

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of report (Date of earliest event reported): January 12, 2026**

**Dyne Therapeutics, Inc.**  
(Exact Name of Registrant as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-39509**  
(Commission  
File Number)

**36-4883909**  
(IRS Employer  
Identification No.)

**1560 Trapelo Road**  
**Waltham, Massachusetts**  
(Address of Principal Executive Offices)

**02451**  
(Zip Code)

**Registrant's telephone number, including area code: (781) 786-8230**

**Not applicable**  
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

Title of each class	Trading symbol(s)	Name of each exchange on which registered
<b>Common stock, \$0.0001 par value per share</b>	<b>DYN</b>	<b>Nasdaq Global Select Market</b>

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

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.

**Item 2.02 Results of Operations and Financial Condition.**

Dyne Therapeutics, Inc. (the “Company”) disclosed on January 12, 2026, that although it has not finalized its full financial results for the fourth quarter and fiscal year ended December 31, 2025, it expects to report cash, cash equivalents and marketable securities of approximately \$1.1 billion as of December 31, 2025.

The estimated cash figure is preliminary and unaudited, represents a management estimate as of the date of this Current Report on Form 8-K and is subject to completion of the Company’s financial closing procedures. The Company’s independent registered public accounting firm has not conducted an audit or review of, and does not express an opinion or any other form of assurance with respect to, the estimated cash figure.

The information furnished under this Item 2.02 shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended (the “Securities Act”), or the Exchange Act, except as expressly set forth by specific reference in such a filing.

**Item 7.01 Regulation FD Disclosure.**

On January 12, 2026, the Company made available a presentation to be used with investors to discuss the Company’s current operations, future plans and other business updates. A copy of the presentation is filed as Exhibit 99.1 hereto and is incorporated herein by reference.

The information furnished under this Item 7.01, including Exhibit 99.1, shall not be deemed “filed” for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act, except as expressly set forth by specific reference in such a filing.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Presentation, dated January 12, 2026</a>
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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**SIGNATURES**

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

DYNE THERAPEUTICS, INC.

Date: January 12, 2026

By: /s/ John G. Cox

Name: John G. Cox

Title: President and Chief Executive Officer

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# Advancing Clinically Validated Platform Toward Commercialization

44<sup>TH</sup> ANNUAL J.P. MORGAN HEALTHCARE CONFERENCE | JANUARY 2026

# Forward-Looking Statements & Disclaimer

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This presentation contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this presentation, including statements regarding Dyne's strategy, future operations, prospects and plans, objectives of management, the potential of the FORCE platform and its conjugates, the therapeutic potential of zelecciment basivarsen (z-basivarsen, also known as DYNE-101), zelecciment rostudirsen (z-rostudirsen, also known as DYNE-251), DYNE-302 and DYNE-401, the anticipated timelines for reporting additional data from the ACHIEVE clinical trial, enrolling registrational cohorts and initiating additional clinical trials, expectations regarding the timing and outcome of interactions with global regulatory authorities and the availability of expedited approval pathways for z-basivarsen and z-rostudirsen, expectations regarding the timing of submitting applications for U.S. Accelerated Approval, plans to provide future updates on pipeline programs, expectations regarding the commercialization of any of Dyne's product candidates, and the sufficiency of Dyne's cash resources for the period anticipated, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," "will" or "would," or the negative of these terms, or other comparable terminology are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Dyne may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the initiation and completion of preclinical studies and clinical trials; uncertainties as to the availability and timing of results from preclinical studies and clinical trials; the timing of and Dyne's ability to enroll patients in clinical trials; whether results from preclinical studies and data from clinical trials will be predictive of the final results of the clinical trials or other trials; whether data from clinical trials will support submission for regulatory approvals; uncertainties as to the FDA's and other regulatory authorities' interpretation of the data from Dyne's clinical trials and acceptance of Dyne's clinical programs and as to the regulatory approval process for Dyne's product candidates; whether Dyne's cash resources will be sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements; as well as the risks and uncertainties identified in Dyne's filings with the Securities and Exchange Commission (SEC), including the Company's most recent Form 10-K and in subsequent filings Dyne may make with the SEC. In addition, the forward-looking statements included in this presentation represent Dyne's views as of the date of this presentation. Dyne anticipates that subsequent events and developments will cause its views to change. However, while Dyne may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Dyne's views as of any date subsequent to the date of this presentation.

This presentation also contains estimates, projections and other statistical data made by independent parties and by Dyne relating to market size and growth and other data about Dyne's industry and business. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. Dyne has not independently verified the accuracy and completeness of the information obtained from third parties included in this presentation. In addition, projections, assumptions and estimates of Dyne's future performance and the future performance of the markets in which Dyne operates are necessarily subject to a high degree of uncertainty and risk.



# Poised to Unlock Significant Commercial Opportunities in Multiple Rare Neuromuscular Diseases

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## LATE-STAGE CLINICAL PIPELINE

- Positive topline results from registrational cohort in DMD
- Ongoing registrational cohort in DM1



## NEAR-TERM VALUE DRIVERS

Steady cadence of expected data readouts and regulatory submissions; first potential commercial launch in Q1 2027



## DIFFERENTIATED PLATFORM

FORCE™ platform enables targeted delivery to muscle and CNS; broader pipeline includes FSHD and Pompe



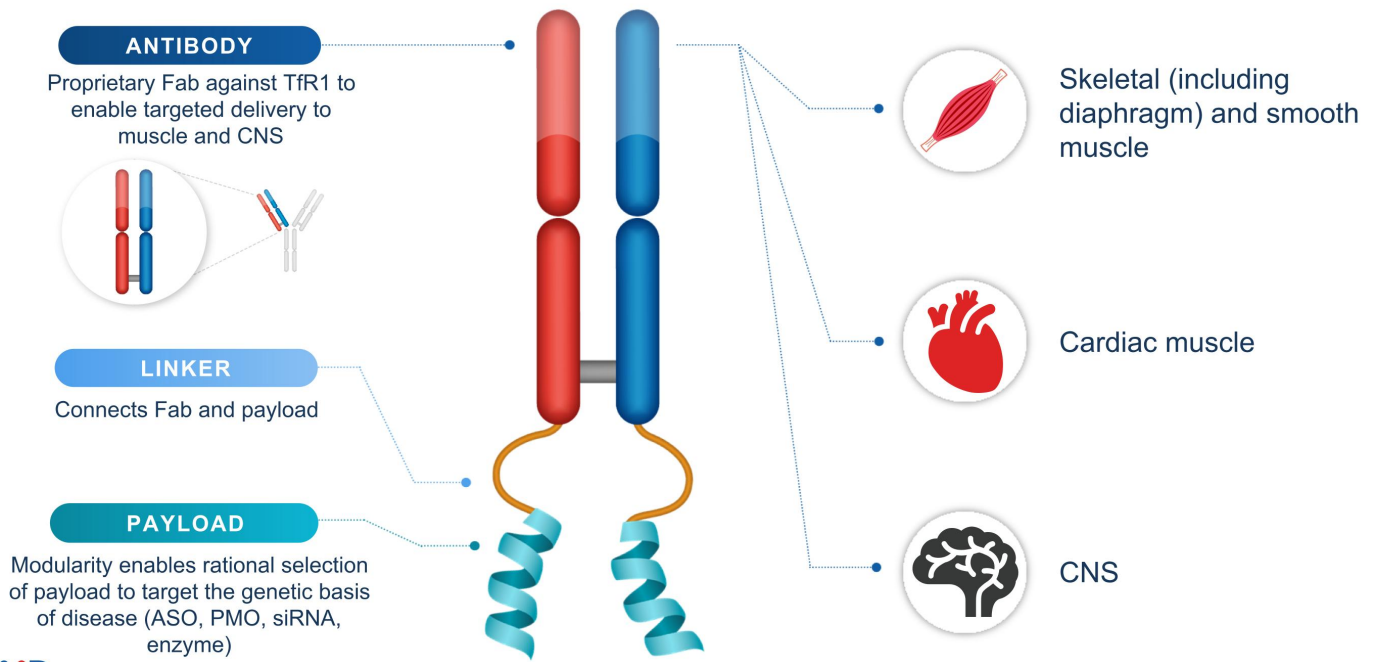
## STRONG FINANCIAL POSITION

Cash position of ~\$1.1 billion (as of 12/31/25)<sup>1</sup> with expected runway into Q1 2028; all assets fully owned



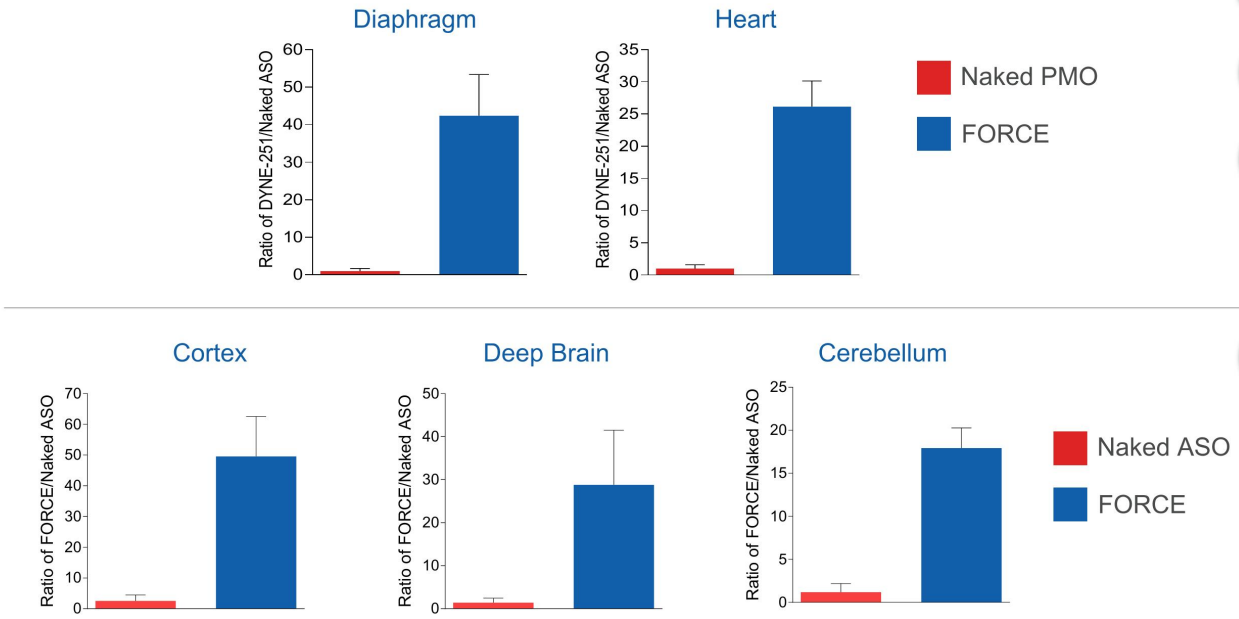
1. Preliminary and unaudited. DMD = Duchenne muscular dystrophy; DM1 = myotonic dystrophy type 1; CNS = central nervous system; FSHD = facioscapulohumeral muscular dystrophy.

# Leveraging Our FORCE™ Platform for Targeted Delivery



Note: Figure depicts oligonucleotide payload; ASO = antisense oligonucleotide; PMO = phosphorodiamidate morpholino oligomer; siRNA = small interfering RNA; CNS = central nervous system.

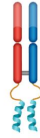
# FORCE Enabled Oligonucleotide Delivery to Muscle and CNS



Notes for PMO: Results after repeat IV dose of naked ASO or DYNE-251 in male cynomolgus monkeys, 2 x 30 mg/kg on day 0 and day 7, analyzed day 28; n = 4 – 5.  
 Notes for ASO: Results after a single IV dose of naked ASO or FORCE in male cynomolgus monkeys, 1 x 10 mg/kg on day 0, analyzed day 3; n = 2. FORCE conjugate consists of lead Fab and surrogate *DMPK*-targeting payload.

# Potential of the FORCE Platform Validated by Recent Z-Rostudirsen Topline Clinical Results

## FORCE



### Design Principles of the FORCE Platform

TfR1-mediated delivery to muscle, including diaphragm and heart, and CNS with rationally selected payload to match disease biology

TfR1-binding Fab to enable robust and widespread tissue distribution

Designed not to interfere with TfR1 function in iron homeostasis

Achievement of target profile with infrequent dosing

### Validation with Z-Rostudirsen DELIVER Data

Statistically significant and robust increase in dystrophin

Early and sustained functional improvement across multiple clinical endpoints

Favorable safety and tolerability<sup>1</sup> with no persistent related anemia<sup>2</sup> or thrombocytopenia at 20 mg/kg

Convenient Q4W dosing

## Dyne's pipeline programs utilize the same TfR1-binding Fab



1. Data as of August 19, 2025. 2. No participants have demonstrated persistent related anemia with Hgb levels <11.2 g/dL (threshold for anemia in children (ref: Powers JM. Approach to the child with anemia. UpToDate, Connor RF (Ed), Wolters Kluwer. Accessed December 2, 2025)). TfR1, transferrin receptor 1; CNS, central nervous system; Fab, fragment antibody-binding; Q4W, every 4 weeks.

# Neuromuscular Pipeline Leveraging Clinically Validated Platform

DISEASE	TARGET	PRECLINICAL	PHASE 1/2	ESTIMATED PATIENTS
Myotonic dystrophy type 1 (DM1)	DMPK	zeleciment basivarsen (z-basivarsen, also known as DYNE-101)		US: ~40,000 EU: ~55,000
Duchenne muscular dystrophy (DMD)	Exon 51	zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251)		US: ~12,000 EU: ~16,000
	Exon 53			
	Exon 45			
	Exon 44			
	Other Exons			
Facioscapulohumeral muscular dystrophy (FSHD)	DUX4	DYNE-302		US: ~15,000 – 40,000 EU: ~20,000 – 50,000
Pompe disease	GAA	DYNE-401		US: ~4,500 EU: ~5,500
<b>PIPELINE EXPANSION OPPORTUNITIES</b>				
CNS, Rare skeletal, Cardiac, Metabolic				



## Broad and Durable Functional Improvement Observed with Dyne's First Clinically Validated Program: Z-Rostudirsen in DMD



# Exon 51 Skip Amenable DMD: A More Severe Duchenne Population with Significant Unmet Need, Despite Approved Therapies



## DMD Population

- ~12,000 (US)
- ~16,000 (EU)
- ~ 13% is exon 51 skip amenable<sup>1</sup>



## Clinical Presentation

- Mutation in *DMD* gene for dystrophin
  - Exon 51 skip amenable DMD is a particularly challenging form
- Muscle weakness and gait abnormalities
- Progressive loss of function
- Cognitive issues
- Respiratory/cardiac failure
- Life expectancy ~30 years<sup>2</sup>



## Current Treatment Limitations

- Limited delivery to muscle and CNS
- High burden due to weekly IV dosing<sup>3</sup>
- <1% dystrophin production with exon 51 skipping therapy<sup>3</sup>
- Microdystrophin lacks domains key for optimal functionality<sup>4</sup>
- Unknown durability and inability to redose with gene therapy
- Safety considerations



## OUR APPROACH

### Potential Best-in-class Targeted Exon Skipping

Increase dystrophin expression and enable less frequent dosing to deliver **functional improvement**



1. Aartsma-Rus et al. *Hum Mutat.* 2009;30:293-299 2. Broomfield et al. *Neurology*, 2021;97:e2304-e2314; 3. Exondys 51 Prescribing Information; 4. Chamberlain et al. *Hum Gene Ther* 2023;34:404-15. DMD, Duchenne muscular dystrophy, CNS, central nervous system; Q1W, every 1 week.

# DELIVER Study to Support Accelerated Approval of Z-Rostudirsen in DMD



Selection of registrational dose (20 mg/kg Q4W) based on multiple ascending dose (MAD) data



Registrational Expansion Cohort met primary endpoint of statistically significant and robust increase in dystrophin at 6 months ( $p < 0.0001$ )



Functional improvement observed across multiple clinical measures out to 24 months



Favorable safety profile<sup>1</sup>



Planned submission for U.S. Accelerated Approval based on positive results from Registrational Expansion Cohort

## Phase 3 study planned to support full approval of z-rostudirsen globally

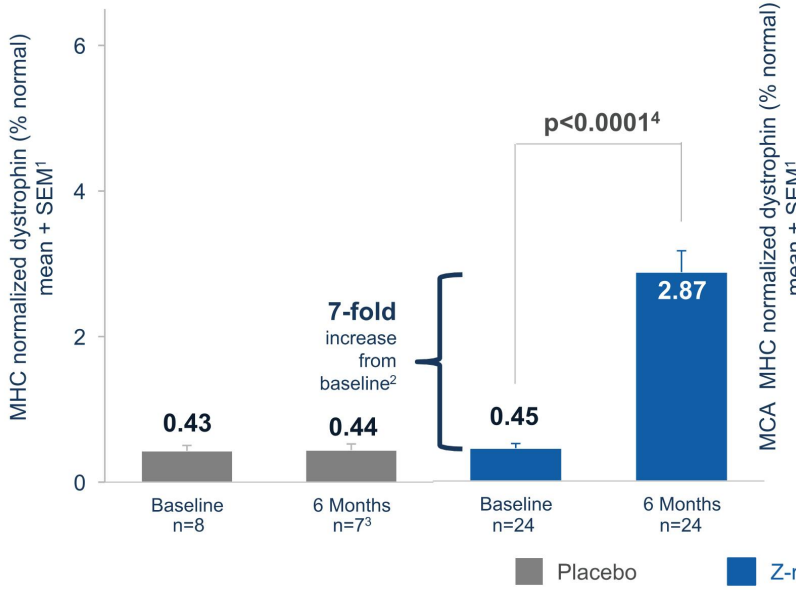


Note: DELIVER is a Phase 1/2 global placebo-controlled clinical trial evaluating multiple doses of z-rostudirsen in ambulant and non-ambulant males with Duchenne muscular dystrophy (DMD) who are ages 4 to 16 and have mutations amenable to exon 51 skipping.

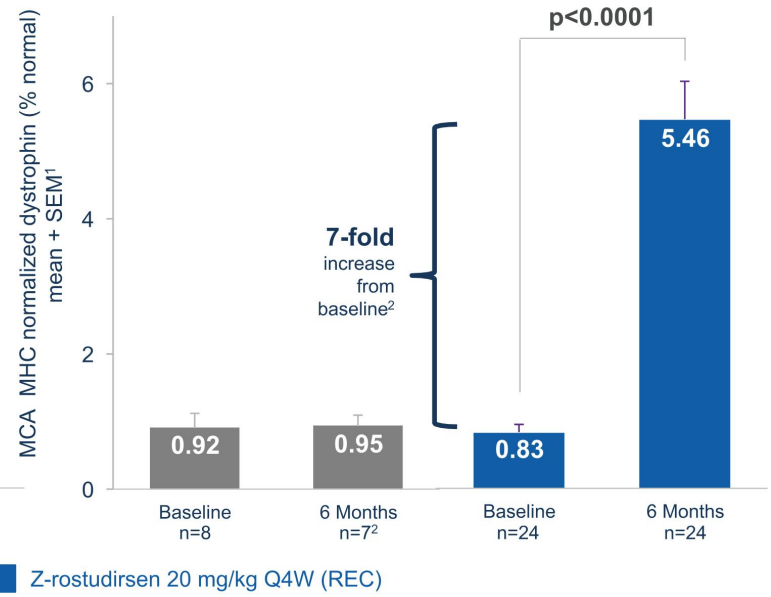
1. Safety data as of August 19, 2025.

# Z-Rostudirsen Achieved a Statistically Significant and Robust Increase in Dystrophin Expression at 6M in Registrational Expansion Cohort

**Unadjusted dystrophin**



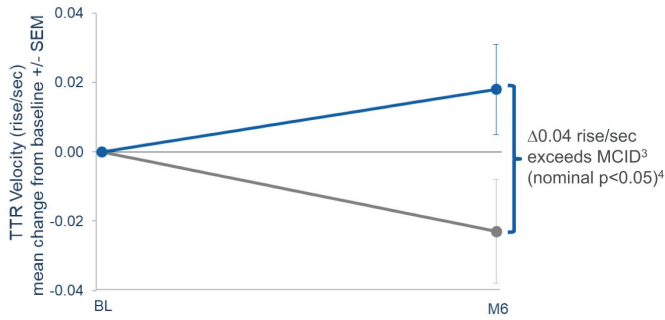
**Muscle content-adjusted dystrophin<sup>5</sup>**



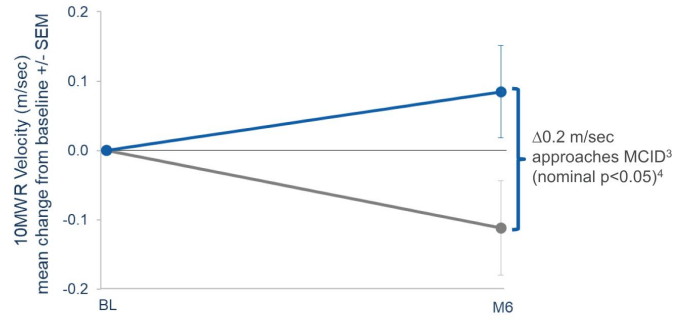
1. Biopsies taken approximately 28 days after most recent dose. 2. Based on geometric mean. 3. One REC placebo participant sample could not be analyzed at Week 25. 4. Prespecified nominal p-value with no adjustment for multiplicity. 5. Muscle content-adjusted dystrophin = MHC normalized dystrophin / % muscle content. 6 months = Week 25 for DELIVER; REC, registrational expansion cohort; MCA, muscle content-adjusted; MHC, myosin heavy chain; Q4W, every 4 weeks; SEM, standard error of the mean.

# Improvement in TTR Velocity and 10MWR Velocity at 6 Months Relative to Baseline and Placebo

**Time to Rise<sup>1</sup> (TTR) Velocity<sup>2</sup>**



**10-Meter Walk/Run (10MWR) Velocity<sup>2</sup>**



- Placebo (REC+MAD) (n=18)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=21)

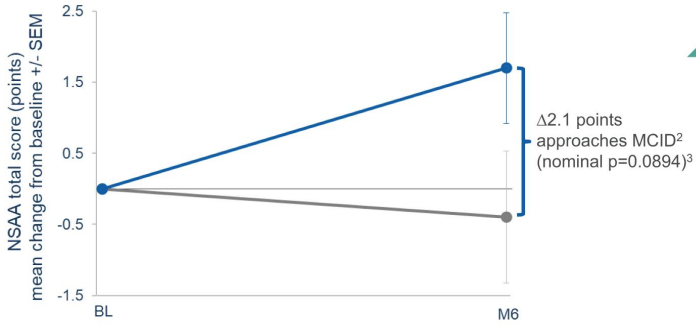
- Placebo (REC+MAD) (n=18)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=21)



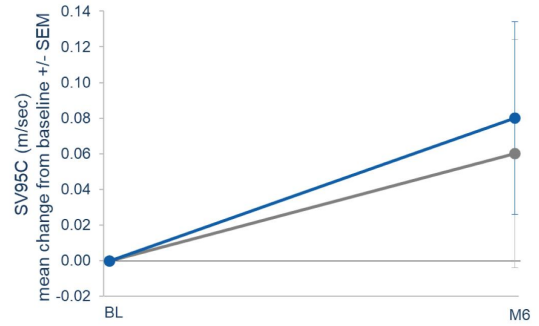
1. Also referred to as rise from floor (RFF). 2. Ambulant participants; out-of-threshold or missing values imputed. 3. Duong et al. *J Neuromusc Dis*. 2021; 8(6):939-948; RFF velocity MCID = 0.023 rise/sec; 10MWR velocity MCID = 0.212 m/sec. 4. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint. BL, baseline; M6 = 169 days; M, monthsec, seconds; SEM, standard error of mean; MCID, minimal clinically important difference; REC, registrational expansion cohort; MAD, multiple ascending dose; Q4W, every 4 weeks.

# Improvement in NSAA and SV95C at 6 Months Relative to Baseline

## North Star Ambulatory Assessment (NSAA)<sup>1</sup>



## Stride Velocity 95<sup>th</sup> Centile (SV95C)<sup>1</sup>



- Placebo (REC+MAD) (n=18)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=21)

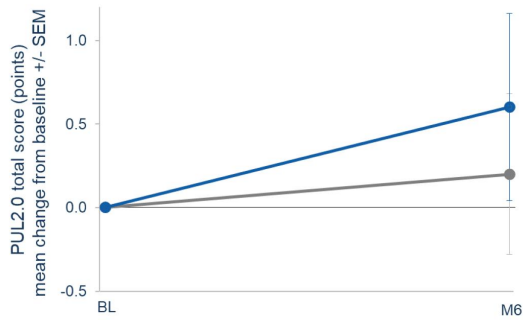
- Placebo (REC+MAD)<sup>4</sup> (n=12)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=20)



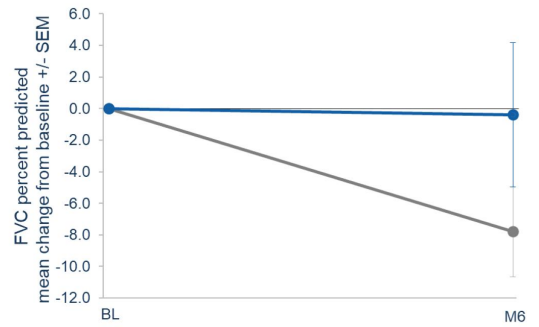
1. Ambulant participants; missing values imputed. 2. Ayyar Gupta et al. *PLoS One*. 2023 Apr 26;18(4):e0283666; NSAA MCID  $\geq 2.3$  points. 3. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint. 4. Placebo impacted by single participant with change from baseline of 0.46 m/sec at 6M; if this participant were excluded, mean change from baseline at 6M for placebo would be approximately 0.02 m/sec. BL, baseline; M6 = 169 days; M, month; sec, seconds; SEM, standard error of mean; MCID, minimal clinically important difference; REC, registrational expansion cohort; MAD, multiple ascending dose; Q4W, every 4 weeks.

# Improvement in PUL2.0 at 6 Months Relative to Baseline and Placebo; Preservation of Lung Function at 6 Months

### Performance Upper Limb v2.0 (PUL2.0)<sup>1</sup>



### Forced Vital Capacity Percent Predicted (FVC%p)<sup>1</sup>



- Placebo (REC+MAD) (n=23)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=22)

- Placebo (REC+MAD) (n=20)
- Z-rostudirsen 20 mg/kg Q4W (REC) (n=15)



1. Ambulant and non-ambulant participants; missing values imputed for PUL2.0. BL, baseline; M6 = 169 days; M, month; sec, seconds; SEM, standard error of mean; REC, registrational expansion cohort; MAD, multiple ascending dose; Q4W, every 4 weeks.

# Unprecedented Breadth and Durability of Functional Improvement with Z-Rostudirsen

BREADTH			DURABILITY	
Endpoint	Patient Population	Muscle System	6-month Functional Improvement vs. Placebo <sup>1</sup>	24-month Functional Improvement vs. Baseline <sup>2</sup>
TTR Velocity	Ambulatory	Trunk & lower limbs	✓	✓
10MWR Velocity	Ambulatory	Lower limbs	✓	✓
NSAA	Ambulatory	Upper limbs, trunk & lower limbs	✓	✓
SV95C	Ambulatory	Lower limbs	✓	✓
PUL2.0	Ambulatory & non-ambulatory	Upper limbs	✓	✓
FVC%p	Ambulatory & non-ambulatory	Diaphragm & trunk	✓	✓



1. Reflects results from 6-month placebo-controlled Registrational Expansion Cohort of DELIVER trial with 20 mg/kg Q4W z-rostudirsen. Check mark represents numerically higher value relative to placebo. 2. Reflects 24-month results from the long-term portions of the DELIVER trial for participants who transitioned from 10 mg/kg Q4W to 20 mg/kg Q4W after 6M; all participants treated with 20 mg/kg Q4W for at least 12M in the 24M assessment. Check mark represents numerically higher value relative to baseline.

# Z-Rostudirsen: Favorable Safety Profile

## Summary of treatment-emergent adverse events (TEAEs)<sup>1</sup>

Study Period	Placebo-Controlled (PC) Period (0 to 6M)		All Study Periods (0 to ≤36M)
	Placebo (MAD+REC) N=24 <sup>2</sup>	Z-rostudirsen 20 mg/kg Q4W (MAD+REC) N=30 <sup>3</sup>	
Participants with ≥1 TEAE – n (%)			Z-rostudirsen Pooled doses <sup>4</sup> (MAD+REC) N=85 <sup>5</sup>
Any TEAE	22 (91.7)	29 (96.7)	80 (94.1)
Any related TEAE	3 (12.5)	10 (33.3)	41 (48.2)
Any serious TEAE	1 (4.2)	2 (6.7)	10 (11.8)
Any serious related TEAE	0	0	4 (4.7)
Any TEAE leading to withdrawal from study	0	0	0
Any TEAE leading to death	0	0	0

### Most related TEAEs were mild or moderate

#### Potentially related serious TEAEs

- 2 participants at 20 mg/kg Q4W (registrational dose)
  - Pyrexia (fever) and malaise<sup>6</sup>
- 2 participants at 40 mg/kg Q4W
  - Acute kidney injury; thrombocytopenia<sup>7</sup>
  - Pancytopenia<sup>8</sup>

#### Most frequent related TEAEs ≥10%<sup>9</sup>

- Pyrexia (fever) (18%)
- Headache (13%)

#### Additional safety data at 20 mg/kg Q4W

- No participants have persistent related anemia<sup>10</sup> or thrombocytopenia

**1,441 doses of z-rostudirsen administered to date representing 113 patient-years of follow-up (up to 36 months)<sup>1</sup>**  
**1,062 doses of z-rostudirsen at 20 mg/kg dose level administered to date<sup>1</sup>**

1. Data as of August 19, 2025; all participants, placebo-controlled period, OLE, and LTE. 2. All placebo participants pooled from MAD and REC. 3. All participants randomized to z-rostudirsen 20 mg/kg Q4W in MAD and REC cohorts. 4. All doses of z-rostudirsen from MAD and REC at doses ranging from 0.7 mg/kg to 40 mg/kg every 4 or 8 weeks. 5. One participant randomized to placebo in REC not yet dosed with z-rostudirsen as of August 19, 2025. 6. One participant with same day onset of pyrexia and malaise in OLE and separate single event of pyrexia in LTE; one participant with single event of pyrexia in LTE; both participants fully recovered and have continued to receive z-rostudirsen without interruption. 7. Events had same day of onset in a single participant with a non-serious related TEAE of anemia in the context of fever, hemolysis, diarrhea, and positive blood in stool; together these events were consistent with hemolytic uremic syndrome with a possible infectious etiology. 8. Participant has a history of hemolytic anemia of unidentified etiology; presented with fever and tonsillitis; symptoms resolved without therapeutic intervention. 9. All cohorts combined; preferred terms reported. 10. No participants have persistent related anemia with Hgb levels <11.2 g/dL (threshold for anemia in children (ref: Powers JM. Approach to the child with anemia. UpToDate, Connor RF (Ed), Wolters Kluwer. Accessed December 2, 2025)). M, months; MAD, multiple ascending dose; REC, registrational expansion cohort; Q4W, every 4 weeks; OLE, open-label extension; LTE, long-term extension.

# Z-Rostudirsen Offers a Compelling Profile for Potential Accelerated Approval and Addressing Unmet Need in DMD

## Z-Rostudirsen: Potential Best-in-class Profile for Exon 51 DMD



Statistically Significant and Robust Increase in Dystrophin at 6 Months



Favorable Safety & Tolerability Profile up to 36 Months<sup>1</sup>



Functional Improvement Observed Across Multiple Clinical Measures



Convenient Q4W Dosing

**“I am highly encouraged by these new results** from the placebo-controlled Registrational Expansion Cohort and the longer-term portions of DELIVER, and **I look forward to being able to offer z-rostudirsen to eligible DMD patients**, if approved.” - Perry Shieh, M.D, Ph.D.



1. Data as of August 19, 2025. 2. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint. DMD, Duchenne muscular dystrophy; CFB, change from baseline; Q4W, every 4 weeks; TTR, time to rise; 10MWR, 10-meter walk/run. Dr. Shieh is a professor of neurology and pediatrics at the David Geffen School of Medicine at UCLA, a neurologist at the Ronald Reagan UCLA Medical Center in Los Angeles, and a principal investigator for the DELIVER trial.

# Preparing to Launch into an Established Rare Disease Market with Well-Characterized Patient Population and Treatment Centers

## Well-Characterized Patient Population

**>50%**

Exon 51 skip amenable patients have been treated with a disease modifying therapy<sup>1</sup>

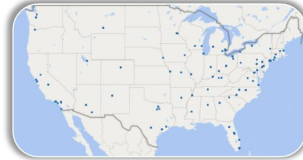
- ~12,000 US DMD population
  - ~1,600 US Exon 51 skip amenable DMD
- Active and educated patient community
- HHS added DMD to RUSP in December 2025

## Concentrated Treatment Centers<sup>1</sup>

**~80%**

of DMD patients cared for at top 100 DMD centers

- Over 80% of top 100 centers have experience prescribing DMTs to exon 51 patients



- Significant overlap expected with DM1 centers

## Established Market with Reimbursement

**~\$1M**

WAC price of currently approved exon skippers for average patient per year<sup>2</sup>

- First exon skipper approved in 2016
- Established reimbursement pathways and clear recognition of unmet need by payers
  - ~ 55% Medicaid, 40% commercial, 5% Medicare/other<sup>1</sup>
- Pricing precedent for exon skippers

# Capital-Efficient Commercial Infrastructure Designed to Deliver Potentially Transformative Therapies and Optimize Value

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## Experienced launch team assembled for targeted execution

- Strong expertise in rare neurological and neuromuscular diseases
  - Broad US launch experience including SPINRAZA, ELEVIDYS, VYONDYS, AMONDYS, and DAYBUE
- Deep expertise across Medical, Commercial, Value & Access, Manufacturing, Program Management, Patient Advocacy and Services, Government Affairs, Digital, Data & Analytics



## Ongoing education & awareness building

- Site of care visits underway across Medical and Commercial field teams
- Ongoing engagement with payer community
- > 5 years of sustained patient community and advocacy organization engagement



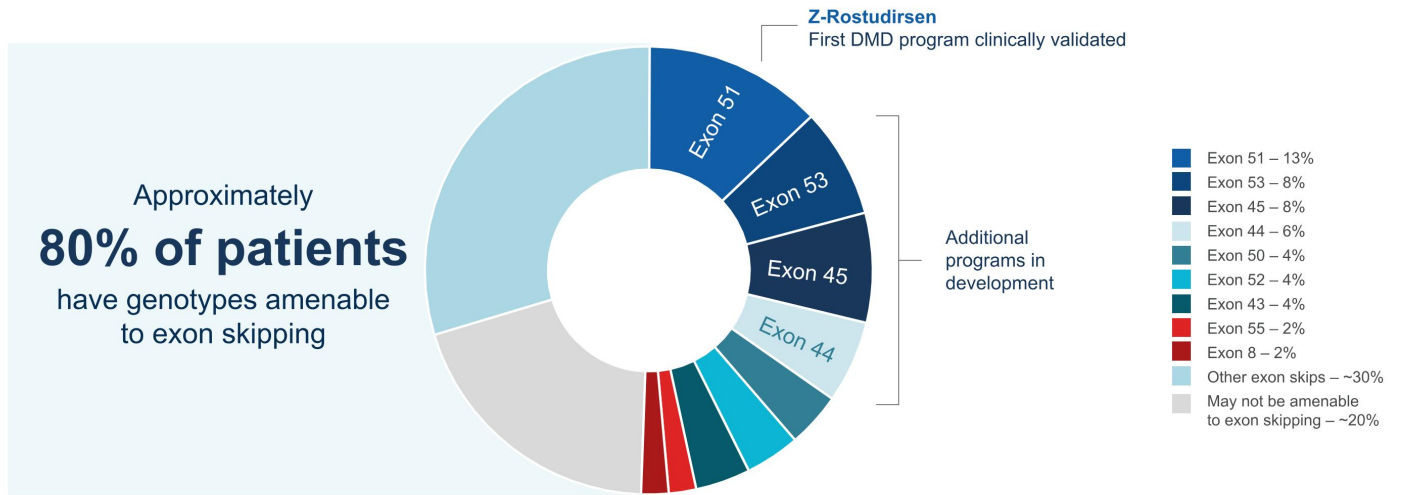
## Manufacturing capabilities and team ready to supply DMD and DM1 potential launches

- Proven track record of providing drug supply across global clinical trials since 2022
- Leveraging same Fab and linker across DMD and DM1 programs
- Commercial-scale manufacturing partnerships in place with sufficient capacity and redundant, geographically-diverse suppliers



Note: Fab = fragment antibody-binding; DMD = Duchenne muscular dystrophy; DM1 = myotonic dystrophy type 1

# De-Risked Opportunity to Build a Broader DMD Franchise Potentially Tripling Addressable Patient Population



## DMD franchise assets designed to leverage same Fab, linker, and payload chemistry as z-rostudirsen



Fletcher, S., et. al. Dystrophin Isoform Induction In Vivo by Antisense-mediated Alternative Splicing. The American Society of Gene & Cell Therapy. 2010;18(6):1218-1223. Aartsma-Rus, et al. Theoretic applicability of antisense-mediated exon skipping for Duchenne muscular dystrophy. Hum Mutat. 2009 Mar;30 (3):293-9. DMD = Duchenne muscular dystrophy; Fab = fragment antibody-binding.

# Drivers for Potential Shareholder Value Creation in DMD

## ATTRACTIVE MARKET CHARACTERISTICS

**Significant**  
unmet needs despite approved therapies



**Identified**  
patient population



**Pricing precedent**  
with established reimbursement



**Concentrated**  
treatment centers



## POSITIONED TO MAXIMIZE VALUE

**Wholly-owned**  
assets



Potential **best-in-class profile** for lead program



**Capital-efficient**  
commercial model



**De-Risked** expansion opportunities



## ON TRACK FOR Q1 2027 POTENTIAL LAUNCH

**Team and capabilities to execute**





## Second Lead Program Leveraging Clinically Validated Platform for Larger Adjacent Indication: Z-Basivarsen in DM1



# DM1 is a Devastating Neuromuscular Splicing Disorder



## Population

- ~40,000 (US)
- ~55,000 (EU)



## Overview

- Mutation in the *DMPK* gene leads to mis-splicing of multiple genes
- Onset at any point, depending on DM1 phenotype
- Life expectancy of 45 - 60 years



## Clinical Presentation

- Muscle weakness & myotonia
- CNS manifestations including fatigue, cognition, and sleep
- Gastrointestinal issues
- Cardiac arrhythmia
- Pulmonary abnormalities



**NO**  
approved  
therapies

## OUR APPROACH

### Functional Improvement via Splicing Correction in Nucleus

Restore normal RNA splicing to achieve **functional improvement** for those living with DM1



Note: DM1 = myotonic dystrophy type 1; CNS = central nervous system.

# ACHIEVE Study to Support Accelerated Approval of Z-Basivarsen in DM1



Selection of registrational dose (6.8 mg/kg Q8W) based on multiple ascending dose (MAD) data



Data support vHOT improvement as early indicator of clinical benefit with z-basivarsen



Proof-of-concept that z-basivarsen can reverse disease progression across multiple functional endpoints



Favorable safety profile<sup>1</sup>



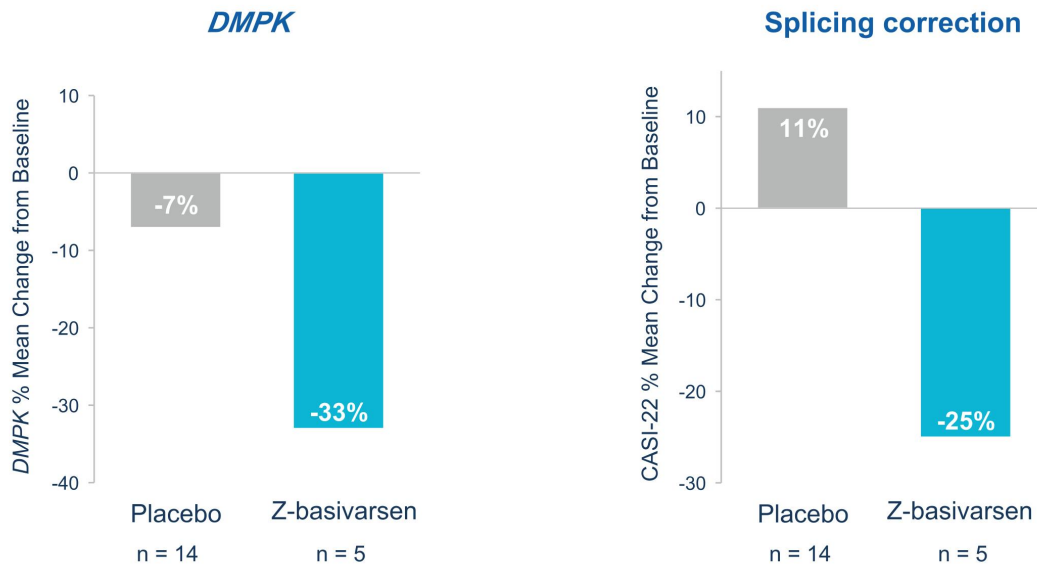
Potential submission for U.S. Accelerated Approval based on data from Registrational Expansion Cohort (REC)

**Expect to complete enrollment of ACHIEVE REC in early Q2 2026**  
**Phase 3 study planned to support full approval of z-basivarsen globally**



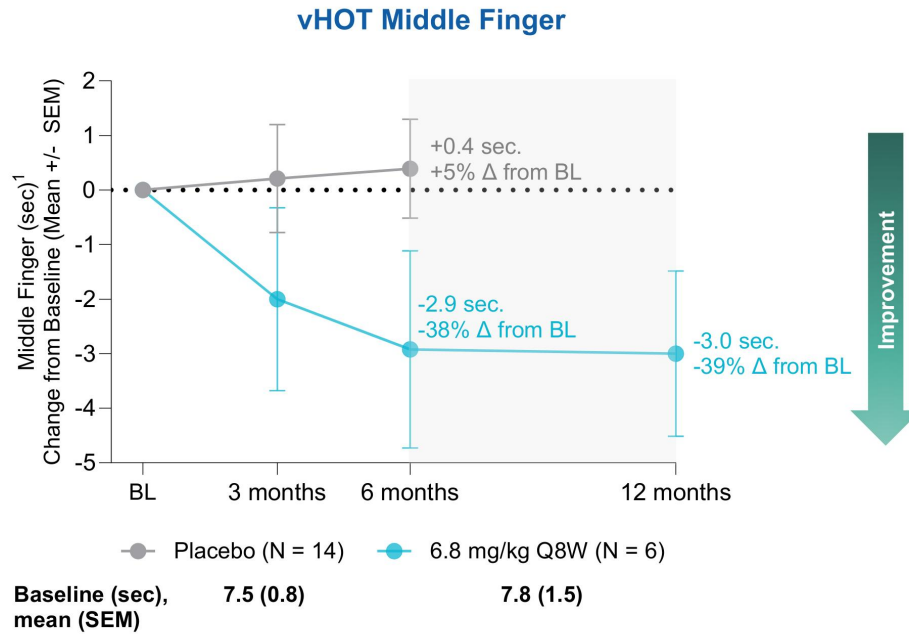
Note: ACHIEVE is a Phase 1/2 global placebo-controlled clinical trial evaluating multiple doses of z-basivarsen in adult patients with myotonic dystrophy.  
1. Data as of April 23, 2025

# Z-Basivarsen at 6.8 mg/kg Q8W Improved Foundational Pathobiology of DM1 at 3 Months



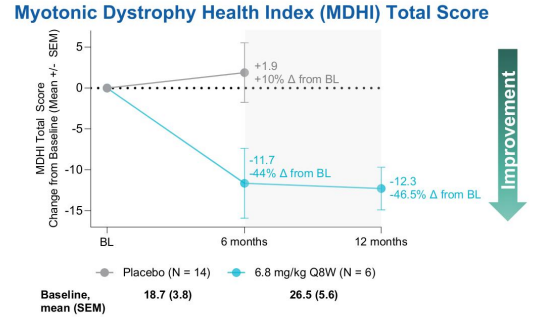
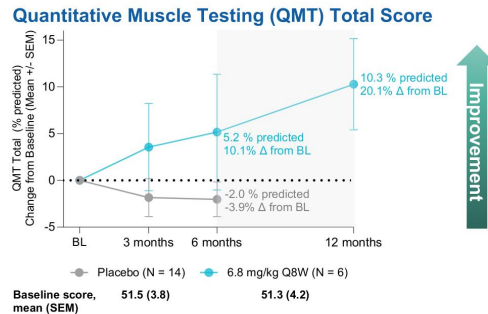
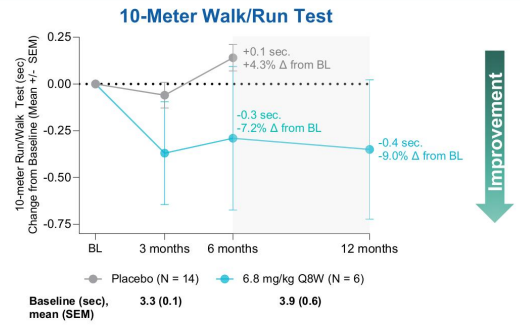
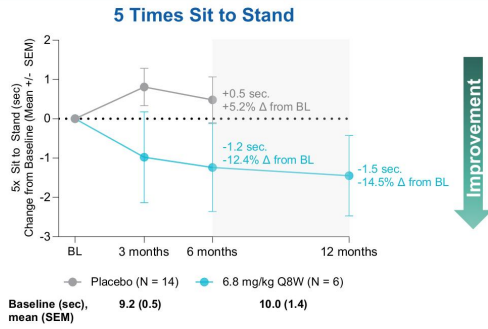
Note: One baseline sample in 6.8 mg/kg treatment groups not included within splicing assay as the sample did not meet QC criteria. 3 months = 85 days. CASI-22 = composite alternative splicing index.

# Robust and Sustained vHOT Improvement with Z-Basivarsen at 6 and 12 Months



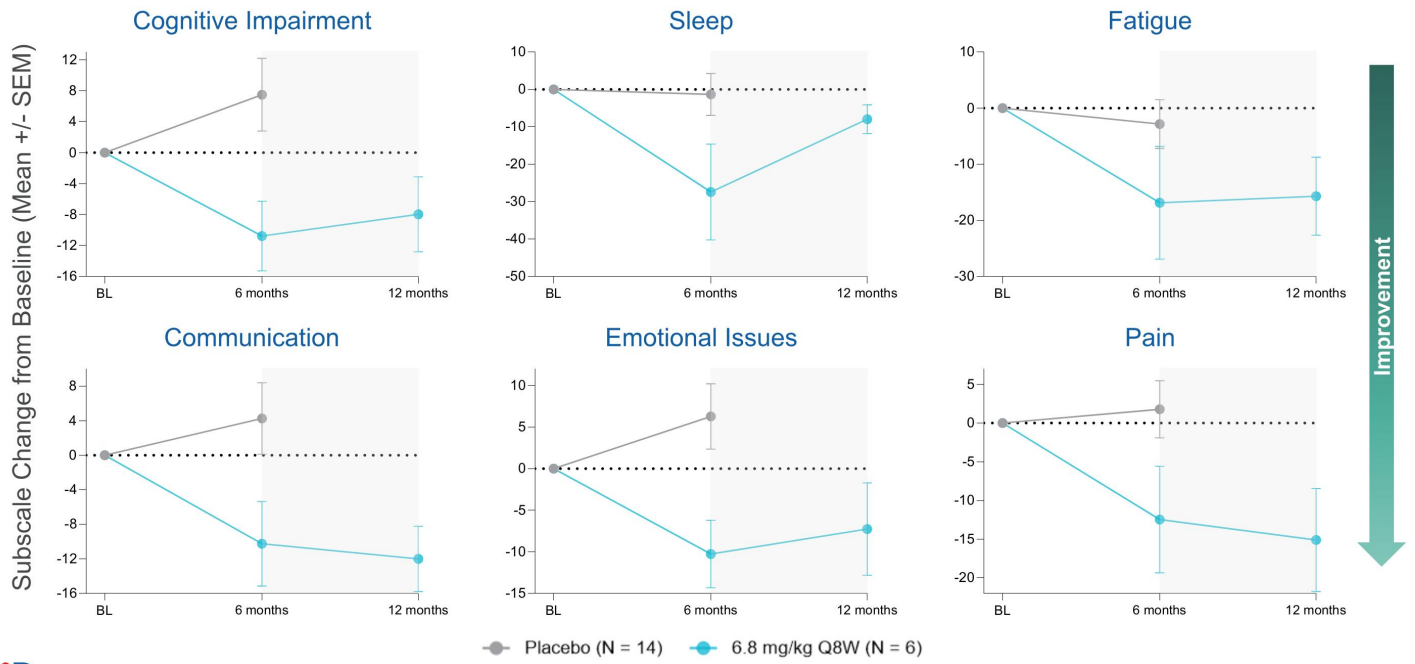
1. vHOT Middle Finger (sec)<sup>1</sup> is the average of all myotonia trials for an individual participant in ACHIEVE; vHOT = video hand opening time; SEM = standard error of the mean; BL = baseline. 3 months = 85 days; 6 months = 169 days; 12 months = 337 days.

# Broad and Sustained Functional and Patient-Reported Improvement with Z-Basivarsen through 12 Months



BL, baseline; Q8W, every 8 weeks dosing; SEM, standard error of the mean.  
3 months = 85 days; 6 months = 169 days; 12 months = 337 days.

# Sustained Improvement in CNS-related MDHI Subscales



SEM = standard error of the mean; BL = baseline; Patient-reported outcomes (PRO) including Myotonic Dystrophy Health Index (MDHI) collected at baseline, 6 months (169 days) and 12 months (337 days).

# Z-Basivarsen: Favorable Safety Profile with No Serious Related TEAEs

## Summary of Treatment Emergent Adverse Events (TEAEs)<sup>1</sup>

TEAE Category	Participants with ≥1 TEAE – n (%)					
	1.8 mg/kg Q4W+Rec. N=16	3.4 mg/kg Q4W+Rec. N=16	3.4 mg/kg Q8W N=8	5.4 mg/kg Q8W N=8	6.8 mg/kg Q8W N=8	Overall (N=56)
Any TEAE	16 (100%)	16 (100%)	8 (100%)	8 (100%)	8 (100%)	56 (100%)
Any related TEAE	9 (56%)	10 (63%)	3 (38%)	6 (75%)	6 (75%)	34 (61%)
Any serious TEAE	4 (25%)	0	1 (13%)	0	0	5 (9%)
Any serious related TEAE	0	0	0	0	0	0
Any TEAE leading to withdrawal from study	0	0	0	0	0	0
Any TEAE leading to death	0	0	0	0	0	0

## Most TEAEs Were Mild or Moderate in Intensity<sup>1</sup>

- 6 serious TEAEs unrelated to study drug
  - Atrioventricular block first degree (1)<sup>2</sup>
  - Pneumonia (2 events in same participant)
  - Pulmonary embolism (1)<sup>3</sup>
  - Hyponatremia (1)
  - Influenza (1)
- Most common TEAEs (≥20% participant incidence)<sup>4</sup>
  - Nasopharyngitis (41%)
  - Procedural pain (34%)
  - Influenza (30%)
  - Infusion-related reaction (29%)
  - Headache (27%)
  - Diarrhea (23%)

## Additional Safety Data




- Liver enzyme elevations have been observed in a minority of participants
  - No impact on liver function (bilirubin or coagulation)
  - Interpretation is complicated by underlying disease and elevated baseline values up to ~2.5x greater than the upper limit of normal
- No participants have demonstrated persistent related anemia or thrombocytopenia

~1000 Doses of Study Drug Administered to Date Representing 93 Patient-Years of Follow-Up<sup>1</sup>



1. Data as of April 23, 2025; 2. Transient worsening of atrioventricular (AV) block in a participant with ongoing medical history of first-degree AV block; 3. Attributed to risk factors for pulmonary embolism; 4. All cohorts combined; preferred terms are reported.

# Transforming Dyne into a Commercial Organization as Early as 2027

Z-Rostudirsen for Exon 51 DMD		Z-Basivarsen for DM1	
Q1 2025	Completed enrollment of Registrational Expansion Cohort 	Early Q2 2026	Complete enrollment planned for Registrational Expansion Cohort
December 2025	Positive topline results from Registrational Expansion Cohort 	Q1 2027	Data planned for Registrational Expansion Cohort
Q2 2026	Planned submission for U.S. Accelerated Approval	Early Q3 2027	Potential submission for U.S. Accelerated Approval
Q1 2027	Potential U.S. launch, assuming Priority Review 	Q1 2028	Potential U.S. launch, assuming Priority Review

**One capital efficient operating model to support multiple potential commercial launches**



DMD = Duchenne muscular dystrophy; DM1 = myotonic dystrophy type 1.

## Q&A

