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Registration No. 333-248414

Prospectus

12,251,578 shares

Common stock

This is an initial public offering of shares of common stock by Dyne Therapeutics, Inc. We are offering 12,251,578 shares of our common stock. The initial public offering price is $19.00 per share.

Prior to this offering, there has been no public market for our common stock. Our common stock has been approved for listing on the Nasdaq Global Select Market under the symbol "DYN." We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012 and are subject to reduced public company reporting requirements.

	Per share	Total
Initial public offering price	$ 19.00	$232,779,982
Underwriting discounts and commissions(1)	$ 1.33	$ 16,294,599
Proceeds to Dyne Therapeutics, Inc., before expenses	$ 17.67	$216,485,383

(1) See "Underwriting" for a description of all compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to 1,837,736 additional shares of common stock from us at the public offering price, less underwriting discounts and commissions.

Investing in our common stock involves a high degree of risk. See "Risk factors" beginning on page 12 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to purchasers on or about September 21, 2020.

Joint Book-Running Managers

J.P. Morgan **Jefferies** **Piper Sandler** **Stifel**

September 16, 2020

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Neither we nor the underwriters have authorized anyone to provide you with any information other than that contained in this prospectus, any amendment or supplement to this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: we have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

Prospectus summary

This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, including our financial statements and the related notes appearing elsewhere in this prospectus and the information set forth in the sections titled "Risk factors" and "Management's discussion and analysis of financial condition and results of operations" before making an investment decision. As used in this prospectus, unless the context otherwise requires, references to "we," "us," "our" and "Dyne" refer to Dyne Therapeutics, Inc.

Overview

We are building a leading muscle disease company focused on advancing innovative life-transforming therapeutics for patients with genetically driven diseases. We are utilizing our proprietary FORCE platform to overcome the current limitations of muscle tissue delivery and advance modern oligonucleotide therapeutics for muscle diseases. Our proprietary FORCE platform therapeutics consist of an oligonucleotide payload that we rationally design to target the genetic basis of the disease we are seeking to treat, a clinically validated linker and an antigen-binding fragment, or Fab, that we attach to the payload using the linker. With our FORCE platform, we have the flexibility to deploy different types of oligonucleotide payloads with specific mechanisms of action that modify target functions. We leverage this modularity to focus on muscle diseases with high unmet need, with etiologic targets and with clear translational potential from preclinical disease models to well-defined clinical development and regulatory pathways. Using our FORCE platform, we are assembling a broad portfolio of muscle disease therapeutics, including our lead programs in myotonic dystrophy type 1, or DM1, Duchenne muscular dystrophy, or DMD, and facioscapulohumeral dystrophy, or FSHD. In addition, we plan to expand our portfolio through development efforts focused on rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases, including some with larger patient populations. Our programs are currently all in the preclinical stage. We expect to submit investigational new drug, or IND, applications to the U.S. Food and Drug Administration, or FDA, for product candidates in each of our DM1, DMD and FSHD programs between the fourth quarter of 2021 and the fourth quarter of 2022.

Oligonucleotide therapeutics are a genetic medicine modality that, using nucleic acids, specifically aims to correct the function of disease-causing genes by either degrading the target gene or modifying expression of a target protein. While some oligonucleotide therapeutics have been approved, the development of oligonucleotide therapeutics has been limited by challenges in the delivery of the oligonucleotide to the tissue that requires therapy. To overcome these limitations, our FORCE platform utilizes the importance of Transferrin 1 receptor, or TfR1, in muscle biology as the foundation of our novel approach of linking therapeutic payloads to our TfR1-binding Fab to deliver targeted therapeutics for muscle diseases. TfR1, which is highly expressed on the surface of muscle cells, is required for iron transport into muscle cells, and evidence to date suggests that there are no other proteins that can substitute for TfR1 function. We believe our FORCE platform may provide several advantages, including targeted delivery to muscle tissue, extended durability, redosable administration and potent targeting of the genetic basis of disease to stop or reverse disease progression.

Our approach

We have designed our proprietary FORCE platform using our deep knowledge of muscle biology and oligonucleotide therapeutics. Our therapeutics consist of three essential components: a proprietary Fab, a clinically validated linker and an oligonucleotide payload that we attach to our Fab using the linker.

Proprietary Antibody (Fab): Our proprietary Fabs are engineered to bind to TfR1 to enable targeted delivery of nucleic acids and other molecules to skeletal, cardiac and smooth muscle. A Fab is the region of an antibody that binds to antigens. We selected a Fab antibody over monoclonal antibodies, or mAbs, due to its potential significant advantages when targeting TfR1 to enable muscle delivery, including enhanced tissue penetration, increased tolerability due to lower protein load and reduced risk of immune system activation due to the lack of the Fc domain on the Fab. To identify the proprietary Fab we plan to use in our product candidates, we generated and screened proprietary antibodies for selectivity to TfR1 in order to enhance muscle specificity and for binding to TfR1 without interfering with the receptor's function of transporting iron into cells.

Clinically Validated Linker: We have selected the Val-Cit linker as the linker for our FORCE platform based on its clinically validated safety and efficacy in approved products, its serum stability and its endosomal release attributes. Additionally, our linker and conjugation chemistry allow us to optimize the ratio of payload molecules attached to the Fab for each type of payload. We believe that our linker and conjugation chemistry will enable us to rapidly design, produce and screen molecules to enable new muscle disease programs.

Optimized Payload: With our FORCE platform, we have the flexibility to deploy different types of therapeutic payloads with specific mechanisms of action that modify target functions. Using this modularity, we rationally select the therapeutic payload for each program to match the biology of the target, with the aim of addressing the genetic basis of disease and stopping or reversing disease progression.

We have demonstrated proof-of-concept of our FORCE platform in multiple *in vitro* and *in vivo* studies. In murine and non-human primate studies, we have delivered antisense oligonucleotides, or ASOs, and phosphorodiamidate morpholino oligomers, or PMOs, to genetic targets within muscle tissue and observed durable, disease-modifying, functional benefit in preclinical models of disease. For instance, in our DM1 program, we observed almost complete reversal of myotonia after a single dose in the HSA-LR mouse DM1 model and reductions in levels of DMPK in wild-type, or WT, mice for up to 12 weeks after a single dose, and in our DMD program, we observed increased muscle function four weeks after a single dose in the mdx mouse DMD model that mirrored levels of muscle function in a control cohort of healthy, wild-type mice. We believe our FORCE platform may provide several advantages, including targeted delivery to muscle tissue, potent targeting of the genetic basis of disease to stop or reverse disease progression, enhanced tolerability, extended durability, redosable administration, well-established and scalable manufacturing and accelerated and efficient development enabled by use of a single Fab and linker across all of our programs.

Our portfolio

We are building a pipeline of programs to address genetically-driven muscle diseases with high unmet need with etiologic targets. Our initial focus is on DM1, DMD and FSHD with potential pipeline expansion opportunities in additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. We have global commercial rights to all of our programs.

DM1 program overview

Our DM1 program is focused on the development of a potentially disease-modifying treatment for DM1. DM1 is a monogenic, autosomal dominant, progressive disease that affects skeletal, cardiac and smooth muscle, resulting in significant physical, cognitive and behavioral impairments and disability. There are currently no disease-modifying therapies to treat DM1 that are approved or in clinical development. DM1 is caused by an abnormal expansion in a region of the DMPK gene and it is estimated to affect over 40,000 people in the United States and over 74,000 people in Europe. Our program candidates consist of a proprietary TfR1-binding Fab conjugated using our linker to an ASO that is designed to address the genetic basis of DM1 by reducing the levels of mutant DMPK RNA in the nucleus, releasing splicing proteins, allowing normal mRNA processing and translation of normal proteins and potentially stopping or reversing disease. In preclinical studies, we have observed reduction of nuclear foci and correction of splicing in DM1 patient cells, reversal of myotonia after a single dose in a DM1 mouse disease model, durability of response up to 12 weeks in WT mice and enhanced muscle distribution as evidenced by reduced levels of cytoplasmic WT DMPK RNA in non-human primates. We expect to submit an IND to the FDA for a product candidate in our DM1 program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

DMD program overview

Our DMD program is focused on the development of potentially disease-modifying treatments for DMD. DMD is a monogenic, X-linked disease caused by mutations in the gene that encodes for the dystrophin protein. In patients with DMD, mutations in the dystrophin gene lead to certain exons being misread resulting in the loss of function of the dystrophin protein, muscle cell death and progressive loss of muscle function. We estimate that the patient population is approximately 12,000 to 15,000 in the United States and approximately 25,000 in Europe. We are developing program candidates to address the genetic basis of DMD by delivering a PMO to muscle tissue to promote the skipping of specific DMD exons in the nucleus, allowing muscle cells to create a more complete, functional dystrophin protein and potentially stop or reverse disease progression. In *in vitro* and *in vivo* preclinical studies, our PMOs when conjugated to a Fab targeting TfR1 have shown increased exon skipping, increased dystrophin expression, reduced muscle damage and increased muscle function. We are seeking to build a DMD franchise by initially focusing on the development of a therapeutic for patients with mutations amenable to skipping Exon 51, to be followed by the development of therapeutics for patients with mutations amenable to skipping other exons, including Exons 53, 45 and 44. We expect to submit an IND to the FDA for a product candidate in our Exon 51 skipping program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

FSHD program overview

Our FSHD program is focused on the development of a potentially disease-modifying therapy for FSHD. FSHD is an autosomal dominant muscular dystrophy characterized by progressive skeletal muscle loss, resulting in significant physical impairments and disability. FSHD is caused by aberrant expression of the double homeobox 4, or DUX4, gene in muscle tissue, which leads to death of muscle and replacement by fat. There are no approved treatments for FSHD. We estimate the patient population is between 16,000 and 38,000 in the United States and approximately 35,000 in Europe. Our FSHD program candidates consist of our proprietary TfR1-binding Fab conjugated using our linker to an ASO that is designed to address the genetic basis of FSHD by reducing DUX4 expression in muscle tissue. In preclinical studies, we observed that administration of our proprietary ASO conjugated to a Fab targeting TfR1 reduced expression of key DUX4 biomarkers in FSHD

patient myotubes. We expect to submit an IND to the FDA for a product candidate in our FSHD program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Discovery programs overview

We intend to expand our FORCE portfolio by pursuing programs in additional indications, including additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. By rationally selecting therapeutic payloads to conjugate with our proprietary Fab and linker, we plan to develop product candidates to address the genetic basis of additional muscle diseases. We have completed screening and identified potent ASO and siRNA payloads against a number of cardiac and metabolic targets. In addition to our muscle disease portfolio, we believe there is an opportunity to leverage our TfR1 antibody expertise to develop novel antibodies that cross the blood-brain barrier and deliver therapeutics to the central nervous system, or CNS, tissue through systemic intravenous administration.

Our strategy

Our goal is to become the leading muscle disease company by advancing innovative life-transforming therapeutics for genetically driven diseases. To accomplish this, we intend to continue building a team that shares our commitment to patients, to continue to enhance our platform and to advance our pipeline. The key elements of our strategy are to:

- Advance our lead programs in DM1, DMD and FSHD to clinical proof-of-concept and approval to offer meaningful benefit to patients;
- Establish a DMD franchise by expanding our DMD program to reach additional DMD patient populations;
- Expand our pipeline of therapeutics for muscle diseases to fully exploit the potential of our proprietary FORCE platform;
- Selectively enter into strategic collaborations to maximize the value of our pipeline and our proprietary FORCE platform; and
- Build a sustainable leadership position in muscle diseases with a deep connection to patients, caregivers, the research community and physicians.

Our culture and team

We have established a patient-focused culture that drives our shared mission of developing life-transforming therapeutics for patients with serious muscle diseases. Our shared definition of success is simple: we do what we say we are going to do. We keep our commitments to patients, employees and Dyne stakeholders. We endeavor to act with integrity and transparency.

Our management team is led by Joshua Brumm, our President and Chief Executive Officer, who brings over 15 years of leadership experience with life sciences companies; Romesh Subramanian, Ph.D., our Chief Scientific Officer and Founder, who is an expert in nucleic acid, antibody and peptide therapeutic development as well as delivery platforms with 20 years of experience across pharmaceutical and biotechnology companies; Susanna High, our Chief Operating Officer, who has more than two decades of experience leading corporate strategy, portfolio management, business planning and operations for biotechnology companies; and Oxana Beskrovnaya, Ph.D., our Senior Vice President, Head of Research, who has extensive experience in

musculoskeletal and renal research. We have also established scientific and clinical advisory boards comprised of leading experts in the fields of muscle disease drug discovery and development and nucleic acid therapeutics, who share our mission of delivering disease-modifying therapeutics for patients with serious muscle diseases.

Since our inception through August 31, 2020, we have raised $167.7 million from a syndicate of leading investors, including Atlas Venture, Forbion, MPM Capital, Vida Ventures, Surveyor Capital (a Citadel company), RA Capital, Wellington Management, Logos Capital, Franklin Templeton and CureDuchenne Ventures.

Risks associated with our business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the “Risk factors” section of this prospectus. These risks include, but are not limited to, the following:

- Even if this offering is successful, we will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts;
- Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability;
- We are very early in our development efforts. We have not identified any product candidates for IND-enabling studies or clinical development, and as a result it will be many years before we commercialize a product candidate, if ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed;
- We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing any product candidates we determine to develop on a timely basis, if at all;
- Our approach to the discovery and development of product candidates based on our FORCE platform is unproven, and we may not be successful in our efforts to identify, discover or develop potential product candidates;
- The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials;
- If any product candidates we may develop cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval;
- We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research, preclinical and clinical testing, and these third parties may not perform satisfactorily;
- We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do;
- Our rights to develop and commercialize any product candidates are subject and may in the future be subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under current or future intellectual property license agreements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business;

- If we or our licensors are unable to obtain, maintain and defend patent and other intellectual property protection for any product candidates or technology, or if the scope of the patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop or our technology may be adversely affected due to such competition; and
- The COVID-19 pandemic may affect our ability to initiate and complete preclinical studies, delay the initiation of our planned clinical trial or future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, which could negatively impact our operations and our ability to raise additional capital following this offering.

Our corporate information

We were incorporated under the laws of the state of Delaware on December 1, 2017 under the name Dyne Therapeutics, Inc. Our principal executive offices are located at 830 Winter Street, Waltham, Massachusetts 02451 and our telephone number is (781) 786-8230. Our website address is https://www.dyne-tx.com. The information contained on, or accessible through, our website does not constitute part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

Trademarks and tradenames

We own or have rights to trademarks, service marks and trade names that we use in connection with the operation of our business, including our corporate name, logos and website names. The service marks and trademarks that we own include the marks Dyne Therapeutics™ and FORCE™. Other trademarks, service marks and trade names appearing in this prospectus are the property of their respective owners. Solely for convenience, some of the trademarks, service marks and trade names referred to in this prospectus are listed without the ® and ™ symbols, but we will assert, to the fullest extent under applicable law, our rights to our trademarks, service marks and trade names.

Implications of being an emerging growth company and a smaller reporting company

We are an “emerging growth company” as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. As a result, we may take advantage of reduced reporting requirements that are otherwise applicable generally to public companies, including delaying auditor attestation of internal control over financial reporting, exemption from the requirements to hold non-binding advisory votes on executive compensation and golden parachute payments, providing only two years of audited financial statements and related Management’s discussion and analysis of financial condition and results of operations in this prospectus and reduced executive compensation disclosures.

We may remain an emerging growth company until December 31, 2025 or until such earlier time as we have more than $1.07 billion in annual revenue, we become a “large accelerated filer” under SEC rules, or we issue more than $1 billion of non-convertible debt over a three-year period.

We have elected to take advantage of certain of the reduced disclosure obligations in the registration statement of which this prospectus is a part. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be

required if we were not an emerging growth company. In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have elected not to "opt out" of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we can adopt the new or revised standard at the time private companies adopt the new or revised standard and may do so until such time that we either (1) irrevocably elect to "opt out" of such extended transition period or (2) no longer qualify as an emerging growth company.

We are also a "smaller reporting company," meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and have reduced disclosure obligations regarding executive compensation, and, similar to emerging growth companies, if we are a smaller reporting company with less than $100 million in annual revenue, we would not be required to obtain an attestation report on internal control over financial reporting issued by our independent registered public accounting firm.

The offering

Common stock offered by us	12,251,578 shares
Option to purchase additional shares	We have granted the underwriters an option for a period of 30 days from the date of this prospectus to purchase up to 1,837,736 additional shares of our common stock at the initial public offering price less underwriting discounts and commissions.
Common stock to be outstanding immediately after this offering	43,585,221 shares (or 45,422,957 shares if the underwriters exercise their option to purchase additional shares in full).
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $213.7 million (or approximately $246.2 million if the underwriters exercise their option to purchase additional shares in full), after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds to us from this offering, together with our existing cash and cash equivalents, for continued research and development of our programs, including preclinical studies, IND-enabling studies and clinical trials; continued development and enhancement of our proprietary FORCE platform; and for working capital and other general corporate purposes. See "Use of proceeds" for more information.
Risk factors	You should read the "Risk factors" section of this prospectus beginning on page 12 for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Proposed Nasdaq Global Select Market symbol	"DYN"

The number of shares of our common stock to be outstanding after this offering is based on 3,247,268 shares of our common stock outstanding as of August 31, 2020, which includes 453,579 shares of unvested restricted stock subject to forfeiture, and after giving effect to the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 28,086,375 shares of common stock upon the closing of this offering, but excludes:

- 5,201,900 shares of common stock issuable upon exercise of stock options outstanding as of August 31, 2020, under our 2018 Stock Incentive Plan, as amended, or the 2018 Plan, at a weighted-average exercise price of $4.03 per share;
- 1,928,394 shares of common stock available for future issuance as of August 31, 2020 under our 2018 Plan;
- 2,955,746 additional shares of common stock available for future issuance under our 2020 Stock Incentive Plan, or the 2020 Plan, as well as any shares which may be reserved pursuant to provisions in the 2020 Plan that automatically increase the number of shares of common stock reserved for issuance under the 2020

Plan, of which we have granted stock options to purchase an aggregate of 1,151,454 shares of common stock, at an exercise price per share equal to the initial public offering price, and restricted stock units for an aggregate of 375,137 shares of common stock to certain of our employees, in connection with this offering; and

- 488,414 additional shares of common stock available for future issuance under our 2020 Employee Stock Purchase Plan, or the 2020 ESPP, as well as any shares which may be reserved pursuant to provisions in the 2020 ESPP that automatically increase the number of shares of common stock reserved for issuance under the 2020 ESPP.

Unless otherwise indicated, all information in this prospectus reflects and assumes:

- a one-for-3.3169 reverse stock split of our common stock, and a proportionate adjustment in the ratio at which our preferred stock is convertible into our common stock, that became effective on September 9, 2020;
- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 28,086,375 shares of our common stock upon the closing of the offering;
- no exercise of the outstanding options described above;
- no exercise by the underwriters of their option to purchase additional shares of our common stock; and
- the filing and effectiveness of our restated certificate of incorporation and the adoption of our amended and restated bylaws upon the closing of this offering.

Summary financial data

The following tables set forth a summary of our historical financial data as of, and for, the periods ended on the dates indicated. We have derived the following statement of operations data for the years ended December 31, 2018 and 2019 from our audited financial statements appearing elsewhere in this prospectus. The statement of operations data for the six months ended June 30, 2019 and 2020 and the balance sheet data as of June 30, 2020 have been derived from our unaudited condensed financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as our audited financial statements, and, in the opinion of management, reflect all adjustments, consisting only of normal, recurring adjustments, necessary for the fair presentation of those unaudited interim financial statements. You should read the following summary financial data, together with our financial statements and the related notes appearing elsewhere in this prospectus and the "Selected financial data" and "Management's discussion and analysis of financial condition and results of operations" sections of this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future, and the results for the six months ended June 30, 2020 are not necessarily indicative of results to be expected for the full year ending December 31, 2020 or any other period.

	Year ended December 31,		Six months ended June 30,	
(in thousands, except share and per share data)	**2018**	**2019**	**2019**	**2020**
Statement of operations data:				
Operating expenses:				
Research and development	$ 4,278	$ 11,040	$ 3,799	$ 13,423
General and administrative	517	2,786	927	3,105
Total operating expenses	4,795	13,826	4,726	16,528
Loss from operations	(4,795)	(13,826)	(4,726)	(16,528)
Other income (expense):				
Interest income	5	290	113	24
Interest expense	—	—	—	(184)
Change in fair value of preferred stock tranche obligations	(21)	(1,323)	1,029	—
Change in success fee obligation	—	—	—	(180)
Total other (expense) income, net	(16)	(1,033)	1,142	(340)
Net loss	$ (4,811)	$ (14,859)	$ (3,584)	$ (16,868)
Net loss per share—basic and diluted(1)	$ (10.15)	$ (6.08)	$ (1.52)	$ (6.31)
Weighted average number of common shares used in computing net loss per share—basic and diluted(1)	474,118	2,442,872	2,355,066	2,675,260
Pro forma net loss per share—basic and diluted(1)		$ (1.31)		$ (1.32)
Pro forma weighted average number of common shares used in computing pro forma net loss per share—basic and diluted(1)		10,324,875		12,818,114

(1) See Note 12 to our financial statements appearing elsewhere in this prospectus for details on the calculation of basic and diluted net loss per share and the calculation of basic and diluted pro forma net loss per share. This calculation excludes the sale by us of 17,500,000 shares of Series A preferred stock in July 2020 and 41,159,724 shares of Series B preferred stock in August 2020, which will convert into an aggregate of 17,685,105 shares of our common stock upon the closing of this offering.

	As of June 30, 2020		
(in thousands)	Actual	Pro forma(1)	Pro forma as adjusted(2)
Balance sheet data:			
Cash and cash equivalents	$ 11,672	$ 144,872	$ 358,107
Working capital(3)	7,747	140,947	354,182
Total assets	13,571	146,771	360,006
Long-term debt—net of unamortized debt discount	9,949	9,949	9,949
Convertible preferred stock	29,401	—	—
Additional paid-in capital	6,493	169,092	382,776
Total stockholders' equity (deficit)	(719)	132,481	345,896

(1) The pro forma balance sheet data give effect to (i) the sale by us of 17,500,000 shares of Series A preferred stock in July 2020 for gross proceeds of $17.5 million, (ii) the sale by us of 41,159,724 shares of Series B preferred stock in August 2020 for gross proceeds of $115.7 million and (iii) the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 28,086,375 shares of common stock upon the closing of this offering.

(2) The pro forma as adjusted balance sheet data give further effect to (i) our issuance and sale of 12,251,578 shares of our common stock in this offering at the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us and (ii) our payment of a success fee in the amount of $0.5 million to our lender under the terms of a loan agreement.

(3) We define working capital as current assets less current liabilities.

Risk factors

Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this prospectus, including our financial statements and the related notes appearing elsewhere in prospectus, before deciding to invest in our common stock. The risks described below are not the only risks facing our company. The occurrence of any of the following risks, or of additional risks and uncertainties not presently known to us or that we currently believe to be immaterial, could cause our business, prospects, operating results and financial condition to suffer materially. In such event, the trading price of our common stock could decline, and you might lose all or part of your investment.

Risks related to our financial position and need for additional capital

We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses for the foreseeable future.

Since inception, we have incurred significant operating losses. Our net losses were $14.9 million for the year ended December 31, 2019 and $16.9 million for the six months ended June 30, 2020. As of June 30, 2020, we had an accumulated deficit of $36.6 million. To date, we have financed our operations with the proceeds raised from the sale of equity securities and borrowings under a loan and security agreement. We have devoted substantially all of our financial resources and efforts to research and development. We are still in the early stages of development of our programs, have not identified a product candidate for preclinical or clinical development and have not commenced or completed clinical development. We expect to continue to incur significant expenses and operating losses for the foreseeable future. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially if and as we:

- continue our current research programs and conduct additional research programs;
- advance any product candidates we identify through our research programs into IND-enabling studies and clinical trials;
- expand the capabilities of our proprietary FORCE platform;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- establish manufacturing sources for any product candidate we may develop, including the Fab antibody, Val-cit linker and therapeutic payload that will comprise the product candidate, and secure supply chain capacity to provide sufficient quantities for preclinical and clinical development and commercial supply;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development and future commercialization efforts, as well as to support our operations as a public company.

Even if we obtain regulatory approval of, and are successful in commercializing, one or more of any product candidates we may develop, we will continue to incur substantial research and development and other costs to

develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue.

We have never generated revenue from product sales and may never achieve or maintain profitability.

We have not identified any product candidates for IND-enabling studies or clinical development or initiated clinical development of any product candidate and expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must succeed in developing, obtaining the necessary regulatory approvals for and eventually commercializing a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including:

- identifying product candidates and completing preclinical and clinical development of any product candidates we may identify;
- obtaining regulatory approval for any product candidates we may develop;
- manufacturing, marketing and selling any products for which we may obtain regulatory approval;
- achieving market acceptance of any products for which we obtain regulatory approval as a viable treatment option; and
- satisfying any post-marketing requirements.

We are only in the preliminary stages of most of these activities. We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. We are currently only in the preclinical stage of our research programs. Because of the numerous risks and uncertainties associated with product development, we are unable to accurately estimate or know the nature, timing or costs of the efforts that will be necessary to complete the preclinical and clinical development and commercialization of any product candidate we may develop or when, or if, we will be able to generate revenues or achieve profitability.

If we are successful in obtaining regulatory approval to market one or more of our products, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to obtain coverage and reimbursement, and whether we own the commercial rights for that territory. If the number of our addressable patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the treatment population is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable could impair our ability to raise capital, maintain our research and development efforts, expand our business or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

Even if this offering is successful, we will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we identify, continue the research and development of, initiate preclinical testing and clinical trials of, arrange for the manufacturing of, and potentially seek marketing approval for any product candidates we may develop. In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur

significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, following the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed, on attractive terms or at all, we may be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

As of June 30, 2020, we had cash and cash equivalents of $11.7 million. In July 2020, we raised an additional $17.5 million in gross proceeds from the sale of 17,500,000 shares of our Series A preferred stock in the third tranche of our Series A preferred stock financing, and in August 2020, we raised an additional $115.7 million in gross proceeds from the sale of 41,159,724 shares of our Series B preferred stock. We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements and debt service obligations into the second half of 2023. However, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. As a result, we could deplete our capital resources sooner than we currently expect and could be forced to seek additional funding sooner than planned.

Our future capital requirements will depend on many factors, including:

- the identification of additional research programs and product candidates;
- the scope, progress, costs and results of preclinical and clinical development for any product candidates we may develop;
- the scope, costs, timing and outcome of regulatory review of any product candidates we may develop;
- the cost and timing of manufacturing activities;
- the costs and scope of the continued development of our FORCE platform;
- the costs and timing of preparing, filing and prosecuting applications for patents, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims, including claims of infringement, misappropriation or other violations of third-party intellectual property;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidates we may develop for which we receive marketing approval;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of product candidates we may develop for which we receive marketing approval;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully identify and develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, may not be sufficient to sustain our operations. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our operations. We cannot be certain that additional funding will be available on acceptable terms, when needed or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts, when needed or on terms acceptable to us, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate we may develop, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations. We could be required to seek collaborators for product candidates we may develop at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates we may develop in markets where we otherwise would seek to pursue development or commercialization ourselves. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

The report of our independent registered public accounting firm included a "going concern" explanatory paragraph.

The report of our independent registered public accounting firm on our financial statements as of and for the year ended December 31, 2019 included an explanatory paragraph indicating that there is substantial doubt about our ability to continue as a going concern. If we are unable to raise sufficient capital in this offering or otherwise as and when needed, our business, financial condition and results of operations will be materially and adversely affected, and we will need to significantly modify our operational plans to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets, and the values we receive for our assets in liquidation or dissolution could be significantly lower than the values reflected in our financial statements. The inclusion of a going concern explanatory paragraph by our independent registered public accounting firm, our lack of cash resources and our potential inability to continue as a going concern may materially adversely affect our share price and our ability to raise new capital, enter into critical contractual relations with third parties and otherwise execute our development strategy.

Raising additional capital may cause dilution to our stockholders, including purchasers of our common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Our loan and security agreement with Pacific Western Bank involves, and any debt financing or preferred equity financing, if available, may involve, agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed or on terms acceptable to us, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We commenced operations in 2017, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, conducting research activities and filing and prosecuting patent applications. All of our research programs are still in the research or preclinical stage of development, and their risk of failure is high. We have not yet demonstrated our ability to initiate or complete any clinical trials, obtain marketing approvals, manufacture product for clinical trials or on a commercial scale or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing products.

Our limited operating history may make it difficult to evaluate our technology and industry and predict our future performance. Our limited history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer.

In addition, as our business grows, we may encounter unforeseen expenses, restrictions, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research focus to a company capable of conducting development activities and then to a company supporting commercial activities. We may not be successful in such transitions.

Our indebtedness may limit our flexibility in operating our business and could have a material adverse effect on our business, prospects, financial condition and results of operations.

As of June 30, 2020, we had $10.0 million of principal balance outstanding under a loan and security agreement with Pacific Western Bank. Interest on the outstanding loan balance will accrue at a variable annual rate equal to the greater of (i) the prime rate plus 0.25% and (ii) 5.00%. We are required to make interest-only payments on the loan on a monthly basis through August 2021. Subsequent to the interest-only periods, we are required to make equal monthly payments of principal plus interest until the term loan matures in February 2024. In the event of a liquidation event, which includes this offering, we will be required to pay a success fee of $0.5 million. The amounts due under the loan agreement are secured by substantially all of our assets, excluding intellectual property rights.

In order to service this indebtedness and any additional indebtedness we may incur in the future, we will need to generate cash. Our ability to generate cash is subject, in part, to our ability to successfully execute our business strategy, as well as general economic, financial, competitive, regulatory and other factors beyond our control. Our business may not be able to generate sufficient cash flow and future borrowings or other financings may not be available to us in an amount sufficient to enable us to service our indebtedness and fund our other liquidity needs. To the extent we are required to use cash from operations or the proceeds of any future financing to service our indebtedness instead of funding working capital, capital expenditures or other general corporate purposes, we will be less able to plan for, or react to, changes in our business, industry and in the economy generally. This could place us at a competitive disadvantage compared to our competitors that have less indebtedness.

In addition, the loan agreement contains, and any agreements evidencing or governing other future indebtedness may contain, certain covenants that limit our ability to engage in certain transactions that may be

in our long-term best interests. Subject to certain limited exceptions, these covenants limit our ability to, among other things:

- sell, lease, convey, transfer, license or otherwise deal with all or any material part of our property, assets or undertaking;
- incur or allow to remain outstanding any indebtedness;
- create or permit to subsist any liens; and
- declare and/or make or agree to make any distribution by way of dividend or otherwise.

Our ability to comply with these covenants may be affected by events and factors beyond our control. In the event that we breach one or more covenants, our lender may choose to declare an event of default and require that we immediately repay all amounts outstanding, terminate any commitment to extend further credit and foreclose on the collateral granted to it to collateralize such indebtedness. The occurrence of any of these events could have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.

Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be subject to limitations.

We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize our net operating losses, or NOLs, or research and development tax credit carryforwards. As of December 31, 2019, we had federal NOL carryforwards of $17.7 million and state NOL carryforwards of $17.7 million.

In general, under Sections 382 and 383 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an “ownership change,” generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year period, is subject to limitations on its ability to utilize its pre-change NOLs and pre-change research and development tax credit carryforwards to offset post-change income or taxes. We have not conducted a study to assess whether any such ownership changes have occurred. We may have experienced such ownership changes in the past and may experience such ownership changes in the future as a result of this offering or of subsequent changes in our stock ownership (which may be outside our control). As a result, if, and to the extent that, we earn net taxable income, our ability to use our NOL carryforwards and research and development tax credit carryforwards to offset such taxable income may be subject to limitations.

There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing NOLs could expire or otherwise become unavailable to offset future income tax liabilities. As described below in “Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition,” legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, or the Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

Risks related to discovery and development

We are very early in our development efforts. We have not identified any product candidates for IND-enabling studies or clinical development, and as a result it will be years before we commercialize a product candidate, if

ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed.

We are very early in our development efforts and have invested our research efforts to date in developing our platform. We have a portfolio of programs that are in early stages of preclinical development and have not yet identified any product candidates for IND-enabling studies or clinical development. We may never identify any product candidates or advance any product candidates to clinical-stage development. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

Commencing clinical trials in the United States is subject to acceptance by the U.S. Food and Drug Administration, or FDA, of an investigational new drug application, or IND, and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In the event that the FDA requires us to complete additional preclinical studies or we are required to satisfy other FDA requests prior to commencing clinical trials, the start of our first clinical trials may be delayed. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to clinical trial applications in other countries, including countries in the European Union.

Commercialization of any product candidates we may develop will require preclinical and clinical development; regulatory and marketing approval in multiple jurisdictions, including by the FDA and the European Medicines Agency, or EMA; manufacturing supply, capacity and expertise; a commercial organization; and significant marketing efforts. The success of product candidates we may identify and develop will depend on many factors, including the following:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable;
- effective INDs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including under the FDA's current Good Clinical Practices, or cGCPs, current Good Laboratory Practices, or cGLPs, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our future clinical trials that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations;
- receipt of marketing approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for any product candidates we may develop;
- commercial launch of any product candidates we may develop, if approved, whether alone or in collaboration with others;

- acceptance of the benefits and use of our product candidates we may develop, including method of administration, if and when approved, by patients, the medical community and third-party payers;
- effective competition with other therapies;
- maintenance of a continued acceptable safety, tolerability and efficacy profile of any product candidates we may develop following approval; and
- establishment and maintenance of healthcare coverage and adequate reimbursement by payers.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may develop, which would materially harm our business. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing any product candidates we determine to develop on a timely basis, if at all.

The risk of failure in developing product candidates is high. It is impossible to predict when or if any product candidate would prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of product candidates in humans. We have not yet conducted a clinical trial of any product candidate. Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses. Even if the clinical trials are successful, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application.

Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our INDs and other regulatory filings. We cannot be certain of the timely identification of a product candidate or the completion or outcome of our preclinical testing and studies and cannot predict whether the FDA will accept our proposed clinical programs or whether the outcome of our preclinical testing and studies will ultimately support the further development of any product candidates. Conducting preclinical testing is a lengthy, time-consuming and expensive process. The length of time may vary substantially according to the type, complexity and novelty of the program, and often can be several years or more per program. As a result, we cannot be sure that we will be able to submit INDs for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs will result in the FDA allowing clinical trials to begin.

Furthermore, product candidates are subject to continued preclinical safety studies, which may be conducted concurrently with our clinical testing. The outcomes of these safety studies may delay the launch of or enrollment in future clinical trials and could impact our ability to continue to conduct our clinical trials.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in trial design, dose selection issues, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CLROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board, or IRB, or independent ethics committee approval, or the equivalent review groups for sites outside the United States, at each clinical trial site;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- negative or inconclusive results observed in clinical trials, including failure to demonstrate statistical significance, which could lead us, or cause regulators to require us, to conduct additional clinical trials or abandon product development programs;
- failure by us, any CLROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's cGCPs;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of any product candidates we may develop to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- failure of our third-party contractors to comply with regulatory requirements or to meet their contractual obligations to us in a timely manner, or at all;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events associated with a product candidate in development by another company, which are viewed to outweigh its potential benefits, and which may negatively impact the perception of our product due to a similarity in technology or approach;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the legal or regulatory regimes domestically or internationally related to patient rights and privacy; or
- lack of adequate funding to continue the clinical trial.

Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and

royalties. In addition, if we make manufacturing or formulation changes to any product candidates we may develop, we may need to conduct additional studies or trials to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize any product candidates we may develop and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of future clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with any product candidates we may develop, we may:

- be delayed in obtaining marketing approval for product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

In particular, each of the conditions for which we plan to develop product candidates are rare genetic diseases with limited patient pools from which to draw for clinical trials. Further, because it can be difficult to diagnose these diseases in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our studies. The eligibility criteria of our clinical trials will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. The treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies.

Our approach to the discovery and development of product candidates based on our FORCE platform is unproven, and we may not be successful in our efforts to identify, discover or develop potential product candidates.

The success of our business depends upon our ability to identify, develop and commercialize products based on our proprietary FORCE platform. Our therapeutics consist of three essential components: a proprietary Fab, a clinically validated linker and an oligonucleotide payload that we attach to our Fab using the linker. The Fab is engineered to bind to TfR1 to enable targeted delivery of nucleic acids and other molecules to skeletal, cardiac and smooth muscle.

All of our product development programs are still in the research or preclinical stage of development and our approach to treating muscle disease is unproven. Our research programs may fail to identify potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates and our potential product candidates may be shown to have harmful side effects in preclinical *in vitro* experiments or animal model studies. In addition, our potential product

candidates may not show promising signals of therapeutic effect in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable or unlikely to receive marketing approval. Further, because all of our development programs are based on our FORCE platform, adverse developments with respect to one of our programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other programs.

In addition, we have not identified any product candidates for IND-enabling studies or clinical development or successfully developed any product candidate and our ability to identify and develop a product candidate may never materialize. The process by which we identify and disclose product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors. In addition:

- we may not be able to assemble sufficient resources to acquire or discover product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;
- potential product candidates we develop may nevertheless be covered by third parties' patents or other intellectual property rights;
- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate may be too complex and difficult to navigate successfully or economically.

If we are unable to identify and discover suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.

We are in the early stage of research in the development of our platform and programs and have not identified any product candidates or conducted any IND-enabling studies or any clinical trials. As a result, our belief in the capabilities of our platform is based on early research and preclinical studies. However, the results of preclinical studies may not be predictive of the results of later preclinical studies or clinical trials, and the results of any early-stage clinical trials may not be predictive of the results of later clinical trials. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are completed. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. Our future clinical trials may not ultimately be successful or support further clinical development of any product candidates we may develop. There is a high failure rate for product candidates proceeding through clinical trials. A number of

companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving encouraging results in earlier studies. Any such setbacks in our clinical development could materially harm our business and results of operations.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our ability to complete clinical trials may be adversely impacted.

Identifying and qualifying patients to participate in clinical trials of any product candidates we may develop is critical to our success. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- perceived risks and benefits of novel unproven approaches;
- size of the patient population, in particular for rare diseases such as the diseases on which we are initially focused, and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease or disorder under investigation;
- proximity and availability of clinical trial sites for prospective patients;
- ability to obtain and maintain patient consent;
- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Our inability to enroll a sufficient number of patients for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing. Furthermore, we rely on and expect to continue to rely on contract research organizations, or CROs, CLROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and we will have limited influence over their performance.

Even if we are able to enroll a sufficient number of patients for our clinical trials, we may have difficulty maintaining patients in our clinical trials. Many of the patients who end up receiving placebo may perceive that they are not receiving the product candidate being tested, and they may decide to withdraw from our clinical trials to pursue other alternative therapies rather than continue the trial with the perception that they are receiving placebo. If we have difficulty enrolling or maintaining a sufficient number of patients to conduct our clinical trials, we may need to delay, limit or terminate clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

If any product candidates we may develop cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

We have not evaluated any product candidates in human clinical trials. It is impossible to predict when or if any product candidates we may develop will prove safe in humans. There can be no assurance that our technologies will not cause undesirable side effects.

Although other oligonucleotide therapeutics have received regulatory approval, our approach, which combines oligonucleotides with a Fab, is a novel approach to oligonucleotide therapy. As a result, there is uncertainty as to the safety profile of product candidates we may develop compared to more well-established classes of therapies, or oligonucleotide therapeutics on their own. Moreover, there have been only a limited number of clinical trials involving the use of conjugated oligonucleotide therapeutics and none involving the proprietary technology used in our FORCE platform.

If any product candidates we develop are associated with serious adverse events, undesirable side effects or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. Many product candidates that initially showed promise in early-stage testing have later been found to cause side effects that prevented further clinical development of the product candidates.

If in the future we are unable to demonstrate that such side effects were caused by factors others than our product candidates, the FDA, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates for any or all targeted indications. Even if we are able to demonstrate that any future serious adverse events are not product-related and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any product candidate, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

We may expend our limited resources to pursue a particular program, product candidate or indication and fail to capitalize on programs, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and expect to focus on product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential, or we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

Clinical trial and product liability lawsuits against us could divert our resources, could cause us to incur substantial liabilities and could limit commercialization of any product candidates we may develop.

We will face an inherent risk of clinical trial and product liability exposure related to the testing of any product candidates we may develop in clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no product candidates in clinical trials or that have been approved for commercial sale, the future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any product candidates we may develop.

We will need to increase our insurance coverage if we commence clinical trials or if we commence commercialization of any product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

Risks related to our dependence on third parties

We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our product manufacturing, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to many of these items, including contract manufacturing organizations, or CMOs, for the manufacturing of any product candidates we test in preclinical or clinical development, as well as CROs for the conduct of our animal testing and research and CLROs for the conduct of our planned clinical trials. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical trials are conducted in

accordance with the study plan and protocols. Moreover, the FDA requires us to comply with cGCPs for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions. If we or any of our CLROs or other third parties, including trial sites, fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA, EMA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials complies with cGCP regulations. In addition, our clinical trials must be conducted with product produced under conditions that comply with the FDA's current Good Manufacturing Practices. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the regulatory approval process.

Although we intend to design the clinical trials for any product candidates we may develop, CLROs will conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less direct control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. In addition, any third parties conducting our clinical trials will not be our employees, and except for remedies available to us under our agreements with such third parties, we cannot control whether or not they devote sufficient time and resources to our clinical programs. If the CROs, CLROs and other third parties do not perform preclinical studies and future clinical trials in a satisfactory manner, if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, or if they breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of any product candidates we may develop may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CROs, CLROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

If third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical studies and clinical trials required to support future IND submissions and approval of any product candidates we may develop.

We currently depend on a small number of third-party suppliers for the manufacture of the Fabs, linker and oligonucleotide payloads that we are evaluating in our research programs. The loss of these or future third-party suppliers, or their inability provide us with sufficient supply, could harm our business.

We do not own or operate manufacturing facilities and have no current plans to develop our own clinical or commercial-scale manufacturing capabilities. We rely on a small number of third-party suppliers for the manufacture of the Fabs, linker and oligonucleotide payloads that we are evaluating in our research programs. We expect to continue to depend on third-party suppliers for the manufacture of any product candidates we advance into preclinical and clinical development, as well as for commercial manufacture if those product candidates receive marketing approval. The facilities used by third-party manufacturers to manufacture our product candidates must be approved by the FDA and any comparable foreign regulatory authority pursuant to inspections that will be conducted after we submit an NDA to the FDA or any comparable filing to a foreign regulatory authority. We do not control the manufacturing process of, and are completely dependent on, third-party manufacturers for compliance with cGMP requirements for manufacture of products. If these third-party manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or any comparable foreign regulatory authority, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities.

Any product candidate we may develop will consist of a proprietary Fab conjugated with the oligonucleotide therapy. Our Fab is manufactured by starting with cells which are stored in a cell bank. If we lose multiple cell banks, our manufacturing will be adversely impacted by the need to replace the cell banks.

In addition, we have no control over the ability of third-party manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA or any comparable foreign regulatory authority does not approve these facilities for the manufacture of any product candidates we may develop or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain regulatory approval for or market any product candidates we may develop, if approved. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our product candidates.

We may also seek to eventually establish our own manufacturing facility for the long-term commercial supply of any product candidates we may develop and which receive regulatory approval. If we determine to establish our own manufacturing facility and manufacture our products on our own, we will need to obtain the resources and expertise in order to build such manufacturing capabilities and to conduct such manufacturing operations. In addition, our conduct of such manufacturing operations will be subject to the extensive regulations and operational risks to which our third-party suppliers are subject. If we are not successful in building these capabilities or complying with the regulations or otherwise operating our manufacturing function, our commercial supply could be disrupted and our business could be materially harmed.

Our or a third party's failure to execute on our manufacturing requirements on commercially reasonable terms and in compliance with cGMP could adversely affect our business in a number of ways, including:

- an inability to initiate preclinical studies or clinical trials of product candidates;
- delays in submitting regulatory applications, or receiving marketing approvals, for product candidates;
- subjecting third-party manufacturing facilities or our manufacturing facilities to additional inspections by regulatory authorities;

- requirements to cease development or to recall batches of product candidates; and
- in the event of approval to market and commercialize any product, an inability to meet commercial demands for the product.

We are party to manufacturing agreements with a number of third-party manufacturers. We may be unable to maintain these agreements or establish any additional agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to maintain or establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- failure of third-party manufacturers to comply with regulatory requirements and maintain quality assurance;
- breach of the manufacturing agreement by the third party;
- failure to manufacture according to our specifications;
- failure to manufacture according to our schedule or at all;
- misappropriation of our proprietary information, including our trade secrets and know-how; and
- termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

We may compete with third parties for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

We do not currently have arrangements in place for redundant supply or a second source for all required raw materials. If our existing or future third-party manufacturers cannot perform as agreed, we may be required to replace such manufacturers and we may be unable to replace them on a timely basis or at all.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in supply. An alternative manufacturer would need to be qualified through a biologics license application, or BLA, supplement which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

Our current and anticipated future dependence upon third parties for the manufacture of any product candidates we develop may adversely affect our development programs and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

We may from time to time be dependent on single-source suppliers for some of the components and materials used in the product candidates we may develop.

We may from time to time depend on single-source suppliers for some of the components and materials used in any product candidates we may develop. For instance, we currently use a single supplier for each of our Fab, linker and payloads. We cannot ensure that these suppliers or service providers will remain in business, have sufficient capacity or supply to meet our needs or that they will not be purchased by one of our competitors or another company that is not interested in continuing to work with us. Our use of single-source suppliers of raw materials, components, key processes and finished goods could expose us to several risks, including disruptions

in supply, price increases or late deliveries. There are, in general, relatively few alternative sources of supply for substitute components. These vendors may be unable or unwilling to meet our future demands for our clinical trials or commercial sale. Establishing additional or replacement suppliers for these components, materials and processes could take a substantial amount of time and it may be difficult to establish replacement suppliers who meet regulatory requirements. Any disruption in supply from any single-source supplier or service provider could lead to supply delays or interruptions which would damage our business, financial condition, results of operations and prospects.

If we have to switch to a replacement supplier, the manufacture and delivery of any product candidates we may develop could be interrupted for an extended period, which could adversely affect our business. Establishing additional or replacement suppliers, if required, may not be accomplished quickly. If we are able to find a replacement supplier, the replacement supplier would need to be qualified and may require additional regulatory authority approval, which could result in further delay. While we seek to maintain adequate inventory of the single source components and materials used in our products, any interruption or delay in the supply of components or materials, or our inability to obtain components or materials from alternate sources at acceptable prices in a timely manner, could impair our ability to meet the demand for our investigational medicines.

We may enter into collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.

We may seek third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop pose numerous risks to us, including the following:

- collaborators would have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay programs, preclinical studies or clinical trials, provide insufficient funding for programs, preclinical studies or clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with any product candidates we may develop if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- collaborators may be acquired by a third party having competitive products or different priorities, causing the emphasis on our product development or commercialization program under such collaboration to be delayed, diminished or terminated;
- collaborators with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of any product candidates we may develop or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under certain circumstances, including if we undergo a change of control;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates we may develop; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this prospectus apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

If conflicts arise between us and our potential collaborators, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.

If conflicts arise between us and our potential collaborators, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Our collaborators may develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop that are the subject of these collaborations with us. Competing products, either developed by the collaborators or to which the collaborators have rights, may result in the withdrawal of support for our product candidates.

Some of our future collaborators could also become our competitors. Our collaborators could develop competing products, preclude us from entering into collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, fail to devote sufficient resources to the development and commercialization of products, or merge with or be acquired by a third party who may do any of these things. Any of these developments could harm our product development efforts.

If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, the EMA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities, or increase our own expenditures on the development of the product candidate.

We are dependent on third-party vendors to provide certain licenses, products and services and our business and operations, including clinical trials, could be disrupted by any problems with our significant third-party vendors.

We engage a number of third-party suppliers and service providers to supply critical goods and services, such as contract research services, contract manufacturing services and IT services. Disruptions to the business, financial stability or operations of these suppliers and service providers, including due to strikes, labor disputes or other disruptions to the workforce, for instance, if, as a result of COVID-19, employees are not able to come to work, or to their willingness and ability to produce or deliver such products or provide such services in a manner that satisfies the requirements put forth by the authorities, or in a manner that satisfies our own requirements, could affect our ability to develop and market our future product candidates on a timely basis. If these suppliers and service providers were unable or unwilling to continue to provide their products or services in the manner expected, or at all, we could encounter difficulty finding alternative suppliers. Even if we are able to secure appropriate alternative suppliers in a timely manner, costs for such products or services could increase significantly. Any of these events could adversely affect our results of operations and our business.

Risks related to commercialization

We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to any product candidates that we may develop from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of many of the disorders for which we are conducting research programs. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render any product candidates that we may develop obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

We expect to face competition from existing products and product candidates in development for each of our programs. There are currently no approved therapies to treat the underlying cause of DM1. Product candidates currently in development to treat DM1 include: tideglusib, a GSK3-ß inhibitor in late-stage clinical development by AMO Pharma Ltd. for the congenital phenotype of DM1; AT466, which is an AAV-antisense candidate in preclinical development by Audentes Therapeutics, Inc.; an antibody linked siRNA in preclinical development by Avidity Biosciences, Inc.; gene editing treatments in preclinical development by Vertex Pharmaceuticals, Inc., or Vertex; an RNA-targeting gene therapy in preclinical development by Locana, Inc.; and small molecules interacting with RNA in preclinical development by Expansion Therapeutics, Inc.

Currently, patients with DMD are treated with corticosteroids to manage the inflammatory component of the disease. EMFLAZA (deflazacort) is an FDA-approved corticosteroid marketed by PTC Therapeutics, Inc., or PTC. In addition, there are three FDA-approved exon skipping drugs: EXONDYS 51 (eteplirsen) and VYONDYS 53 (golodirsen), which are naked PMOs approved for the treatment of DMD patients amenable to Exon 51 and Exon 53 skipping, respectively, and are marketed by Sarepta Therapeutics, Inc., or Sarepta, and VILTEPSO (vitolarsen), a naked PMO approved for the treatment of DMD patients amenable to Exon 53 skipping, which is marketed by Nippon Shinyaku Co. Ltd. Companies focused on developing treatments for DMD that target dystrophin mechanisms, as does our DMD program, include Sarepta with SRP-5051, a peptide-linked PMO currently being evaluated in a Phase 2 clinical trial for patients amenable to Exon 51 skipping, PTC with ataluren, a small molecule targeting nonsense mutations in a Phase 3 clinical trial, and Avidity Biosciences, Inc., which is in preclinical development with an antibody oligonucleotide conjugate that targets dystrophin production. In addition, several companies are developing gene therapies to treat DMD, including Milo Biotechnology (AAV1-FS344), Pfizer Inc. (PF-06939926), Sarepta (SRP-9001 and Galgt2 gene therapy program), and Solid Biosciences Inc. (SGT-001). Gene editing treatments that are in preclinical development are also being pursued by Vertex and Sarepta. We are also aware of several companies targeting non-dystrophin mechanisms for the treatment of DMD.

There are currently no therapies to treat the underlying cause of FSHD. Products currently in development to treat FSHD include: creatine monohydrate, a supplement that enhances muscle performance, which is being evaluated in a Phase 2 clinical trial by Murdoch Children's Research Institute, and losmapimod, a p38 MAPK inhibitor that may modulate DUX4 expression, which is being evaluated in a Phase 2 clinical trial by Fulcrum Therapeutics Inc.

We will also compete more generally with other companies developing alternative scientific and technological approaches, including other companies working to develop conjugates with oligonucleotides for extra-hepatic delivery, including Alnylam Pharmaceuticals, Aro Biotherapeutics, Arrowhead Therapeutics, Avidity Biosciences, Dicerna Pharmaceuticals, Inc., Ionis Pharmaceuticals and Sarepta, as well as gene therapy and gene editing approaches.

Many of the companies against which we compete or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Accordingly, our competitors may be more successful than us in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining approval for treatments and achieving widespread market acceptance, rendering our treatments obsolete or non-competitive.

Additionally, mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If we successfully obtain approval for any product candidate, we will face competition based on many different factors, including the safety and effectiveness of our products, the ease with which our products can be administered and the extent to which patients accept relatively new routes of administration, the timing and scope of regulatory approvals for these products, the availability and cost of manufacturing, marketing and sales capabilities, price, reimbursement coverage and patent position. Competing products could present superior treatment alternatives, including by being more effective, safer, more convenient, less expensive or marketed and sold more effectively than any products we may develop. Competitive products or technological approaches may make any products we develop, or our FORCE platform, obsolete or noncompetitive before we recover the expense of developing and commercializing our product candidates. If we are unable to compete effectively, our opportunity to generate revenue from the sale of our products we may develop, if approved, could be adversely affected.

Even if any product candidate that we may develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payers and others in the medical community necessary for commercial success.

If any product candidate we may develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payers and others in the medical community. Sales of medical products depend in part on the willingness of physicians to prescribe the treatment, which is likely to be based on a determination by these physicians that the products are safe, therapeutically effective and cost-effective. In addition, the inclusion or exclusion of products from treatment guidelines established by various physician groups and the viewpoints of influential physicians can affect the willingness of other physicians to prescribe the treatment. We cannot predict whether physicians, physicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that our product is safe, therapeutically effective and cost-effective as compared with competing treatments. Efforts to educate the

medical community and third-party payers on the benefits of any product candidates we may develop may require significant resources and may not be successful. If any product candidates we may develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential advantages and limitations compared to alternative treatments;
- the effectiveness of sales and marketing efforts;
- the cost of treatment in relation to alternative treatments;
- the clinical indications for which the product is approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

If the market opportunities for any product candidates we develop are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because the target patient populations of our programs are small, and the addressable patient population even smaller, we must be able to successfully identify patients and capture a significant market share to achieve profitability and growth.

We focus our research and product development on treatments for rare diseases. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with any product candidates we may develop, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations or market research that we conducted, and may prove to be incorrect or contain errors. New studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

Our target patient populations are relatively small, and there is currently no standard of care treatment directed at some of our target indications, such as FSHD. As a result, the pricing and reimbursement of any

product candidates we may develop, if approved, is uncertain, but must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell product candidates will be adversely affected.

The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our future product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

The initial target platforms in our pipeline are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payers, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payers will be essential for most patients to be able to afford these treatments. Accordingly, sales of our future product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payers. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payers tend to follow CMS to a substantial degree. However, one payer's determination to provide coverage for a product does not assure that other payers will also provide coverage for the drug product. Further, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in the European Union may be more conservative than CMS.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as any product candidates we may develop. In many countries, particularly the countries of the European Union, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the United States, the reimbursement for any product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payers, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for any product candidates we may develop. We expect to experience pricing pressures in connection with the sale of any product candidates we may develop due to the trend toward managed healthcare, the increasing influence of certain third-party payers, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. There have been instances in which third-party payers have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payers, professional organizations such as the American Medical Association, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payers contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing any product candidates we may develop if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties.

In the future, we may build a sales and marketing infrastructure to market some of the product candidates we may develop if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to educate adequate numbers of physicians on the benefits of any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payers;

- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and we enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute any product candidates we may develop or may be unable to do so on terms that are acceptable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidates we may develop.

The biologic product candidates for which we intend to seek approval may face competition sooner than anticipated.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Amendment, or the ACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biological products.

There is a risk that any product candidates we may develop that are approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider any product candidates we may develop to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for nonbiological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

Risks related to our intellectual property

Although we own and license a number of pending patent applications which have not yet issued as patents, we do not currently own any issued patents. We do, however, in-license one issued U.S. patent and one issued European patent relating to product candidates we may develop. If we or our licensors are unable to obtain, maintain and defend patent and other intellectual property protection for any product candidates or technology, or if the scope of the patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully develop and commercialize any product candidates we may develop or our technology may be adversely affected due to such competition.

Our success depends in large part on our and our licensors' ability to obtain and maintain patent and other intellectual property protection in the United States and other jurisdictions. We currently own and license patent applications relating to our FORCE platform technology, including our Fabs, oligonucleotide payloads and Fab-oligonucleotide conjugates, as well as aspects of our manufacturing and methods of treatment. We and our licensors have sought, and will seek, to protect our proprietary position by filing additional patent applications in the United States and abroad related to certain technologies and our platform that are important to our business. However, our patent portfolio is at an early stage and we currently do not own or exclusively license any issued patents in any jurisdiction other than one issued U.S. patent and one issued European patent which we exclusively license. Moreover, there can be no assurance as to whether or when our patent applications will issue as granted patents. Our ability to stop third parties from making, using, selling, marketing, offering to sell, importing and commercializing any product candidates we may develop and our technology is dependent upon the extent to which we have rights under valid and enforceable patents and other intellectual property that cover our platform and technology. If we are unable to secure, maintain, defend and enforce patents and other intellectual property with respect to any product candidates we may develop and technology, it would have a material adverse effect on our business, financial condition, results of operations and prospects.

Our pending Patent Cooperation Treaty, or PCT, patent applications are not eligible to become issued patents until, among other things, we file a national stage patent application within 30 to 32 months, depending on the jurisdiction, from such application's priority date in the jurisdictions in which we are seeking patent protection. Similarly, our pending provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of such provisional patent application's filing date. If we do not timely file such national stage patent applications or non-provisional patent applications, we may lose our priority date with respect to such PCT or provisional patent applications, respectively, and any patent protection on the inventions disclosed in such PCT or provisional patent applications, respectively. While we and our licensors intend to timely file national stage and non-provisional patent applications relating to our PCT and provisional patent applications, respectively, we cannot predict whether any such patent applications will result in the issuance of patents. If we or our licensors do not successfully obtain issued patents, or, if the scope of any patent protection we or our licensors obtain is not sufficiently broad, we will be unable to prevent others from using any product candidates we may develop or our technology or from developing or commercializing technology and products similar or identical to ours or other competing products and technologies. Any failure to obtain or maintain patent protection with respect to our product candidates or our FORCE platform would have a material adverse effect on our business, financial condition, results of operations and prospects.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, defend, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. We and our licensors may not be able to obtain, maintain or defend patents and patent applications due to the subject matter claimed in such patents and patent applications being

in the public domain. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Consequently, we would not able to prevent any third party from using any of our technology that is in the public domain to compete with any product candidates we may develop.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of patent rights are highly uncertain. Our pending and future owned and licensed patent applications may not result in patents being issued which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and product or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all, and even if such patent applications do issue as patents, they may not issue in a form, or with a scope of claims, that will provide us with any meaningful protection, prevent others from competing with us or otherwise provide us with any competitive advantage. In addition, the scope of claims of an issued patent can be reinterpreted after issuance, and changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions may diminish the value of our patent rights or narrow the scope of our patent protection. Furthermore, our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

Third parties have developed technologies that may be related or competitive to our own technologies and product candidates and may have filed or may file patent applications, or may have obtained issued patents, claiming inventions that may overlap or conflict with those claimed in our owned or licensed patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our current and future product candidates and technology. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know for certain whether the inventors of our owned or licensed patents and patent applications were the first to make the inventions claimed in any owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patent applications may not issue as patents and even if issued, may be challenged and invalidated or ruled unenforceable.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and other jurisdictions. For example, we may be subject to a third-party submission of prior art to the United States Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our owned or licensed patents. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our owned or licensed pending patent applications. We may become involved in opposition, derivation, re-examination, *inter partes* review, post-grant review or interference proceedings and similar proceedings in foreign jurisdictions (for example, opposition proceedings) challenging our owned or licensed patent rights. In addition, a third party may claim that our owned or licensed patent rights are invalid or unenforceable in a litigation. An adverse result in any litigation or patent office proceeding could put one or more of our owned or licensed patents at risk of being invalidated, ruled unenforceable or interpreted narrowly and could allow third parties to commercialize products identical or similar to any product candidates we may

develop and compete directly with us, without payment to us. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. Such challenges and proceedings may result in loss of patent rights, exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and any product candidates we may develop. Such challenges and proceedings may also result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us. Moreover, there could be public announcements of the results of hearings, motions or other interim proceedings or developments related to such challenges and proceedings. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Furthermore, patents have a limited lifespan. In the United States, the expiration of a patent is generally 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. Patent term adjustments and extensions may be available; however, the overall term of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent and other intellectual property rights may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our technology and any product candidates we may develop. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Our rights to develop and commercialize any product candidates are subject and may in the future be subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under our current or future intellectual property license agreements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business.

We are and expect to continue to be reliant upon third-party licensors for certain patent and other intellectual property rights that are important or necessary to the development of our technology and product candidates. For example, we rely on a license from UMONS to certain patent rights and know-how of UMONS. Our license agreement with UMONS imposes, and we expect that any future license agreement will impose, specified diligence, milestone payment, royalty, commercialization, development and other obligations on us and require us to meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. For more information on the terms of the license agreement with UMONS, see “Business—Intellectual property—License agreement with the University of Mons.”

Furthermore, our licensors have, or may in the future have, the right to terminate a license if we materially breach the agreement and fail to cure such breach within a specified period or in the event we undergo certain bankruptcy events. In spite of our best efforts, our current or any future licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements. If our license agreements are terminated, we may lose our rights to develop and commercialize product candidates and technology, lose patent protection, experience significant delays in the development and commercialization of our product candidates and technology, and incur liability for damages. If these in-licenses are terminated, or if the underlying intellectual property fails to provide the intended exclusivity, our competitors or other third parties could have the freedom to seek regulatory approval of, and to market, products and technologies identical or competitive to ours and we may be required to cease our development and commercialization of certain of our product candidates and technology. In addition, we may seek to obtain additional licenses from

our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with any product candidates we may develop and our technology. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our or our licensors' ability to obtain, maintain and defend intellectual property and to enforce intellectual property rights against third parties;
- the extent to which our technology, product candidates and processes infringe, misappropriate or otherwise violate the intellectual property of the licensor that is not subject to the license agreement;
- the sublicensing of patent and other intellectual property rights under our license agreements;
- our diligence, development, regulatory, commercialization, financial or other obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, our license agreement with UMONS is, and future license agreements are likely to be, complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our diligence, development, regulatory, commercialization, financial or other obligations under the relevant agreement. In addition, if disputes over intellectual property that we have licensed or any other dispute related to our license agreements prevent or impair our ability to maintain our current license agreements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technology. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

License agreements we may enter into in the future may be non-exclusive. Accordingly, third parties may also obtain non-exclusive licenses from such licensors with respect to the intellectual property licensed to us under such license agreements. Accordingly, these license agreements may not provide us with exclusive rights to use such licensed patent and other intellectual property rights, or may not provide us with exclusive rights to use such patent and other intellectual property rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and any product candidates we may develop in the future.

Moreover, some of our in-licensed patent and other intellectual property rights may in the future be subject to third party interests such as co-ownership. If we are unable to obtain an exclusive license to such third-party co-owners' interest, in such patent and other intellectual property rights, such third-party co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. We or our licensors may need the cooperation of any such co-owners of our licensed patent and other intellectual property rights in order to enforce them against third parties, and such cooperation may not be provided to us or our licensors.

Additionally, we may not have complete control over the preparation, filing, prosecution, maintenance, enforcement and defense of patents and patent applications that we license from third parties. It is possible that our licensors' filing, prosecution and maintenance of the licensed patents and patent applications, enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, and accordingly, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to file, prosecute, maintain, enforce and defend such patents and patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our technology and any product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors or other third parties from making, using and selling competing products.

Furthermore, our owned and in-licensed patent rights may be subject to a reservation of rights by one or more third parties. When new technologies are developed with government funding, in order to secure ownership of patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. government and timely electing title to such inventions. A failure to meet these obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications. In addition, the U.S. government may have certain rights in such patent rights, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such U.S. government-funded inventions may be subject to certain requirements to manufacture any product candidates we may develop embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations and prospects significantly.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, maintaining, enforcing and defending patents and other intellectual property rights on our technology and any product candidates we may develop in all jurisdictions throughout the world would be prohibitively expensive, and accordingly, our intellectual property rights in some jurisdictions outside the United States could be less extensive than those in the United States. In some cases, we or our licensors may not be able to obtain patent or other intellectual property protection for certain technology and product candidates outside the United States. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we and our licensors may not be able to obtain issued patents or other intellectual property rights covering any product candidates we may develop and our technology in all jurisdictions outside the United States and, as a result, may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Third parties may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent or other intellectual property protection to develop their own products and, further, may export otherwise infringing, misappropriating or violating products to territories where we have patent or other intellectual property protection, but enforcement is not as strong as that in the

United States. These products may compete with any product candidates we may develop and our technology and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Additionally, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain jurisdictions, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement, misappropriation or other violation of our patent and other intellectual property rights or marketing of competing products in violation of our intellectual property rights generally. For example, an April 2019 report from the Office of the United States Trade Representative identified a number of countries, including China, Russia, Argentina, Chile and India, where challenges to the procurement and enforcement of patent rights have been reported. Proceedings to enforce our or our licensors' patent and other intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patent and other intellectual property rights at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We or our licensors may not prevail in any lawsuits that we or our licensors initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many jurisdictions have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many jurisdictions limit the enforceability of patents against government agencies or government contractors. In these jurisdictions, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or patent applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our owned or licensed patent rights. We rely on our outside counsel and other professionals or our licensing partners to pay these fees due to the USPTO and non-U.S. government patent agencies. The USPTO and various non-U.S. government patent agencies also require compliance with several procedural, documentary and other similar provisions during the patent application process. We rely on our outside counsel and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment, loss of priority or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

We may not be successful in obtaining necessary rights to product candidates we may develop through acquisitions and in-licenses.

We currently have rights to certain intellectual property through licenses from third parties. Because our programs may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these intellectual property rights. In addition, with respect to any patent or other intellectual property rights that we co-own with third parties, we may require exclusive licenses to such co-owners' interest in such patent or other intellectual property rights. However, we may be unable to secure such licenses or otherwise acquire or in-license any intellectual property rights related to compositions, methods of use, processes or other components from third parties that we identify as necessary for any product candidates we may develop and our technology on commercially reasonable terms, or at all. Even if we are able to in-license any such necessary intellectual property, it could be on non-exclusive terms, thereby giving our competitors and other third parties access to the same intellectual property licensed to us, and the applicable licensors could require us to make substantial licensing and royalty payments. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to third parties, potentially blocking our ability to pursue our research program and develop and commercialize our product candidates.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign any product candidates we may develop or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Issued patents covering any product candidates we may develop could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.

Our owned and licensed patent rights may be subject to priority, validity, inventorship and enforceability disputes. If we or our licensors are unsuccessful in any of these proceedings, such patent rights may be narrowed, invalidated or held unenforceable, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or we may be required to cease the development, manufacture and commercialization of one or more of our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we or one of our licensors initiate legal proceedings against a third party to enforce a patent covering any of any product candidates we may develop or our technology, the defendant could counterclaim that the patent

covering the product candidate or technology is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, interference proceedings, derivation proceedings, post grant review, *inter partes* review and equivalent proceedings such as opposition, invalidation and revocation proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover any product candidates we may develop or our technology or prevent third parties from competing with any product candidates we may develop or our technology. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates or technology. Such a loss of patent protection could have a material adverse effect on our business, financial condition, results of operations and prospects.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, contractors and other parties who have access to such technology and processes. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees and consultants who are parties to these agreements breach or violate the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets and third parties could use our trade secrets to compete with any product candidates we may develop and our technology. Additionally, we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems; however, such systems and security measures may be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors or other third parties. Competitors or third parties could purchase any product candidates we may develop or our technology and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our intellectual property rights or develop their own competitive technologies that fall outside the scope of our intellectual property rights. If any

of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products, our business, financial condition, results of operations and prospects could be materially and adversely affected.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could harm our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation in which third parties may assert infringement, misappropriation or other violation claims against us, alleging that any product candidates we may develop, manufacturing methods, formulations or administration methods are covered by their patents. Given the vast number of patents and other intellectual property in our field of technology, we cannot be certain or guarantee that we do not infringe, misappropriate or otherwise violate patents or other intellectual property. Other companies and institutions have filed, and continue to file, patent applications that may be related to our technology and, more broadly, to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are pursuing development candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that we may be subject to claims of infringement of the patent rights of third parties. If a patent holder believes the manufacture, use, sale or importation of any product candidates we may develop or our technology infringes its patent, the patent holder may sue us even if we have licensed other patent rights for our technology.

We are aware of certain patents in the United States and other jurisdictions owned by third parties that claim subject matter that relates to our program candidates and the FORCE platform. Although we believe that these patents are invalid and/or not infringed, such third parties may assert these patents against us in litigation in the United States or other jurisdictions. The outcome of any such litigation is uncertain and, even if we prevail, the costs of such litigation could have a material adverse effect on our financial position, result in disclosure of our trade secrets, distract key personnel from the continued development of our business, and adversely affect our ability to enter or maintain commercial relationships with collaborators, clients or customers. If we are unsuccessful in such litigation, we could be prevented from commercializing products or could be required to take licenses from such third parties which may not be available on commercially reasonable terms, if at all.

It is also possible that we have failed to identify relevant third-party patents or applications. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of any product candidates we may develop or our technology and we may not be aware of such patents. Furthermore, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States may remain confidential until a patent issues. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to any product candidates we may develop and our technologies

because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, any product candidates we may develop or the use of any product candidates we may develop.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize any product candidates we may develop or any other of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing any product candidates we may develop and our technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing any product candidates we may develop or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

Intellectual property litigation or other proceedings could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Competitors may challenge the validity and enforceability of our patent rights or those of our licensing partners, infringe, misappropriate or otherwise violate our or our licensors' patent and other intellectual property rights, or we may be required to defend against claims of infringement, misappropriation or other violation. Litigation and other proceedings in connection with any of the foregoing claims can be unpredictable, expensive and time consuming. Even if resolved in our favor, litigation or other proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our scientific, technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors or other third parties may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace and could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or be required to obtain licenses to such intellectual property rights, which may not be available on commercially reasonable terms or at all. An inability to incorporate such intellectual property rights would harm our business and may prevent us from successfully commercializing any product candidates we may develop or at all. In addition, we may lose personnel as a result of such claims and any such litigation or the threat thereof may adversely affect our ability to hire employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize any product candidates we may develop and our technology, which would have a material adverse effect on our business, results of operations, financial condition and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our scientific and management personnel.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Furthermore, individuals executing agreements with us may have pre-existing or competing obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property that we own may have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we or our licensors may in the future be subject to claims by former employees, consultants or other third parties asserting an ownership right in our owned or licensed patent rights. An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar technology and therapeutics, without payment to us, or could limit the duration of the patent protection covering our technology and any product candidates we may develop. Such challenges may also result in our inability to develop, manufacture or commercialize our technology and product candidates without infringing third-party patent rights. In addition, if the breadth or

strength of protection provided by our owned or licensed patent rights are threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future technology and product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Changes in patent law in the United States or worldwide could diminish the value of patents in general, thereby impairing our ability to protect any product candidates we may develop and our technology.

Changes in either the patent laws or interpretation of patent laws in the United States and worldwide, including patent reform legislation such as the Leahy-Smith America Invents Act, or the Leahy-Smith Act, could increase the uncertainties and costs surrounding the prosecution of any owned or in-licensed patent applications and the maintenance, enforcement or defense of any current in-licensed issued patents and issued patents we may own or in-license in the future. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first-to-file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our in-licensed issued patents and issued patents we may own or in-license in the future, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects. Since patent applications in the United States and most other countries are confidential for a period of time after filing or until issuance, we cannot be certain that we or our licensors were the first to either (i) file any patent application related to our product candidates or (ii) invent any of the inventions claimed in our or our licensor's patents or patent applications.

The America Invents Act also includes a number of significant changes that affect the way patent applications will be prosecuted and also may affect patent litigation. These include allowing third party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent by USPTO administered post-grant proceedings, including post-grant review, inter partes review, and derivation proceedings. Because of a lower evidentiary standard in USPTO proceedings compared to the evidentiary standard in United States federal courts necessary to invalidate a patent claim, a third party could potentially provide evidence in a USPTO proceeding sufficient for the USPTO to hold a claim unpatentable even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third party may attempt to use the USPTO procedures to review patentability of our patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. Therefore, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our owned or in-licensed patent applications and the enforcement or defense of our owned or in-licensed issued patents, all of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain

situations. As one example, in the case Assoc. for Molecular Pathology v. Myriad Genetics, Inc., the U.S. Supreme Court held that certain claims to DNA molecules are not patentable simply because they have been isolated from surrounding material. Moreover, in 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. Accordingly, in view of the guidance memo, there can be no assurance that claims in our patent rights covering any product candidates we may develop or our technology will be held by the USPTO or equivalent foreign patent offices or by courts in the United States or in foreign jurisdictions to cover patentable subject matter. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop and our technology, one or more of our U.S. patents that we license or may own in the future may be eligible for limited patent term extension under Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved product, a method for using it or a method for manufacturing it may be extended. The application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

We may be subject to claims challenging the inventorship or ownership of our patent and other intellectual property rights.

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates or technology. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patent rights, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of or right to use intellectual property that is important to any product candidates we may develop or our technology. Even if we are

successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.

We have filed trademark applications with the USPTO for our corporate name. Our current and future trademark applications in the United States and other foreign jurisdictions may not be allowed or may be subsequently opposed. Once filed and registered, our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. As a means to enforce our trademark rights and prevent infringement, we may be required to file trademark claims against third parties or initiate trademark opposition proceedings. This can be expensive and time-consuming, particularly for a company of our size. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make products that are similar to any product candidates we may develop but that are not covered by the intellectual property, including the claims of the patents, that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing, misappropriating or otherwise violating our owned or licensed intellectual property rights;
- it is possible that our or our licensors' current or future pending patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by third parties;

- third parties might conduct research and development activities in jurisdictions where we do not have patent or other intellectual property rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor or other third party will discover our trade secrets or that our trade secrets will be misappropriated or disclosed.

Because we currently rely on certain third parties to manufacture all or part of our drug product and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our product engine and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements and other similar agreements with our collaborators, advisors, employees, consultants and contractors prior to beginning research or disclosing any proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors or other third parties, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's or other third party's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Risks related to regulatory approval and other regulatory and legal compliance matters

Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of any product candidates we may develop. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, product candidates we may develop, and our ability to generate revenue will be materially impaired.

Any product candidates we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate we may develop will prevent us from

commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction. We have no experience as a company in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CLROs to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety, purity and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we may develop may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Of the large number of products in development, only a small percentage successfully complete the FDA or foreign regulatory approval processes and are commercialized. Even if any product candidates we may develop demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

Even if we eventually complete clinical testing and receive approval of a BLA or foreign marketing application for any product candidates, the FDA or the applicable foreign regulatory agency may grant approval or other marketing authorization contingent on the performance of costly additional clinical trials, including post-market clinical trials. The FDA or the applicable foreign regulatory agency also may approve or authorize for marketing a product candidate for a more limited indication or patient population that we originally request, and the FDA or applicable foreign regulatory agency may not approve or authorize the labeling that we believe is necessary or desirable for the successful commercialization of a product candidate. Any of these restrictions or commitments could render an approved product not commercially viable, which would materially adversely impact our business and prospects.

Obtaining and maintaining marketing approval or commercialization of our product candidates in the United States does not mean that we will be successful in obtaining marketing approval of our product candidates in other jurisdictions. Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to

obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Political and socioeconomic factors can also adversely affect our business and our ability to obtain regulatory approvals in other countries. For example, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020 (extendable up to two years). Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult. To date, only an outline of a trade agreement has been reached. Much remains open but the Prime Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for any product candidates we may develop, which could significantly and materially harm our business.

Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of any product candidates we may develop.

If any product candidate we may develop is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet medical need for this condition, the sponsor may apply for FDA fast track designation. However, a fast track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive fast track designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

Breakthrough or RMAT therapy designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.

If any product candidate we may develop is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development, the sponsor may apply for FDA breakthrough designation or a regenerative medicine advanced therapy, or RMAT,

designation. However, neither a breakthrough designation nor an RMAT designation ensures that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive breakthrough or RMAT designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw breakthrough or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Neither breakthrough nor RMAT designation alone guarantees qualification for the FDA's priority review procedures.

Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.

If the FDA determines that a product candidate we may develop offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for any product candidates we may develop. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate we may develop is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

We may not be able to obtain orphan drug exclusivity for any product candidates we may develop, and even if we do, that exclusivity may not prevent regulatory authorities from approving other competing products.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

In order for the FDA to grant orphan drug exclusivity to one of our products, the agency must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two genetic medicine products to be different drugs solely based on minor differences in the transgenes or vectors. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

In 2017, the Congress passed the FDA Reauthorization Act of 2017, or the FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

Even if we, or any collaborators we may have, obtain marketing approvals for any product candidates we may develop, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Any product candidate for which we obtain marketing approval, if ever, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The FDA typically advises that patients treated with genetic medicine undergo follow-up observations for potential adverse events for a 15-year period. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

Accordingly, assuming we, or any third parties we may collaborate with, receive marketing approval for one or more product candidates we may develop, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we and such collaborators are not able to comply with post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition and prospects.

If we fail to comply with applicable regulatory requirements following approval of any product candidates we may develop, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;

- refuse to approve a pending BLA or supplements to a BLA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we may develop and generate revenues.

Any product candidate we may develop for which we obtain marketing approval will be subject to restrictions, such as the laws and regulations prohibiting the promotion of off-label uses, or may need to be withdrawn from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our medicines, when and if any of them are approved.

The FDA and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other regulatory agencies impose stringent restrictions on manufacturers' communications regarding off-label use. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling. If we do not market our medicines for their approved indications, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice. Violation of the Federal Food, Product, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws and state consumer protection laws. The federal government has levied large civil and criminal fines against companies for alleged improper promotion of off-label use and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

In addition, later discovery of previously unknown problems with our medicines, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such medicines, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a medicine;
- restrictions on the distribution or use of a medicine;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the medicines from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of medicines;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;

- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our medicines;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we develop and adversely affect our business, financial condition, results of operations and prospects.

Additionally, if any product candidates we may develop receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to healthcare practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

We and our contract manufacturers are subject to significant regulation. The manufacturing facilities on which we rely may not continue to meet regulatory requirements, which could materially harm our business.

All entities involved in the preparation of product candidates for clinical trials or commercial sale, including any contract manufacturers, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturer must supply all necessary documentation in support of a BLA on a timely basis and must adhere to the FDA's cGLP and cGMP regulations enforced through its facilities inspection program. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of any product candidates we may develop or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a

failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product, or revocation of a pre-existing approval. Any such consequence would severely harm our business, financial condition and results of operations.

If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur significant costs.

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws, regulations and permitting requirements. These current or future laws, regulations and permitting requirements may impair our research, development or production efforts. Failure to comply with these laws, regulations and permitting requirements also may result in substantial fines, penalties or other sanctions or business disruption. Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could in turn have a material adverse effect on our business, financial condition, results of operations and prospects.

We are affected by the political environment and changes that may be made to regulatory regimes. The efforts of the Trump Administration to pursue regulatory reform may limit the FDA's ability to engage in oversight and implementation activities in the normal course, and that could negatively impact our business.

The Trump Administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. On January 30, 2017, the president issued an executive order, applicable to all executive agencies, including the FDA, that required that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This executive order includes a budget neutrality provision that required the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within the Office of Management on February 2, 2017, the administration indicated that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Our relationships with healthcare providers, physicians and third-party payers will be subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payers play a primary role in the recommendation and prescription of any product candidates that we develop for which we obtain marketing approval. Our future arrangements with third-party payers and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research, market, sell and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order, or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the federal False Claims Act, which can be enforced through civil whistleblower or qui tam actions, imposes civil and criminal penalties against individuals or entities for knowingly presenting or causing to be presented, to the federal government, claims for payment or approval from Medicare, Medicaid or other government payers that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which prohibits, among other things, knowingly and willfully executing, or attempting to execute, a scheme or artifice to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit

program, regardless of the payor (e.g., public or private), and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;

- HIPAA, as further amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, which imposes certain requirements, including mandatory contractual terms, on covered entities subject to the rule, such as health plans, healthcare clearinghouses and certain healthcare providers, as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security, and transmission of such individually identifiable health information;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements under the federal Physician Payments Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services, or HHS, information related to payments and other transfers of value to physicians, as defined by such law, and teaching hospitals and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payers, including private insurers, and certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to drug pricing and payments to physicians and other healthcare providers or marketing expenditures and state and local laws that require the registration of sales representatives.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in

violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion from government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of any product candidates we may develop, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payers.

The ACA, which became law in 2010, contains provisions of importance to our business, including, without limitation, our ability to commercialize and the prices we may obtain for any product candidates we may develop and that are approved for sale, the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2030 unless additional Congressional action is taken. The Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been, and continue to be, numerous executive and legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which required most Americans to carry a minimal level of health insurance, became effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." The Congress may consider other legislation to replace elements of the ACA.

The Trump Administration has also taken executive actions to undermine or delay implementation of the ACA. Since January 2017, the president has signed several executive orders designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. One executive order directs federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices. Another executive order terminates the cost-sharing subsidies that reimburse insurers under the ACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the ACA for plans sold through such marketplaces. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in ACA risk corridor payments to third-party payers

who argued were owed to them. On April 27, 2020, the United States Supreme Court reversed the federal circuit decision upholding Congress' denial of such risk corridor funding.

Additionally, on December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. On December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case, and has allotted one hour for oral arguments, which are expected to occur this year. On June 25, 2020, the Trump Administration and a coalition of 18 states asked the court to strike down the entirety of the ACA. It is unclear how such litigation and other efforts to repeal and replace the ACA will impact the ACA and our business.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our potential products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payers.

The costs of prescription pharmaceuticals have also been the subject of considerable discussion in the United States, and members of Congress and the executive branch have stated that they will address such costs through new legislative and administrative measures. To date, there have been several recent U.S. congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the Trump Administration's budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs. While some of these and other measures may require additional authorization to become effective, Congress and the Trump Administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

More recently, President Trump has issued five executive orders that are intended to lower the costs of prescription drug products. The first order would require all federally qualified health centers, or FQHCs, to pass on to patients the discounts the health centers receive on insulin and epinephrine through Medicare's 340B Drug Discount Program.

The second order would establish an international pricing index that would set the price Medicare Part B pays for the costliest medications covered under the program to the lowest price in other economically advanced countries. At the time that the President announced this order, he also indicated that for the time being it would not be implemented.

The third order is intended to reduce the costs of drugs by supporting the safe importation of prescription drugs. Specifically, the order calls upon HHS to facilitate grants to individuals of waivers of the prohibition of importation of prescription drugs that would allow patients to import FDA approved drug products from abroad, so long as doing so would result in lower costs. In addition, the order would allow wholesalers and pharmacies to re-import both biological drugs and insulin that were originally manufactured in the

United States and then exported for international sale. This action follows the publication of a proposed rulemaking on December 23, 2019, that, if finalized, would allow states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants would be required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. At the same time, the FDA issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

The fourth executive order would end drug rebates used by health plan sponsors, pharmacies or pharmacy benefit managers, or PBMs, in operating the Medicare Part D program. Specifically, the order directs HHS to exclude from safe harbor protections under the federal anti-kickback statute retroactive price reductions that are not applied at the point-of-sale. Instead, the order requires HHS to establish new safe harbors that would allow health plan sponsors, pharmacies and PBMs to pass on those discounts to consumers at point-of-sale "in order to lower the patient's out-of-pocket costs" and "permit the use of certain bona fide PBM service fees." Each of these orders directs the federal government to implement the initiatives outlined in the orders, meaning they will not have immediate effects.

Finally, the fifth order instructs the federal government to develop a list of "essential" medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including China. The order is meant to reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for any product candidates we may develop or additional pricing pressures.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for product candidates that we may identify, if we obtain regulatory approval;
- our ability to receive or set a price that we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability;
- the level of taxes that we are required to pay; and
- the availability of capital.

Changes in tax laws or regulations or in their implementation or interpretation may adversely affect our business and financial condition.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could adversely affect our business or financial condition. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, on December 22, 2017, the U.S. government enacted the Tax Act, which significantly reformed the Code. The Tax Act, among other things, contained significant changes to corporate taxation, including a reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for net interest expense to 30% of adjusted taxable income (except for certain small businesses), the limitation of the deduction for NOLs arising in taxable years beginning after December 31, 2017 to 80% of current year taxable income and elimination of NOL carrybacks for losses arising in taxable years beginning after December 31, 2017 (though any such NOLs may be carried forward indefinitely), the imposition of a one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, the elimination of U.S. tax on foreign earnings (subject to certain important exceptions), the allowance of immediate deductions for certain new investments instead of deductions for depreciation expense over time, and the modification or repeal of many business deductions and credits.

In addition, as part of Congress' response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, and the CARES Act was enacted on March 27, 2020. Both laws contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of NOLs, which was enacted as part of the Tax Act. It also provides that NOLs arising in any taxable year beginning after December 31, 2017, and before January 1, 2021 are generally eligible to be carried back up to five years. The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation of the tax deductibility for net interest expense by increasing the limitation from 30 to 50% of adjusted taxable income.

Regulatory guidance under the Tax Act, the FFCR Act and the CARES Act is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. It is also possible that Congress will enact additional legislation in connection with the COVID-19 pandemic, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the FFCR Act or the CARES Act.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants and partners, and, if we commence clinical trials, our principal investigators. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible

to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.

We are subject to numerous laws and regulations in each jurisdiction outside the United States in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, or authorizing the provision of money or anything of value, directly or indirectly through parties, to any foreign official, official of a public international organization, or political party official or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The Securities and Exchange Commission, or SEC, is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA and other anti-corruption laws potentially applicable to our business is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, compliance with the FCPA and other anti-corruption laws presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials.

Various U.S. export and sanctions laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of certain products and technical data relating to those products. Furthermore, such export and sanctions laws include restrictions or prohibitions on the sale or supply of certain products and services to United States embargoed countries or sanctioned countries, governments, persons and entities. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA and export and sanctions laws can result in significant civil and criminal penalties, imprisonment, the loss of export or import privileges, debarment, breach of contract and fraud litigation, reputational harm, and other consequences. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition, results of operations or prospects.

We are subject to data privacy and protection laws, regulations, policies and contractual obligations that apply to the collection, transmission, storage and use of personal information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with laws and regulations governing personal information could result in enforcement actions against us, including fines, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer or other processing of personal data regarding individuals in the United Kingdom and European Union, including personal health data, is subject to the European Union General Data Protection Regulation (EU) 2016/679, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, establishing a legal basis for processing, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data that requires the adoption of administrative, physical and technical safeguards to protect such information, providing notification of data breaches to appropriate data protection authorities or data subjects, establishing means for data subjects to exercise rights in relation to their personal data and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States and, as a result, increases the scrutiny for transfers of personal data from clinical trial sites located in the EEA to the United States. The United Kingdom and Switzerland have adopted similar restrictions. Although there are legal mechanism to allow for the transfer of personal data from the United Kingdom, EEA and Switzerland to the United States, uncertainty about compliance with such data protection laws remains and such mechanisms may not be available or applicable with respect to the personal data processing activities necessary to research, develop and market our any product candidates we develop. For example, legal challenges in Europe to the mechanisms allowing companies to transfer personal data from the EEA to the United States could result in further limitations on the ability to transfer personal data across borders, particularly if governments are unable or unwilling to reach new or maintain existing agreements that support cross-border data transfers, such as the EU-U.S. and Swiss-U.S. Privacy Shield Frameworks. Specifically, on July 16, 2020, the Court of Justice of the European Union invalidated Decision 2016/1250 on the adequacy of the protection provided by the EU-U.S. Privacy Shield Framework. To the extent that we were to rely on the EU-U.S. Privacy Shield Framework, we will not be able to do so in the future, which could increase our costs and limit our ability to process personal data from the European Union. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and/or impose

substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater, and confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data. Further, the United Kingdom's decision to leave the European Union, often referred to as Brexit, has created uncertainty with regard to data protection regulation in the United Kingdom. In particular, while the Data Protection Act of 2018, that "implements" and complements the GDPR achieved Royal Assent on May 23, 2018 and is now effective in the United Kingdom, it is still unclear whether transfer of data from the EEA to the United Kingdom will remain lawful under GDPR. During the period of "transition" (i.e., until December 31, 2020), EU law will continue to apply in the United Kingdom, including the GDPR, after which the GDPR will be converted into UK law. Beginning in 2021, the UK will be a "third country" under the GDPR. Additionally, other countries have passed or are considering passing laws requiring local data residency and/or restricting the international transfer of data.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with the GDPR and similar laws' requirements are rigorous and time intensive and require significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Similar privacy and data security requirements are either in place or underway in the United States. There are a broad variety of data protection laws that may be applicable to our activities, and a range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered or have been implemented at both the state and federal levels. For example, the California Consumer Privacy Act of 2018, or the CCPA, which became effective on January 1, 2020, requires companies that process information on California residents to make new disclosures to consumers about their data collection, use and sharing practices, provides consumers with new data privacy rights, imposes new operational requirements for covered businesses, provides a private right of action for data breaches and creates a statutory damages framework. Many other states are considering similar legislation, and a broad range of legislative measures also have been introduced at the federal level. Although there are limited exemptions for clinical trial data under the CCPA, the CCPA and other similar laws could impact our business activities depending on how it is interpreted and exemplifies the vulnerability of our business to the evolving regulatory environment related to personal data.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information, including certain laws that regulate the use and disclosure of personal health information. In particular, regulations promulgated pursuant to HIPAA, as amended by HITECH, establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. These provisions may be applicable to our business. Determining

whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have violated these privacy and security laws and/or breached certain contracts with our business partners (including as a business associate). Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face significant civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

Any failure by our third-party collaborators, service providers, contractors or consultants to comply with applicable law, regulations or contractual obligations related to data privacy or security could result in proceedings against us by governmental entities or others.

We may publish privacy policies and other documentation regarding our collection, processing, use and disclosure of personal information and/or other confidential information. Although we endeavor to comply with our published policies and other documentation, we may at times fail to do so or may be perceived to have failed to do so. Moreover, despite our efforts, we may not be successful in achieving compliance if our employees or vendors fail to comply with our published policies and documentation. Such failures can subject us to potential international, local, state and federal action if they are found to be deceptive, unfair, or misrepresentative of our actual practices. Moreover, patients or subjects about whom we or our partners obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws or applicable privacy notices even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

It is possible that new and existing laws may be interpreted and applied in a manner that is inconsistent with our practices and our efforts to comply with the evolving data protection rules may be unsuccessful. If so, this could result in government-imposed fines, or penalties or orders requiring that we change our practices, which could adversely affect our business. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with federal, state and international laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines, penalties or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement actions, litigation and significant costs for remediation, reputational harm, diminished profits and earnings, additional reporting requirements and/or oversight, any of which could adversely affect our business, our results of operations or prospects. We also face a threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business, financial condition, results of operations or prospects.

Risks related to employee matters, managing growth and other operational matters

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment offer letters with our executive officers, each of them may terminate their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success.

The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

As of August 31, 2020, we had 36 full-time employees. As our development progresses, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs and, if any product candidate we may develop receives marketing approval, sales, marketing, distribution and coverage and reimbursement capabilities. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a growing biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for, and fully understanding the regulatory and manufacturing pathways to, all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel.

This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

We may acquire additional businesses, technologies or assets, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new products or product candidates resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with collaborators as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

Our internal information technology systems, or those of our vendors, collaborators or other contractors or consultants, may fail or suffer security breaches, loss or leakage of data and other disruptions or compromise, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, trigger contractual and legal obligations, potentially exposing us to liability, reputational harm or otherwise adversely affecting our business and financial results.

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property, proprietary business information and personal information). It is critical that we, our vendors, collaborators or other contractors or consultants, do so in a secure manner to maintain the availability, security, confidentiality, privacy and integrity of such confidential information.

Despite the implementation of security measures, given the size and complexity of our internal information technology systems and those of our current and any future vendors, collaborators and other contractors and consultants, and the increasing amounts of confidential information that they maintain, such information technology systems are vulnerable to damage or interruption from computer viruses, computer hackers, malicious code, employee error, theft or misuse, denial-of-service attacks, sophisticated nation-state and nation-state-supported actors, unauthorized access, natural disasters, terrorism, war, telecommunication and electrical failures or other compromise. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, and we may not be able to implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies.

While we seek to protect our information technology systems from system failure, accident and security breach, our efforts may not be successful. If such an event were to occur, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary or confidential information or other disruptions. For example, the loss of clinical trial data from future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience a significant cybersecurity breach of our information systems or data, the costs associated with the investigation, remediation and potential notification of the breach to counterparties, data subjects, regulators or others could be material. In addition, our remediation efforts may not be successful. Moreover, if the information technology systems of our vendors, collaborators and other contractors and consultants become subject to disruptions or security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. If we do not allocate and effectively manage the resources necessary to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, data loss or the loss of or damage to intellectual property or other proprietary information.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our or our vendors', collaborators' or other contractors' or consultants' data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability including litigation exposure, penalties and fines, we could become the subject of regulatory action or investigation, our competitive position and reputation could be harmed and the further development and commercialization of our product candidates

could be delayed. As a result of such an event, we may be in breach of our contractual obligations. Furthermore, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our customers or employees, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

The financial exposure from the events referenced above could either not be insured against or not be fully covered through any insurance that we maintain and could have a material adverse effect on our business, financial condition, results of operations or prospects. In addition, we cannot be sure that our existing insurance coverage will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages as a result of the events referenced above.

Our operations or those of the third parties upon whom we depend might be affected by the occurrence of a natural disaster, pandemic or other catastrophic event.

We depend on our employees, consultants, CMOs, CROs and CLROs, as well as regulatory agencies and other parties, for the continued operation of our business. While we maintain disaster recovery plans, they might not adequately protect us. Despite any precautions we take for natural disasters or other catastrophic events, these events, including terrorist attack, pandemics, hurricanes, fire, floods and ice and snowstorms, could result in significant disruptions to our research and development, preclinical studies, clinical trials, and, ultimately, commercialization of our products. Long-term disruptions in the infrastructure caused by events, such as natural disasters, the outbreak of war, the escalation of hostilities and acts of terrorism or other ‘‘acts of God,’’ particularly involving cities in which we have offices, manufacturing or clinical trial sites, could adversely affect our businesses. Although we carry business interruption insurance policies and typically have provisions in our contracts that protect us in certain events, our coverage might not respond or be adequate to compensate us for all losses that may occur. Any natural disaster or catastrophic event affecting us, our CMOs, CROs or CLROs, regulatory agencies or other parties with which we are engaged could have a significant negative impact on our operations and financial performance.

The COVID-19 pandemic may affect our ability to initiate and complete preclinical studies, delay the initiation of our planned clinical trial or future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, which could negatively impact our operations and our ability to raise additional capital following this offering.

In December 2019, a novel strain of coronavirus, SARS-CoV-2, causing a disease referred to as COVID-19, emerged in China. Since then, COVID-19 has spread to multiple countries worldwide, including the United States. In March 2020, the World Health Organization declared the COVID-19 outbreak a pandemic. The COVID-19 pandemic is causing many governments to implement measures to slow the spread of the outbreak through quarantines, travel restrictions, heightened border scrutiny and other measures. The outbreak and government measures taken in response have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen.

The future progression of the outbreak and its effects on our business and operations are uncertain. We and our CMOs and CROs have experienced a reduction in the capacity to undertake research-scale production and to execute some preclinical studies, and we may face disruptions that affect our ability to initiate and complete preclinical studies, and disruptions in procuring items that are essential for our research and development activities, such as raw materials used in the manufacture of any product candidates we may develop, laboratory supplies used in our preclinical studies, or animals that are used for preclinical testing for which there are shortages because of ongoing efforts to address the outbreak. We and our CROs and CMOs may face disruptions related to our future IND-enabling studies and clinical trials arising from delays in preclinical studies, manufacturing disruptions, and the ability to obtain necessary IRB, IBC or other necessary site approvals, as well as other delays at clinical trial sites.

The response to the COVID-19 pandemic may also redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions. The pandemic has already caused significant disruptions in worldwide financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business, although for the reasons described above it has the potential to adversely affect our financial condition, results of operations and prospects.

Risks related to this offering, ownership of our common stock and our status as a public company

We do not know whether an active trading market will develop for our common stock or what the market price of our common stock will be, and, as a result, it may be difficult for you to sell your shares of our common stock.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock was determined through negotiations with the underwriters. Although our common stock has been approved for listing on the Nasdaq Global Select Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop, it may be difficult for you to sell shares you purchase in this offering at an attractive price or at all.

If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.

The initial public offering price of our common stock is substantially higher than the pro forma as adjusted net tangible book value per share of our common stock after this offering. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our pro forma as adjusted net tangible book value per share after this offering. Based on our capitalization as of June 30, 2020 and the initial public offering price of $19.00 per share, you will experience immediate dilution of $11.06 per share, representing the difference between our pro forma as adjusted net tangible book value per share after this offering and the initial public offering price. In addition, as of August 31, 2020, we had outstanding stock options to purchase an aggregate of 5,201,900 shares of common stock at a weighted average exercise price of $4.03 per share. To the extent these outstanding options are exercised, you will incur further dilution.

If securities analysts do not publish or cease publishing research or reports or publish misleading, inaccurate or unfavorable research about our business or if they publish negative evaluations of our stock, the price and trading volume of our stock could decline.

The trading market for our common stock will rely, in part, on the research and reports that industry or financial analysts publish about us or our business. We do not currently have, and may never obtain, research coverage by industry or financial analysts. If no, or few, analysts commence coverage of us, the trading price of our stock would likely decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, or provide more favorable relative recommendations about our competitors, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price and trading volume to decline.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.

Our stock price is likely to be volatile. The stock market in general, and the market for smaller biopharmaceutical companies in particular, have experienced extreme price volatility and volume fluctuations that have often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- timing and results of, or developments in, preclinical studies and clinical trials of any product candidates we may develop or those of our competitors or potential collaborators;
- adverse regulatory decisions, including failure to receive marketing approvals for any product candidates we may develop;
- our success in commercializing any product candidates that may be approved;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other intellectual property or proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any product candidates we may develop;
- the results of our efforts to discover, develop, acquire or in-license products, product candidates, technologies or data referencing rights, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to our financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or the financial results of companies that are perceived to be similar to us;
- sales of our common stock by us, our executive officers, directors or principal stockholders or others;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;

- general economic, industry, political and market conditions, including conditions resulting from the effects of the ongoing COVID-19 pandemic; and
- the other factors described in this “Risk factors” section.

Any of the factors listed above could materially adversely affect your investment in our common stock, and our common stock may trade at prices significantly below the initial public offering price, which could contribute to a loss of all or part of your investment. In such circumstances the trading price of our common stock may not recover and may experience a further decline.

In the past, following periods of volatility in the market price of a company’s securities, securities class-action litigation has often been instituted against that company. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Such litigation may also cause us to incur other substantial costs to defend such claims and divert management’s attention and resources.

Unfavorable global economic conditions could adversely affect our business, financial condition, stock price and results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the 2008 global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn resulting from the COVID-19 pandemic could result in a variety of risks to our business, including weakened demand for any product candidates we may develop and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could impair our ability to achieve our growth strategy, could harm our financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that our current or future service providers, manufacturers or other collaborators may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. We cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business.

After this offering, our executive officers, directors and principal stockholders, if they choose to act together, will continue to have the ability to control all matters submitted to stockholders for approval.

Upon the closing of this offering, based on the number of shares outstanding as of August 31, 2020, and giving effect to the issuance of 12,251,578 shares of our common stock in this offering, assuming no exercise by the underwriters of their option to purchase additional shares of our common stock and the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 28,086,375 shares of our common stock upon the closing of this offering, our executive officers and directors and our stockholders who owned more than 5% of our outstanding common stock before this offering will, in the aggregate, beneficially own shares representing approximately 67% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs, even though some of these persons or entities may have interests different than yours. For example, these stockholders, if they choose to act together, could:

- approve a merger, consolidation of sale of all or substantially all of our assets at a price lower than the price that may be desired by other stockholders;
- delay, defer or prevent a change in control transaction involving us that other stockholders may desire;

- entrench our management and board of directors; or
- pursue strategies that deviate from the interests of other stockholders.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. You will not have the opportunity to influence our decisions on how to use our net proceeds from this offering. The failure by our management to apply these funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have 43,585,221 shares of common stock outstanding based on the number of shares outstanding as of August 31, 2020 after giving effect to the automatic conversion of our preferred stock. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates. The remaining 31,333,643 shares are currently restricted as a result of securities laws or lock-up agreements but will become eligible to be sold at various times after the offering as described in the section of this prospectus titled “Shares eligible for future sale.” J.P. Morgan Securities LLC and Jefferies LLC, in their sole discretion, may release some or all of the securities subject to lock-up agreements at any time, which would allow for earlier sales of shares in the public market.

Moreover, subject to the lock-up agreements described below, beginning 180 days after the completion of this offering, holders of an aggregate of 30,301,767 shares of our common stock will have rights, along with holders of an additional 196,890 shares of our common stock issuable upon exercise of outstanding options, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the “Underwriters” section of this prospectus.

We are an “emerging growth company” and a “smaller reporting company,” and the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies may make our common stock less attractive to investors.

We are an “emerging growth company,” or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We may remain an EGC until the end of the fiscal year in which the fifth anniversary of this

offering occurs, although if the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have annual gross revenues of $1.07 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We also would cease to be an EGC if we issue more than $1.0 billion of non-convertible debt over a three-year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- being permitted to provide only two years of audited financial statements in this prospectus, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s discussion and analysis of financial condition and results of operations” disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

Even after we no longer qualify as an emerging growth company, we may continue to qualify as a smaller reporting company, which would allow us to take advantage of many of the same exemptions from disclosure requirements, including reduced disclosure obligations regarding executive compensation. In addition, if we are a smaller reporting company with less than $100 million in annual revenue, we would not be required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404. In reliance on these exemptions, we have taken advantage of reduced reporting obligations in this prospectus. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an EGC or a smaller reporting company.

We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to take advantage of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either irrevocably elect to “opt out” of such extended transition period or no longer qualify as an EGC. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an EGC or a smaller reporting company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing

requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs, particularly as we hire additional financial and accounting employees to meet public company internal control and financial reporting requirements and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us and our business may be harmed.

Pursuant to Section 404, in our second annual report due to be filed with the SEC after becoming a public company, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC or a smaller reporting company with less than $100 million in annual revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, including through hiring additional financial and accounting personnel, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

Effective internal control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal

deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could harm our business and have a negative effect on the trading price of our stock.

We will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an EGC under the JOBS Act or a smaller reporting company with less than $100 million in annual revenue, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404. We could be an EGC for up to five years. An independent assessment of the effectiveness of our internal control over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal control over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation, which could have a negative effect on the trading price of our stock.

Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.

Upon completion of this offering, we will become subject to certain reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management.

Provisions in our restated certificate of incorporation and our amended and restated bylaws that will become effective upon the closing of this offering may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;

- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our certificate of incorporation or bylaws that will become effective upon the closing of this offering.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our restated certificate of incorporation that will become effective upon the closing of this offering designates the Court of Chancery of the State of Delaware and the federal district courts of the United States of America as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers and employees.

Our restated certificate of incorporation that will become effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders;
- any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; or
- any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine.

These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our restated certificate of incorporation that will become effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States of America shall, to the fullest

extent permitted by law, be the sole and exclusive forum for the resolution of any claims arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

These exclusive forum provisions may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees. If a court were to find the either exclusive forum provision contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur further significant additional costs associated with resolving such action in other jurisdictions, all of which could materially adversely affect our business, financial condition and operating results.

Cautionary note regarding forward-looking statements and industry data

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “continue” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus include, among other things, statements about:

- the initiation, timing, progress and results of our research and development programs and preclinical studies and clinical trials;
- the anticipated timing of the submission of INDs for any product candidates we develop;
- the impact of the ongoing COVID-19 pandemic and our response to it;
- our estimates regarding expenses, future revenue, capital requirements, need for additional financing and the period over which we believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to fund our operating expenses, capital expenditure requirements and debt service obligations;
- our plans to develop and, if approved, subsequently commercialize any product candidates we may develop;
- the timing of and our ability to submit applications for, obtain and maintain regulatory approvals for any product candidates we may develop;
- the potential advantages of our FORCE platform;
- our estimates regarding the potential addressable patient populations for our programs;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and our expectations regarding our ability to obtain and maintain intellectual property protection;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- our expectations related to the use of proceeds from this offering and the sufficiency of such proceeds, together with our existing cash and cash equivalents, to fund our operations;
- the impact of government laws and regulations;
- our competitive position and expectations regarding developments and projections relating to our competitors and any competing therapies that are or become available;
- developments and expectations regarding developments and projections relating to our competitors and our industry;

- our ability to maintain and establish collaborations or obtain additional funding; and
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the “Risk factors” section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments we may make or enter into.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this prospectus are made as of the date of this prospectus, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

This prospectus includes statistical and other industry and market data that we obtained from independent industry publications and research, surveys and studies conducted by independent third parties as well as our own estimates of the prevalence of certain diseases and conditions. The market data used in this prospectus involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the patient population with the potential to benefit from treatment with any product candidates we may develop include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and may fail to accurately reflect the addressable patient population. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

Use of proceeds

We estimate that the net proceeds to us from the sale of 12,251,578 shares of our common stock in this offering will be approximately $213.7 million, or approximately $246.2 million if the underwriters exercise their option to purchase additional shares in full, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

As of June 30, 2020, we had cash and cash equivalents of $11.7 million. In July 2020, we raised an additional $17.5 million in gross proceeds from the sale of 17,500,000 shares of our Series A preferred stock in the third tranche of our Series A preferred stock financing, and in August 2020, we raised an additional $115.7 million in gross proceeds from the sale of 41,159,724 shares of our Series B preferred stock. We currently expect to use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately $225.0 million for continued research and development of our programs, including preclinical studies, IND-enabling studies and clinical trials;
- approximately $30.0 million for continued development and enhancement of our proprietary FORCE platform; and
- the remainder for working capital and other general corporate purposes.

Our expected use of net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the scope, progress, costs and results of our research and development efforts, as well as any collaborations that we may enter into with third parties for any product candidates we may develop, and any unforeseen cash needs. We believe opportunities may exist from time to time to expand our current business through acquisitions of complementary companies, products or technologies. While we have no current agreements, commitments or understandings for any specific acquisitions at this time, we may also use a portion of the net proceeds for these purposes.

We expect that the net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to enable us to submit INDs in each of our DM1, DMD and FSHD programs and achieve proof-of-concept data readouts in our DM1 and DMD programs. The expected net proceeds from this offering will not be sufficient for us to fund any product candidate we may develop through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of any product candidates we may develop. All of our programs are currently in the preclinical stage of development and we have not yet identified a product candidate for any of our programs. In light of the early stage of our programs, the specific allocation of the net proceeds from this offering and our existing cash and cash equivalents to any program will depend on, among other things, the results of our research and development efforts for each program, the timing and success of our preclinical studies in the program and the timing and outcome of regulatory submissions. As a result, we are unable to specify the portion of the net proceeds from this offering and our existing cash and cash equivalents that will be allocated to any specific program.

Based on our current plans, we believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to enable us to fund our operating expenses, capital expenditure requirements and debt service obligations into the second half of 2023. We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

Our management will retain broad discretion over the allocation of the net proceeds from this offering. Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

Dividend policy

We have never declared or paid cash dividends on our common stock since our inception. We currently intend to retain all available funds and any future earnings to fund the development and expansion of our business, and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to declare and pay dividends will be made at the discretion of our board of directors and will depend on then-existing conditions, including our results of operations, financial condition, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant. In addition, our ability to pay cash dividends is currently restricted by the terms of our loan and security agreement with Pacific Western Bank, and future debt or other financing arrangements may contain terms prohibiting or limiting the amount of dividends that may be declared or paid on our common stock.

Capitalization

The following table sets forth our cash and cash equivalents and our capitalization as of June 30, 2020:

- on an actual basis;
- on a pro forma basis to give effect to (i) the sale by us of 17,500,000 shares of Series A preferred stock in July 2020 for gross proceeds of $17.5 million, (ii) the sale by us of 41,159,724 shares of Series B preferred stock in August 2020 for gross proceeds of $115.7 million, (iii) the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 28,086,375 shares of common stock upon the closing of this offering and (iv) the filing and effectiveness of our restated certificate of incorporation in connection with the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to (i) our issuance and sale of 12,251,578 shares of common stock in this offering at the initial public offering price of $19.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us and (ii) our payment of a success fee in the amount of $0.5 million to our lender under the terms of a loan agreement.

You should read the information in this table together with our financial statements and the related notes appearing elsewhere in this prospectus and the "Selected financial data" and "Management's discussion and analysis of financial condition and results of operations" sections of this prospectus.

	As of June 30, 2020		
(in thousands, except share and per share data)	**Actual**	**Pro forma**	**Pro forma as adjusted**
Cash and cash equivalents	$ 11,672	$ 144,872	$ 358,107
Long-term debt—net of unamortized debt discount	$ 9,949	$ 9,949	$ 9,949
Stockholders' equity (deficit):			
Convertible preferred stock, $0.0001 par value per share; 52,000,000 shares authorized, 34,500,000 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	29,401	—	—
Preferred stock, $0.0001 par value; no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized, no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, $0.0001 par value per share; 70,000,000 shares authorized, 3,239,770 shares issued and 2,728,365 shares outstanding, actual; 200,000,000 shares authorized, 31,326,145 shares issued and 30,814,740 shares outstanding, pro forma; 200,000,000 shares authorized and 43,577,723 shares issued and 43,066,318 shares outstanding, pro forma as adjusted	1	3	4
Additional paid-in capital	6,493	169,092	382,776
Accumulated deficit	(36,614)	(36,614)	(36,884)
Total stockholders' equity (deficit)	(719)	132,481	345,896
Total capitalization	$ 9,230	$ 142,430	$ 355,845

The table above is based on 3,239,770 shares of our common stock outstanding as of June 30, 2020, which includes 511,405 shares of unvested restricted stock subject to forfeiture, and does not include:

- 1,756,854 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2020 under our 2018 Plan at a weighted average exercise price of $1.06 per share (which does not include stock options to purchase an aggregate of 3,454,806 shares of common stock, at an exercise price of $5.54 per share, that were granted subsequent to June 30, 2020);
- 288,220 shares of common stock available for future issuance as of June 30, 2020 under our 2018 Plan (which does not account for amendments to the 2018 Plan increasing the number of shares available for future issuance by 5,092,779 shares of common stock that were approved by our board of directors and stockholders subsequent to June 30, 2020 and stock options to purchase an aggregate of 3,454,806 shares of common stock, at an exercise price of $5.54 per share, that were granted subsequent to June 30, 2020);
- 2,955,746 additional shares of common stock available for future issuance under the 2020 Plan, as well as any shares which may be reserved pursuant to provisions in the 2020 Plan that automatically increase the number of shares of common stock reserved for issuance under the 2020 Plan, of which we have granted stock options to purchase an aggregate of 1,151,454 shares of common stock, at an exercise price per share equal to the initial public offering price, and restricted stock units for an aggregate of 375,137 shares of common stock to certain of our employees, in connection with this offering; and
- 488,414 additional shares of common stock available for future issuance under the 2020 ESPP, as well as any shares which may be reserved pursuant to provisions in the 2020 ESPP that automatically increase the number of shares of common stock reserved for issuance under the 2020 ESPP.

Dilution

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book value (deficit) as of June 30, 2020 was $(0.7) million, or $(0.22) per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities. Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the 3,239,770 shares of our common stock outstanding as June 30, 2020, which includes 511,405 shares of unvested restricted stock subject to forfeiture.

Our pro forma net tangible book value as of June 30, 2020 was $132.5 million, or $4.23 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, and gives effect to (i) the sale by us of 17,500,000 shares of Series A preferred stock in July 2020 for gross proceeds of $17.5 million, (ii) the sale by us of 41,159,724 shares of Series B preferred stock in August 2020 for gross proceeds of $115.7 million and (iii) the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 28,086,375 shares of common stock upon the closing of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of June 30, 2020, after giving effect to the pro forma adjustments described above.

After giving further effect to our issuance and sale of 12,251,578 shares of our common stock in this offering at the initial public offering price of $19.00 per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us and our payment of a success fee in the amount of $0.5 million to our lender under the terms of a loan agreement, our pro forma as adjusted net tangible book value as of June 30, 2020 would have been $345.9 million, or $7.94 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $3.71 to existing stockholders and an immediate dilution of $11.06 in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Initial public offering price per share		$19.00
Historical net tangible book value (deficit) per share as of June 30, 2020	$(0.22)	
Increase per share attributable to the pro forma adjustments described above	4.45	
Pro forma net tangible book value per share as of June 30, 2020	4.23	
Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing common stock in this offering	3.71	
Pro forma as adjusted net tangible book value per share after this offering		7.94
Dilution per share to new investors purchasing common stock in this offering		$11.06

If the underwriters exercise their option to purchase additional shares in full, our pro forma as adjusted net tangible book value per share after this offering would be $8.33, representing an immediate increase in pro forma as adjusted net tangible book value per share of $4.10 to existing stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of $10.67 to new investors purchasing common stock in this offering at the initial public offering price of $19.00 per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, as of June 30, 2020, on the pro forma as adjusted basis described above, the total number of shares of common stock purchased from us on an as converted to common stock basis, the total consideration paid or to be paid, and the average price per share paid or to be paid by existing stockholders and by new investors in this offering at the initial public offering price of $19.00 per share, before deducting underwriting discounts and commissions and estimated offering expenses payable by us. As the table shows, new investors purchasing common stock in this offering will pay an average price per share substantially higher than our existing stockholders paid.

	Shares purchased		Total consideration		Average price
	Number	Percentage	Amount	Percentage	Per share
Existing stockholders	31,326,145	71.9 %	$167,706,535	41.9%	$ 5.35
New investors	12,251,578	28.1	232,779,982	58.1	19.00
Total	43,577,723	100.0%	$400,486,517	100.0%	

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to 69.0% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors purchasing common stock in this offering would be increased to 31.0% of the total number of shares of our common stock outstanding after this offering.

The tables and discussion above are based on 3,239,770 shares of our common stock outstanding as of June 30, 2020, which includes 511,405 shares of unvested restricted stock subject to forfeiture, and does not include:

- 1,756,854 shares of common stock issuable upon the exercise of stock options outstanding as of June 30, 2020 under our 2018 Plan at a weighted average exercise price of $1.06 per share (which does not include stock options to purchase an aggregate of 3,454,806 shares of common stock, at an exercise price of $5.54 per share, that were granted subsequent to June 30, 2020);
- 288,220 shares of common stock available for future issuance as of June 30, 2020 under our 2018 Plan (which does not account for amendments to the 2018 Plan increasing the number of shares available for future issuance by 5,092,779 shares of common stock that were approved by our board of directors and stockholders subsequent to June 30, 2020 and stock options to purchase an aggregate of 3,454,806 shares of common stock, at an exercise price of $5.54 per share, that were granted subsequent to June 30, 2020);
- 2,955,746 additional shares of common stock available for future issuance under the 2020 Plan, as well as any shares which may be reserved pursuant to provisions in the 2020 Plan that automatically increase the number of shares of common stock reserved for issuance under the 2020 Plan, of which we have granted stock options to purchase an aggregate of 1,151,454 shares of common stock, at an exercise price per share equal to the initial public offering price, and restricted stock units for an aggregate of 375,137 shares of common stock to certain of our employees, in connection with this offering; and
- 488,414 additional shares of common stock available for future issuance under the 2020 ESPP, as well as any shares which may be reserved pursuant to provisions in the 2020 ESPP that automatically increase the number of shares of common stock reserved for issuance under the 2020 ESPP.

To the extent that outstanding stock options are exercised, new stock options are issued, or we issue additional shares of common stock in the future, there may be further dilution to new investors. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.

Selected financial data

The following tables set forth a summary of our historical financial data as of, and for, the periods ended on the dates indicated. We have derived the statement of operations data for the years ended December 31, 2018 and 2019 and the balance sheet data as of December 31, 2018 and 2019 from our audited financial statements appearing elsewhere in this prospectus. The statement of operations data for the six months ended June 30, 2019 and 2020 and the balance sheet data as of June 30, 2020 have been derived from our unaudited condensed financial statements appearing elsewhere in this prospectus and have been prepared on the same basis as the audited financial statements, and, in the opinion of management, reflect all adjustments, consisting only of normal, recurring adjustments, necessary for the fair presentation of those unaudited interim financial statements. You should read the following selected financial data together with our financial statements and the related notes appearing elsewhere in this prospectus and the "Management's discussion and analysis of financial condition and results of operations" section of this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future, and the results for the six months ended June 30, 2020 are not necessarily indicative of results to be expected for the full year ending December 31, 2020 or any other period.

	Year ended December 31,		Six months ended June 30,	
(in thousands, except share and per share data)	**2018**	**2019**	**2019**	**2020**
Statement of operations data:				
Operating expenses:				
Research and development	$ 4,278	$ 11,040	$ 3,799	$ 13,423
General and administrative	517	2,786	927	3,105
Total operating expenses	4,795	13,826	4,726	16,528
Loss from operations	(4,795)	(13,826)	(4,726)	(16,528)
Other income (expense):				
Interest income	5	290	113	24
Interest expense	—	—	—	(184)
Change in fair value of preferred stock tranche obligations	(21)	(1,323)	1,029	—
Change in success fee obligation	—	—	—	(180)
Total other (expense) income, net	(16)	(1,033)	1,142	(340)
Net loss	$ (4,811)	$ (14,859)	$ (3,584)	$ (16,868)
Net loss per share—basic and diluted(1)	$ (10.15)	$ (6.08)	$ (1.52)	$ (6.31)
Weighted average number of common shares used in computing net loss per share—basic and diluted(1)	474,118	2,442,872	2,355,066	2,675,260
Pro forma net loss per share—basic and diluted(1)		$ (1.31)		$ (1.32)
Pro forma weighted average number of common shares used in computing pro forma net loss per share—basic and diluted(1)		10,324,875		12,818,114

(1) See Note 12 to our financial statements appearing elsewhere in this prospectus for details on the calculation of basic and diluted net loss per share and the calculation of basic and diluted pro forma net loss per share.

(in thousands)	As of December 31, 2018	As of December 31, 2019	As of June 30, 2020
Balance sheet data:			
Cash and cash equivalents	$ 8,124	$ 14,632	$ 11,672
Working capital(1)	7,447	12,401	7,747
Total assets	8,268	16,436	13,571
Long-term debt—net of unamortized debt discount	—	—	9,949
Preferred stock tranche obligations	3,375	—	—
Redeemable convertible preferred stock	9,061	—	—
Convertible preferred stock	—	27,429	29,401
Accumulated deficit	(4,887)	(19,746)	(36,614)
Total stockholders' equity (deficit)	(4,879)	14,036	(719)

(1) We define working capital as current assets less current liabilities.

Management's discussion and analysis of financial condition and results of operations

You should read the following discussion and analysis of our financial condition and results of operations together with the "Selected Financial Data" section of this prospectus and our financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. Please also see the "Cautionary note regarding forward-looking statements and industry data" section of this prospectus.

Overview

We are building a leading muscle disease company focused on advancing innovative life-transforming therapeutics for patients with genetically driven diseases. We are utilizing our proprietary FORCE platform to overcome the current limitations of muscle tissue delivery and advance modern oligonucleotide therapeutics for muscle diseases. Our proprietary FORCE platform therapeutics consist of an oligonucleotide payload that we rationally design to target the genetic basis of the disease we are seeking to treat, a clinically validated linker and an antigen-binding fragment, or Fab, that we attach to the payload using the linker. With our FORCE platform, we have the flexibility to deploy different types of oligonucleotide payloads with specific mechanisms of action that modify target functions. We leverage this modularity to focus on muscle diseases with high unmet need, with etiologic targets and with clear translational potential from preclinical disease models to well-defined clinical development and regulatory pathways. Using our FORCE platform, we are assembling a broad portfolio of muscle disease therapeutics, including our lead programs in myotonic dystrophy type 1, or DM1, Duchenne muscular dystrophy, or DMD, and facioscapulohumeral dystrophy, or FSHD. In addition, we plan to expand our portfolio through development efforts focused on rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases, including some with larger patient populations. Our programs are currently all in the preclinical stage. We expect to submit investigational new drug, or IND, applications to the U.S. Food and Drug Administration, or FDA, for product candidates in each of our DM1, DMD and FSHD programs between the fourth quarter of 2021 and the fourth quarter of 2022.

We were incorporated and commenced operations in 2017. Since our incorporation, we have devoted substantially all of our financial resources and efforts to organizing and staffing our company, business planning, raising capital, conducting research activities and filing and prosecuting patent applications. We do not have any products for sale and have not generated any revenue from product sales or otherwise. To date, we have principally raised capital through sales of equity securities and borrowings under a loan and security agreement, or loan agreement, with a commercial bank.

We have incurred significant net losses since inception. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more product candidates. Our net losses were $14.9 million for the year ended December 31, 2019 and $16.9 million for the six months ended June 30, 2020. As of June 30, 2020, we had an accumulated deficit of $36.6 million. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We expect that our expenses and capital requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- continue our current research programs and conduct additional research programs;

- advance any product candidates we identify through our research programs into IND-enabling studies and clinical trials;
- expand the capabilities of our proprietary FORCE platform;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- establish manufacturing sources for any product candidate we may develop, including the Fab antibody, Val-cit linker and therapeutic payload that will comprise the product candidate, and secure supply chain capacity to provide sufficient quantities for preclinical and clinical development and commercial supply;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development and future commercialization efforts, as well as to support our operations as a public company.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for any product candidates we may develop. If we obtain regulatory approval for or otherwise commercialize any product candidates we may develop, we expect to incur significant expenses related to developing our commercialization capabilities to support product sales, marketing and distribution. Further, following this offering, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed, on favorable terms, or at all. If we fail to raise capital or enter into such agreements or arrangements as and when needed, we may have to significantly delay, reduce or eliminate the development or future commercialization of one or more product candidates we may develop.

Because of the numerous risks and uncertainties associated with product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to raise capital, maintain our research and development efforts, expand our business or continue our operations at planned levels, and as a result we may be forced to substantially reduce or terminate our operations.

As of June 30, 2020, our cash and cash equivalents totaled approximately $11.7 million. In July 2020, we raised an additional $17.5 million in gross proceeds from the sale of 17,500,000 shares of our Series A preferred stock in the third tranche of our Series A preferred stock financing, and in August 2020, we raised an additional $115.7 million in gross proceeds from the sale of 41,159,724 shares of our Series B preferred stock. We expect that our existing cash and cash equivalents, together with the anticipated net proceeds from this offering, will enable us to fund our operating expenses, capital expenditure requirements and debt service obligations into the second half of 2023. See “—Liquidity and capital resources.”

Impact of COVID-19 on our business

The worldwide COVID-19 pandemic may affect our ability to initiate and complete preclinical studies, delay the initiation of our future clinical trials, disrupt regulatory activities or have other adverse effects on our business, results of operations, financial condition and prospects. In addition, the pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, both of which could adversely affect our business, operations and ability to raise funds to support our operations.

To date, we have not experienced material business disruptions, including with our vendors, or impairments of any of our assets as a result of the pandemic. We are following, and plan to continue to follow, recommendations from federal, state and local governments regarding workplace policies, practices and procedures. In March 2020, we implemented a remote working policy for many of our employees and began restricting non-essential travel, and on May 18, 2020, when Massachusetts began its staged reopening plan, we began implementing a return-to-work plan, in accordance with the guidance and requirements of federal and state authorities. We expect to continue to take actions as may be required or recommended by government authorities or as we determine are in the best interests of our employees and other business partners. We are continuing to monitor the potential impact of the pandemic, but we cannot be certain what the overall impact will be on our business, financial condition, results of operations and prospects.

Components of our results of operations

Revenue

We have not generated any revenue since our inception and do not expect to generate any revenue from the sale of products in the near future, if at all. If our development efforts are successful and we commercialize products, or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from product sales, as well as upfront, milestone and royalty payments from such collaboration or license agreements, or a combination thereof.

Research and development expenses

Research and development expenses consist primarily of costs incurred for our research activities and development of our programs. These expenses include:

- development and operation of our proprietary FORCE platform;
- employee-related expenses, including salaries, related benefits and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with our research programs, including under agreements with third parties, such as consultants and contract research organizations, or CROs;
- the cost of laboratory supplies and acquiring, developing and manufacturing materials for use in our research and preclinical studies, including under agreements with third parties, such as consultants and contract manufacturing organizations, or CMOs;
- facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance; and
- costs related to compliance with regulatory requirements.

We expense research and development costs as incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our direct external research and development expenses consist of costs that include fees, reimbursed materials and other costs paid to consultants, contractors, CMOs and CROs in connection with our development and manufacturing activities. We do not allocate our research and development costs to specific programs because costs are deployed across multiple programs and our platform and, as such, are not separately classified.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. Accordingly, we expect that our research and development expenses will increase substantially in connection with our preclinical and clinical development activities in the future as we advance our program candidates into IND-enabling studies and clinical trials. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any product candidates we may develop. The successful development of any product candidate is highly uncertain. This is due to the numerous risks and uncertainties associated with product development, including the following:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of programs we decide to pursue and their regulatory paths to market;
- the need to raise funding to complete preclinical and clinical development of any product candidates we may develop;
- our ability to establish new licensing or collaboration arrangements and the progress of the development efforts of third parties with whom we may enter into such arrangements;
- our ability to maintain our current research and development programs and to establish new programs;
- the successful initiation, enrollment and completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the FDA or any comparable foreign regulatory authority;
- the receipt and related terms of regulatory approvals from applicable regulatory authorities for any product candidates;
- the availability of specialty raw materials for use in production of any product candidate we may develop;
- establishing agreements with third-party manufacturers for supply of product candidate components for our clinical trials;
- our ability to obtain and maintain patents, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- our ability to protect our other rights in our intellectual property portfolio;
- commercializing product candidates, if and when approved, whether alone or in collaboration with others; and
- obtaining and maintaining third-party insurance coverage and adequate reimbursement for any approved products.

A change in the outcome of any of these variables with respect to the development of any product candidate we may develop could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any product candidate we may develop.

General and administrative expenses

General and administrative expenses consist primarily of employee-related expenses, including salaries, related benefits and stock-based compensation, for employees in executive, finance, corporate and business development and administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters; professional fees for accounting, auditing, tax and administrative consulting services; insurance costs; administrative travel expenses; and facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our growth strategy. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses associated with our operations as a public company. In addition, if we obtain regulatory approval for a product candidate and do not enter into a third-party commercialization collaboration, we expect to incur significant expenses related to building a sales and marketing team to support product sales, marketing and distribution activities.

Interest income

Interest income consists of interest earned on our cash in bank accounts and cash equivalents, which consist of money market funds. We expect our interest income to increase as we invest the cash received from the net proceeds from this offering.

Interest expense

Interest expense consists of interest on outstanding borrowings under our loan agreement as well as amortization of debt discount and debt issuance costs.

Change in fair value of preferred stock tranche obligations

Under the terms of the stock purchase agreement that we entered into with purchasers of our Series A preferred stock in November 2018, we were contingently obligated to sell, and the purchasers were contingently obligated to purchase, additional shares of Series A preferred stock upon the achievement of specified milestones.

These contingent rights and obligations, which we refer to as the Tranche Rights, were legally detachable and separately exercisable from the Series A preferred stock. Therefore, we allocated the proceeds from the initial closing of the Series A preferred stock financing in November 2018 between the Tranche Rights (as a liability on the balance sheet) and the Series A preferred stock sold at the initial closing (within equity on the balance sheet). The Tranche Rights were subsequently remeasured at their fair value at the end of each reporting period, with changes in fair value recorded within other income (expense) on the statement of operations.

In September 2019, the Tranche Rights ceased to be remeasured at fair value at the end of each reporting period.

Change in success fee obligation

Our loan agreement requires us to pay a success fee upon the occurrence of a specified liquidity event, as defined in the agreement, which includes this offering. At inception of the loan agreement, this contingent obligation was considered immaterial. The fair value of this liability is remeasured at each reporting date, with changes in fair value recorded as a component of other income (expense) on the statement of operations. Upon the closing of this offering, the success fee will be paid and the remaining liability will be eliminated.

Income taxes

Since our inception, we have not recorded any U.S. federal or state income tax benefits for the net losses we have incurred in any year or for our earned research and development tax credits, due to the uncertainty of realizing a benefit from those items. As of December 31, 2019, we had federal net operating loss carryforwards of $17.7 million and state net operating loss carryforwards of $17.7 million. The federal net operating loss carryforwards are indefinite lived and the state net operating loss carryforwards begin to expire in 2038. As of December 31, 2019, we also had federal and state research and development tax credit carryforwards of $0.4 million and $0.2 million, respectively, which begin to expire in 2038 and 2033, respectively.

Results of operations

Comparison of the six months ended June 30, 2019 and 2020

The following table summarizes our results of operations for the six months ended June 30, 2019 and 2020:

	Six months ended June 30,		
(in thousands)	**2019**	**2020**	**Change**
Operating expenses:			
Research and development	$ 3,799	$ 13,423	$ 9,624
General and administrative	927	3,105	2,178
Total operating expenses	4,726	16,528	11,802
Loss from operations	(4,726)	(16,528)	(11,802)
Other income (expense):			
Interest income	113	24	(89)
Interest expense	—	(184)	(184)
Change in fair value of preferred stock tranche obligations	1,029	—	(1,029)
Change in success fee obligation	—	(180)	(180)
Total other income (expense), net	1,142	(340)	(1,482)
Net loss	$(3,584)	$(16,868)	$(13,284)

Research and development expenses

The following table summarizes our research and development expenses for the six months ended June 30, 2019 and 2020:

	Six months ended June 30,		
(in thousands)	**2019**	**2020**	**Change**
Personnel-related	$ 605	$ 2,933	$ 2,328
Stock-based compensation expense	—	55	55
Laboratory supplies and research materials	1,381	3,530	2,149
External manufacturing and research	780	5,411	4,631
Professional and consulting fees	810	520	(290)
Facility-related and other	223	974	751
Total research and development expenses	$3,799	$13,423	$ 9,624

Research and development expenses increased from $3.8 million for the six months ended June 30, 2019 to $13.4 million for the six months ended June 30, 2020. The increase in personnel-related costs was primarily due to increased headcount in our research and development function. The increases in facility-related and other expenses and in costs for laboratory supplies and research materials were primarily due to the increased costs of supporting a larger group of research and development personnel and their research efforts. The increase in external manufacturing and research costs was primarily due to the development of our manufacturing process, increased research activity and the advancement of our programs.

General and administrative expenses

The following table summarizes our general and administrative expenses for the six months ended June 30, 2019 and 2020:

	Six months ended June 30,		
(in thousands)	**2019**	**2020**	**Change**
Personnel-related	$ 407	$ 1,087	$ 680
Stock-based compensation expense	5	86	81
Professional and consulting fees	381	1,548	1,167
Facility-related and other	134	384	250
Total general and administrative expenses	$ 927	$ 3,105	$ 2,178

General and administrative expenses increased from $0.9 million for the six months ended June 30, 2019 to $3.1 million for the six months ended June 30, 2020. The increase in personnel-related costs was primarily the result of an increase in headcount in our general and administrative function. Professional and consulting fees increased primarily due to accounting, audit and legal services as well as costs associated with ongoing business activities and our preparations to operate as a public company.

Interest income

Interest income for the six months ended June 30, 2019 and 2020, was $0.1 million and $24,000, respectively, and was the result of interest earned on invested cash balances.

Interest expense

Interest expense for the six months ended June 30, 2020 was $0.2 million due to interest incurred on outstanding borrowings under our loan agreement. We did not incur any interest expense in the prior year period.

Change in fair value of preferred stock tranche obligations

During the six months ended June 30, 2019, we recorded other income of $1.0 million from a decrease in the fair value of our preferred stock tranche obligations resulting from the decrease in the fair value of the underlying Series A preferred stock. The remaining liability was reclassified to permanent equity in September 2019, and the obligations were no longer required to be remeasured.

Change in success fee obligation

The fair value of the success fee obligation included in our loan agreement increased during the six months ended June 30, 2020 by $0.2 million.

Comparison of the years ended December 31, 2018 and 2019

The following table summarizes our results of operations for the years ended December 31, 2018 and 2019:

(in thousands)	Year ended December 31, 2018	2019	Change
Operating expenses:			
Research and development	$ 4,278	$ 11,040	$ 6,762
General and administrative	517	2,786	2,269
Total operating expenses	4,795	13,826	9,031
Loss from operations	(4,795)	(13,826)	(9,031)
Other income (expense):			
Interest income	5	290	285
Change in fair value of preferred stock tranche obligations	(21)	(1,323)	(1,302)
Total other expense, net	(16)	(1,033)	(1,017)
Net loss	$ (4,811)	$ (14,859)	$ (10,048)

Research and development expenses

The following table summarizes our research and development expenses for the years ended December 31, 2018 and 2019:

(in thousands)	Year ended December 31, 2018	2019	Change
Personnel-related	$ 1,136	$ 2,239	$ 1,103
Stock-based compensation expense	—	12	12
Laboratory supplies and research materials	960	3,464	2,504
External manufacturing and research	702	2,296	1,594
Professional and consulting fees	1,150	1,719	569
Facility-related and other	330	1,310	980
Total research and development expenses	$ 4,278	$ 11,040	$ 6,762

Research and development expenses increased from $4.3 million for the year ended December 31, 2018 to $11.0 million for the year ended December 31, 2019. The increase in personnel-related costs was primarily due to increased headcount in our research and development function. The increases in facility-related and other expenses and in costs for laboratory supplies and research materials were primarily due to the increased costs of supporting a larger group of research and development personnel and their research efforts. The increase in external manufacturing and research costs was primarily due to the development of our manufacturing process, increased research activity and the advancement of our programs.

General and administrative expenses

The following table summarizes our general and administrative expenses for the years ended December 31, 2018 and 2019:

	Year ended December 31,		
(in thousands)	**2018**	**2019**	**Change**
Personnel-related	$ 66	$ 1,177	$ 1,111
Stock-based compensation expense	—	13	13
Professional and consulting fees	413	1,090	677
Facility-related and other	38	506	468
Total general and administrative expenses	$ 517	$ 2,786	$ 2,269

General and administrative expenses increased from $0.5 million for the year ended December 31, 2018 to $2.8 million for the year ended December 31, 2019. The increase in personnel-related costs was primarily due to the 2019 hiring of executive officers and additional personnel in our general and administrative functions as we continued to expand our operations to support the organization. The increase in professional and consulting fees was primarily due to increased legal costs incurred in connection with maintaining and registering worldwide patents and costs associated with our ongoing business operations, as well as higher accounting and consulting costs. The increase in facility-related and other expenses was primarily due to higher facilities costs resulting from entering into a sublease for laboratory and office space in mid-2019.

Interest income

Interest income for the year ended December 31, 2019 was $0.3 million due to interest earned on invested cash balances. Interest income for the prior year was immaterial due to our lower cash balance.

Change in fair value of preferred stock tranche obligations

During the years ended December 31, 2018 and 2019, we recorded other expense of $21,000 and $1.3 million, respectively, from increases in fair value of our preferred stock tranche obligations during the respective periods. The increases in the fair value of the liability resulted from increases in the fair value of the underlying Series A preferred stock during the periods.

Liquidity and capital resources

Sources of liquidity

To date, we have funded our operations primarily with proceeds from sales of equity securities and borrowings under our loan agreement. Through June 30, 2020, we had received net proceeds of $5.0 million from instruments convertible into our Series A preferred stock (which converted into Series A preferred stock in 2018), $29.4 million from sales of our convertible preferred stock and $10.0 million from borrowings under our loan agreement. As of June 30, 2020, $10.0 million remained outstanding and no amounts were available for borrowing under the loan agreement. As of June 30, 2020, we had cash and cash equivalents of $11.7 million. In July 2020, we raised an additional $17.5 million in gross proceeds from the sale of 17,500,000 shares of our Series A preferred stock in the third tranche of our Series A preferred stock financing, and in August 2020, we raised an additional $115.7 million in gross proceeds from the sale of 41,159,724 shares of our Series B preferred stock.

Cash flows

The following table summarizes our sources and uses of cash for each of the periods presented:

	Year ended December 31,		Six months ended June 30,	
(in thousands)	**2018**	**2019**	**2019**	**2020**
Net cash used in operating activities	$ (4,164)	$ (11,834)	$ (4,844)	$ (14,599)
Net cash used in investing activities	(134)	(1,647)	(181)	(282)
Net cash provided by financing activities	12,422	19,989	19,989	11,921
Net increase (decrease) in cash and cash equivalents	$ 8,124	$ 6,508	$ 14,964	$ (2,960)

Operating activities

During the six months ended June 30, 2020, operating activities used $14.6 million of cash, due to our net loss of $16.9 million, partially offset by non-cash charges of $0.7 million and net cash provided by changes in our operating assets and liabilities of $1.6 million. Net cash provided by changes in our operating assets and liabilities primarily consisted of a $1.7 million increase in accounts payable and other liabilities, partially offset by a $0.1 million increase in prepaid expenses and other current assets.

During the six months ended June 30, 2019, operating activities used $4.8 million of cash, due to our net loss of $3.6 million, as well as a decrease of $1.0 million in the fair value of our preferred stock tranche obligations and net cash outflows of $0.2 million resulting from changes in our operating assets and liabilities. Net cash outflows from changes in our operating assets and liabilities primarily consisted of a $0.3 million increase in prepaid expenses and other current assets, partially offset by a $0.1 million increase in accrued expenses and other liabilities.

During the year ended December 31, 2019, operating activities used $11.8 million of cash, due to our net loss of $14.9 million, partially offset by non-cash charges of $1.6 million and net cash provided by changes in our operating assets and liabilities of $1.4 million. Net cash provided by changes in our operating assets and liabilities primarily consisted of a $1.7 million increase in accounts payable and other liabilities, partially offset by a $0.3 million increase in prepaid expenses and other current assets.

During the year ended December 31, 2018, operating activities used $4.2 million of cash, due to our net loss of $4.8 million, partially offset by net cash provided by changes in our operating assets and liabilities of $0.6 million, consisting primarily of an increase in accounts payable and other liabilities.

Changes in our operating assets and liabilities during these periods were generally due to growth in our business, the advancement of our research programs and the timing of vendor invoices and payments.

Investing activities

During the six months ended June 30, 2020 and 2019, net cash used in activities was $0.3 million and $0.2 million, respectively, consisting solely of purchases of property and equipment.

During the years ended December 31, 2019 and 2018, net cash used in activities was $1.6 million and $0.1 million, respectively, consisting solely of purchases of property and equipment.

Financing activities

During the six months ended June 30, 2020, net cash provided by financing activities was $11.9 million, consisting of $2.0 million in proceeds from our issuance of additional shares of Series A preferred stock and borrowings of $10.0 million under our loan agreement.

During the six months ended June 30, 2019 and year ended December 31, 2019, net cash provided by financing activities was $20.0 million, consisting solely of the proceeds from our issuance of Series A preferred stock.

During the year ended December 31, 2018, net cash provided by financing activities was $12.4 million, consisting of $5.0 million in proceeds from our issuance of securities that were later converted to Series A preferred stock and $7.4 million in proceeds from our issuance of Series A preferred stock.

Loan and security agreement

In February 2020, we entered into the loan agreement with Pacific Western Bank, under which we borrowed an aggregate principal amount of $10.0 million in the form of a term loan. Borrowings under the loan agreement are collateralized by substantially all of our assets, excluding intellectual property.

Interest on the outstanding loan balance accrues at a variable annual rate equal to the greater of (i) the bank's prime rate plus 0.25% and (ii) 5.00%. The interest rate was 5.00% at June 30, 2020. We are required to make interest-only payments on the loan on a monthly basis through August 2021. Subsequent to the interest-only period, we are required to make equal monthly payments of principal plus interest until the loan matures in February 2024. We incurred fees associated with establishing the loan facility of $0.1 million. We have an option to prepay the loan in full without a fee. In the event of a specified liquidation event, including this offering, we will be required to pay the bank a success fee of $0.5 million. The loan agreement contains customary representations, warranties and covenants and also includes customary events of default, including payment defaults, breaches of covenants, change of control and occurrence of a material adverse effect. There are no financial covenants associated with the loan agreement.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance our research programs into preclinical and clinical development. In addition, following the closing of this offering, we expect to incur additional costs associated with operating as a public company. The timing and amount of our operating expenditures will depend largely on:

- the identification of additional research programs and product candidates;
- the scope, progress, costs and results of preclinical and clinical development of any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- changes in laws or regulations applicable to any product candidates we may develop, including but not limited to clinical trial requirements for approvals;
- the cost and timing of obtaining materials to produce adequate product supply for any preclinical or clinical development of any product candidate we may develop;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidate we may develop for which we obtain marketing approval;
- the legal costs involved in prosecuting patent applications and enforcing patent claims and other intellectual property claims;

- additions or departures of key scientific or management personnel;
- our ability to establish and maintain collaborations on favorable terms, if at all, as well as the costs and timing of any collaboration, license or other arrangement, including the terms and timing of any milestone payments thereunder; and
- the costs of operating as a public company.

We believe that the anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses, capital expenditure requirements and debt service obligations into the second half of 2023. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and marketing, distribution or licensing arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed, on favorable terms, or at all. If we fail to raise capital or enter into such agreements other arrangements as and when needed, we may have to significantly delay, reduce or eliminate the development or future commercialization of one or more of our product candidates we may develop. See “Risk factors” for additional risks associated with our substantial capital requirements.

Contractual obligations

The following table summarizes our contractual obligations as of December 31, 2019:

		Payments due by period			
(in thousands)	**Total**	**Less than 1 year**	**1 to 3 Years**	**4 to 5 Years**	**More than 5 years**
Operating lease commitments(1)	$1,576	$ 776	$ 800	$ —	$ —
Total	$1,576	$ 776	$ 800	$ —	$ —

(1) Amounts reflect payments due for our subleased laboratory and office space in Waltham, Massachusetts under an operating sublease agreement that expires in December 2021.

We enter into contracts in the normal course of business with CROs, CMOs and other third parties for preclinical research studies, clinical trials and testing and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancelable by us upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation and in the case of certain arrangements with CROs and CMOs may include non-cancelable fees. These payments are not included in the table above as the amount and timing of such payments are not known.

We have also entered into a license agreement with the University of Mons under which we are obligated to make specified milestone and royalty payments. We have not included future payments under this agreement in

the table of contractual obligations above since the payment obligations under this agreement are contingent upon future events, such as our achievement of specified development, regulatory and commercial milestones, or generating product sales. We are unable to estimate the timing or likelihood of achieving these milestones or generating future product sales. For additional information about our license agreement with the University of Mons and amounts that could become payable in the future under that agreement, see “Business—Intellectual Property—License agreement with the University of Mons.”

Critical accounting policies and significant judgments and estimates

Our financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our financial statements appearing at the end of this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our financial statements.

Accrued research and development expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments, if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with preclinical development activities;
- CROs and investigative sites in connection with research activities; and
- CMOs in connection with the production of research materials.

We measure the expense recognized based on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CMOs and CROs that supply, conduct and manage preclinical studies on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the achievement of specified milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses

accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in changes in estimates that increase or decrease amounts recognized in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Stock-based compensation

We measure stock-based awards based on the fair value on the date of the grant using the Black-Scholes option-pricing model. Compensation expense for those awards is recognized over the requisite service period, which is generally the vesting period of the respective award. We use the straight-line method to record the expense of awards with service-based vesting conditions.

The fair value of each stock-based award is estimated on the date of grant using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions we make for the expected volatility of our common stock, the expected term of our stock awards, the risk-free interest rate for a period that approximates the expected term of our stock awards and our expected dividend yield.

Determination of the fair value of common stock

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each option grant, with input from management, considering our most recently available third-party valuations of common stock and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Our common stock valuations were prepared using either an option pricing method, or OPM, or a hybrid method, both of which used market approaches to estimate our enterprise value. The OPM treats common stock and convertible preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeded the value of the convertible preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock.

The hybrid method is a probability-weighted expected return method, or PWERM, by which the equity value in one or more scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock.

These independent third-party valuations were performed at various dates, which resulted in estimated valuations of our common stock by our board of directors of $0.73 per share as of November 30, 2018, $1.03 per share as of May 31, 2019, $1.33 per share as of April 30, 2020 and $5.54 per share as of July 17, 2020. In addition to considering the results of these third-party valuations, our board of directors considered various objective and subjective factors to determine the fair value of our common stock as of each grant date, including:

- the price at which we sold shares, or expected to sell shares, of our preferred stock and the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant;
- the progress of our research and development programs;
- our stage of development and commercialization and our business strategy;
- external market conditions affecting the biopharmaceutical and biotechnology industries, and trends within those industries;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our common stock and our preferred stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or a sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in our industry.

The assumptions underlying these valuations represented management's best estimates, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could be materially different.

Once a public trading market for our common stock has been established following this offering, it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock will be determined based on the quoted market price of our common stock.

Options granted

We did not grant any options from our inception through December 31, 2018. The following table sets forth by grant date the number of shares subject to options granted from January 1, 2019 through the date of this prospectus, as well as the per share exercise price of the options, the fair value of common stock per share on each grant date, and the per share estimated fair value of the options as of the respective grant dates:

Grant date	Number of shares subject option granted	Per share exercise price of options	Fair value of common stock on grant date	Per share estimated fair value of options
February 26, 2019	101,440	$ 0.73	$ 0.73	$ 0.50
March 27, 2019	3,014	0.73	0.73	0.50
July 26, 2019	56,925	1.03	1.03	0.66
September 6, 2019	32,808	1.03	1.03	0.66
September 12, 2019	120,130	1.03	1.03	0.66
January 6, 2020	1,124,317	1.03	1.03	0.66
February 5, 2020	98,426	1.03	1.03	0.66
March 17, 2020	32,808	1.03	1.03	0.66
March 19, 2020	32,808	1.03	1.03	0.66
June 18, 2020	266,168	1.33	1.33	0.86
July 31, 2020	3,454,806	5.54	5.54	3.45

Stock option and restricted stock unit awards in connection with initial public offering

In August 2020, our board of directors approved, subject to the pricing of this offering, grants of stock options to purchase an aggregate of 1,151,454 shares of common stock, at an exercise price per share equal to the initial public offering price, and restricted stock units for an aggregate of 375,137 shares of common stock to certain of our employees.

Valuation of preferred stock tranche obligations

Under the terms of the stock purchase agreement that we entered into with purchasers of our Series A preferred stock in November 2018, we were contingently obligated to sell, and the purchasers were contingently obligated to purchase, additional shares of Series A preferred stock upon the achievement, or waiver of achievement by the purchasers, of specified research and development milestones. In addition, each purchaser had the separate option to purchase the additional shares of Series A preferred stock at any time prior to the achievement or waiver of achievement of such research and development milestones.

We concluded that these Tranche Rights met the definition of a freestanding financial instrument, as the Tranche Rights were legally detachable and separately exercisable from the Series A preferred stock. Therefore, we allocated the proceeds from the initial closing of the Series A preferred stock financing in November 2018 between the Tranche Rights (as a liability on the balance sheet) and the Series A preferred stock sold at the initial closing (within temporary equity on the balance sheet). As the Series A preferred stock was redeemable upon a deemed liquidation event at the election of the board of directors, which was controlled by the holders of Series A preferred stock, and therefore the potential redemption was outside of our control, we initially classified the Tranche Rights as a liability and recorded the obligation at fair value. The Tranche Rights were subsequently remeasured at their fair value at the end of each reporting period, with changes in fair value recorded within other income (expense) on the statement of operations. The estimated fair value of the Tranche Rights at each reporting date was determined using a probability-weighted present value model

that considers the probability of closing a tranche, the estimated future value of Series A preferred stock at each closing, and the investment required at each closing. Future values were converted to present value using a discount rate appropriate for probability adjusted cash flows.

In April 2019, we sold 20,000,000 shares of Series A preferred stock pursuant to the first of the Tranche Rights, and in connection with the closing of such sale, we reclassified the fair value of a portion of Tranche Rights, in the amount of $1.6 million, to the carrying value of the Series A preferred stock.

In September 2019, the size of our board of directors was increased from five to six members, including three independent members, which allowed us to conclude that our board was no longer holder-controlled and the redemption of the Series A preferred stock was within our control. Based on this determination, the Tranche Rights no longer required liability classification, and we reclassified the fair value of the remaining Tranche Rights, in the amount of $6.3 million, from a liability into permanent equity, after which the remaining Tranche Rights ceased to be remeasured at fair value at the end of each reporting period.

Off-balance sheet arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

Recently issued accounting pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our financial statements appearing at the end of this prospectus.

Quantitative and qualitative disclosures about market risks

Interest rate risk

As of June 30, 2020 and December 31, 2019, we had cash and cash equivalents of $11.7 million and $14.6 million, respectively, which consisted of cash and money market funds. Interest income is impacted by changes in the general level of interest rates; however, an immediate 10% change in interest rates would not have a material effect on the fair value of our cash equivalents.

As of June 30, 2020, we had borrowings of $10.0 million outstanding under the loan agreement with Pacific Western Bank. Outstanding borrowings bear interest at a variable rate equal to the greater of i) 0.25% above the bank's prime rate in effect or ii) 5.00%. An immediate 10% change in the variable interest rate would not have had a material impact on our debt-related obligations, financial position or results of operations.

Foreign currency risk

We are not currently exposed to significant market risk related to changes in foreign currency exchange rates; however, we have contracted with, and may continue to contract with, foreign vendors. Our operations may be subject to fluctuations in foreign currency exchange rates in the future. We do not hedge any foreign currency risks.

Inflation risk

Inflation generally affects us by increasing our cost of labor. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2019 and 2018 or the six months ended June 30, 2020.

Emerging growth company and smaller reporting company status

We are an “emerging growth company,” as defined in the Jumpstart Our Business Startups Act, or JOBS Act, and may take advantage of certain exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies. We may take advantage of these exemptions until we are no longer an “emerging growth company.” Section 107 of the JOBS Act provides that an “emerging growth company” can take advantage of the extended transition period afforded by the JOBS Act for the implementation of new or revised accounting standards. We have elected to use the extended transition period for complying with new or revised accounting standards and as a result of this election, our financial statements may not be comparable to companies that comply with public company effective dates. We may take advantage of these exemptions until December 31, 2025 or until such earlier time that we are no longer an “emerging growth company.”

We are also a “smaller reporting company,” meaning that the market value of our shares held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our shares held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100 million during the most recently completed fiscal year and the market value of our shares held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies.

For additional information, see “Prospectus summary—Implications of being an emerging growth company and a smaller reporting company.”

Business

Overview

We are building a leading muscle disease company focused on advancing innovative life-transforming therapeutics for patients with genetically driven diseases. We are utilizing our proprietary FORCE platform to overcome the current limitations of muscle tissue delivery and advance modern oligonucleotide therapeutics for muscle diseases. Our proprietary FORCE platform therapeutics consist of an oligonucleotide payload that we rationally design to target the genetic basis of the disease we are seeking to treat, a clinically validated linker and an antigen-binding fragment, or Fab, that we attach to the payload using the linker. With our FORCE platform, we have the flexibility to deploy different types of oligonucleotide payloads with specific mechanisms of action that modify target functions. We leverage this modularity to focus on muscle diseases with high unmet need, with etiologic targets and with clear translational potential from preclinical disease models to well-defined clinical development and regulatory pathways. Using our FORCE platform, we are assembling a broad portfolio of muscle disease therapeutics, including our lead programs in myotonic dystrophy type 1, or DM1, Duchenne muscular dystrophy, or DMD, and facioscapulohumeral dystrophy, or FSHD. In addition, we plan to expand our portfolio through development efforts focused on rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases, including some with larger patient populations. Our programs are currently all in the preclinical stage. We expect to submit investigational new drug, or IND, applications to the U.S. Food and Drug Administration, or FDA, for product candidates in each of our DM1, DMD and FSHD programs between the fourth quarter of 2021 and the fourth quarter of 2022.

Oligonucleotide therapeutics are a genetic medicine modality that, using nucleic acids, specifically aims to correct the function of disease-causing genes by either degrading the target gene or modifying expression of a target protein. While some oligonucleotide therapeutics have been approved, the development of oligonucleotide therapeutics has been limited by challenges in the delivery of the oligonucleotide to the tissue that requires therapy. To overcome these limitations, our FORCE platform utilizes the importance of Transferrin 1 receptor, or TfR1, in muscle biology as the foundation of our novel approach of linking therapeutic payloads to our TfR1-binding Fab to deliver targeted therapeutics for muscle diseases. TfR1, which is highly expressed on the surface of muscle cells, is required for iron transport into muscle cells, and evidence to date suggests that there are no other proteins that can substitute for TfR1 function. We believe our FORCE platform may provide several advantages, including targeted delivery to muscle tissue, extended durability, redosable administration and potent targeting of the genetic basis of disease to stop or reverse disease progression.

Our approach

We have designed our proprietary FORCE platform using our deep knowledge of muscle biology and oligonucleotide therapeutics. We have demonstrated proof-of-concept of our FORCE platform in multiple *in vitro* and *in vivo* studies. In murine and non-human primate studies, we have delivered antisense oligonucleotides, or ASOs, and phosphorodiamidate morpholino oligomers, or PMOs, to genetic targets within muscle tissue and observed durable, disease-modifying, functional benefit in preclinical models of disease. For instance, in our DM1 program, we observed almost complete reversal of myotonia after a single dose in the HSA-LR mouse DM1 model and reductions in levels of DMPK in wild-type, or WT, mice for up to 12 weeks after a single dose, and in our DMD program, we observed increased muscle function four weeks after a single dose in the mdx mouse DMD model that mirrored levels of muscle function in a control cohort of healthy, wild-type mice.

Our therapeutics consist of three essential components: a proprietary Fab, a clinically validated linker and an oligonucleotide payload that we attach to our Fab using the linker.

Proprietary antibody (Fab)

Our proprietary Fabs are engineered to bind to TfR1 to enable targeted delivery of nucleic acids and other molecules to skeletal, cardiac and smooth muscle. A Fab is the region of an antibody that binds to antigens. We selected a Fab antibody over monoclonal antibodies, or mAbs, due to its potential significant advantages when targeting TfR1 to enable muscle delivery, including enhanced tissue penetration, increased tolerability due to lower protein load and reduced risk of immune system activation due to the lack of the Fc domain on the Fab. The Fc domain is the portion of an antibody that interacts with the immune system. To identify the proprietary Fab we plan to use in our product candidates, we generated and screened proprietary antibodies for selectivity to TfR1 in order to enhance muscle specificity and for binding to TfR1 without interfering with the receptor's function of transporting iron into cells. These proprietary antibodies were also screened to minimize competition with transferrin binding and interference of iron uptake in targeted muscle cells.

Clinically validated linker

The role of the linker is to connect, or conjugate, the Fab and the oligonucleotide payload. As a result, it is critical that the linker maintain stability in serum and provide release kinetics that favor sufficient payload accumulation in the targeted muscle cell. We have selected the Val-Cit linker as the linker for our FORCE platform based on its clinically validated safety and efficacy in approved products, its serum stability and its endosomal release attributes. Additionally, our linker and conjugation chemistry allow us to optimize the ratio of payload molecules attached to the Fab for each type of payload. We believe that our linker and conjugation chemistry will enable us to rapidly design, produce and screen molecules to enable new muscle disease programs.

Optimized payload

With our FORCE platform, we have the flexibility to deploy different types of therapeutic payloads with specific mechanisms of action that modify target functions. Using this modularity, we rationally select the therapeutic payload for each program to match the biology of the target, with the aim of addressing the genetic basis of disease and stopping or reversing disease progression.

Advantages of our FORCE platform

Our FORCE platform is designed to deliver disease-modifying therapeutics for a broad portfolio of serious muscle diseases. We believe that our FORCE platform may provide the following potential advantages:

- Targeted delivery to muscle tissue;
- Potent targeting of the genetic basis of disease to stop or reverse disease progression;
- Enhanced tolerability;
- Extended durability;
- Redosable administration;
- Well-established and scalable manufacturing; and
- Accelerated and efficient development enabled by use of a single Fab and linker across all of our programs.

Our portfolio

We are building a pipeline of programs to address genetically-driven muscle diseases with high unmet need with etiologic targets. Our initial focus is on DM1, DMD and FSHD with potential pipeline expansion opportunities in additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. In selecting diseases to target with our FORCE platform, we seek diseases with clear translational potential from preclinical disease models to well-defined clinical development and regulatory pathways. We have global commercial rights to all of our programs.

DM1 program overview

Our DM1 program is focused on the development of a potentially disease-modifying treatment for DM1. DM1 is a monogenic, autosomal dominant, progressive disease that affects skeletal, cardiac and smooth muscle, resulting in significant physical, cognitive and behavioral impairments and disability. There are currently no disease-modifying therapies to treat DM1 that are approved or in clinical development. DM1 is caused by an abnormal expansion in a region of the DMPK gene and it is estimated to have a genetic prevalence of 1 in 2,500 to 1 in 8,000 people in the United States and Europe, affecting over 40,000 people in the United States and over 74,000 people in Europe. Our program candidates consist of a proprietary TfR1-binding Fab conjugated using our linker to an ASO that is designed to address the genetic basis of DM1 by reducing the levels of mutant DMPK RNA in the nucleus, releasing splicing proteins, allowing normal mRNA processing and translation of normal proteins, and potentially stopping or reversing disease. In preclinical studies, we have observed reduction of nuclear foci and correction of splicing in DM1 patient cells, reversal of myotonia after a single dose in a DM1 mouse disease model, durability of response up to 12 weeks in WT mice and enhanced muscle distribution as evidenced by reduced levels of cytoplasmic WT DMPK RNA in non-human primates. We expect to submit an IND to the FDA for a product candidate in our DM1 program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

DMD program overview

Our DMD program is focused on the development of potentially disease-modifying treatments for DMD. DMD is a monogenic, X-linked disease caused by mutations in the gene that encodes for the dystrophin protein. In patients with DMD, mutations in the dystrophin gene lead to certain exons being misread resulting in the loss of function of the dystrophin protein, muscle cell death and progressive loss of muscle function. We estimate that DMD occurs in approximately one in every 3,500 to 5,000 live male births and that the patient population is approximately 12,000 to 15,000 in the United States and approximately 25,000 in Europe. We are developing program candidates to address the genetic basis of DMD by delivering a PMO to muscle tissue to promote the

skipping of specific DMD exons in the nucleus, allowing muscle cells to create a more complete, functional dystrophin protein and potentially stop or reverse disease progression. In *in vitro* and *in vivo* preclinical studies, our PMOs when conjugated to a Fab targeting TfR1 have shown increased exon skipping, increased dystrophin expression, reduced muscle damage and increased muscle function. We are seeking to build a DMD franchise by initially focusing on the development of a therapeutic for patients with mutations amenable to skipping Exon 51, to be followed by the development of therapeutics for patients with mutations amenable to skipping other exons, including Exon 53, 45 and 44. We expect to submit an IND to the FDA for a product candidate in our Exon 51 skipping program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

FSHD program overview

Our FSHD program is focused on the development of a potentially disease-modifying therapy for FSHD. FSHD is an autosomal dominant muscular dystrophy characterized by progressive skeletal muscle loss, resulting in significant physical impairments and disability. FSHD is caused by aberrant expression of the double homeobox 4, or DUX4, gene in muscle tissue, which leads to death of muscle and replacement by fat. There are no approved treatments for FSHD. We estimate the patient population is between 16,000 and 38,000 in the United States and approximately 35,000 in Europe. Our FSHD program candidates consist of our proprietary TfR1-binding Fab conjugated using our linker to an ASO that is designed to address the genetic basis of FSHD by reducing DUX4 expression in muscle tissue. In preclinical studies, we observed that administration of our proprietary ASO conjugated to a Fab targeting TfR1 reduced expression of key DUX4 biomarkers in FSHD patient myotubes. We expect to submit an IND to the FDA for a product candidate in our FSHD program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Discovery programs overview

We intend to utilize our FORCE platform to expand our portfolio by pursuing the development of programs in additional indications, including additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. By rationally selecting therapeutic payloads to conjugate with our proprietary Fab and linker, we believe we can develop product candidates to address the genetic basis of additional muscle diseases. We have completed screening and identified potent ASO and siRNA payloads against a number of cardiac and metabolic targets. In addition to our muscle disease portfolio, we believe there is an opportunity to leverage our TfR1 antibody expertise to develop novel antibodies that cross the blood-brain barrier and deliver therapeutics to the central nervous system, or CNS, tissue through systemic intravenous administration.

Our strategy

Our goal is to become the leading muscle disease company by advancing innovative life-transforming therapeutics for genetically driven diseases. To accomplish this, we intend to continue building a team that shares our commitment to patients, to continue to enhance our platform and to advance our pipeline. The key elements of our strategy are to:

- **Advance our lead programs in DM1, DMD and FSHD to clinical proof-of-concept and approval to offer meaningful benefit to patients.** We are developing our lead programs for the treatment of DM1, DMD and FSHD. By applying our FORCE platform, we are able to optimize product candidates for each indication based on extensive preclinical data, including disease-specific models and biomarkers, thus enhancing the probability of clinical success of our programs. Our immediate focus is on our programs for DM1 and DMD followed by our program for FSHD, and we expect to submit INDs to the FDA for product candidates in each of our DM1, DMD and FSHD programs between the fourth quarter of 2021 and the fourth quarter of 2022. We

intend to conduct our clinical studies in a genetically-defined patient population and to leverage learnings from other therapeutics in clinical development or approved by the FDA to inform the clinical and regulatory pathways for our programs. We believe our DM1, DMD and FSHD programs have the potential to stop or reverse the progression of the disease and offer meaningful benefit to patients in need.

- **Establish a DMD franchise by expanding our DMD program to reach additional DMD patient populations.** We are developing our DMD program to treat the genetic basis of DMD. Approximately 80% of patients with DMD have mutations amenable to exon skipping in the nucleus. Exons 51, 53, 45 and 44 represent nearly half of the total mutations observed in DMD that are amenable to exon skipping. We are seeking to build a DMD franchise by initially focusing on the development of a therapeutic for patients with mutations amenable to skipping Exon 51, to be followed by the development of therapeutics for patients with mutations amenable to skipping other exons, including Exons 53, 45 and 44.
- **Expand our pipeline of therapeutics for muscle diseases to fully exploit the potential of our proprietary FORCE platform.** Our FORCE platform leverages the pivotal role played by TfR1 in muscle biology as the foundation of our novel approach of linking therapeutic payloads to TfR1-targeted Fabs to deliver precision therapeutics for muscle diseases. We believe there are many muscle diseases with significant unmet need and we aim to expand our portfolio by pursuing additional programs where our FORCE platform could improve clinical efficacy relative to current therapeutic approaches. We have completed screening and identified potent ASO and siRNA payloads against a number of cardiac and metabolic targets. In addition to our muscle disease portfolio, we believe there is an opportunity to leverage our TfR1 antibody expertise to develop novel antibodies that cross the blood-brain barrier and deliver therapeutics to CNS tissue through systemic intravenous administration.
- **Selectively enter into strategic collaborations to maximize the value of our pipeline and our proprietary FORCE platform.** Given the potential of our platform to generate novel product candidates addressing a wide variety of muscle diseases, we may opportunistically enter into strategic collaborations around certain targets, programs or muscle tissues. We may seek strategic collaborations where we believe we can utilize our FORCE platform to enhance delivery of third-party payloads to muscle tissue. We may also explore collaboration arrangements to commercialize any product candidates where we believe the resources and expertise of the third party could be beneficial. These collaborations could advance and accelerate our programs to maximize their market potential and expand our FORCE platform capabilities.
- **Build a sustainable leadership position in muscle diseases with a deep connection to patients, caregivers, the research community and physicians.** We have global commercial rights to all of our programs and intend to build a fully integrated biotechnology company and independently pursue the development and commercialization of our lead programs. Our mission is to expand our portfolio into a wide range of muscle diseases and become a leader in this area by advancing life-transforming therapeutics for patients with serious muscle diseases. To achieve this, we plan to continue to evaluate and invest in enhancing our platform and technologies that may accelerate the development of our therapeutics, to build out our capabilities to commercialize our therapeutics on our own and to cultivate a strong network with patient advocacy groups and thought leaders.

Our culture and team

We have established a patient-focused culture that drives our shared mission of developing life-transforming therapeutics for patients with serious muscle diseases. Our shared definition of success is simple: we do what we say we are going to do. We keep our commitments to patients, employees and Dyne stakeholders. We endeavor to act with integrity and transparency.

Our management team is led by Joshua Brumm, our President and Chief Executive Officer, who brings over 15 years of leadership experience with life sciences companies; Romesh Subramanian, Ph.D., our Chief Scientific Officer and Founder, who is an expert in nucleic acid, antibody and peptide therapeutic development as well as delivery platforms with 20 years of experience across pharmaceutical and biotechnology companies; Susanna High, our Chief Operating Officer, who has more than two decades of experience leading corporate strategy, portfolio management, business planning and operations for biotechnology companies; and Oxana Beskrovnaya, Ph.D., our Senior Vice President, Head of Research, who has extensive experience in musculoskeletal and renal research. Our organization is comprised of 36 talented individuals with significant experience across discovery, preclinical research, manufacturing, clinical development and operations. We have also established scientific and clinical advisory boards comprised of leading experts in the fields of muscle disease drug discovery and development and nucleic acid therapeutics, who share our mission of delivering disease-modifying therapeutics for patients with serious muscle diseases.

Since our inception through August 31, 2020, we have raised $167.7 million from a syndicate of leading investors, including Atlas Venture, Forbion, MPM Capital, Vida Ventures, Surveyor Capital (a Citadel company), RA Capital, Wellington Management, Logos Capital, Franklin Templeton and CureDuchenne Ventures.

Genetic medicines background

Overview

Each person's genetic material, or genome, consists of deoxyribonucleic acid, or DNA, in sequences of genetic code called genes. A genetic disease is caused by a change, or a mutation, in an individual's DNA sequence. Genetic diseases can be caused by a mutation in a single gene, known as a monogenic disorder, or by mutations in multiple genes, known as a multifactorial inheritance disorder. Current estimates suggest that there are more than 10,000 monogenic diseases. Many of these are rare muscle diseases, affecting thousands of patients worldwide, such as DM1, DMD and FSHD. There are also a number of more prevalent genetic muscle diseases, including many types of cardiac disease.

Genetic medicines are designed to correct disease-causing dysfunction at the genetic level and include multiple therapeutic modalities, such as oligonucleotide therapeutics, including ASOs and siRNAs, viral gene therapy and small molecules. Significant progress has been made in the field of genetic medicine over the last decade as a number of genetic medicines have been approved or are in clinical development. However, the nature and fundamental limitations of these genetic medicines, such as poor tissue specificity, reduced efficacy, unknown durability and immunogenicity, make them poorly suited to effectively address the majority of genetic muscle diseases.

Viral gene therapy, in which viral vectors are employed to deliver therapeutic genes to defective cells or tissues, has made significant progress in the past decade. The most advanced method for systemic administration is adeno-associated virus, or AAV, gene therapy, which has demonstrated durable transduction of cells in several organ systems, with long-lasting expression in non-dividing cells. Several AAV gene therapy products have been approved, including LUXTURNA (voretigene neparvovec-rzyl) for the treatment of biallelic RPE65 mutation-associated retinal dystrophy, a rare inherited blindness disorder, and ZOLGENSMA (onasemnogene abeparvovec-xioi) for spinal muscular atrophy, and a number of AAV gene therapy products are in clinical development for muscle diseases, including microdystrophin gene therapy candidates for DMD.

However, current AAV gene therapy has significant limitations, including:

- *Limited durability*: AAV gene therapies, which are administered in a single dose, have shown limited durability. This limited durability is problematic because following a single dose of AAV, antibodies are induced against the AAV capsid, the protein shell of the virus used for delivery, with the result that the therapy cannot be re-administered after the first dose.

- *Pre-existing immunity*: Up to half of patients have antibodies against AAV due to naturally acquired infections. These antibodies prevent them from receiving AAV gene therapy due to pre-existing AAV immunity to the capsid encapsulating the AAV.
- *Variable safety profile*: Multiple clinical trials have reported significant adverse events associated with systemic administration of AAV to treat muscle diseases, particularly at higher doses.
- *Limited payload capacity*: AAV constructs are limited to 4.7 kb in length, restricting both the size of genes and complexity of regulatory sequences that can be delivered. For example, AAV payload capacity prevents the delivery of the full dystrophin gene. As a result, AAV gene therapies being developed for DMD use a microdystrophin, a smaller, less complete version of the dystrophin protein, as the therapeutic payload.
- *Off-target, multi-tissue delivery*: Due to the inherent nature of AAVs, off-target delivery to unintended tissues and cell-types can lead to adverse events.
- *Limited manufacturing scale*: The production systems for AAV gene therapies are limited in scale to 2,000 liters per batch or less. In general, the high doses required by AAV gene therapies and the low productivity of these systems combine to limit treatment to rare disease populations at a higher cost relative to other treatment modalities.

Evolution of oligonucleotide therapeutics

Oligonucleotide therapeutics are a genetic medicine modality that, using nucleic acids, specifically aims to correct the function of disease-causing genes by either degrading the disease-causing gene or modifying expression of a gene or a protein. Oligonucleotides are rationally designed genetic medicines which have demonstrated clinical benefit and been approved for marketing in multiple diseases, such as spinal muscular atrophy, elevated cholesterol and hereditary transthyretin-mediated amyloidosis. Oligonucleotides are designed based on genomic data, using Watson-Crick base pairing rules, to bind to and decrease or modify the expression of specific disease-causing RNA or proteins which ultimately results in disease modification.

Oligonucleotide therapeutics include single-stranded nucleic acids, known as antisense oligonucleotides or ASOs, as well as double-stranded nucleic acids, known as small interfering RNA, or siRNAs. The ASOs are generally less than 30 nucleobases in length. ASOs can have either charged nucleobases or uncharged bases. ASOs with uncharged bases are referred to as phosphorodiamidate morpholinos oligomers, or PMOs. The charged nucleobases impart an overall negative charge to ASOs that enhances their ability to cross membranes and enter into cells without the assistance of a delivery formulation. Once inside the cell, the charged ASOs can degrade RNA if they are gapmers, which have a DNA region in the center flanked by RNA wings, by recruiting the RNAseH1 enzyme to cleave heteroduplexes of DNA and RNA. ASOs can also alter splicing or exon skipping if they are non-gapmers. ASOs are functional in the nucleus and the cytoplasm. In contrast, a PMO is neutral in charge and unable to enter into cells without a delivery enhancement. PMOs are capable of exon skipping and are functional in the nucleus. In addition to ASOs and PMOs, siRNAs are a third type of oligonucleotide therapy which are generally less than 23 nucleobases in length and are a negatively charged duplex molecule comprised of a guide strand and a passenger strand. In order to modulate RNA or protein expressions, siRNAs need to be loaded into an RNA-induced silencing complex which is generally present in the cytoplasm but not the nucleus.

While some oligonucleotide therapeutics have been approved, the development of oligonucleotide therapeutics has been limited by challenges in the delivery of the oligonucleotide to the tissue that requires therapy. These challenges have been particularly evident in the delivery of oligonucleotides to muscle tissue. Unconjugated, or naked, oligonucleotides bind non-specifically to plasma proteins which increases their plasma half-life and circulation time through filtering organs such as the liver and kidney, leading the oligonucleotides to

accumulate primarily in the liver and kidney, and resulting in increased toxicity in these organs. As a result, many oligonucleotide therapeutics are limited to organs where direct delivery to the target organ can be an effective approach, such as intravitreal administration in the retina or intrathecal administration in the central nervous system. There have been efforts to enhance delivery of siRNAs to tissue by utilizing lipid nanoparticles, or LNPs, and other encapsulation vehicles. Although these approaches can increase the effectiveness of an siRNA therapeutic, they are largely limited to filtering organs such as the liver and kidney and increase the safety risk of the therapeutic. Despite these limitations, both naked ASOs and encapsulated siRNAs have been approved and commercialized.

Subsequent efforts using targeted delivery of ASOs or siRNAs have demonstrated that conjugation to a delivery moiety can effectively deliver these compounds to target tissues and provide significant health benefits to patients. For instance, third-party developers have leveraged proteins on cell surfaces to enhance the delivery of oligonucleotides using a process called receptor-mediated uptake. These developers have conjugated sugar molecules referred to as GalNAcs to oligonucleotides in order to engage asiaglycoprotein, or ASGPR, a transporter protein expressed primarily on the surface of liver cells, and facilitate intracellular delivery of oligonucleotide therapeutics, resulting in increased target engagement as compared to naked oligonucleotides. The emerging preclinical and clinical data around the GalNAc-ASGPR approach and recent FDA approval of GIVLAARI (givosiran), which uses this approach, supports receptor-mediated uptake as a delivery strategy for oligonucleotide therapeutics.

In order to increase the uptake of oligonucleotides, third-party developers have also used antibody conjugates as a means of delivering oligonucleotides into cells. Antibodies are naturally occurring proteins produced by the immune system that first identify and then neutralize or clear antigens, such as bacteria, viruses and other substances, by selectively binding to these foreign substances. Antibodies can be engineered for desired characteristics, such as high selectivity and high affinity for their target cell surface proteins and antibody format, such as mAb or Fab, in order to facilitate the delivery of oligonucleotide therapeutics into those cells. The use of engineered antibodies as a conjugate to oligonucleotide therapeutics is being studied for the delivery of oligonucleotides to muscle tissue and tumors.

Developers have sought to use the TfR1 receptor, which is highly expressed on the surface of muscle cells, to deliver oligonucleotides to muscle tissue. TfR1 is required for iron transport into muscle cells, and evidence to date suggests that there are no other proteins that can substitute for TfR1 function. For instance, in third-party studies, a conditional knock-out of TfR1 in cardiac muscle was lethal in mice, and a conditional knock-out of TfR1 in skeletal muscle resulted in significant metabolic imbalance in mice. These studies provide evidence that TfR1 is critical for muscle function. However, we believe that previous efforts to develop muscle disease therapeutics based on TfR1-mediated delivery have been unsuccessful because receptor-mediated uptake requires the optimization of each component of the conjugate molecule (the antibody, the linker and the oligonucleotide), which has not yet been achieved.

Our FORCE platform utilizes the importance of TfR1 in muscle biology as the foundation of our novel approach of linking therapeutic payloads to TfR1-targeted Fabs to deliver targeted therapeutics for muscle diseases.

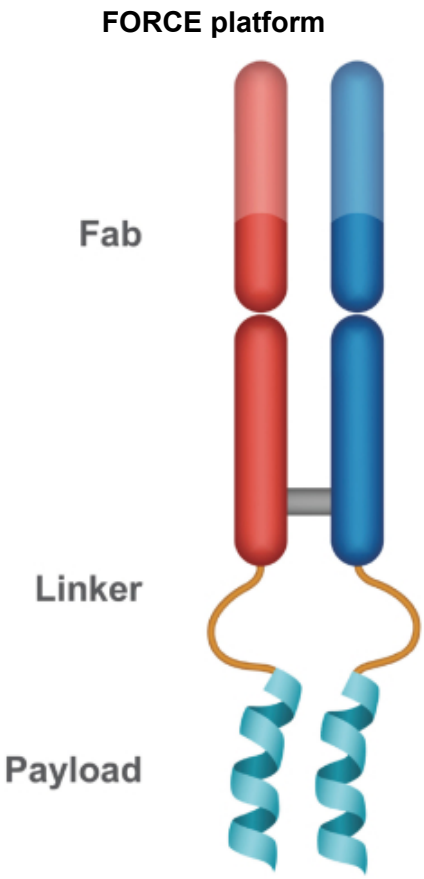
Our approach

We are using our proprietary FORCE platform to develop targeted, life-transforming therapeutics for serious muscle diseases. We have designed our proprietary FORCE platform using our deep knowledge of muscle biology and oligonucleotide therapeutics with the goal of overcoming the current limitations of muscle tissue delivery and advancing modern oligonucleotide therapeutics for muscle diseases. Our therapeutics consist of three essential components: a proprietary Fab, a clinically validated linker and an oligonucleotide payload that we attach to our Fab using the linker. We engineered our proprietary Fab to bind to TfR1 to enable targeted

delivery to skeletal, cardiac and smooth muscle. We selected the linker for our platform based on its clinically validated safety and efficacy in approved products, its serum stability and its ability to release the therapeutic payload within the muscle cell. Finally, we attach the Fab and linker to a therapeutic payload that can be an ASO, siRNA, PMO or small molecule that we rationally select to target the genetic basis of disease to potentially stop or reverse disease progression.

We have demonstrated proof-of-concept of our FORCE platform in multiple *in vitro* and *in vivo* studies. In murine and non-human primate (NHP) studies, we have delivered ASOs and PMOs to genetic targets within muscle tissue and observed durable, disease-modifying, functional benefit in preclinical models of disease. For instance, in our DM1 program, we observed almost complete reversal of myotonia after a single dose in the HSA-LR mouse DM1 model and reductions in levels of DMPK in WT mice for up to 12 weeks after a single dose, and in our DMD program, we observed increased muscle function four weeks after a single dose in the mdx mouse DMD model that mirrored levels of muscle function in a control cohort of healthy, wild-type mice.

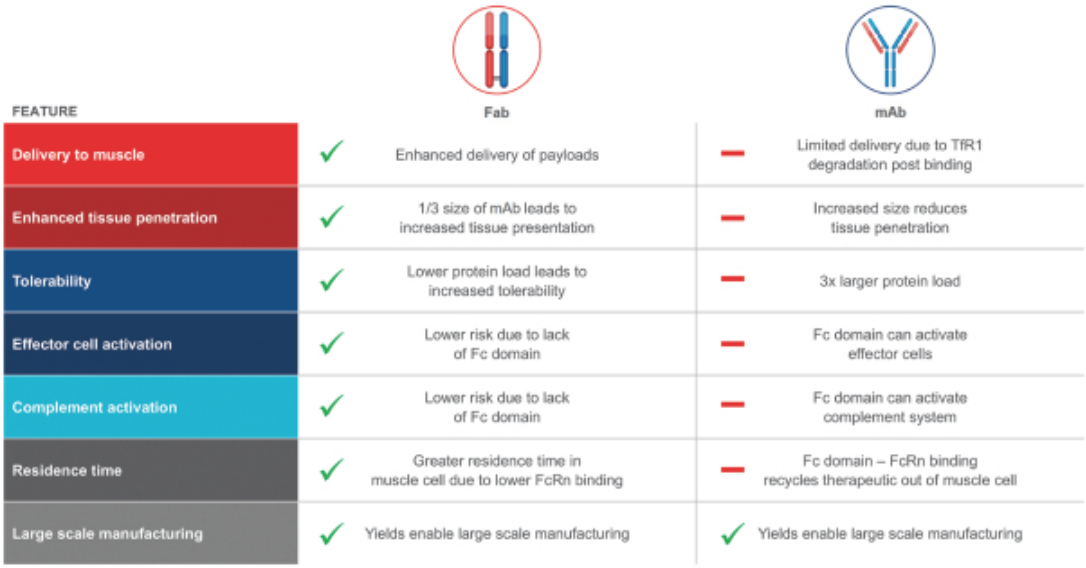
The following graphic illustrates the three components of our therapeutics:



Proprietary antibody (Fab)

Our proprietary Fabs are engineered to bind to TfR1 to enable targeted delivery of nucleic acids and other molecules to skeletal, cardiac and smooth muscle. A Fab is the region of an antibody that binds to antigens. Although we have engineered a number of Fabs, we plan to use the same Fab to target TfR1 across all of our muscle programs. We chose to develop our programs using a Fab rather than using mAbs because we believe Fabs provide the following potential significant advantages:

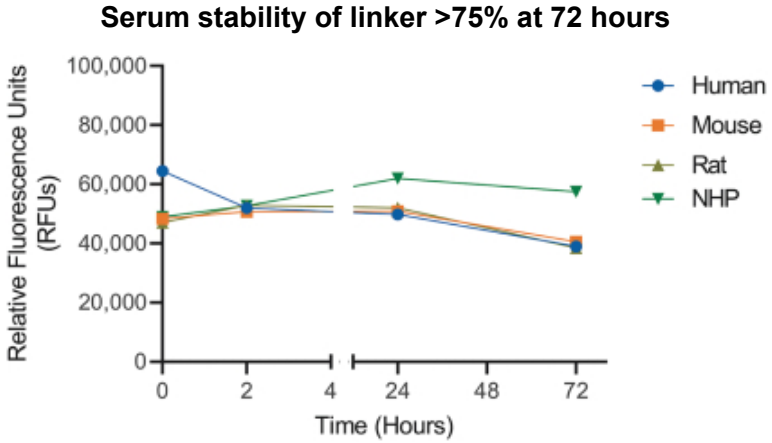
Potential advantages of Fabs targeting TfR1



To identify the proprietary Fab we plan to use in our product candidates, we generated and screened proprietary antibodies for selectivity to TfR1 in order to enhance muscle specificity and for binding to TfR1 without interfering with the receptor's function of transporting iron into cells.

Clinically validated linker

The role of the linker is to connect the Fab and the oligonucleotide therapy. As a result, it is critical that the linker maintain stability in serum and provide release kinetics that favor sufficient payload accumulation in the targeted muscle cell. We have selected the Val-Cit linker for our FORCE platform based on its clinically validated safety and efficacy in approved products, its serum stability and its endosomal release attributes. As shown in the figure below, serum stability of our linker was comparable across multiple species, showing at least 75% stability measured at 72 hours after intravenous dosing.



We believe that serum stability is necessary to enable systemic intravenous administration, stability of the conjugated oligonucleotide in the bloodstream, delivery to muscle tissue and internalization of the therapeutic payload in the muscle cells. In preclinical studies, our Val-Cit linker facilitated precise conjugation of multiple types of payloads to our proprietary Fabs, including ASOs, siRNAs and PMOs. This flexibility enables us to rationally select the appropriate type of payload to address the genetic basis of each muscle disease. Additionally, our linker and conjugation chemistry allow us to optimize the ratio of payload molecules attached to each Fab for each type of payload. We believe that our linker and conjugation chemistry will enable us to rapidly design, produce and screen molecules to enable new muscle disease programs.

Optimized payload

With our FORCE platform, we have the flexibility to deploy different types of therapeutic payloads with specific mechanisms of action that modify target functions. Using this modularity, we rationally select the therapeutic payload for each program to match the biology of the target, with the aim of addressing the genetic basis of disease and stopping or reversing disease progression. For instance, in our DM1 program, where the genetic driver of DM1 is mutant DMPK pre-mRNA located in the nucleus, we have determined to use an ASO because ASOs have advantages in degrading RNA in the nucleus when compared to siRNAs. In the case of our DMD program, we are utilizing an exon skipping PMO payload with the goal of enhancing dystrophin expression. In the case of certain cardiac and metabolic programs where the genetic targets are focused in the cytoplasm, we have engineered proprietary siRNA payloads to reduce the expression of these cytoplasmic targets.

Advantages of our FORCE platform

We are using our FORCE platform to develop disease-modifying therapeutics for a broad portfolio of serious muscle diseases. We believe that these therapeutics may provide the following potential advantages:

- *Targeted delivery to muscle tissue*: Using our FORCE platform, we are designing our oligonucleotide therapeutics to leverage TfR1 expression on skeletal, cardiac and smooth muscle cells to deliver muscle-targeted therapeutics to benefit patients with serious muscle disease.
- *Potent targeting of the genetic basis of disease*: The flexibility of our FORCE platform allows us to deploy different types of payloads with specific mechanisms of action to modify target function. This enables us to rationally select payloads that match the biology of the target, with the aim of addressing the genetic basis of disease and stopping or reversing disease progression.

- *Enhanced tolerability*: We engineered our Fabs to enhance the tolerability of oligonucleotide therapeutics by limiting systemic drug exposure through targeted delivery of potent therapeutic payloads to muscle tissue. Additionally, we engineered our Fabs to minimize competition with transferrin binding and interference of iron uptake in targeted muscle cells.
- *Extended durability*: Our program candidates have demonstrated in preclinical studies the ability to deliver oligonucleotides to muscle cells at concentrations that we believe could produce prolonged disease-modifying pharmacodynamic effects. We believe that the potential durability of our program candidates may enable less frequent dosing of patients.
- *Redosable administration*: Our program candidates are engineered to be redosable, not just administered one time, which may enable individualized patient titration to reach the desired level of therapeutic expression and to potentially maintain efficacy throughout a patient's life.
- *Well-established and scalable manufacturing*: Our program candidates can be manufactured using well-established and scalable methods for manufacturing antibodies, linkers and oligonucleotides. In addition, we expect our manufacturing costs will be reduced by our use of the same Fab and linker in each product candidate we develop.
- *Accelerated and efficient development*: We believe our use of a single Fab and linker across all of our programs reduces the development risk and cost of each product candidate and enables us to more quickly expand our portfolio of programs through either internally or externally developed payloads as the focus of our development will remain with the selection and optimization of each program-specific therapeutic payload.

Our portfolio

Our mission is to develop life-transforming medicines for patients with serious muscle diseases. We are creating a pipeline of programs to address diseases with high unmet need with etiologic targets. Our initial focus is on DM1, DMD and FSHD with potential pipeline expansion opportunities in additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. In selecting diseases to target with our FORCE platform, we seek diseases with clear translational potential from preclinical disease models to well-defined clinical development and regulatory pathways, and where we believe that we would be able to commercialize any products that we develop and are approved with an efficient, targeted sales force. We have global commercial rights to all of our programs.

Myotonic dystrophy type 1 (DM1)

Overview

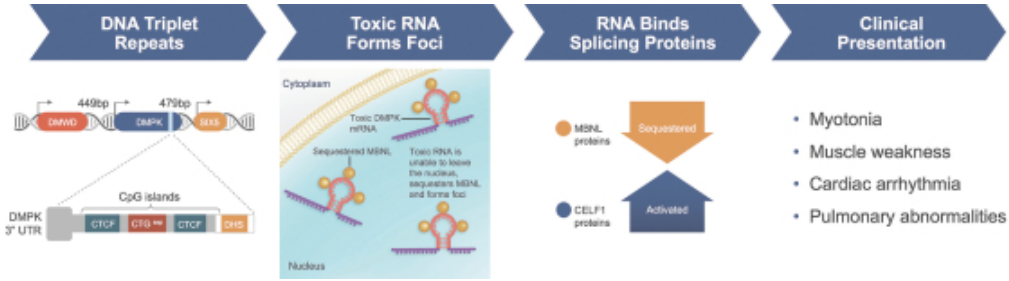
We are developing program candidates under our DM1 program to address the genetic basis of DM1 by targeting the toxic nuclear DMPK RNA that causes the disease. Our DM1 program is designed to deliver an ASO to muscle tissue to reduce the accumulation of DMPK pre-mRNA in the nucleus, release splicing proteins and potentially stop or reverse disease progression. In *in vitro* and *in vivo* preclinical studies, our ASOs when conjugated to Fabs targeting TfR1 have shown reduction in nuclear foci, correction of splicing changes, reversal of myotonia, which is a neuromuscular condition in which the relaxation of a muscle is impaired, and enhanced muscle distribution as evidenced by reduced levels of cytoplasmic wild type, or WT, DMPK RNA. We anticipate submitting an IND to the FDA for a product candidate in our DM1 program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Disease overview and prevalence

DM1 is a monogenic, autosomal dominant, progressive disease that primarily affects skeletal, cardiac and smooth muscles. DM1 patients can suffer from various manifestations of the disease including myotonia, muscle weakness, cardiac arrhythmias, respiratory problems, fatigue, cardiac abnormalities, gastrointestinal, or GI, complications, early cataracts and cognitive and behavioral impairment.

DM1 is caused by an abnormal expansion in a region of the DMPK gene. Specifically, DM1 is caused by an increase in the number of CTG triplet repeats found in the 3' non-coding region of the DMPK gene. The number of repeats ranges from up to approximately 35 in healthy individuals to many thousands in DM1 patients. The higher than normal number of triplet repeats form large hairpin loops that entrap the DMPK pre-mRNA in the nucleus and impart toxic activity, referred to as a toxic gain-of-function mutation. The mutant DMPK pre-mRNA sequesters in the nucleus, forming nuclear foci that bind splicing proteins. This inhibits the ability of splicing proteins to perform their normal function in the nucleus of guiding pre-mRNA processing of gene transcripts from many other genes. As a result, multiple pre-mRNAs that encode key proteins are mis-spliced. This mis-splicing in the nucleus results in the translation of atypical proteins which ultimately cause the clinical presentation of DM1. When nuclear DMPK levels are reduced, the nuclear foci that bind splicing proteins are diminished, releasing splicing proteins, allowing normal mRNA processing and translation of normal proteins, and potentially stopping or reversing disease progression. This disease process is illustrated below:

DM1: Genetic basis and clinical presentation



DM1 is estimated to have a genetic prevalence of 1 in 2,500 to 1 in 8,000 people in the United States and Europe, affecting over 40,000 people in the United States and over 74,000 people in Europe. However, we believe that the patient population is currently underdiagnosed due to lack of available therapies as is observed for other rare diseases. DM1 is highly variable with respect to disease severity, presentation and age of onset.

We are advancing our own efforts to better characterize the actual DM1 patient population through a natural history study that we are sponsoring. We believe that the introduction of new therapies for DM1 will cause the diagnosis rate to improve, resulting in an increase in the overall prevalence estimates for the disease. Based on age of onset and severity of symptoms, DM1 is typically categorized into four overlapping phenotypes: late-onset; classical (adult-onset); childhood; and congenital (cDM1):

Overview of DM1 phenotypes

Phenotype	Clinical presentation	Estimated % of DM1 patients	Age of onset
Late-onset	• Myotonia • Muscle weakness • Cataracts	10%	40 - 70 years
Classical (Adult-onset)	• Muscle weakness and wasting • Myotonia • Cardiac conduction abnormalities • Respiratory insufficiency • Fatigue/Excessive daytime sleepiness • GI disturbance • Cataracts	65%	Early teens - 50 years
Childhood	• Psychological problems • Low IQ • Incontinence	15%	1 - 10 years
Congenital (cDM1)	• Infantile hypotonia • Severe generalized weakness • Respiratory deficits • Intellectual disability • Classic signs present in adults	10%	Birth

All DM1 phenotypes, except the late-onset form, are associated with high levels of disease burden and premature mortality. The clinical course of DM1 is progressive, and may become extremely disabling, especially when more generalized limb weakness and respiratory muscle involvement develops. Systemic manifestations such as fatigue, GI complications, cataracts and excessive daytime sleepiness greatly impact a patient's quality of life. As a result, DM1 leads to physical impairment, activity limitations and decreased participation in social activities and work. Excluding congenital DM1 deaths, life expectancy ranges from 45 years to 60 years. Approximately 80% of early mortality is caused by cardiorespiratory complications. Respiratory failure due to muscle weakness (especially diaphragmatic weakness) causes at least 50% of early mortality, and cardiac abnormalities, including sudden death, account for approximately 30% of early mortality.

Current approaches and limitations

There are currently no disease-modifying therapies to treat DM1 that are approved or in clinical development, and treatment is focused largely on symptom management or palliative therapies. There are a number of product candidates in development, including product candidates in late stage clinical development that also are focused on symptom management or palliative therapies and do not target toxic nuclear DMPK RNA, which is the genetic basis of the disease. There remains a high unmet medical need for new disease-modifying therapies.

Our approach

Our program is designed to address the genetic basis of DM1 by targeting the toxic nuclear DMPK RNA that is the cause of the disease. We are developing program candidates linking our proprietary Fab to a proprietary ASO to address the genetic basis of DM1 by reducing the levels of mutant DMPK RNA in the nucleus, thereby releasing splicing proteins, allowing normal mRNA processing and translation of normal proteins, and potentially stopping or reversing disease progression. We expect that the proprietary ASO will be a gapmer oligonucleotide that is designed to translocate to the nucleus, bind its complementary sequence on the DMPK RNA, recruit RNAseH1 to degrade DMPK RNA and thus reduce toxic nuclear DMPK RNA. We have chosen to develop our program candidates for DM1 with an ASO because single-stranded ASOs preferentially target nuclear RNAs, which is essential for degradation of toxic nuclear DMPK RNA.

Another company previously attempted to develop a naked ASO to treat DM1 but discontinued its program due to challenges related to delivery. Specifically, the program was unable to reach the oligonucleotide muscle tissue concentration required to reduce nuclear DMPK levels, correct splicing abnormalities and address the clinical presentation. The other company believed there was a need to focus on more potent delivery to muscle. We believe that our FORCE platform has the potential to overcome the limitations faced by the other company and address the genetic basis of DM1 by achieving enhanced delivery of therapeutic ASOs to muscle.

Preclinical data

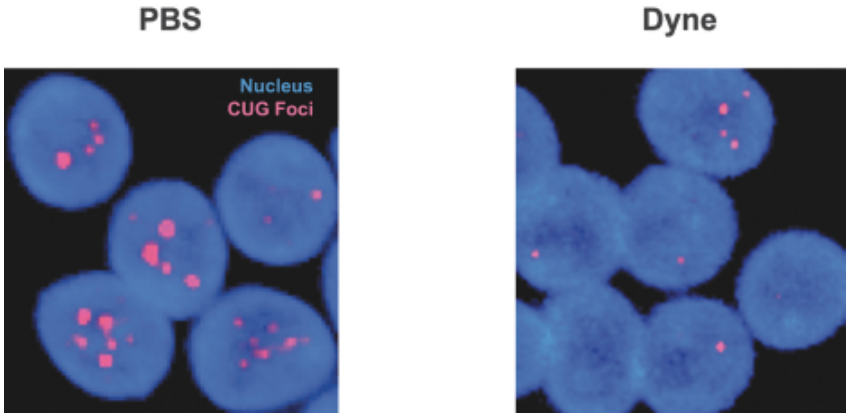
We are conducting preclinical studies of our ASOs conjugated to Fabs targeting TfR1 in DM1 patient cells and in the HSA-LR DM1 mouse model, which are disease models in which toxic DMPK RNA is observed. In *in vitro* and *in vivo* preclinical studies, our conjugated ASOs have shown reduction of nuclear foci, correction of splicing changes and reversal of myotonia in disease models, as well as enhanced muscle distribution as evidenced by reduced levels of cytoplasmic WT DMPK RNA. We believe these data support the potential for our oligonucleotide therapy to be a disease-modifying therapy for patients with DM1.

Reduction of nuclear foci

In preclinical studies in DM1 patient cells that contained toxic nuclear DMPK RNA, we observed that administration of an ASO conjugated to a Fab targeting TfR1 resulted in reduction of nuclear foci. In DM1, the higher than normal number of CUG repeats form large hairpin loops that remain trapped in the nucleus, forming nuclear foci that bind splicing proteins and inhibit the ability of splicing proteins to perform their normal function. When toxic nuclear DMPK levels are reduced, the nuclear foci are diminished, releasing splicing proteins, allowing restoration of normal mRNA processing, and potentially stopping or reversing disease progression. As illustrated in the figure below, in this study in DM1 patient cells, a single dose of conjugated ASO (shown as Dyne in the figure below) reduced nuclear DMPK foci as determined through a fluorescence in situ hybridization (FISH) analysis. The reduced nuclear DMPK foci are indicated by the reduction in red punctate staining in the figure below for Dyne as compared to a saline control (shown as PBS). We believe

the approximately 40% reduction in nuclear foci observed in this study supports the potential for our approach to advance a disease-modifying therapy for patients with DM1.

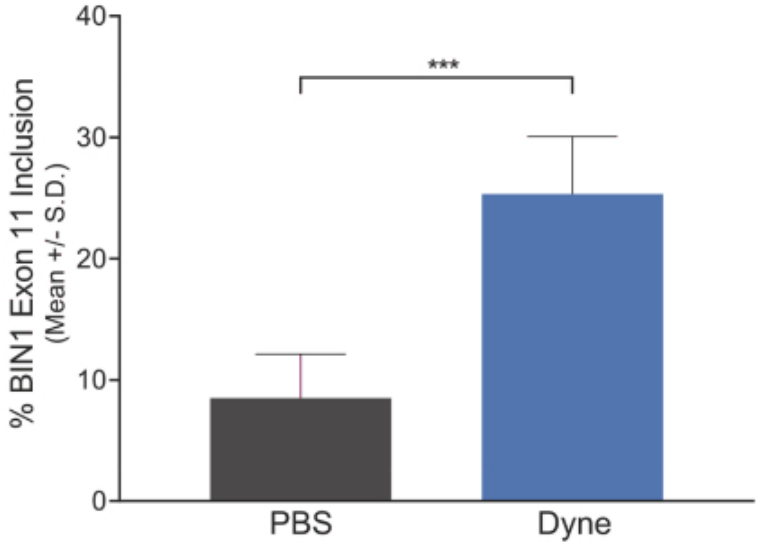
FORCE targeted nuclear DMPK and reduced nuclear foci in DM1 patient cells



Correction of splicing

In preclinical studies in DM1 patient cells that contained toxic nuclear DMPK RNA, we observed that administration of an ASO conjugated to a Fab targeting TfR1 resulted in correction of splicing of downstream RNAs such as Bridging Integrator Protein 1, or BIN1. Toxic DMPK RNA in the nucleus binds splicing proteins, inhibiting splicing protein function, and thereby reducing Exon 11 in BIN1 RNA. As illustrated in the figure below, in this study in DM1 patient cells, a single dose of the conjugated ASO (shown as Dyne in the figure) resulted in a statistically significant increase ($p < 0.001$) in Exon 11 inclusion compared to saline (shown as PBS), indicating that our conjugated ASO had reduced toxic DMPK RNA in the nucleus, causing the release of splicing proteins and the correction of BIN1 splicing. A p-value is a conventional statistical method for measuring the statistical significance of study results. A p-value of less than 0.05 is generally considered to represent statistical significance, meaning that there is a less than 5% likelihood that the observed results occurred by chance.

FORCE targeted nuclear DMPK and corrected BIN1 splicing in DM1 patient cells



*** *P < 0.001*

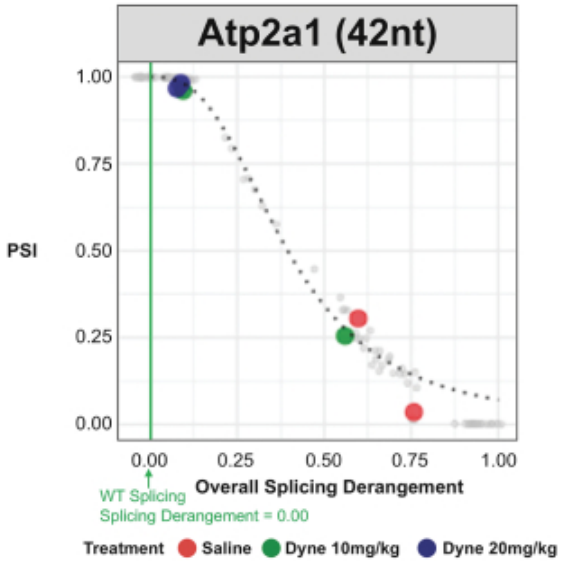
We have also observed correction of splicing in the HSA-LR DM1 mouse model. The HSA-LR DM1 mouse model is a well-validated model of DM1 that exhibits pathologies that are very similar to human DM1 patients. This model accumulates toxic DMPK RNA within the nucleus and sequesters proteins responsible for splicing such as Muscleblind-like Protein, or MBNL, thus causing mis-splicing of multiple RNAs such as CLCN1 (chloride channel) and Atp2a1 (calcium channel), among others. This mis-splicing causes the mice to exhibit myotonia, which is a hallmark of the DM1 clinical presentation in humans.

In blinded preclinical studies, single doses of one of our ASOs conjugated to a Fab targeting TfR1 delivered intravenously demonstrated dose-dependent correction of splicing in multiple RNAs and multiple muscles and was well tolerated by HSA-LR mice. In these studies, we tested the ability of the conjugated ASO to correct splicing in more than 30 different RNAs that are critical for contraction and relaxation of muscle in HSA-LR mice and observed dose-dependent correction of splicing. In DM1, significant RNA mis-splicing of these RNAs reduces muscle function.

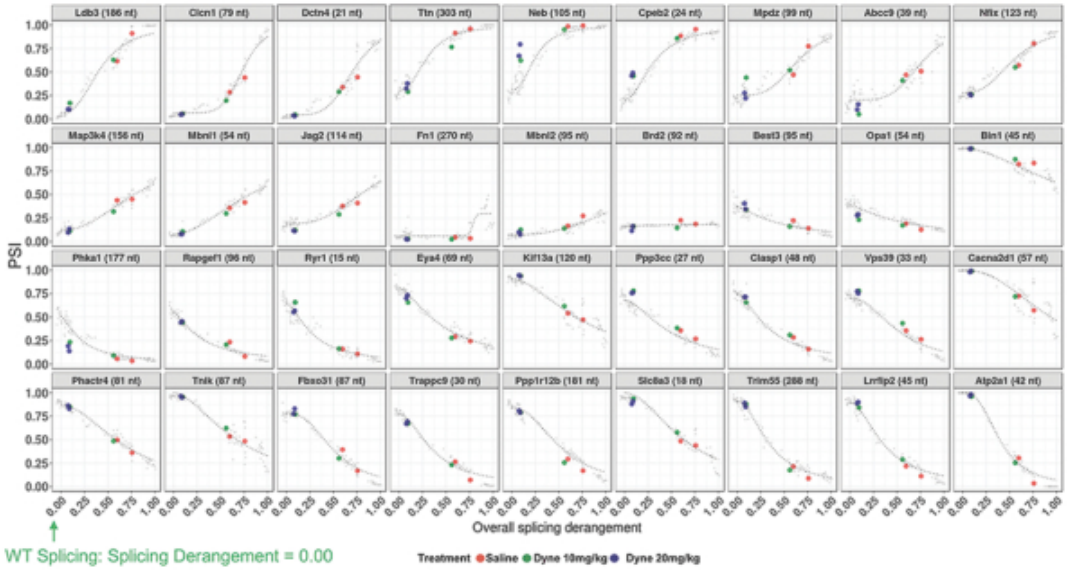
The first figure below presents the data from these studies with respect to the Atp2a1 RNA, which encodes a calcium channel and contributes to muscle contraction and relaxation. The X-axis in the figure below represents splice derangement with 1.00 on the right side of the figure representing severe mis-splicing and 0.00 on the left side of the figure representing a normal or WT splice pattern. Hence, progression from right to left on the X-axis in the figure represents a correction of splicing. The Y-axis of the figure below represents the percent of the gene spliced in, or PSI. Severe mis-splicing of Atp2a1 is caused by the lack of Exon 22 inclusion in the Atp2a1 RNA as reflected in a PS1 close to 0.00, while WT splicing reflects near complete inclusion of Exon 22 as reflected in a PSI close to 1.00. Accordingly, the blue dots in the figure indicate a near-WT splicing pattern and near-WT Exon 22 inclusion. As the figure shows, our conjugated ASO (shown as Dyne) corrected splicing of Atp2a1 in a dose-dependent manner in the gastrocnemius muscle. Two doses were administered in this study in order to evaluate dose dependence.

The second figure below presents the same data from these studies with respect to the more than 30 different RNAs that were tested, showing similar dose-dependent correction of splicing for all of the tested RNAs in gastrocnemius muscle. For some of these RNAs, correction of splicing toward WT reflects an increase in PSI, like Atp2a1 RNA in the first figure below, and for others it reflects a decrease in PSI.

FORCE dose-dependently corrected Atp2a1 splicing in HSA-LR DM1 mouse model



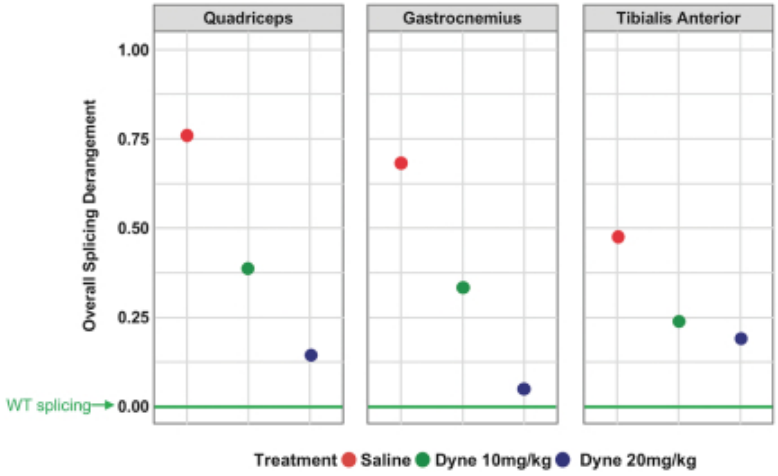
FORCE dose-dependently corrected splicing in multiple RNAs in HSA-LR DM1 mouse model



In addition to the dose-dependent changes shown in the gastrocnemius muscle, we also observed similar dose-dependent correction of splicing across the same panel of RNAs in the quadriceps and tibialis anterior muscles.

The figure below presents the levels of splicing derangement observed for saline and different doses of one of our conjugated ASOs, presented on a composite basis across the more than 30 RNAs that we tested in each muscle type.

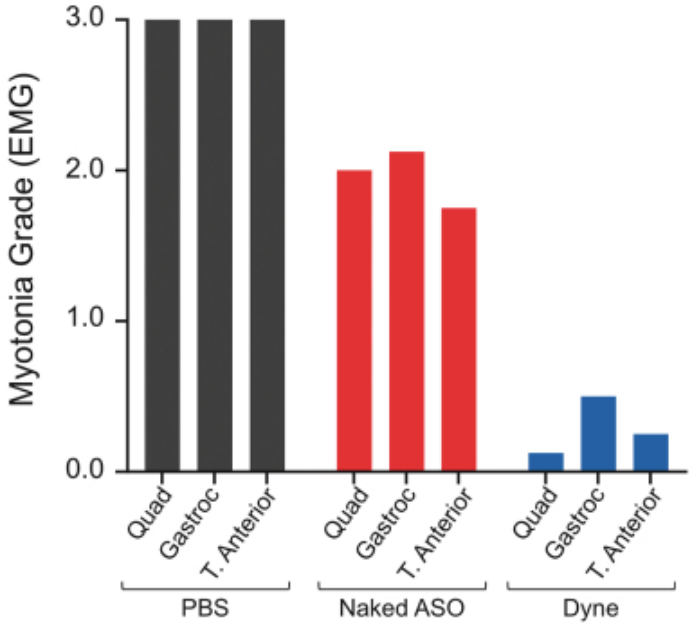
FORCE dose-dependently corrected splicing in multiple muscles in HSA-LR DM1 mouse model



Reversal of myotonia

In addition to these reductions in splicing derangement across multiple genes and muscles in the HSA-LR model, we observed in the HSA-LR model disease modification and almost complete reversal of myotonia after a single dose (20 mg/kg) of one of our ASOs conjugated to a Fab targeting TfR1. As shown in the figure below, we evaluated the severity of myotonia on a four-point scale 14 days following dosing with saline (PBS), naked ASO and the conjugated ASO, with grade 0 representing no myotonia, grade 1 representing myotonic discharge as measured by electromyography (EMG) in less than 50% of needle insertions, grade 2 representing myotonic discharge in greater than 50% of needle insertions and grade 3 representing myotonic discharge with nearly every needle insertion. We conducted this evaluation in quadriceps, gastrocnemius and tibialis anterior muscles.

Single dose of FORCE reversed myotonia in HSA-LR DM1 mouse model



Separately, in a third-party study of a naked ASO conducted in the same HSA-LR model by the company that discontinued its program due to delivery challenges, reductions in myotonia were observed after one month following eight 25 mg/kg doses of a naked ASO administered biweekly for a total dose of 200 mg/kg.

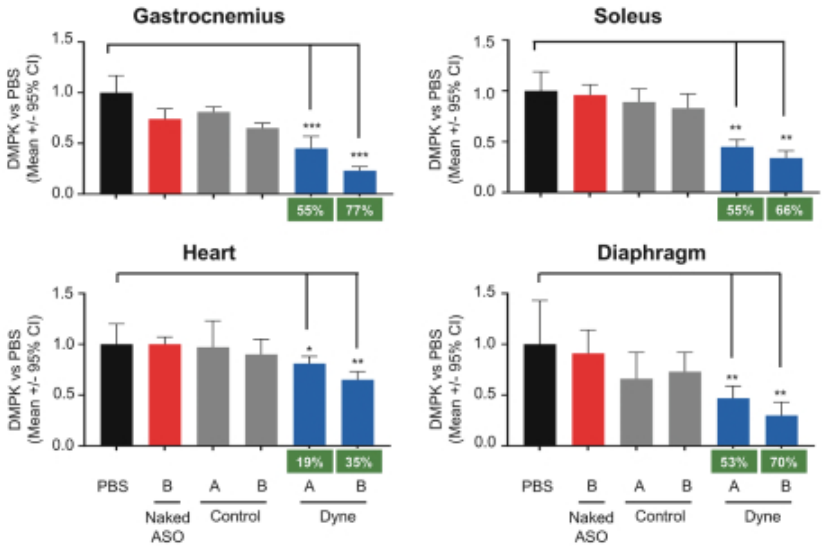
We believe the correction of splicing and reduction of myotonia we observed in our studies show that the intravenously administered ASO conjugated to a Fab was internalized into multiple muscles, enabling the ASO payload to enter the nucleus and degrade toxic DMPK RNA, thereby releasing splicing proteins to correct splicing of multiple RNAs and reverse myotonia.

Enhanced muscle distribution

While oligonucleotides are rationally designed genetic medicines, the current limitations with respect to delivery of oligonucleotides to muscle tissue present a significant limitation to advancing modern oligonucleotide therapeutics for muscle diseases. We have designed our FORCE platform to overcome these limitations. In preclinical studies, we observed that a single intravenous dose of one of our ASOs conjugated to a Fab targeting TfR1 was able to deliver its DMPK-targeted ASO payload to skeletal, cardiac and smooth muscle cells in mice and non-human primates and decrease WT DMPK RNA in the cytoplasm of different muscle cells.

We have observed dose-dependent and long-lasting reductions in the levels of DMPK in WT mice. In preclinical studies shown in the figures below, one of our ASOs when conjugated to a Fab targeting TfR1 resulted in a dose-dependent reduction in levels of cytoplasmic WT DMPK RNA that was greater than the reductions observed with the naked ASO. In this study we also evaluated our ASO conjugated to a Fab “scramble control” which does not bind to any known murine cell receptor in the gastrocnemius, soleus, heart and diaphragm muscles, at two dose levels, to demonstrate the advantage of leveraging TfR1 to deliver oligonucleotides to muscle. The percentages in the green boxes reflect the amount by which DMPK RNA decreased with the conjugated ASO compared to saline (PBS) administration.

FORCE dose-dependently decreased DMPK RNA

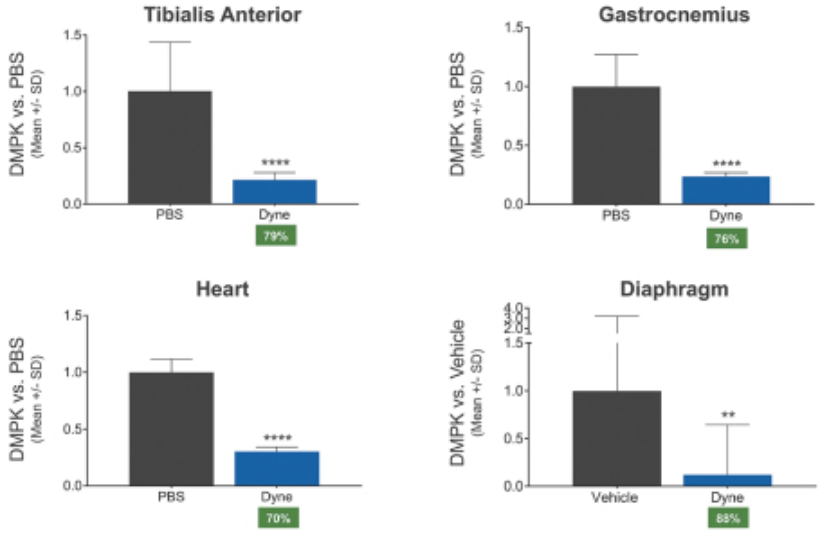


Dose A: low dose
Dose B: high dose
** P < 0.05*
*** P < 0.01*
**** P < 0.001*

We have also observed results in preclinical studies important to our efforts to develop redosable, titratable and durable therapeutics under our FORCE platform. Specifically, we observed durability of response up to 12 weeks in the tibialis anterior muscle and other muscles after a single dose in WT mice. In addition, repeat doses in WT mice were well tolerated and resulted in increased reductions in cytoplasmic WT DMPK RNA as compared to single doses.

We also developed a hTfR1 mouse model which expresses a human TfR1 rather than the murine TfR1 expressed in WT mice. This model was designed to accelerate the development of our FORCE platform and programs. In preclinical studies using the hTfR1 mouse model, as shown below, we observed that two doses of an ASO conjugated to a Fab targeting human TfR1 resulted in significant reductions in cytoplasmic WT DMPK RNA in the tibialis anterior, gastrocnemius, heart and diaphragm muscles.

FORCE decreased DMPK RNA in hTfR1 mouse model



*** P < 0.01*
**** P < 0.0001*

We also are evaluating our DM1 program in non-human primates. Currently there are no known DM1 disease models in non-human primates. As a result, any therapeutic candidate that reduces DMPK RNA expression in non-human primates is a result of targeting cytoplasmic WT DMPK RNA and not reflective of disease modification, which would require targeting toxic DMPK RNA in the nucleus. Consequently, we utilize non-human primate studies to evaluate muscle delivery, pharmacokinetics and tolerability of our FORCE platform. As shown in the figure below, in studies in non-human primates, a single intravenous dose of one of our ASOs when conjugated to a Fab targeting TfR1 reduced cytoplasmic DMPK RNA in multiple skeletal, cardiac and smooth muscles. In addition, and also as shown in the figure below, our conjugated ASO produced greater reductions, or knockdown (KD), in DMPK RNA than an equivalent dose of the naked ASO. Furthermore, our conjugated ASO produced less knockdown in DMPK RNA in non-muscle tissues, including kidney, liver and spleen tissues, as compared to a naked ASO, which we believe indicates specificity of our conjugated ASO for muscle tissue. Treatment with our conjugated ASO in these non-human primates was well tolerated with no clinically meaningful changes in hematology, serum biochemistry, platelet numbers, iron homeostasis, or kidney or liver function.

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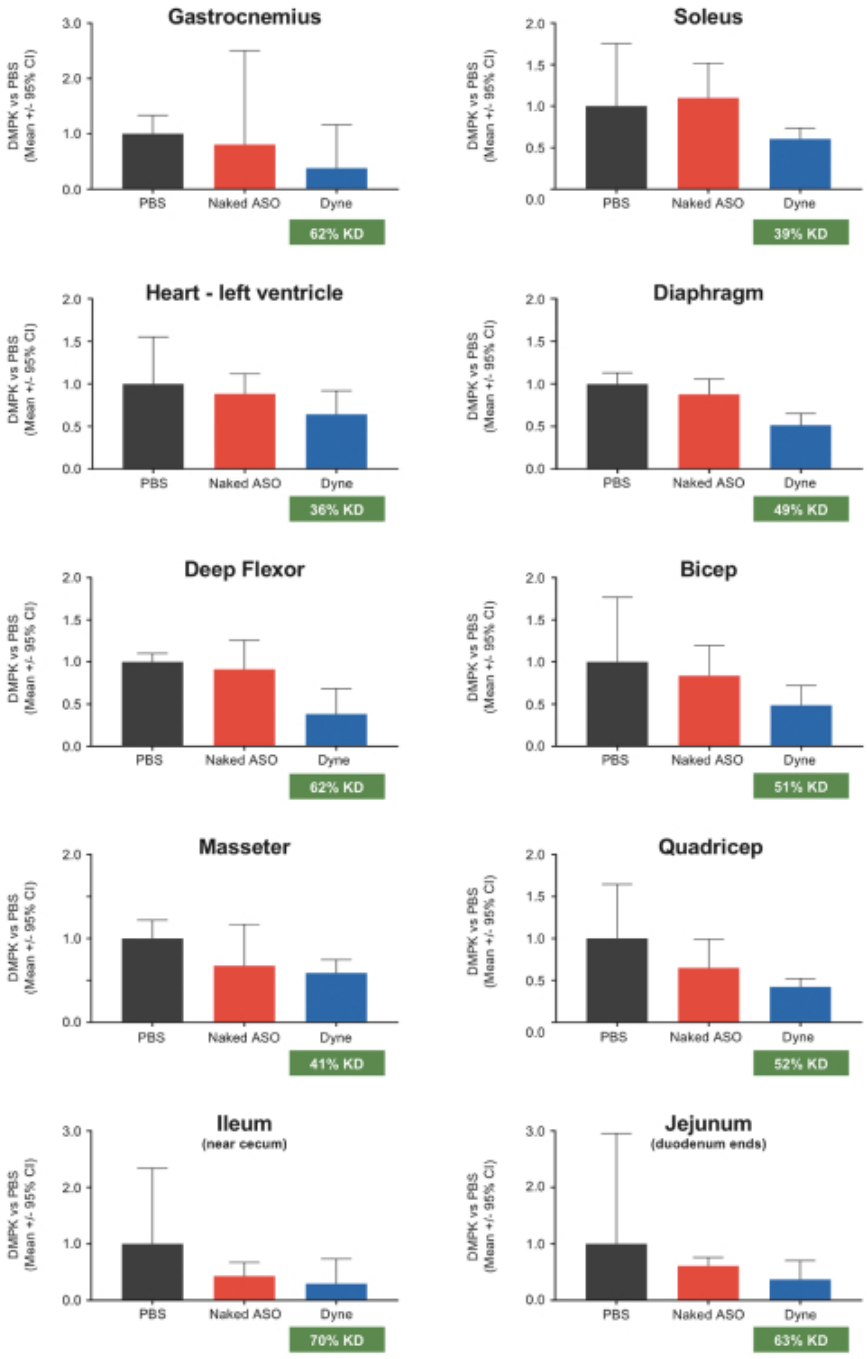
FORCE significantly decreased cytoplasmic DMPK RNA in non-human primate skeletal, cardiac and smooth muscle



Next steps

In our preclinical studies, we have observed targeting of mutant DMPK RNA in the nucleus, reduction of nuclear foci and splicing correction of key genes in DM1 patient cells, dose-dependent correction of splicing and reversal of myotonia after a single dose in the HSA-LR mouse DM1 model, durability of DMPK RNA knockdown up to 12 weeks, enhanced DMPK RNA knockdown with repeat dosing in WT and hTfr1 mice and enhanced delivery of DMPK-targeting ASO payload to murine and non-human primate skeletal, cardiac and smooth muscle tissue.

Based on these results, we plan to conduct IND-enabling safety studies in non-human primates and anticipate submitting an IND to the FDA for a product candidate in our DM1 program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Duchenne muscular dystrophy (DMD)

Overview

We are developing program candidates under our DMD program to address the genetic basis of DMD by delivering a PMO to muscle tissue to promote the skipping of specific DMD exons in the nucleus, allowing muscle cells to create a more complete, functional dystrophin protein and to potentially stop or reverse disease progression. We believe that PMOs, with their preferential targeting of nuclear mechanisms, are the best payload to address nuclear exon skipping. In *in vitro* and *in vivo* preclinical studies, our PMOs when conjugated to a Fab targeting TfR1 have shown increased exon skipping, increased dystrophin expression, reduced muscle damage and increased muscle function. We are seeking to build a DMD franchise by initially focusing on the development of a therapeutic for patients with mutations amenable to skipping Exon 51, to be followed by the development of therapeutics for patients with mutations amenable to skipping other exons, including Exon 53, 45 and 44. We expect to submit an IND to the FDA for a product candidate in our Exon 51 skipping program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

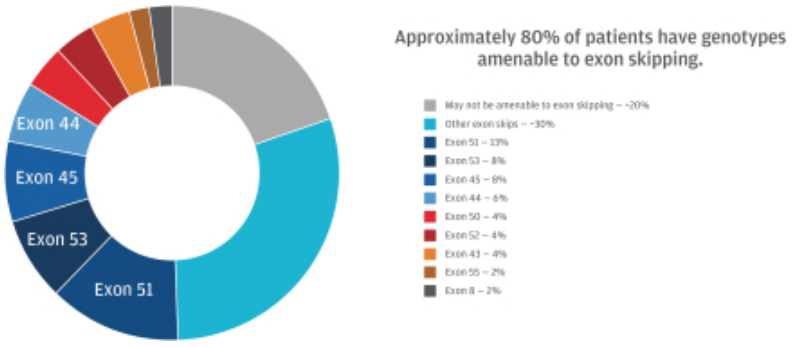
Disease overview and prevalence

DMD is a monogenic, X-linked, disease caused by mutations in the gene that encodes for the dystrophin protein. Dystrophin protein is essential to maintain the structural integrity and normal function of muscle cells for walking, breathing and cardiac function. In patients with DMD, mutations in the dystrophin gene lead to certain exons being misread, resulting in the loss of function of the dystrophin protein. The reduction or absence of dystrophin leads to damage to muscle cell membranes, resulting in muscle cell death and progressive loss of muscle function.

DMD symptoms typically begin to manifest with weakness and progressive loss of muscle function beginning in the first few years of life. Young boys experience progressive muscle wasting and have difficulty standing up, climbing stairs, running, breathing and performing daily functions. As the disease progresses the severity of damage to skeletal and cardiac muscles results in patients experiencing total loss of ambulation in the pre-teenage or early teenage years. Progressive loss of upper extremity function is often observed in the mid-to-late teens followed by respiratory and/or cardiac failure, resulting in death before the age of 30.

We estimate that DMD occurs in approximately one in every 3,500 to 5,000 live male births and that approximately 12,000 to 15,000 patients in the United States, and approximately 25,000 patients in Europe, have DMD. Approximately 80% of patients with DMD have DMD mutations amenable to exon skipping in the nucleus. Exons 51, 53, 45 and 44 represent nearly half of the total mutations observed in DMD that are amenable to exon skipping, as illustrated in the figure below.

Overview of DMD exons amenable to skipping



Current approaches and limitations

Currently, patients with DMD are treated with corticosteroids to manage the inflammatory component of the disease. There are three FDA-approved naked PMO-based oligonucleotide therapies, each addressing a specific mutation: EXONDYS 51 (eteplirsen), which is approved for the treatment of DMD patients amenable to Exon 51 skipping, VYONDYS 53 (golodirsen), which is approved for the treatment of DMD patients amenable to Exon 53 skipping, and VILTEPSO (vitolarsen), which is approved for the treatment of DMD patients amenable to Exon 53 skipping. However, each of the drugs requires weekly intravenous infusions. Eteplirsen and golodirsen have demonstrated a less than 1% mean increase in dystrophin in clinical trials and vitolarsen has demonstrated an approximately 3% increase in dystrophin in clinical trials. The FDA-approved labels for all three drugs state that a clinical benefit has not yet been established and that continued approval may be contingent upon the verification of such clinical benefit in confirmatory clinical trials. In Europe, the European Medicines Agency has rejected an application for approval of eteplirsen citing insufficient evidence of clinical benefit. In addition, a fourth drug, TRANSLARNA (ataluren), has only been conditionally approved in the European Union, Iceland and South Korea for non-sense mutations in DMD in ambulatory patients aged five years and older. Each of these approved products seeks to address DMD through the exon skipping approach we are pursuing, but we believe their limited efficacy is due to poor muscle uptake and biodistribution. There are a number of product candidates in development, including product candidates in late stage clinical development, which seek to address DMD through the exon skipping approach we are pursuing, including naked oligonucleotides, targeted oligonucleotides and PMOs conjugated to charged peptides, as well as product candidates that seek to address DMD through gene editing and gene replacement with viral gene therapies and with other approaches. We believe that each of these approaches currently have significant limitations, and that there continues to be a high unmet medical need for new disease-modifying therapies.

Our approach

Our DMD program is designed to address the genetic basis of DMD by promoting the skipping of specific DMD exons in the nucleus, allowing muscle cells to create more complete, functional dystrophin protein. Under our DMD program, we are developing program candidates for intravenous infusion that incorporate a Fab targeting TfR1 conjugated to a PMO designed to promote the skipping of specific DMD exons in the nucleus. Existing clinical data generated by others supports the benefits of utilizing a single stranded ASO or PMO to skip the faulty exon in the nucleus of DMD patient cells. We believe the Fab targeting TfR1 allows for more efficient delivery of a PMO to skeletal, cardiac and smooth muscle cells, creating an opportunity to increase dystrophin expression, enable less frequent dosing and provide greater clinical benefit compared to current therapeutic

approaches. We plan to develop our program candidates for DMD with a PMO, initially for Exon 51 and in the future for other exon mutations including Exons 53, 45 and 44. We have identified potential PMOs for mutations amenable to skipping Exons 51, 53 and 45 and plan to identify PMOs for mutations amenable to skipping Exon 44.

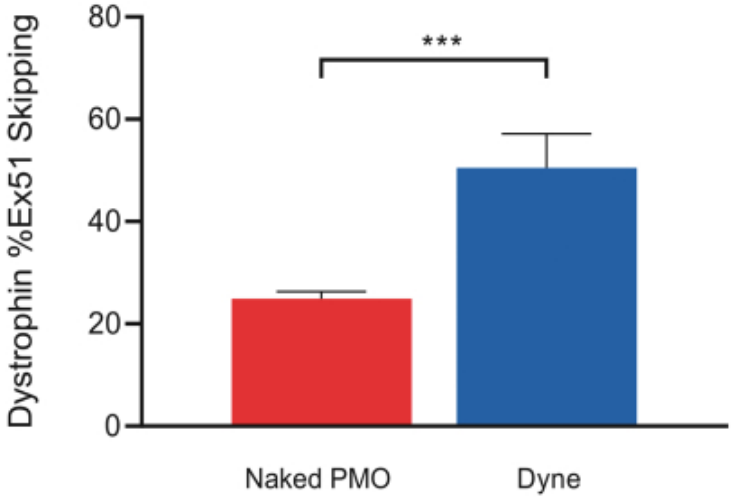
Preclinical data

We are conducting preclinical studies of our PMOs conjugated to Fabs targeting TfR1 in human DMD cell lines and in mouse models of DMD. In *in vitro* and *in vivo* preclinical studies, these PMOs when conjugated to a Fab targeting TfR1 have shown increased exon skipping, increased dystrophin expression, reduced muscle damage and increased muscle function. We believe these data support the potential for our oligonucleotide therapy to be a disease-modifying therapy for patients with DMD.

Increased exon skipping

We evaluated a candidate Exon 51-targeting PMO conjugated to a Fab targeting TfR1 in human DMD myotubes, a type of muscle cell, with a mutation amenable to Exon 51 skipping. We observed that treatment with the conjugated PMO resulted in a 50% increase in exon skipping as compared to a 25% increase in exon skipping following treatment with an equimolar dose of the naked PMO (p=0.001), as illustrated in the figure below.

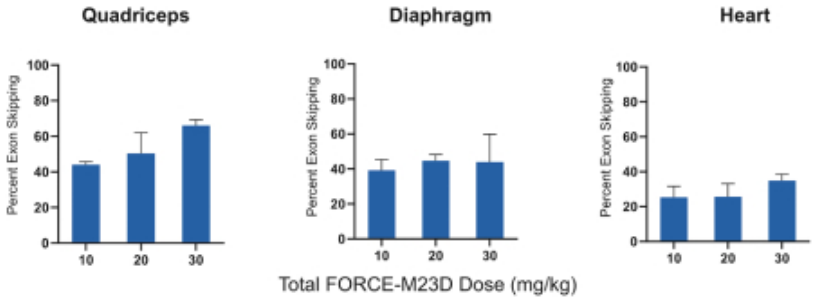
FORCE increased exon skipping in human DMD myotubes with exon 51 mutation



*** *P < 0.001*

In studies in the mdx mouse DMD model, a validated and widely accepted mouse model in DMD which has a mutation in Exon 23, we observed that single intravenous doses of an Exon 23-targeting PMO conjugated to a Fab targeting TfR1 which we refer to as FORCE-M23D, achieved effective, dose-dependent exon skipping in multiple muscles, including in the quadriceps, diaphragm and cardiac muscles, 14 days following treatment, as illustrated in the figure below.

FORCE achieved effective exon skipping in mdx mouse DMD model

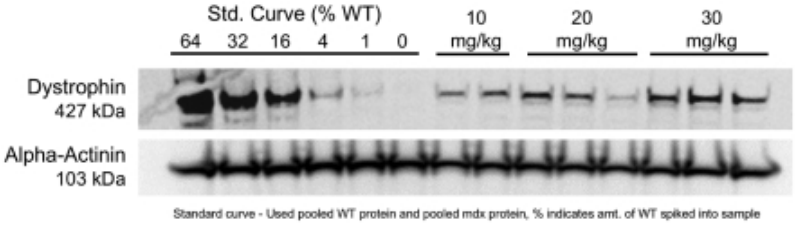


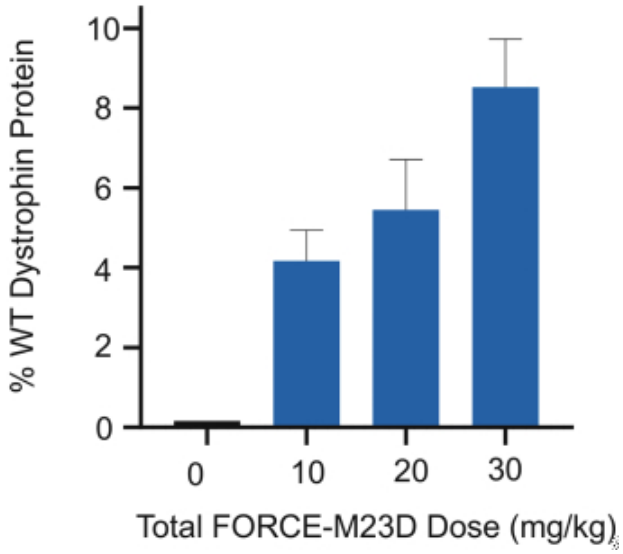
Levels of exon skipping of FORCE-M23D in this study were also higher than levels of exon skipping observed in third-party studies of a naked PMO or a stereopure ASO in the same mdx mouse model.

Increased dystrophin expression

In the same mdx mouse DMD model, we observed that the exon skipping promoted by a Exon 23-targeting PMO when conjugated to a Fab targeting TfR1 resulted in dose-dependent production of dystrophin protein in the quadriceps muscle, as illustrated in the western blot (top) and bar graph (bottom) below. Levels of dystrophin expression in this study were also higher than levels of dystrophin expression observed in a third-party study of a naked PMO in the same mdx mouse model. In this study, we utilized Alpha-Actinin as a control protein.

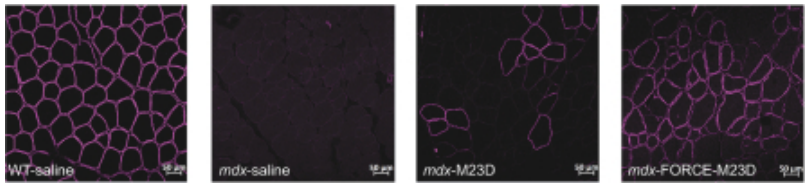
FORCE dose-dependently increased dystrophin expression in mdx mouse DMD model quadriceps muscle





We also observed in the mdx mouse DMD model that a single dose of FORCE-M23D restored dystrophin expression to the muscle cell membrane based on immunohistochemistry in the quadricep. In addition, FORCE-M23D restored more dystrophin compared to an equivalent dose of the naked PMO which we refer to as naked M23D, as indicated by the greater purple shading in the figure for FORCE-M23D as compared to naked M23D and a saline control.

Dystrophin expression in quadriceps muscle

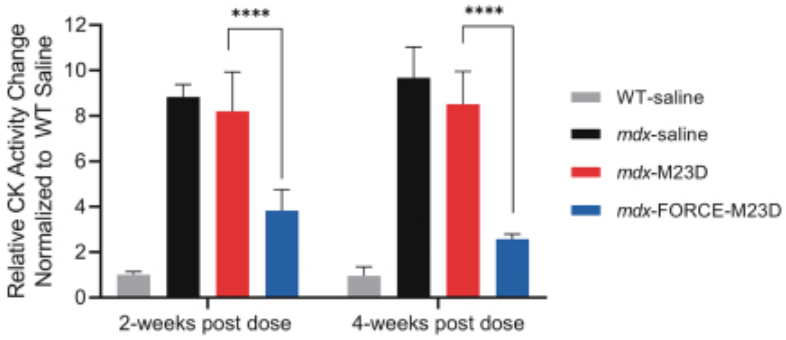


We believe localization of dystrophin to the muscle cell membrane is critical to restore muscle cell structural integrity and function in patients with DMD and that these data show more effective PMO delivery to muscle when using our conjugated PMO as compared to the naked PMO.

Reduced muscle damage

In DMD patients, the reduction of muscle cell structural integrity leads to muscle cell membrane damage and the leakage of creatine kinase (CK), an enzyme normally found inside muscle cells, into serum. As a result, the presence of creatine kinase in serum is utilized as a biomarker for muscle damage in DMD. In preclinical studies in the mdx mouse DMD model, we observed that levels of creatine kinase decreased significantly more in mice treated with a single dose of FORCE-M23D when measured two and four weeks following dosing compared to mice treated with naked M23D, as illustrated in the figure below.

Single dose of FORCE significantly reduced serum creatine kinase in mdx mouse DMD model

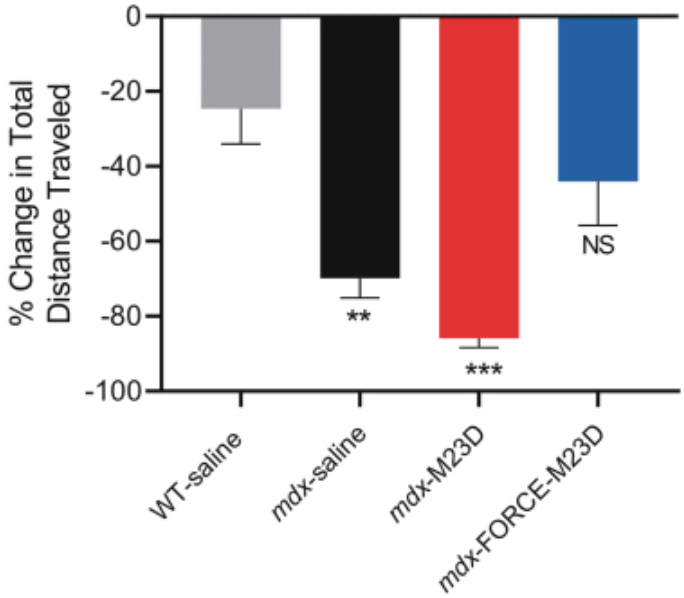


**** *P < 0.0001*

Increased muscle function

In additional preclinical studies in the mdx mouse DMD model, we observed that a single dose of FORCE-M23D demonstrated a functional benefit in multiple standardized assessments conducted in blinded conditions. In a hind limb fatigue challenge, mice treated with a single dose of FORCE-M23D performed significantly better than those treated with naked M23D two weeks following treatment. In addition, the performance of the mdx mice treated with FORCE-M23D was statistically equivalent to the performance of a control cohort of healthy, wild-type mice treated with saline, as illustrated in the figure below.

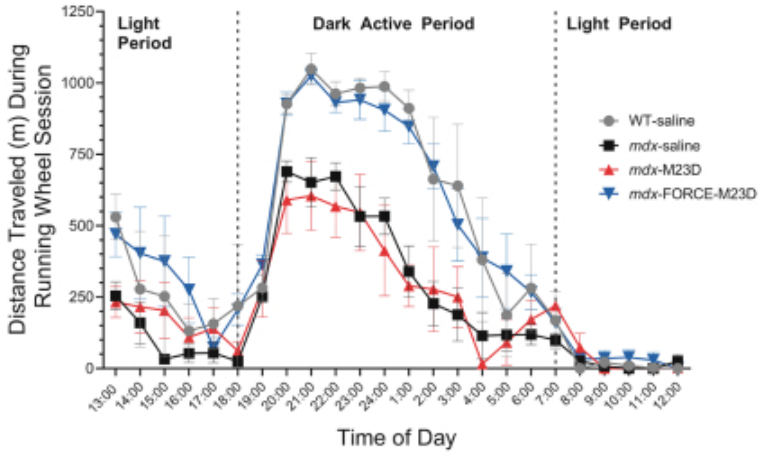
Single dose of FORCE demonstrated functional benefit in mdx mouse DMD model: hind limb fatigue challenge



** *P < 0.01*
*** *P < 0.001*
NS: not statistically significant

In the home cage running wheel study, the distance traveled by mice is measured over a 24-hour period. In this functional assessment, mdx mice treated with a single dose of FORCE-M23D performed better than those treated with naked M23D four weeks following treatment, with the performance of the mdx mice treated with FORCE-M23D mirroring the performance of a control cohort of healthy, wild-type mice treated with saline, as illustrated in the figure below. In this study, the mice were subjected to both light and dark periods to simulate the course of a day, and activity was higher during dark periods reflecting the nocturnal nature of mice.

Single dose of FORCE demonstrated functional benefit in mdx mouse DMD model: home cage running wheel



We believe the results from these preclinical studies are consistent with improvements in both skeletal and cardiac muscle function resulting from targeted delivery of PMOs in muscle tissue, enhanced exon skipping, increased dystrophin expression and improved muscle health.

Next steps

We are seeking to build a DMD franchise by initially focusing on the development of a therapeutic for patients with mutations amenable to skipping Exon 51, to be followed by the development of therapeutics for patients with mutations amenable to skipping other exons, including Exons 53, 45 and 44. We plan to conduct IND-enabling safety studies in non-human primates and anticipate submitting an IND to the FDA for a product candidate in our Exon 51 skipping program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Facioscapulohumeral Dystrophy (FSHD)

Overview

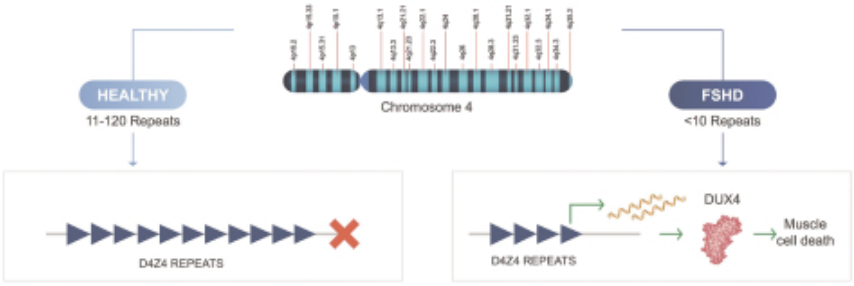
We are developing program candidates under our FSHD program that are designed to address the genetic basis of FSHD by reducing DUX4 expression in muscle tissue. We exclusively licensed from the University of Mons, or UMONS, intellectual property covering multiple ASOs that have been shown to potently target DUX4 in preclinical studies. We anticipate submitting an IND to the FDA for a product candidate in our FSHD program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Disease overview and prevalence

FSHD is one of the most common muscular dystrophies and affects both sexes equally, with onset typically in teens and young adults. FSHD is characterized by progressive skeletal muscle loss that initially causes weakness in muscles in the face, shoulders, arms and trunk and progresses to weakness in muscles in lower extremities and the pelvic girdle. Skeletal muscle weakness results in significant physical limitations, including progressive loss of facial muscles that can cause an inability to smile or communicate, difficulty using arms for activities of daily living and difficulty getting out of bed, with many patients ultimately becoming dependent upon the use of a wheelchair for daily mobility activities. We estimate that the patient population is between 16,000 and 38,000 in the United States and approximately 35,000 in Europe. We believe that there may be additional patients who are not formally diagnosed due to a perceived difficulty of obtaining a diagnosis and the fact that there are no approved treatments. Approximately two-thirds of cases are familial-inherited in an autosomal dominant fashion and one-third of cases occur randomly or as a result of environmental factors. FSHD affects all ethnic groups with similar incidence and prevalence.

FSHD is caused by aberrant expression of the DUX4 gene in muscle resulting in inappropriate presence of the DUX4 protein, a transcription factor causing the expression of other genes. Normally, DUX4-driven gene expression is limited to early embryonic development, after which time the DUX4 gene is silenced. In patients with FSHD, a genetic mutation causes expression of DUX4 protein to continue after embryonic development. The DUX4 protein regulates the expression of multiple genes encoding other proteins, some of which are toxic to muscle. Evidence of aberrant expression of DUX4 and the genes it activates, including ZSCAN4, MBD3L2, and TRIM43, is a major molecular signature that distinguishes muscles affected by FSHD from healthy muscle. The aberrant expression of DUX4 in FSHD results in muscle death and replacement by fat, which leads to the progressive muscle weakness and disability which characterize the disease, as shown in the figure below.

FSHD: genetic basis and disease process



Current approaches and limitations

There are currently no approved therapies for FSHD, and patients are treated with pain management and physical therapy. There are a number of product candidates in clinical development, including losmapimod, a p38 MAPK inhibitor that is intended to modulate DUX4 expression and is being evaluated in a Phase 2 clinical trial. To aid in the development of therapies for FSHD, we are sponsoring an ongoing natural history study seeking to validate new clinical outcome assessments and evaluate physiological biomarkers to support the design and implementation of future clinical trials.

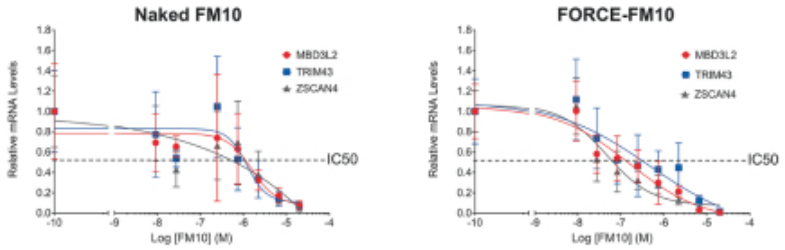
Our approach

We are developing program candidates under our FSHD program that are designed to address the genetic basis of FSHD by reducing DUX4 expression in muscle tissue. We exclusively licensed from UMONS intellectual

property covering multiple DUX-targeting ASOs that have been shown to potently target DUX4 in preclinical studies. We are currently evaluating these ASOs conjugated to a Fab targeting TfR1 in patient-derived FSHD myotubes.

As shown in the figure below, we administered to patient-derived myotubes an ASO conjugated to a Fab targeting TfR1, which we refer to as FORCE-FM10, and naked FM10 and observed reduced DUX4-associated gene expression, as measured by the mRNA transcribed by three genes that are known to be only expressed following DUX4 activation, MBD3L2, TRIM43 and ZSCAN4. In addition, IC_{50} concentrations for the conjugated FM10 were up to 9.6 times lower than those observed for naked FM10, indicating that conjugated FORCE-FM10 was up to 9.6 times more potent than naked FM10. IC_{50} concentration is a measure of the potency of a substance that indicates how much of the substance is needed to inhibit a biological process.

FORCE-FM10 suppressed expression of key DUX4 biomarkers in FSHD patient myotubes



Transcript	IC_{50} (nM) FORCE-FM10	IC_{50} (nM) Naked FM10	Fold Advantage of FORCE-FM10
ZSCAN4	67	643	9.6 x
MBD3L2	144	1208	8.4 x
TRIM43	352	1117	3.2 x

Next steps

We plan to conduct IND-enabling safety studies in non-human primates and anticipate submitting an IND to the FDA for a product candidate in our FSHD program as one of the three INDs we expect to submit between the fourth quarter of 2021 and the fourth quarter of 2022.

Discovery programs

We intend to expand our FORCE portfolio by pursuing programs in additional indications, including additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. By rationally selecting therapeutic payloads to conjugate with our proprietary Fab and linker, we plan to develop product candidates to address the genetic basis of additional muscle diseases. In selecting these payloads, we plan to prioritize ASOs for indications driven by nuclear genetic targets and siRNAs for indications driven by cytoplasmic targets. We have completed screening and identified potent ASO and siRNA payloads against a number of cardiac and metabolic targets. We may selectively establish strategic collaborations for certain of these programs where we believe we could benefit from the resources or capabilities of other biopharmaceutical companies. We may also seek strategic collaborations where we believe we can utilize our FORCE platform to enhance delivery of third-party payloads to muscle tissue.

In addition to our muscle disease portfolio, we believe there is an opportunity to leverage our TfR1 antibody expertise to develop novel antibodies that cross the blood-brain barrier and deliver therapeutics to CNS tissue

through systemic intravenous administration. These antibodies will likely have different characteristics, such as TfR1 affinity, than the antibodies we have optimized for muscle delivery.

Manufacturing

We do not own or operate manufacturing facilities. We currently rely on third-party contract manufacturing organizations, or CMOs, and suppliers for the Fab antibodies, linker and oligonucleotide payloads that comprise our program candidates and the conjugation of these components. We plan to use third-party CMOs to support our IND-enabling studies and to fully supply our clinical trials and commercial activities, but may also seek to eventually establish our own manufacturing facility for long-term commercial supply. As we scale manufacturing, we intend to continue to expand and strengthen our network of CMOs. We believe there are multiple sources for all of the materials required for the manufacture of our product candidates, as well as multiple CMOs who could assemble the antibody, linker and payload that comprise our program candidates.

Manufacturing is subject to extensive regulations that impose procedural and documentation requirements. These regulations govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance. Our CMOs are required to comply with these regulations and are assessed through regular monitoring and formal audits. Our third-party manufacturers are required to manufacture any product candidates we develop under current Good Manufacturing Practice, or cGMP, requirements and other applicable laws and regulations.

We have personnel with extensive technical, manufacturing, analytical and quality experience to oversee all contracted manufacturing and testing activities. We have established a CMC Advisory Board to support our manufacturing personnel.

Intellectual property

We strive to protect our proprietary technology, inventions, improvements, platforms, program candidates, product candidates and components thereof, their methods of use and processes for their manufacture that we believe are important to our business, including by obtaining, maintaining, defending and enforcing patent and other intellectual property rights for the foregoing in the United States and in foreign jurisdictions. We also rely on trade secrets and confidentiality agreements to protect our confidential information and know-how and other aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our future commercial success depends in part on our ability to:

- obtain, maintain, enforce and defend patent and other intellectual property rights for our important technology, inventions and know-how;
- preserve the confidentiality of our trade secrets and other confidential information;
- obtain and maintain licenses to use and exploit intellectual property owned or controlled by third parties;
- operate without infringing, misappropriating or otherwise violating any valid and enforceable patents and other intellectual property rights of third parties; and
- defend against challenges and assertions by third parties challenging the validity or enforceability of our intellectual property rights, or our rights in our intellectual property, or asserting that the operation of our business infringes, misappropriates or otherwise violates their intellectual property rights.

As of June 30, 2020, we owned 37 patent application families related to our business, comprised of 33 U.S. provisional patent applications and 11 pending Patent Cooperation Treaty, or PCT, patent applications, and we

exclusively licensed one patent family, comprised of one issued U.S. patent, one U.S. patent application and one issued European patent that has been validated in Belgium, Switzerland, Germany, Denmark, Spain, France, the United Kingdom, Ireland, Italy, the Netherlands and Sweden. None of our pending PCT patent applications has entered the national stage.

Our owned and licensed patent estate covers various aspects of our programs and technology, including our FORCE platform, proprietary antibodies, oligonucleotide conjugates, methods of treatment and aspects of manufacturing. Any U.S. or foreign patents issued from national stage filings of our PCT patent applications and any U.S. patents issued from non-provisional applications we may file in connection with our provisional patent applications would be scheduled to expire on various dates from 2039 through 2041, without taking into account any possible patent term adjustments or extensions and assuming payment of all appropriate maintenance, renewal, annuity and other governmental fees. Further details on certain segments of our patent portfolio are included below.

FORCE platform

With regard to our FORCE platform, as of June 30, 2020, we owned two pending PCT patent applications and eight pending U.S. provisional patent applications. These applications relate to various aspects of our FORCE platform including proprietary antibodies, oligonucleotide conjugates, methods of manufacture and methods of treatment. Any patents issued from these applications are expected to expire from 2039 to 2041; however, patent term extension may be available.

DM1 program

With regard to our DM1 program, as of June 30, 2020, we owned one pending PCT patent application and four pending U.S. provisional patent applications. These applications relate to composition of matter and methods of treating disease involving our FORCE platform in the context of DM1. Any patents issued from these applications are expected to expire from 2039 to 2041; however, patent term extension may be available.

DMD program

With regard to our DMD program, as of June 30, 2020, we owned one pending PCT patent application and four pending U.S. provisional patent applications. These applications relate to composition of matter and methods of treating disease involving our FORCE platform in the context of DMD. Any patents issued from these applications are expected to expire in 2039 and 2041; however, patent term extension may be available.

FSHD program

With regard to our FSHD Program, as of June 30, 2020, we owned one pending PCT patent application and three pending U.S. provisional patent applications. These applications relate to composition of matter and methods of treating disease involving our FORCE platform in the context of FSHD. Any patents issued from these applications are expected to expire in 2039 and 2041; however, patent term extension may be available. We also in-license a patent family from UMONS comprised of one issued U.S. patent, one pending U.S. patent application and one issued European patent that has been validated in Belgium, Switzerland, Germany, Denmark, Spain, France, the United Kingdom, Ireland, Italy, the Netherlands and Sweden. The issued patents expire in 2031; however, a patent term extension may be available.

Discovery programs

With regard to our discovery programs, as of June 30, 2020, we owned one pending PCT patent application and twelve pending U.S. provisional patent applications. These applications relate to composition of matter and

methods of treating disease involving our FORCE platform in the context of a variety of additional rare skeletal muscle diseases, as well as cardiac and metabolic muscle diseases. Any patents issued from these applications are expected to expire in 2039 and 2041; however, patent term extension may be available.

Patent prosecution

A PCT patent application is not eligible to become an issued patent until, among other things, we file one or more national stage patent applications in the jurisdictions in which we seek patent protection and do so within prescribed timelines of the PCT application's priority date. These prescribed timelines are generally 30 months, 31 months or 32 months, depending on the jurisdiction. If we do not timely file any national stage patent applications, we may lose our priority date with respect to our PCT patent application and any potential patent protection on the inventions disclosed in such PCT patent application.

Moreover, a provisional patent application is not eligible to become an issued patent. A provisional patent application may serve as a priority filing for a non-provisional patent application we file within 12 months of such provisional patent application. If we do not timely file non-provisional patent applications, we may lose our priority date with respect to our existing provisional patent applications and any potential patent protection on the inventions disclosed in our provisional patent applications.

While we intend to timely file additional provisional patent applications and national stage and non-provisional patent applications relating to our PCT patent applications, we cannot predict whether any of our patent applications will result in the issuance of patents. If we do not successfully obtain patent protection, or if the scope of the patent protection we or our licensors obtain with respect to our product candidates or technology is insufficient, we will be unable to use patent protection to prevent others from using our technology or from developing or commercializing technology and products similar or identical to ours or other similar competing products and technologies. Our ability to stop third parties from making, using, selling, offering to sell, importing or otherwise commercializing any of our technology, inventions and improvements, either directly or indirectly, will depend in part on our success in obtaining, maintaining, defending and enforcing patent claims that cover our technology, inventions and improvements.

The patent positions of companies like ours are generally uncertain and involve complex legal and factual questions. The protection afforded by a patent varies on a product-by-product basis, from jurisdiction-to-jurisdiction, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of patent term adjustments and regulatory-related patent term extensions, the availability of legal remedies in a particular jurisdiction and the validity and enforceability of the patent. Patent laws and related enforcement in various jurisdictions outside of the United States are uncertain and may not protect our rights to the same extent as the laws of the United States. Changes in the patent laws and rules, whether by legislation, judicial decisions or regulatory interpretation, in the United States and other jurisdictions may have uncertain affects that could improve or diminish our ability to protect our inventions and obtain, maintain, defend and enforce our patent rights, and could therefore affect the value of our business in uncertain ways.

The area of patent and other intellectual property rights in biotechnology is evolving and has many risks and uncertainties, and third parties may have blocking patents and other intellectual property that could be used to prevent us from commercializing our platform and product candidates and practicing our proprietary technology. Our patent rights may be challenged, narrowed, circumvented, invalidated or ruled unenforceable, which could limit our ability to stop third parties from marketing and commercializing related platforms or product candidates or limit the term of patents that cover our platform and any product candidates. In addition, the rights granted under any issued patents may not provide us with protection or competitive advantages against third parties with similar technology, and third parties may independently develop similar technologies.

Moreover, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that before any of our product candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any competitive advantage provided by the patent. For this and other risks related to our proprietary technology, inventions, improvements, platforms and product candidates and intellectual property rights related to the foregoing, please see the section entitled “Risk factors—Risks related to our intellectual property.”

Patent term

The term of individual patents depends upon the laws of the jurisdictions in which they are obtained. In most jurisdictions in which we file, the patent term is 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. However, the term of U.S. patents may be extended or adjusted for delays incurred due to compliance with FDA requirements or by delays encountered during prosecution that are caused by the United States Patent and Trademark Office, or the USPTO. For example, in the United States, a patent claiming a new biologic product, its method of use or its method of manufacture may be eligible for a limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, for up to five years beyond the normal expiration date of the patent. Patent term extension cannot be used to extend the remaining term of a patent past a total of 14 years from the product’s approval date in the United States. Only one patent applicable to an approved product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. During the period of extension, if granted, the scope of exclusivity is limited to the approved product for approved uses. Some foreign jurisdictions, including Europe and Japan, have analogous patent term extension provisions, which allow for extension of the term of a patent that covers a drug approved by the applicable foreign regulatory agency. For more information on patent term extensions, see “Business—Government regulation—Patent term restoration and extension.” In the future, if and when any product candidates we may develop receive FDA approval, we expect to apply for patent term extensions on issued patents covering those product candidates. Moreover, we intend to seek patent term adjustments and extensions for any of our issued patents in any jurisdiction where such adjustments and extensions are available. However, there is no guarantee that the applicable authorities, including the USPTO and FDA, will agree with our assessment of whether such adjustments and extensions should be granted, and even if granted, the length of such adjustments and extensions.

Trade secrets

In addition to patent protection, we also rely on trade secrets, know-how, unpatented technology and other proprietary information to strengthen our competitive position. We currently, and may continue in the future continue to, rely on third parties to assist us in developing and manufacturing our products. Accordingly, we must, at times, share trade secrets, know-how, unpatented technology and other proprietary information, including those related to our platform, with them. We may in the future also enter into research and development collaborations with third parties that may require us to share trade secrets, know-how, unpatented technology and other proprietary information under the terms of research and development partnerships or similar agreements. Nonetheless, we take steps to protect and preserve our trade secrets and other confidential and proprietary information and prevent the unauthorized disclosure of the foregoing, including by entering into non-disclosure and invention assignment agreements with parties who have access to our trade secrets or other confidential and proprietary information, such as employees, consultants, outside scientific collaborators, contract research and manufacturing organizations, sponsored researchers and other

advisors, at the commencement of their employment, consulting or other relationships with us. In addition, we take other appropriate precautions, such as maintaining physical security of our premises and physical and electronic security of our information technology systems, to guard against any misappropriation or unauthorized disclosure of our trade secrets and other confidential and proprietary information by third parties.

Despite these efforts, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or other confidential or proprietary information. In addition, we cannot provide any assurances that all of the foregoing non-disclosure and invention assignment agreements have been duly executed, and any of the counterparties to such agreements may breach them and disclose our trade secrets and other confidential and proprietary information. Although we have confidence in the measures we take to protect and preserve our trade secrets and other confidential and proprietary information, they may be inadequate, our agreements or security measures may be breached, and we may not have adequate remedies for such breaches. Moreover, to the extent that our employees, contractors, consultants, collaborators and advisors use intellectual property owned by others in their work for us, disputes may arise as to our rights in any know-how or inventions arising out of such work. For more information, please see the section entitled “Risk factors—Risks related to our intellectual property.”

License agreement with the University of Mons

In April 2020, we entered into a license agreement with UMONS, or the UMONS Agreement, pursuant to which UMONS granted to us an exclusive, worldwide license to certain patents and patent applications related to oligonucleotides for our FSHD program and a non-exclusive, worldwide license to existing, related know-how. Each of the issued patents licensed to us under the UMONS Agreement is scheduled to expire in 2031. The licenses under the UMONS Agreement confer on us the right to research, develop and commercialize products, which we refer to as licensed products, and to practice processes, in each case, covered by the licensed patents and existing, related know-how.

Under the UMONS Agreement, we are obligated to use commercially reasonable efforts to develop at least one licensed product and, to the extent regulatory approval is obtained in such jurisdictions, to commercialize at least one licensed product in the United States and the United Kingdom or a member country of the European Union. Unless terminated earlier, the UMONS Agreement will remain in effect until the last to expire of the licensed patent rights on a licensed product-by-licensed product and country-by-country basis. UMONS may terminate the UMONS Agreement in the event of a material breach by us and our failure to cure such breach within a specified time period. We may voluntarily terminate the UMONS Agreement with prior notice to UMONS.

In connection with our entry into the UMONS Agreement, we paid UMONS an upfront payment of €50,000. We also agreed to make milestone payments to UMONS upon the achievement of specified development and regulatory milestones up to a maximum aggregate total of €400,000 for the first licensed product to achieve such milestones and up to a maximum aggregate total of €200,000 for each subsequent licensed product to achieve each such milestones, as well as a low single-digit percentage royalty on net sales of licensed products by us, our affiliates and sublicensees. These royalty obligations continue on a licensed product-by-licensed product and country-by-country basis until the expiration of the last licensed patent rights covering such licensed product in such country. In addition, if we sublicense rights under the UMONS Agreement, we are required to pay a low double-digit percentage of the sublicense revenue to UMONS. Additionally, if we choose to file, prosecute or maintain any patents included in the licensed patent rights under the UMONS Agreement, we will be required to bear the full cost and expenses of preparing, filing, prosecuting and maintaining any such patents.

Competition

The biotechnology and biopharmaceutical industries generally, and the muscle disease field specifically, are characterized by rapid evolution of technologies, sharp competition and strong defense of intellectual property. Any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our technology, development experience and scientific knowledge in the field of muscle diseases, oligonucleotide therapeutics and manufacturing provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions.

There are currently no approved therapies to treat the underlying cause of DM1. Product candidates currently in development to treat DM1 include: tideglusib, a GSK3-ß inhibitor in late-stage clinical development by AMO Pharma Ltd. for the congenital phenotype of DM1; AT466, which is an AAV-antisense candidate in preclinical development by Audentes Therapeutics, Inc.; an antibody linked siRNA in preclinical development by Avidity Biosciences, Inc.; gene editing treatments in preclinical development by Vertex Pharmaceuticals, Inc., or Vertex; an RNA-targeting gene therapy in preclinical development by Locana, Inc.; and small molecules interacting with RNA in preclinical development by Expansion Therapeutics, Inc.

Currently, patients with DMD are treated with corticosteroids to manage the inflammatory component of the disease. EMFLAZA (deflazacort) is an FDA-approved corticosteroid marketed by PTC Therapeutics, Inc., or PTC. In addition, there are three FDA-approved exon skipping drugs: EXONDYS 51 (eteplirsen) and VYONDYS 53 (golodirsen), which are naked PMOs approved for the treatment of DMD patients amenable to Exon 51 and Exon 53 skipping, respectively, and are marketed by Sarepta Therapeutics, Inc., or Sarepta, and VILTEPSO (vitolarsen), a naked PMO approved for the treatment of DMD patients amenable to Exon 53 skipping, which is marketed by Nippon Shinyaku Co. Ltd. Companies focused on developing treatments for DMD that target dystrophin mechanisms, as does our DMD program, include Sarepta with SRP-5051, a peptide-linked PMO currently being evaluated in a Phase 2 clinical trial for patients amenable to Exon 51 skipping, PTC with ataluren, a small molecule targeting nonsense mutations in a Phase 3 clinical trial, and Avidity Biosciences, Inc., which is in preclinical development with an antibody oligonucleotide conjugate that targets dystrophin production. In addition, several companies are developing gene therapies to treat DMD, including Milo Biotechnology (AAV1-FS344), Pfizer Inc. (PF-06939926), Sarepta (SRP-9001 and Galgt2 gene therapy program), and Solid Biosciences Inc. (SGT-001). Gene editing treatments that are in preclinical development are also being pursued by Vertex and Sarepta. We are also aware of several companies targeting non-dystrophin mechanisms for the treatment of DMD.

There are currently no therapies to treat the underlying cause of FSHD. Products currently in development to treat FSHD include: creatine monohydrate, a supplement that enhances muscle performance, which is being evaluated in a Phase 2 clinical trial by Murdoch Children's Research Institute, and losmapimod, a p38 MAPK inhibitor that may modulate DUX4 expression, which is being evaluated in a Phase 2 clinical trial by Fulcrum Therapeutics Inc.

We will also compete more generally with other companies developing alternative scientific and technological approaches, including other companies working to develop conjugates with oligonucleotides for extra-hepatic delivery, including Alnylam Pharmaceuticals, Aro Biotherapeutics, Arrowhead Therapeutics, Avidity Biosciences, Dicerna Pharmaceuticals, Inc., Ionis Pharmaceuticals and Sarepta, as well as gene therapy and gene editing approaches.

Many of our competitors, either independently or with strategic partners, have substantially greater financial, technical and human resources than we do. Accordingly, our competitors may be more successful than we are

in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approval for treatments and achieving widespread market acceptance. Merger and acquisition activity in the biotechnology and biopharmaceutical industries may result in resources being concentrated among a smaller number of our competitors. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials and acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be substantially limited if our competitors develop and commercialize products that are more effective, safer, less toxic, more convenient or less expensive than products we may develop. In geographies that are critical to our commercial success, competitors may also obtain regulatory approvals before us, resulting in our competitors building a strong market position in advance of the entry of our products. In addition, our ability to compete may be affected in many cases by insurers or other third-party payers seeking to encourage the use of other drugs. The key competitive factors affecting the successful of all any products we may develop are likely to be their efficacy, safety, convenience, price and availability of reimbursement.

Government regulation

Government authorities in the United States, at the federal, state and local level and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, pricing, reimbursement, sales, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting and import and export of pharmaceutical products, including biological products. The processes for obtaining marketing approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

Licensure and regulation of biologics in the United States

In the United States, any product candidates we may develop would be regulated as biological products, or biologics, under the Public Health Service Act, or PHSA, and the Federal Food, Drug and Cosmetic Act, or FDCA, and their implementing regulations and guidance. The failure to comply with the applicable U.S. requirements at any time during the product development process, including preclinical testing, clinical testing, the approval process, or post-approval process, may subject an applicant to delays in the conduct of the study, regulatory review and approval and/or administrative or judicial sanctions. These sanctions may include, but are not limited to, the FDA's refusal to allow an applicant to proceed with clinical testing, refusal to approve pending applications, license suspension, or revocation, withdrawal of an approval, warning letters, adverse publicity, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines and civil or criminal investigations and penalties brought by the FDA or the Department of Justice, or DOJ, and other governmental entities, including state agencies.

An applicant seeking approval to market and distribute a new biologic in the United States generally must satisfactorily complete each of the following steps:

- preclinical laboratory tests, animal studies and formulation studies all performed in accordance with the FDA's Good Laboratory Practices, or GLP regulations;
- completion of the manufacture, under cGMP conditions, of the drug substance and drug product that the sponsor intends to use in human clinical trials along with required analytical and stability testing;

- submission to the FDA of an IND application for human clinical testing, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety, potency and purity of the product candidate for each proposed indication, in accordance with current Good Clinical Practices, or GCP;
- preparation and submission to the FDA of a BLA for a biologic product requesting marketing for one or more proposed indications, including submission of detailed information on the manufacture and composition of the product in clinical development and proposed labelling;
- review of the product by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities, including those of third parties, at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of any FDA audits of the preclinical studies and clinical trial sites to assure compliance with GLP, as applicable, and GCP, and the integrity of clinical data in support of the BLA;
- payment of user Prescription Drug User Fee Act, or PDUFA, securing FDA approval of the BLA and licensure of the new biologic product; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and any post-approval studies or other post-marketing commitments required by the FDA.

Preclinical studies and investigational new drug application

Before testing any biologic product candidate in humans, including an antibody, the product candidate must undergo preclinical testing. Preclinical tests include laboratory evaluations of product chemistry, formulation and stability, as well as studies to evaluate the potential for efficacy and toxicity in animal studies. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND application.

An IND is an exemption from the FDCA that allows an unapproved product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer such investigational product to humans. The IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions about the product or conduct of the proposed clinical trial, including concerns that human research subjects will be exposed to unreasonable health risks. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns before the clinical trials can begin or recommence.

As a result, submission of the IND may result in the FDA not allowing the trials to commence or allowing the trial to commence on the terms originally specified by the sponsor in the IND. If the FDA raises concerns or questions either during this initial 30-day period, or at any time during the IND review process, it may choose to impose a partial or complete clinical hold. Clinical holds are imposed by the FDA whenever there is concern for

patient safety, may be a result of new data, findings, or developments in clinical, preclinical and/or chemistry, manufacturing and controls or where there is non-compliance with regulatory requirements. This order issued by the FDA would delay either a proposed clinical trial or cause suspension of an ongoing trial, until all outstanding concerns have been adequately addressed and the FDA has notified the company that investigations may proceed. This could cause significant delays or difficulties in completing our planned clinical trial or future clinical trials in a timely manner.

Expanded access to an investigational drug for treatment use

Expanded access, sometimes called “compassionate use,” is the use of investigational products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational products for patients who may benefit from investigational therapies. FDA regulations allow access to investigational products under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the investigational product under a treatment protocol or treatment IND application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its drug products available for expanded access; however, as required by the 21st Century Cures Act, or Cures Act, passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Although these requirements were rolled out over time, they have now come into full effect. This provision requires drug and biologic companies to make publicly available their policies for expanded access for individual patient access to products intended for serious diseases. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 trial; or 15 days after the investigational drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a manufacturer to make its investigational products available to eligible patients as a result of the Right to Try Act.

Human clinical trials in support of a BLA

Clinical trials involve the administration of the investigational product candidate to healthy volunteers or patients with the disease or condition to be treated under the supervision of a qualified principal investigator in

accordance with GCP requirements. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, inclusion and exclusion criteria, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND.

A sponsor who wishes to conduct a clinical trial outside the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. When a foreign clinical trial is conducted under an IND, all FDA IND requirements must be met unless waived. When a foreign clinical trial is not conducted under an IND, the sponsor must ensure that the trial complies with certain regulatory requirements of the FDA in order to use the trial as support for an IND or application for marketing approval. Specifically, the FDA requires that such trials be conducted in accordance with GCP, including review and approval by an independent ethics committee and informed consent from participants. The GCP requirements encompass both ethical and data integrity standards for clinical trials. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical trials, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign trials are conducted in a manner comparable to that required for clinical trials in the United States.

Further, each clinical trial must be reviewed and approved by an IRB either centrally or individually at each institution at which the clinical trial will be conducted. The IRB will consider, among other things, clinical trial design, patient informed consent, ethical factors, the safety of human subjects, and the possible liability of the institution. An IRB must operate in compliance with FDA regulations. The FDA, IRB, or the clinical trial sponsor may suspend or discontinue a clinical trial at any time for various reasons, including a finding that the clinical trial is not being conducted in accordance with FDA requirements or that the participants are being exposed to an unacceptable health risk. Clinical testing also must satisfy extensive GCP rules and the requirements for informed consent.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board, or DSMB. This group may recommend continuation of the trial as planned, changes in trial conduct, or cessation of the trial at designated check points based on certain available data from the trial to which only the DSMB has access. Finally, research activities involving infectious agents, hazardous chemicals, recombinant DNA and genetically altered organisms and agents may be subject to review and approval of an Institutional Biosafety Committee, or IBC, in accordance with NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules.

Clinical trials typically are conducted in three sequential phases, but the phases may overlap or be combined. Additional studies may be required after approval.

- *Phase 1* clinical trials are initially conducted in a limited population to test the product candidate for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion and pharmacodynamics in healthy subjects or patients.
- *Phase 2* clinical trials are generally conducted in a limited patient population to identify possible adverse effects and safety risks, evaluate the efficacy of the product candidate for specific targeted indications and determine dose tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials.
- *Phase 3* clinical trials proceed if the Phase 2 clinical trials demonstrate that a dose range of the product candidate is potentially effective and has an acceptable safety profile. Phase 3 clinical trials are undertaken within an expanded patient population to further evaluate dosage, provide substantial evidence of clinical efficacy and further test for safety in an expanded and diverse patient population at multiple, geographically dispersed clinical trial sites. A well-controlled, statistically robust Phase 3 trial may be designed to deliver the

data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a biologic; such Phase 3 studies are referred to as “pivotal.”

In some cases, the FDA may approve a BLA for a product but require the sponsor to conduct additional clinical trials to further assess the product’s safety and effectiveness after approval. Such post-approval trials are typically referred to as Phase 4 clinical trials. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of biologics approved under accelerated approval regulations. If the FDA approves a product while a company has ongoing clinical trials that were not necessary for approval, a company may be able to use the data from these clinical trials to meet all or part of any Phase 4 clinical trial requirement or to request a change in the product labeling. The failure to exercise due diligence with regard to conducting Phase 4 clinical trials could result in withdrawal of approval for products.

Under the Pediatric Research Equity Act of 2003, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA’s internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of an applicant, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments. In addition, FDA will meet early in the development process to discuss pediatric study plans with sponsors and FDA must meet with sponsors by no later than the end-of-phase 1 meeting for serious or life-threatening diseases and by no later than 90 days after FDA’s receipt of the study plan.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the Food and Drug Administration Safety and Innovation Act. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Information about applicable clinical trials must be submitted within specific timeframes to the NIH for public dissemination on its ClinicalTrials.gov website.

Compliance with cGMP requirements

Before approving a BLA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in full compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. The PHSA emphasizes the importance of manufacturing control for products like biologics whose attributes cannot be precisely defined.

Manufacturers and others involved in the manufacture and distribution of products must also register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing

establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Any product manufactured by or imported from a facility that has not registered, whether foreign or domestic, is deemed misbranded under the FDCA. Establishments may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. Inspections must follow a "risk-based schedule" that may result in certain establishments being inspected more frequently. Manufacturers may also have to provide, on request, electronic or physical records regarding their establishments. Delaying, denying, limiting, or refusing inspection by the FDA may lead to a product being deemed to be adulterated.

Review and approval of a BLA

The results of product candidate development, preclinical testing and clinical trials, including negative or ambiguous results as well as positive findings, are submitted to the FDA as part of a BLA requesting license to market the product. The BLA must contain extensive manufacturing information and detailed information on the composition of the product and proposed labeling as well as payment of a user fee. Under federal law, the submission of most BLAs is subject to an application user fee. The sponsor of a licensed BLA is also subject to an annual program fee. Certain exceptions and waivers are available for some of these fees, such as an exception from the application fee for products with orphan designation and a waiver for certain small businesses.

The FDA has 60 days after submission of the application to conduct an initial review to determine whether to accept it for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission has been accepted for filing, the FDA begins an in-depth review of the application. Under the goals and policies agreed to by the FDA under the PDUFA, the FDA has ten months in which to complete its initial review of a standard application and respond to the applicant, and six months for a priority review of the application. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs. The review process may often be significantly extended by FDA requests for additional information or clarification. The review process and the PDUFA goal date may be extended by three months if the FDA requests or if the applicant otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Under the PHSA, the FDA may approve a BLA if it determines that the product is safe, pure and potent, and the facility where the product will be manufactured meets standards designed to ensure that it continues to be safe, pure and potent. On the basis of the FDA's evaluation of the application and accompanying information, including the results of the inspection of the manufacturing facilities and any FDA audits of preclinical and clinical trial sites to assure compliance with GCPs, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. If the application is not approved, the FDA will issue a complete response letter, which will contain the conditions that must be met in order to secure final approval of the application, and when possible will outline recommended actions the sponsor might take to obtain approval of the application. Sponsors that receive a complete response letter may submit to the FDA information that represents a complete response to the issues identified by the FDA. Such resubmissions are classified under PDUFA as either Class 1 or Class 2. The classification of a resubmission is based on the information submitted by an applicant in response to an action letter. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has two months to review a Class 1 resubmission and six months to review a Class 2 resubmission. The FDA will not approve an application until issues identified in the complete response letter have been addressed.

The FDA may also refer the application to an advisory committee for review, evaluation and recommendation as to whether the application should be approved. In particular, the FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee.

Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

If the FDA approves a new product, it may limit the approved indication(s) for use of the product. It may also require that contraindications, warnings, or precautions be included in the product labeling. In addition, the FDA may call for post-approval studies, including Phase 4 clinical trials, to further assess the product's efficacy and/or safety after approval. The agency may also require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, to help ensure that the benefits of the product outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patent registries. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Fast track, breakthrough therapy, priority review and regenerative medicine advanced therapy designations

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs are referred to as fast track designation, breakthrough therapy designation, priority review designation and regenerative medicine advanced therapy, or RMAT, designation. These designations are not mutually exclusive, and a product candidate may qualify for one or more of these programs. While these programs are intended to expedite product development and approval, they do not alter the standards for FDA approval.

Specifically, the FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the application is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Second, in 2012, Congress enacted the Food and Drug Administration Safety and Innovation Act. This law established a new regulatory scheme allowing for expedited review of products designated as "breakthrough therapies." A product may be designated as a breakthrough therapy if it is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review

process; assigning a cross-disciplinary project lead for the review team; and taking other steps to design the clinical trials in an efficient manner. Breakthrough designation may be rescinded if a product no longer meets the qualifying criteria.

Third, the FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines, on a case-by-case basis, whether the proposed product represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting product reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months. Priority designation may be rescinded if a product no longer meets the qualifying criteria.

With passage of the Cures Act in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative medicine advanced therapies. A product is eligible for RMAT designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative medicine advanced therapy designation include early interactions with FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review, and accelerated approval based on surrogate or intermediate endpoints. RMAT designation may be rescinded if a product no longer meets the qualifying criteria.

Accelerated approval pathway

The FDA may grant accelerated approval to a product for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Products granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a product, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a product.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a product, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used

extensively in the development and approval of products for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. As a result, a product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, confirm a clinical benefit during post-marketing studies or dissemination of false or misleading promotional materials would allow the FDA to withdraw the product from the market on an expedited basis. All promotional materials for product candidates approved under accelerated regulations are subject to prior review by the FDA.

Post-approval regulation

If regulatory approval for marketing of a product or new indication for an existing product is obtained, the sponsor will be required to comply with all regular post-approval regulatory requirements as well as any post-approval requirements that the FDA have imposed as part of the approval process. The sponsor will be required to report certain adverse reactions and production problems to the FDA, provide updated safety and efficacy information and comply with requirements concerning advertising and promotional labeling requirements. Manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMP regulations, which impose certain procedural and documentation requirements upon manufacturers. Accordingly, the sponsor and its third-party manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMP regulations and other regulatory requirements.

A product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official lot release, the manufacturer must submit samples of each lot, together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot, to the FDA. The FDA may in addition perform certain confirmatory tests on lots of some products before releasing the lots for distribution. Finally, the FDA will conduct laboratory research related to the safety, purity, potency and effectiveness of pharmaceutical products.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;

- product recall, seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

Pharmaceutical products may be promoted only for the approved indications and in accordance with the provisions of the approved label. Although healthcare providers may prescribe products for uses not described in the drug's labeling, known as off-label uses, in their professional judgment, drug manufacturers are prohibited from soliciting, encouraging or promoting unapproved uses of a product. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

The FDA strictly regulates the marketing, labeling, advertising and promotion of prescription drug products placed on the market. This regulation includes, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities and promotional activities involving the Internet and social media. Promotional claims about a drug's safety or effectiveness are prohibited before the drug is approved. After approval, a drug product generally may not be promoted for uses that are not approved by the FDA, as reflected in the product's prescribing information. In the United States, healthcare professionals are generally permitted to prescribe drugs for such off-label uses because the FDA does not regulate the practice of medicine. However, FDA regulations impose rigorous restrictions on manufacturers' communications, prohibiting the promotion of off-label uses. It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information.

If a company is found to have promoted off-label uses, it may become subject to adverse public relations and administrative and judicial enforcement by the FDA, the DOJ, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

Orphan drug designation

Orphan drug designation in the United States is designed to encourage sponsors to develop products intended for treatment of rare diseases or conditions. In the United States, a rare disease or condition is statutorily defined as a condition that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available the biologic for the disease or condition will be recovered from sales of the product in the United States.

Orphan drug designation qualifies a company for tax credits and market exclusivity for seven years following the date of the product's marketing approval if granted by the FDA. An application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. A product becomes an orphan when it receives orphan drug designation from the Office of Orphan Products Development at the FDA based on acceptable confidential requests made under the regulatory provisions. The product must then go through the review and approval process like any other product.

A sponsor may request orphan drug designation of a previously unapproved product or new orphan indication for an already marketed product. In addition, a sponsor of a product that is otherwise the same product as an

already approved orphan drug may seek and obtain orphan drug designation for the subsequent product for the same rare disease or condition if it can present a plausible hypothesis that its product may be clinically superior to the first drug. More than one sponsor may receive orphan drug designation for the same product for the same rare disease or condition, but each sponsor seeking orphan drug designation must file a complete request for designation.

If a product with orphan designation receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will receive orphan drug exclusivity. Orphan drug exclusivity means that the FDA may not approve another sponsor's marketing application for the same product for the same indication for seven years, except in certain limited circumstances. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity.

The period of exclusivity begins on the date that the marketing application is approved by the FDA and applies only to the indication for which the product has been designated. The FDA may approve a second application for the same product for a different use or a second application for a clinically superior version of the product for the same use. The FDA cannot, however, approve the same product made by another manufacturer for the same indication during the market exclusivity period unless it has the consent of the sponsor or the sponsor is unable to provide sufficient quantities.

Pediatric exclusivity

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity that cover the product are extended by six months.

Biosimilars and exclusivity

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or, collectively, the ACA, which was signed into law in March 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. The BPCIA established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. A biosimilar is a biological product that is highly similar to an existing FDA-licensed "reference product." As of January 1, 2020, the FDA has approved 26 biosimilar products for use in the United States. No interchangeable biosimilars, however, have been approved. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars. Additional guidances are expected to be finalized by the FDA in the near term. Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the agency must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one

has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of approval of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was approved. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law. Since the passage of the BPCIA, many states have passed laws or amendments to laws, including laws governing pharmacy practices, which are state-regulated, to regulate the use of biosimilars.

Federal and state data privacy and security laws

Under the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, the U.S. Department of Health and Human Services, or HHS, has issued regulations to protect the privacy and security of protected health information, or PHI, used or disclosed by covered entities including certain healthcare providers, health plans and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 or HITECH, and their regulations, including the final omnibus rule published on January 25, 2013, also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that are applicable to our business. In addition to possible federal administrative, civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. Accordingly, state attorneys general have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. New laws and regulations governing privacy and security may be adopted in the future as well.

Additionally, California recently enacted legislation that has been dubbed the first "GDPR-like" law in the United States. Known as the California Consumer Privacy Act, or CCPA, it creates new individual privacy rights for consumers (as that word is broadly defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA went into effect on January 1, 2020 and requires covered companies to provide new disclosures to California consumers, provide such consumers new ways to opt-out of certain sales of personal information, and allow for a new cause of action for data breaches. The CCPA could impact our business activities depending on how it is interpreted and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and protected health information.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply

with different privacy compliance and/or reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil and administrative penalties, damages, fines, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a consent decree or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

Patent term restoration and extension

In the United States, a patent claiming a new biologic product, its method of use or its method of manufacture may be eligible for a limited patent term extension under the Hatch-Waxman Act, which permits a patent extension of up to five years for patent term lost during product development and FDA regulatory review. Assuming grant of the patent for which the extension is sought, the restoration period for a patent covering a product is typically one-half the time between the effective date of the IND involving human beings and the submission date of the BLA, plus the time between the submission date of the BLA and the ultimate approval date. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date in the United States. Only one patent applicable to an approved product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. The USPTO reviews and approves the application for any patent term extension in consultation with the FDA.

Regulation and procedures governing approval of medicinal products in the European Union

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, an applicant will need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. Specifically, the process governing approval of medicinal products in the European Union generally follows the same lines as in the United States. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing authorization by these authorities before the product can be marketed and sold in the European Union.

Clinical trial approval

Pursuant to the currently applicable Clinical Trials Directive 2001/20/EC and the Directive 2005/28/EC on GCP, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the member states. Under this system, an applicant must obtain approval from the competent national authority of a European Union member state in which the clinical trial is to be conducted, or in multiple member states if the clinical trial is to be conducted in a number of member states. Furthermore, the applicant

may only start a clinical trial at a specific site after the competent ethics committee has issued a favorable opinion. The clinical trial application must be accompanied by an investigational medicinal product dossier with supporting information prescribed by Directive 2001/20/EC and Directive 2005/28/EC and corresponding national laws of the member states and further detailed in applicable guidance documents.

In April 2014, the European Union adopted a new Clinical Trials Regulation (EU) No 536/2014, but it has not yet become effective. It will overhaul the current system of approvals for clinical trials in the European Union. Specifically, the new legislation, which will be directly applicable in all member states, aims at simplifying and streamlining the approval of clinical trials in the European Union. For instance, the new Clinical Trials Regulation provides for a streamlined application procedure via a single-entry point and strictly defined deadlines for the assessment of clinical trial applications. As of January 1, 2020, the website of the European Commission reported that the implementation of the new Clinical Trials Regulation was dependent on the development of a fully functional clinical trials portal and database, which would be confirmed by an independent audit, and that the new legislation would come into effect six months after the European Commission publishes a notice of this confirmation. The website indicated that the audit was expected to commence in December 2020. Parties conducting certain clinical trials must, as in the United States, post clinical trial information in the European Union at the EudraCT website: https://eudract.ema.europa.eu.

PRIME designation in the European Union

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRIority MEdicines, or PRIME, scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated marketing authorization application assessment once a dossier has been submitted. Importantly, a dedicated EMA contact and rapporteur from the Committee for Human Medicinal Products, or CHMP, or Committee for Advanced Therapies are appointed early in the PRIME scheme facilitating increased understanding of the product at the EMA's Committee level. A kick-off meeting initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies.

Marketing authorization

To obtain a marketing authorization for a product under the European Union regulatory system, an applicant must submit an MAA, either under a centralized procedure administered by the EMA or one of the procedures administered by competent authorities in European Union Member States (decentralized procedure, national procedure, or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the European Union. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the European Union, an applicant must demonstrate compliance with all measures included in an EMA-approved Pediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, class waiver or a deferral for one or more of the measures included in the PIP.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all European Union member states. Pursuant to Regulation (EC) No. 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain

biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases, including products for the treatment of cancer. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional. Manufacturers must demonstrate the quality, safety and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

Under the centralized procedure, the CHMP established at the EMA is responsible for conducting an initial assessment of a product. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated evaluation may be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts such a request, the time limit of 210 days will be reduced to 150 days, but it is possible that the CHMP may revert to the standard time limit for the centralized procedure if it determines that it is no longer appropriate to conduct an accelerated assessment.

Regulatory data protection in the European Union

In the European Union, new chemical entities approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity pursuant to Regulation (EC) No 726/2004, as amended, and Directive 2001/83/EC, as amended. Data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application for a period of eight years. During the additional two-year period of market exclusivity, a generic marketing authorization application can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to authorization, is held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity so that the innovator gains the prescribed period of data exclusivity, another company may market another version of the product if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

Patent term extensions in the European Union and other jurisdictions

The European Union also provides for patent term extension through Supplementary Protection Certificates, or SPCs. The rules and requirements for obtaining a SPC are similar to those in the United States. An SPC may extend the term of a patent for up to five years after its originally scheduled expiration date and can provide up to a maximum of fifteen years of marketing exclusivity for a drug. In certain circumstances, these periods may be extended for six additional months if pediatric exclusivity is obtained. Although SPCs are available throughout the European Union, sponsors must apply on a country-by-country basis. Similar patent term extension rights exist in certain other foreign jurisdictions outside the European Union.

Periods of authorization and renewals

A marketing authorization is valid for five years, in principle, and it may be renewed after five years on the basis of a reevaluation of the risk-benefit balance by the EMA or by the competent authority of the authorizing

member state. To that end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. Once renewed, the marketing authorization is valid for an unlimited period, unless the European Commission or the competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal period. Any authorization that is not followed by the placement of the drug on the European Union market (in the case of the centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid.

Regulatory requirements after marketing authorization

Following approval, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of the medicinal product. These include compliance with the European Union's stringent pharmacovigilance or safety reporting rules, pursuant to which post-authorization studies and additional monitoring obligations can be imposed. In addition, the manufacturing of authorized products, for which a separate manufacturer's license is mandatory, must also be conducted in strict compliance with the EMA's GMP requirements and comparable requirements of other regulatory bodies in the European Union, which mandate the methods, facilities and controls used in manufacturing, processing and packing of drugs to assure their safety and identity. Finally, the marketing and promotion of authorized products, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the European Union under Directive 2001/83EC, as amended.

Orphan drug designation and exclusivity

Regulation (EC) No 141/2000 and Regulation (EC) No. 847/2000 provide that a product can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the European Union when the application is made, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that the marketing of the drug in the European Union would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or, if such method exists, the drug will be of significant benefit to those affected by that condition.

An orphan drug designation provides a number of benefits, including fee reductions, regulatory assistance and the possibility to apply for a centralized European Union marketing authorization. Marketing authorization for an orphan drug leads to a ten-year period of market exclusivity. During this market exclusivity period, neither the EMA nor the European Commission or the member states can accept an application or grant a marketing authorization for a "similar medicinal product." A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation because, for example, the product is sufficiently profitable not to justify market exclusivity.

Brexit and the regulatory framework in the United Kingdom

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020, which is extendable up to two years. Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult to date. To date, only an outline of a trade agreement has been reached. Much remains open but the Prime Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption. The Prime Minister has also indicated that the United Kingdom will not accept high regulatory alignment with the European Union.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for any product candidates we may develop, which could significantly and materially harm our business.

Furthermore, while the Data Protection Act of 2018 in the United Kingdom that “implements” and complements the European Union’s General Data Protection Regulation, or GDPR, has achieved Royal Assent on May 23, 2018 and is now effective in the United Kingdom, it is still unclear whether transfer of data from the European Economic Area, or EEA, to the United Kingdom will remain lawful under GDPR. During the period of “transition” (i.e., until December 31, 2020), European Union law will continue to apply in the United Kingdom, including the GDPR, after which the GDPR will be converted into United Kingdom law. Beginning in 2021, the United Kingdom will be a “third country” under the GDPR. We may, however, incur liabilities, expenses, costs and other operational losses under GDPR and applicable European Union Member States and the United Kingdom privacy laws in connection with any measures we take to comply with them.

General Data Protection Regulation

The collection, use, disclosure, transfer or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance.

Coverage, pricing and reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we may seek regulatory approval by the FDA or other government authorities. In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payers to reimburse all or part of the associated healthcare costs. Patients are unlikely to use any product candidates we may develop unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of such product candidates. Even if any product candidates we may develop are approved, sales of such product candidates will depend, in part, on the extent to which third-party payers, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage and establish adequate reimbursement levels for, such product candidates. The process for determining whether a payer will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payer will pay for the product once coverage is approved. Third-party payers are increasingly challenging the prices charged, examining the medical necessity, and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payers may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable marketing approvals. Nonetheless, product candidates may not be considered medically necessary or cost effective. A decision by a third-party payer not to cover any product candidates we may develop could reduce physician utilization of such product candidates once approved and have a material adverse effect on our sales, results of operations and financial condition. Additionally, a payer's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payer's determination to provide coverage for a product does not assure that other payers will also provide coverage and reimbursement for the product, and the level of coverage and reimbursement can differ significantly from payer to payer. Third-party reimbursement and coverage may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. In addition, any companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to any companion diagnostics.

The containment of healthcare costs also has become a priority of federal, state and foreign governments and the prices of pharmaceuticals have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit a company's revenue generated from the sale of any approved products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which a company or its collaborators receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If we obtain approval in the future to market in the United States any product candidates we may develop, we may be required to provide discounts or rebates under government healthcare programs or to certain
government and private purchasers in order to obtain coverage under federal healthcare programs such as

Medicaid. Participation in such programs may require us to track and report certain drug prices. We may be subject to fines and other penalties if we fail to report such prices accurately.

Outside the United States, ensuring adequate coverage and payment for any product candidates we may develop will face challenges. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us to conduct a clinical trial that compares the cost effectiveness of any product candidates we may develop to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on healthcare costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states, and parallel trade (arbitrage between low-priced and high-priced member states), can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

Healthcare law and regulation

Healthcare providers and third-party payers play a primary role in the recommendation and prescription of pharmaceutical products that are granted marketing approval. Arrangements with providers, consultants, third-party payers and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, reporting of payments to physicians and teaching hospitals and patient privacy laws and regulations and other healthcare laws and regulations that may constrain our business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, false statement laws and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly

presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious or fraudulent or knowingly making, using or causing to made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal regulations relating to pricing and submission of pricing information for government programs, including penalties for knowingly and intentionally overcharging 340b eligible entities and the submission of false or fraudulent pricing information to government entities;
- HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by HITECH, and their respective implementing regulations, including the final omnibus rule published in January 2013, which impose obligations, including mandatory contractual terms, on certain covered healthcare providers, health plans and healthcare clearinghouses, as well as their respective business associates that perform services for them, that involve the use, or disclosure of, individually identifiable health information, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the Foreign Corrupt Practices Act, which prohibits companies and their intermediaries from making, or offering or promising to make improper payments to non-U.S. officials for the purpose of obtaining or retaining business or otherwise seeking favorable treatment;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the ACA, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the U.S. Department of Health and Human Services, information related to payments and other transfers of value made by that entity to physicians, as defined by such law, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payers, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring pharmaceutical manufacturers to report information related to payments to physicians and other healthcare providers, drug pricing or marketing expenditures. In addition, certain state and local laws require drug manufacturers to register pharmaceutical sales representatives. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, exclusion from government funded healthcare programs, such as Medicare and Medicaid, imprisonment, integrity oversight and reporting obligations and the curtailment or restructuring of our operations.

Healthcare reform

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and

biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the ACA, which, among other things, includes changes to the coverage and payment for products under government healthcare programs.

Since enactment of the ACA, there have been, and continue to be, numerous judicial, administrative, executive, and legislative challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, various portions of the ACA are currently undergoing legal and constitutional challenges in the U.S. Supreme Court. It is unclear whether the ACA will be overturned, repealed, replaced, or further amended. We cannot predict what affect further changes to the ACA would have on our business.

Further, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the Trump Administration's budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower cost generic and biosimilar drugs. While some of these and other measures may require additional authorization to become effective, Congress and the Trump Administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

More recently, President Trump has issued five executive orders that are intended to lower the costs of prescription drug products. The first order would require all federally qualified health centers, or FQHCs, to pass on to patients the discounts the health centers receive on insulin and epinephrine through Medicare's 340B Drug Discount Program.

The second order would establish an international pricing index that would set the price Medicare Part B pays for the costliest medications covered under the program to the lowest price in other economically advanced countries. At the time that the President announced this order, he also indicated that for the time being it would not be implemented.

The third order is intended to reduce the costs of drugs by supporting the safe importation of prescription drugs. Specifically, the order calls upon HHS to facilitate grants to individuals of waivers of the prohibition of importation of prescription drugs that would allow patients to import FDA approved drug products from abroad, so long as doing so would result in lower costs. In addition, the order would allow wholesalers and pharmacies to re-import both biological drugs and insulin that were originally manufactured in the United States and then exported for international sale. This action follows the publication of a proposed rulemaking on December 23, 2019, that, if finalized, would allow states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants would be required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. At the same time, the FDA issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

The fourth executive order would end drug rebates used by health plan sponsors, pharmacies or pharmacy benefit managers, or PBMs, in operating the Medicare Part D program. Specifically, the order directs HHS to

exclude from safe harbor protections under the federal anti-kickback statute retroactive price reductions that are not applied at the point-of-sale. Instead, the order requires HHS to establish new safe harbors that would allow health plan sponsors, pharmacies and PBMs to pass on those discounts to consumers at point-of-sale “in order to lower the patient’s out-of-pocket costs” and “permit the use of certain bona fide PBM service fees.” Each of these orders directs the federal government to implement the initiatives outlined in the orders, meaning they will not have immediate effects.

Finally, the fifth order instructs the federal government to develop a list of “essential” medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including China. The order is meant to reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States.

These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for any product candidates we may develop or additional pricing pressures.

Employees

As of August 31, 2020, we had 36 full-time employees, including a total of 12 employees with M.D. or Ph.D. degrees. Of these full-time employees, 28 employees are engaged in research and development. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Facilities

Our principal facilities consist of office and laboratory space. We occupy approximately 15,000 square feet of office and laboratory space in Waltham, Massachusetts under a sublease that currently expires in December 2021. We believe our facilities are adequate and suitable for our current needs and that should it be needed, suitable additional or alternative space will be available to accommodate our operations.

Legal proceedings

We are not currently subject to any material legal proceedings.

Management

Executive officers and directors

Executive Officers

The following table sets forth certain information concerning our executive officers, including their ages as of August 31, 2020:

Name	Age	Position
Joshua Brumm	42	Chief Executive Officer, President and Director
Romesh Subramanian, Ph.D.	55	Chief Scientific Officer
Susanna High	53	Chief Operating Officer
Oxana Beskrovnaya, Ph.D.	59	Senior Vice President, Head of Research
Jonathan McNeill, M.D.	35	Vice President, Business Development
Richard Scalzo	34	Vice President of Accounting and Administration

The following is a biographical summary of the experience of our executive officers.

Joshua Brumm has served as our Chief Executive Officer and President and as a director since October 2019. Prior to joining us, Mr. Brumm served as Chief Operating Officer and Chief Financial Officer at Kaleido Biosciences, Inc., a healthcare company, from April 2018 to October 2019. Prior to joining Kaleido, Mr. Brumm served as Chief Operating Officer and Chief Financial Officer at Versartis, Inc., a biopharmaceutical company, from November 2013 until December 2017. Mr. Brumm served as Executive Vice President of Finance at Pharmacyclics, Inc., a biopharmaceutical company, from August 2012 to August 2013. Prior to joining Pharmacyclics, Mr. Brumm served in various roles at ZELTIQ Aesthetics, Inc., a medical technology company, from December 2009 to August 2012, including as Senior Vice President and Chief Financial Officer, Vice President of Corporate Development and Investor Relations, Senior Managing Director of International Sales and Director of Corporate Development and Strategy. Prior to his service at ZELTIQ Aesthetics, Mr. Brumm served as Director of Finance at Proteolix, Inc. and held investment banking roles at Citigroup Global Markets, Inc. and Morgan Stanley. Mr. Brumm holds a B.A. in business administration from the University of Notre Dame. We believe that Mr. Brumm is qualified to serve on our board of directors based on his experience, qualifications, attributes and skills, including experience in operations management and executive leadership.

Romesh Subramanian, Ph.D. is one of our co-founders and has served as our Chief Scientific Officer since October 2019. Prior to becoming Chief Scientific Officer, Dr. Subramanian served as our President and Chief Executive Officer from November 2018 to October 2019 and as our Chief Scientific Officer from April 2018 to November 2018. Dr. Subramanian also served as one of our directors from November 2018 to October 2019. Prior to joining us, Dr. Subramanian led new modality discovery research as Sr. Director, Discovery Research at Alexion Pharmaceuticals, Inc., a biopharmaceutical company, focusing on nucleic acid, antibody and enzyme replacement therapies from 2014 to October 2017. Previously, Dr. Subramanian co-founded RaNA Therapeutics, Inc., now Translate Bio, Inc., a biotechnology company, where he served as Senior Director from 2011 to 2014. Prior to co-founding RaNA, Dr. Subramanian held positions of increasing responsibility at Pfizer Inc. and Thrasos Therapeutics, Inc., a biotechnology company. Since October 2017, Dr. Subramanian has been an entrepreneur-in-residence at Atlas Venture. Dr. Subramanian also serves on the advisory board of the Harvard Medical School Initiative for RNA Medicine. Dr. Subramanian earned his B.S. in zoology from Loyola University, his M.S. in biology from Duke University, and his Ph.D. from Emory University.

Susanna High has served as our Chief Operating Officer since July 2020. Prior to joining us, she was an independent adviser to biopharmaceutical organizations, with a focus on strategy and operations, from February 2019 until July 2020. Prior to that, Ms. High served as Chief Operating Officer of bluebird bio, Inc., a biopharmaceutical company, from December 2016 to January 2019. Previously, Ms. High served as Senior Vice President, Strategy and Business Integration at Alnylam Pharmaceuticals, Inc. from February 2015 to September 2016, and as Vice President, Business Planning and Program Management from June 2008 to January 2015 and Senior Director, Business Planning and Program Management from February 2007 to June 2008. Prior to joining Alnylam, she supported corporate strategy and business operations at Millennium Pharmaceuticals (now Takeda Oncology). Ms. High holds an undergraduate degree in economics and business management from Bocconi University in Italy and an M.B.A. from the MIT Sloan School of Management.

Oxana Beskrovnaya, Ph.D. has served as our Senior Vice President, Head of Research since January 2020. Prior to joining us, Dr. Beskrovnaya served as head of musculoskeletal and renal research in Sanofi's rare disease and neurological unit from July 2011 to January 2020. Dr. Beskrovnaya is the author of numerous patents, invited reviews, editorials, book chapters and original research articles in major scientific journals. Dr. Beskrovnaya received her Ph.D. in genetics from Moscow Genetics Institute, followed by postdoctoral fellowship training in neuromuscular diseases at the Howard Hughes Medical Institute at the University of Iowa.

Jonathan McNeill, M.D. has served as our Vice President, Business Development since February 2019. Prior to joining us, Dr. McNeill served as Associate Director, Business Development at Editas Medicine, a biotechnology company, from August 2015 to January 2019. Prior to joining Editas, Dr. McNeill served as a Consultant at Boston Consulting Group from March 2014 to August 2015. Dr. McNeill earned his B.A. in public policy and economics from the University of North Carolina and his M.D. from the University of Pennsylvania.

Richard Scalzo has served as our Vice President of Accounting and Administration since July 2020. He previously served as our Corporate Controller from December 2019 to July 2020. Prior to joining us, Mr. Scalzo served as Corporate Controller at several biotechnology companies, including Kaleido Biosciences, Inc. from August 2018 to November 2019, X4 Pharmaceuticals, Inc. from September 2016 to August 2018 and Ocata Therapeutics, Inc. (acquired by Astellas Pharma Inc. in February 2016) from August 2014 to September 2016. Mr. Scalzo started his career with PricewaterhouseCoopers in its health industries practice. Mr. Scalzo is a certified public accountant in the Commonwealth of Massachusetts and holds a B.S. in accounting from Boston College and an M.B.A. from the University of Massachusetts.

Significant employees

The following table sets forth certain information concerning our significant employees, including their ages as of August 31, 2020:

Name	Age	Position
Thomas-Christian Mix, M.D.	53	Senior Vice President, Clinical Development
John Davis, Ph.D.	52	Vice President, Head of Preclinical Development
Debra Feldman	50	Vice President, Head of Regulatory Affairs
Gene Kim	44	Vice President, Finance
John Najim	44	Vice President, Chemistry, Manufacturing and Controls
Mohammed Qatanani, Ph.D.	47	Vice President, Program and Alliance Management
Amy Reilly	47	Vice President, Corporate Communications and Investor Relations
Molly White	60	Vice President, Medical Communications and Advocacy
Daniel Wilson	49	Vice President, Head of Intellectual Property

The following is a biographical summary of the experience of these significant employees.

Thomas-Christian Mix, M.D. has served as our Senior Vice President, Clinical Development since February 2020. Prior to joining us, Dr. Mix served as Vice President of rare genetic disease clinical development at Agios Pharmaceuticals, Inc., from May 2018 to October 2019. Prior to joining Agios, Dr. Mix served as Vice President of Clinical Development at Sarepta Therapeutics, Inc., from June 2017 to May 2018. Prior to Sarepta, Dr. Mix served as Executive Director, Global Medical Sciences at Alexion Pharmaceuticals from January 2015 to May 2017. Prior to Alexion, Dr. Mix served as Executive Medical Director, Global Development at Amgen from 2003 to 2015. Dr. Mix received his B.A. in chemistry from Haverford College, an M.D. from the University of Massachusetts Medical School and an M.S. in Clinical Care Research from Tufts Sackler School of Biomedical Sciences.

John Davis, Ph.D. has served as our Vice President, Head of Preclinical Development since July 2020. Prior to joining us, Dr. Davis was Vice President of Preclinical Development at Wave Life Sciences Ltd. from May 2016 to July 2020. Previously, he served as Director of Investigative Toxicology at Pfizer, Inc. from 2007 to May 2016. Dr. Davis received his B.S. from the University of Wisconsin-Madison and his Ph.D. in molecular toxicology from Purdue University.

Debra Feldman has served as our Vice President, Head of Regulatory Affairs since May 2020. Prior to joining us, Ms. Feldman served as Vice President, Regulatory Affairs at Sage Therapeutics, Inc. from March 2016 to May 2020. Prior to joining Sage, Ms. Feldman served as Senior Director, Regulatory Affairs at Medivector, Inc. from July 2014 to March 2016. Prior to Medivector, Ms. Feldman held various regulatory affairs and regulatory consulting roles at several biotechnology companies including FoldRx Pharmaceuticals, Artisan Pharma, AMAG Pharmaceuticals and EPIX Pharmaceuticals. Ms. Feldman holds a B.A. from the University of Massachusetts, a degree in nutritional science from Simmons College and a Master of Public Health from Boston University.

Gene Kim has served as our Vice President, Finance, since January 2020. Prior to joining us, Mr. Kim served as Vice President, Finance at Kaleido Biosciences, Inc. from May 2018 to January 2020. Prior to joining Kaleido, Mr. Kim served as Executive Director, Financial Planning and Analysis at Versartis, Inc. from November 2013 to May 2018. Prior to Versartis, Mr. Kim held various roles within finance organizations at Pharmacyclics, Onyx

Pharmaceuticals and Gilead Sciences. Mr. Kim began his career in investment banking supporting the global healthcare division at Lehman Brothers. Mr. Kim holds a B.A. in electrical engineering from the University of California at Los Angeles.

John Najim has served as our Vice President, Chemistry, Manufacturing and Controls since August 2019. Prior to joining us, Mr. Najim served as an independent consultant at CMC Ready, LLC, a chemistry, manufacturing and controls consulting firm, from May 2019 to August 2019. Prior to that, Mr. Najim served as Director of Manufacturing, Senior Director of Technical Operations and then Vice President of Manufacturing and Process Development at Proteon Therapeutics, a pharmaceutical company, from May 2009 to May 2019. Mr. Najim previously held senior positions in manufacturing at Dyax and GTC Biotherapeutics, companies involved in biotechnology and pharmaceutical development. Mr. Najim received his B.S. in biochemistry from Merrimack College and an M.B.A. from Bentley University.

Mohammed Qatanani, Ph.D. has served as our Vice President, Program and Alliance Management since May 2020 and previously served as our Vice President, Discovery and Translational Research from February 2018 to May 2020. Prior to joining us, Dr. Qatanani served as Director, Discovery Research at Alexion Pharmaceuticals from July 2015 to January 2018. Prior to Alexion, Dr. Qatanani led drug discovery at Synageva BioPharma, a biopharmaceutical company. Dr. Qatanani began his drug discovery work at the cardiometabolic disease division at Merck Research Laboratories. Dr. Qatanani received his B.S. and M.S. in biology from the American University of Beirut and holds a Ph.D. in molecular and human genetics from Baylor College of Medicine.

Amy Reilly has served as our Vice President, Corporate Communications and Investor Relations since July 2020. Prior to joining us, she was at Kaleido Biosciences, Inc. from November 2017 to May 2020, most recently serving as Vice President, Communications and Investor Relations. Previously, Ms. Reilly served from March 2017 to October 2017 as Senior Director, Internal Communications for Patheon, and then following its acquisition, Thermo Fisher Scientific, Inc., and from October 2015 to March 2017, as Director, Corporate Communications at ImmunoGen, Inc. From September 2011 to June 2015, Ms. Reilly served as Director, Employee Communications and Philanthropy at Cubist Pharmaceuticals, Inc., which was acquired by Merck & Co., Inc. in January 2015. Prior to that, she spent nearly 10 years at Biogen, Inc. serving in various roles, including managing product communications and media relations as well as overseeing the company's foundation. Ms. Reilly received her A.B. in English and American Literature from Bowdoin College.

Molly White has served as our Vice President, Medical Communications and Advocacy since January 2020. Prior to joining us, Ms. White served as Chief Executive Officer at Myotonic, a patient advocacy organization focused on accelerating drug development and improving quality of life for people living with myotonic dystrophy, from January 2012 to December 2019. Prior to Myotonic, Ms. White served in corporate social responsibility leadership roles at Nike, Inc., Gap, Inc. and Apollo Group and as a consultant building corporate social responsibility programs for clients. Ms. White received her B.A. from the University of Montana and M.A. from the University of Iowa.

Daniel Wilson has served as our Vice President, Head of Intellectual Property since June 2020. Prior to joining us, Mr. Wilson served in roles of increasing responsibility at Celgene Corporation, a pharmaceutical company, from August 2012 to April 2020. Prior to Celgene, Mr. Wilson worked in the legal organization at Sunovion Pharmaceuticals after beginning his career at Goodwin Procter LLP and Testa, Hurwitz & Thibeault, LLP. Mr. Wilson received his B.A. in biology at Swarthmore College, M.S. in pharmacology at University of Pennsylvania and J.D. at Boston University School of Law.

Non-employee directors

The following table sets forth certain information concerning our non-employees who serve on our board of directors, including their ages as of August 31, 2020:

Name	Age	Position
Jason Rhodes(3)	51	Director and Chairman of the Board
Edward Hurwitz(2)	56	Director
Dirk Kersten(1)(2)	45	Director
Lawrence Klein, Ph.D.(1)(2)	38	Director
David Lubner(1)(3)	56	Director
Catherine Stehman-Breen, M.D.	57	Director

(1) Member of the audit committee.

(2) Member of the compensation committee.

(3) Member of the nominating and corporate governance committee.

The following is a biographical summary of the experience of our non-employee directors.

Jason Rhodes has served as a director of our company since December 2017 and chairman of our board of directors since November 2018. Mr. Rhodes also served as our founding President and Chief Executive Officer from December 2017 to November 2018. Mr. Rhodes has been a partner at Atlas Venture since 2014. From 2010 to 2014, Mr. Rhodes was employed at Epizyme, Inc., a biotechnology company, where he most recently served as President and Chief Financial Officer. Mr. Rhodes serves as a member of the board of directors of Replimune Group, Inc., Generation Bio Co. and several private companies, and previously served as a director at Bicycle Therapeutics, Inc. from 2016 to 2019. Mr. Rhodes earned a B.A. in history from Yale University and an M.B.A. from the Wharton School of the University of Pennsylvania. We believe Mr. Rhodes is qualified to serve on our board based on his extensive knowledge of our company from his roles as our founding chief executive officer and chairman of our board of directors as well as his extensive leadership experience, his biotechnology company board experience and his experience investing in life science companies.

Edward Hurwitz has served as a director of our company since November 2018. Mr. Hurwitz has served as a Managing Director at MPM Capital since January 2017 and as Managing Director of Precision BioVentures since he founded the firm in 2013. Prior to joining MPM Capital, Mr. Hurwitz was a Director at Alta Partners from 2002 through December 2014. Prior to joining Alta Partners, Mr. Hurwitz served as Senior Vice President and CFO of Affymetrix from 1997 to 2002. Prior to his service at Affymetrix, Mr. Hurwitz was a biotechnology research analyst for Robertson Stephens & Company and Smith Barney Shearson and a lawyer at Cooley Godward LLP. Mr. Hurwitz serves as a member of the board of directors of MacroGenics, Inc. and Applied Genetic Technologies Corporation and several private companies. Mr. Hurwitz earned his J.D. and M.B.A. degrees from the University of California, Berkeley, and his B.A. in molecular biology from Cornell University. We believe Mr. Hurwitz is qualified to serve on our board based on his education and professional background in science, business management and law, his work as a research analyst, senior executive, and lawyer in the biotechnology industry, his experience investing in life science companies, and his experience as a director of public and private biotechnology companies.

Dirk Kersten has served as a director of our company since November 2018. Mr. Kersten has served as a general partner at Forbion since October 2018. Mr. Kersten is a physicist by training and previously was a managing director at INKEF Capital from May 2014 to August 2018, where he was responsible for all healthcare

investment activities. Prior to joining INKEF Capital, Mr. Kersten was a partner at Gilde Healthcare Partners from 2006 to 2014, including a period of three years when he led its U.S. operations. Mr. Kersten served as a director of a number of private life science companies, including Ascendis Pharma, Profibrix, Lanthio Pharma, Nightbalance, Audion Therapeutics, GTX and Vicentra. Mr. Kersten received his M.S. in physics from the University of Groningen in the Netherlands. We believe Mr. Kersten is qualified to serve on our board based on his scientific background, his extensive experience investing in life science companies and his experience as a director of biotechnology companies.

Lawrence Klein, Ph.D. has served as a director of our company since September 2019. Dr. Klein has served in various positions at CRISPR Therapeutics AG, a biotechnology company, including Chief Business Officer since January 2019 and Chief Business Officer and Chief Operating Officer since January 2020, Senior Vice President, Business Development and Strategy from November 2017 to December 2018 and as Vice President, Strategy from February 2016 to November 2017. Before joining CRISPR, Dr. Klein was an Associate Partner at McKinsey & Company, a global management consulting firm, from October 2014 to February 2016. Dr. Klein received his B.S. in biochemistry and physics from the University of Wisconsin-Madison and his Ph.D. in biophysics from Stanford University. We believe Dr. Klein is qualified to serve on our board based on his scientific background and his business development and operational experience as a senior executive in the biotechnology industry.

David Lubner has served as a director of our company since March 2020. Mr. Lubner served as Executive Vice President and Chief Financial Officer of Ra Pharmaceuticals, Inc., a biotechnology company acquired by UCB S.A. in April 2020, from January 2016 until June 2020. Before joining Ra Pharmaceuticals, Mr. Lubner served as Chief Financial Officer of Tetraphase Pharmaceuticals, Inc., a biotechnology company, from its inception in 2006 to 2016, as Chief Financial Officer of PharMetrics Inc., a patient-based pharmacy and medical claims data informatics company, from 1999 until it was acquired by IMS Health in 2005 and as Vice President and Chief Financial Officer of ProScript, Inc. from 1996 to 1999. Mr. Lubner serves as a member of the board of directors of Therapeutics Acquisition Corp. and several other private companies, and Mr. Lubner previously served on the board of directors of Nightstar Therapeutics plc from 2017 until it was acquired by Biogen Inc. in June 2019. Mr. Lubner is a Certified Public Accountant. He received his B.S. in business administration from Northeastern University and an M.S. in taxation from Bentley University. We believe Mr. Lubner is qualified to serve on our board based on his financial and leadership experience as a senior executive in the biotechnology industry and his experience as a director of a public biotechnology company, including serving as chair of the audit committee.

Catherine Stehman-Breen, M.D. has served as a director of our company since June 2019. Dr. Stehman-Breen has served as Chief Development Officer of Obsidian Therapeutics, Inc., a biotechnology company, since July 2019. Previously, she served as an entrepreneur-in-residence at Atlas Venture, serving as Chief Medical Officer of our company from March 2018 to July 2019 and as Chief Medical Officer of Disarm Therapeutics, Inc., a biotechnology company, from April 2018 to July 2019. Dr. Stehman-Breen also served as Chief Medical Officer of Sarepta Therapeutics, Inc. from March 2017 to December 2017. Prior to that, Dr. Stehman-Breen served as Vice President, Clinical Development and Regulatory Affairs at Regeneron Pharmaceuticals, Inc., a biotechnology company, initially as head, pain therapeutic area, and subsequently as head, clinical project management and operations from January 2015 to March 2017. Dr. Stehman-Breen serves as a member of the board of directors of Generation Bio Co. and Tenaya Therapeutics, Inc. Dr. Stehman-Breen earned a B.A. in biology and psychology from Colby College, an M.Sc. degree in epidemiology from the University of Washington, where she also conducted her residency and fellowship training, and an M.D. from the University of Chicago. We believe Dr. Stehman-Breen is qualified to serve on our board based on her extensive leadership experience, including her experience with clinical development and regulatory matters and in the life science industry.

Our board of directors

Our board of directors currently consists of seven members. Our directors hold office until their successors have been elected and qualified or until the earlier of their death, resignation or removal. Our restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering provide that the authorized number of directors may be changed only by resolution of our board of directors. Our restated certificate of incorporation and amended and restated bylaws will also provide that our directors may be removed only for cause by the affirmative vote of the holders of 75% of our shares of capital stock present in person or by proxy and entitled to vote, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

In accordance with the terms of our restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering, our board of directors will be divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms. As a result, only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the class I directors will be Catherine Stehman-Breen, M.D. and Lawrence Klein, Ph.D., and their term will expire at the first annual meeting of stockholders to be held after the closing of this offering;
- the class II directors will be Edward Hurwitz and Dirk Kersten, and their term will expire at the second annual meeting of stockholders to be held after the closing of this offering; and
- the class III directors will be Jason Rhodes, David Lubner and Joshua Brumm, and their term will expire at the third annual meeting of stockholders to be held after the closing of this offering.

Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

The classification of our board of directors may have the effect of delaying or preventing changes in our control or management. See “Description of capital stock—Delaware anti-takeover law and certain charter and bylaw provisions.”

Director independence

The rules of the Nasdaq Stock Market, or Nasdaq, require a majority of a listed company’s board of directors to be comprised of independent directors within one year of listing. In addition, Nasdaq rules require that, subject to specified exceptions, each member of a listed company’s audit, compensation and nominating and corporate governance committees be independent. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under applicable Nasdaq rules, a director will only qualify as an “independent director” if, in the opinion of the listed company’s board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for

each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In August 2020, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of our directors, with the exception of Joshua Brumm and Catherine Stehman-Breen, is an "independent director" as defined under applicable Nasdaq rules, including, in the case of all the members of our audit committee, the independence criteria set forth in Rule 10A-3 under the Exchange Act, and in the case of all the members of our compensation committee, the independence criteria set forth in Rule 10C-1 under the Exchange Act. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director. Joshua Brumm is not an independent director under these rules because he is an executive officer of our company. Catherine Stehman-Breen is not an independent director under these rules because she has received more than $120,000 in consulting fees from us during a twelve-month period within the past three years.

There are no family relationships among any of our directors or executive officers.

Board leadership structure

Currently, the roles of chair of our board of directors and Chief Executive Officer are separated. Jason Rhodes is the chairman of our board of directors and Joshua Brumm is our Chief Executive Officer. We believe that separating these positions allows our Chief Executive Officer to focus on our day-to-day business, while allowing our chairman of the board to lead the board of directors in providing advice to, and independent oversight of, our management. While our amended and restated bylaws and corporate governance guidelines that will become effective upon the completion of this offering will not require that chair of our board of directors and Chief Executive Officer positions be separate, our board of directors believes that having separate positions is the appropriate leadership structure for us at this time and demonstrates our commitment to good corporate governance.

Board's role in risk oversight

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure, and our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements.

Committees of our board of directors

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee, each of which operates under a charter approved by our board of directors. We have posted current copies of each committee's charter on our website, www.dyne-tx.com.

Audit committee

The members of our audit committee are Dirk Kersten, Lawrence Klein, Ph.D. and David Lubner, and David Lubner is the chair of the audit committee. Our board of directors has determined that David Lubner is an "audit committee financial expert" as defined by applicable SEC rules and that each of the members of our audit committee possesses the financial sophistication required for audit committee members under Nasdaq rules. We believe that the composition of our audit committee meets the requirements for independence under current Nasdaq and SEC rules and regulations. Our audit committee's responsibilities include:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from such firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our internal audit function;
- overseeing our risk assessment and risk management policies;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting-related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management;
- reviewing and approving or ratifying any related person transactions; and
- preparing the audit committee report required by SEC rules.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Compensation committee

The members of our compensation committee are Edward Hurwitz, Dirk Kersten and Lawrence Klein, Ph.D., and Dirk Kersten is the chair of the compensation committee. Our compensation committee's responsibilities include:

- reviewing and approving, or making recommendations to our board of directors with respect to, the compensation of our chief executive officer and our other executive officers;

- overseeing an evaluation of our senior executives;
- overseeing and administering our cash and equity incentive plans;
- reviewing and making recommendations to our board of directors with respect to director compensation;
- reviewing and discussing annually with management our "Compensation Discussion and Analysis" disclosure if and to the extent required by SEC rules; and
- preparing the compensation committee report if and to the extent then required by SEC rules.

We believe that the composition of our compensation committee meets the requirements for independence under current Nasdaq and SEC rules and regulations.

Nominating and corporate governance committee

The members of our nominating and corporate governance committee are David Lubner and Jason Rhodes, and Jason Rhodes is the chair of the nominating and corporate governance committee. Our nominating and corporate governance committee's responsibilities include:

- recommending to our board of directors the persons to be nominated for election as directors and to each of our board's committees;
- reviewing and making recommendations to our board with respect to our board leadership structure;
- reviewing and making recommendations to our board with respect to management succession planning;
- developing and recommending to our board of directors corporate governance principles; and
- overseeing a periodic evaluation of our board of directors.

We believe that the composition of our nominating and corporate governance committee meets the requirements for independence under current Nasdaq and SEC rules and regulations.

Compensation committee interlocks and insider participation

No member of our compensation committee is or has been a current or former officer or employee of our company. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any entity that has one or more of its executive officers serving on our board of directors or our compensation committee.

Code of business conduct and ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers, employees and designated agents, including our principal executive officer, principal financial officer, principal accounting officer, or persons performing similar functions. A current copy of the code is posted on the investor relations section of our website, which is located at http://www.dyne-tx.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website.

Executive compensation

The following discussion relates to the compensation of our President and Chief Executive Officer, Joshua Brumm, our Chief Scientific Officer, Romesh Subramanian, and our Vice President, Business Development, Jonathan McNeill, for the fiscal year ended December 31, 2019. Mr. Brumm, Dr. Subramanian and Dr. McNeill are collectively referred to in this prospectus as our named executive officers.

In preparing to become a public company, we have begun a thorough review of all elements of our executive compensation program, including the function and design of our equity incentive programs. We have begun, and expect to continue in the coming months, to evaluate the need for revisions to our executive compensation program to ensure that our program is competitive with the companies with which we compete for executive talent and is appropriate for a public company.

Summary compensation table

The following table sets forth information regarding compensation awarded to, earned by or paid to each of our named executive officers during our fiscal year ended December 31, 2019.

Name and principal position	Year	Salary ($)	Bonus ($)	Option awards ($)(1)	Non-equity incentive plan compensation ($)(2)	All other compensation ($)	Total ($)
Joshua Brumm *President and Chief Executive Officer*	2019	97,212(3)	180,000(3)	—	—	102(4)	277,314
Romesh Subramanian, Ph.D.(5) *Chief Scientific Officer*	2019	350,002	—	—	61,250	408(4)	411,660
Jonathan McNeill, M.D. *Vice President, Business Development*	2019	223,333(6)	—	48,055	58,560	408(4)	330,356

(1) The amounts reported represent the aggregate grant date fair value of stock options awarded in 2019, calculated in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value are set forth in Note 10 to our audited financial statements appearing elsewhere in this prospectus.

(2) The amounts reported represent performance-based cash bonuses earned by our named executive officers in the year ended December 31, 2019, which amounts were paid in February 2020. See "—Narrative Disclosure to 2019 Summary Compensation Table—Annual bonus" below for a general description of the criteria that our board of directors used to determine the performance-based cash bonuses.

(3) Mr. Brumm commenced employment with us in October 2019. His annual base salary for 2019 was $450,000, and his bonus for 2019 was fixed at $180,000 as of the commencement of his employment. Mr. Brumm is also a member of our board of directors but does not receive any additional compensation in his capacity as a director.

(4) The amount reported represents long-term disability insurance premiums in excess of statutory limits.

(5) Dr. Subramanian currently serves as our Chief Scientific Officer and ceased to be our Chief Executive Officer upon Mr. Brumm's assuming the role of our Chief Executive Officer in October 2019.

(6) Dr. McNeill commenced employment with us in February 2019. His annual base salary for 2019 was $260,000.

Narrative disclosure to 2019 summary compensation table

Base salary. During 2019, the annualized base salaries for Mr. Brumm, Dr. Subramanian and Dr. McNeill were $450,000, $350,000 and $260,000, respectively. We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our named executive officers. None of our named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary. Effective upon the effectiveness of the registration statement of which this prospectus forms a part, Mr. Brumm's, Dr. Subramanian's and Dr. McNeill's annual base salaries were increased to $562,000, $425,000 and $325,000, respectively.

Annual bonus. Our board of directors may, in its discretion, award bonuses to our named executive officers from time to time. We typically establish annual bonus targets based on specified corporate goals and individual performance and conduct annual performance reviews to assess individual performance. Each of our named executive officers was eligible to receive an annual bonus for 2019, with the target amount of such bonus for each named executive officer set forth in his employment or letter agreement with us. For 2019, the bonus amount for Mr. Brumm was set at $180,000 pursuant to the terms of his offer letter, and the target bonus amount, expressed as a percentage of base salary, for each of Dr. Subramanian and Dr. McNeill were as follows: 35% and 25%, respectively. Effective upon the effectiveness of the registration statement of which this prospectus forms a part, Mr. Brumm's, Dr. Subramanian's and Dr. McNeill's target bonus amounts for the remainder of 2020, expressed as a percentage of base salary, will be 55%, 40% and 30%, respectively.

With respect to 2019, our board of directors awarded bonuses of $180,000, $61,250 and $58,560 to Mr. Brumm, Dr. Subramanian and Dr. McNeill, respectively.

Equity incentives. Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executive officers with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executive officers and our stockholders. We believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our executive officers to remain in our employment during the vesting period and that equity grants with performance-based vesting help to align incentives for our executive officers with our key performance objectives. We have used stock options to compensate our executive officers in the form of initial grants in connection with the commencement of employment. In addition, our board of directors periodically reviews the equity incentive compensation of our executive officers, including our named executive officers, and from time to time may grant equity incentive awards to them in the form of stock options, restricted stock or restricted stock units.

Prior to this offering, our executive officers were eligible to participate in our 2018 Stock Incentive Plan, as amended, or the 2018 Plan. All stock options were granted pursuant to the 2018 Plan. We did not grant any restricted stock awards during 2019. Following this offering, our employees and executive officers will be eligible to receive stock options and other stock-based awards pursuant to our 2020 Stock Incentive Plan, or the 2020 Plan.

The options, restricted stock and restricted stock units that we have granted to our executive officers that are subject to time-based vesting generally vest over four years following the vesting commencement date. Upon certain terminations of employment in connection with a change of control, vesting is fully accelerated. Prior to the exercise of a stock option, the holder has no rights as a stockholder with respect to the shares subject to such option, including no voting rights and no right to receive dividends or dividend equivalents.

We have historically awarded stock options with exercise prices that are equal to the fair market value of our common stock on the date of grant as determined by our board of directors.

We granted an option to purchase 557,749 shares of our common stock to Mr. Brumm in January 2020, under the 2018 Plan, in connection with him joining the company as President and Chief Executive Officer in October 2019. This option is exercisable at a price per share of $1.03 and vests as to 25% of the shares underlying the option on October 12, 2020, and then an additional 6.25% of the shares underlying the option vest in quarterly installments thereafter, subject to continuous service. We also granted an option to purchase 98,426 shares of our common stock to Dr. McNeill in February 2019, under the 2018 Plan, in connection with him joining our company as Vice President, Business Development. This option is exercisable at a price per share of $0.73 and vested as to 25% of the shares underlying the option on February 1, 2020, and then an additional 6.25% of the shares underlying the option vests in quarterly installments thereafter, subject to continuous service.

In July 2020, we granted Mr. Brumm, Dr. Subramanian and Dr. McNeill options to purchase 687,181, 278,308 and 98,468 shares of our common stock, respectively, under the 2018 Plan. Each of these options is exercisable at a price per share of $5.54 and vests as to 6.25% of the shares underlying such option in equal quarterly installments until all shares are vested on the fourth anniversary of the date of grant, subject to continuous service. At the same time, we also granted performance-based options to these officers to purchase 153,931, 88,390 and 21,666 shares of our common stock, respectively, under the 2018 Plan. Each of these options is exercisable at a price per share of $5.54 and will vest in full only upon the effective date of an IND application filed by us, if we submit such application and it becomes effective on or prior to December 31, 2023.

In August 2020, subject to the pricing of this offering, we granted Mr. Brumm, Dr. Subramanian and Dr. McNeill options to purchase 320,781, 128,185 and 52,787 shares of our common stock, respectively, under the 2020 Plan. Each of these options will be exercisable at a price per share equal to the initial offering price and vests in equal monthly installments until all shares are vested on the fourth anniversary of the date of grant, subject to continuous service. At the same time, subject to the pricing of this offering, we also granted restricted stock units to these officers for 107,027, 41,309 and 17,012 shares of our common stock, respectively, under the 2020 Plan. Each of these restricted stock units vests in equal annual installments until all shares are vested on the fourth anniversary of the date of grant, subject to continuous service. We also granted to Mr. Brumm, under the 2020 Plan and subject to the pricing of this offering, performance-based options to purchase 78,386 shares of our common stock and performance-based restricted stock units for 24,118 shares of our common stock. The performance-based option will be exercisable at a price per share equal to the initial offering price and each of the performance-based option and performance-based restricted stock units will vest in full only upon the effective date of an IND application filed by us, if we submit such application and it becomes effective on or prior to June 30, 2022.

Outstanding equity awards at 2019 fiscal year-end

The following table sets forth information concerning outstanding equity awards held by our named executive officers as of December 31, 2019.

	Option Awards				Stock Awards	
Name	**Number of securities underlying unexercised options (#) exercisable**	**Number of securities underlying unexercised options (#) unexercisable**	**Option exercise price ($)**	**Option expiration date**	**Number of shares or units of stock that have not vested (#)**	**Market value of shares or units of stock that have not vested ($)(1)**
Joshua Brumm *President & Chief Executive Officer*	—	—	—	—	—	—
Romesh Subramanian, Ph.D. *Chief Scientific Officer*	—	—	—	—	295,279(2)	$5,610,301
Jonathan McNeill, M.D. *Vice President, Business Development*	—	98,426(3)	0.73	2/25/2029	—	—

(1) The market value of our common stock is based on the initial public offering price of $19.00 per share.

(2) Represents the unvested portion of a total original grant of 524,940 shares of our common stock subject to a restricted stock agreement. The restricted shares vested as to 25% on January 1, 2019, and are schedule to vest thereafter in 12 equal quarterly installments through January 1, 2022, subject to continuous service.

(3) The shares underlying this option vested as to 25% of the shares on February 1, 2020, and are scheduled to vest in 12 equal quarterly installments thereafter through February 1, 2023, subject to continuous service.

Employment arrangements with our named executive officers

Offer letters

Joshua Brumm

In September 2019, in connection with our appointment of Mr. Brumm as our President and Chief Executive Officer, we entered into an offer letter with Mr. Brumm. The offer letter established Mr. Brumm's title, his base salary, his eligibility for an annual bonus, and his eligibility for benefits made available to employees generally and also provided for certain benefits upon termination of his employment under specified conditions. Mr. Brumm's employment is at will. Pursuant to the offer letter, we granted Mr. Brumm an option to purchase 557,749 shares of our common stock, which is subject to service-based vesting, and agreed to grant to him additional stock options to maintain his target ownership percentage in our company until such time as we had sold equity having an aggregate purchase price of $50,000,000. Pursuant to the offer letter, Mr. Brumm's base salary was initially set at $450,000, his annual bonus for 2019 was fixed at $180,000 and his target bonus percentage for 2020 is set at 40%.

Romesh Subramanian

In March 2018, in connection with our appointment of Dr. Subramanian as our Chief Scientific Officer, we entered into an offer letter with Dr. Subramanian. The offer letter established Dr. Subramanian's title, his base salary, his eligibility for an annual bonus and his eligibility for benefits made available to employees generally and also provided for certain benefits upon termination of his employment under specified conditions. Dr. Subramanian's employment is at will. Pursuant to the offer letter, we granted Dr. Subramanian an award of 524,940 shares of restricted common stock, which is subject to service-based vesting, and agreed to grant to him stock options or restricted stock awards to maintain a specified target ownership percentage in our company until such time as we had sold equity having an aggregate purchase price of $30,000,000.

In November 2018, in connection with our appointment of Dr. Subramanian as our President and Chief Executive Officer, we entered into an employment side letter with Dr. Subramanian to modify his title, base salary, annual bonus and target ownership percentage in the company. Pursuant to the side letter, Dr. Subramanian's base salary was increased to $350,000, his target annual bonus percentage was increased to 35% and his target ownership percentage in our company was increased. Pursuant to the employment side letter, for so long as Dr. Subramanian served as our Chief Executive Officer, he ceased to serve as our Chief Scientific Officer and would resume his position as our Chief Scientific Officer as determined by our Board with no reduction to his then-current base salary or annual incentive bonus target.

Jonathan McNeill

In December 2018, in connection with our appointment of Dr. McNeill as our Vice President, Business Development, we entered into an offer letter with Dr. McNeill. The offer letter established Dr. McNeill's title, his base salary, his eligibility for an annual bonus, and his eligibility for benefits made available to employees generally. Dr. McNeill's employment is at will. Pursuant to the offer letter, we granted Dr. McNeill an option to purchase 98,426 shares of our common stock, which is subject to service-based vesting, and agreed to grant to him additional stock options to maintain his target ownership percentage in our company until such time as we

had sold equity having an aggregate purchase price of $30,000,000. Pursuant to the offer letter, Dr. McNeill's base salary was set at $240,000 and his target bonus percentage for 2020 is set at 25%.

Severance upon termination of employment; change in control

In August 2020, we adopted our Executive Severance and Change in Control Benefits Plan, or the severance benefits plan, for certain of our employees, including each of our executive officers. The severance benefits provided in the severance benefits plan supersede the separation benefits, if any, provided under the terms of each covered employee's offer letter, except, in the case of Dr. Subramanian, as described below. Under the terms of the severance benefits plan, if the employment of any of our officers is terminated by us without cause or by the officer for good reason prior to or more than 12 months following a change in control, each as defined in the plan, and subject to the officer's execution of a general release of potential claims against us, we have agreed to continue to pay the officer's then-current base salary for a period of 12 months, in the case of our chief executive officer, and nine months, in the case of our other senior officers, and to pay premiums for continuation of health coverage under COBRA for up to 12 months, in the case of our chief executive officer, and up to nine months, in the case of our other senior officers.

Alternatively, if a covered employee's employment is terminated by us without cause or by the employee for good reason within one year following a change in control, and subject to the employee's execution of a general release of potential claims against us, we have agreed, in the case of our chief executive officer, to pay a lump sum payment in an amount equal to 18 months of his then-current base salary, in the case of our other senior officers, to pay a lump sum payment in an amount equal to 12 months of his or her then-current base salary and, in the case of our other covered employees, to pay a lump sum payment in an amount equal to nine months of his or her then-current base salary; to pay premiums for continuation of health coverage under COBRA for up to 18 months, in the case of our chief executive officer, up to 12 months, in the case of our other senior officers, and up to nine months in the case of our other covered employees; to pay a lump sum payment in an amount equal to 150%, in the case of our chief executive officer, and 100%, in the case of our other covered employees, of the employee's target annual bonus for the year in which his or her employment is terminated; and to accelerate the vesting of any outstanding equity grants in full.

In addition, pursuant to his existing offer letter, if Dr. Subramanian's employment is terminated by us without cause or by Dr. Subramanian for good reason, each as defined in his offer letter, prior to or more than 12 months following a change in control, we have agreed to accelerate the vesting of any of his outstanding equity grants such that 25% of the then unvested portion of such equity grants will be fully vested.

Employee invention and non-disclosure agreements and non-competition and non-solicitation agreements

We have also entered into employee invention and non-disclosure agreements and non-competition and non-solicitation agreements with each of our named executive officers. Under the invention and non-disclosure agreements, each named executive officer has agreed to protect our confidential and proprietary information and to assign to us related intellectual property developed during the course of his employment. Under the non-competition and non-solicitation agreements, each named executive officer has agreed not to compete with us during his employment and for a period of one year after the termination of his employment, and not to solicit our employees during his employment and for a period of one year after the termination of his employment.

Employee benefit and equity compensation plans

In this section we describe our 2018 Plan, our 2020 Plan, and our 2020 Employee Stock Purchase Plan, or the 2020 ESPP. Prior to this offering, we granted awards to eligible participants under the 2018 Plan. Following this

offering, we expect to grant awards to eligible participants from time to time only under the 2020 Plan. These summaries of the equity incentive plans are qualified in their entirety by reference to the actual text of the plans, which are filed as exhibits to the registration statement of which this prospectus is a part.

2018 Stock incentive plan

The 2018 Plan was initially approved by our board of directors in October 2018 and by our stockholders in November 2018, and was subsequently amended in January 2020, March 2020, July 2020 and August 2020, in each case solely to increase the total number of shares reserved for issuance under the 2018 Plan. The 2018 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, awards of restricted stock, restricted stock units and other stock-based awards. Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2018 Plan; however, incentive stock options may only be granted to our employees. The type of award granted under the 2018 Plan and the terms of such award are set forth in the applicable award agreement. Pursuant to the terms of the 2018 Plan, our board of directors (or a committee delegated by our board of directors) administers the plan and, subject to any limitations in the plan, selects the recipients of awards and determines:

- the number of shares of our common stock covered by options and the dates upon which the options become exercisable;
- the type of options to be granted;
- the duration of options, which may not be in excess of ten years;
- the exercise price of options, which must be at least equal to the fair market value of our common stock on the date of grant; and
- the number of shares of our common stock subject to, and the terms and conditions of, any stock appreciation rights, awards of restricted stock, restricted stock units or other stock-based awards, including conditions for repurchase or cancellation, measurement price, issue price and repurchase price, if any (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years) and any performance conditions.

The maximum number of shares of common stock authorized for issuance under the 2018 Plan is 8,267,252 shares. Our board of directors may amend, suspend or terminate the 2018 Plan at any time, except that stockholder approval may be required to comply with applicable law.

Effect of certain changes in capitalization

In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, we are required by the 2018 Plan to make equitable adjustments (or make substitute awards, if applicable), in the manner determined by our board of directors, to:

- the number and class of securities available under the 2018 Plan;
- the number and class of securities and exercise price per share of each outstanding option;
- the share and per-share provisions and measurement price of each outstanding stock appreciation right;

- the number of shares and the repurchase price per share subject to each outstanding award of restricted stock; and
- the share and per-share related provisions and purchase price, if any, of each outstanding restricted stock unit award and each other stock-based award.

Effect of certain corporate transactions

Upon the occurrence of a merger or other reorganization event (as defined in the 2018 Plan), our board of directors may, on such terms as our board of directors determines (except to the extent specifically provided otherwise in an applicable award agreement or other agreement between the participant and us), take any one or more of the following actions pursuant to the 2018 Plan as to all or any (or any portion of) outstanding awards, other than awards of restricted stock:

- provide that outstanding awards will be assumed, or substantially equivalent awards will be substituted, by the acquiring or succeeding corporation (or an affiliate of the acquiring or succeeding corporation);
- upon written notice to a participant, provide that all of the participant's unexercised and/or unvested awards will terminate immediately prior to the consummation of such transaction unless exercised, to the extent exercisable, by the participant within a specified period following the date of such notice;
- provide that outstanding awards will become exercisable, realizable or deliverable, or restrictions applicable to an award will lapse, in whole or in part, prior to or upon the reorganization event;
- in the event of a reorganization event pursuant to which holders of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award;
- provide that, in connection with our liquidation or dissolution, awards convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings); or
- any combination of the foregoing.

In taking any of the foregoing actions, our board of directors is not obligated by the 2018 Plan to treat all awards, all awards held by a participant, or all awards of the same type, identically.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than our liquidation or dissolution, our repurchase and other rights with respect to outstanding awards of restricted stock will continue for the benefit of the succeeding company and will, unless our board of directors determines otherwise, apply to the cash, securities or other property which our common stock was converted into or exchanged for pursuant to the reorganization event in the same manner and to the same extent as they applied to the common stock subject to the restricted stock award. However, our board of directors may provide for the termination or deemed satisfaction of such repurchase or other rights under the restricted stock award agreement or any other agreement between a

participant and us, either initially or by amendment, or provide for forfeiture of such restricted stock if issued at no cost. Upon the occurrence of a reorganization event involving our liquidation or dissolution, except to the extent specifically provided to the contrary in the restricted stock award agreement or any other agreement between the participant and us, all restrictions and conditions on all outstanding restricted stock awards will automatically be deemed terminated or satisfied.

Our board of directors may at any time provide that any award under the 2018 Plan shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

As of August 31, 2020, there were options to purchase an aggregate of 5,201,900 shares of common stock outstanding under the 2018 Plan at a weighted-average exercise price of $4.03 per share, 453,579 shares of unvested restricted common stock were outstanding under the 2018 Plan and 1,928,394 shares of common stock were available for grant under future awards under the 2018 Plan. No further awards will be made under the 2018 Plan; however, awards outstanding under the 2018 Plan will continue to be governed by their existing terms.

2020 Stock incentive plan

In August 2020 our board of directors adopted and our stockholders approved the 2020 Plan, which became effective immediately prior to the effectiveness of the registration statement for this offering. The 2020 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. The number of shares of our common stock reserved for issuance under the 2020 Plan is the sum of (1) 2,955,746; plus (2) the number of shares (up to a maximum of 7,687,901 shares) as is equal to the sum of (x) the number of shares of our common stock reserved for issuance under the 2018 Plan that remained available for grant under the 2018 Plan immediately prior to the effectiveness of the registration statement for this offering and (y) the number of shares of our common stock subject to outstanding awards granted under the 2018 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right; plus (3) an annual increase, to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2021 and continuing until, and including, the fiscal year ending December 31, 2030, equal to the lesser of (i) 5% of the number of shares of our common stock outstanding on such date, and (ii) an amount determined by our board of directors. Up to 15,074,316 of the shares of common stock available for issuance under the 2020 Plan may be issued as incentive stock options.

Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2020 Plan; however, incentive stock options may only be granted to our employees. We granted stock options to purchase an aggregate of 1,151,454 shares of common stock, at an exercise price per share equal to the initial public offering price, and restricted stock units for an aggregate of 375,137 shares of common stock to certain of our employees, in connection with this offering.

Pursuant to the terms of the 2020 Plan, our board of directors (or a committee delegated by our board of directors) administers the 2020 Plan and, subject to any limitations set forth in the 2020 Plan, selects the recipients of awards and determines:

- the number of shares of our common stock covered by options and the dates upon which the options become exercisable;
- the type of options to be granted;

- the exercise price of options, which price must be at least equal to the fair market value of our common stock on the date of grant;
- the duration of options, which may not be in excess of ten years;
- the methods of payment of the exercise price of options; and
- the number of shares of our common stock subject to and the terms and conditions of any stock appreciation rights, awards of restricted stock, restricted stock units or other stock-based awards, including conditions for repurchase, measurement price, issue price and repurchase price, if any (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years) and any performance conditions.

If our board of directors delegates authority to one or more of our officers to grant awards under the 2020 Plan, the officer will have the power to make awards to all of our employees, except officers and executive officers (as such terms are defined in the 2020 Plan). Our board of directors will fix the terms of the awards to be granted by any such officer, the maximum number of shares subject to awards that any such officer may grant, and the time period in which such awards may be granted.

The 2020 Plan contains limits on the compensation that may be paid to our non-employee directors. The maximum amount of cash and value (calculated based on grant date fair value for financial reporting purposes) of awards granted under the plan in any calendar year to any individual non-employee director in his or her capacity as a non-employee director may not exceed $1,000,000, or in the case of a new director during his or her first year of service, $1,000,000; provided, however, that fees paid by us on behalf of any non-employee director in connection with regulatory compliance and any amounts paid to a non-employee director as reimbursement of an expense shall not count against the foregoing limit. Our board of directors may make additional exceptions to this limit for individual non-employee directors in extraordinary circumstances, as the board of directors may determine in its discretion, provided that the non-employee director receiving such additional compensation may not participate in the decision to award such compensation. For the avoidance of doubt, the maximum amount set forth above will not apply to cash or awards granted under the 2020 Plan to a non-employee director in his or her capacity as a consultant or advisor to us.

Effect of certain changes in capitalization

In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, we are required by the 2020 Plan to make equitable adjustments (or make substitute awards, if applicable), in the manner determined by our board of directors, to:

- the number and class of securities available under the 2020 Plan;
- the share counting rules of the 2020 Plan;
- the number and class of securities and exercise price per share of each outstanding option;
- the share and per-share provisions and measurement price of each outstanding stock appreciation right;
- the number of shares and the repurchase price per share subject to each outstanding award of restricted stock; and
- the share and per-share related provisions and purchase price, if any, of each outstanding restricted stock unit award and each other stock-based award.

Effect of certain corporate transactions

Upon the occurrence of a merger or other reorganization event (as defined in the 2020 Plan), our board of directors may, on such terms as our board of directors determines (except to the extent specifically provided otherwise in an applicable award agreement or other agreement between the participant and us), take any one or more of the following actions pursuant to the 2020 Plan as to all or any (or any portion of) outstanding awards, other than awards of restricted stock:

- provide that outstanding awards will be assumed, or substantially equivalent awards will be substituted, by the acquiring or succeeding corporation (or an affiliate of the acquiring or succeeding corporation);
- upon written notice to a participant, provide that all of the participant's unvested awards will be forfeited immediately prior to the consummation of the reorganization event and/or vested but unexercised awards will terminate immediately prior to the consummation of such transaction unless exercised, to the extent exercisable, by the participant within a specified period following the date of such notice;
- provide that outstanding awards will become exercisable, realizable or deliverable, or restrictions applicable to an award will lapse, in whole or in part, prior to or upon the reorganization event;
- in the event of a reorganization event pursuant to which holders of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award;
- provide that, in connection with our liquidation or dissolution, awards convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings); or
- any combination of the foregoing.

In taking any of the foregoing actions, our board of directors is not obligated by the 2020 Plan to treat all awards, all awards held by a participant, or all awards of the same type, identically.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than our liquidation or dissolution, our repurchase and other rights with respect to each outstanding award of restricted stock will continue for the benefit of the acquiring or succeeding company (or any affiliate of the acquiring or succeeding corporation) and will, unless our board of directors determines otherwise, apply to the cash, securities, or other property which our common stock is converted into or exchanged for pursuant to the reorganization event in the same manner and to the same extent as they applied to the common stock subject to the restricted stock award. However, our board of directors may provide for the termination or deemed satisfaction of such repurchase or other rights under the restricted stock award agreement or in any other agreement between a participant and us, either initially or by amendment. Upon the occurrence of a reorganization event involving our liquidation or dissolution, except to the extent specifically provided to the contrary in the restricted stock award agreement or any other agreement between the participant and us, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied.

Our board of directors may, at any time, provide that any award under the 2020 Plan will become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

Except with respect to certain actions requiring stockholder approval under the Code, or Nasdaq Stock Market rules, our board of directors may amend, modify or terminate any outstanding award under the 2020 Plan, including but not limited to, substituting for the award another award of the same or a different type, changing the date of exercise or realization, and converting an incentive stock option to a nonstatutory stock option, subject to certain participant consent requirements. However, unless our stockholders approve such action, the 2020 Plan provides that we may not (except as otherwise permitted in connection with a change in capitalization or reorganization event):

- amend any outstanding stock option or stock appreciation right granted under the 2020 Plan to provide an exercise or measurement price per share that is lower than the then-current exercise or measurement price per share of such outstanding award;
- cancel any outstanding stock option or stock appreciation right (whether or not granted under the 2020 Plan) and grant a new award under the 2020 Plan in substitution for the cancelled award (other than substitute awards permitted in connection with a merger or consolidation of an entity with us or our acquisition of property or stock of another entity) covering the same or a different number of shares of our common stock and having an exercise or measurement price per share lower than the then-current exercise or measurement price per share of the cancelled award;
- cancel in exchange for a cash payment any outstanding option or stock appreciation right with an exercise or measurement price per share above the then-current fair market value of our common stock (valued in the manner determined by (or in the manner approved by) our board of directors); or
- take any other action that constitutes a “repricing” within the meaning of Nasdaq Stock Market rules or rules of any other exchange or marketplace on which our common stock is listed or traded.

No award may be granted under the 2020 Plan on or after the date that is ten years from the effectiveness of the 2020 Plan. Our board of directors may amend, suspend or terminate the 2020 Plan at any time, except that stockholder approval may be required to comply with applicable law or stock market requirements.

2020 Employee stock purchase plan

In August 2020 our board of directors adopted and our stockholders approved the 2020 ESPP, which became effective immediately prior to the effectiveness of the registration statement for this offering. The 2020 ESPP is administered by our board of directors or by a committee appointed by our board of directors. The 2020 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 488,414 shares of our common stock. The number of shares of our common stock reserved for issuance under the 2020 ESPP will automatically increase on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including the fiscal year commencing on, January 1, 2030, in an amount equal to the lowest of (i) 1,953,656 shares of our common stock, (ii) 1% of the number of shares of our common stock outstanding on such date, and (iii) an amount determined by our board of directors.

All of our employees and employees of any designated subsidiary, as defined in the 2020 ESPP, are eligible to participate in the 2020 ESPP, provided that:

- such person is customarily employed by us or a designated subsidiary for more than 20 hours a week and for more than five months in a calendar year;

- such person has been employed by us or by a designated subsidiary for at least three months prior to enrolling in the 2020 ESPP; and
- such person was our employee or an employee of a designated subsidiary on the first day of the applicable offering period under the 2020 ESPP.

We retain the discretion to determine which eligible employees may participate in an offering under applicable regulations.

We expect to make one or more offerings to our eligible employees to purchase stock under the 2020 ESPP beginning at such time and on such dates as our board of directors may determine, or on the first business day thereafter. Each offering will consist of a six-month offering period during which payroll deductions will be made and held for the purchase of our common stock at the end of the offering period. Our board of directors or a committee designated by the board of directors may, at its discretion, choose a different period of not more than 12 months for offerings.

On each offering commencement date, each participant will be granted an option to purchase, on the last business day of the offering period, up to a number of shares of our common stock determined by multiplying $2,083 by the number of full months in the offering period and dividing that product by the closing price of our common stock on the first day of the offering period. No employee may be granted an option under the 2020 ESPP that permits the employee's rights to purchase shares under the 2020 ESPP and any other employee stock purchase plan of ours or of any of our subsidiaries to accrue at a rate that exceeds $25,000 of the fair market value of our common stock (determined as of the first day of each offering period) for each calendar year in which the option is outstanding. In addition, no employee may purchase shares of our common stock under the 2020 ESPP that would result in the employee owning 5% or more of the total combined voting power or value of our stock or the stock of any of our subsidiaries.

Each eligible employee may authorize up to a maximum of 15% of his or her compensation to be deducted by us during the offering period. Each employee who continues to be a participant in the 2020 ESPP on the last business day of the offering period will be deemed to have exercised an option to purchase from us the number of whole shares of our common stock that his or her accumulated payroll deductions on such date will pay for, not in excess of the maximum numbers set forth above. Under the terms of the 2020 ESPP, the purchase price will be determined by our board of directors or the committee for each offering period and will be at least 85% of the applicable closing price of our common stock. If our board of directors or the committee does not make a determination of the purchase price, the purchase price will be 85% of the lesser of the closing price of our common stock on the first business day of the offering period or on the last business day of the offering period.

An employee may at any time prior to the close of business on the fifteenth business day (or such other number of days as is determined by us) prior to the end of the offering period, and for any reason, permanently withdraw from participating in the offering and permanently withdraw the balance accumulated in the employee's account. Partial withdrawals are not permitted. If an employee elects to discontinue his or her payroll deductions during an offering period but does not elect to withdraw his or her funds, funds previously deducted will be applied to the purchase of common stock at the end of the offering period. If a participating employee's employment ends before the last business day of an offering period, no additional payroll deductions will be taken and the balance in the employee's account will be paid to the employee.

We will be required to make equitable adjustments to the extent determined by our board of directors or a committee thereof to the number and class of securities available under the 2020 ESPP, the share limitations under the 2020 ESPP, and the purchase price for an offering period under the 2020 ESPP to reflect stock splits, reverse stock splits, stock dividends, recapitalizations, combinations of shares, reclassifications of shares, spin-

offs and other similar changes in capitalization or events or any dividends or distributions to holders of our common stock other than ordinary cash dividends.

In connection with a merger or other reorganization event, as defined in the 2020 ESPP, our board of directors or a committee of our board of directors may take any one or more of the following actions as to outstanding options to purchase shares of our common stock under the 2020 ESPP on such terms as our board of directors or committee thereof determines:

- provide that options will be assumed, or substantially equivalent options will be substituted, by the acquiring or succeeding corporation (or an affiliate of the acquiring or succeeding corporation);
- upon written notice to employees, provide that all outstanding options will be terminated immediately prior to the consummation of such reorganization event and that all such outstanding options will become exercisable to the extent of accumulated payroll deductions as of a date specified by the board of directors or committee thereof in such notice, which date will not be less than ten days preceding the effective date of the reorganization event;
- upon written notice to employees, provide that all outstanding options will be cancelled as of a date prior to the effective date of the reorganization event and that all accumulated payroll deductions will be returned to participating employees on such date;
- in the event of a reorganization event under the terms of which holders of our common stock will receive upon consummation thereof a cash payment for each share surrendered in the reorganization event, change the last day of the offering period to be the date of the consummation of the reorganization event and make or provide for a cash payment to each employee equal to (1) the cash payment for each share surrendered in the reorganization event times the number of shares of our common stock that the employee's accumulated payroll deductions as of immediately prior to the reorganization event could purchase at the applicable purchase price, where the cash payment for each share surrendered in the reorganization event is treated as the fair market value of our common stock on the last day of the applicable offering period for purposes of determining the purchase price and where the number of shares that could be purchased is subject to the applicable limitations under the 2020 ESPP minus (2) the result of multiplying such number of shares by the purchase price; and/or
- provide that, in connection with our liquidation or dissolution, options convert into the right to receive liquidation proceeds (net of the purchase price thereof).

Our board of directors may at any time, and from time to time, amend or suspend the 2020 ESPP or any portion of the 2020 ESPP. We will obtain stockholder approval for any amendment if such approval is required by Section 423 of the Code. Further, our board of directors may not make any amendment that would cause the 2020 ESPP to fail to comply with Section 423 of the Code. The 2020 ESPP may be terminated at any time by our board of directors. Upon termination, we will refund all amounts in the accounts of participating employees.

401(k) Plan

We maintain a defined contribution employee retirement plan for our employees, including our named executive officers. The plan is intended to qualify as a tax-qualified 401(k) plan so that contributions to the 401(k) plan, and income earned on such contributions, are not taxable to participants until withdrawn or distributed from the 401(k) plan (except in the case of contributions under the 401(k) plan designated as Roth contributions). Under the 401(k) plan, each employee is fully vested in his or her deferred salary contributions and any qualified nonelective contributions made by us. Employee contributions are held and invested by the plan's trustee as directed by participants. The 401(k) plan provides us with the discretion to match employee contributions.

Health/welfare plans

All of our full-time employees, including our named executive officers, are eligible to participate in our health and welfare plans, including medical and dental benefits, short-term and long-term disability insurance, and life insurance. We believe these benefits are necessary and appropriate to provide a competitive compensation package to our named executive officers.

Rule 10b5-1 plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. It also is possible that the director or officer could amend the plan in certain circumstances when not in possession of material, nonpublic information or terminate the plan. In addition, our directors and executive officers may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

Limitations on liability and indemnification matters

Our restated certificate of incorporation, which will become effective upon the closing of this offering, limits the liability of our directors for breach of fiduciary duty to the maximum extent permitted by the Delaware General Corporation Law, or the DGCL, and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;
- for voting for or assenting to unlawful payments of dividends, stock repurchases or other distributions; or
- for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the DGCL is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the DGCL.

In addition, our restated certificate of incorporation, which will become effective upon the closing of this offering, provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers specified liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers. In addition, we intend to enter into indemnification agreements with all of our executive officers and directors prior to the completion of this offering. These indemnification agreements may require us, among other things, to indemnify each such executive officer or director for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him or her in any action or proceeding arising out of his or her service as one of our executive officers or directors.

Some of our non-employee directors may, through their relationships with their employers, be insured or indemnified against specified liabilities incurred in their capacities as members of our board of directors.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, executive officers or persons controlling us, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act of 1933, or the Securities Act, and is therefore unenforceable.

Director compensation

The table below shows all compensation to our non-employee directors during the year ended December 31, 2019. Joshua Brumm, our President and Chief Executive Officer, is also a member of our board of directors, but he did not receive any additional compensation for service as a director. Mr. Brumm's compensation as an executive officer is set forth above under "—Summary Compensation Table."

Name	Fees earned or paid in cash ($)	Stock awards ($) (1)	Option awards ($) (2)	All other compensation ($)	Total ($)
Jason Rhodes	—	—	—	—	—
Edward Hurwitz	—	—	—	—	—
Dirk Kersten	—	—	—	—	—
Catherine Stehman-Breen, M.D.	12,500	—	22,275	77,868(3)	112,643
Lawrence Klein, Ph.D.	6,250	—	22,275	—	28,525
David Lubner(4)	—	—	—	—	—

(1) As of December 31, 2019, none of our non-employee directors held common stock other than Dr. Stehman-Breen who holds 104,988 shares of common stock pursuant to a restricted stock award.

(2) The amounts reported represent the aggregate grant date fair value of stock options awarded in 2019, calculated in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value are set forth in Note 10 to our audited financial statements appearing elsewhere in this prospectus. These amounts reflect the accounting cost for these stock options and do not reflect the actual economic value that may be realized by the directors upon the vesting of the stock options, the exercise of the stock options or the sale of the common underlying such stock options. As of December 31, 2019, none of our non-employee directors held common stock subject to outstanding option awards other than Dr. Stehman-Breen and Dr. Klein who each held 32,808 shares of common stock subject to outstanding option awards. In March 2020, Mr. Lubner received an option award for 32,808 shares of common stock.

(3) Consists of consulting fees paid to Dr. Stehman-Breen through Atlas Venture for consulting services. For further information, see "Certain Relationships and Related Party Transactions—Atlas Venture services."

(4) Mr. Lubner was elected to our board of directors in March 2020.

Prior to this offering, we granted options to purchase 32,808 shares of our common stock to certain of our non-employee directors upon their initial election to our board of directors and paid annual cash fees of $25,000 to certain of our non-employee directors for their service on our board of directors. However, we did not have a formal non-employee director compensation policy. We have historically reimbursed our non-employee directors for reasonable travel and out-of-pocket expenses incurred in connection with attending board of director and committee meetings.

Non-employee director compensation policy

In August 2020, our board of directors approved a non-employee director compensation program, which became effective upon the effectiveness of the registration statement of which this prospectus is a part, that is designed to enable us to attract and retain, on a long-term basis, highly qualified non-employee directors. Under this director compensation program, we will pay our non-employee directors a cash retainer for service on the board of directors and for service on each committee on which the director is a member. The chair of the board and of each committee will receive higher retainers for such service. These fees are payable in arrears in four equal quarterly installments on the last day of each quarter, provided that the amount of such payment will be prorated for any portion of such quarter that the director is not serving on our board of directors and no fee will be payable in respect of any period prior to the effectiveness of the registration statement for this

offering. The fees paid to non-employee directors for service on the board of directors and for service on each committee of the board of directors on which the director is a member are as follows:

	Member annual fee	Chair additional annual fee
Board of Directors	$35,000	$ 30,000
Audit Committee	7,500	15,000
Compensation Committee	5,000	10,000
Nominating and Corporate Governance Committee	4,000	8,000

We also will continue to reimburse our non-employee directors for reasonable travel and other expenses incurred in connection with attending meetings of our board of directors and any committee of our board of directors on which they serve.

In addition, under our director compensation program, each non-employee director will receive, upon his or her initial election or appointment to our board of directors, an option to purchase 39,193 shares of our common stock under the 2020 Plan. Each of these options will vest in equal monthly installments until all shares are vested on the third anniversary of the date of grant, subject to the non-employee director's continued service as a director. Further, on the date of each annual meeting of stockholders, each non-employee director that has served on our board of directors for at least six months will receive an option to purchase 19,596 shares of our common stock under the 2020 Plan. Each of these options will vest in full on the earlier of the first anniversary of the date of grant and the next annual meeting of stockholders following the date of grant, subject to the non-employee director's continued service as a director. All options issued to our non-employee directors under our director compensation program will be issued at exercise prices equal to the fair market value of our common stock on the date of grant and will have a term of ten years.

Certain relationships and related party transactions

Since our formation on December 1, 2017, we have engaged in the following transactions in which the amounts involved exceeded $120,000 and any of our directors, executive officers or holders of more than 5% of our voting securities, or any member of the immediate family of, or person sharing the household with, the foregoing persons, had or will have a direct or indirect material interest. We believe that all of the transactions described below were made on terms no less favorable to us than could have been obtained from unrelated third parties.

SAFEs

Between January 2018 and October 2018, we issued Simple Agreements for Future Equity, or SAFEs, in an aggregate principal amount of $5,000,000 to Atlas Venture Fund XI, L.P., a holder of more than 5% of our voting securities. On November 29, 2018, these agreements, which we refer to as the Atlas SAFEs, were converted into 5,000,000 shares of our Series A preferred stock.

Atlas Venture services

We have received professional services from Atlas Venture Life Science Advisors, LLC, an affiliate of Atlas Venture Fund XI, L.P., a holder of more than 5% of our voting securities. We paid Atlas Venture a total of $0.2 million and $0.1 million related to these services for the years ended December 31, 2018 and 2019, including consulting fees paid to Dr. Catherine Stehman-Breen prior to her election to our board of directors. We do not expect to receive any further services from Atlas Venture Life Science Advisors, LLC.

Common stock issuance

In October 2018, we issued and sold 2,110,404 shares of our common stock to Atlas Venture Fund XI, L.P., a holder of more than 5% of our voting securities, at a purchase price of $0.003 per share, for an aggregate purchase price of $7,000.

Series A preferred stock financing

In November 2018, in connection with the initial closing of our Series A preferred stock financing, we issued and sold 7,500,000 shares of our Series A preferred stock at a price per share of $1.00 in cash, for an aggregate purchase price of $7,500,000 and also issued 5,000,000 shares of our Series A preferred stock upon conversion of the Atlas SAFEs. The following table sets forth the aggregate number of shares of our Series A preferred stock that we issued and sold in November 2018 to our directors, officers and 5% stockholders and their affiliates in the initial closing of our Series A preferred stock financing and the aggregate amount of consideration for such shares:

Purchaser(1)	Shares of Series A preferred stock	Total purchase price
Atlas Venture Fund XI, L.P.	5,000,000	$ 5,000,000(2)
Forbion Capital Fund IV Cooperatief U.A.	3,750,000	$ 3,750,000
Entities affiliated with MPM Bioventures	3,750,000	$ 3,750,000

(1) See “Principal stockholders” for additional information about shares held by these entities.

(2) We issued 5,000,000 shares of our Series A preferred stock upon conversion of the Atlas SAFEs. See “—SAFEs.”

In April 2019, in connection with the second closing of our Series A preferred stock financing, we issued an aggregate of 20,000,000 additional shares of our Series A preferred stock at a price per share of $1.00 in cash, for an aggregate purchase price of $20,000,000. The following table sets forth the aggregate number of shares of our Series A preferred stock that we issued in April 2019 to our directors, officers and 5% stockholders and their affiliates in the second closing of our Series A preferred stock financing and the aggregate amount of consideration for such shares:

Purchaser(1)	Shares of Series A preferred stock	Total purchase price
Atlas Venture Fund XI, L.P.	8,000,000	$ 8,000,000
Forbion Capital Fund IV Cooperatief U.A.	6,000,000	$ 6,000,000
Entities affiliated with MPM Bioventures	6,000,000	$ 6,000,000

(1) See “Principal stockholders” for additional information about shares held by these entities.

In July 2020, in connection with the third closing of our Series A preferred stock financing, we issued an aggregate of 17,500,000 additional shares of our Series A preferred stock at a price per share of $1.00 in cash, for an aggregate purchase price of $17,500,000. The following table sets forth the aggregate number of shares of our Series A preferred stock that we issued in July 2020 to our directors, officers and 5% stockholders and their affiliates in the third closing of our Series A preferred stock financing and the aggregate amount of consideration for such shares:

Purchaser(1)	Shares of Series A preferred stock	Total Purchase Price
Atlas Venture Fund XI, L.P.	7,000,000	$ 7,000,000
Forbion Capital Fund IV Cooperatief U.A.	5,250,000	$ 5,250,000
Entities affiliated with MPM Bioventures	5,250,000	$ 5,250,000

(1) See “Principal stockholders” for additional information about shares held by these entities.

Each share of Series A preferred stock is convertible into 0.3015 shares of common stock.

Series B preferred stock financing

In August 2020, we issued an aggregate of 41,159,724 shares of our Series B preferred stock at a price per share of $2.811 in cash, for an aggregate purchase price of $115,699,984. The following table sets forth the aggregate number of shares of our Series B preferred stock that we issued to our directors, officers and 5% stockholders and their affiliates and the aggregate amount of consideration for such shares:

Purchaser(1)	Shares of Series B preferred stock	Total Purchase Price
Atlas Venture Opportunity Fund I, L.P.	5,336,179	$ 14,999,999
Forbion Capital Fund IV Cooperatief U.A.	5,336,179	$ 14,999,999
Entities affiliated with MPM Bioventures	1,422,981	$ 4,000,000
Catherine Stehman-Breen, M.D.	35,574	$ 99,999
David C. Lubner	177,872	$ 499,998
Lawrence Klein, Ph.D.	35,574	$ 99,999

(1) See “Principal stockholders” for additional information about shares held by these entities and persons.

Each share of Series B preferred stock is convertible into 0.3015 shares of common stock.

Registration rights

We are a party to an investors' rights agreement with the holders of our preferred stock, including our 5% stockholders and their affiliates. This investors' rights agreement provides these stockholders the right, subject to certain conditions, beginning six months following the completion of this offering, to demand that we file a registration statement or to request that their shares be covered by a registration statement that we are otherwise filing.

See "Description of capital stock—Registration rights" for additional information regarding these registration rights.

Indemnification agreements

Our restated certificate of incorporation, which will become effective upon the closing of this offering, provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we intend to enter into new indemnification agreements with all of our directors and executive officers prior to the completion of this offering. These indemnification agreements may require us, among other things, to indemnify each such director or executive officer for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him or her in any action or proceeding arising out of his or her service as one of our directors or executive officers. See "Executive compensation—Limitations on liability and indemnification matters" for additional information.

Offer letters and severance benefits plan

We have entered into offer letters with our executive officers and have adopted a severance benefits plan for certain of our employees, including each of our executive officers. For more information regarding these arrangements with our named executive officers, see the section entitled "Executive compensation—Employment arrangements with our named executive officers."

Policies and procedures for related person transactions

Our board of directors has adopted written policies and procedures for the review of any transaction, arrangement or relationship in which our company is a participant, the amount involved exceeds $120,000, and one of our executive officers, directors, director nominees or 5% stockholders (or their immediate family members), each of whom we refer to as a "related person," has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a "related person transaction," the related person must report the proposed related person transaction to our principal financial officer. The policy calls for the proposed related person transaction to be reviewed and, if deemed appropriate, approved by our audit committee. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the audit committee will review, and, in its discretion, may ratify the related person transaction. The policy also permits the chair of the audit committee to review and, if deemed appropriate, approve proposed related person transactions that arise between audit committee meetings, subject to ratification by the audit committee at its next meeting. Any related person transactions that are ongoing in nature will be reviewed annually.

A related person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the audit committee after full disclosure of the related person's interest in the transaction. As appropriate for the circumstances, the audit committee will review and consider:

- the related person's interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;
- the approximate dollar value of the amount of the related person's interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the transaction; and
- any other information regarding the related person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

Our audit committee may approve or ratify the transaction only if it determines that, under all of the circumstances, the transaction is in, or is not inconsistent with, our best interests. Our audit committee may impose any conditions on the related person transaction that it deems appropriate.

In addition to the transactions that are excluded by the instructions to the SEC's related person transaction disclosure rule, our board of directors has determined that the following transactions do not create a material direct or indirect interest on behalf of related persons and, therefore, are not related person transactions for purposes of this policy:

- interests arising solely from the related person's position as an executive officer of another entity, whether or not the person is also a director of such entity, that is a participant in the transaction, where the related person and all other related persons own in the aggregate less than a 10% equity interest in such entity, the related person and his or her immediate family members are not involved in the negotiation of the terms of the transaction and do not receive any special benefits as a result of the transaction, and the amount involved in the transaction is less than the greater of $200,000 or 5% of the annual gross revenues of the company receiving payment under the transaction; and
- a transaction that is specifically contemplated by provisions of our certificate of incorporation or bylaws.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by our compensation committee in the manner specified in the compensation committee's charter.

We did not have a written policy regarding the review and approval of related person transactions prior to this offering. Nevertheless, with respect to such transactions, it has been the practice of our board of directors to consider the nature of and business reasons for such transactions, how the terms of such transactions compared to those which might be obtained from unaffiliated third parties and whether such transactions were otherwise fair to and in the best interests of, or not contrary to, our best interests.

Principal stockholders

The following table sets forth information with respect to the beneficial ownership of our common stock as of August 31, 2020 by:

- each of our directors;
- each of our named executive officers;
- all of our executive officers and directors as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

The column entitled “Percentage of shares beneficially owned—Before offering” is based on a total of 31,333,643 shares of our common stock outstanding as of August 31, 2020, including 453,579 shares of unvested restricted stock subject to forfeiture, and assuming the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 28,086,375 shares of our common stock upon the closing of this offering. The column entitled “Percentage of shares beneficially owned—After offering” is based on 43,585,221 shares of our common stock to be outstanding after this offering, including the shares of our common stock that we are selling in this offering and the 453,579 shares of unvested restricted stock subject to forfeiture, but not including any additional shares issuable upon exercise of outstanding options or any additional shares issuable upon the underwriters’ option to purchase additional shares.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock that an individual has a right to acquire within 60 days after August 31, 2020 are considered outstanding and beneficially owned by the person holding such right for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person, except with respect to the percentage ownership of all directors and executive officers. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Unless otherwise indicated, the address of each beneficial owner is c/o Dyne Therapeutics, Inc., 830 Winter Street, Waltham, MA 02451.

Name of beneficial owner	Number of shares beneficially owned prior to offering	Percentage of shares beneficially owned	
		Before offering	After offering
5% stockholders:			
Entities affiliated with Atlas Venture Fund XI, L.P.(1)	9,748,915	31.11%	22.37%
Forbion Capital Fund IV Cooperatief U.A.(2)	6,131,079	19.57	14.07
Entities affiliated with MPM Bioventures(3)	4,951,303	15.80	11.36
Entities affiliated with RA Capital Management, L.P.(4)	2,145,045	6.85	4.92
Entities affiliated with Vida Ventures, LLC(5)	2,145,046	6.85	4.92
Citadel Multi-Strategy Equities Master Fund Ltd.(6).	1,608,785	5.13	3.69
Wellington Biomedical Innovation Master Investors (Cayman) I L.P.(7)	1,608,785	5.13	3.69
Named executive officers and directors:			
Jason Rhodes(1)	9,748,915	31.11	22.37
Edward Hurwitz(3)	4,951,303	15.80	11.36
Dirk Kersten(2)	6,131,079	19.57	14.07
Lawrence Klein, Ph.D.(8).	18,927	*	*
David Lubner(9)	53,625	*	*
Catherine Stehman-Breen, M.D.(10)	125,965	*	*
Joshua Brumm(11)	139,437	*	*
Romesh Subramanian, Ph.D.(12)	524,940	1.68	1.20
Jonathan McNeill, M.D.(13)	36,909	*	*
All executive officers and directors as a group(14) (12 persons)	21,731,100	68.93	49.64

* Represents beneficial ownership of less than 1%.

(1) Consists of (i) 6,029,726 shares of common stock issuable upon the conversion of shares of Series A preferred stock held by Atlas Venture Fund XI, L.P., or Atlas Fund XI, (ii) 2,110,404 shares of common stock held by Atlas Fund XI and (iii) 1,608,785 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by Atlas Venture Opportunity Fund I, L.P., or Atlas Fund I. Atlas Venture Associates XI, L.P. is the general partner of Atlas Fund XI, and Atlas Venture Associates XI, LLC is the general partner of Atlas Venture Associates XI, L.P. Bruce Booth, Jean- Francois Formela, David Grayzel, Jason Rhodes and Kevin Bitterman are the members of Atlas Venture Associates XI, LLC and collectively make investment decisions on behalf of Atlas Venture Fund XI, LLC. Each of Atlas Fund XI, Atlas Venture Associates XI, L.P., and Atlas Venture Associates XI, LLC may be deemed to beneficially own the shares held by Atlas Fund XI. Atlas Venture Associates Opportunity I, L.P. is the general partner of Atlas Fund I, and Atlas Venture Associates Opportunity I, LLC, or AVAO, LLC, is the general partner of Atlas Venture Associates Opportunity I, L.P. Bruce Booth, Jean-Francois Formela, David Grayzel, Jason Rhodes and Kevin Bitterman are the members of AVAO, LLC and collectively make investment decisions on behalf of AVAO, LLC. Each of Atlas Fund I, Atlas Venture Associates Opportunity I, L.P. and AVAO, LLC may be deemed to beneficially own the shares held by Atlas Fund I. Jason Rhodes is also a member of our board of directors. Mr. Rhodes disclaims beneficial ownership of the shares listed, except to the extent of his pecuniary interest therein, if any. The mailing address of Atlas Fund XI and Atlas Fund I is 400 Technology Square, 10th Floor, Cambridge, MA 02139.

(2) Consists of (i) 4,522,293 shares of common stock issuable upon the conversion of shares of Series A preferred stock and (ii) 1,608,786 shares of common stock issuable upon the conversion of shares of Series B preferred stock. All shares are held directly by Forbion Capital Fund IV Coöperatief U.A., or FCF IV. Forbion IV Management B.V., or Forbion Management, the director of FCF IV, may be deemed to have voting and dispositive power over the shares held by FCF IV. Investment decisions with respect to the shares held by FCF IV can be made by FCPM III Services B.V., the director of Forbion Management, which may delegate such powers to its investment committee which may delegate such powers to the authorized representatives of Forbion Management. Messrs. Slootweg, van Osch, Mulder, van Houten, Reithinger and Boorsma, or the Partners, are partners of FCPM III Services B.V., which acts as the investment advisor to the directors of FCF IV. Each of the Partners

disclaim beneficial ownership of such shares, except to the extent of his pecuniary interest therein. Dirk Kersten is a partner of Forbion Management and a member of the investment committee of Forbion Management and is also a member of our board of directors. The address for FCF IV, Forbion Management and FCPM III Services B.V. is Gooimeer 2-35, 1411 DC Naarden, the Netherlands.

(3) Consists of (i) 4,215,076 shares of common stock issuable upon the conversion of shares of Series A preferred stock held by MPM Bioventures 2018, L.P., or MPM 2018, (ii) 83,188 shares of common stock issuable upon the conversion of shares of Series A preferred stock held by MPM Asset Management Investors BV2018 LLC or MPM BV2018, (iii) 224,025 shares of common stock issuable upon the conversion of shares of Series A preferred stock held by MPM Bioventures 2018 (B), L.P., or MPM 2018 (B), (iv) 399,867 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by MPM 2018, (v) 7,893 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by MPM BV2018 and (vi) 21,254 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by MPM 2018 (B). MPM 2018, MPM BV2018 and MPM 2018 (B) are collectively referred to as the MPM Entities. Edward Hurwitz, a member of our board of directors, Luke Evnin, Ansbert Gadicke, and Todd Foley are the Managing Directors of MPM BioVentures 2018 LLC, or BV2018 LLC. BV2018 LLC is the Managing Member of MPM BioVentures 2018 GP LLC, which is the General Partner of MPM 2018 and MPM 2018 (B). MPM BV2018 invests alongside MPM 2018 and MPM 2018 (B). Each of Dr. Evnin, Dr. Gadicke, Mr. Foley and Mr. Hurwitz shares power to vote, acquire, hold and dispose of the shares held by each of the MPM Entities. Each of the entities and individuals listed above expressly disclaims beneficial ownership of the securities listed above except to the extent of any pecuniary interest therein. The address of each of the MPM Entities is 450 Kendall Street, Cambridge, MA 02142.

(4) Consists of (i) 1,458,068 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by RA Capital Healthcare Fund, L.P., or RA Capital (ii) 536,261 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by RA Capital Nexus Fund, L.P., or Nexus Fund and (iii) 150,716 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by Blackwell Partners LLC—Series A, or Blackwell. RA Capital Management, L.P. is the investment manager for RA Capital, Nexus Fund and Blackwell. The general partner of RA Capital Management, L.P. is RA Capital Management GP, LLC, of which Peter Kolchinsky and Rajeev Shah are managing members. RA Capital Management, L.P., RA Capital Management GP, LLC, Peter Kolchinsky and Rajeev Shah may be deemed to have voting and investment power over the shares held of record by RA Capital, Nexus Fund and Blackwell. RA Capital Management, L.P., RA Capital Management GP, LLC, Peter Kolchinsky and Rajeev Shah disclaim beneficial ownership of such shares, except to the extent of any pecuniary interest therein. The address of the entities listed above is 200 Berkeley Street, 18th Floor, Boston, Massachusetts 02116.

(5) Consists of (i) 2,087,130 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by Vida Ventures II, LLC, or Vida II, and (ii) 57,916 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by Vida Ventures II-A, LLC, or Vida II-A. VV Manager II LLC, or VV Manager II, is the manager of Vida II and Vida II-A. Arie Belldegrun, Fred Cohen, and Leonard Potter, the members of the management committee of VV Manager II, along with the other members of the investment committee, Stefan Vitorovic, Arjun Goyal, Helen Kim, Rajul Jain, and Joshua Kazam, may be deemed to share voting and dispositive power over the shares held by Vida II and Vida II-A. The address of Vida II and Vida II-A is 40 Broad Street, Suite 201, Boston, Massachusetts 02109.

(6) Consists of 1,608,785 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by Citadel Multi-Strategy Equities Master Fund Ltd., or Citadel. Citadel Advisors LLC, or Citadel Advisors, acts as the portfolio manager of Citadel. Citadel Advisors Holdings LP, or CAH, is the sole member of Citadel Advisors, and Citadel GP LLC, or CGP, is the general partner of CAH. Kenneth Griffin owns a controlling interest in CGP and may be deemed to share voting and dispositive power over shares held by Citadel. The address for this entity is c/o Citadel Advisors LLC, 601 Lexington Avenue, New York, New York 10022.

(7) Consists of 1,608,785 shares of common stock issuable upon the conversion of shares of Series B preferred stock held by Wellington Biomedical Innovation Master Investors (Cayman) I L.P., or Wellington Biomedical Fund. Wellington Management Company LLP, a registered investment company under the Investment Company Act of 1940, as amended, is the investment advisor to Wellington Biomedical Fund, and Wellington Alternative Investments LLC is its general partner. Wellington Management Investment, Inc. is the managing member of Wellington Alternative Investments LLC. Wellington Management Company LLP is an indirect subsidiary of Wellington Management Group LLP. Wellington Management Group LLP and Wellington Management Company LLP may be deemed beneficial owners with shared voting and investment power over the shares held by Wellington Biomedical Fund. The address for Wellington Biomedical Fund and the Wellington entities is 280 Congress Street, Boston, Massachusetts 02210.

(8) Consists of (i) 10,725 shares of common stock issuable upon the conversion of shares of Series B preferred stock and (ii) 8,202 shares of common stock underlying options exercisable within 60 days of August 31, 2020.

(9) Consists of 53,625 shares of common stock issuable upon the conversion of shares of Series B preferred stock.

(10) Consists of (i) 104,988 shares of restricted common stock, (ii) 10,725 shares of common stock issuable upon the conversion of shares of Series B preferred stock and (iii) 10,252 shares of common stock underlying options exercisable within 60 days of August 31, 2020.

(11) Consists of 139,437 shares of common stock underlying options exercisable within 60 days of August 31, 2020.

(12) Consists of 524,940 shares of restricted common stock.

(13) Consist of 36,909 shares of common stock underlying options exercisable within 60 days of August 31, 2020.

(14) Consists of (i) 2,110,404 shares of common stock, (ii) 629,928 shares of restricted common stock, (iii) 15,074,308 shares of common stock issuable upon conversion of shares of Series A preferred stock, (iv) 3,721,660 shares of common stock issuable upon conversion of shares of Series B preferred stock and (v) 194,800 shares of common stock underlying options exercisable within 60 days of August 31, 2020.

Description of capital stock

The following description of our capital stock and provisions of our restated certificate of incorporation and amended and restated bylaws are summaries and are qualified by reference to the restated certificate of incorporation and amended and restated bylaws that will become effective upon the closing of this offering. We have filed copies of these documents with the SEC as exhibits to our registration statement of which this prospectus is a part. The description of our common stock reflects changes to our capital structure that will occur upon the closing of this offering.

Upon the closing of this offering, our authorized capital stock will consist of 200,000,000 shares of our common stock, par value $0.0001 per share, and 10,000,000 shares of our preferred stock, par value $0.0001 per share, all of which preferred stock will be undesignated.

As of August 31, 2020, we had issued and outstanding:

- 3,247,268 shares of our common stock held by 20 stockholders of record, which includes 453,579 shares of unvested restricted stock subject to forfeiture;
- 52,000,000 shares of our Series A preferred stock held by six stockholders of record, which shares are convertible into an aggregate of 15,677,280 shares of our common stock; and
- 41,159,724 shares of our Series B preferred stock held by 19 stockholders of record, which shares are convertible into an aggregate of 12,409,095 shares of our common stock.

Upon the closing of this offering, all of the outstanding shares of our preferred stock will automatically convert into an aggregate of 28,086,375 shares of our common stock.

Common stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. Each election of directors by our stockholders will be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of outstanding preferred stock that we may designate and issue in the future.

In the event of our liquidation or dissolution, the holders of our common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any of our outstanding preferred stock. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

Preferred stock

Under the terms of our restated certificate of incorporation that will become effective upon the closing of this offering, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other

corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Upon the closing of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

Options and unvested restricted common stock

As of August 31, 2020, options to purchase an aggregate of 5,201,900 shares of our common stock were outstanding under our 2018 Plan, at a weighted average exercise price of $4.03 per share, and 453,579 shares of unvested restricted common stock were outstanding. See "Executive compensation—Employee benefit and equity compensation plans" for additional information regarding the terms of our 2018 Plan.

Registration rights

We have entered into an amended and restated investors' rights agreement dated as of August 7, 2020, or the investors' rights agreement, with holders of our preferred stock. Subject to the lock-up agreements described below, beginning 180 days following the closing of this offering, holders of a total of 30,301,767 shares of our common stock will have the right, along with holders of an additional 196,890 shares of our common stock issuable upon exercise of outstanding options, to require us to register these shares under the Securities Act under specified circumstances. We refer to the shares with these registration rights as registrable securities. After registration pursuant to these rights, the registrable securities will become freely tradable without restriction under the Securities Act. The registration rights under the investors' rights agreement terminate upon the earliest to occur of:

- the closing of a "Deemed Liquidation Event," as such term is defined in our certificate of incorporation;
- following the closing of this offering, with respect to any holder party to the investors' rights agreement, such time as Rule 144 of the Securities Act or another similar exemption under the Securities Act is available for the sale of all of the shares held by such holder without limitation during a three-month period without registration; or
- the fifth anniversary of the closing of this offering.

Demand registration rights

Beginning 180 days after the effective date of the registration statement of which this prospectus is a part, subject to specified limitations set forth in the investors' rights agreement, at any time, the holders of a majority of the then outstanding registrable securities may demand that we register at least 30% of the registrable securities then outstanding under the Securities Act for purposes of a public offering.

In addition, subject to specified limitations set forth in the investors' rights agreement, at any time after we become eligible to file a registration statement on Form S-3, certain holders of at least 20% of the registrable securities then outstanding may request that we register their registrable securities on Form S-3 for purposes of a public offering for which the anticipated aggregate offering price to the public would exceed, net of selling expenses, $5.0 million.

We are required to use our commercially reasonable efforts to cause such registration statements to become effective.

Incidental registration rights

If, at any time after the closing of this offering, we propose to register any of our securities under the Securities Act in connection with a public offering of such securities solely for cash, the holders of registrable securities

will be entitled to notice of the registration and, subject to specified exceptions, have the right to require us to register all or a portion of the registrable securities then held by them in that registration. We have the right to terminate or withdraw any registration initiated by us before the effective date of such registration.

In the event that any registration in which the holders of registrable securities participate pursuant to our investors' rights agreement is an underwritten public offering, we have agreed to enter into an underwriting agreement in usual and customary form.

Expenses

Pursuant to the investors' rights agreement, we are required to pay all registration expenses, including all registration, filing and qualification fees; printing and accounting fees; and fees and disbursements not to exceed $35,000 of one counsel representing the selling stockholders, but excluding underwriting discounts, selling commissions and stock transfer taxes applicable to the sale of registrable securities and the fees and expenses of the selling stockholders' own counsel (other than the counsel selected to represent all selling stockholders).

The investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the registration statement attributable to us or any violation or alleged violation whether by action or inaction by us under the Securities Act, the Exchange Act, any state securities or Blue Sky law or any rule or regulation promulgated under the Securities Act, the Exchange Act or any state securities or Blue Sky law in connection with such registration statement or the qualification or compliance of the offering, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

Delaware anti-takeover law and certain charter and bylaw provisions

Delaware law

We are subject to Section 203 of the Delaware General Corporation Law, or DGCL. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless either the interested stockholder attained such status with the approval of our board of directors, the business combination is approved by our board of directors and stockholders in a prescribed manner or the interested stockholder acquired at least 85% of our outstanding voting stock in the transaction in which it became an interested stockholder. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person. The restrictions contained in Section 203 are not applicable to any of our existing stockholders that will own 15% or more of our outstanding voting stock upon the closing of this offering.

Staggered board; removal of directors

Our restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing of this offering divide our board of directors into three classes with staggered three-year terms. In addition, our restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing of this offering provide that directors may be removed only for cause and only by the affirmative vote of the holders of at least 75% of our shares of capital stock present in person or by proxy and entitled to vote. Under our restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing

of this offering, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Furthermore, our restated certificate of incorporation to be effective upon the closing of this offering provides that the authorized number of directors may be changed only by the resolution of our board of directors. The classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Super-majority voting

The DGCL provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our amended and restated bylaws to be effective upon the closing of this offering may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our restated certificate of incorporation described above.

Stockholder action; special meeting of stockholders; advance notice requirements for stockholder proposals and director nominations

Our restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing of this offering provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our restated certificate of incorporation and our amended and restated bylaws to be effective upon the closing of this offering also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by our board of directors. In addition, our bylaws to be effective upon the closing of this offering establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors, or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities. These provisions also could discourage a third party from making a tender offer for our common stock because even if the third party acquired a majority of our outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

Indemnification agreements

Our restated certificate of incorporation, which will become effective upon the closing of this offering, provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we intend to enter into indemnification agreements with all of our directors and executive officers prior to the completion of this offering. These indemnification agreements may require us, among other things, to

indemnify each such director or executive officer for some expenses, including attorneys' fees, judgments, fines, and settlement amounts incurred by him or her in any action or proceeding arising out of his or her service as one of our directors or executive officers.

Exclusive forum provision

Our restated certificate of incorporation to be effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) shall be the sole and exclusive forum for the following types of proceedings: (1) any derivative action or proceeding brought on behalf of our company, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the General Corporation Law of the State of Delaware or as to which the General Corporation Law of the State of Delaware confers jurisdiction on the Court of Chancery of the State of Delaware, or (4) any action asserting a claim arising pursuant to any provision of our restated certificate of incorporation or amended and restated bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine. These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Exchange Act. Furthermore, Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all such Securities Act actions. Accordingly, both state and federal courts have jurisdiction to entertain such claims. To prevent having to litigate claims in multiple jurisdictions and the threat of inconsistent or contrary rulings by different courts, among other considerations, our restated certificate of incorporation that will become effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the federal district courts of the United States of America shall, to the fullest extent permitted by law, be the sole and exclusive forum for the resolution of any claims arising under the Securities Act. While the Delaware courts have determined that such choice of forum provisions are facially valid, a stockholder may nevertheless seek to bring a claim in a venue other than those designated in the exclusive forum provisions. In such instance, we would expect to vigorously assert the validity and enforceability of the exclusive forum provisions of our restated certificate of incorporation. This may require significant additional costs associated with resolving such action in other jurisdictions and there can be no assurance that the provisions will be enforced by a court in those other jurisdictions.

Nasdaq Global Select Market

Our common stock has been approved for listing on the Nasdaq Global Select Market under the trading symbol "DYN."

Transfer agent and registrar

The transfer agent and registrar for our common stock is Computershare Trust Company, N.A. The transfer agent and registrar's address is 250 Royall Street, Canton, Massachusetts 02021, and its telephone number is (800) 884-4225.

Shares eligible for future sale

Prior to this offering, there has been no public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options, or the anticipation of these sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of equity securities.

Upon the closing of this offering, we will have outstanding 43,585,221 shares of our common stock, based on the 3,247,268 shares of our common stock that were outstanding on August 31, 2020, including 453,579 shares of unvested restricted stock subject to forfeiture, and after giving effect to the issuance of 12,251,578 shares of our common stock in this offering, assuming no exercise by the underwriters of their option to purchase additional shares of our common stock and the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 28,086,375 shares of our common stock upon the closing of this offering. Of these shares, all shares sold in this offering will be freely tradable without restriction or further registration under the Securities Act unless purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act.

The remaining 31,333,643 shares of our common stock will be "restricted securities" under Rule 144, and we expect that substantially all of these restricted securities will be subject to the 180-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market upon release or waiver of any applicable lock-up agreements and only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

Lock-up agreements

We and each of our directors and executive officers and holders of substantially all of our outstanding equity securities have agreed that, without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC, on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus, subject to extension in specified circumstances:

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for common stock, or make any public announcement of an intention to do any of the foregoing; or
- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of our common stock, whether any transaction described above is to be settled by delivery of our common stock or such other securities, in cash or otherwise.

These agreements are subject to certain exceptions, as described in the section of this prospectus entitled "Underwriting."

Rule 144

In general, under Rule 144 of the Securities Act, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell those shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least one

year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 435,852 shares immediately after this offering; and
- the average weekly trading volume in our common stock on the Nasdaq Global Select Market during the four calendar weeks preceding the date of filing of a Notice of Proposed Sale of Securities Pursuant to Rule 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us.

Upon waiver or expiration of the lock-up period described above, approximately 31,333,643 shares of our common stock will be eligible for sale under Rule 144. We cannot estimate the number of shares of our common stock that our existing stockholders will elect to sell under Rule 144.

Rule 701

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell these shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the various restrictions, including the availability of public information about us, holding period and volume limitations, contained in Rule 144. Subject to the 180-day lock-up period described above, approximately 3,629,244 shares of our common stock, based on shares outstanding as of August 31, 2020, will be eligible for sale in accordance with Rule 701.

Stock options and Form S-8 registration statement

Following this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding awards and reserved for future issuance under the 2018 Plan, the 2020 Plan and the 2020 ESPP. See “Executive compensation—Employee benefit and equity compensation plans” for additional information regarding these plans. Accordingly, shares of our common stock registered under the registration statements will be available for sale in the open market, subject to Rule 144 volume limitations applicable to affiliates, and subject to any vesting restrictions and lock-up agreements applicable to these shares.

Registration rights

Subject to the lock-up agreements described above, beginning 180 days following the closing of this offering, the holders of 30,301,767 shares of common stock will have rights, along with holders of an additional 196,890 shares of our common stock issuable upon exercise of outstanding options, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act. See “Description of capital stock—Registration rights” for additional information regarding these registration rights.

Material U.S. tax considerations for non-U.S. holders of common stock

The following is a discussion of material U.S. federal income and estate tax considerations relating to ownership and disposition of our common stock by a non-U.S. holder (as defined below). For purposes of this discussion, the term “non-U.S. holder” means a beneficial owner (other than a partnership or other entity or arrangement treated as a pass-through entity for U.S. federal income tax purposes) of our common stock that is not, for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation, or other entity treated as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States, any state thereof or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust, if a U.S. court is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have authority to control all substantial decisions of the trust or if the trust has a valid election in effect to be treated as a U.S. person under applicable U.S. Treasury Regulations.

This discussion is based on current provisions of the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, each as in effect as of the date of this prospectus, and all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any such change or different interpretation could alter the tax considerations to non-U.S. holders described in this prospectus. In addition, there can be no assurance that the Internal Revenue Service, or the IRS, will not challenge one or more of the tax considerations described in this prospectus.

This discussion addresses only non-U.S. holders that hold shares of our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all aspects of U.S. federal income and estate taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder’s individual circumstances nor does it address the alternative minimum tax, the Medicare contribution tax on net investment income or any aspects of U.S. state, local or non-U.S. taxes. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt organizations;
- government organizations;
- financial institutions;
- brokers or dealers in securities;
- pension plans;
- who hold or receive our common stock pursuant to the exercise of employee stock options or otherwise as compensation;
- controlled foreign corporations;
- passive foreign investment companies;

- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment; and
- certain U.S. expatriates.

In addition, this discussion does not address the tax treatment of partnerships (or entities or arrangements that are treated as pass-through entities for U.S. federal income tax purposes) or persons who hold their common stock through such partnerships or such entities or arrangements. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of the purchase, ownership and disposition of our common stock through a partnership or other pass-through entity, as applicable.

Prospective investors should consult their own tax advisors regarding the U.S. federal, state, local and non-U.S. income and other tax considerations of acquiring, holding and disposing of our common stock.

Dividends

If we pay distributions on our common stock, those distributions generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such non-U.S. holder's tax basis in the common stock. Any amount distributed in excess of basis will be treated as capital gain, subject to the tax treatment described below under the heading "—Gain on Disposition of Our Common Stock."

Dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence. A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such non-U.S. holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy applicable certification and other requirements. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim with the IRS. Non-U.S. holders are urged to consult their own tax advisors regarding their entitlement to benefits under a relevant income tax treaty.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. To claim the exemption, the non-U.S. holder must furnish to us or the applicable withholding agent a valid IRS Form W-8ECI (or applicable successor form), certifying that the dividends are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States. However, such U.S. effectively connected income is taxed on a net income basis at the same U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such non-U.S. holder's country of residence.

Gain on disposition of our common stock

A non-U.S. holder generally will not be subject to U.S. federal income tax on gain recognized on a disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the United States and, if an applicable income tax treaty so provides, the gain is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the United States; in these cases, the non-U.S. holder will be taxed on a net income basis at the same U.S. federal income tax rates applicable to United States persons (as defined in the Code), and if the non-U.S. holder is a foreign corporation, an additional branch profits tax at a 30% rate, or such lower rate as may be specified by an applicable income tax treaty, may also apply;
- the non-U.S. holder is a nonresident alien individual present in the United States for 183 days or more in the taxable year of the disposition and certain other requirements are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty) on the net gain derived from the disposition, which may be offset by U.S.-source capital losses of the non-U.S. holder, if any, provided the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses; or
- we are, or have been at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter), a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder held no more than 5% of our outstanding common stock, directly or indirectly, actually or constructively, during the shorter of the five year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. If we are determined to be a U.S. real property holding corporation and the foregoing exception does not apply, then the non-U.S. holder generally will be taxed on its net gain derived from the disposition at the income tax rates applicable to United States persons (as defined in the Code). Generally, a corporation is a "U.S. real property holding corporation" if the fair market value of its "U.S. real property interests" equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we believe that we are not currently, and we do not anticipate becoming, a "U.S. real property holding corporation" for U.S. federal income tax purposes. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rule described above.

Information reporting and backup withholding

We must report annually to the IRS and to each non-U.S. holder the gross amount of the dividends on our common stock paid to such holder and the tax withheld, if any, with respect to such dividends. Non-U.S. holders may have to comply with specific certification procedures to establish that the non-U.S. holder is not a U.S. person (as defined in the Code) in order to avoid backup withholding at the applicable rate, with respect to dividends on our common stock. Generally, a non-U.S. holder will comply with such procedures if it provides a properly executed IRS Form W-8BEN or W-8BEN-E (or other applicable Form W-8) or otherwise meets documentary evidence requirements for establishing that it is a non-U.S. holder, or otherwise establishes an exemption. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above under the heading "—Dividends," will generally be exempt from U.S. backup withholding.

Information reporting and backup withholding generally will apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the non-U.S. holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to

a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Rather, any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder may be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

FATCA

Provisions of the Code commonly referred to as the Foreign Account Tax Compliance Act, or FATCA, generally impose a 30% withholding tax on dividends on, and gross proceeds from the sale or other disposition of, our common stock if paid to a foreign entity unless (1) if the foreign entity is a "foreign financial institution," the foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (2) if the foreign entity is not a "foreign financial institution," the foreign entity identifies certain of its U.S. investors, or (3) the foreign entity is otherwise excepted under FATCA.

Withholding under FATCA generally applies to payments of dividends on our common stock. While withholding under FATCA may apply to payments of gross proceeds from a sale or other disposition of our common stock, under proposed U.S. Treasury Regulations, withholding on payments of gross proceeds is not required. Although such regulations are not final, applicable withholding agents may rely on the proposed regulations until final regulations are issued.

If withholding under FATCA is required on any payment related to our common stock, investors not otherwise subject to withholding (or that otherwise would be entitled to a reduced rate of withholding) on such payment may be able to seek a refund or credit from the IRS. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this section. Non-U.S. holders should consult their own tax advisors regarding the possible implications of FATCA on their investment in our common stock and the entities through which they hold our common stock.

Federal estate tax

Common stock owned or treated as owned by an individual who is a non-U.S. holder (as specially defined for U.S. federal estate tax purposes) at the time of death will be included in the individual's gross estate for U.S. federal estate tax purposes and, therefore, may be subject to U.S. federal estate tax, unless an applicable estate tax or other treaty provides otherwise.

The preceding discussion of material U.S. federal tax considerations is for prospective investors' information only. It is not tax advice. Prospective investors should consult their own tax advisors regarding the particular U.S. federal, state, local, and non-U.S. tax consequences of purchasing, holding, and disposing of our common stock, including the consequences of any proposed changes in applicable laws.

Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, Jefferies LLC, Piper Sandler & Co. and Stifel, Nicolaus & Company, Incorporated are acting as lead book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	5,145,663
Jefferies LLC	3,307,927
Piper Sandler & Co.	1,898,994
Stifel, Nicolaus & Company, Incorporated	1,898,994
Total	12,251,578

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $0.798 per share. After the initial offering of the shares to the public, if all of the shares of common stock are not sold at the initial public offering price, the underwriters may change the offering price and the other selling terms. Sales of any shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to 1,837,736 additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $1.33 per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without option to purchase additional shares exercise	With full option to purchase additional shares exercise
Per Share	$ 1.33	$ 1.33
Total	$ 16,294,599	$ 18,738,788

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $2,800,000. We have agreed to reimburse the underwriters for expenses relating to the clearance of this offering with the Financial Industry Regulatory Authority, Inc. in an amount up to $40,000.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, or submit to or file with the Securities and Exchange Commission a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exercisable or exchangeable for any shares of our common stock, or publicly disclose the intention to undertake any of the foregoing, or (ii) enter into any swap, hedging or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of any shares of common stock or any such other securities (regardless of whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold in this offering.

The restrictions on our actions, as described above, do not apply to certain transactions, including (i) the issuance of shares of common stock or securities convertible into or exercisable for shares of our common stock pursuant to the conversion or exchange of convertible or exchangeable securities or the exercise of warrants or options (including net exercise) or the settlement of RSUs (including net settlement), in each case outstanding on the date of the underwriting agreement and described in this prospectus; (ii) grants of stock options, stock awards, restricted stock, RSUs, or other equity awards and the issuance of shares of our common stock or securities convertible into or exercisable or exchangeable for shares of our common stock (whether upon the exercise of stock options or otherwise) to our employees, officers, directors, advisors, or consultants pursuant to the terms of an equity compensation plan in effect as of the closing of this offering and described in this prospectus, provided that such recipients enter into a lock-up agreement with the underwriters; or (iii) our filing of any registration statement on Form S-8 relating to securities granted or to be granted pursuant to any plan in effect on the date of the underwriting agreement and described in this prospectus or any assumed benefit plan pursuant to an acquisition or similar strategic transaction.

Our directors and executive officers, and substantially all of our stockholders (such persons, the “lock-up parties”) have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each lock-up party, with limited exceptions, for a period of 180 days after the date of this prospectus (such period, the “restricted period”), may not (and may not cause any of their direct or indirect affiliates to), without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such lock-up parties in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant (collectively with the common stock, the “lock-up securities”)), (2) enter into any hedging, swap or other agreement or transaction that transfers, in whole or in part, any of the economic consequences of ownership of the lock-up securities, whether any such transaction

described in clause (1) or (2) above is to be settled by delivery of common stock or any other lock-up securities, in cash or otherwise, (3) make any demand for, or exercise any right with respect to, the registration of any lock-up securities, or (4) publicly disclose the intention to do any of the foregoing. Such persons or entities have further acknowledged that these undertakings preclude them from engaging in any hedging or other transactions or arrangements (including, without limitation, any short sale or the purchase or sale of, or entry into, any put or call option, or combination thereof, forward, swap or any other derivative transaction or instrument, however described or defined) designed or intended, or which could reasonably be expected to lead to or result in, a sale or disposition or transfer (by any person or entity, whether or not a signatory to such agreement) of any economic consequences of ownership, in whole or in part, directly or indirectly, of any lock-up securities, whether any such transaction or arrangement (or instrument provided for thereunder) would be settled by delivery of our common stock or other securities, in cash or otherwise.

The restrictions described in the immediately preceding paragraph and contained in the lock-up agreements between the underwriters and the lock-up parties do not apply, subject in certain cases to various conditions, to certain transactions, including (a) transfers or dispositions of lock-up securities (i) as bona fide gifts, or for bona fide estate planning purposes, (ii) by will, other testamentary document or intestacy, (iii) to any trust for the direct or indirect benefit of the lock-up party or any immediate family member, (iv) to a corporation, partnership, limited liability company, trust or other entity of which the lock-up party and/or one or more members of its immediate family members are the legal and beneficial owner of all of the outstanding equity securities or similar interests, (v) to a nominee or custodian of a person or entity to whom a disposition or transfer would be permissible under clauses (i) through (iv), (vi) in the case of a corporation, partnership, limited liability company, trust or other business entity, (A) to another corporation, partnership, limited liability company, trust or other business entity that is an affiliate of the lock-up party, or to any investment fund or other entity controlling, controlled by, managing or managed by or under common control with the lock-up party or its affiliates or (B) as part of a distribution or other transfer to general or limited partners, members or stockholders of, or other holders of equity in, the lock-up party; (vii) by operation of law, (viii) to us from an employee or other service provider upon death, disability or termination of employment or service relationship of such employee or service provider, (ix) as part of a sale of lock-up securities acquired in this offering (other than, in the case of one of our officers or directors, any securities such officer or director may purchase in this offering) or in open market transactions after the completion of this offering, (x) to us in connection with the vesting, settlement or exercise of restricted stock units, options, warrants or other rights to purchase shares of our common stock (including “net” or “cashless” exercise), including for the payment of exercise price and tax and remittance payments, or (xi) pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction approved by our board of directors and made to all stockholders involving a change in control, provided that if such transaction is not completed, all such lock-up securities would remain subject to the restrictions in the immediately preceding paragraph; (b) exercise of the options, settlement of RSUs or other equity awards granted pursuant to plans or other equity compensation arrangements or exercise warrants, in each case described in in this prospectus, provided that any lock-up securities received upon such exercise, vesting or settlement would be subject to restrictions similar to those in the immediately preceding paragraph; (c) the conversion of outstanding preferred stock, warrants to acquire preferred stock or convertible securities or warrants to acquire shares of our common stock into shares of our common stock, provided that any common stock or warrants received upon such conversion would be subject to restrictions similar to those in the immediately preceding paragraph; and (d) the establishment by lock-up parties of one or more trading plans under Rule 10b5-1 under the Exchange Act, provided that such plan does not provide for the transfer or disposition of lock-up securities during the restricted period and no filing by any party under the Exchange Act or other public announcement would be required or made voluntarily in connection with such trading plan.

J.P. Morgan Securities LLC and Jefferies LLC, in their sole discretion, may release the securities subject to any of the lock-up agreements with the underwriters described above, in whole or in part at any time.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act.

Our common stock has been approved for listing on the Nasdaq Global Select Market under the symbol “DYN.”

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be “covered” shorts, which are short positions in an amount not greater than the underwriters’ option to purchase additional shares referred to above, or may be “naked” shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on the Nasdaq Global Select Market, in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price was determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters considered a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;

- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common stock, or that the shares will trade in the public market at or above the initial public offering price.

Other relationships

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

Selling restrictions

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Notice to prospective investors in Canada

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

Notice to prospective investors in the European Economic Area and United Kingdom

In relation to each Member State of the European Economic Area and the United Kingdom, or each a "Relevant State," no shares have been offered or will be offered pursuant to this offering to the public in that Relevant State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation, except that offers of shares may be made to the public in that Relevant State at any time under the following exemptions under the Prospectus Regulation:

- to any legal entity which is a qualified investor as defined under the Prospectus Regulation;
- to fewer than 150 natural or legal persons (other than qualified investors as defined under the Prospectus Regulation), subject to obtaining the prior consent of the underwriters; or
- in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

provided that no such offer of shares shall require us or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation and each person who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the underwriters and the Company that it is a "qualified investor" within the meaning of Article 2(e) of the Prospectus Regulation. In the case of any shares being offered to a financial intermediary as that term is used in the Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Relevant State to qualified investors as so defined or in circumstances in which the prior consent of the underwriters have been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an "offer to the public" in relation to shares in any Relevant State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares, and the expression "Prospectus Regulation" means Regulation (EU) 2017/1129.

Notice to prospective investors in the United Kingdom

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are "qualified investors" (as defined in the Prospectus Regulation) (i) who have professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended or the Order, and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons") or otherwise in circumstances which have not resulted and will not result in an offer to the public of the shares in the United Kingdom within the meaning of the Financial Services and Markets Act 2000 as amended.

Any person in the United Kingdom that is not a relevant person should not act or rely on the information included in this document or use it as basis for taking any action. In the United Kingdom, any investment or investment activity that this document relates to may be made or taken exclusively by relevant persons.

Notice to prospective investors in Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX or on any other stock exchange or regulated trading facility in Switzerland. This document does not constitute a prospectus within the meaning of, and has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, the Company or the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Notice to prospective investors in the Dubai International Financial Centre

This document relates to an Exempt Offer in accordance with the Markets Rules 2012 of the Dubai Financial Services Authority, or DFSA. This document is intended for distribution only to persons of a type specified in the Markets Rules 2012 of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus supplement nor taken steps to verify the information set forth herein and has no responsibility for this document. The securities to which this document relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the securities offered should conduct their own due diligence on the securities. If you do not understand the contents of this document you should consult an authorized financial advisor.

In relation to its use in the Dubai International Financial Centre, or DIFC, this document is strictly private and confidential and is being distributed to a limited number of investors and must not be provided to any person other than the original recipient, and may not be reproduced or used for any other purpose. The interests in the securities may not be offered or sold directly or indirectly to the public in the DIFC.

Notice to prospective investors in the United Arab Emirates

The shares have not been, and are not being, publicly offered, sold, promoted or advertised in the United Arab Emirates (including the DIFC) other than in compliance with the laws of the United Arab Emirates (and the DIFC) governing the issue, offering and sale of securities. Further, this prospectus does not constitute a public offer of securities in the United Arab Emirates (including the DIFC) and is not intended to be a public offer. This prospectus has not been approved by or filed with the Central Bank of the United Arab Emirates, the Securities and Commodities Authority or the DFSA.

Notice to prospective investors in Australia

This prospectus:

- does not constitute a disclosure document or a prospectus under Chapter 6D.2 of the Corporations Act 2001, or the Corporations Act;

- has not been, and will not be, lodged with the Australian Securities and Investments Commission, or ASIC, as a disclosure document for the purposes of the Corporations Act and does not purport to include the information required of a disclosure document for the purposes of the Corporations Act; and
- may only be provided in Australia to select investors who are able to demonstrate that they fall within one or more of the categories of investors, available under section 708 of the Corporations Act, or Exempt Investors.

The shares may not be directly or indirectly offered for subscription or purchased or sold, and no invitations to subscribe for or buy the shares may be issued, and no draft or definitive offering memorandum, advertisement or other offering material relating to any shares may be distributed in Australia, except where disclosure to investors is not required under Chapter 6D of the Corporations Act or is otherwise in compliance with all applicable Australian laws and regulations. By submitting an application for the shares, you represent and warrant to us that you are an Exempt Investor.

As any offer of shares under this document will be made without disclosure in Australia under Chapter 6D.2 of the Corporations Act, the offer of those securities for resale in Australia within 12 months may, under section 707 of the Corporations Act, require disclosure to investors under Chapter 6D.2 if none of the exemptions in section 708 applies to that resale. By applying for the shares you undertake to us that you will not, for a period of 12 months from the date of issue of the shares, offer, transfer, assign or otherwise alienate those shares to investors in Australia except in circumstances where disclosure to investors is not required under Chapter 6D.2 of the Corporations Act or where a compliant disclosure document is prepared and lodged with ASIC.

Notice to prospective investors in Japan

The shares have not been and will not be registered pursuant to Article 4, Paragraph 1 of the Financial Instruments and Exchange Act. Accordingly, none of the shares nor any interest therein may be offered or sold, directly or indirectly, in Japan or to, or for the benefit of, any “resident” of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to or for the benefit of a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Act and any other applicable laws, regulations and ministerial guidelines of Japan in effect at the relevant time.

Notice to prospective investors in Hong Kong

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to “professional investors” as defined in the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong), or the SFO, of Hong Kong and any rules made thereunder; or (b) in other circumstances which do not result in the document being a “prospectus” as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong, or the CO or which do not constitute an offer to the public within the meaning of the CO. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to “professional investors” as defined in the SFO and any rules made thereunder.

Notice to prospective investors in Singapore

Each Joint Lead Manager has acknowledged that this prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, each Joint Lead Manager has represented and agreed that it

has not offered or sold any shares or caused the shares to be made the subject of an invitation for subscription or purchase and will not offer or sell any shares or cause the shares to be made the subject of an invitation for subscription or purchase, and has not circulated or distributed, nor will it circulate or distribute, this prospectus or any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares, whether directly or indirectly, to any person in Singapore other than:

(a) to an institutional investor (as defined in Section 4A of the Securities and Futures Act (Chapter 289) of Singapore, as modified or amended from time to time, or the SFA) pursuant to Section 274 of the SFA;

(b) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA; or

(c) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

(a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

(b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

securities or securities-based derivatives contracts (each term as defined in Section 2(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:

(i) to an institutional investor or to a relevant person, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;

(ii) where no consideration is or will be given for the transfer;

(iii) where the transfer is by operation of law;

(iv) as specified in Section 276(7) of the SFA; or

(v) as specified in Regulation 37A of the Securities and Futures (Offers of Investments) (Securities and Securities-based Derivatives Contracts) Regulations 2018.

Singapore SFA Product Classification—In connection with Section 309B of the SFA and the CMP Regulations 2018, unless otherwise specified before an offer of Notes, we have determined, and hereby notify all relevant persons (as defined in Section 309A(1) of the SFA), that the shares are "prescribed capital markets products" (as defined in the CMP Regulations 2018) and Excluded Investment Products (as defined in MAS Notice SFA 04-N12: Notice on the Sale of Investment Products and MAS Notice FAA-N16: Notice on Recommendations on Investment Products).

Notice to prospective investors in Bermuda

Shares may be offered or sold in Bermuda only in compliance with the provisions of the Investment Business Act of 2003 of Bermuda which regulates the sale of securities in Bermuda. Additionally, non-Bermudian persons (including companies) may not carry on or engage in any trade or business in Bermuda unless such persons are permitted to do so under applicable Bermuda legislation.

Notice to prospective investors in Saudi Arabia

This document may not be distributed in the Kingdom of Saudi Arabia except to such persons as are permitted under the Offers of Securities Regulations as issued by the board of the Saudi Arabian Capital Market Authority, or CMA pursuant to resolution number 2-11-2004 dated 4 October 2004 as amended by resolution number 1-28-2008, as amended. The CMA does not make any representation as to the accuracy or completeness of this document and expressly disclaims any liability whatsoever for any loss arising from, or incurred in reliance upon, any part of this document. Prospective purchasers of the securities offered hereby should conduct their own due diligence on the accuracy of the information relating to the securities. If you do not understand the contents of this document, you should consult an authorized financial adviser.

Notice to prospective investors in the British Virgin Islands

The shares are not being, and may not be offered to the public or to any person in the British Virgin Islands for purchase or subscription by or on our behalf. The shares may be offered to companies incorporated under the BVI Business Companies Act, 2004 (British Virgin Islands), or BVI Companies, but only where the offer will be made to, and received by, the relevant BVI Company entirely outside of the British Virgin Islands.

Notice to prospective investors in China

This prospectus will not be circulated or distributed in the PRC and the shares will not be offered or sold, and will not be offered or sold to any person for re-offering or resale directly or indirectly to any residents of the PRC except pursuant to any applicable laws and regulations of the PRC. Neither this prospectus nor any advertisement or other offering material may be distributed or published in the PRC, except under circumstances that will result in compliance with applicable laws and regulations.

Notice to prospective investors in Korea

The shares have not been and will not be registered under the Financial Investments Services and Capital Markets Act of Korea and the decrees and regulations thereunder, or the FSCMA, and the shares have been and will be offered in Korea as a private placement under the FSCMA. None of the shares may be offered, sold or delivered directly or indirectly, or offered or sold to any person for re-offering or resale, directly or indirectly, in Korea or to any resident of Korea except pursuant to the applicable laws and regulations of Korea, including the FSCMA and the Foreign Exchange Transaction Law of Korea and the decrees and regulations thereunder, or the FETL. The shares have not been listed on any of securities exchanges in the world including, without limitation, the Korea Exchange in Korea. Furthermore, the purchaser of the shares shall comply with all applicable regulatory requirements (including but not limited to requirements under the FETL) in connection with the purchase of the shares. By the purchase of the shares, the relevant holder thereof will be deemed to represent and warrant that if it is in Korea or is a resident of Korea, it purchased the shares pursuant to the applicable laws and regulations of Korea.

Notice to prospective investors in Malaysia

No prospectus or other offering material or document in connection with the offer and sale of the shares has been or will be registered with the Securities Commission of Malaysia, orCommission, for the Commission's approval pursuant to the Capital Markets and Services Act 2007. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Malaysia other than (i) a

closed end fund approved by the Commission; (ii) a holder of a Capital Markets Services Licence; (iii) a person who acquires the shares, as principal, if the offer is on terms that the shares may only be acquired at a consideration of not less than RM250,000 (or its equivalent in foreign currencies) for each transaction; (iv) an individual whose total net personal assets or total net joint assets with his or her spouse exceeds RM3 million (or its equivalent in foreign currencies), excluding the value of the primary residence of the individual; (v) an individual who has a gross annual income exceeding RM300,000 (or its equivalent in foreign currencies) per annum in the preceding twelve months; (vi) an individual who, jointly with his or her spouse, has a gross annual income of RM400,000 (or its equivalent in foreign currencies), per annum in the preceding twelve months; (vii) a corporation with total net assets exceeding RM10 million (or its equivalent in a foreign currencies) based on the last audited accounts; (viii) a partnership with total net assets exceeding RM10 million (or its equivalent in foreign currencies); (ix) a bank licensee or insurance licensee as defined in the Labuan Financial Services and Securities Act 2010; (x) an Islamic bank licensee or takaful licensee as defined in the Labuan Financial Services and Securities Act 2010; and (xi) any other person as may be specified by the Commission; provided that, in the each of the preceding categories (i) to (xi), the distribution of the shares is made by a holder of a Capital Markets Services Licence who carries on the business of dealing in securities. The distribution in Malaysia of this prospectus is subject to Malaysian laws. This prospectus does not constitute and may not be used for the purpose of public offering or an issue, offer for subscription or purchase, invitation to subscribe for or purchase any securities requiring the registration of a prospectus with the Commission under the Capital Markets and Services Act 2007.

Notice to prospective investors in Taiwan

The shares have not been and will not be registered with the Financial Supervisory Commission of Taiwan pursuant to relevant securities laws and regulations and may not be sold, issued or offered within Taiwan through a public offering or in circumstances which constitutes an offer within the meaning of the Securities and Exchange Act of Taiwan that requires a registration or approval of the Financial Supervisory Commission of Taiwan. No person or entity in Taiwan has been authorized to offer, sell, give advice regarding or otherwise intermediate the offering and sale of the shares in Taiwan.

Notice to prospective investors in South Africa

Due to restrictions under the securities laws of South Africa, no “offer to the public” (as such term is defined in the South African Companies Act, No. 71 of 2008 (as amended or re-enacted), or the South African Companies Act) is being made in connection with the issue of the shares in South Africa. Accordingly, this document does not, nor is it intended to, constitute a “registered prospectus” (as that term is defined in the South African Companies Act) prepared and registered under the South African Companies Act and has not been approved by, and/or filed with, the South African Companies and Intellectual Property Commission or any other regulatory authority in South Africa. The shares are not offered, and the offer shall not be transferred, sold, renounced or delivered, in South Africa or to a person with an address in South Africa, unless one or other of the following exemptions stipulated in section 96 (1) applies:

Section 96 (1) (a) the offer, transfer, sale, renunciation or delivery is to:

(i) persons whose ordinary business, or part of whose ordinary business, is to deal in securities, as principal or agent;

(ii) the South African Public Investment Corporation;

(iii) persons or entities regulated by the Reserve Bank of South Africa;

(iv) authorised financial service providers under South African law;

(v) financial institutions recognised as such under South African law;

(vi) a wholly-owned subsidiary of any person or entity contemplated in (c), (d) or (e), acting as agent in the capacity of an authorised portfolio manager for a pension fund, or as manager for a collective investment scheme (in each case duly registered as such under South African law); or

(vii) any combination of the person in (i) to (vi); or

Section 96 (1) (b) the total contemplated acquisition cost of the securities, for any single addressee acting as principal is equal to or greater than ZAR1,000,000 or such higher amount as may be promulgated by notice in the Government Gazette of South Africa pursuant to section 96(2)(a) of the South African Companies Act.

Information made available in this prospectus should not be considered as "*advice*" as defined in the South African Financial Advisory and Intermediary Services Act, 2002.

Legal matters

The validity of the shares of common stock offered hereby is being passed upon for us by Wilmer Cutler Pickering Hale and Dorr LLP, Boston, Massachusetts. Cooley LLP, Reston, Virginia, is acting as counsel for the underwriters in connection with this offering.

Experts

The financial statements as of December 31, 2018 and 2019 and for each of the two years in the period ended December 31, 2019 included in this prospectus have been audited by Deloitte & Touche LLP, an independent registered public accounting firm, as stated in their report appearing herein (which report expresses an unqualified opinion on the financial statements and includes an explanatory paragraph referring to the entity's ability to continue as a going concern). Such financial statements have been so included in reliance upon the report of such firm given upon their authority as experts in accounting and auditing.

Where you can find more information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock we are offering to sell. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement or the exhibits, schedules and amendments to the registration statement. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus about the contents of any contract, agreement or other document are not necessarily complete, and in each instance, we refer you to the copy of the contract, agreement or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference to such contract, agreement or document.

Upon completion of this offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at www.sec.gov. We also maintain a website at www.dyne-tx.com and upon completion of this offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and any amendments to those reports, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

Index to financial statements

Report of independent registered public accounting firm

To the stockholders and board of directors of Dyne Therapeutics, Inc.

Opinion on the financial statements

We have audited the accompanying balance sheets of Dyne Therapeutics, Inc. (the "Company") as of December 31, 2018 and 2019, and the related statements of operations, redeemable convertible preferred stock and stockholders' equity (deficit), and cash flows for the years then ended and the related notes to the financial statements (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2019, and the results of its operations and its cash flows for each of the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Going concern

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has suffered recurring losses from operations and recurring negative operating cash flows that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulation of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits, we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Deloitte & Touche LLP

Boston, Massachusetts
July 23, 2020 (September 9, 2020, as to the effects of the reverse stock split discussion in Note 15)

We have served as the Company's auditor since 2020.

Dyne Therapeutics, Inc.
Balance sheets
(in thousands, except share and per share data)

	December 31,		June 30,	Pro forma June 30,
	2018	2019	2020	2020
			(unaudited)	(unaudited)
Assets				
Current assets:				
Cash and cash equivalents	$ 8,124	$ 14,632	$ 11,672	$ 144,872
Prepaid expenses and other current assets	34	127	209	209
Total current assets	8,158	14,759	11,881	145,081
Property and equipment, net	110	1,486	1,499	1,499
Other assets	—	191	191	191
Total assets	$ 8,268	$ 16,436	$ 13,571	$ 146,771
Liabilities, Redeemable Convertible Preferred Stock and Stockholders' Equity (Deficit)				
Current liabilities:				
Accounts payable	$ 518	$ 1,256	$ 2,192	$ 2,192
Accrued expenses and other current liabilities	193	1,102	1,942	1,942
Total current liabilities	711	2,358	4,134	4,134
Long-term debt—net of unamortized debt discount	—	—	9,949	9,949
Preferred stock tranche obligations	3,375	—	—	—
Deferred rent	—	42	27	27
Success fee obligation	—	—	180	180
Total liabilities	4,086	2,400	14,290	14,290
Redeemable convertible preferred stock (Note 8)	9,061	—	—	—
Commitments and contingencies (Note 13)				
Stockholders' equity (deficit)				
Convertible preferred stock (Note 8)	—	27,429	29,401	—
Common stock, $0.0001 par value; 68,000,000, 68,000,000 and 70,000,000 shares authorized at December 31, 2018 and 2019 and June 30, 2020 (unaudited), respectively; 3,252,140, 3,239,017 and 3,239,770 shares issued at December 31, 2018 and 2019 and June 30, 2020 (unaudited), respectively; 2,110,404, 2,586,535 and 2,728,365 shares outstanding at December 31, 2018 and 2019 and June 30, 2020 (unaudited), respectively; 131,408,815 shares authorized, 31,326,145 shares issued and 30,814,740 shares outstanding at June 30, 2020, pro forma (unaudited)	1	1	1	3
Additional paid-in capital	7	6,352	6,493	169,092
Accumulated deficit	(4,887)	(19,746)	(36,614)	(36,614)
Total stockholders' equity (deficit)	(4,879)	14,036	(719)	132,481
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 8,268	$ 16,436	$ 13,571	$ 146,771

The accompanying notes are an integral part of these financial statements.

Dyne Therapeutics, Inc.
Statements of operations
(in thousands, except share and per share data)

	Year ended December 31,		Six months ended June 30,	
	2018	2019	2019	2020
				(unaudited)
Operating expenses:				
Research and development	$ 4,278	$ 11,040	$ 3,799	$ 13,423
General and administrative	517	2,786	927	3,105
Total operating expenses	4,795	13,826	4,726	16,528
Loss from operations	(4,795)	(13,826)	(4,726)	(16,528)
Other (expense) income:				
Interest income	5	290	113	24
Interest expense	—	—	—	(184)
Change in fair value of preferred stock tranche obligations	(21)	(1,323)	1,029	—
Change in success fee obligation	—	—	—	(180)
Total other (expense) income, net	(16)	(1,033)	1,142	(340)
Net loss	$ (4,811)	$ (14,859)	$ (3,584)	$ (16,868)
Net loss per share—basic and diluted	$ (10.15)	$ (6.08)	$ (1.52)	$ (6.31)
Weighted-average common shares outstanding used in net loss per share—basic and diluted	474,118	2,442,872	2,355,066	2,675,260
Pro forma net loss per share—basic and diluted (unaudited)		$ (1.31)		$ (1.32)
Pro forma weighted-average number of common shares used in computing pro forma net loss per share—basic and diluted (unaudited)		10,324,875		12,818,114

The accompanying notes are an integral part of these financial statements.

Dyne Therapeutics, Inc.
Statements of redeemable convertible preferred stock and stockholders' equity (deficit)
(in thousands, except share data)

	Redeemable convertible preferred stock		Convertible preferred stock		Common stock		Additional paid-in capital	Accumulated deficit	Stockholders' equity (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at January 1, 2018	—	$ —	—	$ —	—	$ —	$ —	$ (76)	$ (76)
Common stock issuance	—	—	—	—	2,110,404	1	6	—	7
Issuance of Series A redeemable convertible preferred stock, net of issuance costs of $0.1 million	12,500,000	9,061	—	—	—	—	—	—	—
Stock-based compensation	—	—	—	—	—	—	1	—	1
Net loss	—	—	—	—	—	—	—	(4,811)	(4,811)
Balance at December 31, 2018	12,500,000	9,061	—	—	2,110,404	1	7	(4,887)	(4,879)
Issuance of Series A redeemable convertible preferred stock, net of issuance costs of $0.1 million	20,000,000	19,989	—	—	—	—	—	—	—
Settlement of Tranche Right I	—	(1,621)	—	—	—	—	—	—	—
Reclassification of redeemable convertible preferred stock and preferred stock tranche obligations	(32,500,000)	(27,429)	32,500,000	27,429	—	—	6,319	—	33,748
Stock-based compensation	—	—	—	—	—	—	26	—	26
Vesting of restricted shares	—	—	—	—	476,131	—	—	—	—
Net loss	—	—	—	—	—	—	—	(14,859)	(14,859)
Balance at December 31, 2019	—	—	32,500,000	27,429	2,586,535	1	6,352	(19,746)	14,036
Issuance of Series A convertible preferred stock, net of issuance costs of $0.1 million	—	—	2,000,000	1,972	—	—	—	—	1,972
Exercise of stock options	—	—	—	—	753	—	—	—	—
Stock-based compensation	—	—	—	—	—	—	141	—	141
Vesting of restricted shares	—	—	—	—	141,077	—	—	—	—
Net loss	—	—	—	—	—	—	—	(16,868)	(16,868)
Balance at June 30, 2020 (unaudited)	—	$ —	34,500,000	$29,401	2,728,365	$ 1	$ 6,493	$ (36,614)	$ (719)

The accompanying notes are an integral part of these financial statements.

Dyne Therapeutics, Inc.
Statements of cash flows
(in thousands)

	Year ended December 31,		Six months ended June 30,	
	2018	2019	2019	2020
				(unaudited)
Cash flows from operating activities:				
Net loss	$ (4,811)	$ (14,859)	$ (3,584)	$ (16,868)
Adjustments to reconcile net loss to net cash used in operating activities:				
Stock-based compensation expense	1	26	5	141
Depreciation and amortization expense	24	271	22	308
Changes in fair value of preferred stock tranche obligations	21	1,323	(1,029)	—
Changes in success fee obligation	—	—	—	180
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(34)	(284)	(324)	(82)
Accounts payable and other liabilities	635	1,689	66	1,722
Net cash used in operating activities	(4,164)	(11,834)	(4,844)	(14,599)
Cash flows from investing activities:				
Purchases of property and equipment	(134)	(1,647)	(181)	(282)
Net cash used in investing activities	(134)	(1,647)	(181)	(282)
Cash flows from financing activities:				
Proceeds from issuance of common stock	7	—	—	—
Proceeds from issuance of debt, net of issuance costs	—	—	—	9,949
Proceeds from SAFEs (Note 7)	5,000	—	—	—
Proceeds from issuance of preferred stock, net of issuance costs	7,415	19,989	19,989	1,972
Net cash provided by financing activities	12,422	19,989	19,989	11,921
Net increase (decrease) in cash and cash equivalents	8,124	6,508	14,964	(2,960)
Cash and cash equivalents at beginning of period	—	8,124	8,124	14,632
Cash and cash equivalents at end of period	$ 8,124	$ 14,632	$ 23,088	$ 11,672
Supplemental cash flow information:				
Cash paid for interest and taxes	$ —	$ —	$ —	$ 183
Supplemental disclosure of non-cash investing and financing information:				
Reclassification of preferred stock tranche obligation liability to permanent equity	$ —	$ 6,319	$ —	$ —
Settlement of SAFEs with the issuance of Series A Preferred Stock	$ 5,000	$ —	$ —	$ —
Purchase of property and equipment in accounts payable	$ —	$ —	$ 14	$ 39
Settlement of Tranche Right I	$ —	$ 1,621	$ 1,621	$ —

The accompanying notes are an integral part of these financial statements.

Dyne Therapeutics, Inc.
Notes to financial statements

1. Nature of business and basis of presentation

Nature of business

Dyne Therapeutics, Inc. (the "Company") is a healthcare company that was incorporated in Delaware on December 1, 2017 and has a principal place of business in Waltham, Massachusetts. The Company's focus is on advancing innovative life-transforming therapies for genetically driven muscle diseases.

Risks, uncertainties and going concern

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, successful development of technology, obtaining additional funding, protection of proprietary technology, compliance with government regulations, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for its product candidates, fluctuations in operating results, dependence on key personnel, risks associated with changes in technologies and development by competitors of technological innovations.

To date, the Company has principally raised capital through the private placement of convertible preferred stock. The Company has incurred recurring losses since its inception, including net losses of $14.9 million for the year ended December 31, 2019 and $16.9 million for the six months ended June 30, 2020 (unaudited). In addition, as of December 31, 2019 and June 30, 2020 (unaudited), the Company had an accumulated deficit of $19.7 million and $36.6 million, respectively. The Company expects to incur additional losses and negative operating cash flows at least for the development period and possibly into the commercialization stage. Based on its recurring losses from operations incurred since inception, expectation of continuing operating losses for the foreseeable future, and need to raise additional capital to finance its future operations, the Company has concluded that there is substantial doubt regarding the Company's ability to continue as a going concern within one year after the date that these financial statements are issued.

The Company will require substantial additional capital to fund its research and development and ongoing operating expenses. These capital requirements are expected to be funded through debt and equity offerings as well as possible strategic collaborations with other companies. If the Company is unable to raise additional funds when needed, it may be required to delay, reduce or eliminate its product development or future commercialization efforts, or grant rights to develop and market product candidates that the Company would otherwise prefer to develop and market itself. While there can be no assurance the Company will be able to reduce operating expenses or raise additional capital, management believes its historical success in managing cash flows and obtaining capital will continue in the foreseeable future.

The Company is seeking to complete an initial public offering ("IPO") of its common stock. Upon the completion of an IPO on specified terms, the Company's outstanding convertible preferred stock will automatically convert into shares of common stock (see Note 8). In the event the Company does not complete an IPO, the Company expects to seek additional funding through private equity financings, debt financings or other capital sources, including collaborations with other companies or other strategic transactions. The Company may not be able to obtain funding on acceptable terms, or at all. The terms of any future financing may adversely affect the holdings or the rights of the Company's existing stockholders.

The accompanying financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. These financial statements do not include any adjustments that might result from the outcome of this uncertainty.

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COVID-19

In March 2020, the spread of the novel coronavirus began to cause business disruptions for the Company and many of the Company's vendors. In addition, the Company has taken a series of actions aimed at safeguarding the Company's employees and business associates, including implementing a work-at-home policy. These disruptions could result in increased costs of execution of development plans or may negatively impact the quality, quantity, timing and regulatory usability of data that the Company would otherwise be able to collect. While these disruptions are currently expected to be temporary, there is considerable uncertainty around the duration of these disruptions. Therefore, the related financial impact and duration cannot be reasonably estimated at this time.

Basis of presentation

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("U.S. GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative U.S. GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Updates ("ASUs") issued by the Financial Accounting Standards Board ("FASB").

2. Summary of significant accounting policies

Use of estimates

The preparation of the financial statements in accordance with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Actual results could differ from those estimates.

Unaudited interim financial information

The accompanying balance sheet as of June 30, 2020, the statements of operations and of cash flows for the six months ended June 30, 2019 and 2020, and the statement of convertible preferred stock and stockholders' equity (deficit) for the six months ended June 30, 2020 are unaudited. The unaudited interim financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of June 30, 2020, the results of its operations and its cash flows for the six months ended June 30, 2019 and 2020 and the changes in its convertible preferred stock and stockholders' equity (deficit) for the six months ended June 30, 2020. The financial data and other information disclosed in these notes related to the six months ended June 30, 2019 and 2020 are also unaudited. The results for the six months ended June 30, 2020 are not necessarily indicative of results to be expected for the year ending December 31, 2020, any other interim periods, or any future year or period.

Unaudited pro forma information

The accompanying unaudited pro forma balance sheet as of June 30, 2020 has been prepared to give effect to (i) the sale of 17,500,000 shares of Series A Preferred Stock in July 2020 at a price of $1.00 per share, for gross proceeds of $17.5 million (see Note 15), (ii) the sale of 41,159,724 shares of Series B Preferred Stock in August

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2020 at a price of $2.81 per share, for gross proceeds of $115.7 million (see Note 15), and (iii) upon the closing of the proposed IPO, the conversion of all outstanding shares of convertible preferred stock into 28,086,375 shares of common stock as if the conversion due to the proposed IPO had occurred on June 30, 2020.

In the accompanying statements of operations, the unaudited pro forma net loss per share for the year ended December 31, 2019 and six months ended June 30, 2020 has been computed using the weighted-average number of common shares outstanding after giving pro forma effect to the conversion of all outstanding shares of convertible preferred stock into 10,401,270 shares of common stock as if the conversion due to the proposed IPO had occurred on the later of January 1, 2019 or the issuance date of the convertible preferred stock. Additionally, the changes in the fair value of the preferred stock tranche obligations have been removed from the determination of pro forma net loss per share as these obligations and the changes in fair value would not be recognized after the preferred stock has been converted to common stock. This calculation excludes the sale by us of 17,500,000 shares of Series A Preferred Stock in July 2020 and 41,159,724 shares of Series B Preferred Stock in August 2020, which will convert into 17,685,105 shares of common stock upon the closing of the proposed IPO.

Cash and cash equivalents

Cash includes cash in readily available checking accounts and cash equivalents include all highly liquid investments maturing within 90 days from the date of purchase. Financial instruments that subject the Company to significant concentrations of credit risk consist primarily of cash and cash equivalents as the Company's cash deposits on hand at one financial institution often exceed federally insured limits. The Company places its cash in a financial institution that management believes to be of high credit quality.

Property and equipment

Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization expense is recognized using the straight-line method over the estimated useful life of each asset as follows:

	Estimated useful life
Laboratory equipment	3 years
Furnitures and fixtures	5 years
Computer equipment	3 years
Leasehold improvements	Shorter of life of lease or 10 years

Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance are charged to expense as incurred.

Impairment of long-lived assets

Long-lived assets are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amount of the asset may not be recoverable. When such events occur, the Company compares the carrying amounts of the assets to their undiscounted expected future cash flows. If this comparison indicates

that there is an impairment, the amount of the impairment is calculated as the difference between the carrying value and the fair value. The Company has not recorded any impairment charges in the periods presented in these financial statements.

Segment information

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker, or decision-making group, in making decisions on how to allocate resources and assess performance. The Company's chief operating decision maker is the chief executive officer ("CEO"). The CEO and other members of senior management of the Company view the Company's operations and manage its business as one operating segment.

Research and development costs

Research and development costs are expensed as incurred. Research and development costs that are paid in advance of performance (if any) are capitalized as a prepaid expense and amortized over the service period as the services are provided.

Income taxes

Deferred tax assets and liabilities are recognized for the expected future tax consequences of events that have been included in the financial statements or tax returns. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is provided to reduce the deferred tax asset to an amount, which, more likely than not, will be realized.

The Company recognizes the tax benefit from any uncertain tax positions only if it is more likely than not that the tax position will be sustained on examination by the taxing authorities, based on the technical merits of the position. The tax benefits recognized in the financial statements from such a position are measured based on the largest benefit that has a greater than fifty percent likelihood of being realized upon ultimate settlement.

Fair value measurements

Certain assets and liabilities are carried at fair value. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Unadjusted quoted prices in active markets that are accessible to the reporting entity at the measurement date for identical assets and liabilities.

Dyne Therapeutics, Inc.
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- Level 2—Inputs other than quoted prices in active markets for identical assets and liabilities that are observable either directly or indirectly for substantially the full term of the asset or liability. Level 2 inputs include the following:
    - quoted prices for similar assets and liabilities in active markets;
    - quoted prices for identical or similar assets or liabilities in markets that are not active;
    - observable inputs other than quoted prices that are used in the valuation of the asset or liabilities (e.g., interest rate and yield curve quotes at commonly quoted intervals); and
    - inputs that are derived principally from or corroborated by observable market data by correlation or other means.
- Level 3—Unobservable inputs for the assets or liability (i.e., supported by little or no market activity). Level 3 inputs include management's own assumptions about the assumptions that market participants would use in pricing the asset or liability (including assumptions about risk).

Net loss per share

The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed. During periods of loss, the Company does not allocate loss to participating securities because they have no contractual obligation to share in the losses of the Company.

Basic net loss per share is computed by dividing the net income loss by the weighted average number of shares of common stock outstanding for the period. Diluted net loss is computed by adjusting net loss to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share is computed by dividing the diluted net loss by the weighted average number of shares of common stock outstanding for the period, including potential dilutive common shares assuming the dilutive effect of common stock equivalents.

Redeemable convertible preferred stock

The Company recorded redeemable convertible preferred stock at fair value upon issuance, net of any issuance costs. The Company's redeemable convertible preferred stock was subject to a dividend when, as and if declared by the Company's board of directors (the "Board"). Since the issuance of the Company's outstanding redeemable convertible preferred stock, no dividends have been declared on any shares of redeemable convertible preferred stock. The Company classified stock that was redeemable in circumstances outside of the Company's control outside of permanent equity. No accretion was recognized as the contingent events that could give rise to redemption were not deemed probable.

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Stock-based compensation

The Company accounts for stock-based awards at fair value, and measures fair value using the Black-Scholes option-pricing model. Stock-based compensation costs are recognized as expense over the requisite service period, which is generally the vesting period, on a straight-line basis for all time-vested awards. Forfeitures are recognized as they occur.

Success fee obligation

The Company's loan and security agreement (the "Loan Agreement") with Pacific Western Bank ("PWB") entered into in 2020 requires the Company to pay a success fee of $0.5 million upon the occurrence of a specified liquidity event, as described in the Loan Agreement, which includes the proposed IPO. The Company classifies this contingent obligation to pay a success fee as a liability on its balance sheet. The liability was initially deemed immaterial upon entering into the Loan Agreement and is subsequently remeasured to fair value at each reporting date. Changes in the success fee obligation will continue to be recognized until the liability is settled.

Comprehensive loss

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. The Company's comprehensive net loss equals the reported net loss for all periods presented.

Accounting pronouncements issued and not adopted

In February 2016, the FASB issued ASU No. 2016-02, *Leases* ("ASU 2016-02"), which applies to all leases and will require lessees to record most leases on the balance sheet but recognize expense in a manner similar to the previous standard. The Company will use a modified retrospective approach of adoption for its leases. The Company plans to adopt this standard on January 1, 2022 and is currently evaluating the impact that the adoption will have on its financial statements. The Company expects to recognize a lease obligation and right to use asset upon adoption of ASU 2016-02.

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3. Fair value measurements

The following tables set forth by level, within the fair value hierarchy (see Note 2), the assets and liabilities carried at fair value on a recurring basis for the periods presented:

	Fair value measurements as of December 31, 2018			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents—money market funds	$ 7,005	—	—	$ 7,005
Total	$ 7,005	—	—	$ 7,005
Liabilities:				
Preferred stock tranche obligations	$ —	—	$ 3,375	$ 3,375
Total	$ —	—	$ 3,375	$ 3,375

	Fair value measurements as of December 31, 2019			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents—money market funds	$ 14,420	—	—	$ 14,420
Total	$ 14,420	—	—	$ 14,420

	Fair value measurements as of June 30, 2020 (unaudited)			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Liabilities:				
Success fee obligation	$ —	—	$ 180	$ 180
Total	$ —	—	$ 180	$ 180

Money market funds were valued by the Company based on quoted market prices. There were no transfers among Level 1, Level 2, or Level 3 categories during the periods presented.

The fair value of the preferred stock tranche obligations in connection with the unfunded tranches associated with the Series A Preferred Stock Purchase Agreement (see Note 8) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of the preferred stock tranche obligations was determined by considering as inputs the future value of the Series A Preferred Stock, the difference between the future value of the Series A Preferred Stock and the contract price, the number of shares underlying the contract, the probability of achieving the funding of the tranches, expected timing of the tranche closing and the discount rate. The future value of Series A Preferred Stock was estimated by calibrating, or backsolving, to the price of the Series A Preferred Stock as set forth in the Series A Preferred Stock Purchase Agreement. The contractual price of the shares to be issued in addition to the number of shares underlying the contract are both provided for by the Series A Preferred Stock Purchase Agreement. The probability of achieving the funding of the tranches and the expected timing of those closings were estimates made by the Company. The discount rate was equal to the risk-free rate for the estimated timing of each tranche closing.

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The fair value of the liability recognized in connection with the contingent success fee associated with the Loan Agreement (see Note 6) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of the liability was determined using the probability-weighted expected return method ("PWERM"), which considered as inputs the probability of occurrence of a specified liquidity event, the expected timing of a liquidity event, the amount of the success fee and a risk-adjusted discount rate. As of June 30, 2020, the assumed probability of occurrence of the event that was most probable of triggering the payment was 40%, the expected timing of such an event was estimated to be less than one year (0% discount rate) and the amount of the success fee was $0.5 million. Based on these inputs, the Company determined that the fair value of the liability was $0.2 million at June 30, 2020 (unaudited).

The following table presents a roll-forward of the aggregate fair values of the Company's liabilities for which fair value is determined by Level 3 inputs:

(in thousands)	Preferred stock tranche obligations	Success fee obligation
Balance—January 1, 2018	$ —	$ —
Initial fair value of preferred stock tranche obligations	3,354	—
Change in fair value	21	—
Balance—December 31, 2018	3,375	—
Settlement of Tranche Right I	1,621	—
Change in fair value	1,323	—
Reclassification of Tranche Right II to equity	(6,319)	—
Balance—December 31, 2019	—	—
Initial fair value of success fee obligation	—	—
Change in fair value	—	180
Balance—June 30, 2020 (unaudited)	$ —	$ 180

Financial instruments not recorded at fair value

The carrying values of cash, cash equivalents, accounts payable and accrued expenses that are reported on the balance sheets approximate their fair value due to the short-term nature of these assets and liabilities. The carrying value of the long-term debt at June 30, 2020 (unaudited) approximates fair value given that the debt was recently issued.

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4. Property and equipment

Property and equipment consisted of the following:

(in thousands)	December 31, 2018	December 31, 2019
Laboratory equipment	$ 134	$1,705
Furnitures and fixtures	—	41
Computer equipment	—	20
Leasehold improvements	—	14
Property and equipment—at cost	134	1,781
Less accumulated depreciation and amortization	(24)	(295)
Property and equipment—net	$ 110	$1,486

Depreciation expense totaled $24,000 and $0.3 million for the years ended December 31, 2018 and 2019, respectively. Depreciation expense totaled $22,000 and $0.3 million for the six months ended June 30, 2019 and 2020 (unaudited), respectively.

5. Accrued expenses and other current liabilities

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	December 31, 2018	December 31, 2019	June 30, 2020 (unaudited)
Payroll and benefits	$184	$ 540	$ 624
Consulting services	—	60	38
Legal services	—	—	202
Research and development	8	484	996
Facility costs	—	18	30
Other	1	—	52
Total	$193	$1,102	$ 1,942

6. Debt financing

On February 20, 2020, the Company entered into the Loan Agreement with PWB pursuant to which PWB made term loans to the Company in an aggregate principal amount of $10.0 million. Borrowings under the Loan Agreement are collateralized by substantially all of the Company's assets, excluding intellectual property.

Interest on the outstanding loan balance will accrue at a variable annual rate equal to the greater of (i) PWB's prime rate plus 0.25% and (ii) 5.00%. The interest rate was 5.00% at June 30, 2020. The Company is required to make interest-only payments on the loans on a monthly basis through February 2021. Upon receipt of at least $17.5 million of gross cash proceeds from the closing of the third tranche of the Series A Preferred Stock, interest-only payments will be extended until August 2021. Subsequent to the interest-only periods, the Company will be required to make equal monthly payments of principal plus interest until the loan matures in

February 2024. The Company incurred fees associated with establishing the facility of $0.1 million. The Company has an option to prepay the loan in full without a fee. In the event of a specified liquidity event, including the proposed IPO, the Company will be required to pay a success fee of $0.5 million. The fair value of the success fee and the transaction fees created a debt discount, which will be amortized over the facility term. The Loan Agreement contains customary representations, warranties and covenants and also includes customary events of default, including payment defaults, breaches of covenants, change of control and occurrence of a material adverse effect. There are no financial covenants associated with the Loan Agreement.

The scheduled principal maturity of the amounts borrowed under the Loan Agreement is $2.8 million in 2021, $3.3 million in 2022, $3.3 million in 2023 and $0.6 million in 2024.

7. Simple agreement for future equity

In January 2018, June 2018 and October 2018, the Company entered into simple agreements for future equity (the “SAFEs”) with an investor, receiving $5.0 million of gross proceeds in aggregate in exchange for the investor’s right to participate in a future equity financing. The SAFEs contained a number of conversion and redemption provisions, including settlement upon liquidity or dissolution events. In November 2018, the investor exercised its right to convert the SAFEs in connection with the Company’s equity financing (See Note 8) and exchanged the SAFEs for an aggregate of 5,000,000 shares of Series A Redeemable Convertible Preferred Stock, with a fair value of $5.0 million at issuance.

8. Redeemable convertible preferred stock

Series A preferred stock

On November 29, 2018, the Company entered into the Series A Preferred Stock Purchase Agreement with its initial investors committing to purchase an aggregate of $50.0 million in shares of Series A Preferred Stock. At the initial closing, 12,500,000 shares of Series A Preferred Stock were issued by the Company at a purchase price of $1.00 per share, for gross cash proceeds of $7.5 million. Of the 12,500,000 shares issued, 5,000,000 shares were issued upon conversion of the then outstanding SAFEs, which had a fair value of $5.0 million (See Note 7). In April 2019, the Company issued an additional 20,000,000 shares of Series A Preferred Stock under the terms of the Series A Preferred Stock Purchase Agreement at a purchase price of $1.00 per share for total gross proceeds of $20.0 million. Issuance costs associated with each closing of the Series A Preferred Stock financing were $0.1 million. On March 18, 2020, the Company entered into a separate Series A Preferred Stock Purchase Agreement with an additional investor and issued 2,000,000 shares of Series A Preferred Stock at a purchase price $1.00 per share for gross cash proceeds of $2.0 million.

Included in the terms of the November 2018 Series A Preferred Stock Purchase Agreement were certain rights (“Tranche Rights”) granted to the investors who purchased the Series A Preferred Stock purchased in November 2018. The Tranche Rights contingently obligated the investors to purchase, and the Company to sell, up to an aggregate of 37,500,000 shares of Series A Preferred Stock at $1.00 per share upon the satisfaction of specified research and development milestones by the Company. The Tranche Rights are also exercisable at the option of the holders of the Series A Preferred Stock. The Tranche Rights were divided into separate rights and obligations to purchase 20,000,000 shares (“Tranche Right I”) and 17,500,000 shares (“Tranche Right II”) based on the achievement of specified milestones for each tranche.

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The Company concluded that the Tranche Rights met the definition of a freestanding financial instrument, as the Tranche Rights were legally detachable and separately exercisable from the Series A Preferred Stock. Therefore, the Company allocated the proceeds from the November 2018 issuance between the Tranche Rights and the Series A Preferred Stock. As the Series A Preferred Stock was redeemable upon a deemed liquidation event at the election of the holder-controlled Board, and therefore outside of the control of the Company, the Tranche Rights were initially classified as a liability and were initially recorded at their fair value of $3.4 million. The Tranche Rights were then remeasured at fair value at each reporting period, with changes in fair value recorded in the statement of operations. The estimated fair value of the Tranche Rights was determined at each reporting date using a probability-weighted present value model that considers the probability of closing a tranche, the estimated future value of the Series A Preferred Stock to be issued at each closing, and the investment required at each closing. Future values were converted to present value using a discount rate appropriate for probability-adjusted cash flows.

In April 2019, Tranche Right I was settled when the Company closed on the issuance of 20,000,000 shares Series A Preferred Stock. The fair value of the Tranche Right I as of the closing date was reclassified from a liability to Series A Preferred Stock within equity.

In September 2019, the size of the Board was increased from five to six members, including three independent members, which allowed the Company to conclude that the redemption rights of the holders of the Series A Preferred Stock was within the control of the Company. As a result, the Company concluded that Tranche Right II qualified for equity classification as of that date. The preferred stock tranche obligation liability, with a fair value of $6.3 million, was reclassified into permanent equity and was no longer remeasured at fair value at each reporting period after that date.

As of each balance sheet date, convertible preferred stock consisted of the following:

	As of December 31, 2018				
(in thousands, except share data)	Preferred stock authorized	Preferred stock issued and outstanding	Carrying value	Liquidation preference	Common shares issuable upon conversion
Series A Preferred Stock	50,000,000	12,500,000	$ 9,061	$ 12,500	3,768,575
Total	50,000,000	12,500,000	$ 9,061	$ 12,500	3,768,575

	As of December 31, 2019				
(in thousands, except share data)	Preferred stock authorized	Preferred stock issued and outstanding	Carrying value	Liquidation preference	Common shares issuable upon conversion
Series A Preferred Stock	50,000,000	32,500,000	$27,429	$ 32,500	9,798,298
Total	50,000,000	32,500,000	$27,429	$ 32,500	9,798,298

	As of June 30, 2020 (unaudited)				
(in thousands, except share data)	Preferred stock authorized	Preferred stock Issued and outstanding	Carrying value	Liquidation preference	Common shares issuable upon conversion
Series A Preferred Stock	52,000,000	34,500,000	$29,401	$ 34,500	10,401,270
Total	52,000,000	34,500,000	$29,401	$ 34,500	10,401,270

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The Series A Preferred Stock has the followings rights and privileges:

Dividends

Holders of the Series A Preferred Stock are entitled to receive non-cumulative dividends when, as and if declared by the Board at a rate of 6% of the Original Issue Price (as defined below) per share (the “Dividend Rate”), subject to adjustment. Holders of Series A Preferred Stock will have preference to any dividends being declared or paid on common stock. The Company has not, and has no plans to, declare dividends on any class of preferred or common stock.

Liquidation

In the event of any liquidation, dissolution, or winding-up of the Company, which would include the sale of the Company, the Series A Preferred Stock is senior to common stock. The holders of the Series A Preferred Stock would be entitled to preferential payment in the amount of $1.00 per share (the “Original Issue Price”). In the event that there are additional assets to be distributed, the holders of the Series A Preferred Stock will share in the distribution along with common stockholders as if the shares of Series A Preferred Stock had converted to common stock immediately prior to the distribution. If the amounts to be distributed to the holders of the Series A Preferred Stock exceeds $2.00 per share, subject to certain adjustments, then the holders of the Series A Preferred Stock will receive the greater of $2.00 or the amount such holder would have received if all shares of Series A Preferred Stock had converted to common stock immediately prior to the distribution.

Voting

The holders of the Series A Preferred Stock are entitled to the number of votes equal to the number of shares of common stock into which the shares of Series A Preferred Stock held by each holder are then convertible.

Conversion

The holders of the Series A Preferred Stock may convert, at any time, each share of Series A Preferred Stock into shares of common stock at the Series A Conversion Price. The Series A Conversion Price is initially equal to the Original Issue Price, subject to adjustment. Upon either (a) the closing of the sale of shares of common stock to the public at a price of at least $3.00 per share (subject to adjustment) in an initial public offering with net proceeds to the Company of at least $50.0 million (“Qualified IPO”) or (b) the written consent of the holders of a majority of the outstanding shares of Series A Preferred Stock, the Series A Preferred Stock will automatically convert into common stock at the Series A Conversion Price then in effect.

In the event that a holder of Series A Preferred Stock does not purchase the shares of Series A Preferred Stock that the holder is obligated to purchase pursuant to Tranche Right II, that holder will have its outstanding shares of Series A Preferred Stock automatically converted into common stock at a conversion price of $2.00 per share, meaning that the holder would receive 50% fewer shares than would be received upon an optional or mandatory conversion as set forth in the preceding paragraph.

9. Common stock

As of December 31, 2018 and 2019, the Company had authorized 68,000,000 shares of common stock. As of and June 30, 2020 (unaudited), the Company had authorized 70,000,000 shares of common stock. The voting,

dividend and liquidation rights of the holders of the Company's common stock is subject to and qualified by the rights, powers and preferences of the holders of the Series A Preferred Stock as set forth above.

As of December 31, 2018 and 2019 and June 30, 2020 (unaudited), the Company had reserved an aggregate of 5,737,103, 11,290,676 and 12,957,749 shares of common stock, respectively, for the conversion of outstanding shares of Series A Preferred Stock, the exercise of outstanding stock options, the vesting of restricted shares of common stock and the potential issuance of shares available for grant under the Company's 2018 Stock Incentive Plan (see Note 10).

10. Stock-based compensation

2018 Stock incentive plan

During 2018, the Company adopted the 2018 Stock Incentive Plan (the "2018 Plan"). The 2018 Plan, as amended, provided for the issuance of up to 1,968,528 shares of common stock as of December 31, 2019 and up to 3,174,473 shares of common stock as of June 30, 2020 (unaudited) to employees, officers, directors, consultants, and advisors in the form of nonqualified and incentive stock options, restricted stock awards, and other stock-based awards. Options typically vest over four years and have a maximum term of 10 years. There were no awards granted during the year ended December 31, 2018. At December 31, 2019 and June 30, 2020 (unaudited), there were 528,593 shares and 288,220 shares, respectively, of common stock available for issuance under the 2018 Plan.

Stock option valuation

The Company typically grants stock options to employees and nonemployees at exercise prices deemed to be equal to the fair value of the common stock at the time of grant. The fair value of the common stock has been determined by the Board at each measurement date based on a variety of different factors, including the results obtained from independent third-party appraisals, the Company's financial position and historical financial performance, the status of development of the Company's programs, the current climate in the marketplace, the illiquid nature of the common stock, the effect of the rights and preferences of the holders of Series A Preferred Stock, and the prospects of a liquidity event, among others.

The Company utilized the Black-Scholes option-pricing model to estimate the fair value of stock options awarded. The Black-Scholes option-pricing model requires several key assumptions. The assumptions that the Company used to determine the grant date fair value of options granted were as follows:

	Year ended December 31, 2019	Six months ended June 30, 2020 (unaudited)
Expected volatility	74%	75%
Risk-free interest rate	1.46% — 2.50%	0.45% — 1.67%
Expected term (in years)	6	6
Expected dividend yield	—	—

The risk-free interest rates are based on rates associated with U.S. Treasury issues approximating the expected life of the stock options. The expected term of options granted to employees was determined using the simplified method, which represents the midpoint of the contractual term of the option and the weighted-

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average vesting period of the option. The Company uses the simplified method because it does not have sufficient historical option exercise data to provide a reasonable basis upon which to estimate the expected term. The expected dividend-yield assumption was based on the Company's expectation of no future dividend payments. The expected volatility of the underlying stock was based on the average historical volatility of comparable publicly traded companies based on weekly price returns as reported by a pricing service, as the Company does not have a trading history for its stock.

The weighted-average grant date fair value of the options granted during the year ended December 31, 2019 was $0.62 per share. The weighted-average grant date fair value of the options granted during the six months ended June 30, 2020 (unaudited) was $0.71 per share. As of December 31, 2019, there was $0.2 million of unrecognized compensation expense which will be recognized over a weighted-average period of 3.4 years. As of June 30, 2020 (unaudited), there was $1.1 million of unrecognized compensation expense which will be recognized over a weighted-average period of 3.4 years.

The following table summarizes the option activity under the 2018 Plan for the periods presented:

(in thousands, except share and per share data)	Options	Weighted average exercise price	Weighted average remaining life (in years)	Aggregate intrinsic value
Outstanding January 1, 2019	—	$ —	—	$ —
Granted	314,317	0.93		
Exercised	—	—		
Canceled	(3,014)	0.73		
Outstanding December 31, 2019	311,303	$ 0.93	9.5	$ 30
Granted	1,554,527	1.08		
Exercised	(753)	0.73		
Canceled	(108,223)	1.04		
Outstanding June 30, 2020 (unaudited)	1,756,854	$ 1.06	9.5	$ 476
Options exercisable—December 31, 2019	—	$ —	—	$ —
Options exercisable—June 30, 2020 (unaudited)	44,422	$ 0.82	8.5	$ 23
Options vested or expected to vest—December 31, 2019	311,303	$ 0.93	9.5	$ 30
Options vested or expected to vest—June 30, 2020 (unaudited)	1,756,854	$ 1.06	9.5	$ 476

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Restricted common stock

During the year ended December 31, 2018, the Company granted restricted common stock with service-based vesting conditions. Shares of unvested restricted common stock may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award. The grant date fair value of these shares was immaterial. The following table summarizes the Company's restricted common stock award activity for the year ended December 31, 2019 and six months ended June 30, 2020 (unaudited):

	Number of restricted shares
Issued and unvested as of January 1, 2019	1,141,736
Vested	(476,131)
Forfeited	(13,123)
Issued and unvested as of December 31, 2019	652,482
Vested	(141,077)
Issued and unvested as of June 30, 2020 (unaudited)	511,405

The Company recorded stock-based compensation expense in the following expense categories of its statements of operations:

	Year ended December 31,		Six months ended June 30,	
(in thousands)	2018	2019	2019	2020
				(unaudited)
Research and development	$ —	$ 13	$ —	$ 55
General and administrative	1	13	5	86
Total	$ 1	$ 26	$ 5	$ 141

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11. Income taxes

There is no provision for income taxes because the Company has historically incurred net operating losses and maintains a full valuation allowance against its deferred tax assets. The reported amount of income tax expense/benefit for the years ended December 31, 2018 and 2019 differs from the amount that would result from applying domestic federal statutory rates to pretax losses primarily because of changes in the valuation allowance.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year ended December 31,	
	2018	2019
Federal income tax expense at statutory rate	21%	21%
State taxes—net of federal benefit	6	6
Change in fair value of preferred stock tranche obligations	—	(2)
Other permanent differences	—	(1)
Federal & state R&D credits	2	4
Increase in valuation allowance	(29)	(28)
Effective income tax rate	0%	0%

Significant components of the Company's net deferred tax assets at December 31, 2018 and 2019 are as follows:

	Year ended December 31,	
(in thousands)	2018	2019
Deferred tax assets:		
Stock-based compensation	$ —	$ 1
Net operating loss carryforwards	1,264	4,848
Credit carryforwards	66	612
Intangible assets	4	12
Accrued expenses	50	161
Total deferred tax assets	1,384	5,634
Valuation allowance	(1,382)	(5,607)
Total net deferred tax assets	2	27
Deferred tax liabilities:		
Fixed assets	(2)	(27)
Total deferred tax liability	(2)	(27)
Total deferred tax assets (liabilities)	$ —	$ —

Since its inception in 2017, the Company has not recorded any U.S. federal or state income tax benefits for the net losses it has incurred in any year or for its earned research and development tax credits, due to the uncertainty of realizing a benefit from those items. The valuation allowance increased by $1.4 million and $4.2 million during the years ended December 31, 2018 and 2019, respectively. As of December 31, 2019, the

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Company had federal net operating loss carryforwards of $17.7 million and state net operating loss carryforwards of $17.7 million. The federal net operating loss carryforwards are indefinite lived, and the state net operating loss carryforwards begin to expire in 2038. As of December 31, 2019, the Company also had federal and state research and development tax credit carryforwards of $0.4 million and $0.2 million, respectively, which begin to expire in 2038 and 2033, respectively.

Utilization of the net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986, as amended, due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50% over a three-year period. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards would be subject to an annual limitation under Section 382, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization. Further, until a study is completed and any limitation is known, no amounts are being presented as an uncertain tax position.

The Company's policy is to record estimated interest and penalties related to uncertain tax positions in income tax expense. The Company has no amounts recorded for any unrecognized tax positions, accrued interest or penalties as of December 31, 2018 and 2019.

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates, including the United States and the Commonwealth of Massachusetts. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending tax examinations. The Company's tax returns are open under statute from 2018 to the present.

12. Net loss per share

Basic and diluted net loss per share was calculated as follows:

	Year ended December 31,		Six months ended June 30,	
(in thousands, except share and per share data)	**2018**	**2019**	**2019**	**2020**
				(unaudited)
Numerator:				
Net loss	$ (4,811)	$ (14,859)	$ (3,584)	$ (16,868)
Denominator:				
Weighted-average common shares outstanding—basic and diluted	474,118	2,442,872	2,355,066	2,675,260
Net loss per share—basic and diluted	$ (10.15)	$ (6.08)	$ (1.52)	$ (6.31)

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The following potentially dilutive common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the computation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	Year ended December 31,		Six months ended June 30,	
	2018	2019	2019	2020
				(unaudited)
Options to purchase common stock	—	311,303	104,454	1,756,854
Unvested restricted common stock	1,141,736	652,482	804,632	511,405
Convertible preferred stock (as converted to common stock)	3,768,575	9,798,298	9,798,298	10,401,270
Total	4,910,311	10,762,083	10,707,384	12,669,529

Unaudited pro forma net loss per share

The unaudited pro forma basic and diluted net loss per share for the year ended December 31, 2019 and six months ended June 30, 2020 have been prepared to give effect to adjustments arising upon the closing of a qualified IPO. The unaudited pro forma net loss used in the calculation of unaudited pro forma basic and diluted net loss per share removes the effects of changes in fair value of the Tranche Rights because the changes in fair value would not be recognized after the preferred stock has been converted to common stock.

Pro forma net loss per share was calculated as follows:

(in thousands, except share and per share data)	Year ended December 31, 2019	Six months ended June 30, 2020
		(unaudited)
Numerator:		
Net loss	$ (14,859)	$ (16,868)
Change in fair value of preferred stock tranche obligations	1,323	—
Pro forma net loss	$ (13,536)	$ (16,868)
Denominator:		
Weighted-average number of common shares used in computing net loss per share	2,442,872	2,675,260
Pro forma adjustment to reflect assumed conversion of convertible preferred stock into common stock	7,882,003	10,142,854
Pro forma weighted-average common shares outstanding—basic and diluted	10,324,875	12,818,114
Pro forma net loss per share—basic and diluted	$ (1.31)	$ (1.32)

The pro forma net loss per share does not include the sales of 17,500,000 shares of Series A Preferred Stock in July 2020 and 41,159,724 shares of Series B Preferred Stock in August 2020, which will convert into 17,685,105 shares of common stock upon the closing of the proposed IPO and will result in additional dilution in the periods subsequent to the issuance of those shares.

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13. Commitments and contingencies

Operating leases

In May 2019, the Company entered into a sublease agreement for a portion of the laboratory and office space in Waltham, Massachusetts. The term of the sublease commenced on July 1, 2019 and expires on December 31, 2021.

Rent expense for the years ended December 31, 2018 and 2019 totaled $0.3 million and $0.7 million, respectively. Rent expense for the six months ended June 30, 2019 and 2020 (unaudited) totaled $0.2 million and $0.4 million, respectively.

Future minimum lease payments under the non-cancelable operating leases consisted of the following as of December 31, 2019:

Year ending December 31,	(in thousands)
2020	$ 776
2021	800
Total	$ 1,576

Legal proceedings

The Company is not currently party to any material legal proceedings. At each reporting date, the Company evaluates whether a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company expenses as incurred the costs related to any such legal proceedings.

Other contractual obligations

The Company enters into contracts in the normal course of business with third parties for preclinical research studies, clinical trials and testing and manufacturing services. These contracts typically do not contain minimum purchase commitments and are generally cancelable by the Company upon written notice. Payments due upon cancellation consist of payments for services provided or expenses incurred, including noncancelable obligations of the service providers, up to the date of cancellation and in the case of certain arrangements may include non-cancelable fees.

The Company has also entered into a license agreement (the “UMONS Agreement”) with the University of Mons (“UMONS”) in April 2020, pursuant to which UMONS granted to the Company an exclusive, worldwide license to certain patents and patent applications and a non-exclusive, worldwide license to existing, related know-how. Each of the issued patents licensed to the Company under the UMONS Agreement is scheduled to expire in 2031. The licenses under the UMONS Agreement confer on the Company the right to research, develop and commercialize products (“licensed products”), and to practice processes, in each case, covered by the licensed patents and existing, related know-how.

Under the UMONS Agreement, the Company is obligated to use commercially reasonable efforts to develop at least one licensed product and, to the extent regulatory approval is obtained in such jurisdictions, to commercialize at least one licensed product in the United States and the United Kingdom or a member country

of the European Union. Unless terminated earlier, the UMONS Agreement will remain in effect until the last to expire of the licensed patent rights on a licensed product-by-licensed product and country-by-country basis. UMONS may terminate the UMONS Agreement in the event of a material breach by the Company and the Company's failure to cure such breach within a specified time period. The Company may voluntarily terminate the UMONS Agreement with prior notice to UMONS.

In connection with the Company's entry into the UMONS Agreement, the Company paid UMONS an upfront payment of €0.1 million. The Company also agreed to make milestone payments to UMONS upon the achievement of specified development and regulatory milestones up to a maximum aggregate total of €0.4 million for the first licensed product to achieve such milestones and up to a maximum aggregate total of €0.2 million for each subsequent licensed product to achieve each such milestones, as well as a low single-digit percentage royalty on net sales of licensed products by the Company, its affiliates and sublicensees. These royalty obligations continue on a licensed product-by-licensed product and country-by-country basis until the expiration of the last licensed patent rights covering such licensed product in such country. In addition, if the Company sublicenses rights under the UMONS Agreement, the Company is required to pay a low double-digit percentage of the sublicense revenue to UMONS. Additionally, if the Company chooses to file, prosecute or maintain any patents included in the licensed patent rights under the UMONS Agreement, it will be required to bear the full cost and expenses of preparing, filing, prosecuting and maintaining any such patents.

No amounts have been accrued under this arrangement with respect to potential milestone payment obligations or royalty obligations given the uncertainty of achieving the specified milestones and commercial sales of any licensed products.

14. Related parties

The Company has received professional services from a major stockholder, Atlas Venture Life Science Advisors, LLC. The Company recorded expenses totaling $0.2 million and $0.1 million related to these services for the years ended December 31, 2018 and 2019, respectively. The Company recorded expenses of $0.1 million related to these services for the six months ended June 30, 2019 (unaudited). There were no expenses recorded related to these services for the six months ended June 30, 2020 (unaudited). As of December 31, 2018, the Company had $0.1 million in accounts payable representing amounts owed to Atlas Venture Life Science Advisors, LLC. There were no amounts owed to Atlas Venture Life Science Advisors, LLC as of December 31, 2019 and June 30, 2020 (unaudited).

15. Subsequent events

For the year ended December 31, 2019, subsequent events were evaluated through July 23, 2020, the date on which the audited financial statements were issued and September 9, 2020 as to the effects of the reverse stock split discussion below.

For the six months ended June 30, 2020 (unaudited), subsequent events were evaluated through July 23, 2020, the date on which the unaudited interim financial statements were first issued, August 25, 2020 as to the equity transactions and equity awards referenced below and September 9, 2020 as to the effects of the reverse stock split discussion below. The Company has concluded that no events have occurred subsequent to June 30, 2020 that require disclosure, except for those referenced below, which are unaudited.

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Notes to financial statements

Issuance and sale of Series A Preferred Stock

In July 2020, the Company issued and sold 17,500,000 shares of Series A Preferred Stock at a price of $1.00 per share, for gross proceeds of $17.5 million. The terms of the Series A Preferred Stock sold in 2020 are identical to those of the existing shares of Series A Preferred Stock described in Note 8.

Issuance and sale of Series B Preferred Stock

In August 2020, the Company issued and sold 41,159,724 shares of Series B Preferred Stock at a price of $2.81 per share, for gross proceeds of $115.7 million. Except for the original issuance price, the terms of the Series B Preferred Stock are substantially the same as the terms of the Series A Preferred Stock. In connection with the issuance, the Company increased the number of authorized shares of Preferred Stock from 52,000,000 shares to 93,159,724 shares and the number of authorized shares of common stock was increased to 131,408,815 shares.

The effects of the sales of Series A Preferred Stock and the Series B Preferred Stock have been included in the presentation of the unaudited pro forma balance sheet at June 30, 2020.

Increase in shares available for issuance under the 2018 Plan

In July 2020, the number of shares of common stock authorized for issuance under the 2018 Plan was increased from 3,174,473 shares to 6,341,119 shares. In August 2020, the number of shares of common stock authorized for issuance under the 2018 Plan was further increased from 6,341,119 shares to 8,267,252 shares.

Grant of stock options under the 2018 Plan

On July 31, 2020, the Company granted options with service-based vesting criteria for the purchase of an aggregate of 2,815,981 shares of common stock, at an exercise price of $5.54 per share. The aggregate grant date fair value of these options was $10.1 million, which is expected to be recognized over approximately four years.
On July 31, 2020, the Company granted options with performance-based vesting criteria for the purchase of an aggregate of 638,825 shares of common stock, at an exercise price of $5.54 per share. The aggregate grant date fair value of these options was $1.8 million, which will be recognized once it becomes probable that the milestone will be achieved, and the shares will vest. The performance milestone relates to the Company's submission of an Investigational New Drug application with the U.S. Food and Drug Administration.

2020 Equity Plans

In August 2020 the Company's board of directors adopted and the Company's stockholders approved the 2020 Stock Incentive Plan (the "2020 Plan"), which will become effective immediately prior to the effectiveness of the registration statement for the proposed IPO. The 2020 Plan provides for the grant of awards with respect to an additional 2,955,746 shares of common stock. Subject to the effectiveness of the 2020 Plan, the Company will cease the grant of additional awards under the 2018 Plan.

In August 2020 the Company's board of directors adopted and the Company's stockholders approved the 2020 Employee Stock Purchase Plan (the "2020 ESPP"), which will become effective immediately prior to the effectiveness of the registration statement for the proposed IPO. The 2020 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 488,414 shares of common stock.

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Reverse stock split

The Company's board of directors and stockholders approved a one-for-3.3169 reverse split of the Company's issued and outstanding common stock and a proportional adjustment to the existing conversion ratios for the outstanding shares of convertible preferred stock, which became effective on September 9, 2020. Accordingly, all share and per share amounts for all periods presented in the accompanying financial statements and notes thereto have been retroactively adjusted, where applicable, to reflect the reverse stock split.

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12,251,578 shares

Common stock

Prospectus

Joint Book-Running Managers

J.P. Morgan **Jefferies** **Piper Sandler** **Stifel**

September 16, 2020

Through and including October 11, 2020, (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.