

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

- (Mark One)
- QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the quarterly period ended September 30, 2021
- OR
- TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**
For the transition period from _____ to _____
Commission File Number: 001-39509

Dyne Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware
(State or other jurisdiction of
incorporation or organization)
830 Winter Street
Waltham, Massachusetts
(Address of principal executive offices)

36-4883909
(I.R.S. Employer
Identification No.)

02451
(Zip Code)

(781) 786-8230

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	DYN	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input checked="" type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

As of October 31, 2021, the registrant had 51,528,469 shares of common stock, \$0.0001 par value per share, outstanding.

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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 Quarterly Report on Form 10-Q are the property of their respective owners. Solely for convenience, some of the trademarks, service marks and trade names referred to in this Quarterly Report on Form 10-Q 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.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or this Quarterly Report, contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act and Section 21E of the Securities Exchange Act of 1934, as amended, that involve substantial risk and uncertainties. All statements other than statements of historical fact, contained in this Quarterly Report, 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,” or the negative of these words or other 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 Quarterly Report 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 investigational new drug applications, or INDs, for any product candidates we may 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 our cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses and capital expenditure requirements;
- 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 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;
- 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 establish and maintain 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 Quarterly Report, particularly in Item 1A. “Risk Factors” in this Quarterly Report, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Moreover, we operate in a competitive and rapidly changing environment. 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 Quarterly Report and the documents that we have filed or incorporated by reference as exhibits to this Quarterly Report with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report are made as of the date of this Quarterly Report, 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.

RISK FACTOR SUMMARY

Our business is subject to a number of risks that, if realized, could materially affect our business, prospects, operating results and financial condition. These risks are discussed more fully in the “Risk Factors” section of this Quarterly Report. These risks include, but are not limited to, the following:

- our limited operating history may make it difficult to evaluate the success of our business to date and to assess our future viability;
- we are very early in our development efforts. Our product candidates are in varying stages of preclinical development, and we have not advanced any product candidate into clinical development. As a result it will be many years before we commercialize a product candidate, if ever. If we are unable to 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 develop our 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 our product candidates 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 develop and commercialize our product candidates 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 trials 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 to supply chains and may adversely impact economies worldwide, which could negatively impact our operations.

PART I—FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements (Unaudited)

Dyne Therapeutics, Inc.
Condensed Consolidated Balance Sheets (Unaudited)
(in thousands, except share and per share data)

	September 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	\$ 229,042	\$ 300,852
Marketable securities	178,481	44,462
Prepaid expenses and other current assets	17,132	3,773
Total current assets	424,655	349,087
Property and equipment, net	2,988	1,946
Right-of-use assets	36,927	—
Restricted cash and other assets	2,308	2,301
Total assets	\$ 466,878	\$ 353,334
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	7,088	3,440
Accrued expenses and other current liabilities	11,674	7,527
Lease liabilities	4,589	—
Total current liabilities	23,351	10,967
Lease liabilities, net of current portion	29,411	—
Total liabilities	52,762	10,967
Commitments and contingencies (Note 9)		
Stockholders' equity		
Preferred stock, \$0.0001 par value; 10,000,000 shares authorized at September 30, 2021 and December 31, 2020	—	—
Common stock, \$0.0001 par value; 200,000,000 shares authorized at September 30, 2021 and December 31, 2020; 51,607,908 and 45,446,903 shares issued and 51,483,643 and 45,076,574 shares outstanding at September 30, 2021 and December 31, 2020, respectively	6	5
Additional paid-in capital	590,832	421,572
Accumulated other comprehensive loss	(34)	(27)
Accumulated deficit	(176,688)	(79,183)
Total stockholders' equity	414,116	342,367
Total liabilities and stockholders' equity	\$ 466,878	\$ 353,334

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Dyne Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss (Unaudited)
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	\$ 36,510	\$ 9,679	\$ 79,007	\$ 23,102
General and administrative	6,256	3,841	19,058	6,945
Total operating expenses	42,766	13,520	98,065	30,047
Loss from operations	(42,766)	(13,520)	(98,065)	(30,047)
Other (expense) income:				
Interest income	184	—	560	24
Interest expense	—	(130)	—	(315)
Change in success fee obligation	—	(270)	—	(450)
Total other (expense) income, net	184	(400)	560	(741)
Net loss	\$ (42,582)	\$ (13,920)	\$ (97,505)	\$ (30,788)
Net loss per share—basic and diluted	\$ (0.83)	\$ (2.01)	\$ (1.92)	\$ (7.51)
Weighted-average common shares outstanding used in net				
loss per share—basic and diluted	51,320,940	6,920,008	50,676,668	4,100,504
Comprehensive loss:				
Net loss	\$ (42,582)	\$ (13,920)	\$ (97,505)	\$ (30,788)
Other comprehensive loss:				
Unrealized gains (losses) on marketable securities, net	7	—	(7)	—
Comprehensive loss	\$ (42,575)	\$ (13,920)	\$ (97,512)	\$ (30,788)

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Dyne Therapeutics, Inc.
Condensed Consolidated Statements of Stockholders' Equity (Deficit) (Unaudited)
(in thousands, except share data and issuance costs)

(in thousands, except per share data)	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balance at January 1, 2021	—	\$ —	45,076,574	\$ 5	\$ 421,572	\$ (27)	\$ (79,183)	\$ 342,367
Issuance of common stock upon follow-on public offering, net of issuance costs of \$0.7 million	—	—	6,000,000	1	157,236	—	—	157,237
Exercise of stock options	—	—	11,163	—	13	—	—	13
Stock-based compensation	—	—	—	—	3,652	—	—	3,652
Vesting of restricted shares	—	—	70,539	—	—	—	—	—
Unrealized losses on marketable securities	—	—	—	—	—	(41)	—	(41)
Net loss	—	—	—	—	—	—	(24,968)	(24,968)
Balance at March 31, 2021	—	\$ —	51,158,276	\$ 6	\$ 582,473	\$ (68)	\$ (104,151)	\$ 478,260
Exercise of stock options	—	—	10,088	—	25	—	—	25
Stock-based compensation	—	—	—	—	4,239	—	—	4,239
Vesting of restricted shares	—	—	63,977	—	—	—	—	—
Unrealized gains on marketable securities	—	—	—	—	—	27	—	27
Net loss	—	—	—	—	—	—	(29,955)	(29,955)
Balance at June 30, 2021	—	\$ —	51,232,341	\$ 6	\$ 586,737	\$ (41)	\$ (134,106)	\$ 452,596
Exercise of stock options	—	—	99,679	—	457	—	—	457
Stock-based compensation	—	—	—	—	3,638	—	—	3,638
Vesting of restricted shares	—	—	63,977	—	—	—	—	—
Vesting of restricted stock units	—	—	87,646	—	—	—	—	—
Unrealized gains on marketable securities	—	—	—	—	—	7	—	7
Net loss	—	—	—	—	—	—	(42,582)	(42,582)
Balance at September 30, 2021	—	\$ —	51,483,643	\$ 6	\$ 590,832	\$ (34)	\$ (176,688)	\$ 414,116

(in thousands, except per share data)	Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
Balance at January 1, 2020	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
Vesting of restricted shares	—	—	70,538	—	—	—	—	—
Stock-based compensation	—	—	—	—	66	—	—	66
Net loss	—	—	—	—	—	—	(7,886)	(7,886)
Balance at March 31, 2020	34,500,000	\$ 29,401	2,657,073	\$ 1	\$ 6,418	\$ —	\$ (27,632)	\$ 8,188
Exercise of stock options	—	—	753	—	—	—	—	—
Stock-based compensation	—	—	—	—	75	—	—	75
Vesting of restricted shares	—	—	70,539	—	—	—	—	—
Net loss	—	—	—	—	—	—	(8,982)	(8,982)
Balance at June 30, 2020	34,500,000	\$ 29,401	2,728,365	\$ 1	\$ 6,493	\$ —	\$ (36,614)	\$ (719)
Issuance of Series A convertible preferred stock, net of issuance costs of \$0.1 million	17,500,000	17,495	—	—	—	—	—	17,495
Issuance of Series B convertible preferred stock, net of issuance costs of \$0.4 million	41,159,724	115,319	—	—	—	—	—	115,319
Conversion of convertible preferred stock into common stock upon initial public offering	(93,159,724)	(162,215)	28,086,375	3	162,212	—	—	—
Issuance of common stock upon initial public offering, net of issuance costs of \$2.6 million	—	—	14,089,314	1	246,410	—	—	246,411
Exercise of stock options	—	—	29,656	—	24	—	—	24
Stock-based compensation	—	—	—	—	2,235	—	—	2,235
Vesting of restricted shares	—	—	70,537	—	—	—	—	—
Net loss	—	—	—	—	—	—	(13,920)	(13,920)
Balance at September 30, 2020	—	\$ —	45,004,247	\$ 5	\$ 417,374	\$ —	\$ (50,534)	\$ 366,845

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Dyne Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows (Unaudited)
(in thousands)

	Nine Months Ended September 30,	
	2021	2020
Cash flows from operating activities:		
Net loss	\$ (97,505)	\$ (30,788)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	11,529	2,376
Depreciation and amortization expense	747	483
Non-cash lease expense	(28)	—
Amortization (accretion) of premium (discount) on marketable securities	1,843	—
Changes in success fee obligation	—	450
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(12,816)	(107)
Right-of-use assets	(2,940)	—
Accounts payable and other liabilities	7,675	1,114
Net cash used in operating activities	(91,495)	(26,472)
Cash flows from investing activities:		
Purchases of property and equipment	(1,628)	(438)
Purchases of marketable securities	(208,207)	—
Maturities of marketable securities	72,178	—
Net cash used in investing activities	(137,657)	(438)
Cash flows from financing activities:		
Proceeds from initial public offering of common stock, net of issuance costs	—	247,454
Proceeds from follow-on public offering of common stock, net of issuance costs	157,237	—
Proceeds from issuance of debt, net of issuance costs	—	9,936
Settlement of success fee obligation	—	(450)
Proceeds from issuance of convertible preferred stock, net of issuance costs	—	134,920
Proceeds from exercise of stock options	495	24
Net cash provided by financing activities	157,732	391,884
Net increase (decrease) in cash and cash equivalents	(71,420)	364,974
Cash, cash equivalents and restricted cash, beginning of period	303,153	14,632
Cash, cash equivalents and restricted cash, end of period	\$ 231,733	\$ 379,606
Supplemental cash flow information:		
Cash paid for interest and taxes	\$ —	\$ 311
Supplemental disclosures of cash flow information:		
Purchase of property and equipment in accounts payable	\$ 161	\$ —
Right-of-use assets acquired under operating leases	\$ 33,799	\$ —
Issuance costs from convertible preferred stock included in accounts payable or accrued expenses	\$ —	\$ 134
Conversion of convertible preferred stock to common stock	\$ —	\$ 162,215
Public offering costs included in accounts payable or accrued expenses	\$ —	\$ 1,043

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Dyne Therapeutics, Inc.
Notes to Condensed Consolidated Financial Statements (Unaudited)

1. Nature of the Business and Basis of Presentation

Dyne Therapeutics, Inc. (the “Company”) is building a leading muscle disease company focused on advancing innovative life-transforming therapeutics for people living with genetically driven diseases that was incorporated in Delaware on December 1, 2017 and has a principal place of business in Waltham, Massachusetts.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, risks of failure of preclinical studies and clinical trials, the need to obtain marketing approval for its product candidates, fluctuations in operating results, compliance with government regulations, the ability to establish clinical- and commercial-scale manufacturing processes, the impact of the COVID-19 pandemic and the ability to secure additional capital to fund operations. Product candidates and programs currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization of a product. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company’s development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

On September 21, 2020, the Company completed its initial public offering (“IPO”) pursuant to which it issued and sold 14,089,314 shares of its common stock, including 1,837,736 shares pursuant to the full exercise of the underwriters’ option to purchase additional shares, at a public offering price of \$19.00 per share, resulting in net proceeds of \$246.4 million, after deducting underwriting discounts and commissions and offering expenses. Upon the closing of the IPO, all of the Company’s then outstanding convertible preferred stock automatically converted into shares of common stock.

On January 25, 2021, the Company completed a follow-on public offering of common stock pursuant to which it issued and sold 6,000,000 shares of its common stock at a public offering price of \$28.00 per share, resulting in net proceeds of \$157.2 million, after deducting underwriting discounts and commissions and offering expenses.

The accompanying condensed consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. Since inception, the Company has funded its operations with proceeds from the sales of preferred stock and common stock, including the IPO completed in September 2020 and the follow-on offering completed in January 2021. The Company expects to continue to generate operating losses for the foreseeable future. The Company expects that its cash, cash equivalents and marketable securities will be sufficient to fund its operating expenses and capital expenditure requirements for at least 12 months from the issuance of these condensed consolidated financial statements.

To continue its development efforts, the Company will need to obtain substantial additional funding through public or private equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements in order to fund its research and development and ongoing operating expenses. The Company may not be able to obtain financing on acceptable terms, when needed or at all, and the Company may not be able to enter into collaborations, strategic alliances or licensing arrangements. The terms of any financing may adversely affect the holdings or the rights of the Company’s stockholders. Any collaborations, strategic alliances or licensing arrangements may require the Company to relinquish rights to certain of its technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to the Company. If the Company is unable to obtain funding, the Company could be forced to delay, limit, reduce or eliminate some or all of its research and development programs, pipeline expansion or future commercialization efforts or grant rights to develop and market product candidates, which could adversely affect its business prospects. Although management will continue to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations when needed or at all.

To date, the Company has not experienced material business disruptions, including with its vendors, as a result of the COVID-19 pandemic. In March 2020, the Company implemented a remote working policy for many of its employees and began restricting non-essential travel, and in May 2020, when Massachusetts began its staged reopening plan, the Company began implementing a return-to-work plan, in accordance with the guidance and requirements of federal and state authorities. The Company continues to take actions as may be required or recommended by government authorities or as it determines are in the best interests of its employees and other business partners. The Company is continuing to

monitor the potential impact of the pandemic, but cannot be certain what the overall impact will be on its business, financial condition, results of operations and prospects.

2. Summary of Significant Accounting Policies

The accompanying financial statements have been prepared in conformity with generally accepted accounting principles ("GAAP") in the United States of America. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

The financial statements of the Company included herein have been prepared, without audit, pursuant to the rules and regulations of the Securities and Exchange Commission (the "SEC"). The unaudited interim financial statements have been prepared on the same basis as audited annual financial statements, except certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted from this report, as is permitted by such rules and regulations. In the opinion of management, the interim financial information reflects all adjustments, all of which are of a normal and recurring nature, necessary for a fair representation of the results for the reported periods. Accordingly, these financial statements should be read in conjunction with the financial statements and notes thereto included in the Company's annual report on Form 10-K filed with the SEC on March 4, 2021. The results for the nine months ended September 30, 2021 are not necessarily indicative of results to be expected for the year ending December 31, 2021, any other interim periods, or any future year or period.

Marketable securities

The Company's marketable securities as of September 30, 2021 consisted of commercial paper, certificates of deposit, corporate debt securities and US treasury securities and are classified as available-for-sale and are reported at fair value. Unrealized losses on available-for-sale debt securities are reported as a component of accumulated other comprehensive loss in stockholders' equity. Realized gains and losses are based on the specific identification method and are included as a component of other income (expense), net in the condensed consolidated statements of operations and comprehensive loss.

The Company evaluates its marketable securities with unrealized losses for other-than-temporary impairment. When assessing marketable securities for other-than-temporary declines in value, the Company considers such factors as, among other things, how significant the decline in value is as a percentage of the original cost, how long the market value of the investment has been less than its original cost, the Company's ability and intent to retain the investment for a period of time sufficient to allow for any anticipated recovery in fair value and market conditions in general. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the statement of operations and comprehensive loss. No such adjustments were necessary during the periods presented.

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	5 years
Furniture and fixtures	5 years
Computer equipment	3 years
Leasehold improvements	Shorter of life of lease or 10 years

Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated once placed into service. 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 which do not improve or extend the life of the respective assets are charged to expense as incurred.

Leases

Prior to January 1, 2021, the Company accounted for leases under ASC 840, *Leases* ("ASC 840"). The Company recorded monthly rent expense on a straight-line basis, equal to the total of the payments due over the lease term, divided by the number of months of the lease term. The difference between rent expense recorded and the amount paid was charged to deferred rent.

Effective January 1, 2021, the Company adopted ASU 2016-02, *Leases (Topic 842)* (“ASC 842”), using the modified retrospective approach transition method as of the date of adoption. Under this method, financial statements for reporting periods after adoption are presented in accordance with ASC 842 and prior-period financial statements continue to be presented in accordance with ASC 840. Upon adoption, the Company recognized lease liabilities totaling \$0.8 million and right-of-use assets totaling \$0.7 million.

Under ASC 842, at the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances presented in the arrangement, including whether the Company controls the use of identified assets. The Company classifies leases with a term greater than one year as either operating or finance leases at the lease commencement date and records a right-of-use assets and current and non-current lease liabilities, as applicable on the balance sheet. The Company has elected not to recognize on the balance sheet leases with terms of one year or less, but payments are recognized as expense on a straight-line basis over the lease term. If a lease includes options to extend the lease term, the Company does not assume the option will be exercised in its initial lease term assessment unless there is reasonable certainty that the Company will renew based on an assessment of economic factors present as of the lease commencement date. The Company monitors its plans to renew its material leases each reporting period.

Lease liabilities and the corresponding right-of-use assets are recorded based on the present value of lease payments over the remaining lease term. The present value of future lease payments are discounted using the interest rate implicit in lease contracts if that rate is readily determinable; otherwise the Company utilizes its incremental borrowing rate (“IBR”), which reflects the fixed rate at which the Company could borrow on a collateralized basis over a similar term, the amount of the lease payments in a similar economic environment. After lease commencement and the establishment of a right-to-use asset and operating lease liability, lease expense is recorded on a straight-line basis over the lease term.

The Company enters into contracts that contain both lease and non-lease components. Non-lease components include costs that do not provide a right-to-use a leased asset but instead provide a service, such as maintenance costs. The Company has elected to account for the lease and non-lease components together as a single component for all classes of underlying assets. Variable costs associated with the lease, such as maintenance and utilities, are not included in the measurement of right-to-use assets and lease liabilities but rather are expensed when the events determining the amount of variable consideration to be paid have occurred.

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 views the Company’s operations and manages the business as one operating segment.

Fair value measurements

Certain assets and liabilities are carried at fair value. Fair value is defined as the amount 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.
- 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

In the periods prior to the IPO, the Company followed the two-class method when computing net loss per share as the Company had issued shares that met 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. Following the IPO, there were no participating securities outstanding.

Basic net loss per share is computed by dividing the net 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.

3. Cash, Cash Equivalents and Restricted Cash

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.

Amounts included in restricted cash represent amounts pledged as collateral for letters of credit required for security deposits on the Company's leased facilities. Restricted cash totaled \$2.7 million and \$2.3 million at September 30, 2021 and December 31, 2020, respectively. These amounts are classified as a component of other current assets and as restricted cash on the Company's condensed consolidated balance sheets.

Cash, cash equivalents and restricted cash consisted of the following:

(in thousands)	September 30, 2021	December 31, 2020
Cash and cash equivalents	\$ 229,042	\$ 300,852
Short term restricted cash	383	—
Restricted cash	2,308	2,301
Total	\$ 231,733	\$ 303,153

4. Fair Value Measurements

The following tables set forth by security type, marketable securities for the periods presented:

(in thousands)	As of September 30, 2021			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
Commercial paper	\$ 11,509	\$ —	\$ —	\$ 11,509
Certificates of deposit	4,490	—	—	4,490
Corporate debt securities	135,985	12	(48)	135,949
US treasury notes	26,531	4	(2)	26,533
Total	\$ 178,515	\$ 16	\$ (50)	\$ 178,481

(in thousands)	As of December 31, 2020			
	Amortized Cost	Unrealized Gains	Unrealized Losses	Total
Commercial paper	\$ 6,412	\$ —	\$ —	\$ 6,412
Certificates of deposit	1,504	—	—	1,504
Corporate debt securities	36,573	—	(27)	36,546
Total	\$ 44,489	\$ —	\$ (27)	\$ 44,462

The following tables set forth by level, within the fair value hierarchy, the assets carried at fair value on a recurring basis for the periods presented:

(in thousands)	Fair value measurements as of September 30, 2021			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents				
Money market funds	\$ 120,408	\$ —	\$ —	\$ 120,408
Corporate debt securities	—	524	—	524
Marketable securities				
Commercial paper	11,508	—	—	11,508
Certificates of deposit	—	4,490	—	4,490
Corporate debt securities	—	135,949	—	135,949
US treasury notes	26,534	—	—	26,534
Total	\$ 158,450	\$ 140,963	\$ —	\$ 299,413

(in thousands)	Fair Value Measurements as of December 31, 2020			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents				
Money market funds	\$ 155,017	\$ —	\$ —	\$ 155,017
Commercial paper	2,999	—	—	2,999
Certificates of deposit	—	5,309	—	5,309
Marketable securities				
Commercial paper	6,412	—	—	6,412
Certificates of deposit	—	1,504	—	1,504
Corporate debt securities	—	36,545	—	36,545
Total	\$ 164,428	\$ 43,358	\$ —	\$ 207,786

The fair value of money market funds, commercial paper and US treasury notes were determined by the Company based on quoted market prices, which represent a Level 1 measurement within the fair value hierarchy. Certificates of deposit and corporate debt securities notes were valued by the Company using quoted prices in active markets for similar securities, which represent a Level 2 measurement within the fair value hierarchy.

There were no transfers between Level 1, Level 2, or Level 3 during the periods presented.

The following table summarizes the scheduled maturity for the Company's marketable securities at September 30, 2021:

(in thousands)	September 30, 2021
Maturing in one year or less	\$ 131,877
Maturing in one year through two years	46,604
Maturing after two years	—
Total	\$ 178,481

Financial instruments not recorded at fair value

The carrying values of cash, 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.

5. Property and Equipment

Property and equipment consisted of the following:

(in thousands)	September 30,		December 31,	
	2021		2020	
Laboratory equipment	\$	3,393	\$	2,744
Office and computer equipment		79		78
Leasehold improvements		14		14
Construction in process		1,244		105
Property and equipment—at cost		4,730		2,941
Less accumulated depreciation and amortization		(1,742)		(995)
Property and equipment—net	\$	2,988	\$	1,946

Depreciation and amortization expense for the three and nine months ended September 30, 2021 was \$0.3 million and \$0.7 million, respectively. Depreciation and amortization expense for the three and nine months ended September 30, 2020, was \$0.2 million and \$0.5 million, respectively.

6. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	September 30,		December 31,	
	2021		2020	
Payroll and benefits	\$	2,693	\$	2,105
Consulting services		694		263
Legal services		136		325
Research and development		8,151		4,782
Facility costs		-		52
Total	\$	11,674	\$	7,527

7. Stock-Based Awards

2018 Stock Incentive Plan

The Company's 2018 Stock Incentive Plan (the "2018 Plan") provided for the Company to sell or issue incentive stock options or nonqualified stock options, restricted stock, and other equity awards to employees, directors and consultants of the Company. The 2018 Plan was administered by the board of directors or, at the discretion of the board of directors, by a committee of the board of directors. The exercise prices, vesting and other restrictions were determined at the discretion of the board of directors.

The total number of shares of common stock authorized under the 2018 Plan was 8,267,252 shares. Upon the effectiveness of the 2020 Stock Incentive Plan (the "2020 Plan"), the Company ceased granting awards under the 2018 Plan, and the 1,928,487 shares of common stock remaining under the 2018 Plan became available for future issuance under the 2020 Plan.

2020 Stock Incentive Plan

In August 2020 the Company's board of directors adopted and the Company's stockholders approved the 2020 Plan, which became effective on September 16, 2020. 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 to employees, directors, consultants and advisors of the Company. The 2020 Plan is administered by the Company's board of directors or by a committee appointed by the board of directors. Upon the effectiveness of the 2020 Plan, the Company ceased granting awards under the 2018 Plan. The number of shares initially reserved for issuance under the 2020 Plan was 4,884,233. The number of shares of common stock reserved for issuance under the 2020 Plan 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 lower of (1) 5% of the shares of common stock outstanding on such date and (2) an amount determined by the Company's board of directors. On January 1, 2021, 2,272,345 shares were added to the shares reserved for issuance under the 2020 Plan in the first of these annual increases.

As of September 30, 2021, 4,289,104 shares remained available for future issuance under the 2020 Plan.

2020 Employee Stock Purchase 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 became effective September 16, 2020. The 2020 ESPP is administered by the Company's board of directors or by a committee appointed by the board of directors. The 2020 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 488,414 shares of common stock. The number of shares of 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 (1) 1,953,656 shares of common stock, (2) 1% of the shares of common stock outstanding on such date, and (3) an amount determined by the board of directors. As of September 30, 2021, no offering periods have commenced under the 2020 ESPP and 488,414 shares remained available for issuance.

Stock option valuation

The Company typically grants stock options at exercise prices deemed by the Board to be equal to the fair value of the common stock at the time of grant. In the periods prior to the IPO, the fair value of the common stock was 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 preferred stockholders, and the prospects of a liquidity event, among others. In the periods following the IPO, the fair value is determined based upon the quoted price of the Company's common stock on Nasdaq.

The fair value of stock option grants is estimated using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The assumptions that the Company used to determine the grant-date fair value of options granted were as follows:

	Nine Months Ended September 30,	
	2021	2020
Expected volatility	74%	75%
Risk-free interest rate	0.82% — 1.17%	0.31% — 1.67%
Expected term (in years)	6	6
Expected dividend yield	—	—

Stock option activity

A summary of the Company's stock option activity and related information for the nine months ended September 30, 2021 is as follows:

(in thousands, except share and per share data)	Options	Weighted Average Exercise Price	Weighted Average Remaining Life (in years)	Aggregate Intrinsic Value
Outstanding as of January 1, 2021	6,421,589	\$ 6.97	9.5	\$ 90,112
Granted	1,160,176	18.15		
Exercised	(120,930)	4.10		
Canceled	(135,163)	9.83		
Outstanding as of September 30, 2021	7,325,672	\$ 8.73	8.3	\$ 60,678
Options exercisable as of September 30, 2021	1,567,826	\$ 5.91	8.3	\$ 16,944
Options vested or expected to vest as of September 30, 2021	6,932,411	\$ 8.73	8.9	\$ 57,499

The weighted-average grant date fair value of the options granted during the nine months ended September 30, 2021 and 2020 was \$11.68 and \$4.27 per share, respectively. As of September 30, 2021 there was \$26.3 million of unrecognized compensation expense, which the Company expects to recognize over a weighted-average period of 2.7 years. Additionally, as of September 30, 2021, the Company has unrecognized compensation cost related to unvested stock-based awards with performance-based vesting conditions for which performance has not been deemed probable of \$2.2 million.

Restricted stock units

A restricted stock unit (“RSU”) represents the right to receive one share of common stock upon vesting of the RSU. The Company grants RSUs with service conditions that vest in four equal annual installments provided that the employee remains employed with the Company. The Company also grants RSUs with performance-based vesting conditions for which performance was not deemed probable at September 30, 2021. The fair value of each RSU is based on the closing price of the Company’s common stock on the date of grant. A summary of the Company’s RSU activity and related information for the 2020 Plan for the nine months ended September 30, 2021:

	Number of Shares Underlying RSUs	Weighted Average Grant Date Fair Value
Issued and unvested as of January 1, 2021	403,355	\$ 23.60
Granted	256,450	17.83
Vested	(87,646)	23.90
Forfeited	(13,407)	22.55
Issued and unvested as of September 30, 2021	558,752	\$ 20.98

As of September 30, 2021, there was \$8.5 million of unrecognized compensation costs related to unvested RSUs, which are expected to be recognized over a weighted-average period of 2.9 years. Additionally, as of September 30, 2021, the Company has unrecognized compensation cost of \$0.6 million related to unvested stock-based awards with performance-based vesting conditions for which performance has not been deemed probable.

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 aggregate grant date fair value of these awards was immaterial. The following table summarizes the Company’s restricted common stock award activity for the nine months ended September 30, 2021:

	Number of Restricted Shares
Issued and unvested as of January 1, 2021	370,329
Vested	(198,493)
Forfeited	(47,571)
Issued and unvested as of September 30, 2021	124,265

The Company recorded stock-based compensation expense in the following expense categories of its statements of operations:

(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Research and development	\$ 1,422	\$ 929	\$ 5,141	\$ 984
General and administrative	2,216	1,306	6,388	1,392
Total	\$ 3,638	\$ 2,235	\$ 11,529	\$ 2,376

In June 2021, the Company entered into a severance agreement with its former chief scientific officer resulting in a modification to previously issued equity awards. This modification resulted in recognition of \$0.4 million of stock-based compensation expense at the time of modification.

8. Net Loss per Share

Basic and diluted net loss per share was calculated as follows:

(in thousands, except share and per share data)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Numerator:				
Net loss	\$ (42,582)	\$ (13,920)	\$ (97,505)	\$ (30,788)
Denominator:				
Weighted-average common shares outstanding used in net loss per share—basic and diluted	51,320,940	6,920,008	50,676,668	4,100,504
Net loss per share—basic and diluted	\$ (0.83)	\$ (2.01)	\$ (1.92)	\$ (7.51)

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:

	September 30,	
	2021	2020
Options to purchase common stock	7,325,672	6,337,779
Unvested restricted common stock	124,265	440,868
Unvested restricted stock units	558,752	375,137
Total	8,008,689	7,153,784

9. Commitments and Contingencies

Operating leases

In May 2019, the Company entered into a sublease agreement for laboratory and office space in Waltham, Massachusetts. The term of the sublease commenced on July 1, 2019 and expires on December 31, 2021.

In December 2020, the Company entered into a lease agreement, which was amended in January 2021, March 2021, and June 2021 (the "Lease"), pursuant to which the Company has agreed to lease approximately 68,187 square feet of office and laboratory space located in Waltham, Massachusetts (the "Premises"). The Lease has a term of eight years and six months that commenced under the standards of ASC 842 when the Company gained access to the Premises in September 2021. The Company's obligation for the payment of base rent for the Premises begins in April 2022, and will be \$0.4 million per month, increasing up to \$0.5 million during the term of the Lease. The Company has two options to extend the term of the Lease, each for a period of an additional five years.

Rent expense for the three and nine months ended September 30, 2021 totaled \$0.2 million and \$0.6 million, respectively. Rent expense for the three and nine months ended September 30, 2020 totaled \$0.2 million and \$0.4 million, respectively.

Summary of lease costs

The Company did not have any material finance leases as of September 30, 2021.

The components of lease cost under ASC 842 for the leases were as follows:

(in thousands)	Three Months Ended September 30, 2021	Nine Months Ended September 30, 2021
Operating lease cost	\$ 189	\$ 569
Short term lease cost	—	—
Variable lease cost	135	381
Total lease cost	\$ 324	\$ 950

Supplemental disclosure of cash flow information related to leases under ASC 842 for the leases were as follows:

(in thousands)	Nine Months Ended September 30, 2021
Operating cash payments for operating leases	\$ 597

The weighted-average remaining lease term and discount rate for the leases were as follows:

(in thousands)	As of September 30, 2021
Weighted-average remaining lease term - operating leases	8.5 years
Weighted-average discount rate - operating leases	5.79%

Future minimum lease payments under non-cancelable operating leases under ASC 842 consisted of the following as of September 30, 2021:

Year Ending December 31,	(in thousands)
2021 (remaining 3 months)	\$ 2,391
2022	3,452
2023	4,706
2024	4,847
2025	4,993
Thereafter	22,930
Total future minimum lease payments	43,319
Less: imputed interest	(9,319)
Present value of lease liabilities	\$ 34,000

Included in the condensed consolidated balance sheet (in thousands)	September 30, 2021
Lease liability	\$ 4,589
Lease liability, net of current portion	29,411
Total lease liability	\$ 34,000

Future minimum lease payments under non-cancelable operating leases under ASC 840 for the sublease and Lease consisted of the following as of December 31, 2020:

Year Ending December 31,	(in thousands)
2021	\$ 800
2022	3,452
2023	4,706
2024	4,847
2025	4,993
Thereafter	22,930
Total	\$ 41,728

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q, or this Quarterly Report, and our audited condensed consolidated financial statements and related notes for the year ended December 31, 2020, including in our Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, on March 4, 2021. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, including information with respect to our plans and strategy for our business, 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 Quarterly Report, 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.

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. We have identified lead product candidates for each of our lead programs that are in varying stages of preclinical development. We expect to submit investigational new drug, or IND, applications to the U.S. Food and Drug Administration, or FDA, for our DMD product candidate, DYNE-251, in the fourth quarter of 2021, for our DM1 product candidate, DYNE-101, in the first quarter of 2022 and for our FSHD product candidate, DYNE-301, in the second half of 2022. Following the IND submissions and review with regulators, we plan to initiate global, placebo-controlled multiple ascending dose, or MAD, clinical trials for DYNE-251 and DYNE-101 evaluating safety, key disease markers and muscle function in patients with DMD and DM1, with the intention of dosing patients in both trials by the middle 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 our loan agreement, with a commercial bank. On September 21, 2020, we completed our initial public offering, or IPO, pursuant to which we issued and sold 14,089,314 shares of our common stock, including 1,837,736 shares pursuant to the full exercise of the underwriters' option to purchase additional shares. We received net proceeds of \$246.4 million, after deducting underwriting discounts and commissions and offering expenses payable by us. On January 25, 2021, we completed a follow-on public offering, which we refer to as the January 2021 offering, pursuant to which we issued and sold 6,000,000 shares of our common stock. We received net proceeds of \$157.2 million, after deducting underwriting discounts and commissions and offering expenses payable by us.

Since our inception, we have incurred significant operating losses. Our ability to generate any product revenue or product revenue sufficient to achieve profitability will depend on the successful development and eventual commercialization of one or more product candidates. For the three and nine months ended September 30, 2021 we reported net losses of \$42.6 million and 97.5 million, respectively. For the three and nine months ended September 30, 2020 we reported net

losses of \$13.9 million and \$30.8 million, respectively. As of September 30, 2021, we had an accumulated deficit of \$176.7 million.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We expect that our expenses and capital expenditure requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- continue our current lead programs in DM1, DMD and FSHD and conduct research programs in additional indications;
- advance our product candidates and any additional product candidates we identify through and 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, we expect to continue 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.

We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2024. 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. See “—Liquidity and capital resources” below.

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 to supply chains and may adversely impact economies worldwide, both of which could adversely affect our business and 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 in May 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 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 and development of our product candidates, 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 or product candidates because costs are deployed across multiple programs and product candidates as well as 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 through IND-enabling studies and into 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 we may develop;
- 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 and move into our new facility in the fourth quarter of 2021. 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, cash equivalents and marketable securities. We expect our interest income to increase during 2021 due to our increased cash resources.

Interest expense

We did not incur any interest expense in the three or nine months ended September 30, 2021. For the three and nine months ended September 30, 2020, interest expense consisted of interest on borrowings under our loan agreement as well as amortization of debt discount and debt issuance costs. In October 2020, all outstanding borrowings under our loan agreement were repaid.

Change in success fee obligation

In February 2020, we entered into a loan agreement with Pacific Western Bank, or PWB, under which we borrowed an aggregate principal amount of \$10.0 million in the form of a term loan. Our loan agreement required us to pay a success fee upon the occurrence of a specified liquidity event, as defined in the agreement, which included our initial public

offering. In September 2020, following the completion of our initial public offering, we paid the success fee of \$0.5 million, and in October 2020, we paid PWB \$10.0 million as repayment of the outstanding principal and less than \$0.1 million as accrued interest on the term loan. At inception of the loan agreement, this contingent obligation was considered immaterial. The fair value of the liability was remeasured at each reporting date, with changes in fair value recorded as a component of other income (expense) on the statement of operations.

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 our uncertainty of realizing a benefit from those items. As of December 31, 2020, we had federal and state net operating loss carryforwards of \$69.1 million and \$68.9 million, respectively. 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, 2020, we also had federal and state research and development tax credit carryforwards \$0.8 million and \$0.4 million which begin to expire in 2039 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 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. We completed a Section 382 study of transactions in our stock through December 31, 2020 and concluded that we have experienced ownership changes since inception that we believe under Section 382 and 383 of the Code will result in limitations on our ability to use certain pre-change NOLs and credits. In addition, we may experience subsequent ownership changes as a result of future equity offerings or other changes in the ownership of our stock, some of which are beyond our control. As a result, the amount of the NOLs and tax credit carryforwards presented in our financial statements could be limited. Similar provisions of state tax law may also apply to limit the use of accumulated state tax attributes.

Results of operations

Comparison of the three months ended September 30, 2021 and 2020

The following table summarizes our results of operations for the three months ended September 30, 2021 and 2020:

(in thousands)	Three Months Ended September 30,		Change
	2021	2020	
Operating expenses:			
Research and development	\$ 36,510	\$ 9,679	\$ 26,831
General and administrative	6,256	3,841	2,415
Total operating expenses	42,766	13,520	29,246
Loss from operations	(42,766)	(13,520)	(29,246)
Other income (expense):			
Interest income	184	—	184
Interest expense	—	(130)	130
Change in success fee obligation	—	(270)	270
Total other income (expense), net	184	(400)	584
Net loss	\$ (42,582)	\$ (13,920)	\$ (28,662)

Research and development expenses

The following table summarizes our research and development expenses for the three months ended September 30, 2021 and 2020:

(in thousands)	Three Months Ended September 30,		Change
	2021	2020	
Personnel-related	\$ 4,260	\$ 1,875	\$ 2,385
Stock-based compensation expense	1,422	929	493
Laboratory supplies and research materials	687	878	(191)
External manufacturing and research	27,908	5,092	22,816
Professional and consulting fees	883	277	606
Facility-related and other	1,350	628	722
Total research and development expenses	\$ 36,510	\$ 9,679	\$ 26,831

The increase in personnel-related costs and stock-based compensation expense was primarily due to increased headcount in our research and development function. The increase in facility-related and other expenses was primarily due to the increased costs of supporting a larger number of research and development personnel and their research efforts. The increase in external manufacturing and research costs and professional and consulting fees was primarily due to the development of our manufacturing process, increased research activity, and the advancement of our programs and product candidates, including conducting IND-enabling studies.

General and administrative expenses

The following table summarizes our general and administrative expenses for the three months ended September 30, 2021 and 2020:

(in thousands)	Three Months Ended September 30,		Change
	2021	2020	
Personnel-related	\$ 1,779	\$ 928	\$ 851
Stock-based compensation expense	2,216	1,306	910
Professional and consulting fees	1,518	1,133	385
Facility-related and other	743	474	269
Total general and administrative expenses	\$ 6,256	\$ 3,841	\$ 2,415

The increase in personnel-related costs and stock-based compensation expense 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 the costs of operating as a public company. The increase in facility-related and other expenses was primarily due to the increased costs of supporting a larger number of general and administrative personnel.

Interest income

Interest income for the three months ended September 30, 2021 was \$0.2 million due to interest earned on invested cash balances. We did not earn interest income in the three months ended September 30, 2020.

Interest expense

We did not incur any interest expense in the three months ended September 30, 2021. Interest expense was \$0.1 million for the three months ended September 30, 2020, due to interest incurred on outstanding borrowings under our loan agreement, which was repaid in October 2020.

Change in success fee obligation

The fair value of the success fee obligation included in our loan agreement increased during three months ended September 30, 2020 by \$0.3 million.

Comparison of the nine months ended September 30, 2021 and 2020

The following table summarizes our results of operations for the nine months ended September 30, 2021 and 2020:

(in thousands)	Nine Months Ended September 30,		Change
	2021	2020	
Operating expenses:			
Research and development	\$ 79,007	\$ 23,102	\$ 55,905
General and administrative	19,058	6,945	12,113
Total operating expenses	98,065	30,047	68,018
Loss from operations	(98,065)	(30,047)	(68,018)
Other income (expense):			
Interest income	560	24	536
Interest expense	—	(315)	315
Change in success fee obligation	—	(450)	450
Total other income (expense), net	560	(741)	1,301
Net loss	\$ (97,505)	\$ (30,788)	\$ (66,717)

Research and development expenses

The following table summarizes our research and development expenses for the nine months ended September 30, 2021 and 2020:

(in thousands)	Nine Months Ended September 30,		Change
	2021	2020	
Personnel-related	\$ 10,748	\$ 4,808	\$ 5,940
Stock-based compensation expense	5,141	984	4,157
Laboratory supplies and research materials	2,004	2,181	(177)
External manufacturing and research	55,186	12,724	42,462
Professional and consulting fees	2,115	804	1,311
Facility-related and other	3,813	1,601	2,212
Total research and development expenses	\$ 79,007	\$ 23,102	\$ 55,905

The increase in personnel-related costs and stock-based compensation expense was primarily due to increased headcount in our research and development function. The increase in facility-related and other expenses was primarily due to the increased costs of supporting a larger number of research and development personnel and their research efforts. The increase in external manufacturing and research costs and professional and consulting fees was primarily due to the development of our manufacturing process, increased research activity, and the advancement of our programs and product candidates, including conducting IND-enabling studies.

General and administrative expenses

The following table summarizes our general and administrative expenses for the nine months ended September 30, 2021 and 2020:

(in thousands)	Nine Months Ended September 30,		Change
	2021	2020	
Personnel-related	\$ 4,737	\$ 2,015	\$ 2,722
Stock-based compensation expense	6,388	1,392	4,996
Professional and consulting fees	5,319	2,682	2,637
Facility-related and other	2,614	856	1,758
Total general and administrative expenses	\$ 19,058	\$ 6,945	\$ 12,113

The increase in personnel-related costs and stock-based compensation expense 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 the costs of operating as a public company. The increase in facility-related and other expenses was primarily due to the increased costs of supporting a larger number of general and administrative personnel.

Interest income

Interest income for the nine months ended September 30, 2021 and 2020 was \$0.6 million and less than \$0.1 million, respectively, due to interest earned on invested cash balances.

Interest expense

We did not incur any interest expense in the nine months ended September 30, 2021. Interest expense was \$0.3 million for the nine months ended September 30, 2020, due to interest incurred on outstanding borrowings under our loan agreement, which was repaid in October 2020.

Change in success fee obligation

The fair value of the success fee obligation included in our loan agreement increased during nine months ended September 30, 2020 by \$0.5 million.

Liquidity and capital resources

Sources of liquidity

Since our inception, we have incurred significant operating losses. We expect to incur significant expenses and operating losses for the foreseeable future as we support our continued research activities and development of our programs and platform. We have not yet commercialized any product candidates, and we do not expect to generate revenue from sales of any product candidates for several years, if at all. To date, we have funded our operations primarily with proceeds from sales of equity securities, most recently, with proceeds from the sale of common stock in our IPO and the January 2021 offering, and borrowings under our loan agreement which we have since repaid. As of September 30, 2021 we had cash, cash equivalents and marketable securities of \$407.5 million.

In November 2021, we filed a universal shelf registration on Form S-3 to register for sale from time to time up to \$400.0 million of common stock, preferred stock, debt securities, warrants and/or units in one or more offerings. Further, in November 2021, we entered into an Open Market Sale AgreementSM with Jefferies LLC, or Jefferies, pursuant to which, from time to time, we may offer and sell shares of our common stock having an aggregate offering price of up to \$150.0 million after such time as the universal shelf registration statement is declared effective by the SEC. Sales of common stock through Jefferies may be made by any method that is deemed an "at-the-market" offering as defined in Rule 415(a)(4) under the Securities Act of 1933, as amended.

Cash flows

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

<i>(in thousands)</i>	Nine Months Ended September 30,	
	2021	2020
Net cash used in operating activities	\$ (91,495)	\$ (26,472)
Net cash used in investing activities	(137,657)	(438)
Net cash provided by financing activities	157,732	391,884
Net increase (decrease) in cash, cash equivalents and restricted cash	\$ (71,420)	\$ 364,974

Operating activities

During the nine months ended September 30, 2021, operating activities used \$91.5 million of cash, due to our net loss of \$97.5 million and net cash used by changes in our operating assets and liabilities of \$8.1 million, partially offset by non-cash charges of \$14.1 million. Net cash used by changes in our operating assets and liabilities consisted of a \$12.8 million increase in prepaid expenses and other current assets and a \$2.9 million increase in prepayments made for lessor-owned lease assets, partially offset by a \$7.7 million increase in accounts payable and other liabilities. During the nine months ended September 30, 2020, operating activities used \$26.5 million of cash, due to our net loss of \$30.8 million, partially offset by non-cash charges of \$3.3 million and net cash provided by changes in our operating assets and liabilities of \$1.0 million. Net cash provided by changes in our operating assets and liabilities primarily consisted of a \$1.1 million increase in accounts payable and other liabilities, partially offset by a \$0.1 million increase in prepaid expenses and other current assets. 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 nine months ended September 30, 2021, net cash used in investing activities was \$137.7 million due to the purchase of marketable securities of \$208.2 million and purchases of property and equipment of \$1.6 million, partially offset by maturities of marketable securities of \$72.2 million. During the nine months ended September 30, 2020, net cash used in investing activities was \$0.4 million, due to purchases of property and equipment.

Financing activities

During the nine months ended September 30, 2021, net cash provided by financing activities was \$157.7 million, consisting primarily of \$157.2 million in aggregate net proceeds from our follow-on offering completed in January 2021.

During the nine months ended September 30, 2020, net cash provided by financing activities was \$391.9 million, consisting of \$19.5 million in net proceeds from our issuance of Series A preferred stock, \$115.3 million in net proceeds from our issuance of Series B preferred stock, \$246.4 million in net proceeds from our IPO and borrowings of \$10.0 million under our 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 and 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 our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2024. We expect that our liquidity will be sufficient to enable us to submit INDs for product candidates in each of our DM1, DMD and FSHD programs and achieve proof-of-concept data readouts in our initial clinical trials for our DM1, DMD, and FSHD programs. We have based these estimates 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, the ownership interest of our stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect the rights of holders of our common stock. 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. See Item 1A. "Risk factors" in this Quarterly Report for additional risks associated with our substantial capital requirements.

Contractual obligations

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.

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. 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.

In May 2019, we entered into a sublease agreement for our laboratory and office space. The term of the sublease commenced on July 1, 2019 and expires on December 31, 2021. Our lease payments for the remainder of the term of the sublease will be \$0.1 million per month.

On December 4, 2020, we entered into a lease agreement for office and laboratory space, which was amended in January 2021, March 2021, and June 2021. The lease has a term of 8.5 years that commenced when we gained access to the office and laboratory space in September, 2021. Our obligation for the payment of the base rent begins in April 2022 and will be \$0.4 million per month, increasing up to \$0.5 million during the term of the lease. We have two options to extend the term of the lease, each for a period of an additional five years.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our condensed consolidated 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.

We define our critical accounting policies as those accounting principles generally accepted in the United States of America that are most critical to the judgments and estimates used in the preparation of our condensed consolidated financial statements. While our significant accounting policies are described in more detail in Note 2 "Summary of Significant Accounting Policies" to our condensed consolidated financial statements, we believe that our most critical accounting policies are those relating to accrued research and development expenses and stock-based compensation.

There have been no significant changes to our critical accounting policies from those described in our Annual Report on Form 10-K filed with the SEC on March 4, 2021.

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 SEC.

Recently Issued and Adopted Accounting Pronouncements

There were no recently issued accounting pronouncements that are expected to materially impact our financial statements as of September 30, 2021.

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” as defined in Rule 12b-2 under the Exchange Act. We may continue to be a smaller reporting company if either (i) the market value of our shares held by non-affiliates is less than \$250 million or (ii) our annual revenue is 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 as of the last business day of our most recently completed second fiscal quarter. 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.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

Not Required.

Item 4. Controls and Procedures.***Evaluation of Disclosure Controls and Procedures***

Our management, under the supervision and with the participation of our Principal Executive Officer (our Chief Executive Officer) and Principal Financial Officer (our Vice President of Accounting and Administration, Treasurer), has evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2021. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2021, our Principal Executive Officer and Principal Financial Officer have concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2021 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION**Item 1A. Risk Factors.**

Our business is subject to numerous risks. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this Quarterly Report on Form 10-Q, or this Quarterly Report, including our condensed consolidated financial statements and the related notes thereto in evaluating our company. 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.

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 \$30.8 million for the nine months ended September 30, 2020 and \$97.5 million for the nine months ended September 30, 2021. As of September 30, 2021, we had an accumulated deficit of \$176.7 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 and product candidates. Our product candidates are in varying stages of preclinical development, and we have not commenced or completed clinical development of any product candidates. 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 lead programs in DM1, DMD and FSHD and conduct research programs in additional indications;
- advance our product candidates and any additional product candidates we identify through and into investigational new drug; or 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.

Our product candidates are in varying stages of preclinical development, we have not advanced any product candidate into clinical development and we 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. Our product candidates are only in the preclinical stage of development. 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 our stockholders to lose all or part of their investment.

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 activities 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, we expect to continue 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 September 30, 2021, we had cash, cash equivalents and marketable securities of \$407.5 million. We believe that our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements into the second half of 2024. 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 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 candidates 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 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 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.

Raising additional capital may cause dilution to our stockholders, 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, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of common stockholders. 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 eliminate some or all of our research and development programs, pipeline expansion 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 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. Our product candidates are in varying stages of preclinical 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 our stockholders 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 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, 2020, we had federal NOL carryforwards of \$69.1 million and state NOL carryforwards of \$68.9 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 completed a Section 382 study of transactions in our stock through December 31, 2020 and concluded that we have experienced ownership changes since inception that we believe under Section 382 and 383 of the Code will result in limitation on our ability to use certain pre-change NOLs and credits. In addition, we may experience subsequent ownership changes as a result of future equity offerings or other changes in the ownership of our stock, some of which are beyond 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. Our product candidates are in varying stages of preclinical development, and we have not advanced any product candidate into clinical development. 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 and our lead programs. Our product candidates are in varying stages of preclinical development. We have not advanced any product candidates to clinical-stage development and may never do so. 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 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 any jurisdiction where we seek to commercialize such product candidates, such as 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 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 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 product candidates 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 of our product candidates, 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 candidates are still in varying stages of preclinical development and our approach to treating muscle disease is unproven. Our research programs may fail to identify additional 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 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 product candidates and programs are based on our FORCE platform, adverse developments with respect to one of our product candidates and programs may have a significant adverse impact on the actual or perceived likelihood of success and value of our other product candidates and programs.

In addition, we have not advanced any product candidates for clinical development or successfully developed any product candidate and our ability to identify and develop a product candidates may never materialize. The process by which we identify and discover 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 product candidates obsolete or less attractive;
- product candidates we develop may nevertheless be covered by third parties' patents or other intellectual property rights;
- 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;
- product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a product candidate may change so that the continued development of that product candidate is no longer reasonable;

- a 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 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, programs and product candidates, and have not advanced any product candidates into clinical development. 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 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 Fabs, the linker and oligonucleotide payloads. 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. We expect to continue to depend on third-party suppliers for the manufacture of any product candidates that we evaluate in preclinical studies and clinical trials, 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.

Our product candidates 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 candidate 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 “Risk Factors” section 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 research programs and product candidates 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 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 and product candidates. 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- β inhibitor in late-stage clinical development by AMO Pharma Ltd. for the congenital phenotype of DM1; pitolisant, a selective histamine 3 receptor antagonist / inverse agonist currently being evaluated in a Phase 2 clinical trial for non-muscular symptoms of DM1 by Harmony Biosciences; AT466, which is an AAV-antisense candidate in preclinical development by Audentes Therapeutics, Inc.; AOC-1001, an antibody linked siRNA being evaluated in a Phase 1/2 clinical trial by Avidity Biosciences, Inc.; gene editing treatments in preclinical development by Biogen, Inc. and Vertex Pharmaceuticals, Inc., or Vertex; an RNA-targeting gene therapy in preclinical development by Locana, Inc.; small molecules interacting with RNA in preclinical development by Expansion Therapeutics, Inc.; therapeutics based on biomolecular condensate biology in preclinical development by Dewpoint Therapeutics, Inc. and Faze Medicines, Inc.; gene targeted chimera small molecules in preclinical development by Design Therapeutics; EDODM1, a peptide-linked PMO in preclinical development by Pepgen, Inc.; NT0200, a peptide-nucleic acid (PNA) in preclinical development by Neubase Therapeutics; and antisense oligonucleotides and siRNA candidates by Triplet Therapeutics.

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 four FDA-approved exon skipping drugs: EXONDYS 51 (eteplirsen), VYONDYS 53 (golodirsen) and AMONDYS 45 (casimersen), which are naked PMOs approved for the treatment of DMD patients amenable to Exon 51, Exon 53 and Exon 45 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, Wave Life Sciences Ltd. with WVE-N531, a stereopure oligonucleotide currently being evaluated in a Phase 1 clinical trial for patients amenable to Exon 53 skipping, PTC with ataluren, a small molecule targeting nonsense mutations in a Phase 3 clinical trial, Entrada with endosomal escape vehicle technology which targets dystrophin production, Pepgen, Inc., with EDO51, a peptide-linked PMO for patients amenable to Exon 51 skipping which is in preclinical development; BioMarin Pharmaceuticals, Inc. with BMN 351, an oligonucleotide therapy that targets dystrophin production which is in preclinical development and Avidity Biosciences, Inc. with AOC 1044, an antibody oligonucleotide conjugate that targets dystrophin production for patients amenable to Exon 44 skipping which is in preclinical development. 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), Solid Biosciences Inc. (SGT-001) and REGENXBIO Inc. 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: ARO-DUX4, an siRNA therapy in preclinical development by Arrowhead Pharmaceuticals, AOC FSHD, an antibody oligonucleotide conjugate in preclinical development by Avidity Biosciences, Inc., MC-DX4, a microRNA targeting therapy in preclinical development by miRecale, Inc., 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 Alynham Pharmaceuticals, Aro Biotherapeutics, Arrowhead Therapeutics, Avidity Biosciences, Dicerna Pharmaceuticals, Inc., DTx Pharma, Gennao Bio, Inc., Ionis Pharmaceuticals, NeuBase Therapeutics, Inc. 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

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 patents and 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 which we own and two issued U.S. patents 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 be 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 the University of Mons, or 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.

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 product candidates 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.

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, logos and tagline. 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.

Additionally, we could face heightened risks with respect to seeking marketing approval in the United Kingdom as a result of the recent withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the United Kingdom and the European Union, the United Kingdom withdrew from the European Union, effective December 31, 2020. On December 24, 2020, the United Kingdom and the European Union entered into a Trade and Cooperation Agreement. The agreement sets out certain procedures for approval and recognition of medical products in each jurisdiction.

Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of the Trade and Cooperation Agreement or otherwise, would prevent us from commercializing any product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or the European Union 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 cGMP 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.

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, and civil monetary penalty laws 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 and their subcontractors 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 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.

Legislative and regulatory changes 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 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, and other COVID-19 relief legislation suspended the 2% Medicare sequester from May 1, 2020 through March 31, 2021. In January 2013, the American Taxpayer Relief Act of 2012 became law, which, 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 Act, which was signed by President Trump on December 22, 2017, Congress effectively repealed the "individual mandate" by reducing the applicable penalty to zero dollars. The modification 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 eliminated 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 Trump Administration also took executive actions to undermine or delay implementation of the ACA. During the Trump presidency, the president 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. 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.

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 November 10, 2020, the United States Supreme Court heard oral argument in this case. On June 17, 2021, the Supreme Court rejected this challenge to the ACA after finding that plaintiffs do not have standing to challenge the constitutionality of the statute. 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.

In addition, President Trump issued several executive orders intended to lower the costs of prescription drug products. Certain of these orders are reflected in recently promulgated regulations, including an interim final rule implementing President Trump's most favored nation model, but such final rule is currently subject to a nationwide preliminary injunction. The Biden Administration has frozen certain of the previous administration's measures to reform drug prices, pending further review. It remains to be seen how the Biden Administration will address this issue but, under Medicare Part D, the new administration may seek to establish a ceiling for the launch prices of all branded, biologic, and certain generic drugs by referencing the average price of these drugs in other developed countries. At the same time, the administration may seek to limit Medicare Part D and public option drug prices through a tax penalty on manufacturers for increases in the cost of drugs and biologics above the general inflation rate. The American Rescue Plan Act of 2021, comprehensive COVID-19 relief legislation recently enacted under the Biden administration, includes a number of healthcare-related provisions, such as support to rural health care providers, increased tax subsidies for health insurance purchased through insurance exchange marketplaces, financial incentives to states to expand Medicaid programs and elimination of the Medicaid drug rebate cap effective in 2024.

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.

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 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

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; and continues to have effect in amended form in the United Kingdom by operation of the so-called “UK GDPR” (i.e., the GDPR as it continues to form part of law in the United Kingdom by virtue of section 3 of the European Union (Withdrawal) Act 2018, as amended (including by the various Data Protection, Privacy and Electronic Communications (Amendments etc) (EU Exit) Regulations)). 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 and United Kingdom 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 and United Kingdom to the United States. Switzerland has adopted similar restrictions. Although there are legal mechanisms 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, or CJEU, invalidated Decision 2016/1250 on the adequacy of the protection provided by the EU-U.S. Privacy Shield Framework, in a case known colloquially as “Schrems II”. Following this decision, the Swiss Federal Data Protection and Information Commissioner, or the FDPIC, announced that the Swiss-U.S. Privacy Shield does not provide adequate safeguards for the purposes of personal data transfers from Switzerland to the United States. While the FDPIC does not have authority to invalidate the Swiss-U.S. Privacy Shield regime, the FDPIC’s announcement casts doubt on the viability of the Swiss-U.S. Privacy Shield as a future compliance mechanism for Swiss-U.S. data transfers. Following the decision in Schrems II, the United Kingdom government has also invalidated use of the EU-U.S. Privacy Shield as a mechanism for lawful transfers of personal data from the United Kingdom to the United States under the UK GDPR. The CJEU’s decision in Schrems II also raised questions about whether one of the primary alternatives to the EU-U.S. Privacy Shield, namely, the European Commission’s Standard Contractual Clauses, can lawfully be used for personal data transfers from Europe to the United States or other third countries that are not the subject of an adequacy decision of the European Commission. While the CJEU upheld the adequacy of the Standard Contractual Clauses in principle in Schrems II, it made clear that reliance on those Clauses alone may not necessarily be sufficient in all circumstances. Use of the Standard Contractual Clauses must now be assessed on a case-by-case basis taking into account the legal regime applicable in the destination country, in particular regarding applicable surveillance laws and relevant rights of individuals with respect to the transferred data. In the context of any given transfer, where the legal regime applicable in the destination country may or does conflict with the intended operation of the Standard Contractual Clauses and/or applicable European law, the decision in Schrems II and subsequent draft guidance from the European Data Protection Board, or EDPB, would require the parties to that transfer to implement certain supplementary technical, organizational and/or contractual measures to rely on the Standard Contractual Clauses as a compliant ‘transfer mechanism.’ However, the aforementioned draft guidance from the EDPB on such supplementary technical, organizational and/or contractual measures appears to conclude that no combination of such measures could be sufficient to allow effective reliance on the Standard Contractual Clauses in the context of transfers of personal data ‘in the clear’ to recipients in countries where the power granted to public authorities to access the transferred data goes beyond that which is ‘necessary and proportionate in a democratic society’ — which may, following the CJEU’s conclusions in Schrems II on relevant powers of United States public authorities and commentary in that draft EDPB guidance, include the United States in certain circumstances (e.g., where Section 702 of the US Foreign Intelligence Surveillance Act applies). At present, there are few, if any, viable alternatives to the EU-U.S. Privacy Shield and the Standard Contractual Clauses. As such, if we are unable to implement a valid solution for personal data transfers from Europe, including, for example, obtaining individuals’ explicit consent to transfer their personal data from Europe to the United States or other countries, we will face increased exposure to regulatory actions, substantial fines and injunctions against processing personal data from Europe. Inability to import personal data from the EEA, United Kingdom or Switzerland may also restrict our clinical trials activities in Europe; limit our ability to collaborate with contract research organizations as well as other service providers, contractors and other companies subject to European data protection laws; and require us to

increase our data processing capabilities in Europe at significant expense. Additionally, other countries outside of Europe have enacted or are considering enacting similar cross-border data transfer restrictions and laws requiring local data residency, which could increase the cost and complexity of delivering our services and operating our business.

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. Furthermore, following the United Kingdom's withdrawal from the European Union on January 31, 2020, pursuant to the transitional arrangements agreed to between the United Kingdom and European Union, the GDPR continued to have effect in law in the United Kingdom, and continued to do so until December 31, 2020 as if the United Kingdom remained a Member State of the European Union for such purposes. Following December 31, 2020, and the expiry of those transitional arrangements, the data protection obligations of the GDPR continue to apply to United Kingdom-related processing of personal data in substantially unvaried form under the UK GDPR. However, going forward, there will be increasing scope for divergence in application, interpretation and enforcement of the data protection law as between the United Kingdom and EEA. Furthermore, the relationship between the United Kingdom and the EEA in relation to certain aspects of data protection law remains unclear. For example, it is still unclear whether the transfer of data from the EEA to the United Kingdom will in the future remain lawful under the GDPR. For the meantime, under the post-Brexit Trade and Cooperation Agreement between the European Union and the United Kingdom, it has been agreed, that transfers of personal data to the United Kingdom from European Union Member States will not be treated as "restricted transfers" to a non-EEA country for a period of up to four months from January 1, 2021, which may be extended by a further two months — such period, the adequacy assessment period. This will also apply to transfers to the United Kingdom from EEA Member States, assuming those Member States accede to the relevant provision of the Trade and Cooperation Agreement. Although the current maximum duration of the adequacy assessment period is six months it may end sooner, for example, in the event that the European Commission adopts an adequacy decision in respect of the United Kingdom, or the United Kingdom amends the UK GDPR and/or makes certain changes regarding data transfers under the UK GDPR or the Data Protection Act 2018 without the consent of the European Union (unless those amendments or decisions are made simply to keep relevant United Kingdom laws aligned with the European Union's data protection regime). Unless the European Commission makes an 'adequacy finding' in respect of the United Kingdom prior to the expiry of the adequacy assessment period, from that point onwards the United Kingdom will be an 'inadequate third country' under the GDPR and transfers of data from the EEA to the United Kingdom will require a 'transfer mechanism,' such as the European Commission's Standard Contractual Clauses issued and approved from time-to-time. Additionally, the transposition of the GDPR into United Kingdom domestic law by way of the UK GDPR could expose us to two parallel regimes, each of which potentially authorizes similar fines and other potentially divergent enforcement actions for certain violations. In addition to such parallel United Kingdom and European Union regimes, following the expiry of the post-Brexit transitional arrangements agreed between the United Kingdom and European Union, the United Kingdom Information Commissioner's Office is not able to be our 'lead supervisory authority' in respect of any 'cross border processing' for the purposes of the GDPR. In the event that we are unable to, and/or do not, designate a lead supervisory authority in an EEA Member State, with effect from January 1, 2021, we are not able to benefit from the GDPR's 'one stop shop' mechanism. Amongst other things, this would mean that, in the event of a violation of the GDPR affecting data subjects across the United Kingdom and the EEA, we could be investigated by, and ultimately fined by the United Kingdom Information Commissioner's Office and the supervisory authority in each and every EEA member state where data subjects have been affected by such violation. 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. Additionally, effective starting on January 1, 2023, the California Privacy Rights Act, or the CPRA, will significantly modify the CCPA, including by expanding consumers' rights with respect to certain sensitive personal information. The CPRA also creates a new state agency that will be vested with authority to implement and enforce the CCPA and the CPRA. 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 September 30, 2021, we had 88 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 trials 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 to supply chains and may adversely impact economies worldwide, which could negatively impact our operations.

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 countries worldwide, including the United States. In March 2020, the World Health Organization declared the COVID-19 outbreak a pandemic. The COVID-19 pandemic caused 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 may 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 ownership of our common stock and our status as a public company

We do not know whether an active trading market will continue to develop or be sustained for our common stock and, as a result, it may be difficult for our stockholders to sell their shares of our common stock.

Our common stock began trading on the Nasdaq Global Select Market on September 17, 2020. Prior to September 17, 2020, there was no public market for our common stock, and we cannot assure you that an active trading market for our shares will continue to develop or be sustained. As a result, it may be difficult for our stockholders to sell their shares without depressing the market price of our common stock, or at all.

The price of our common stock is volatile and fluctuates substantially, which could result in substantial losses for our stockholders.

Our stock price has been, and is likely to continue 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, our stockholders may not be able to sell their common stock at or above the price they paid for their shares. 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.

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.

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 relies, in part, on the research and reports that industry or financial analysts publish about us or our business. We do not have control over these analysts. There can be no assurance that existing analysts will continue to cover us or that new analysts will begin to cover us. There is also no assurance that any covering analysts will provide favorable 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.

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. 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.

Our executive officers and directors and their affiliates, if they choose to act together, have the ability to significantly influence all matters submitted to stockholders for approval.

Our executive officers and directors and their affiliates, in the aggregate, beneficially owned shares representing approximately 43.0% of our common stock as of September 30, 2021. As a result, if these stockholders were to choose to act together, they would be able to significantly influence 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 significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of ownership may:

- delay, defer or prevent a merger, consolidation or sale of all or substantially all of our assets that may be desired by other stockholders;
- delay, defer or prevent a change in control transaction involving us that other stockholders may desire; or
- entrench our management and board of directors.

We have broad discretion in the use of our cash and cash equivalents and may not use them effectively.

Our management has broad discretion in the application of our cash, cash equivalents and marketable securities and could use such funds in ways that do not improve our results of operations or enhance the value of our common stock or in ways that our stockholders may not agree with. 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 these funds 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 the sole source of gain for our stockholders.

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 the sole source of gain for our stockholders for the foreseeable future.

A significant portion of our total outstanding shares 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 or impair our ability to raise capital through the sale of equity securities in the future. As of October 31, 2021, we had 51,528,469 shares of common stock outstanding.

All of our outstanding shares of common stock are available for sale in the public market, subject to applicable securities laws.

Moreover, holders of a substantial number of shares of our common stock and shares of our common stock issuable upon exercise of outstanding options have rights, 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 have also filed a registration statement on Form S-8 to register all of the shares of common stock that we are able to issue under our equity compensation plans. Shares registered under these registration statements on Form S-8 can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates, vesting arrangements and exercise of options.

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 December 31, 2025, 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:

- 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 our periodic reports and proxy statements. 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.

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 have incurred and will continue to incur increased costs as a result of operating as a public company, and our management has devoted and will continue to 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 previously 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 devote and will need to continue 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 compared to when we were a private company. 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, we will be required to furnish a report by our management on our internal control over financial reporting beginning with our filing of an Annual Report on Form 10-K with the SEC for the year ended December 31, 2021. 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 are 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 are required to disclose changes made in our internal controls and procedures on a quarterly basis and our management is 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.

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 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 our stockholders might otherwise receive a premium for their 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.

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 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 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 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.

General Risk Factors

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.

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 and COVID relief provisions were included in the Consolidated Appropriations Act 2021, or CAA, which was enacted on December 27, 2020. Each of these laws contains 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, the CARES Act and the CAA 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, the CARES Act or the CAA.

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

We are 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

On September 21, 2020, we closed our initial public offering, or IPO, of our common stock pursuant to which we issued and sold 14,089,314 shares of our common stock, including 1,837,736 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares at a price to the public of \$19.00 per share for aggregate gross proceeds of \$267.7 million.

All of the shares issued and sold in the IPO were registered under the Securities Act of 1933, as amended, pursuant to a Registration Statement on Form S-1 (File No. 333-248414), which was declared effective by the SEC on September 16, 2020. J.P. Morgan Securities LLC, Jefferies LLC, Piper Sandler & Co. and Stifel, Nicolaus & Company, Incorporated acted as joint book-running managers for our IPO.

We received aggregate net proceeds of approximately \$246.4 million after deducting underwriting discounts and commissions and offering expenses payable by us.

As of September 30, 2021, we have used approximately \$123.9 million of the net proceeds from the IPO for the continued research and development of our programs, continued development and enhancement of our proprietary FORCE platform and for working capital and other general corporate purposes. There has been no material change in our planned use of the net proceeds from the offering as described in our final prospectus filed pursuant to Rule 424(b)(4) under the Securities Act with the SEC on September 17, 2020.

Item 3. Defaults Upon Senior Securities.

Not Applicable.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

Exhibit Number	Description
31.1*	<u>Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
31.2*	<u>Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</u>
32.1†	<u>Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
32.2†	<u>Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</u>
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	The cover page from the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, has been formatted in Inline XBRL.

* Filed herewith.

† The certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report, are deemed furnished and not filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of Dyne Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

DYNE THERAPEUTICS, INC.

Date: November 4, 2021

By:

/s/ Joshua Brumm

Joshua Brumm

President and Chief Executive Officer
(Principal Executive Officer)

Date: November 4, 2021

By:

/s/ Richard Scalzo

Richard Scalzo
Vice President, Accounting and Administration and Treasurer

(Principal Financial and Accounting Officer)

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Joshua Brumm, hereby certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Dyne Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2021

/s/ Joshua Brumm

Joshua Brumm
President and Chief Executive Officer
(Principal Executive Officer)

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002**

I, Richard Scalzo, hereby certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Dyne Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
 - (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - (b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 4, 2021

/s/ Richard Scalzo

Richard Scalzo
Vice President of Accounting and
Administration and Treasurer
(Principal Financial and Accounting Officer)

**CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

I, Joshua Brumm, certify pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge, the Quarterly Report on Form 10-Q of Dyne Therapeutics, Inc. for the fiscal quarter ended September 30, 2021 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Dyne Therapeutics, Inc.

/s/ Joshua Brumm

Joshua Brumm

President and Chief Executive Officer

(Principal Executive Officer)

November 4, 2021

**CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

I, Richard Scalzo, certify pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge, the Quarterly Report on Form 10-Q of Dyne Therapeutics, Inc. for the fiscal quarter ended September 30, 2021 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Dyne Therapeutics, Inc.

/s/ Richard Scalzo

Richard Scalzo

Vice President of Accounting and Administration and
Treasurer

(Principal Financial and Accounting Officer)

November 4, 2021
