Positive Topline Results from Phase 1/2 DELIVER Trial of Zeleciment Rostudirsen (DYNE-251) in DMD to Support Potential U.S. Accelerated Approval

DECEMBER 8, 2025

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Program

Opening Remarks

John Cox, President & CEO

Positive Topline Results from DELIVER Trial of Z-Rostudirsen Supporting Potential for U.S. Accelerated Approval

Doug Kerr, M.D., Ph.D., Chief Medical Officer

Closing Remarks

John Cox, President & CEO

Available for Q&A

Erick Lucera, Chief Financial Officer

Transforming Exon 51 Skip Amenable DMD; Validating the FORCE Platform and Dyne Pipeline

Data to Support Potential Accelerated Approval

- Positive results from Registrational Expansion Cohort of DELIVER trial of z-rostudirsen in exon 51 DMD
- **Met primary endpoint:** Statistically significant and robust increase in dystrophin at 6 months ($p<0.0001$)
- Continued favorable safety profile[1]
- Clear trends relative to placebo across multiple functional endpoints

Sustained Functional Improvement

- Sustained functional improvement from baseline across multiple clinical endpoints out to 24 months, including:
  - TTR Velocity
  - 10MWR Velocity
  - NSAA
  - SV95C
  - PUL2.0
  - FVC%p

Validation of FORCE™ Platform

- Data show that FORCE platform can deliver payloads to targeted tissues, with favorable therapeutic window
- Leveraging same delivery approach for z-basivarsen in DM1 and early-stage pipeline in FSHD, Pompe disease and other DMD exons

Advancing z-rostudirsen toward Dyne's first approval

Planned BLA submission for U.S. Accelerated Approval on track for Q2 2026

Potential U.S. launch in Q1 2027, assuming FDA grants Priority Review

Phase 3 study initiation planned for Q2 2026

1. Safety data as of August 19, 2025; DMD, Duchenne muscular dystrophy; DM1, myotonic dystrophy type 1; FSHD, facioscapulohumeral muscular dystrophy; BLA, biologics license application; TTR, time to rise; 10MWR, 10-meter walk/run; NSAA, north star ambulatory assessment; SV95C, stride velocity 95th centile; PUL2.0, performance upper limb v2.0; FVC%p, forced vital capacity percent predicted.

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DELIVER Clinical Update Agenda

Unmet Need in DMD and Potential for Z-Rostudirsen

Positive Topline Results from Registrational Expansion Cohort (REC) of DELIVER Trial to Support Planned Submission for U.S. Accelerated Approval

New Positive Long-Term Results from DELIVER Trial Showed Sustained Functional Improvement Across Multiple Functional Measures

Results Validate the Potential of FORCE™ Platform

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

Progressive Clinical Presentation

- Muscle weakness and gait abnormalities
- Progressive loss of upper and lower limb strength and function
- Cognitive function impairment and neuropsychiatric disorders
- Respiratory/cardiac failure (leading cause of death)
- Life expectancy ~30 years[1]

Exon 51 Skip Amenable DMD – Challenging Form of Duchenne

- Greatly reduced or absent dystrophin protein expression
  - Low baseline dystrophin levels compared with most other DMD mutations[2-8]
- Faster functional decline[9-11]
- Earlier loss of ambulation (average ~11.5 years)[10]
- ~13% of DMD patient population[12]

Limitations with Current Therapies

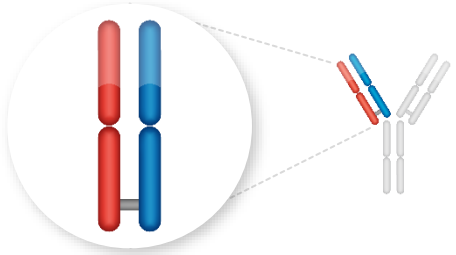
- Limited delivery to skeletal muscle, heart, and CNS
- High patient and caregiver burden due to frequent IV dosing (e.g., Q1W)[2]
- <1% dystrophin production with currently approved exon 51 skipping therapy[2]
- Microdystrophin lacks domains key for optimal functionality[13]
- Unknown durability and inability to redose with gene therapy
- Safety considerations

1. Broomfield et al. *Neurology*. 2021;97:e2304-e2314; 2. Exondys 51 Prescribing Information; 3. McMillan et al. 2025 WMS Annual Meeting, 19O; 4. Vyondys53 Prescribing Information; 5. Amondys45 Prescribing Information; 6. Viltepso Prescribing Information; 7. Veerapandiyan et al. 2025 MDA Clinical and Scientific Conference O72; 8. Aoki et al. 2025 ASGCT Annual Meeting, Abstract 1351; 9. Muntoni et al. *Neurology*. 2023;100:e1540–e1554; 10. Bello et al. *Neurology*. 2016;87:401-409. 11. Zygmunt et al. *Muscle Nerve*. 2024 Nov;70(5):1053-1061. 12. Aartsma-Rus et al. *Hum Mutat*. 2009;30:293-299; 13. Chamberlain et al. *Hum Gene Ther* 2023;34:404–15. DMD, Duchenne muscular dystrophy, CNS, central nervous system; Q1W, every 1 week.

Z-Rostudirsen Leverages FORCE™ Platform for Targeted Delivery to Tissues That Matter in DMD

ANTIBODY

Proprietary Fab against TfR1 to enable targeted delivery to muscle and CNS

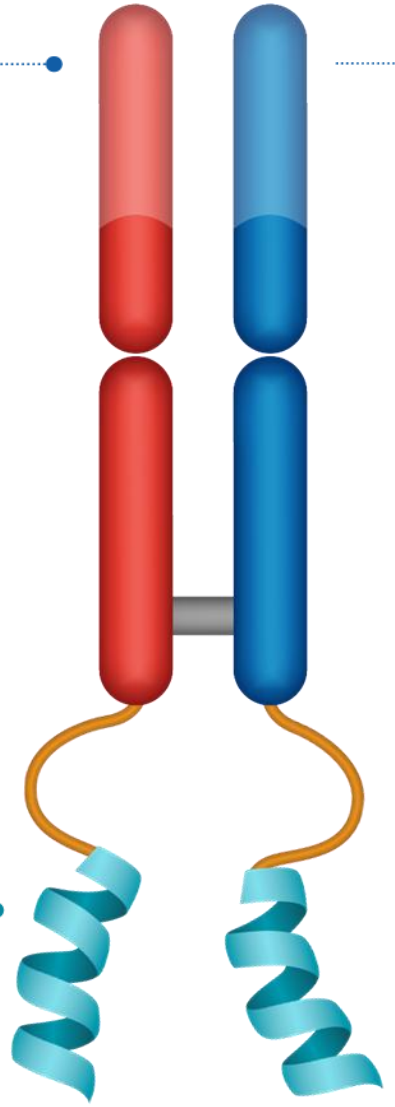


LINKER

Connects Fab and payload

PAYLOAD

PMO designed to enable production of near full-length dystrophin



Skeletal (including diaphragm) and smooth muscle

Cardiac muscle

CNS

Note: Figure depicts oligonucleotide payload; DMD, Duchenne muscular dystrophy; Fab, fragment antibody-binding; TfR1, transferrin receptor 1; PMO, phosphorodiamidate morpholino oligomer; CNS, central nervous system.

DELIVER Clinical Update Agenda

Unmet Need in DMD and Potential for Z-Rostudirsen

Positive Topline Results from Registrational Expansion Cohort (REC) of DELIVER Trial to Support Planned Submission for U.S. Accelerated Approval

New Positive Long-Term Results from DELIVER Trial Showed Sustained Functional Improvement Across Multiple Functional Measures

Results Validate the Potential of FORCE™ Platform

DELIVER Trial Designed to Support Potential U.S. Accelerated Approval

Trial Design Focused on U.S. Accelerated Approval

- Broad, representative patient population with *DMD* mutations amenable to exon 51 skipping
  - Ages 4–16 years; ambulant and non-ambulant
- Statistically significant change in dystrophin in REC
- Sufficient characterization of safety profile
- Trends in functional outcome measures in REC

Endpoints

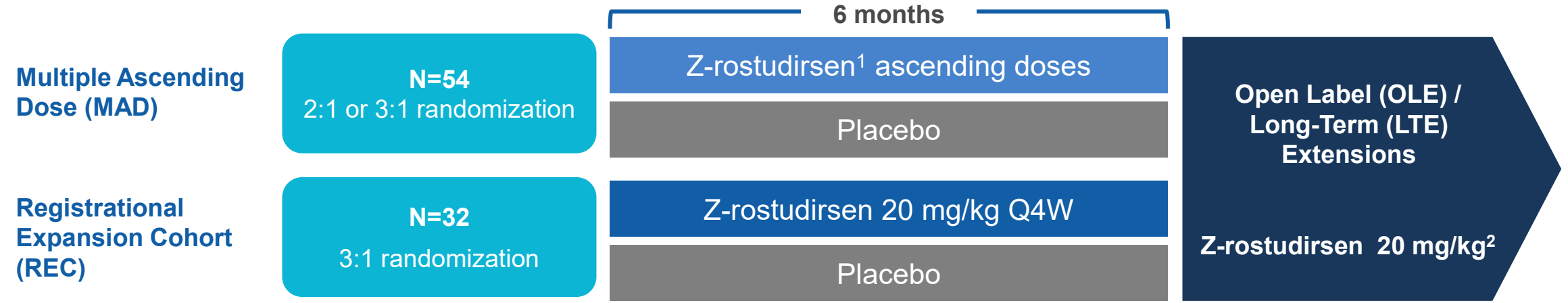
Primary endpoints

- Change from baseline in dystrophin protein levels by Western Blot at 6 months
- Safety and tolerability

Key functional endpoints

- TTR Velocity, 10MWR Velocity, NSAA, SV95C, PUL2.0, FVC%p

DELIVER (N=86)



1. Z-rostudirsen doses in the MAD cohorts ranged from 0.7 mg/kg to 40 mg/kg every 4 or 8 weeks. 2. Transition to 20 mg/kg dose occurred at non-uniform times during OLE or LTE; for participants initiated at 40 mg/kg, transition started either in the placebo-controlled period or OLE. Q4W, every 4 weeks; DMD, Duchenne muscular dystrophy; TTR, time to rise; 10MWR, 10-meter walk/run; NSAA, North Star Ambulatory Assessment; SV95C, stride velocity 95th centile; PUL2.0, performance upper limb v2.0; FVC%p, forced vital capacity percent predicted; Q4W, every 4 weeks.

DELIVER Baseline Participant Characteristics and Function Generally Well Balanced Across Treated and Pooled Placebo Cohorts

Mean (SD) or n (%)	Placebo (MAD+REC) N=24[5]	Z-rostudirsen		
		20 mg/kg Q4W (REC) N=24	10 → 20 mg/kg[6] Q4W (MAD) N=6	20 mg/kg Q4W (MAD) N=6
Age (years)	8.2 (2.5)	7.8 (3.6)	6.8 (2.5)	7.7 (2.5)
BMI (kg/m^2)	19.8 (4.7)	17.6 (4.5)	17.9 (3.7)	17.5 (2.9)
Age of symptom onset (years)	3.4 (1.8)	2.5 (1.7)	3.0 (1.8)	2.0 (0.9)
Most recent corticosteroid dosing regimen, n (%)[1] Daily Other	 20 (83.3) 4 (16.7)	 20 (83.3) 4 (16.7)	 6 (100) 0	 6 (100) 0
Duration of corticosteroid treatment (years)[2]	2.1 (2.4)	2.4 (2.5)	1.5 (2.0)	2.4 (2.2)
Prior DMD therapy, n (%) Eteplirsen Other	 4 (16.7) 2 (8.3)	 2 (8.3) 5 (20.8)	 0 0	 0 2 (33.3)
PUL2.0 total score[3]	36.3 (4.0)	36.3 (5.0)	37.2 (5.9)	33.8 (3.5)
FVC%p	92.7 (17.6)	90.0 (22.2)	89.8 (22.7)	90.7 (11.2)
Ambulant (%)	19 (79.2)	21 (87.5)	5 (83.3)	6 (100)
TTR velocity (rise/sec)[4]	0.20 (0.10)	0.22 (0.12)	0.23 (0.06)	0.17 (0.14)
10MWR velocity (m/sec)[4]	2.0 (0.5)	1.8 (0.5)	2.5 (0.7)	1.5 (0.6)
NSAA total score[4]	21.6 (6.3)	20.6 (5.0)	26.6 (5.4)	15.0 (5.3)
SV95C (m/sec)[4]	1.7 (0.5)	1.5 (0.4)	2.0 (0.3)	1.4 (0.5)

1. Most recent corticosteroid regimen refers to corticosteroid at time of randomization. 2. Cumulative duration of previous and most recent corticosteroid treatment at the time of randomization. 3. Missing values imputed. 4. Ambulant participants; out-of-threshold and/or missing values imputed. 5. All placebo participants pooled from MAD and REC. 6. Participants transitioned from 10 mg/kg Q4W to 20 mg/kg Q4W after 6 months. SD, standard deviation; BMI, body mass index; kg, kilogram; m, meter; DMD, Duchenne muscular dystrophy; PUL2.0, performance upper limb v2.0; FVC%p, forced vital capacity percent predicted; TTR, time to rise; 10MWR, 10-meter walk/run; NSAA, north star ambulatory assessment; SV95C, stride velocity 95th centile; MAD, multiple ascending dose; REC, registrational expansion cohort; Q4W, every 4 weeks.

Safety Profile of Z-Rostudirsen 20 mg/kg Q4W Remains Favorable

Summary of treatment-emergent adverse events (TEAEs)[1]

Study Period	Placebo-Controlled (PC) Period (0 to 6M)		All Study Periods (0 to ≤36M)
Participants with ≥1 TEAE – n (%)	Placebo (MAD+REC) N=24[2]	Z-rostudirsen 20 mg/kg Q4W (MAD+REC) N=30[3]	Z-rostudirsen Pooled doses[4] (MAD+REC) N=85[5]
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)[6]
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
  - Pyrexia (fever) and malaise[7]
- Previously disclosed: 2 participants at 40 mg/kg Q4W
  - Acute kidney injury; thrombocytopenia[8]
  - Pancytopenia[9]

Most frequent related TEAEs ≥10%[10]

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

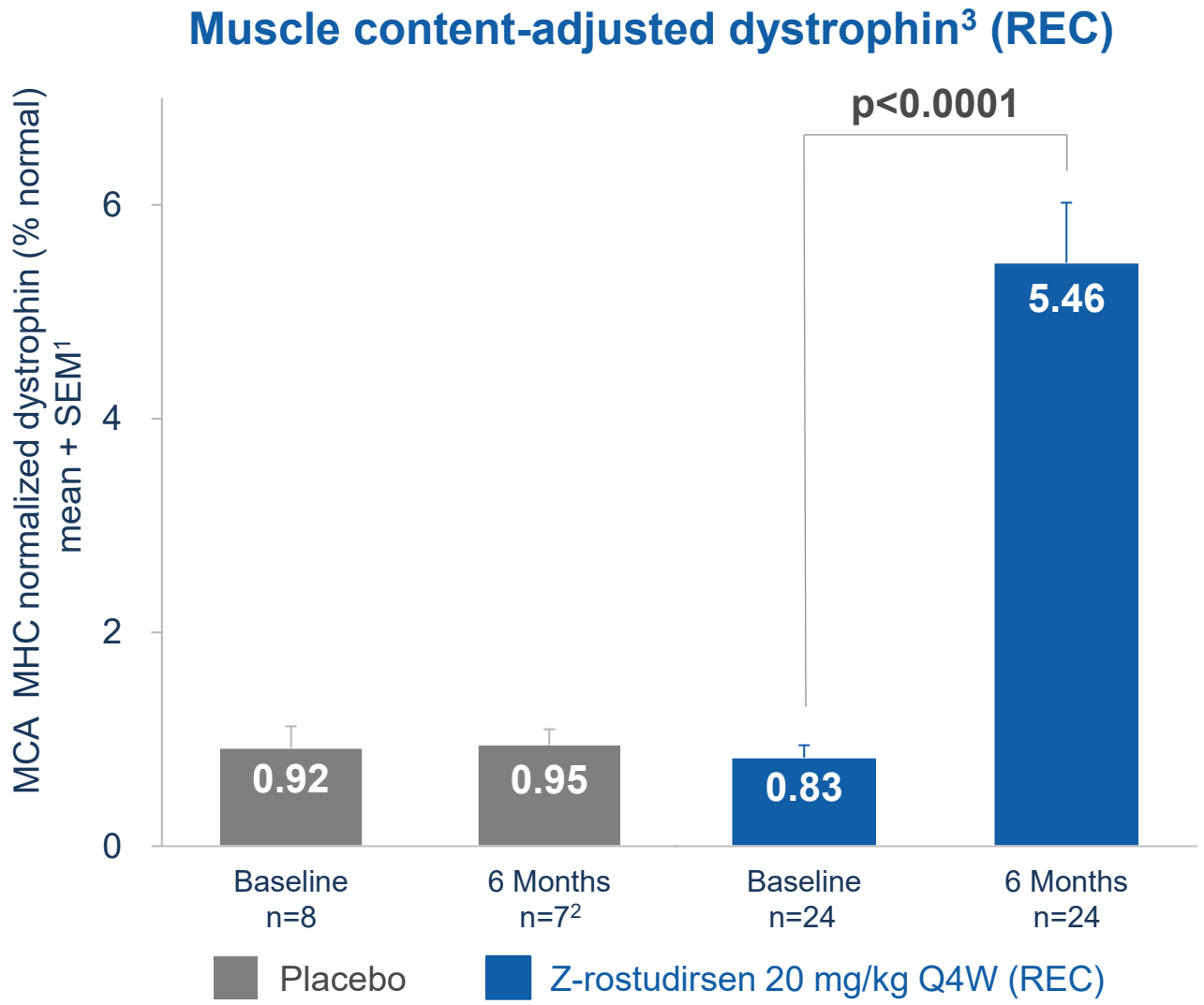
Additional safety data at 20 mg/kg Q4W

- No participants have persistent related anemia[11] or thrombocytopenia

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

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. Includes previously disclosed 2 participants with serious related TEAEs. 7. 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. 8. 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. 9. Participant has a history of hemolytic anemia of unidentified etiology; presented with fever and tonsilitis; symptoms resolved without therapeutic intervention. 10. All cohorts combined; preferred terms reported. 11. 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.

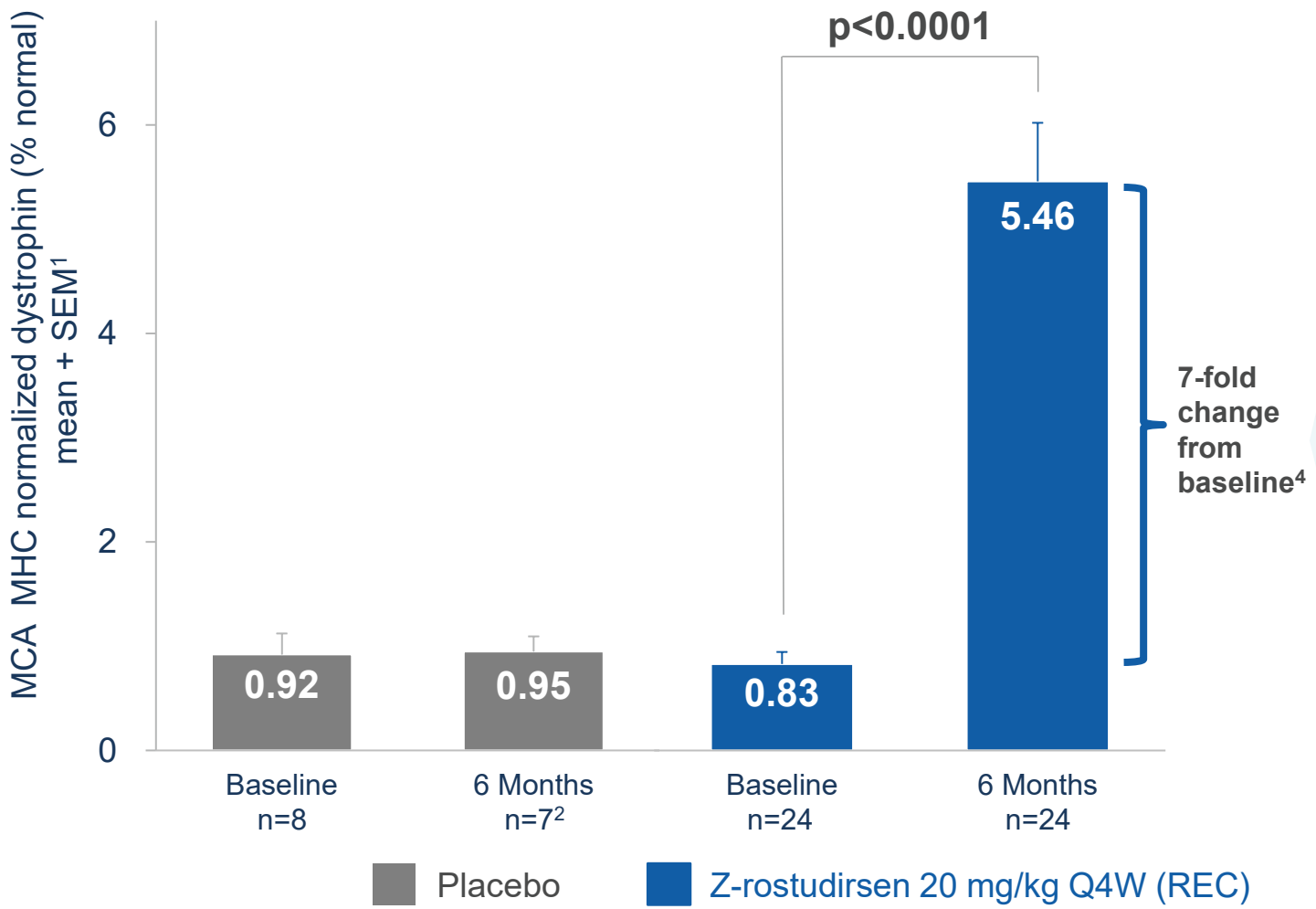
Z-Rostudirsen Achieved Statistically Significant and Robust Increase in Dystrophin Expression at 6 Months in REC



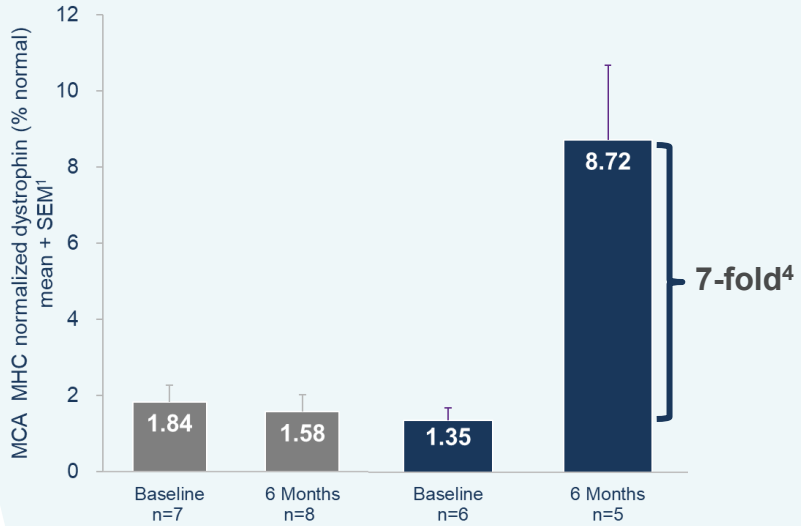
1. Biopsies taken approximately 28 days after most recent dose. 2. One REC placebo participant sample could not be analyzed at Week 25. 3. Muscle content-adjusted dystrophin = MHC normalized dystrophin / % muscle content. 6 months = Week 25 for DELIVER; REC, registrational expansion cohort; MCA, muscle content-adjusted; MHC, myosin heavy chain; Q4W, every 4 weeks; SEM, standard error of the mean.

REC Replicated Same 7-Fold Increase in Dystrophin Originally Observed in 20 mg/kg Q4W MAD Cohort

Muscle content-adjusted dystrophin[3] (REC)



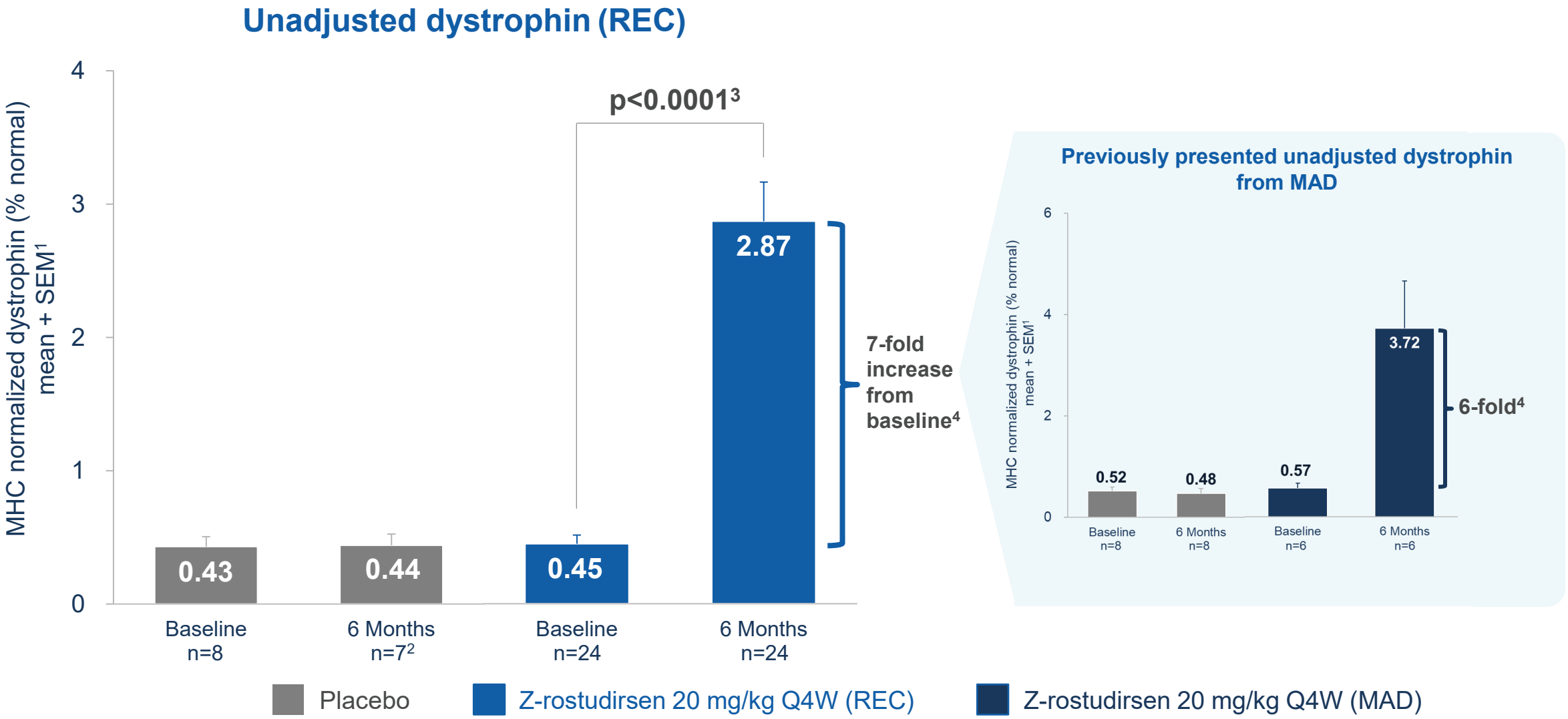
Previously presented muscle content-adjusted dystrophin from MAD



Placebo | Z-rostudirsen 20 mg/kg Q4W (REC) | Z-rostudirsen 20 mg/kg Q4W (MAD)

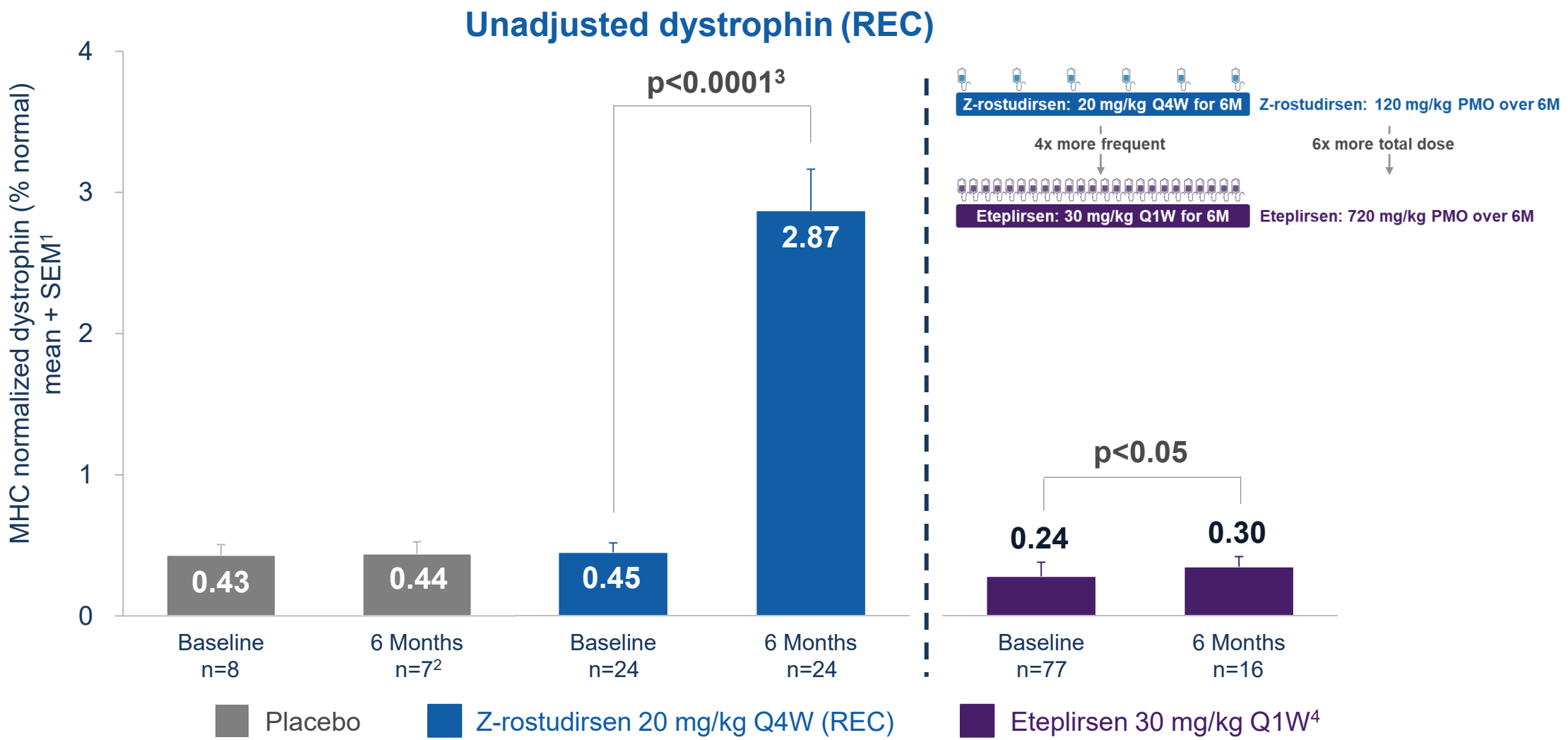
1. Biopsies taken approximately 28 days after most recent dose. 2. One REC placebo participant sample could not be analyzed at Week 25. 3. Muscle content-adjusted dystrophin = MHC normalized dystrophin / % muscle content. 4. Based on geometric mean. 6 months = Week 25 for DELIVER; MAD, multiple ascending dose; REC, registrational expansion cohort; MCA, muscle content-adjusted; MHC, myosin heavy chain; Q4W, every 4 weeks; SEM, standard error of the mean.

REC Exceeded Fold Increase in Unadjusted Dystrophin Observed in 20 mg/kg Q4W MAD Cohort



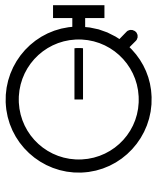
1. Biopsies taken approximately 28 days after most recent dose. 2. One REC placebo participant sample could not be analyzed at Week 25. 3. Prespecified nominal p-value with no adjustment for multiplicity. 4. Based on geometric mean. 6 months = Week 25 for DELIVER; MAD, multiple ascending dose; REC, registrational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

Z-Rostudirsen Achieved ~10-Fold Higher Level of Unadjusted Dystrophin Relative to Level Reported in Phase 3 Trial of SoC



1. Biopsies taken approximately 28 days after most recent dose. 2. One REC placebo participant sample could not be analyzed at Week 25. 3. Prespecified nominal p-value with no adjustment for multiplicity. 4. No head-to-head trials have been conducted comparing z-rostudirsen to eteplirsen; eteplirsen data may not be directly comparable due to differences in trial protocols, dosing regimens, methodologies for calculating mean dystrophin expression, and patient populations. Accordingly, these cross-trial comparisons may not be reliable. Eteplirsen data from McDonald et al. *J Neuromuscul Dis* 2021; 8(6):989–1001. 6 months = Week 25 for DELIVER; SoC, standard of care; REC, registrational expansion cohort; MHC, myosin heavy chain; PMO, phosphorodiamidate morpholino oligonucleotide; Q4W, every 4 weeks; Q1W, every 1 week; SEM, standard error of the mean.

Clinically Validated Prespecified Outcome Measures Used to Assess Functional Improvement



TTR Velocity

Time to Rise[1] Velocity

Speed of rising from floor

10MWR Velocity

10-Meter Walk/Run Velocity

Speed of walking or running 10 meters

NSAA

North Star Ambulatory Assessment

17-item scale measuring ambulatory function[2]

SV95C

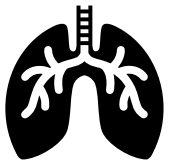
Stride Velocity 95th Centile

5% fastest strides taken during everyday living[3,4]

PUL2.0

Performance Upper Limb v2.0

22-item scale measuring upper limb function[5]



FVC%p

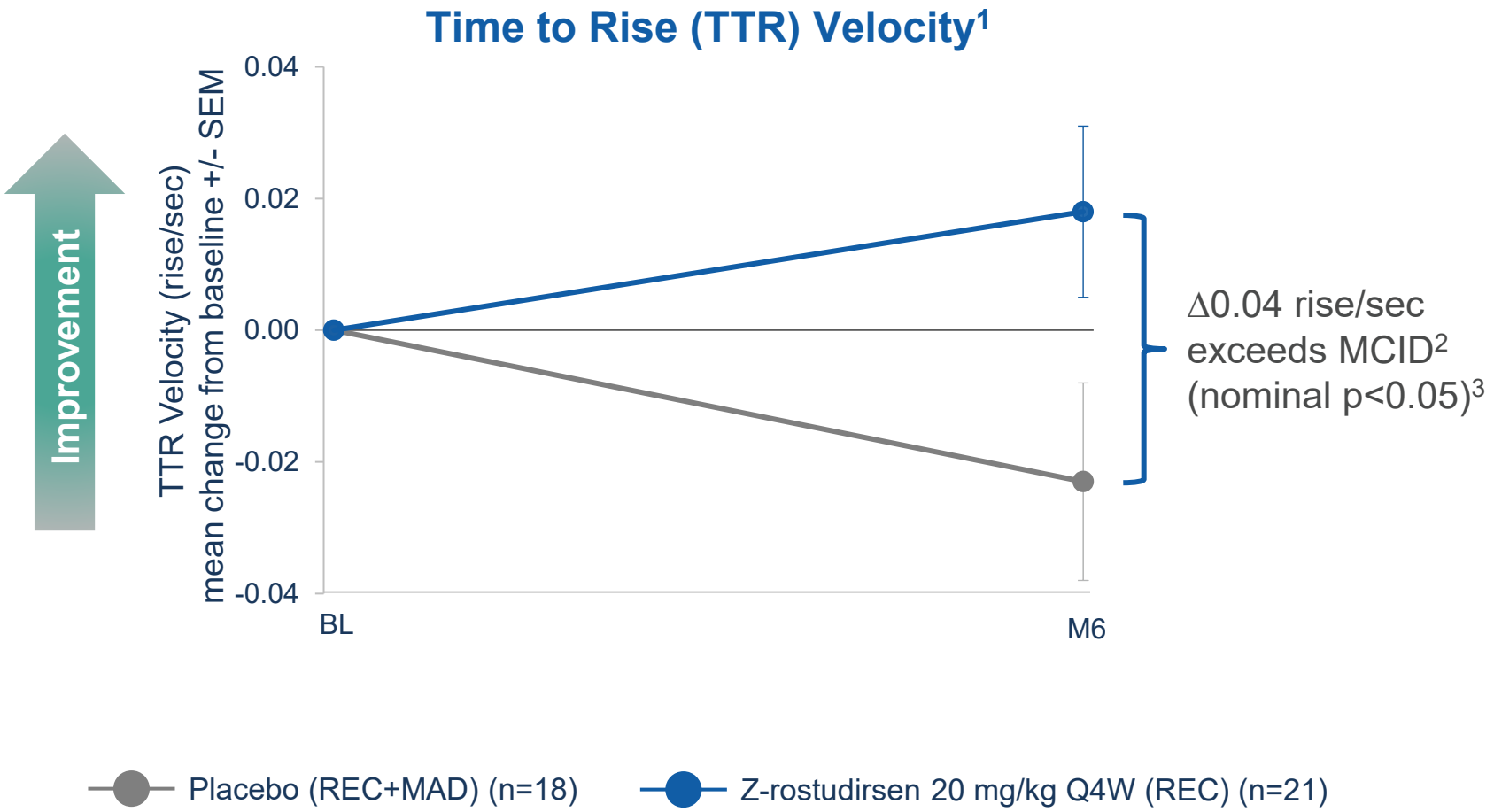
Forced Vital Capacity Percent Predicted

Global assessment of lung function

For functional assessments, placebo data pooled from MAD and REC per prespecified statistical analysis plan

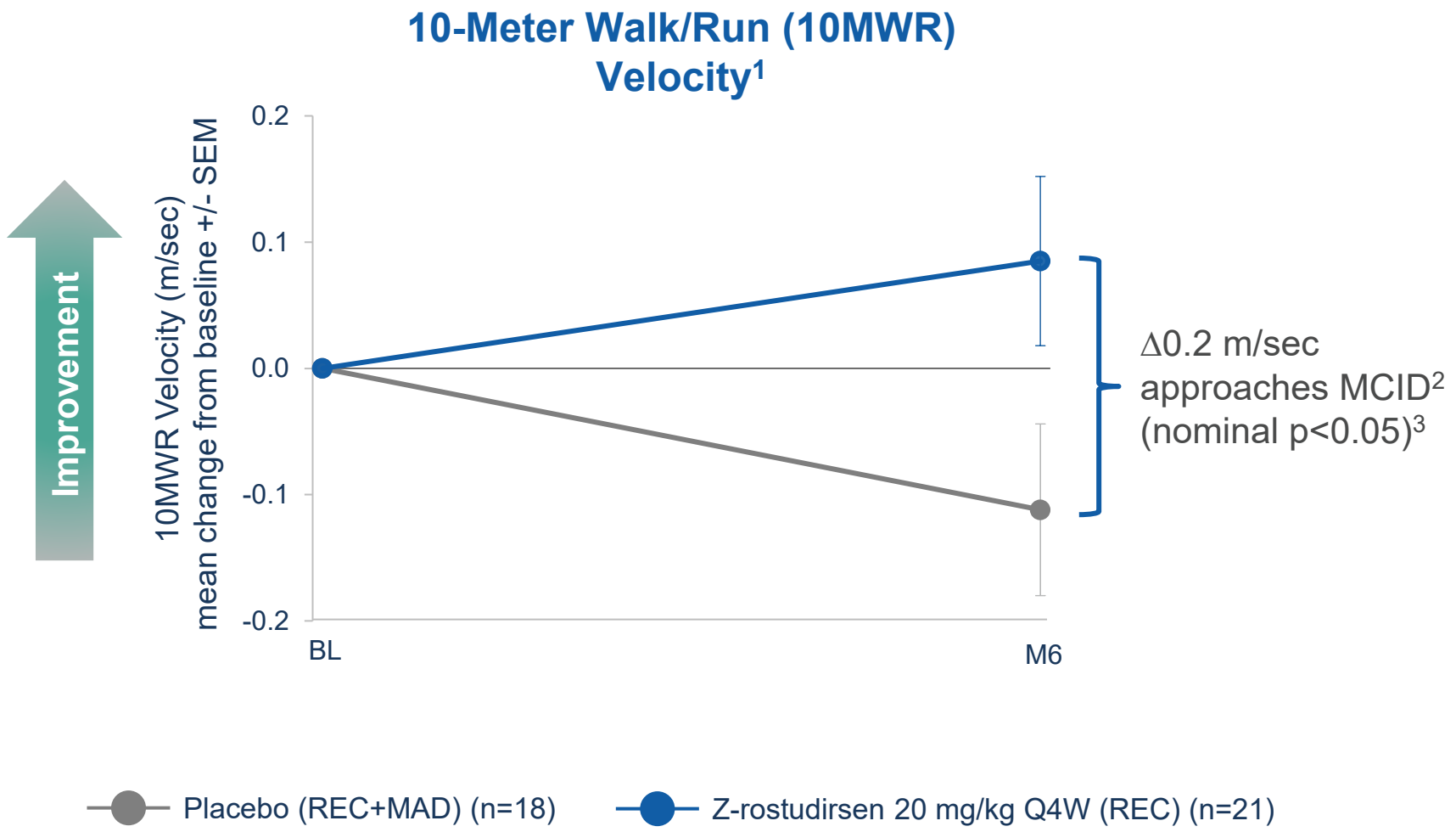
1. Also referred to as rise from floor (RFF). 2. Scott et al. *Physiother Res Int*. 2012; 17:101-109. 3. EMA. Opinion on SV95C. July 2023. https://www.ema.europa.eu/en/documents/scientific-guideline/qualification-opinion-stride-velocity-95th-centile-primary-endpoint-studies-ambulatory-duchenne-muscular-dystrophy-studies_en.pdf. Accessed December 2, 2025. 4, Servais, et al. *Nat Med*. 2023;29(10):2391–2. 5. Mayhew et al. *Dev Med Child Neurol*. 2020; 62(5): 633-639. MAD, multiple ascending dose; REC, registrational expansion cohort.

Improvement in TTR Velocity at 6 Months Exceeded MCID Relative to Placebo with Nominal p<0.05[3]



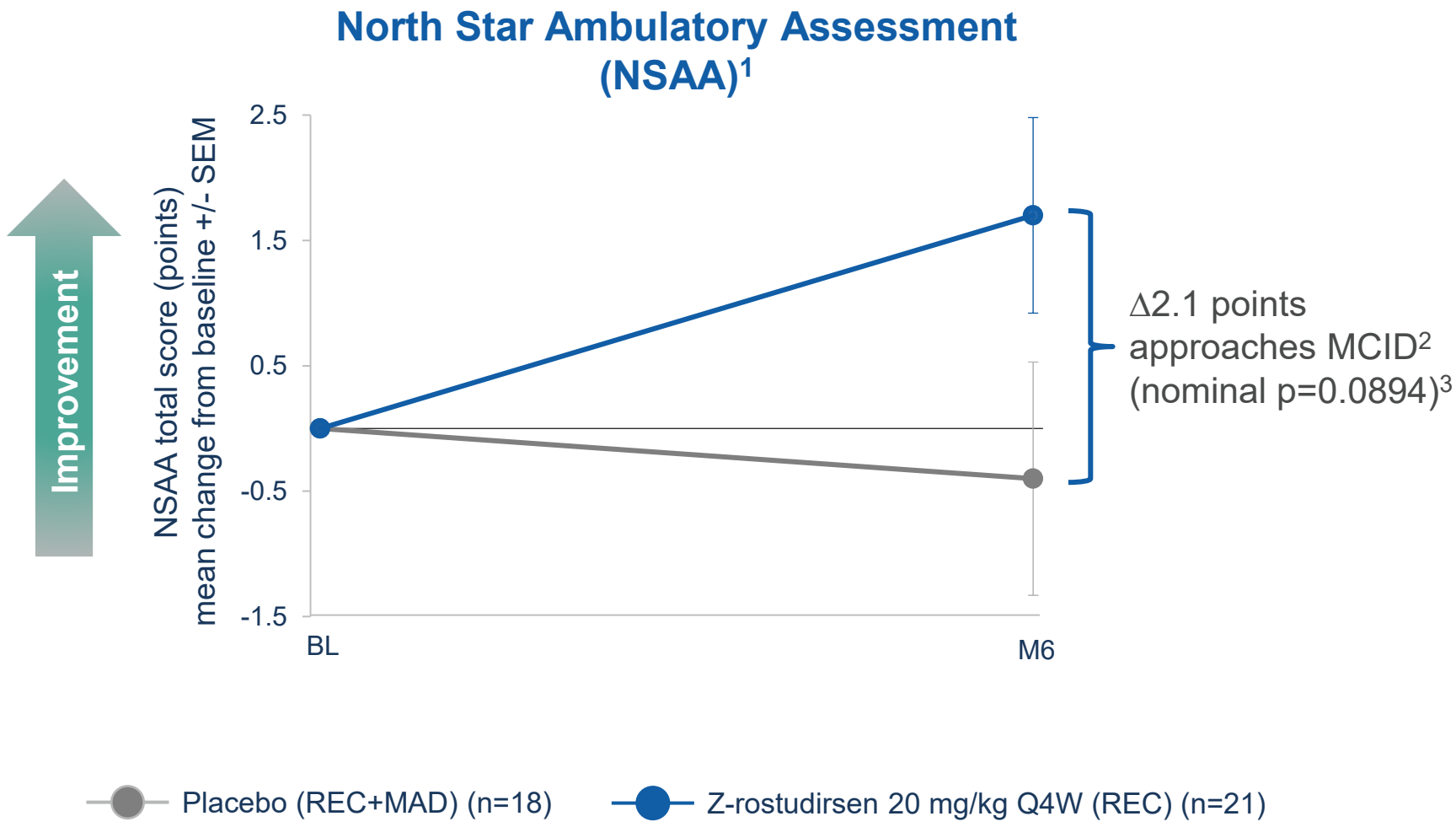
1. Ambulant participants; out-of-threshold or missing values imputed. 2. Duong et al. *J Neuromusc Dis.* 2021; 8(6):939-948; MCID = 0.023 rise/sec. 3. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint. BL, baseline; M6 = 169 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 10MWR Velocity at 6 Months Relative to Baseline and Placebo with Nominal p<0.05[3]



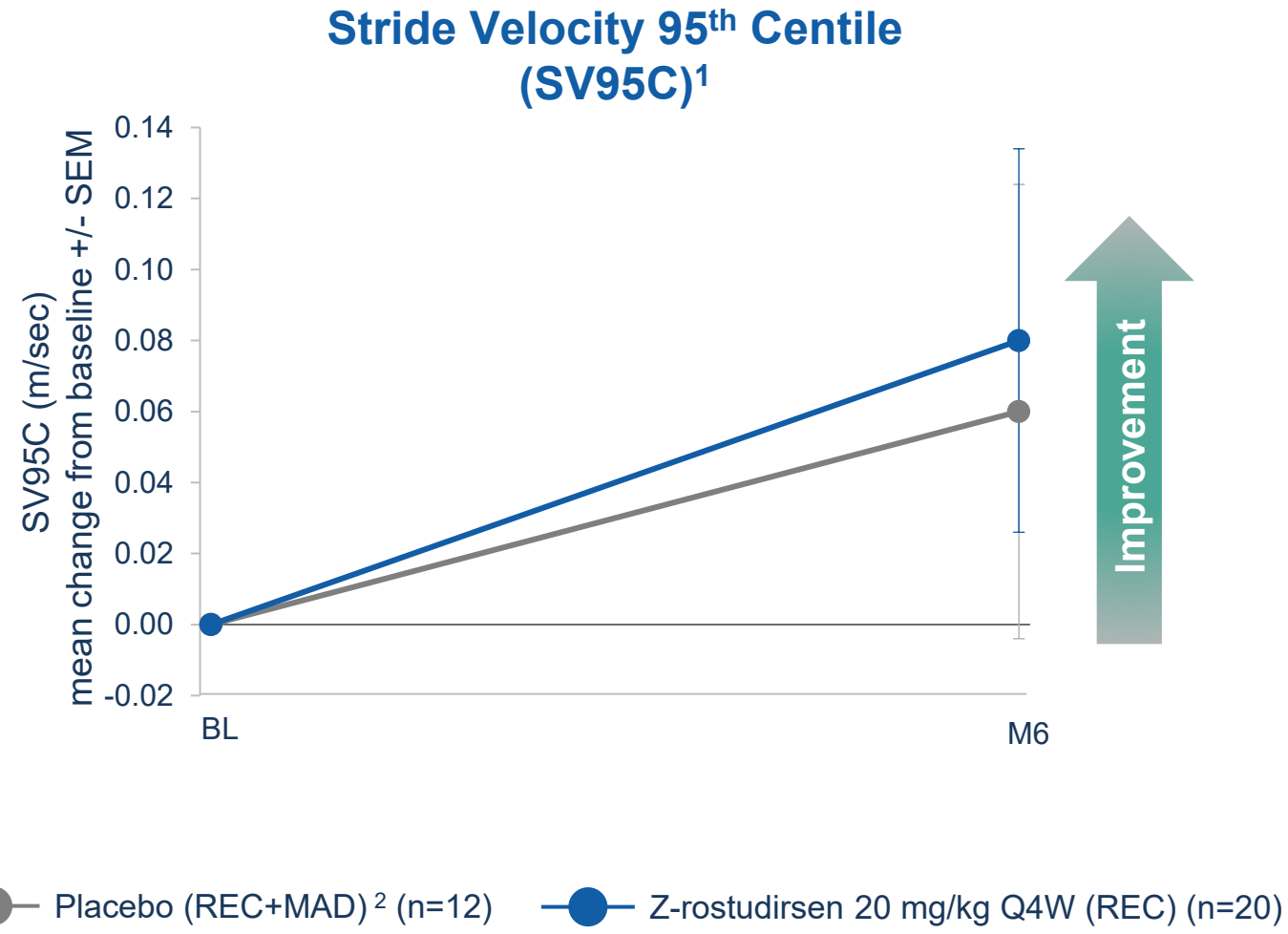
1. Ambulant participants; out-of-threshold or missing values imputed. 2. Duong et al. *J Neuromusc Dis.* 2021; 8(6):939-948; MCID = 0.212 m/sec. 3. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint. BL, baseline; M6 = 169 days; 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 NSAA at 6 Months Relative to Baseline and Placebo



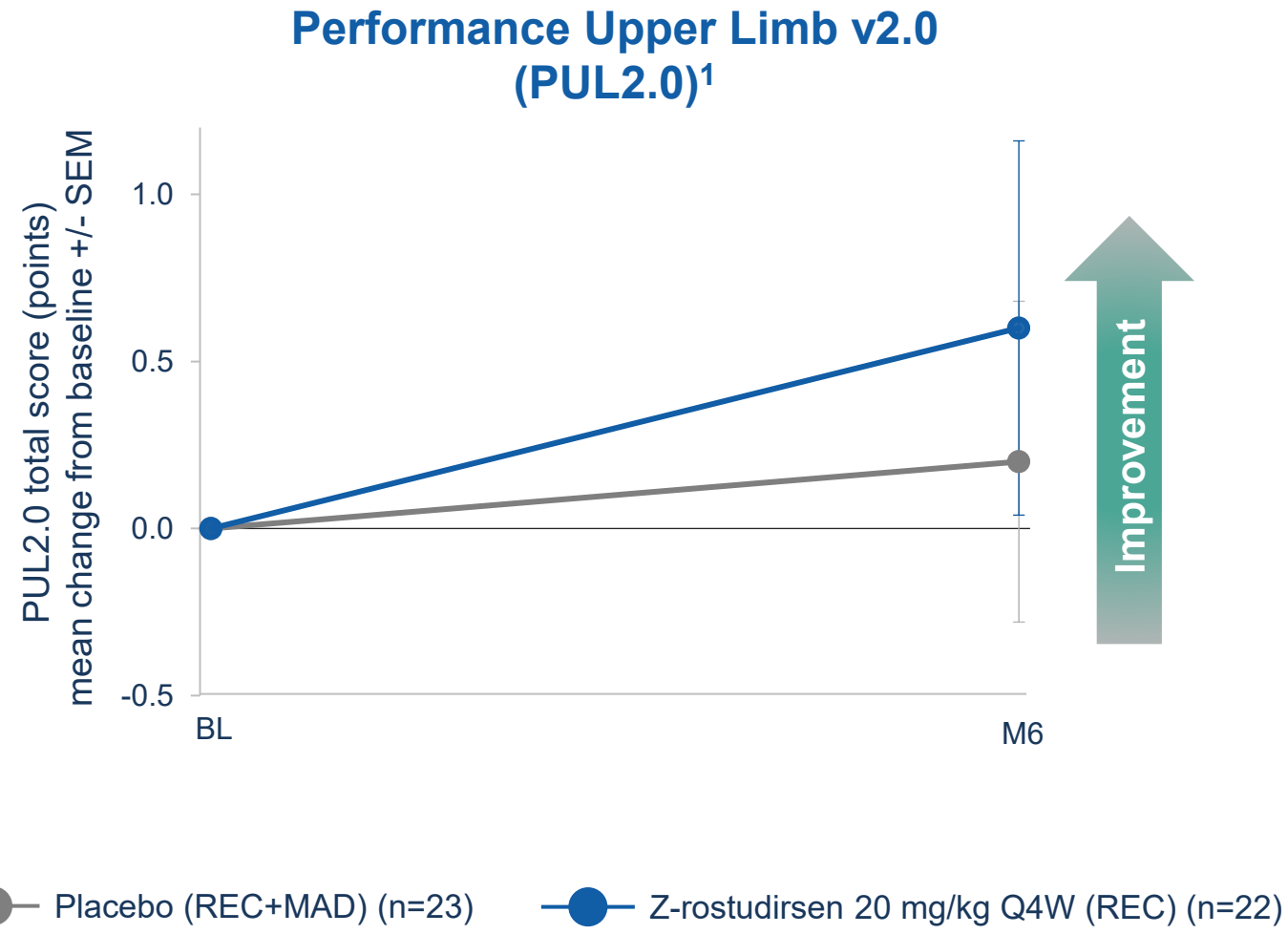
1. Ambulant participants; missing values imputed. 2. Ayyar Gupta et al. *PLoS One.* 2023 Apr 26;18(4):e0283669; MCID ≥2.3 points. 3. Post-hoc analysis; prespecified statistical analysis plan did not include formal hypothesis testing for any functional endpoint. BL, baseline; M6 = 169 days; M, month; 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 SV95C at 6 Months Relative to Baseline



1. Ambulant participants; missing values imputed. 2. 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; REC, registrational expansion cohort; MAD, multiple ascending dose; Q4W, every 4 weeks.

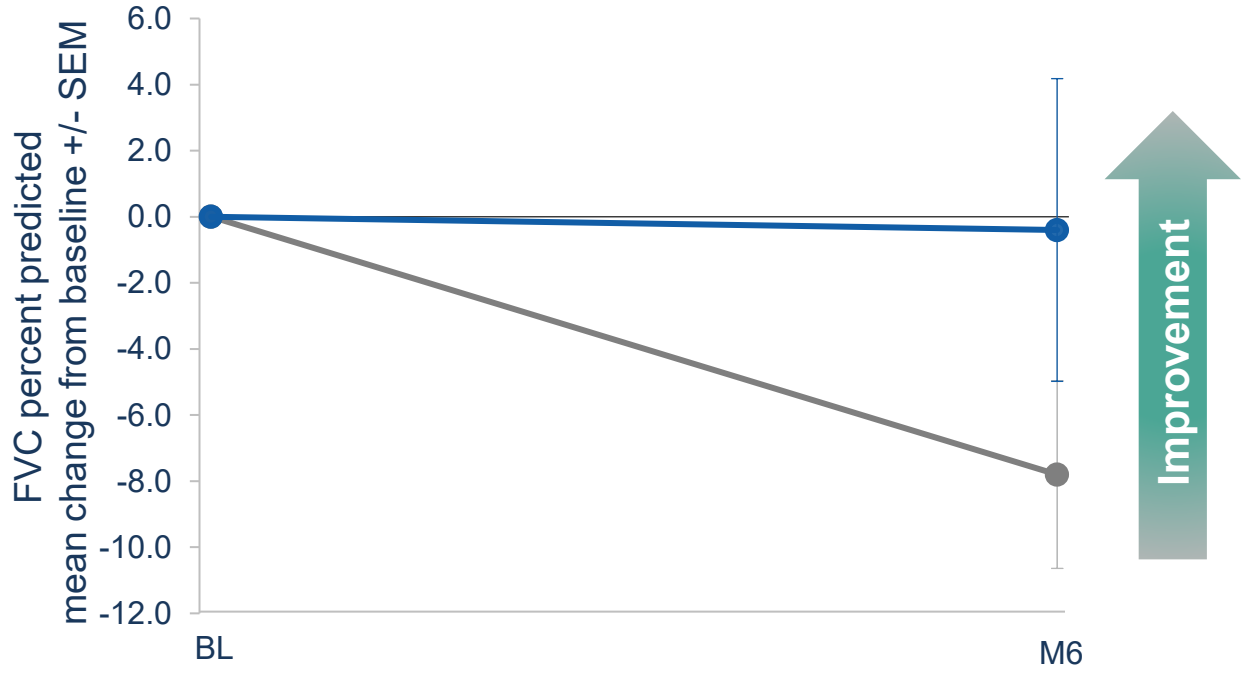
Improvement in PUL2.0 at 6 Months in Ambulant and Non-Ambulant Participants Relative to Baseline and Placebo



1. Ambulant and non-ambulant participants; missing values imputed. 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.

Preservation of Lung Function at 6 Months

Forced Vital Capacity Percent Predicted (FVC%p)[1]



Placebo (REC+MAD) (n=20)

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

1. Ambulant and non-ambulant participants. 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.

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

Z-Rostudirsen for Exon 51 DMD

- **Statistically Significant and Robust Increase in Dystrophin at 6 Months**
- **Favorable Safety & Tolerability Profile up to 36 Months[1]**
- **Functional Improvement Observed Across Multiple Clinical Measures**
- **Convenient Q4W Dosing**

Post-hoc statistical analysis comparing the REC to pooled placebo group at 6 months resulted in ***nominal p<0.05[2] for TTR velocity and 10MWR velocity***

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.

DELIVER Clinical Update Agenda

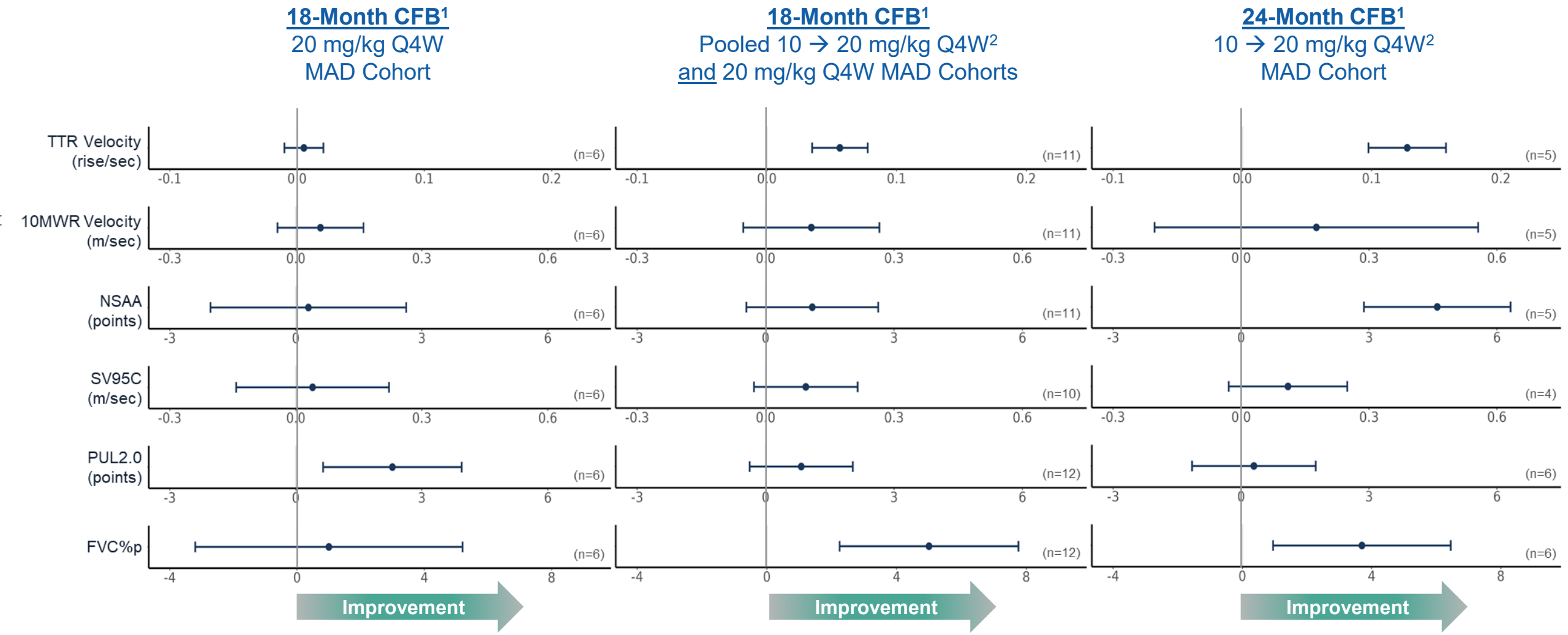
Unmet Need in DMD and Potential for Z-Rostudirsen

Positive Topline Results from Registrational Expansion Cohort (REC) of DELIVER Trial to Support Planned Submission for U.S. Accelerated Approval

New Positive Long-Term Results from DELIVER Trial Showed Sustained Functional Improvement Across Multiple Functional Measures

Results Validate the Potential of FORCE™ Platform

Sustained Functional Improvement Compared to Baseline Across All 6 Measures up to 24 Months in a Broad Participant Population



1. Mean change from baseline +/- SEM; TTR velocity, 10MWR velocity, NSAA, and SV95C analyzed from ambulant participants; PUL2.0 and FVC%p analyzed from ambulant and non-ambulant participants; Out-of-threshold and/or missing values imputed except for FVC%p. 2. Participants 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. Q4W, every 4 weeks; CFB, change from baseline; MAD, multiple ascending dose; TTR, time to rise; 10MWR, 10-meter walk/run; NSAA, north star ambulatory assessment; SV95C, stride velocity 95th centile; PUL2.0, performance upper limb v2.0; FVC%p, forced vital capacity percent predicted.

DELIVER Clinical Update Agenda

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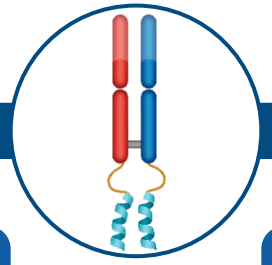
Z-Rostudirsen Data Validate Potential of the FORCE Platform

FORCE



Design Principles of the FORCE Platform

TfR1-mediated delivery to muscle, 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[1] with no persistent related anemia[2] or thrombocytopenia at 20 mg/kg

Convenient Q4W dosing

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.

Advancing Robust Portfolio Focused on Neuromuscular Diseases

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**

PIPELINE EXPANSION OPPORTUNITIES

Rare skeletal, CNS, Cardiac, Metabolic

Program

Opening Remarks

John Cox, President & CEO

Positive Topline Results from DELIVER Trial of Z-Rostudirsen Supporting Potential for U.S. Accelerated Approval

Doug Kerr, M.D., Ph.D., Chief Medical Officer

Closing Remarks

John Cox, President & CEO

Available for Q&A

Erick Lucera, Chief Financial Officer

Executing on a Compelling Commercial Opportunity

Strong Value Proposition to Address Unmet Need in DMD

Z-Rostudirsen for Exon 51 DMD

- **Statistically Significant and Robust Increase in Dystrophin at 6M**
- **Favorable Safety & Tolerability Profile up to 36 Months**[1]
- **Sustained Functional Improvement Observed Across Multiple Clinical Measures**
- **Convenient Q4W Dosing**

Capital Efficient Operating Model

- Leadership team in place with rare neuromuscular disease expertise
- DMD commercial infrastructure planned to be leveraged for future DM1 opportunity
- CMC activities on track to support planned BLA submission in Q2 2026 and potential launch in Q1 2027, assuming Priority Review
- Disciplined capital allocation

1. Data as of August 19, 2025. DMD, Duchenne muscular dystrophy; DM1, myotonic dystrophy type 1; M, month; Q4W, every 4 weeks; CMC, chemistry, manufacturing, and controls; BLA, biologics license application.

Transforming Exon 51 Skip Amenable DMD; Validating the FORCE Platform and Dyne Pipeline

Data to Support Potential Accelerated Approval

- Positive results from Registrational Expansion Cohort of DELIVER trial of z-rostudirsen in exon 51 DMD
- **Met primary endpoint:** Statistically significant and robust increase in dystrophin at 6 months ($p<0.0001$)
- Continued favorable safety profile[1]
- Clear trends relative to placebo across multiple functional endpoints

Sustained Functional Improvement

- Sustained functional improvement from baseline across multiple clinical endpoints out to 24 months, including:
  - TTR Velocity
  - 10MWR Velocity
  - NSAA
  - SV95C
  - PUL2.0
  - FVC%p

Validation of FORCE™ Platform

- Data show that FORCE platform can deliver payloads to targeted tissues, with favorable therapeutic window
- Leveraging same delivery approach for z-basivarsen in DM1 and early-stage pipeline in FSHD, Pompe disease and other DMD exons

Advancing z-rostudirsen toward Dyne's first approval

Planned BLA submission for U.S. Accelerated Approval on track for Q2 2026

Potential U.S. launch in Q1 2027, assuming FDA grants Priority Review

Phase 3 study initiation planned for Q2 2026

1. Safety data as of August 19, 2025; DMD, Duchenne muscular dystrophy; DM1, myotonic dystrophy type 1; FSHD, facioscapulohumeral muscular dystrophy; BLA, biologics license application; TTR, time to rise; 10MWR, 10-meter walk/run; NSAA, north star ambulatory assessment; SV95C, stride velocity 95th centile; PUL2.0, performance upper limb v2.0; FVC%p, forced vital capacity percent predicted.

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 *1st potential launch for Dyne*	**Q1 2028**	Potential U.S. launch, assuming Priority Review

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

THANK YOU

to all who participated

DELIVER

Dyne THERAPEUTICS