



# Building the World's Leading Neuromuscular Disease Company

COMPANY OVERVIEW | MARCH 2026



Sarah, living with DM1

# 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, the therapeutic potential of zeleciment basivarsen (z-basivarsen, also known as DYNE-101), zeleciment rostudirsen (z-rostudirsen, also known as DYNE-251), DYNE-302, DYNE-401, DYNE-253, DYNE-245, DYNE-244, and DYNE-255, 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.

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# Dyne is on a Mission to Deliver Functional Improvement for the Neuromuscular Community



## DYNE IS AIMING TO DELIVER FUNCTIONAL IMPROVEMENT

for people living with genetically driven neuromuscular diseases

Dyne was built to address one of the most significant challenges in biotech: how to effectively and broadly deliver a therapeutic payload that results in measurable and meaningful functional improvement to patients



### DYNE SNAPSHOT

FORCE™ platform uses an antigen-binding fragment (Fab) to target the transferrin receptor 1 (TfR1), which is expressed on muscle cells and the blood brain barrier

The FORCE Fab harnesses TfR1 to deliver a therapeutic payload, leveraging a naturally occurring mechanism

Dyne is advancing a broad pipeline, including two clinical programs, z-basivarsen (DYNE-101) for myotonic dystrophy type 1 (DM1) and z-rostudirsen (DYNE-251) for Duchenne muscular dystrophy (DMD)

**We are on a mission to deliver functional improvement for individuals, families and communities living with serious neuromuscular diseases**



### DYNE MILESTONES

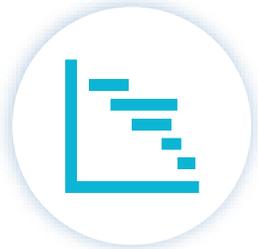
- **2019 | RESEARCH**  
Dyne launches
- **2020-2021 | PRECLINICAL**  
DMD candidate selected  
DM1 candidate selected
- **2022 | CLINIC**  
FIH trials for DYNE-101 & DYNE-251
- **2023 | CLINICAL PROGRESS**  
DYNE-101 & DYNE-251 dose escalation based on safety results
- **2024 | CLINICAL POC**  
DYNE-101 & DYNE-251 early functional improvement
- **2025 | SUSTAINED FUNCTIONAL IMPROVEMENT & SAFETY RESULTS**

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



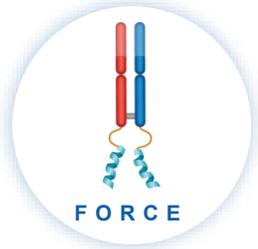
## LATE-STAGE CLINICAL PIPELINE

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



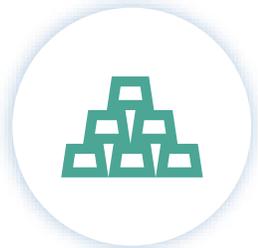
## NEAR-TERM VALUE DRIVERS

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, Pompe, and additional DMD exons



## STRONG FINANCIAL POSITION

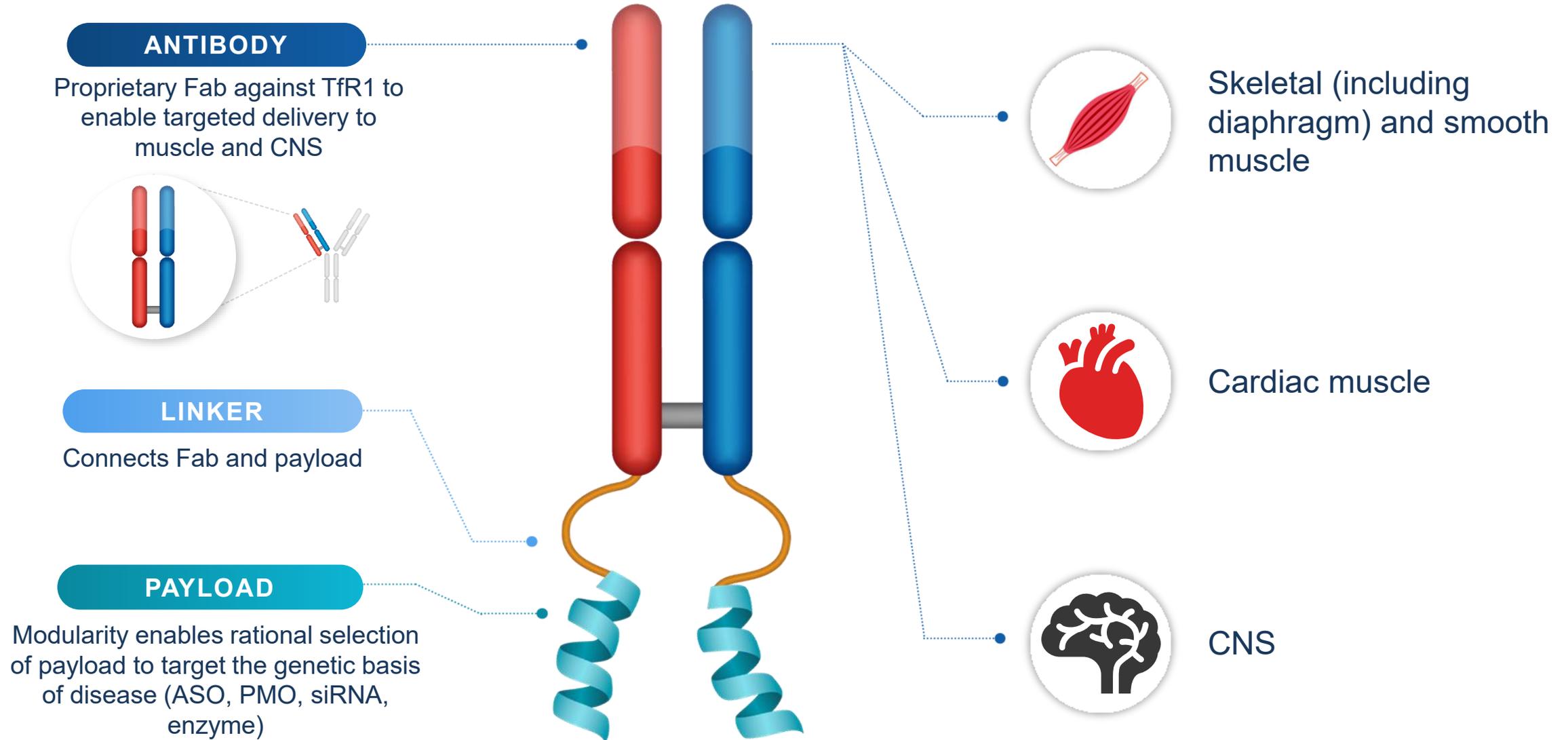
Cash position of ~\$1.1 billion (as of 12/31/25) with expected runway into Q1 2028; all assets fully owned

# 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 	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

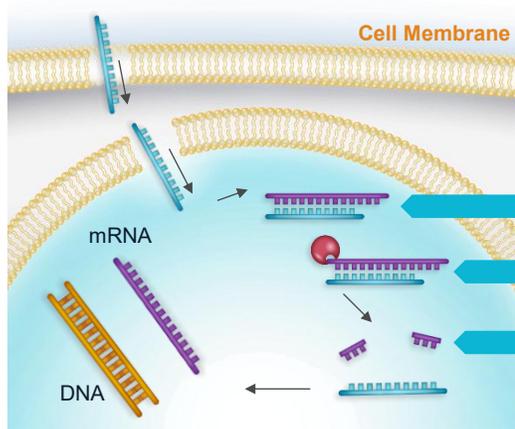
**One capital efficient operating model to support multiple potential commercial launches**

# Leveraging Our FORCE™ Platform for Targeted Delivery

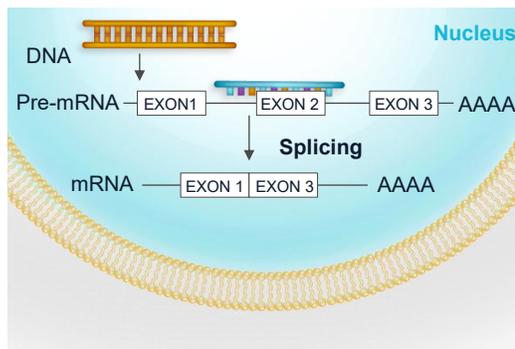


# Rationally Select Payload to Target Genetic Basis of Disease

## ASO acts in the nucleus and cytoplasm

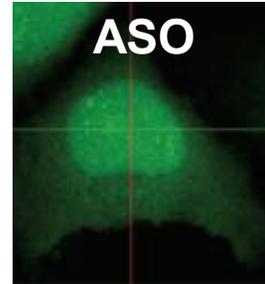


## Splice-modulating ASO



Single-Stranded Antisense

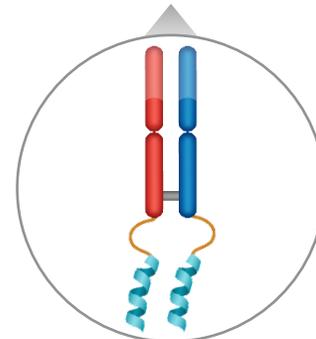
## Subcellular distribution of ASO and siRNA



Nuclear localization

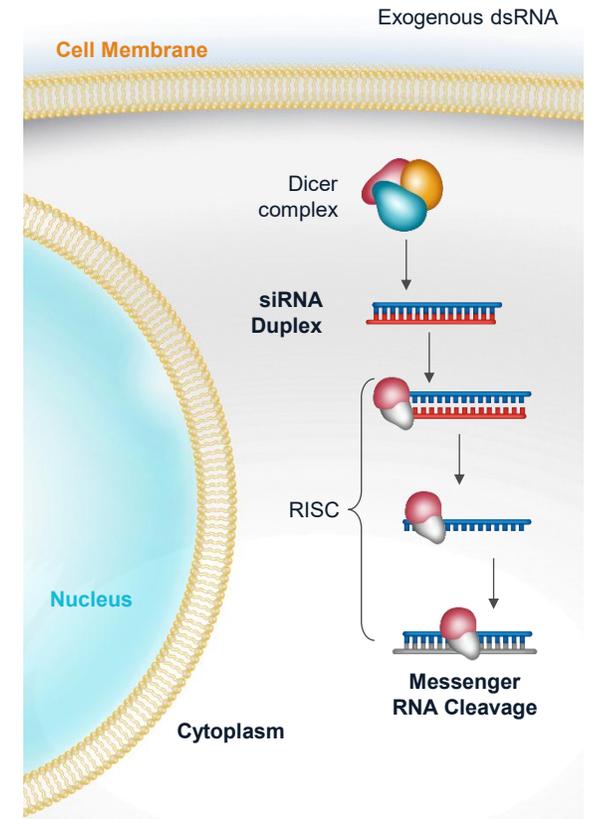


Cytoplasmic localization



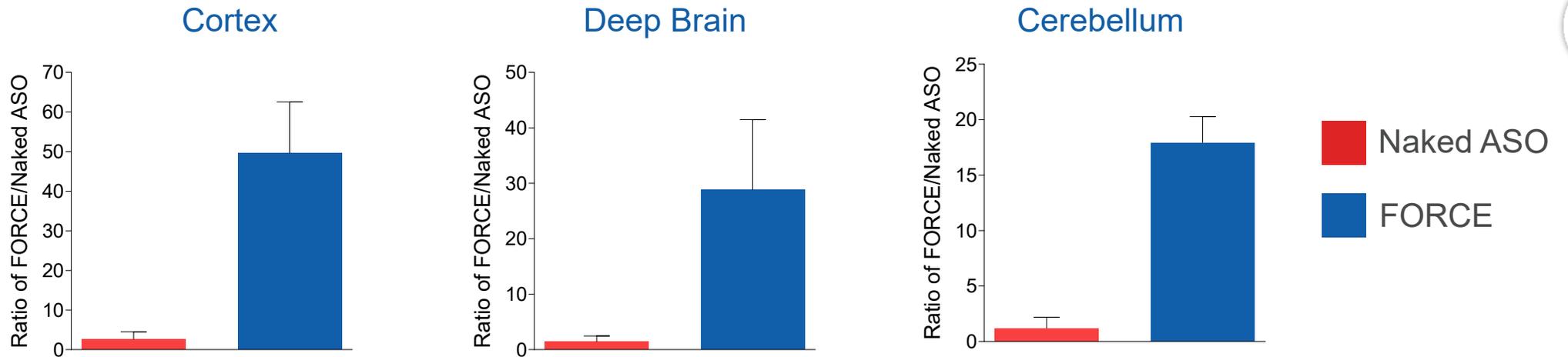
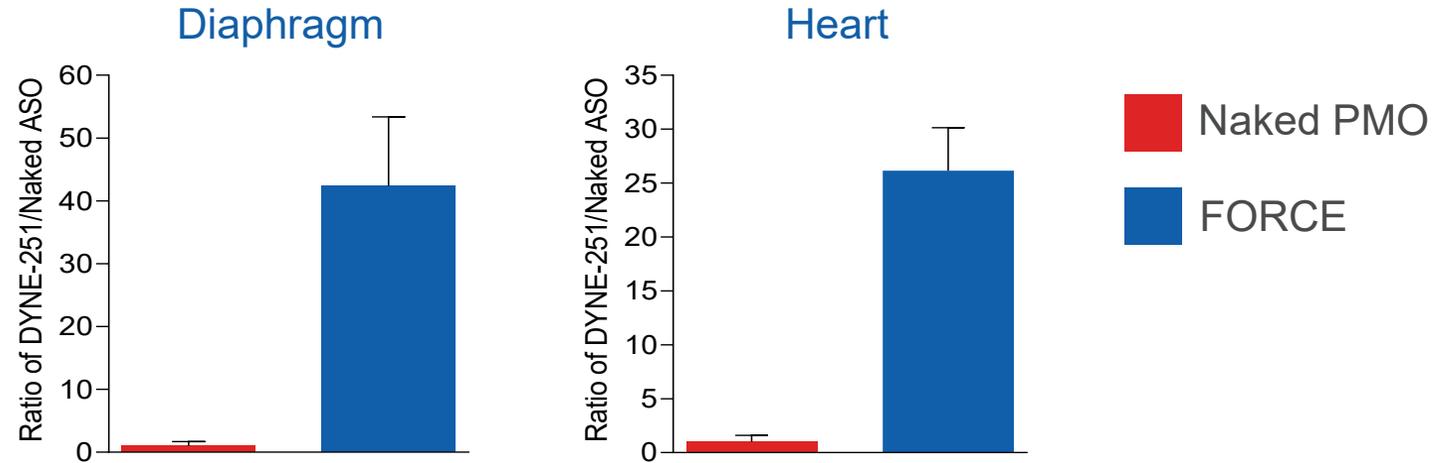
**FORCE** delivers **ASO** payload for nuclear targets, **siRNA** payload for cytoplasmic targets

## siRNA acts in the cytoplasm



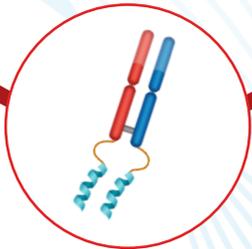
Double-Stranded Antisense (siRNA)

# FORCE Enabled Oligonucleotide Delivery to Muscle and CNS



# FORCE Platform Designed to Deliver Significant Advantages

**Provide  
Functional  
Improvement**



- ✓ **Targeted Delivery**  
Leverages TfR1 expression on skeletal, cardiac, and smooth muscle as well as blood-brain-barrier
- ✓ **Redosable administration**  
Durable efficacy and treatment optionality
- ✓ **Extended Durability**  
Potential for prolonged disease-modifying effects, enabling less frequent dosing
- ✓ **Targets Genetic Basis of Disease**  
Rationally select payloads to match target biology
- ✓ **Enhanced Tolerability**  
Targeted delivery limits systemic drug exposure and use of Fab minimizes disruption to TfR1 receptor
- ✓ **Reduced Development and Manufacturing Costs**  
A single Fab utilized across all programs

# Neuromuscular Pipeline Leveraging Clinically Validated Platform

DISEASE & PREVALENCE	TARGET	PRECLINICAL	CLINICAL	REGISTRATIONAL	COMMERCIAL
<b>Duchenne muscular dystrophy (DMD)</b> US: ~12,000 Europe: ~16,000	Exon 51	zeleciment rostudirsen (z-rostudirsen)			Planned BLA submission Q2 2026
<b>Myotonic dystrophy type 1 (DM1)</b> US: ~40,000 Europe: ~55,000	DMPK	zeleciment basivarsen (z-basivarsen)			
<b>Facioscapulohumeral muscular dystrophy (FSHD)</b> US: ~15,000-40,000 Europe: ~20,000-50,000	DUX4	DYNE-302			
<b>Duchenne muscular dystrophy (DMD)</b> US: ~12,000 Europe: ~16,000	Exon 53	DYNE-253			
	Exon 45	DYNE-245			
	Exon 44	DYNE-244			
	Exon 55	DYNE-255			
<b>Pompe disease</b> US: ~4,500 Europe: ~5,500	GAA	DYNE-401			

PIPELINE EXPANSION OPPORTUNITIES: CNS, Rare skeletal, Cardiac, Metabolic

# 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**

# DELIVER Trial 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>

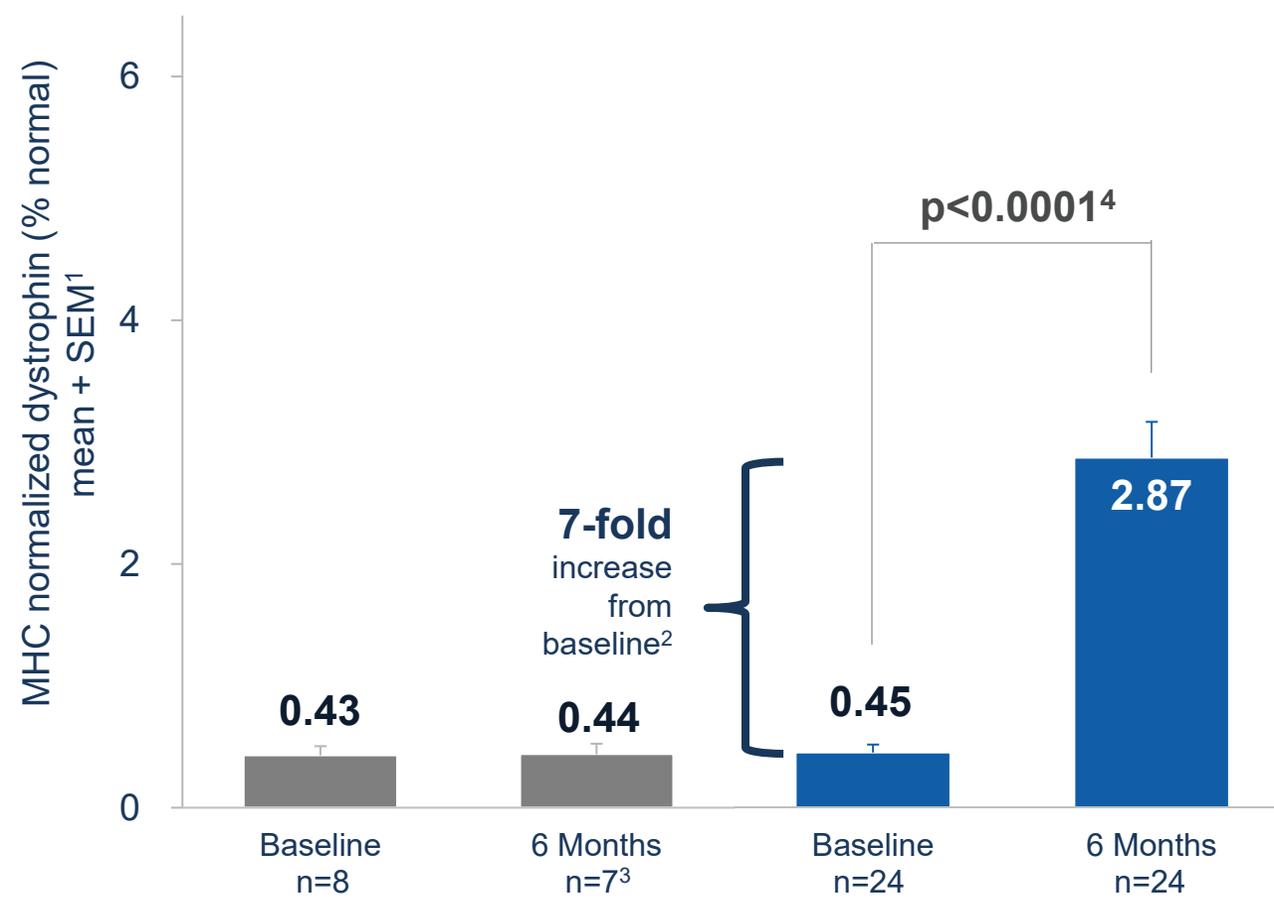


Planned submission for U.S. Accelerated Approval based on positive results from Registrational Expansion Cohort

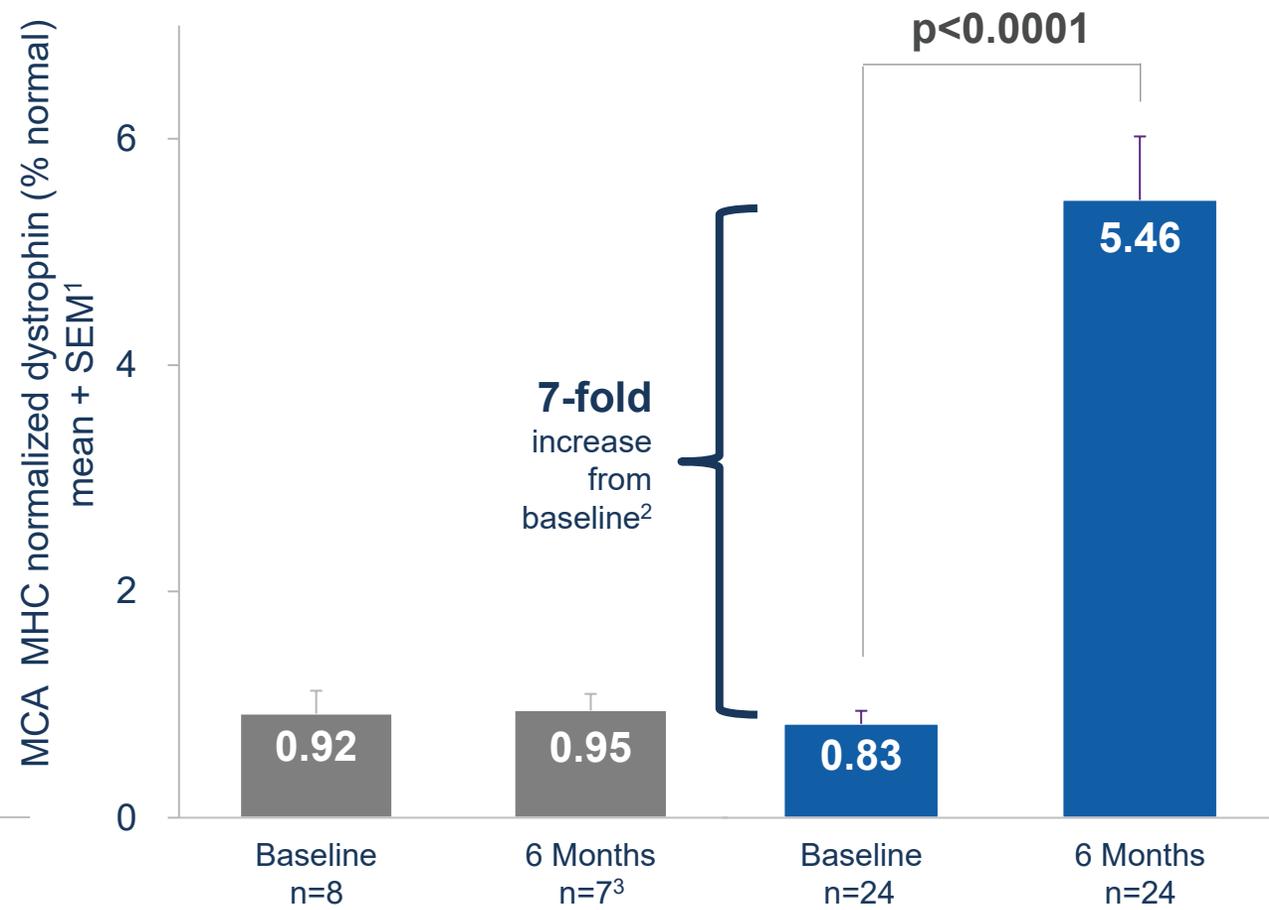
**Confirmatory Phase 3 trial planned to support full approval of z-rostudirsen globally**

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

## Unadjusted dystrophin



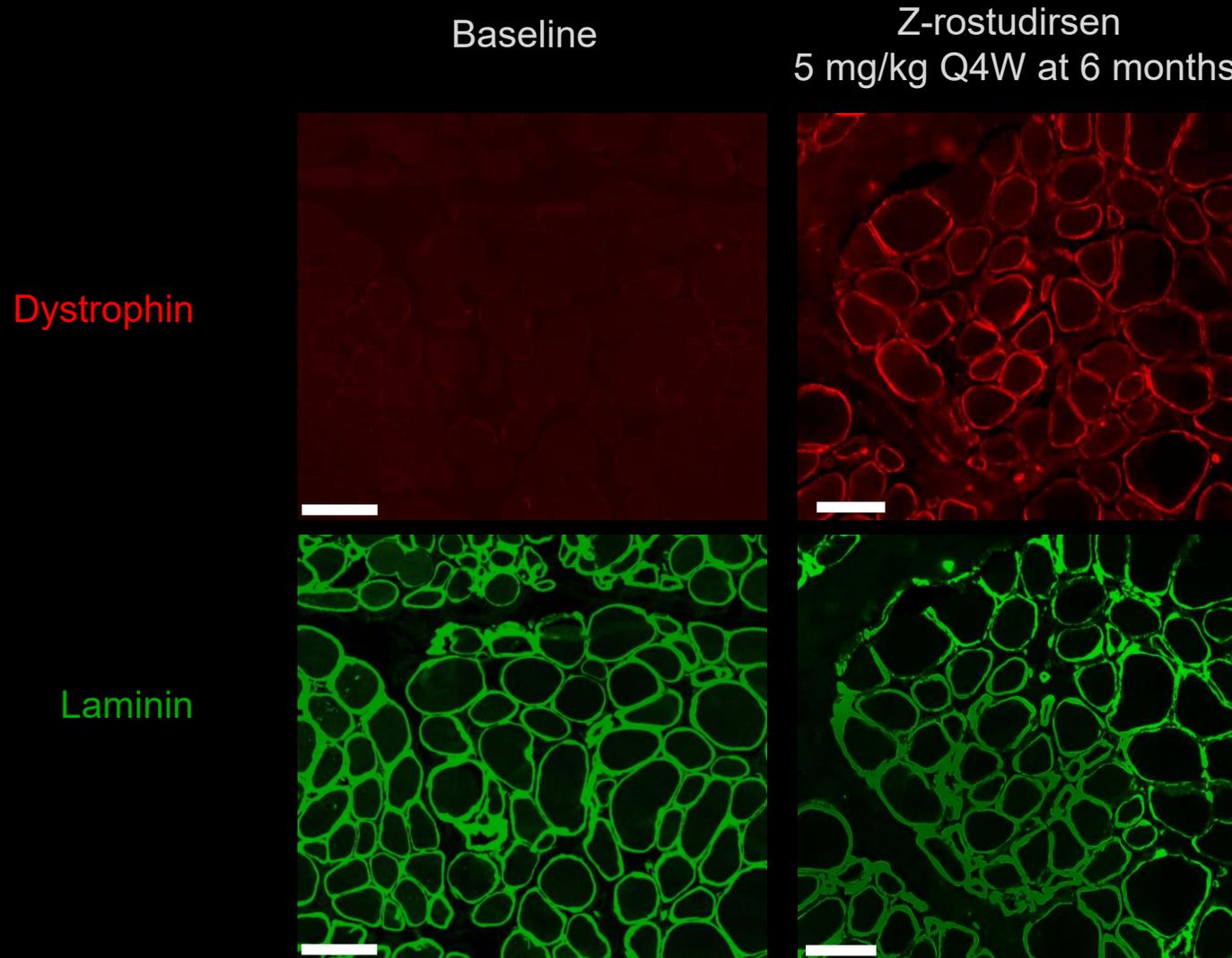
## Muscle content-adjusted dystrophin<sup>5</sup>



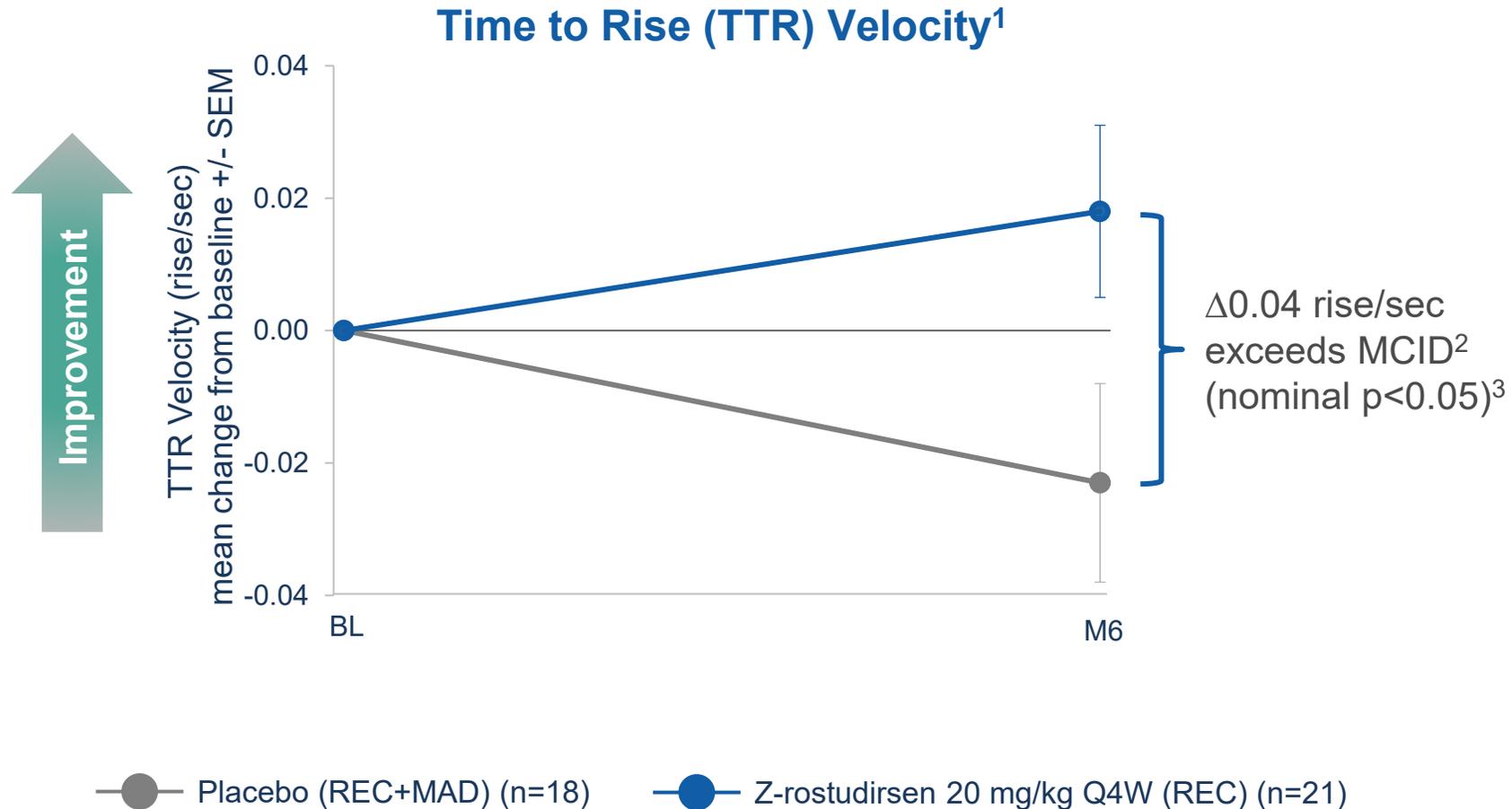
■ Placebo

■ Z-rostudirsen 20 mg/kg Q4W (REC)

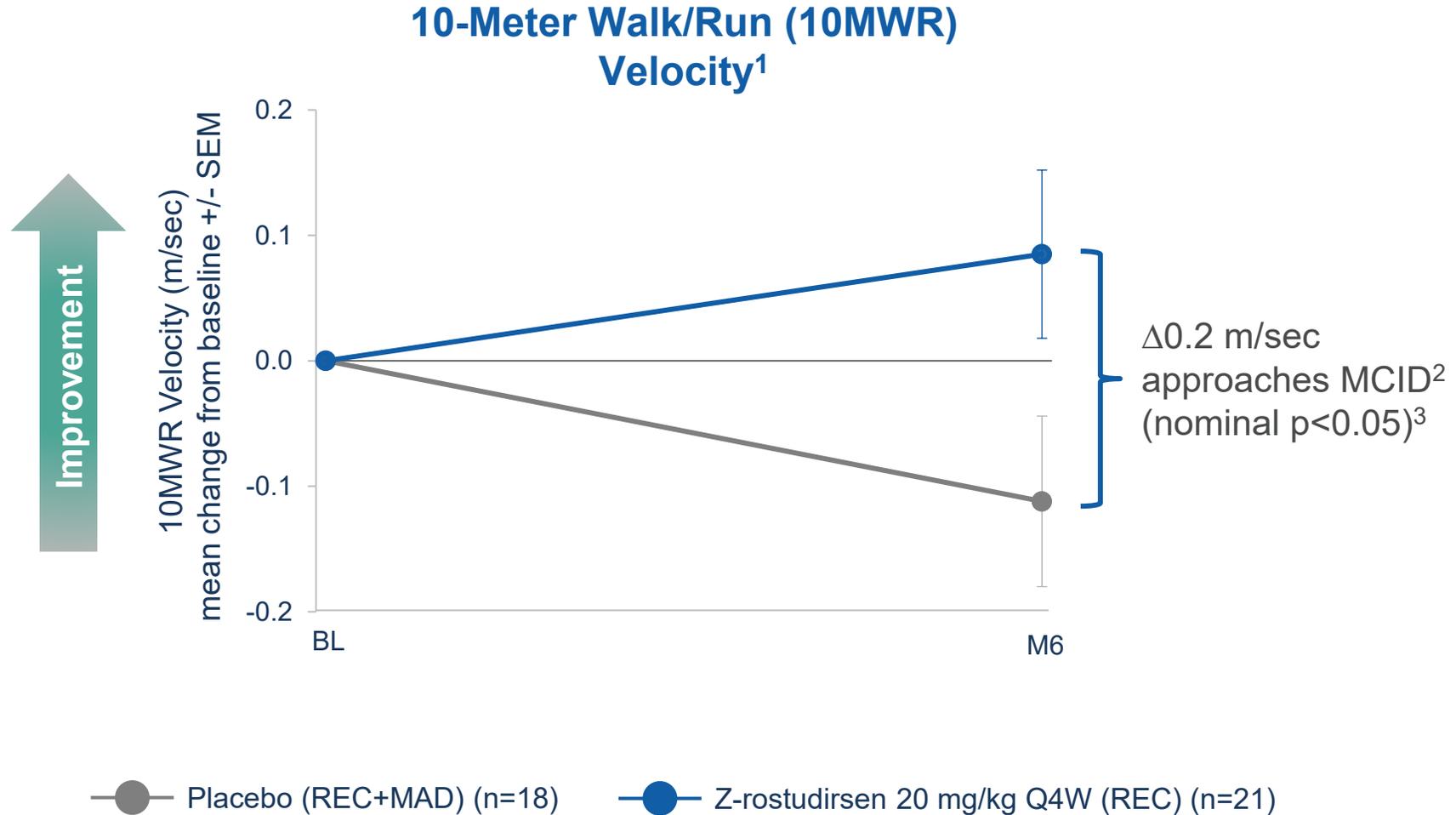
# Clear Improvement in Dystrophin Localization to Sarcolemma



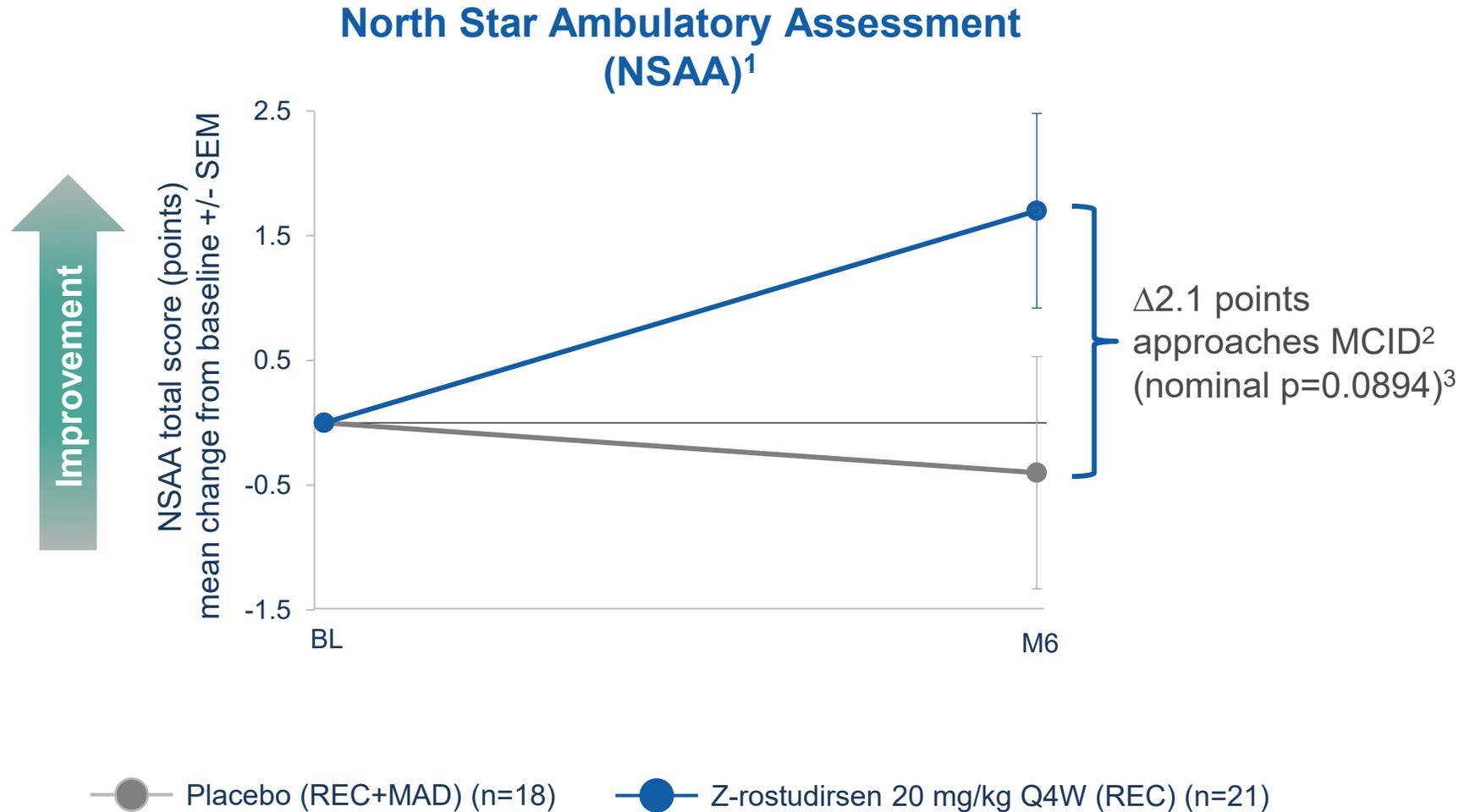
# Improvement in TTR Velocity at 6 Months Exceeded MCID Relative to Placebo with Nominal $p < 0.05^3$



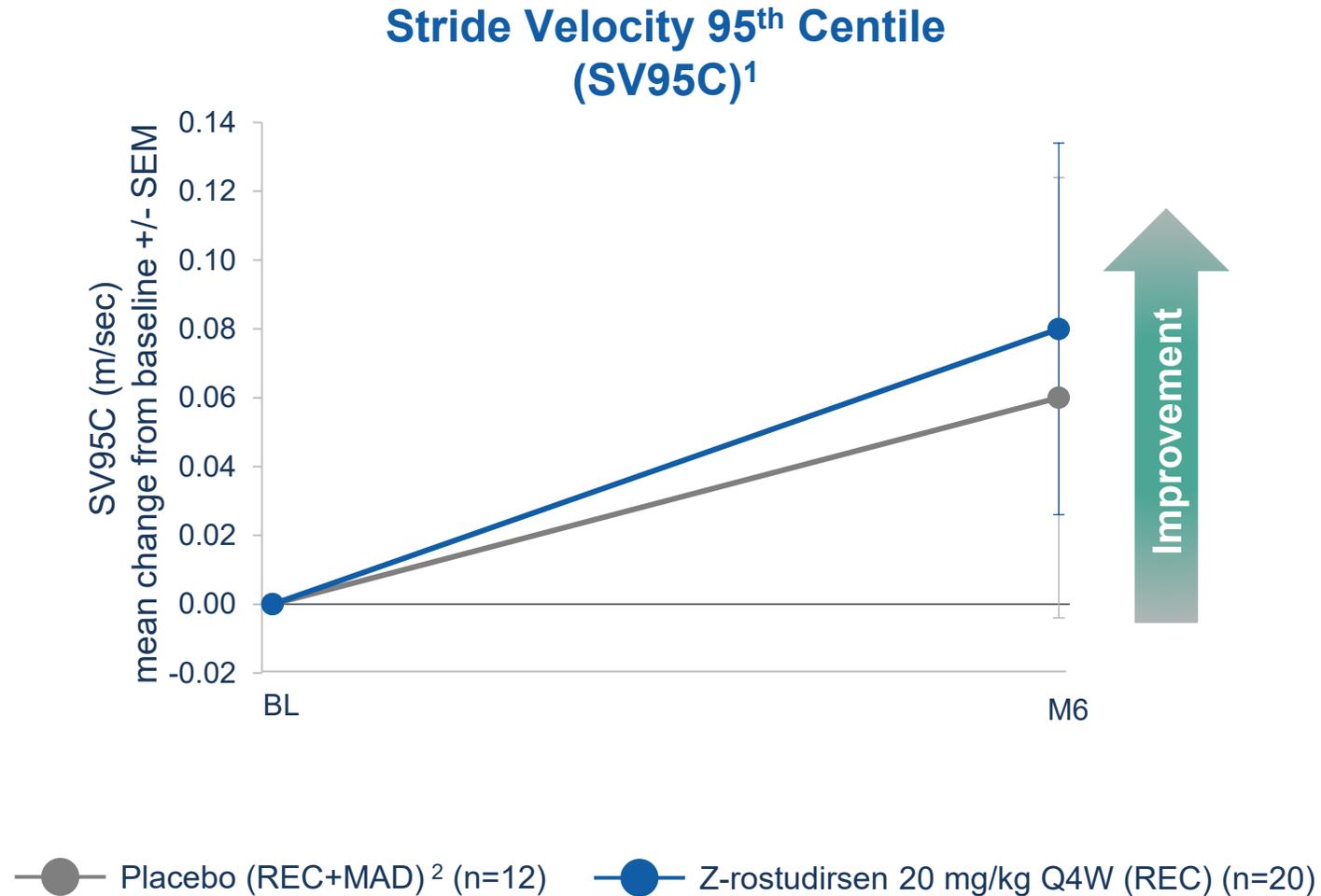
# Improvement in 10MWR Velocity at 6 Months Relative to Baseline and Placebo with Nominal $p < 0.05^3$



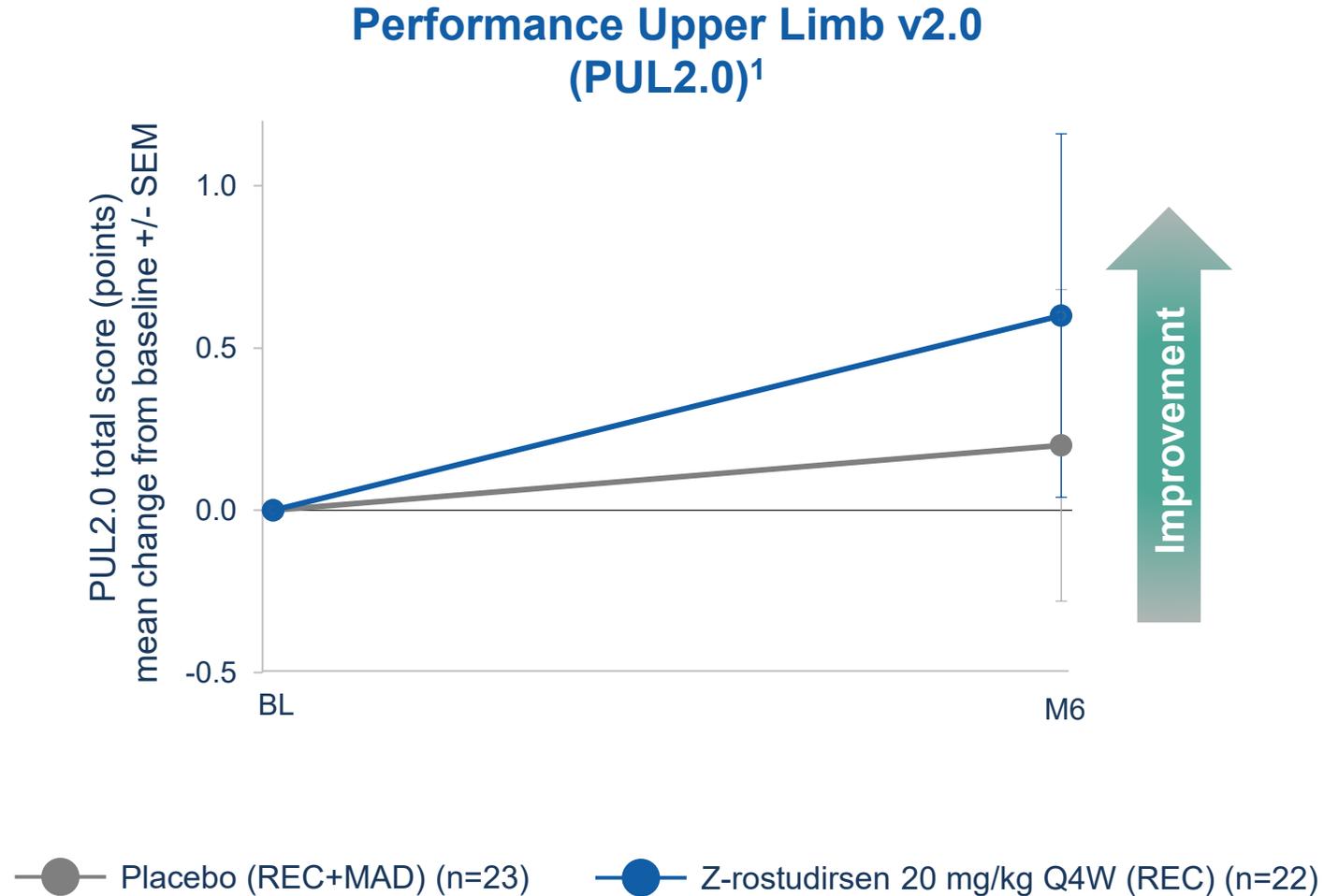
# Improvement in NSAA at 6 Months Relative to Baseline and Placebo



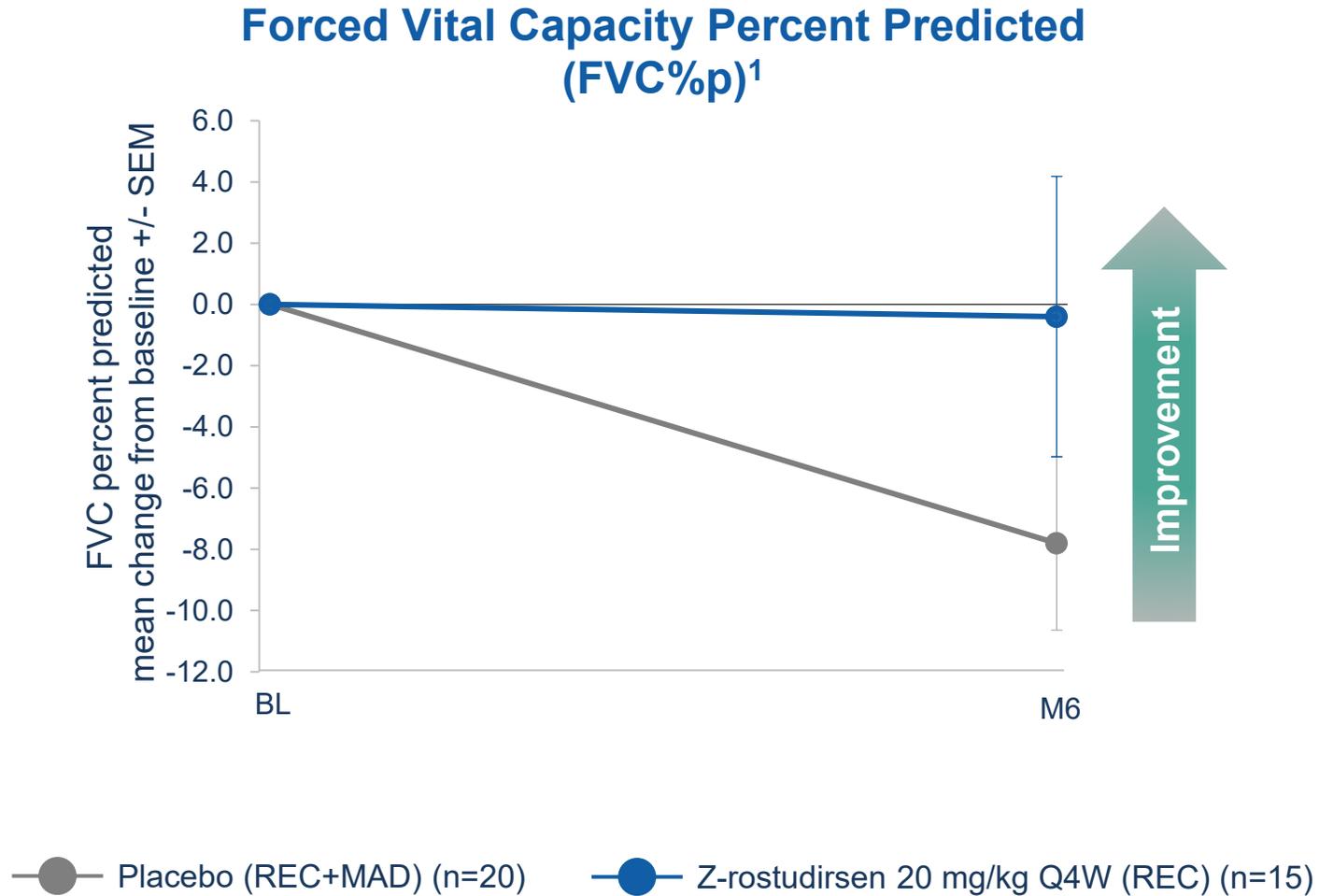
# Improvement in SV95C at 6 Months Relative to Baseline



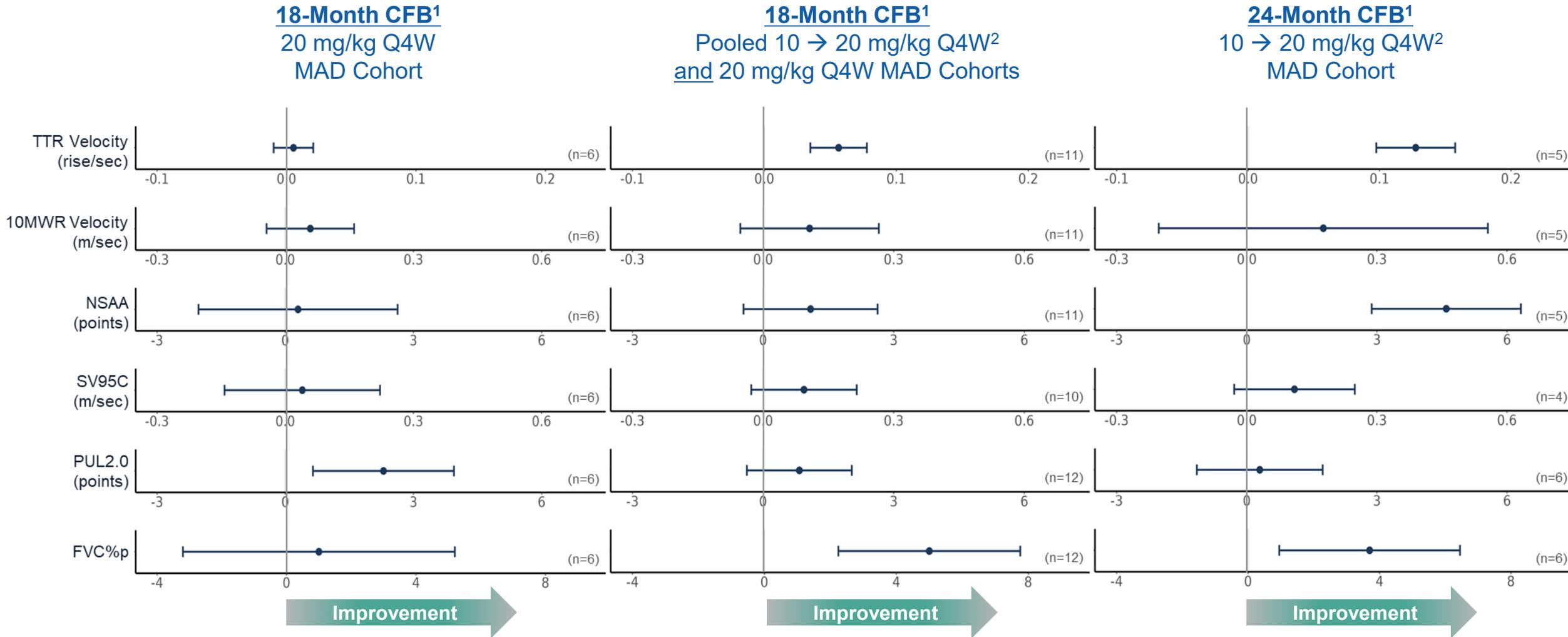
# Improvement in PUL2.0 at 6 Months in Ambulant and Non-Ambulant Participants Relative to Baseline and Placebo



# Preservation of Lung Function at 6 Months



# 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 95<sup>th</sup> centile; PUL2.0, performance upper limb v2.0; FVC%p, forced vital capacity percent predicted.

# 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	✓	✓

# 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

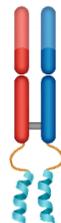
- No participants have persistent related anemia<sup>10</sup> or thrombocytopenia

**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.

# 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

# 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>

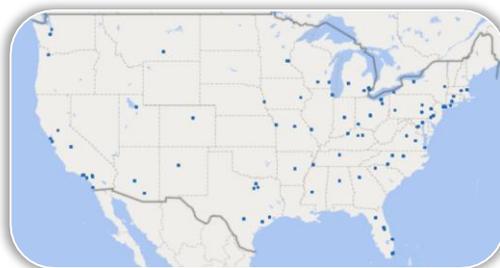
- ~12,000 US DMD population
  - ~1,600 US Exon 51 skip amenable DMD
- Active and educated patient community
- HHS added DMD to RUSP in December 2025

## 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

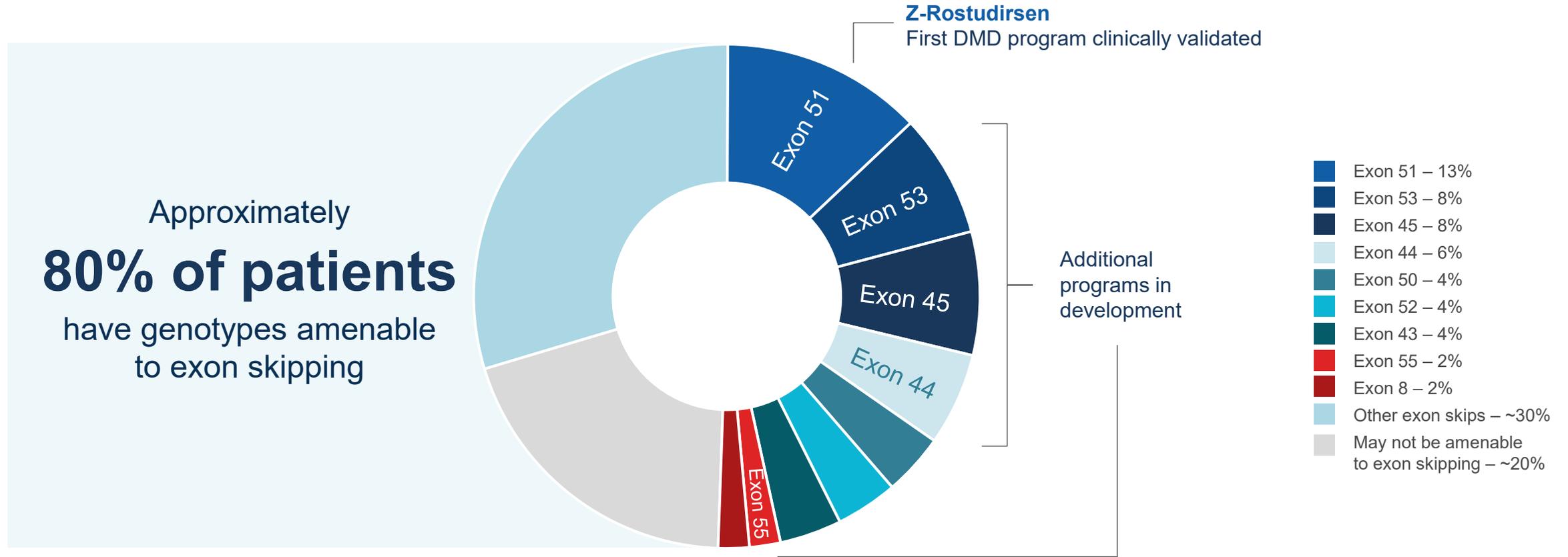
## Established Market with Reimbursement

**~\$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

# 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**

# 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

# Z-Basivarsen Addressing the Central Pathobiology of DM1 to Enable Broad Functional Improvement<sup>1</sup>

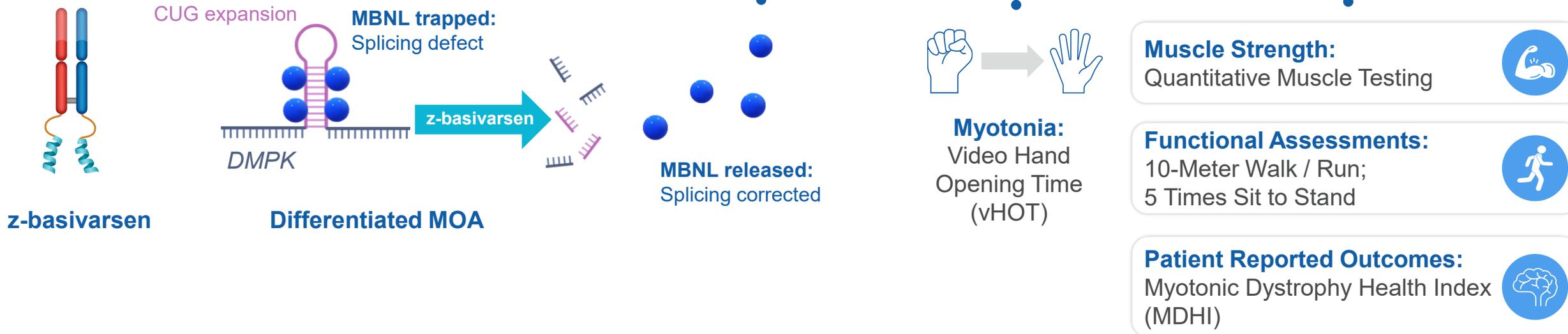
Robust and widespread delivery

DMPK degradation in the nucleus

MBNL release and splicing correction

Early clinical effect

Broad functional improvement



# ACHIEVE Trial 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



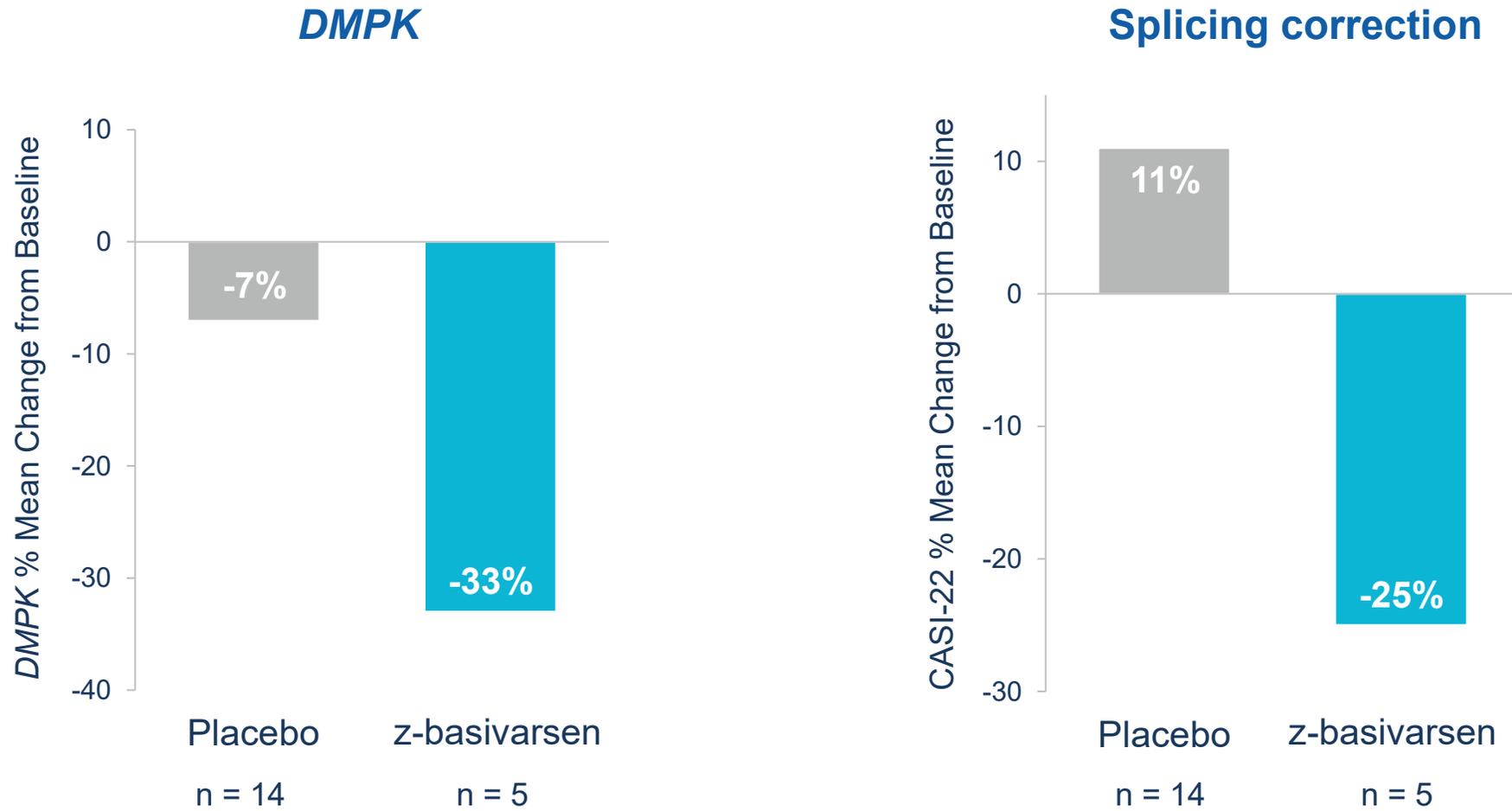
Favorable safety profile<sup>1</sup>



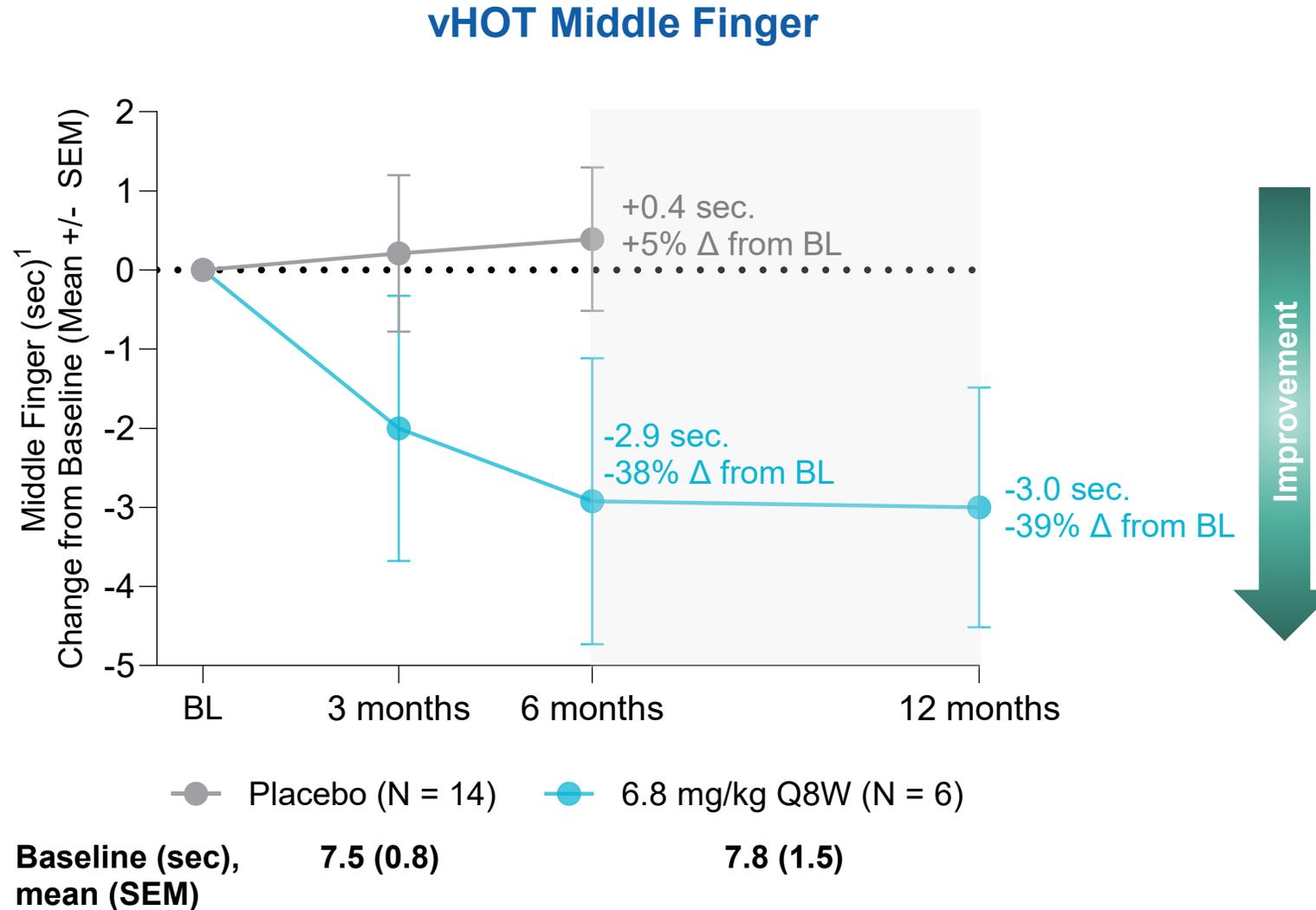
Potential submission for U.S. Accelerated Approval based on data from Registrational Expansion Cohort (REC)

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

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

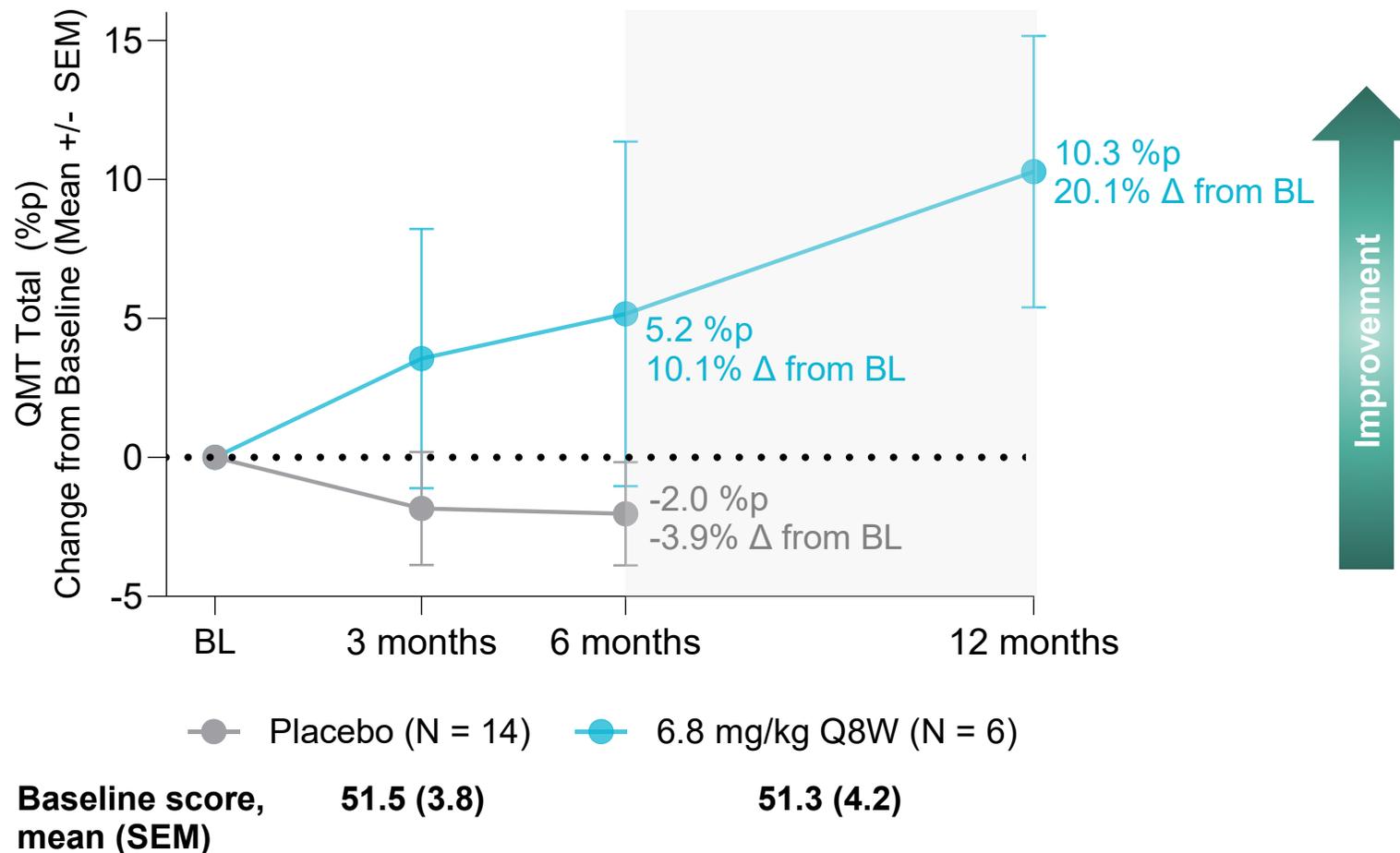


# Robust and Sustained vHOT Improvement with Z-Basivarsen at 6 and 12 Months

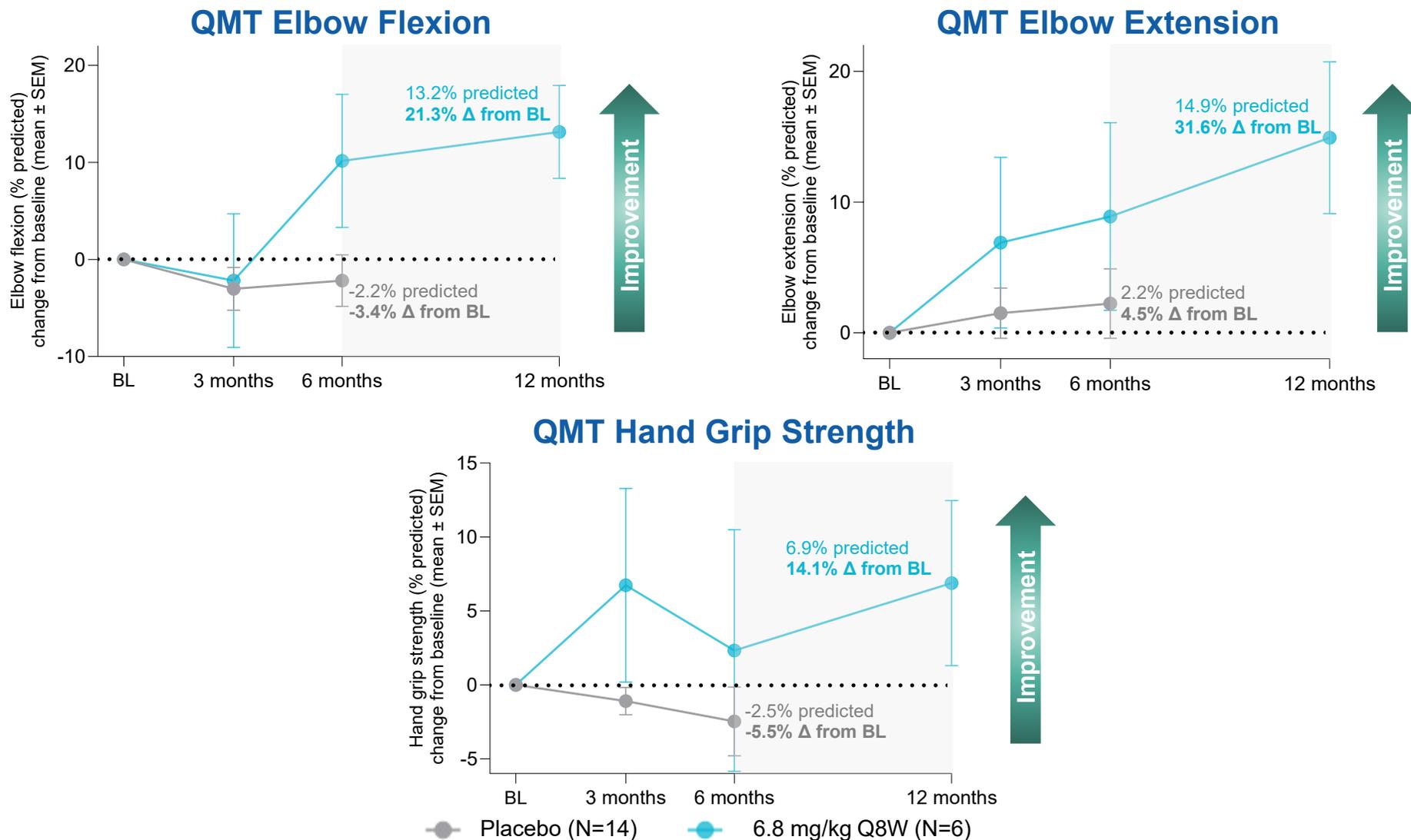


# Strength Continued to Improve with Z-Basivarsen from Month 6 to Month 12

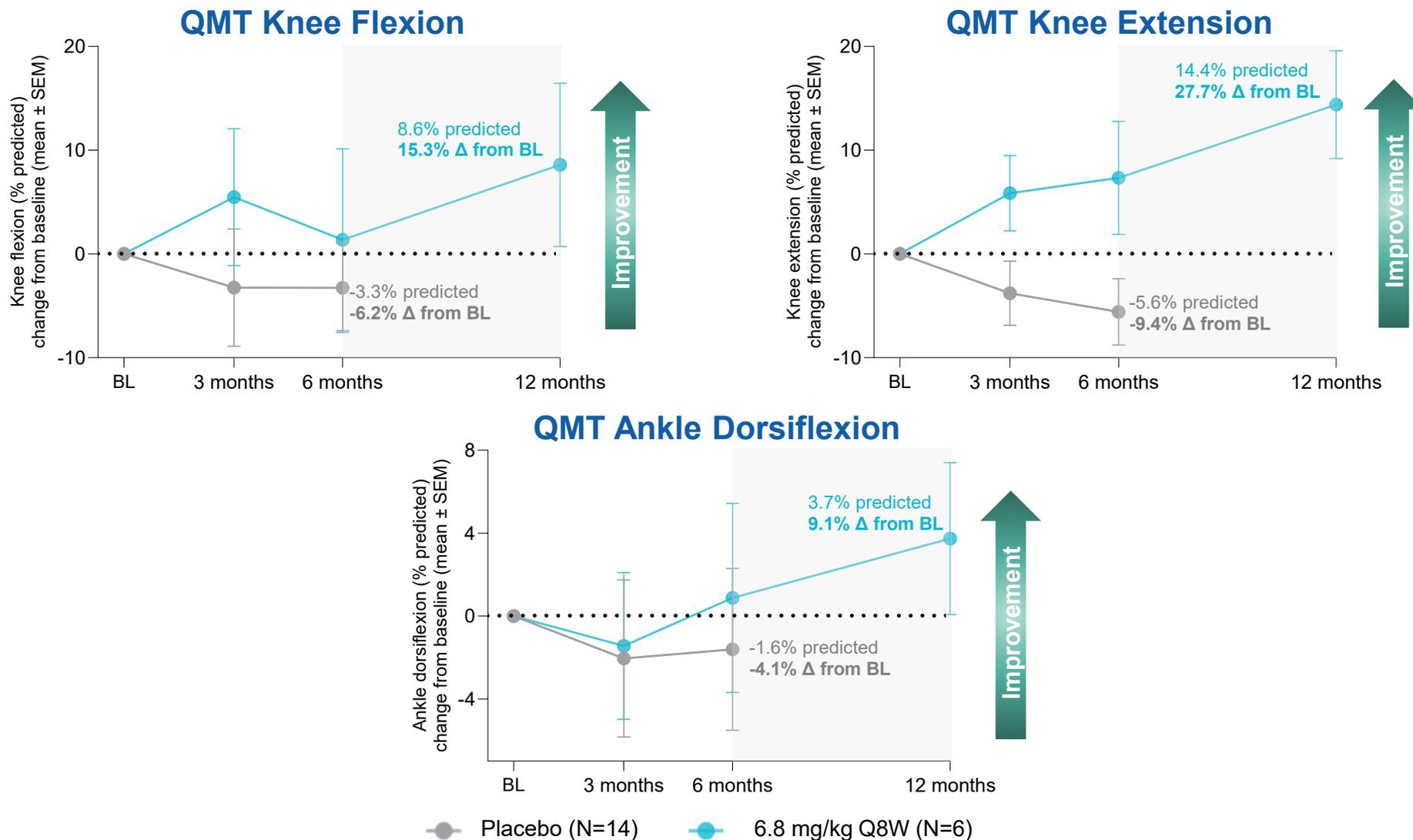
## Quantitative Muscle Testing (QMT) Total Score



# Strength Improved with Z-Basivarsen Across Both Proximal and Distal Muscles of the Upper Body

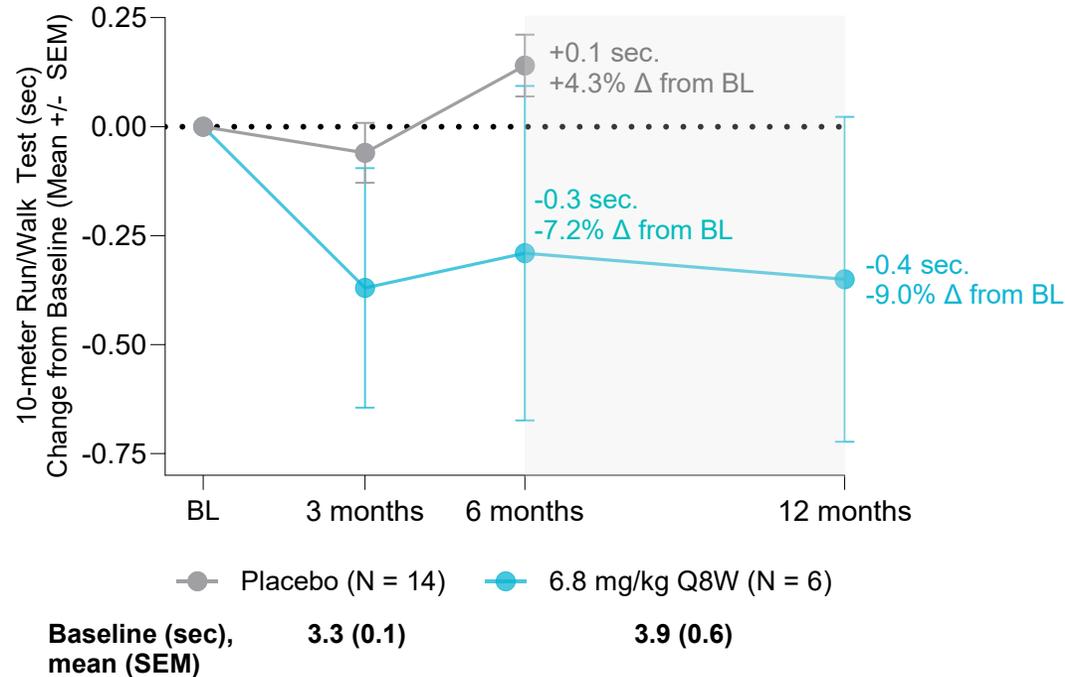


# Strength Improved with Z-Basivarsen Across Both Proximal and Distal Muscles of the Lower Body

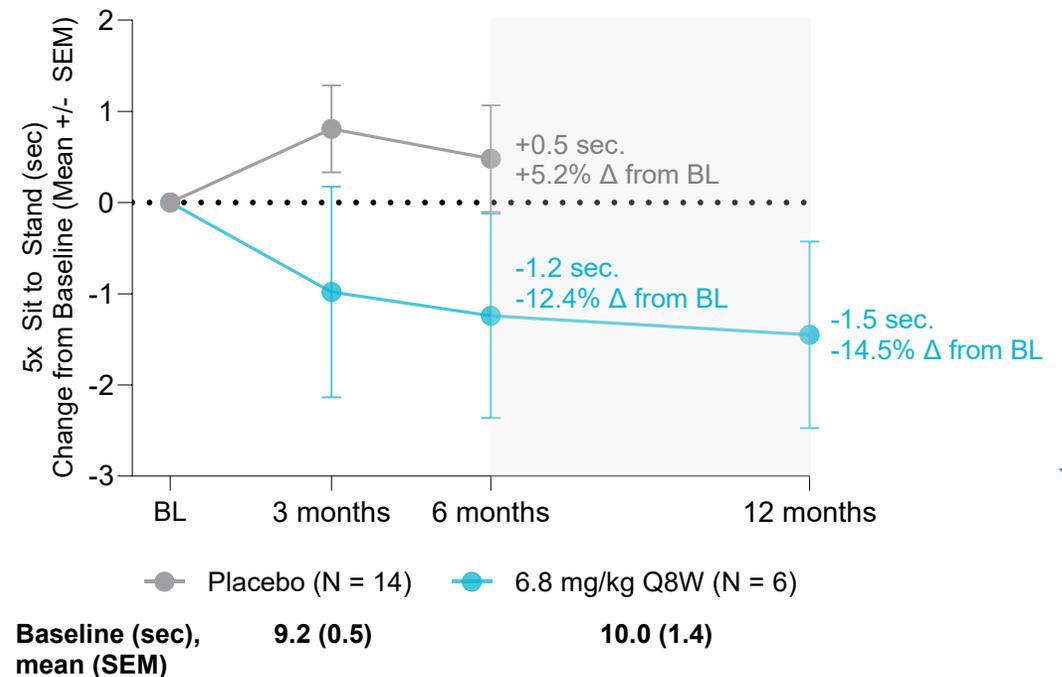


# Robust Benefit Across Multiple Timed Function Tests Sustained with Z-Basivarsen at 6 and 12 Months

## 10-Meter Walk/Run Test

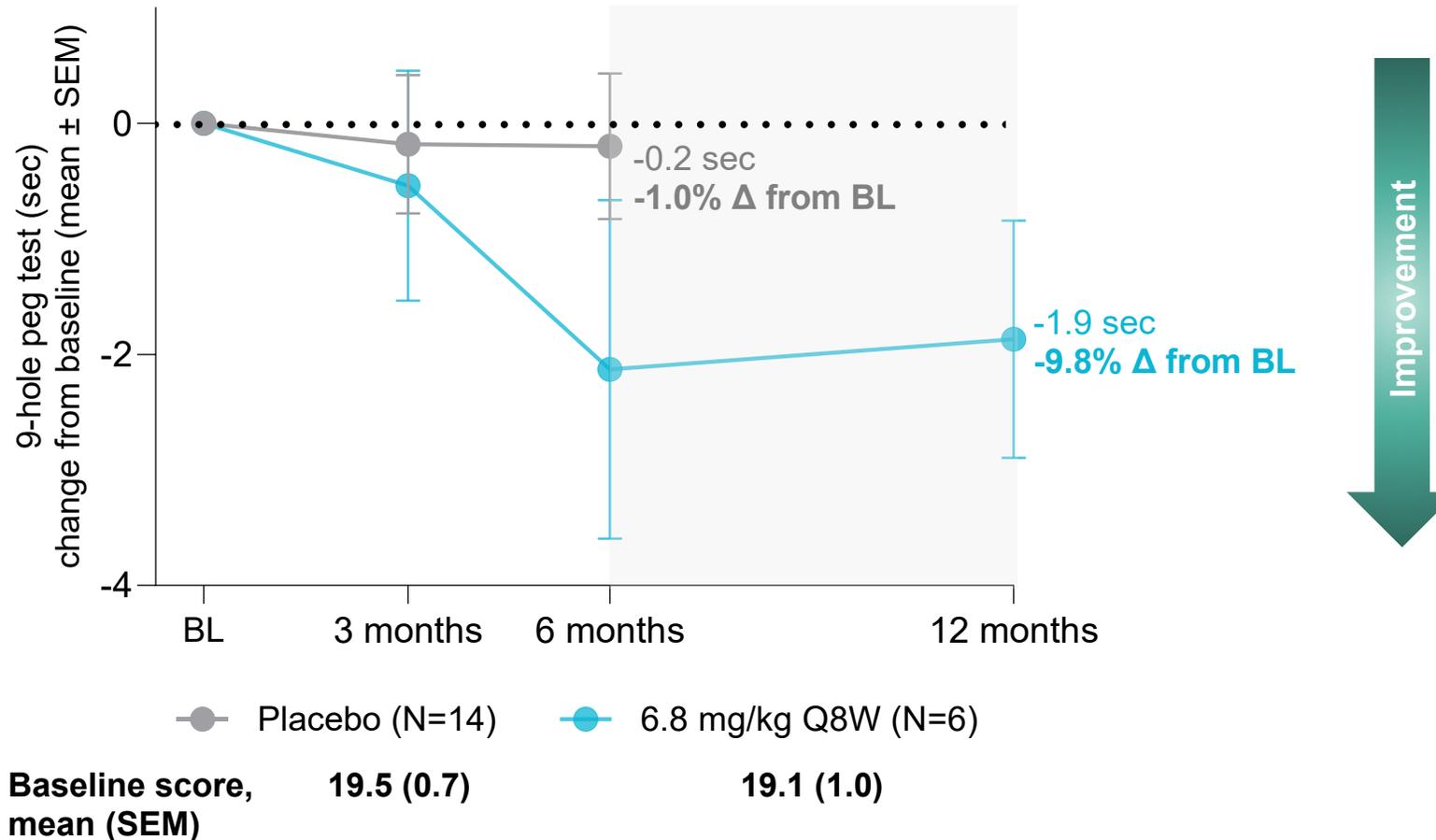


## 5 Times Sit to Stand Test



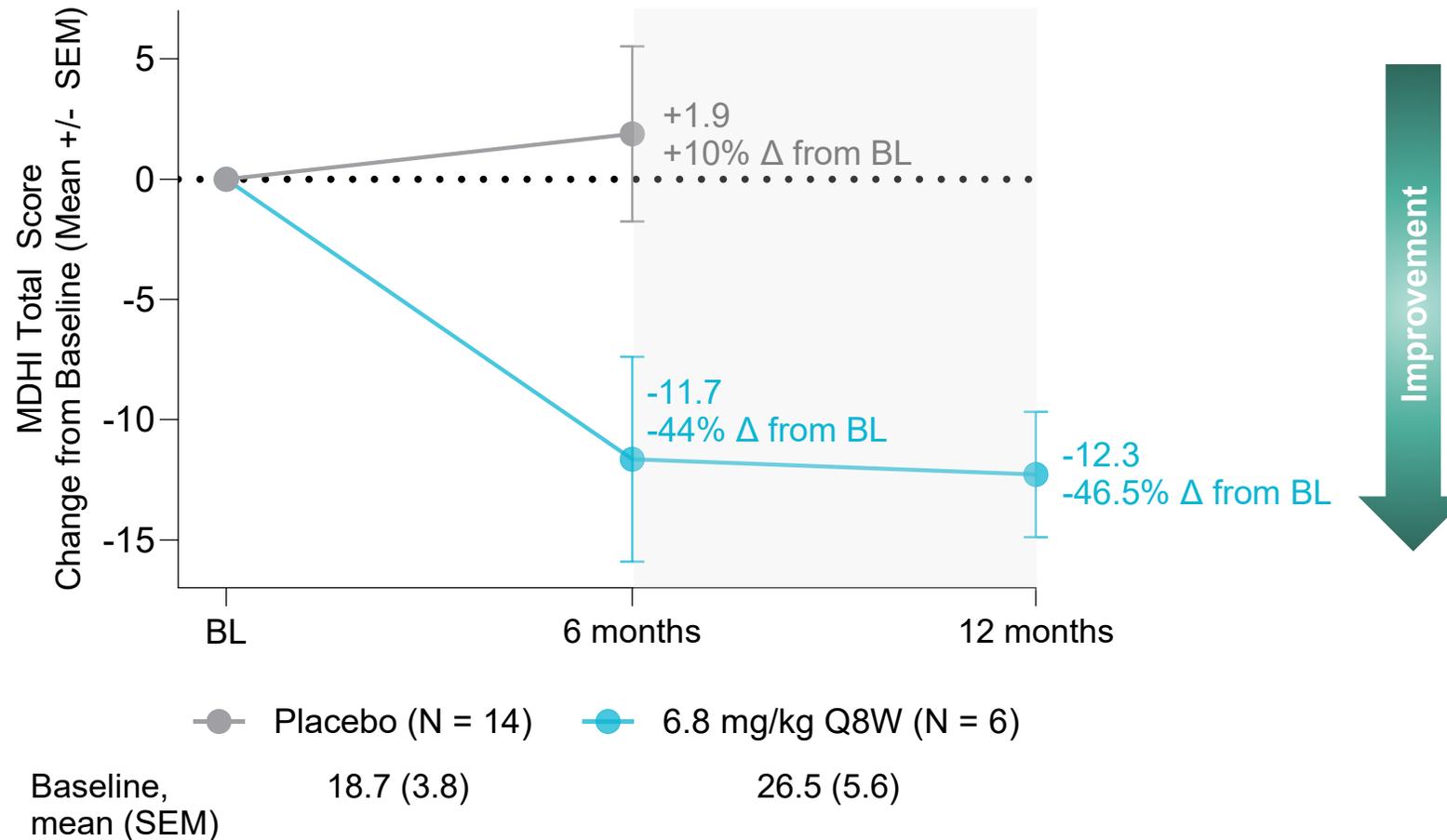
# Meaningful Improvement with Z-Basivarsen in 9-Hole Peg Test, a Measure of Upper Limb Function

## 9-Hole Peg Test

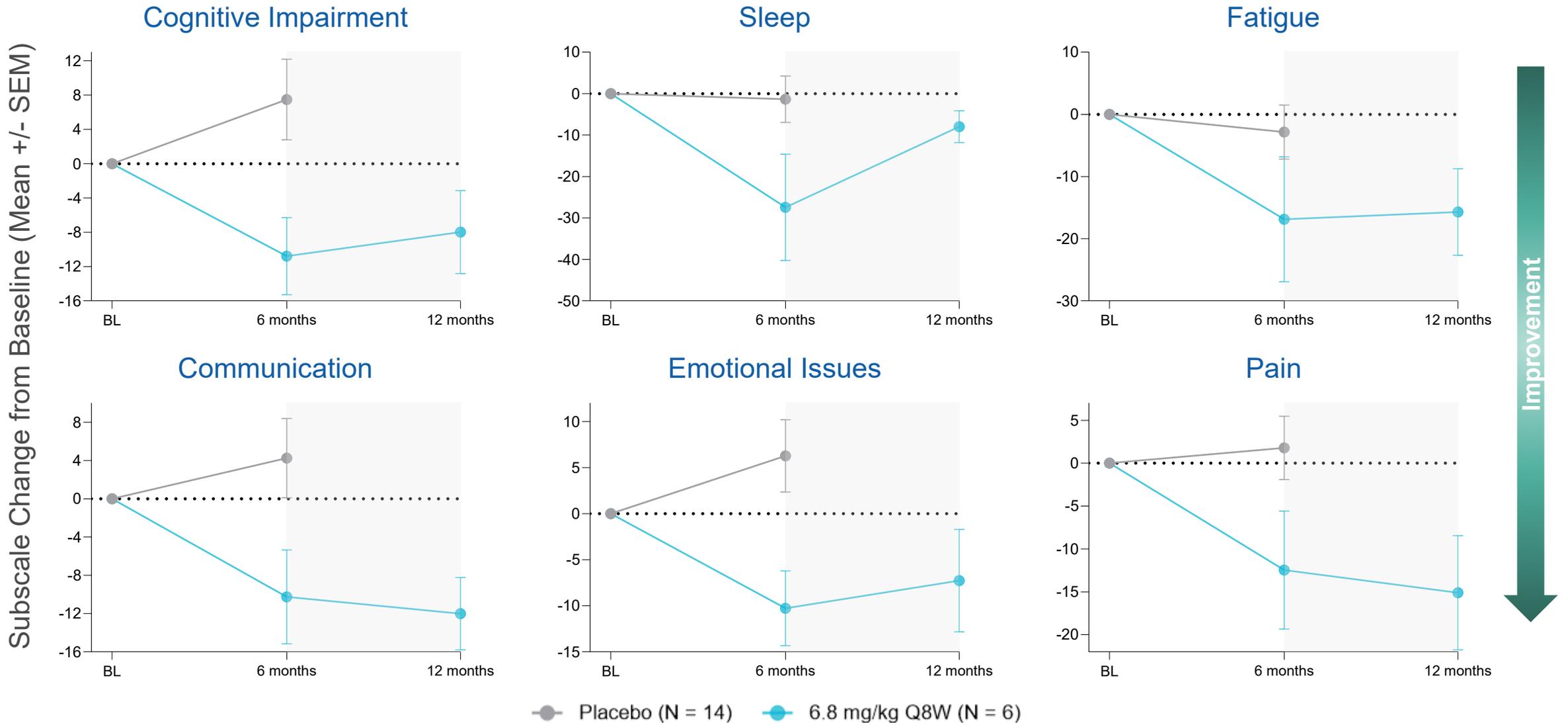


# Deep Improvement with Z-Basivarsen in Patient Reported Outcome Sustained at 6 and 12 Months

## Myotonic Dystrophy Health Index (MDHI) Total Score

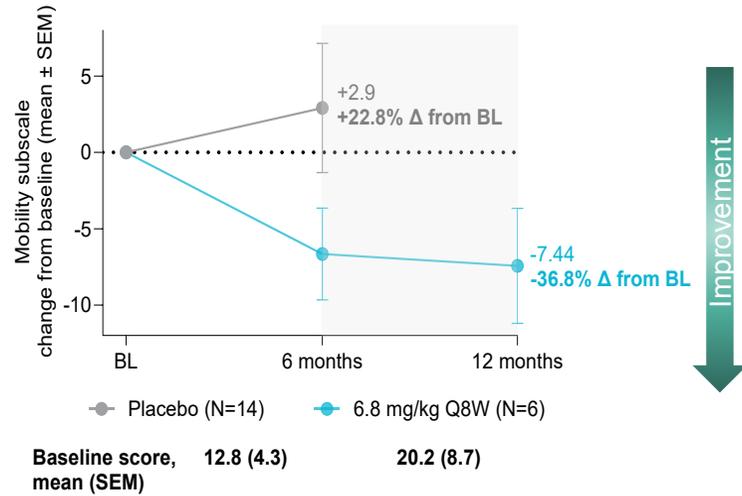


# Sustained Improvement in CNS-related MDHI Subscales Over Time

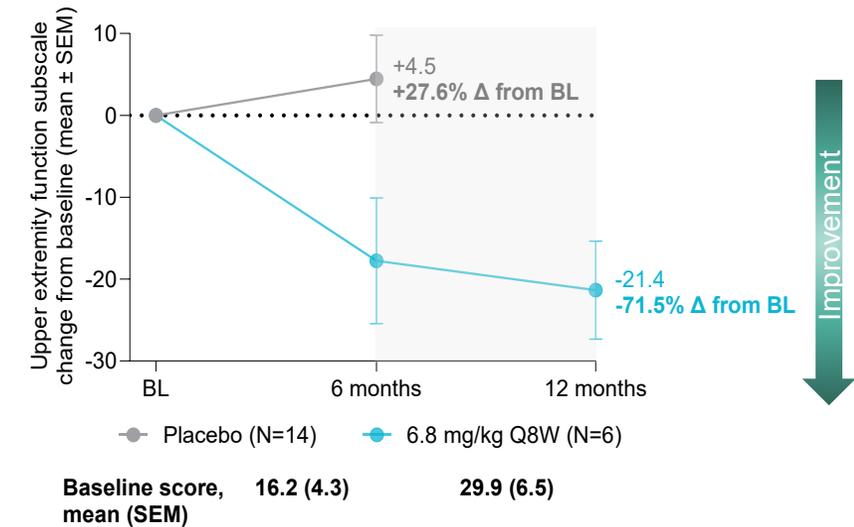


# Other MDHI Subscales Show Clinical Meaningfulness of Improvements with Z-Basivarsen in Strength and Function

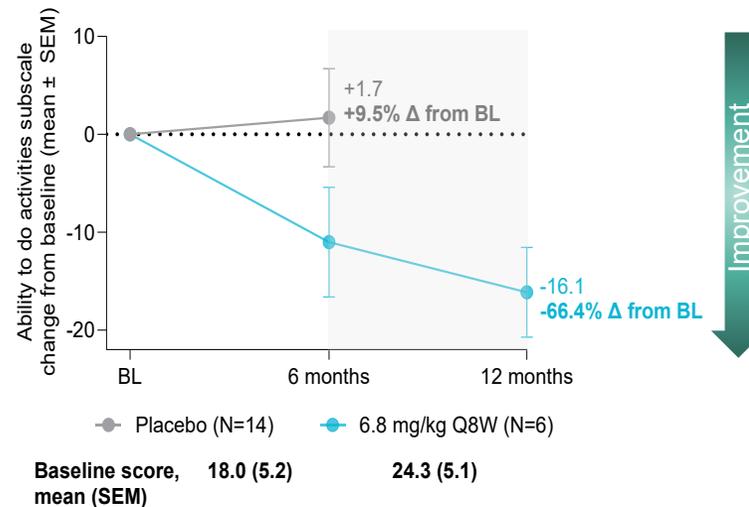
### MDHI Mobility Subscale



### MDHI Upper Extremity Function Subscale

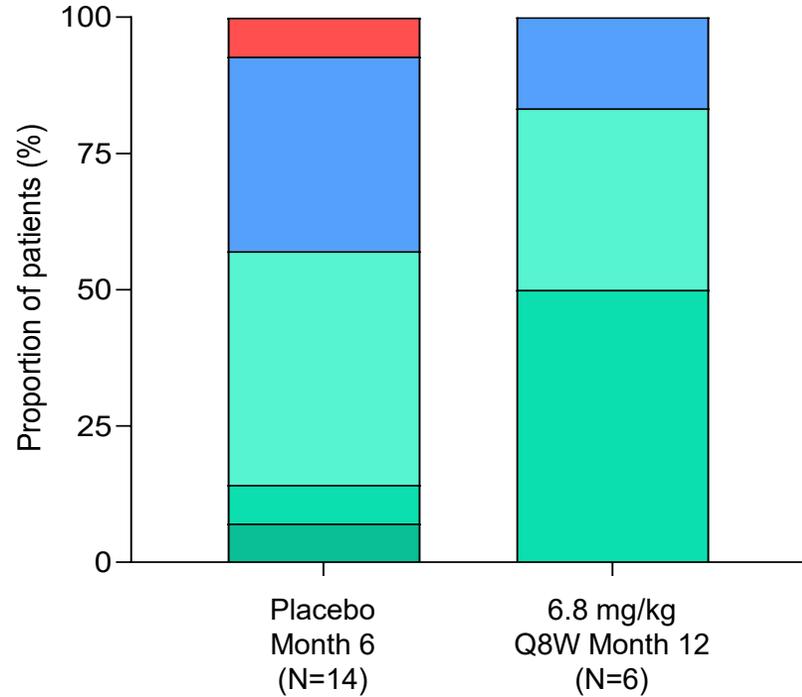


### MDHI Ability to Do Activities Subscale

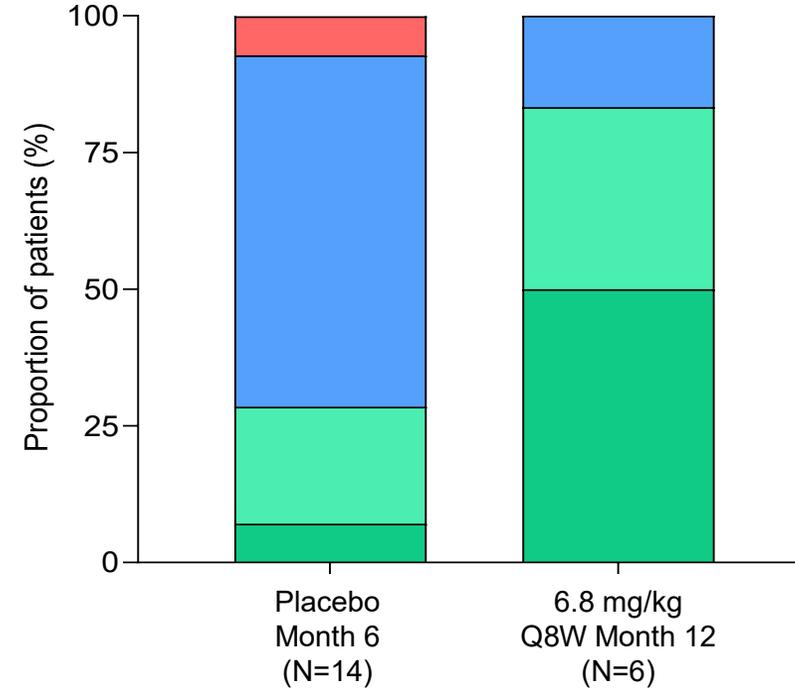


# Questionnaires Showed Improvements in Patient and Clinician Impressions of Global Functioning From Baseline with Z-Basivarsen

Patient Global Impression of Change

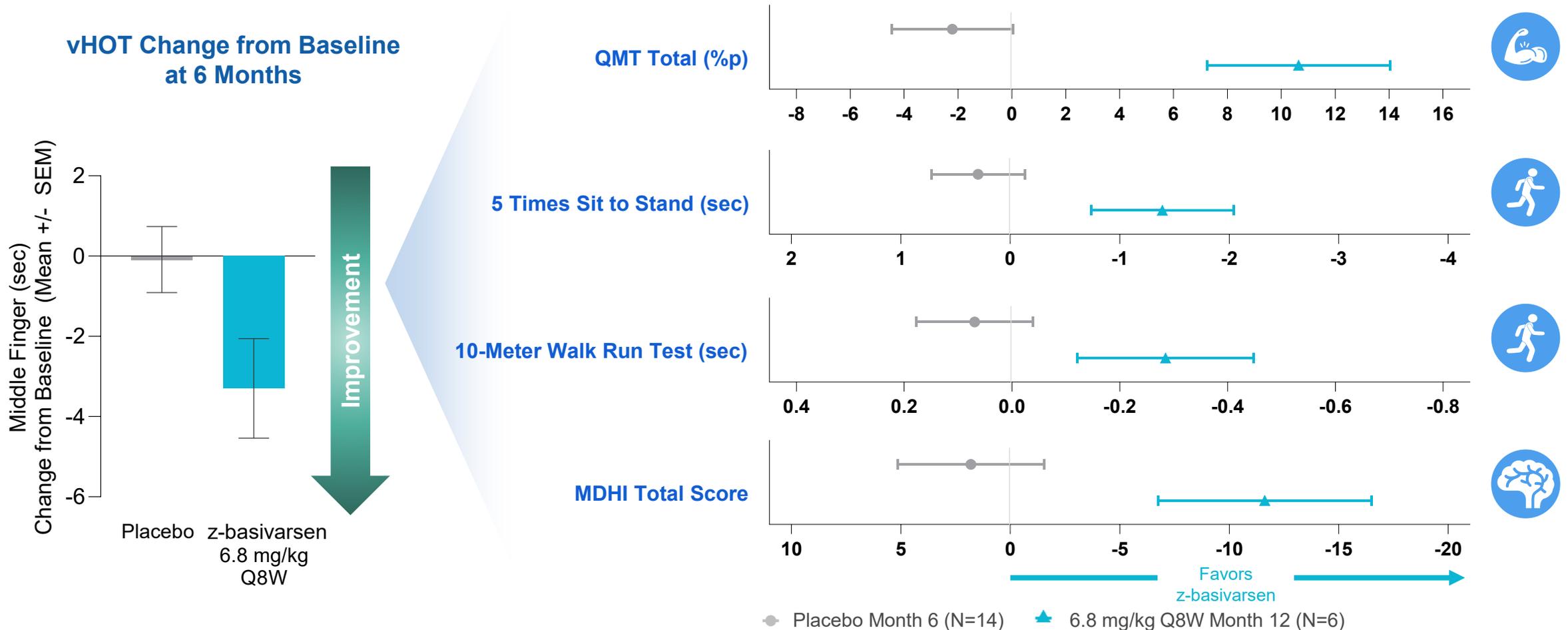


Clinician Global Impression of Change



■ Very much improved   
 ■ Much improved   
 ■ Minimally improved   
 ■ No change   
 ■ Minimally worse   
 ■ Much worse   
 ■ Very much worse

# Data Support vHOT Improvement as an Early Indicator of Clinical Benefit with Z-Basivarsen



**With z-basivarsen, all participants at highest 3 doses who improved in 6M vHOT improved in 12M strength, 5xSTS, and/or 10MWR**

Notes: Mixed model for repeated measures (MMRM): fixed effects: dose, visit, baseline, dose by visit interaction, baseline by visit interaction. Data: all dose groups except recovery group; excluding placebo data after 6 months; Data presented are least squares (LS) mean change from baseline ± SEM (standard error of the mean); 6 months = 169 days, 12 months = 337 days; vHOT = video hand opening time; QMT = quantitative muscle testing; 10MWR = 10-meter walk/run test; 5xSTS = 5 times sit to stand test; MDHI = Myotonic Dystrophy Health Index; %p = percent predicted.

# 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>

# Comprehensive Development Program for Z-Basivarsen in DM1



## Demonstrated Proof of Concept in MAD and LTE

- ✓ Selected registrational dose of 6.8 mg/kg Q8W
- ✓ Data support vHOT improvement as early indicator of clinical benefit with z-basivarsen
- ✓ Potential best-in-class profile across myotonia, strength, timed function tests, and patient reported outcomes, including CNS-related
- ✓ Favorable safety profile<sup>1</sup>; no serious related TEAEs

## Registrational Expansion Cohort to Support U.S. Accelerated Approval

- 60 participants (3:1); 6.8 mg/kg Q8W
- Primary endpoint: Change from baseline in middle finger myotonia as measured by vHOT at 6 months, compared to placebo
- Secondary endpoints include: CASI-22, QMT, 10MWR, 5xSTS, and MDHI at 6 months
- Enrollment completion planned for Q2 2026
- Data planned for Q1 2027 to support potential U.S. Accelerated Approval submission in early Q3 2027

## Planned Phase 3 Trial

- Planning to initiate confirmatory Phase 3 clinical trial in March 2026

# 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 	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**

# FSHD Program



## Population

- ~15,000 – 40,000 (US)
- ~20,000 – 50,000 (EU)



## Overview

- Aberrant expression of DUX4
- Onset in teen years or young adulthood



## Clinical Presentation

- Progressive wasting and skeletal muscle loss
- Significant physical limitations



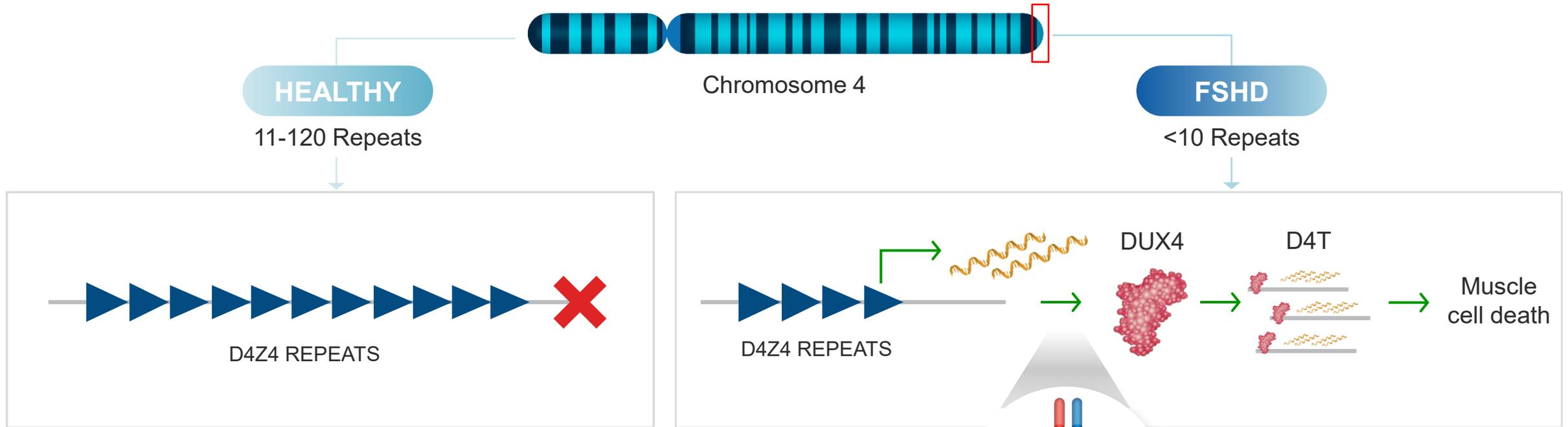
**NO  
approved  
therapies**

## OUR APPROACH

# DUX4 knockdown to achieve functional improvement

Targeting toxic *DUX4* mRNA expression to potentially **stop or reverse disease progression and enable functional improvement**

# DYNE-302 Targets the Genetic Basis of FSHD



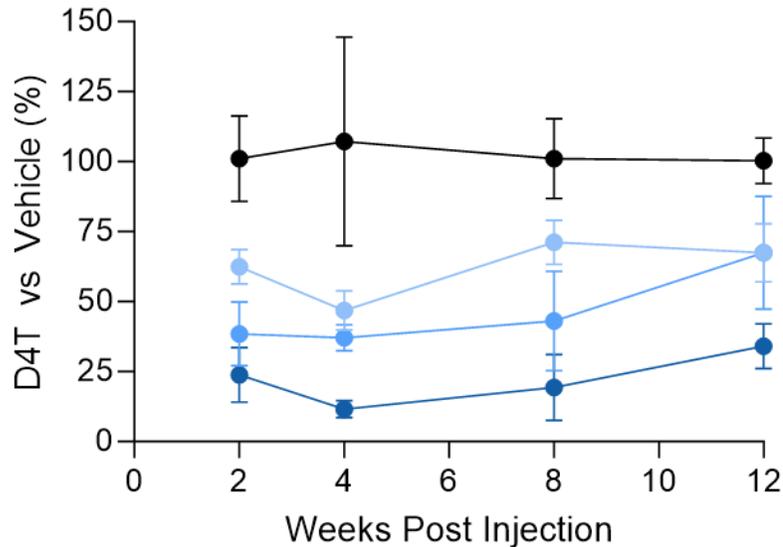
DYNE-302: a FORCE-siRNA conjugate designed to address the genetic basis of disease by **targeting toxic *DUX4* expression**

- Highly selective *DUX4* siRNA payload with favorable *in vitro* off-target and *in vitro* tolerability profile
- Extended duration of action intended to overcome sporadic *DUX4* activation

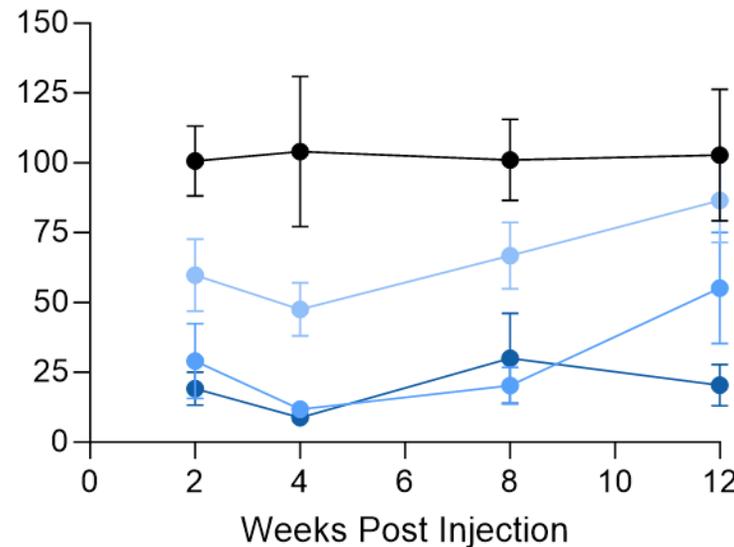
# DYNE-302 Achieved Robust, Durable, and Dose-Dependent D4T KD in Skeletal Muscle of hTfR1/iFLEXD FSHD Mice



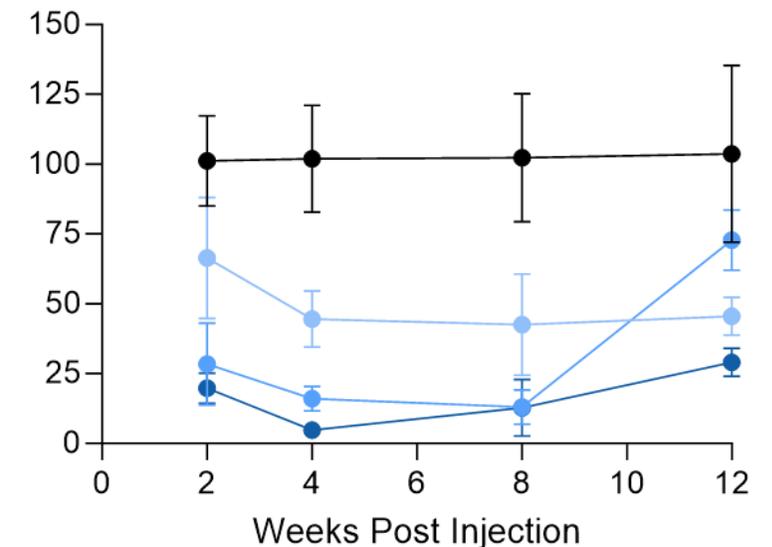
### Quadriceps



### Gastrocnemius



### Tibialis anterior



Vehicle
  DYNE-302 1 mg/kg
  DYNE-302 2 mg/kg
  DYNE-302 6 mg/kg

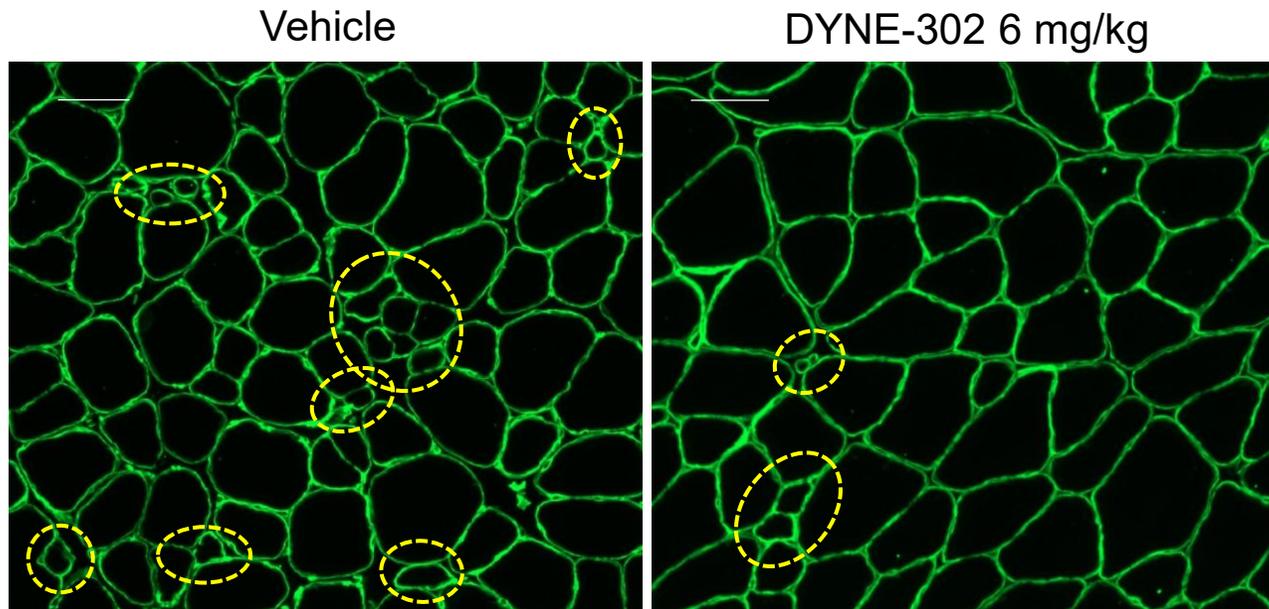
DYNE-302 demonstrates potential for infrequent dosing, out to Q12W

# Single Dose of DYNE-302 Corrected Muscle Pathology in Quadriceps of hTfR1/iFLExD FSHD Mice at 12 Weeks



DYNE-302 reduces hypotrophic myofibers

Quantification of hypotrophic myofiber reduction

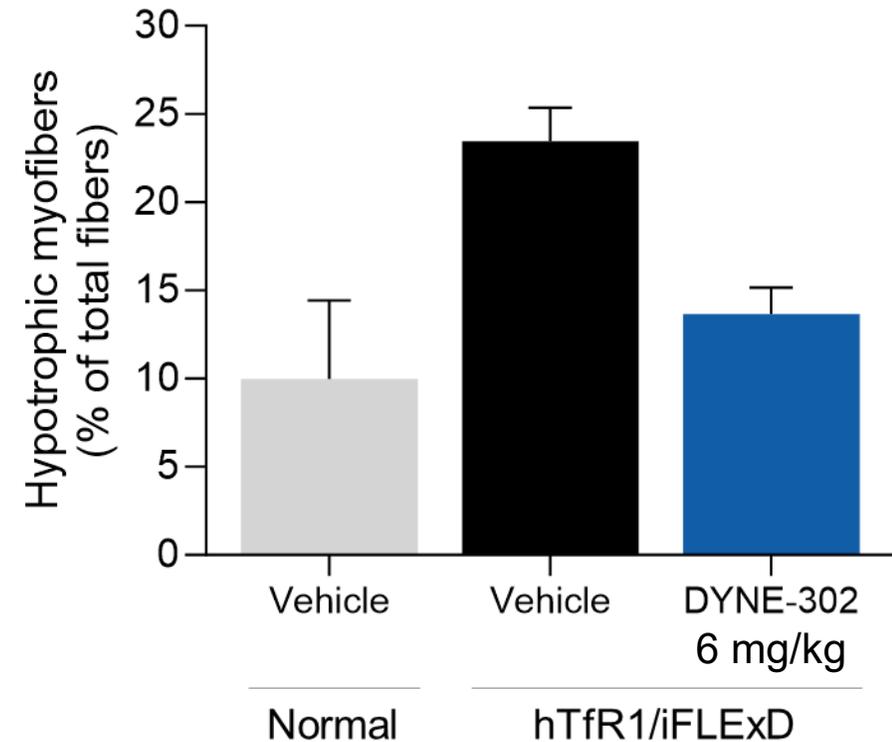


hTfR1/iFLExD

Laminin



Fiber splitting (hypotrophic myofibers)



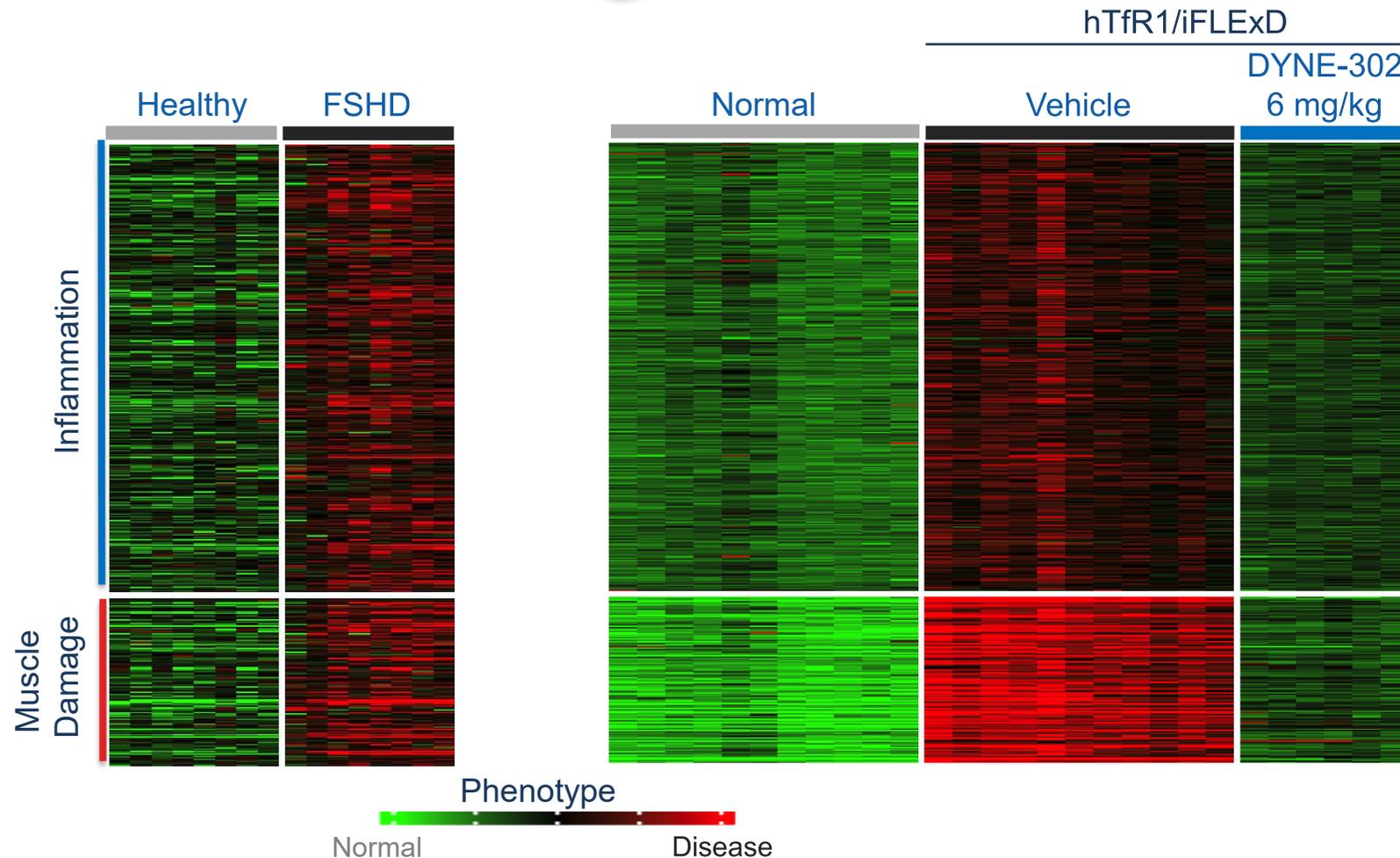
# DYNE-302 Normalized Transcriptional Profiles of Inflammation and Muscle Damage in hTfR1/iFLExD Mice



Human transcriptome  
in skeletal muscle biopsies



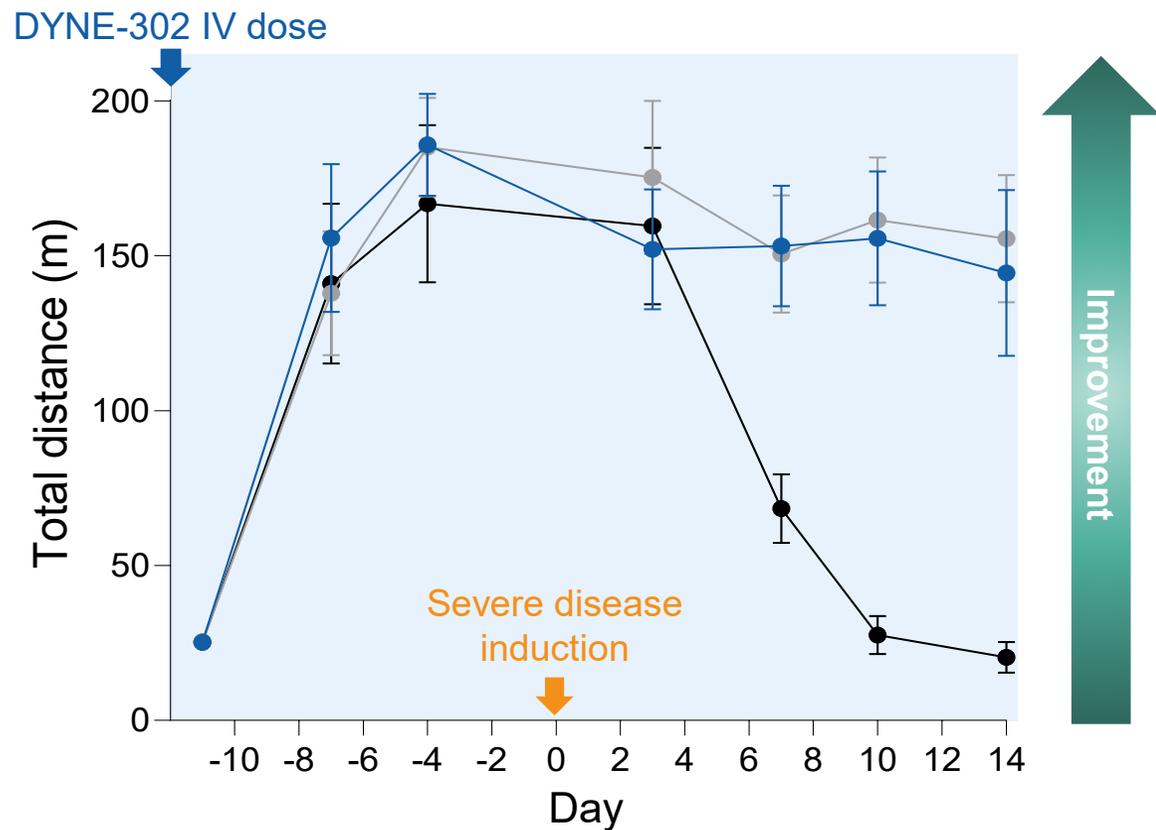
Mouse ortholog transcriptome  
in quadriceps



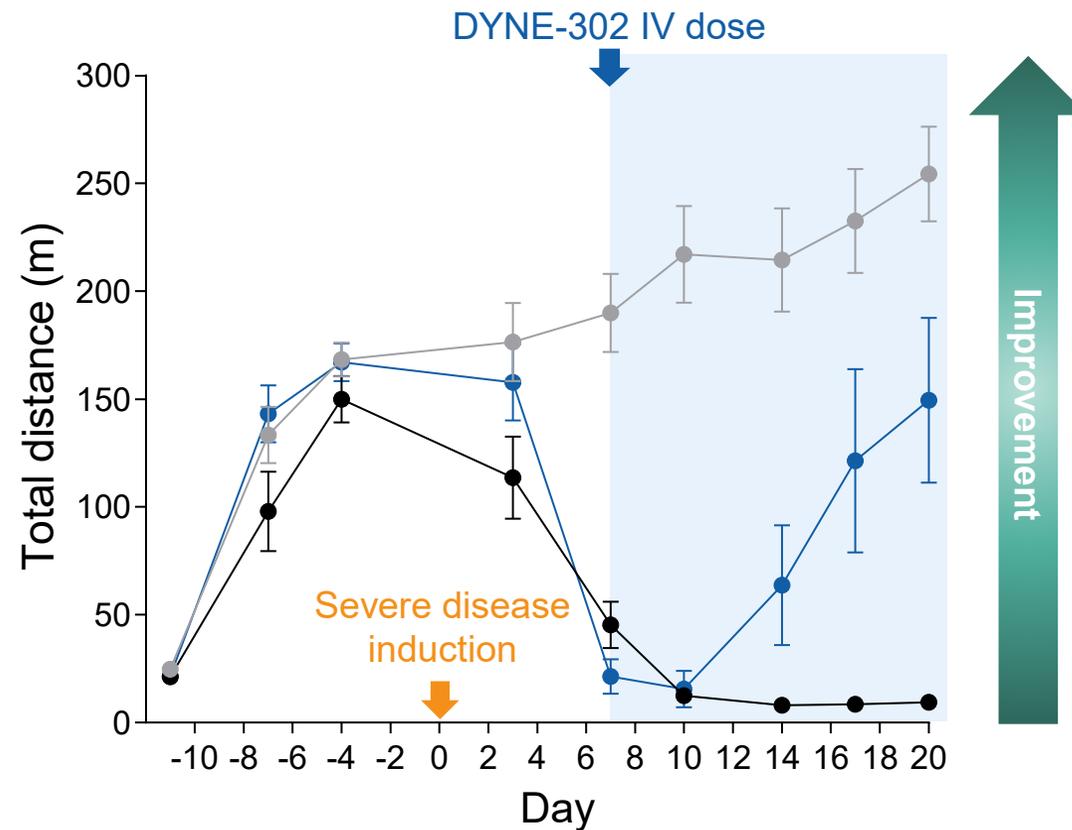
# DYNE-302 Led to Functional Improvement in hTfR1/iFLExD FSHD Mice



DYNE-302 led to functional improvement in mice by preventing onset of severe disease



DYNE-302 led to functional improvement in mice with pre-existing, severe disease



Normal mice: ● Vehicle

FSHD mice: ● Vehicle ● 6 mg/kg DYNE-302

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



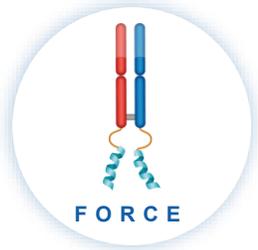
## LATE-STAGE CLINICAL PIPELINE

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



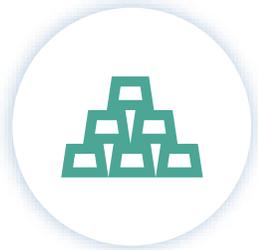
## NEAR-TERM VALUE DRIVERS

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, Pompe, and additional DMD exons



## STRONG FINANCIAL POSITION

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



# Building the World's Leading Neuromuscular Disease Company