



ACHIEVE

Clinical Update

JANUARY 10, 2025



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Program



Opening Remarks

John Cox, President & CEO



New Data from DYNE-101 ACHIEVE Trial in DM1 Update on DYNE-251 DELIVER Trial in DMD

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



Closing Remarks

John Cox, President & CEO

Aiming to Deliver Transformative Therapies for Neuromuscular Diseases



LATE-STAGE PIPELINE

Two clinical programs moving to registrational expansion cohorts for DM1 and DMD following positive proof-of-concept data



NEAR-TERM VALUE DRIVERS

Key data readouts in 2025 & 2026 potentially enabling two submissions for U.S. Accelerated Approval in 2026



DIFFERENTIATED PLATFORM

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



STRONG FINANCIAL POSITION

Cash position of ~ \$642 million (as of 12/31/24)* with expected runway into H2 2026; all assets fully owned

Myotonic Dystrophy Type 1 (DM1) Clinical Data Show Broad Functional Impact and Support Potential for Accelerated Approval

1

Initiating Registrational Expansion Cohort in ACHIEVE trial to support potential 1H2026 submission for U.S. Accelerated Approval

2

Potential best-in-class profile with meaningful improvement in myotonia, strength, timed function tests, and patient reported outcomes

3

Continued favorable safety profile¹; no serious related TEAEs

Advancing Next-Generation Exon 51-Skipping Therapeutic for Duchenne Muscular Dystrophy (DMD)

- 1** Ongoing Registrational Expansion Cohort in DYNE-251 DELIVER trial to support potential submission for U.S. Accelerated Approval early 2026, using dystrophin as surrogate endpoint based on recent FDA feedback
- 2** Unprecedented dystrophin expression and functional benefit previously demonstrated at registrational dose of 20 mg/kg Q4W
- 3** DELIVER Registrational Expansion Cohort on-track for full enrollment in Q1 2025
- 4** Continued favorable safety profile¹ since last update

Program



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Closing Remarks

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Developing Transformative Therapeutics for People Living with DM1



Overview

- Mutation in the *DMPK* gene
- Onset at any point, depending on DM1 phenotype
- Life expectancy of 45 - 60 years



Clinical Presentation

- Myotonia
- Muscle weakness
- Cardiac arrhythmia
- Pulmonary abnormalities
- CNS manifestations



Population

- >40,000 (US)
- >74,000 (Europe)



**NO
approved
therapies**

OUR APPROACH

Disease-Modifying Nuclear *DMPK* Knockdown

Targeting toxic gain of function *DMPK* RNA to potentially **stop or reverse** disease progression

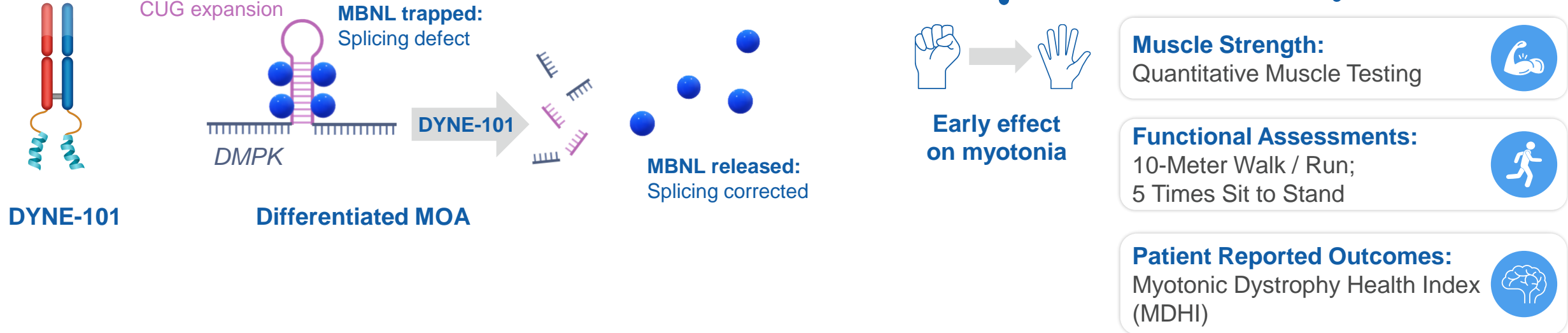
DYNE-101 Addressing the Central Pathobiology of DM1 to Enable Broad Functional Improvement¹

Robust and widespread delivery

DMPK degradation in the nucleus

MBNL release and splicing correction

Correction of myotonia and broad functional improvement



DYNE-101

Differentiated MOA

DM1 is a Heterogeneous Disease with Widespread Muscle and CNS Manifestations

CNS¹⁻⁴

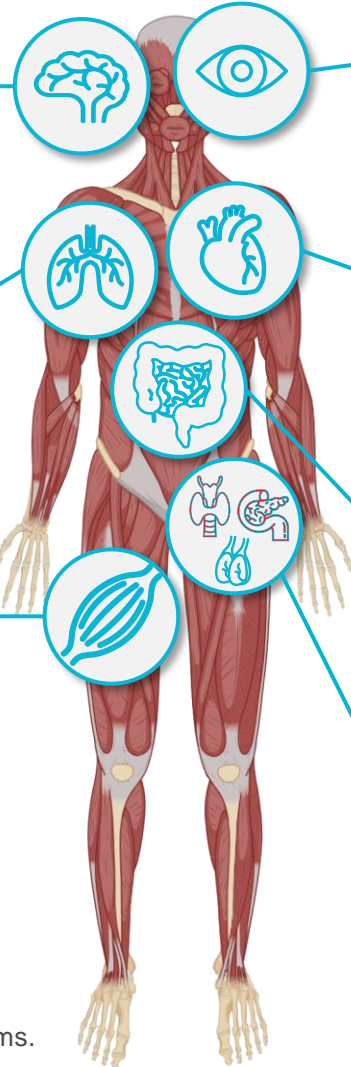
- Fatigue
- Excessive daytime sleepiness
- Difficulty concentrating
- Behavioral/personality changes

Skeletal muscle (respiratory)¹⁻⁴

- Restrictive ventilatory pattern
- Shortness of breath

Skeletal muscle¹⁻⁴

- Muscle weakness
- Myotonia
- Balance issues
- Muscle pain
- Atrophy



Ocular¹⁻⁴

- Cataracts
- Ptosis

Cardiac¹⁻⁴

- Conduction disturbances
- Arrhythmia
- Cardiomyopathy
- Sudden death

Smooth muscle¹⁻⁴

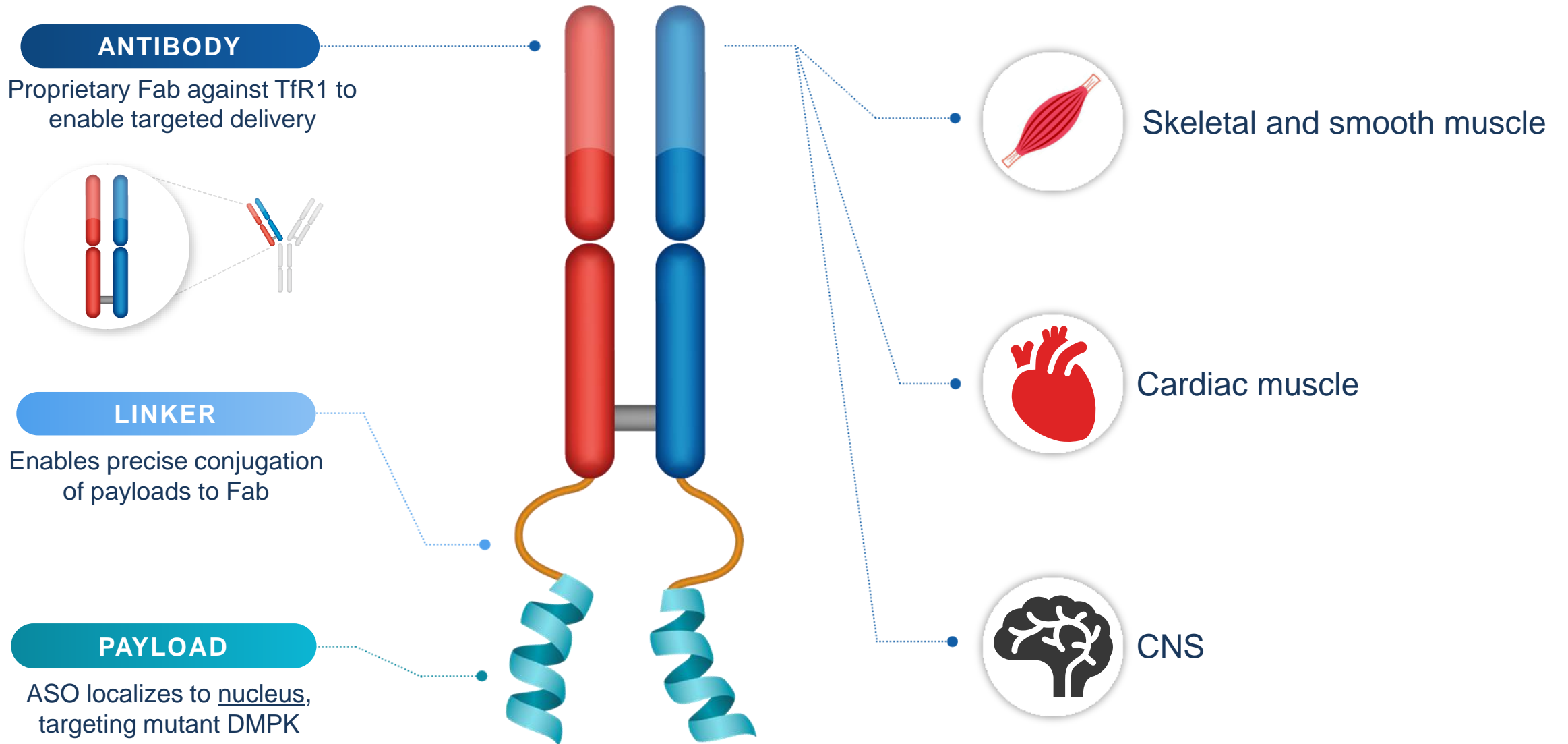
- Dysphagia
- Constipation
- Heartburn
- Regurgitation

Endocrine¹⁻⁴

- Thyroid disorders
- Diabetes
- Male hypogonadism
- Vitamin D deficiency


Slide does not represent an exhaustive list of symptoms.

DYNE-101 Leverages FORCE™ Platform for Targeted Delivery



Multiple Ascending Dose (MAD) Portion of ACHIEVE is Complete



Population	Primary Endpoints	Additional Endpoints	Stages of ACHIEVE
<ul style="list-style-type: none">• Adult patients living with DM1• Ages 18 to 49 years	<ul style="list-style-type: none">• Safety and tolerability	<ul style="list-style-type: none">• Pharmacokinetics• Change from baseline of:<ul style="list-style-type: none">– Splicing– <i>DMPK</i> RNA expression– Multiple assessments of muscle strength and function– Patient-reported outcomes, including DM1-ACTIV^c and MDHI	<ul style="list-style-type: none">✓ Multiple Ascending Dose (MAD): 24 weeks• Open-Label Extension (OLE): 24 weeks• Long-Term Extension (LTE): 96 weeks <p style="text-align: center;"></p> <p style="text-align: center;">Registrational Expansion Cohort</p>

DYNE-101 Addresses Central Pathobiology: Differentiated Pharmacology with Potential to Lead to Broad Functional Benefit

Delivery and exposure

DMPK knockdown

Splicing correction

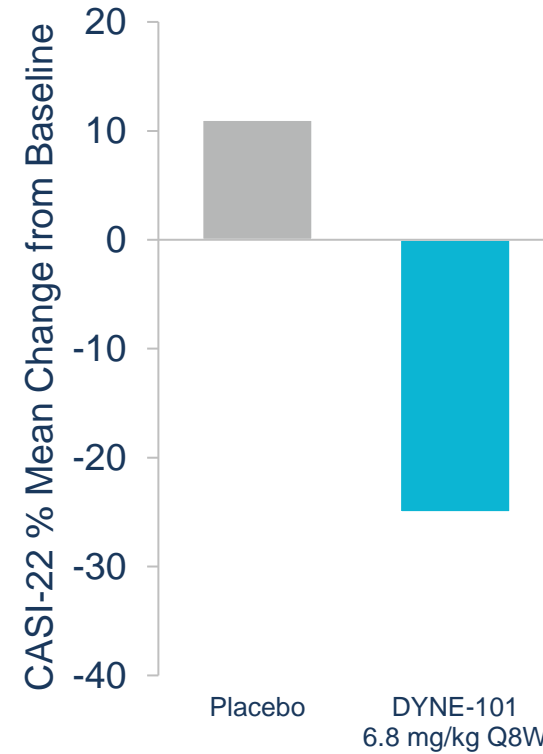
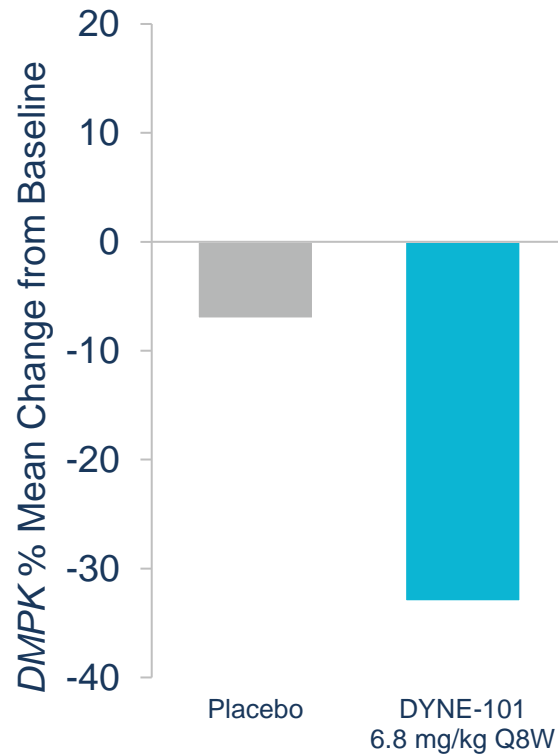
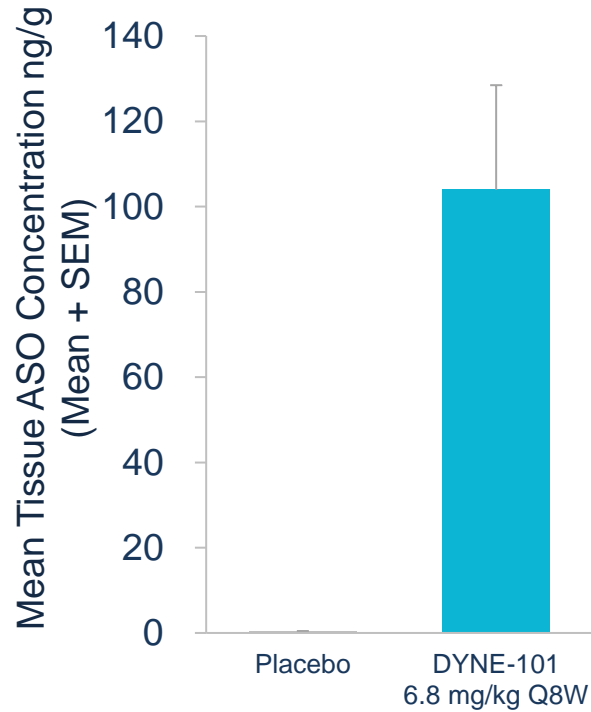
Broad functional benefit

Muscle delivery at 3 mo

Target engagement at 3 mo

Biomarker response at 3 mo

Benefit at 6 mo



DYNE-101: Favorable Safety Profile with No Serious Related TEAEs

Summary of Treatment Emergent Adverse Events (TEAEs)¹

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%)	9 (56%)	2 (25%)	3 (38%)	6 (75%)	29 (52%)
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 Intensity¹

- 6 serious TEAEs unrelated to study drug
 - Atrioventricular block first degree (1)²
 - Pneumonia (2 events in same participant)
 - Pulmonary embolism (1)³
 - Hyponatremia (1)
 - Influenza (1)
- Most common TEAEs (≥20% participant incidence)⁴
 - Nasopharyngitis (38%)
 - Procedural pain (30%)
 - Influenza (27%)
 - Infusion-related reaction (25%)
 - Diarrhea; headache (each 21%)

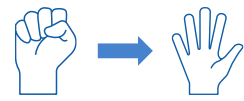
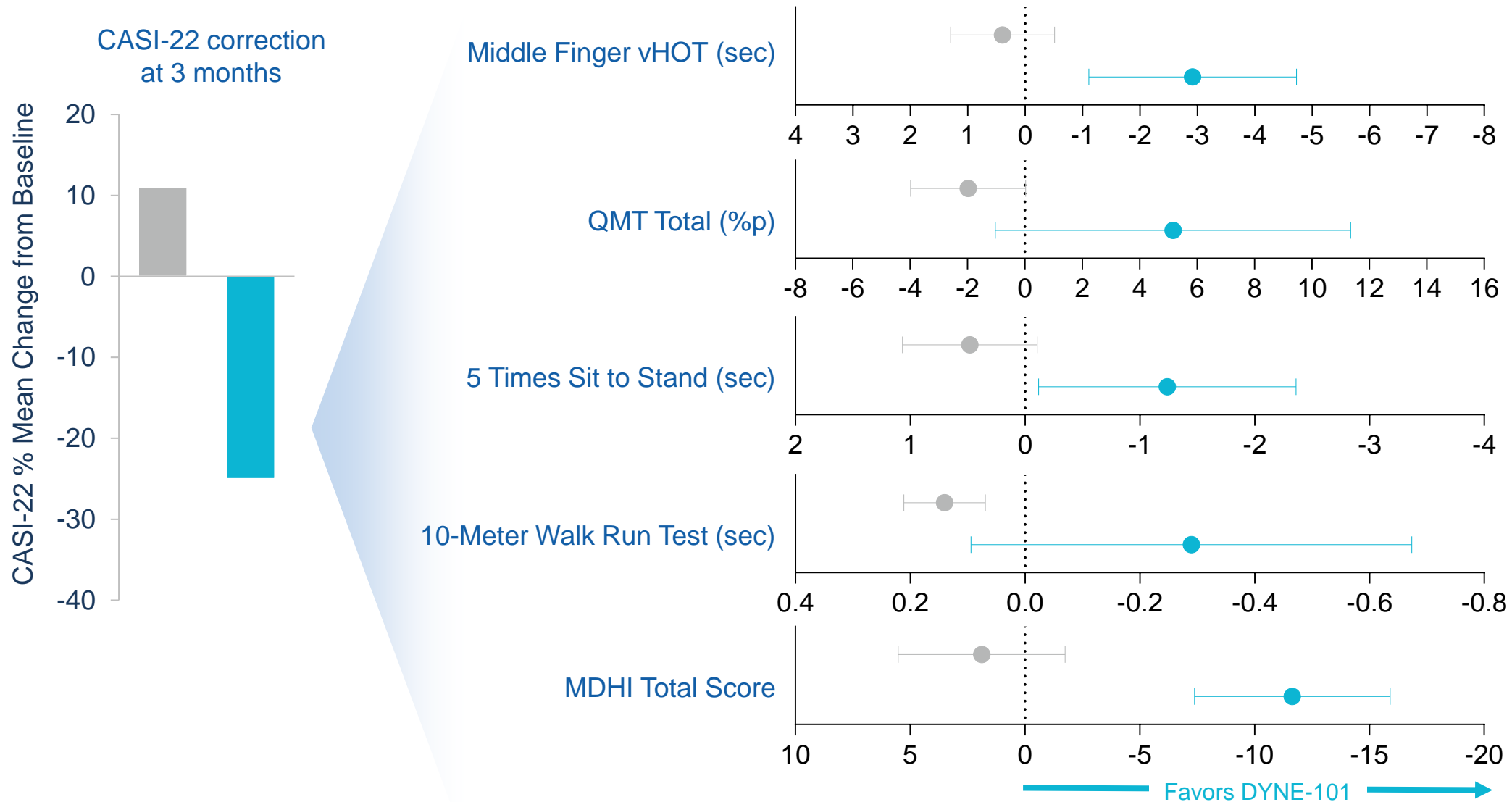
Additional Safety Data

- 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

~855 Doses Administered to Date Representing Over 72 Patient-Years of Follow-Up¹

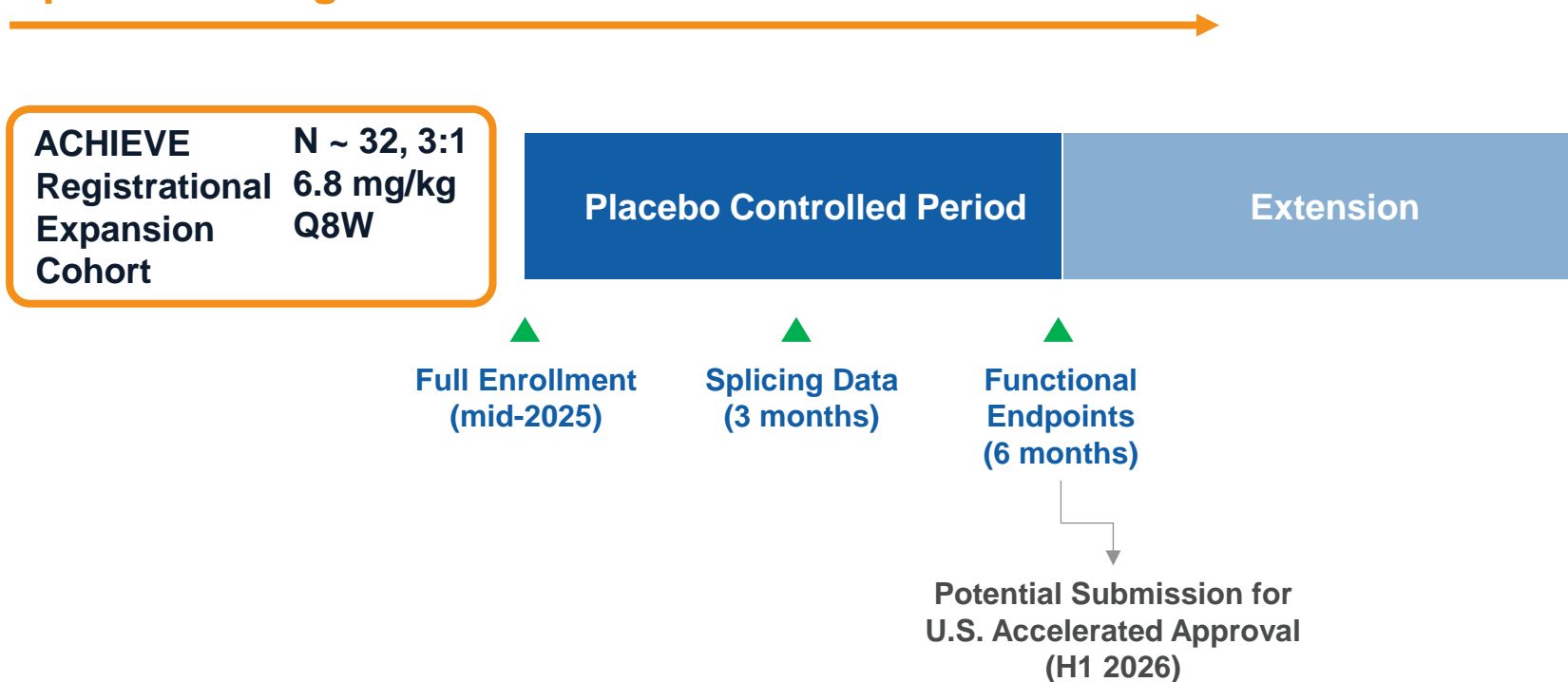
1. Data as of December 6, 2024; 2. Transient worsening of atrioventricular (AV) block in a participant with ongoing medical history of first-degree AV block; 3. Attributed to risk factors for pulmonary embolism; 4. All cohorts combined; preferred terms are reported.

Broad Improvement Demonstrated at 6 Months with Planned Registrational Dose of 6.8 mg/kg Q8W



Registrational Expansion Cohort Timeline Enables Potential Submission for U.S. Accelerated Approval in H1 2026

Accelerated Approval Path Enables Speed to Filing with Functional Benefit



Potential Profile

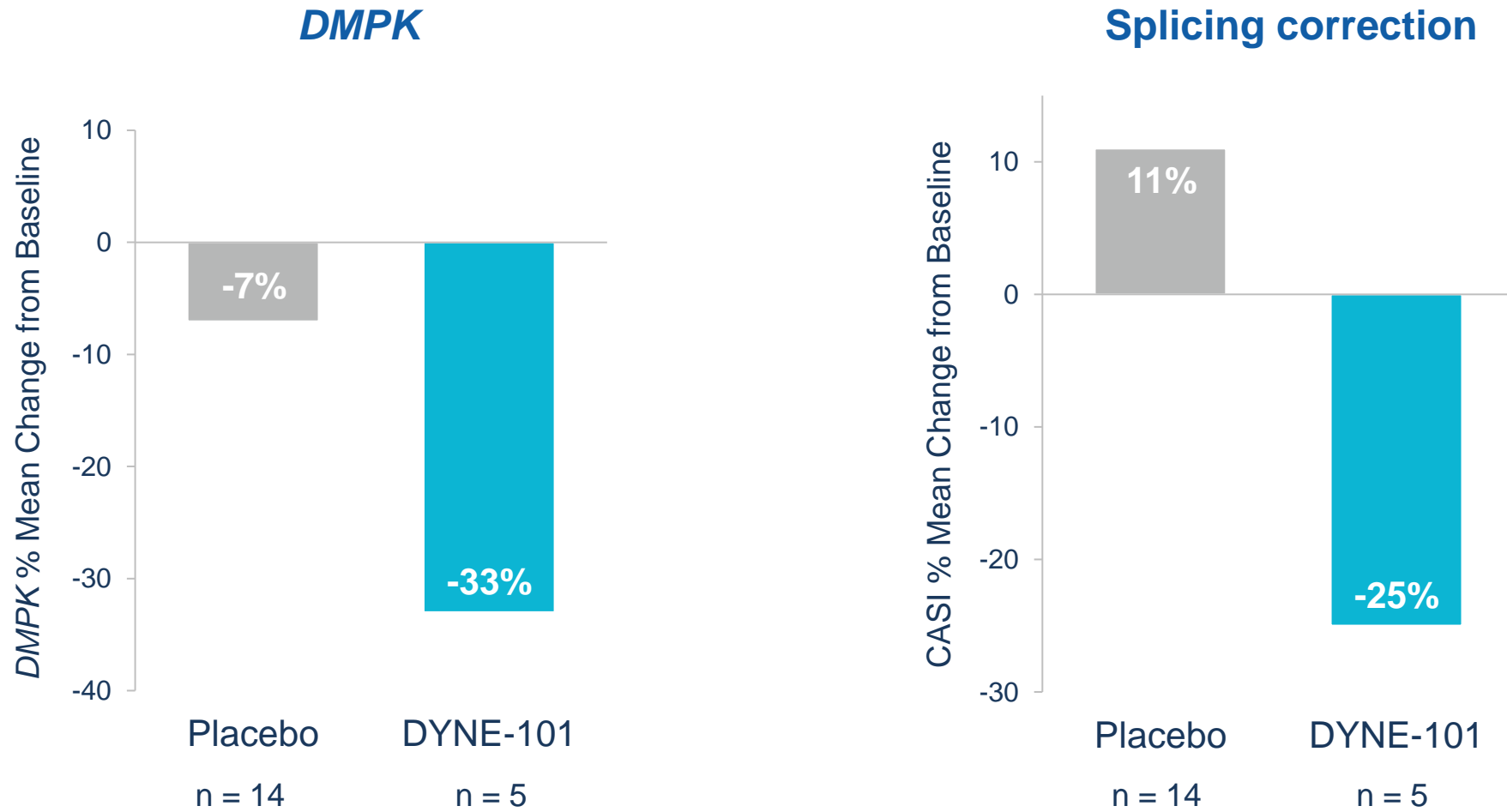
Planned Primary Endpoint (3 months)

- Change from baseline in CASI

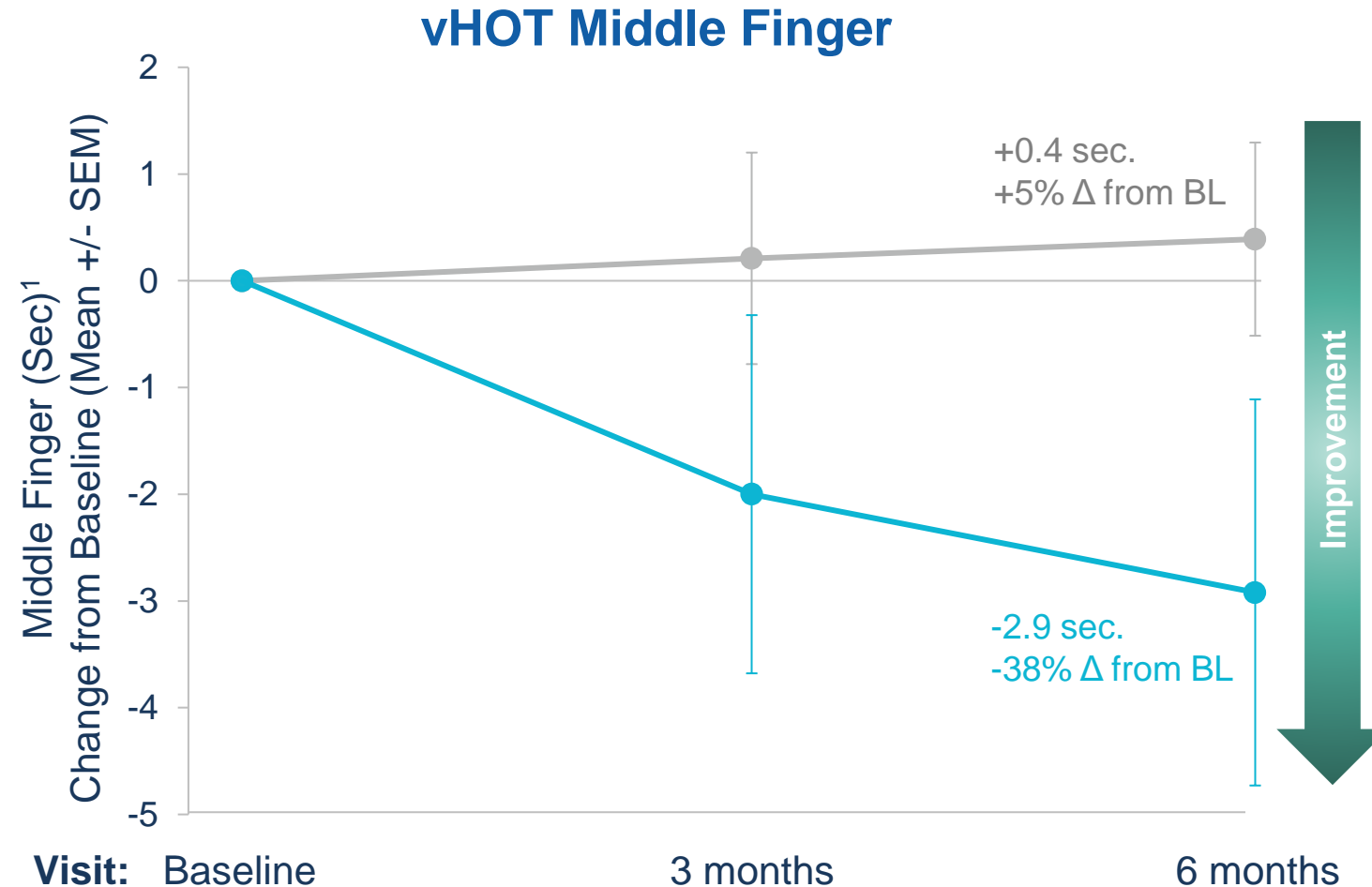
Planned Secondary Endpoints (6 months)

- Change from baseline in
 - vHOT (middle finger)
 - 10MWR
 - QMT
 - 5xSTS
 - MDHI Total Score

DYNE-101 at 6.8 mg/kg Q8W Improved Foundational Pathobiology of DM1 at 3 Months



Early and Robust Improvement in Functional Myotonia

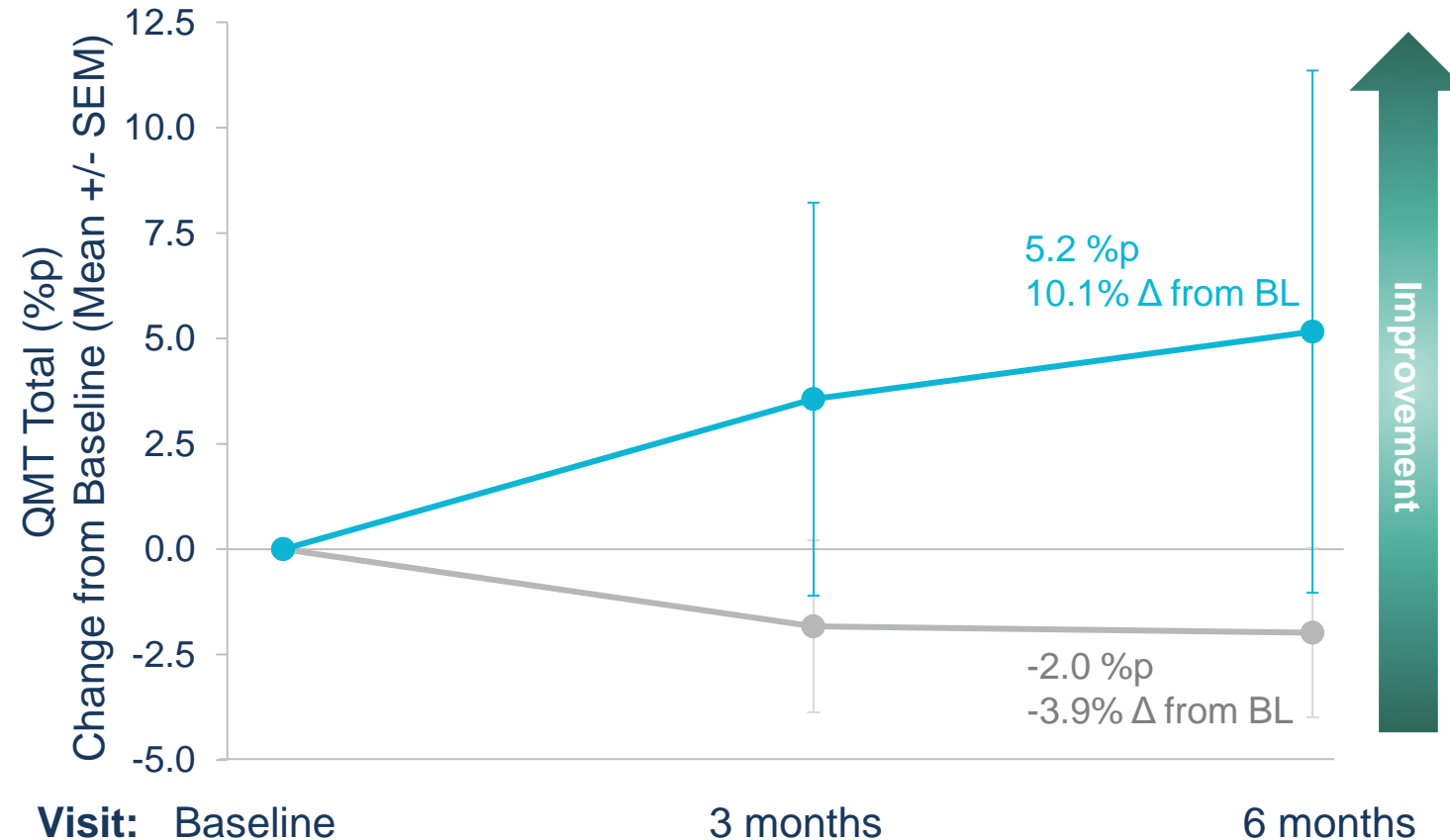


Cohort: ● Placebo (N = 14) ● DYNE-101 6.8 mg/kg Q8W (N = 6)

Baseline (sec), mean (SEM): 7.5 (0.8) 7.8 (1.5)

Improved Muscle Strength at 6 Months

Quantitative Muscle Testing (QMT) Total Score



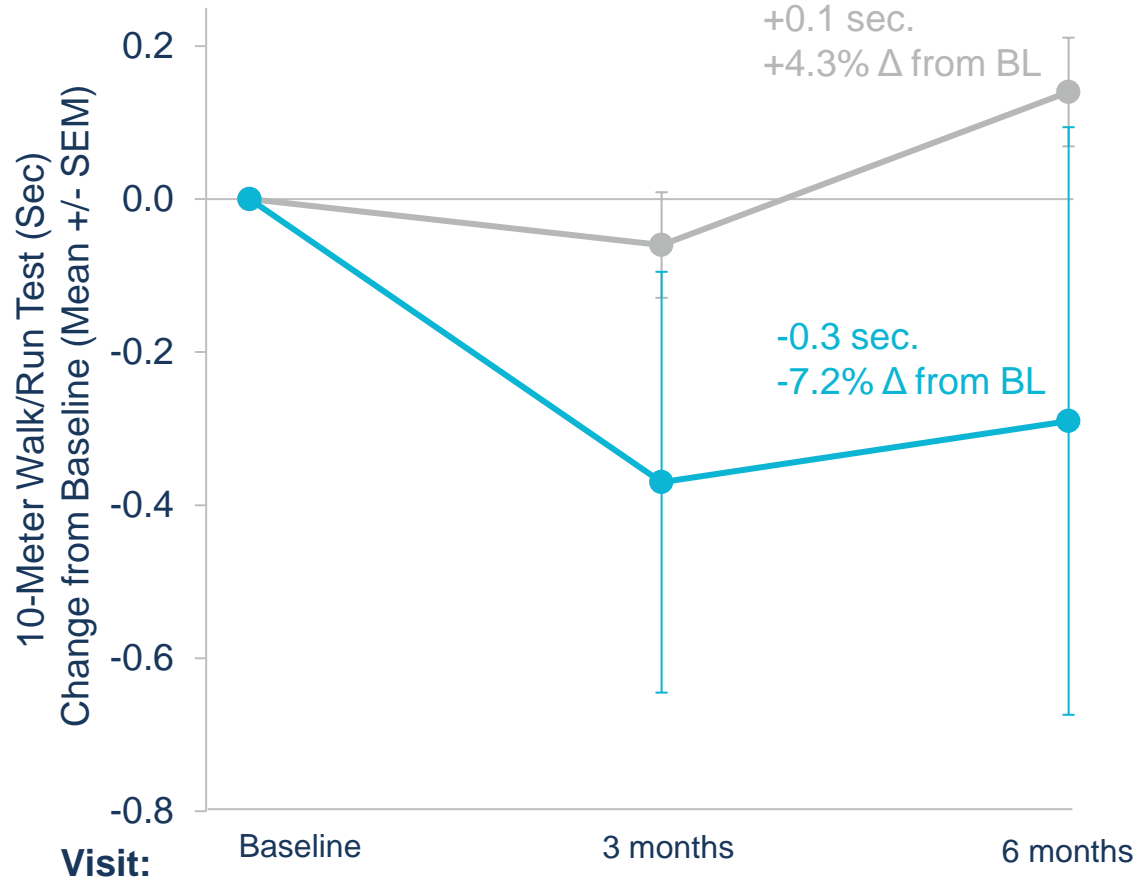
Cohort: ● Placebo (N = 14) ● DYNE-101 6.8 mg/kg Q8W (N = 6)

Baseline, mean (SEM): 51.5 (3.8)

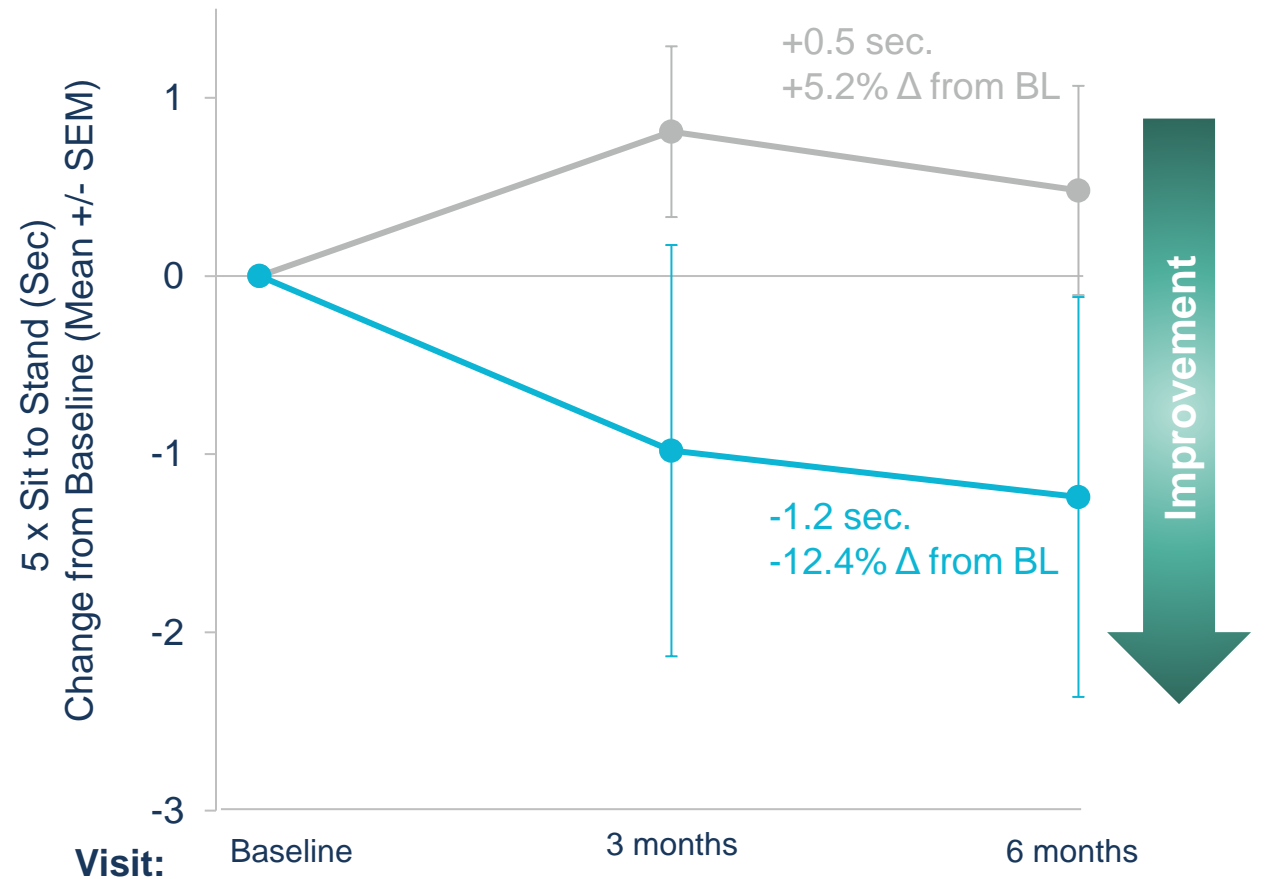
51.3 (4.2)

Early and Robust Benefit Across Multiple Timed Function Tests

10-Meter Walk/Run Test



5 Times Sit to Stand



Cohort: ● Placebo (N = 14) ● DYNE-101 6.8 mg/kg Q8W (N = 6)

Cohort: ● Placebo (N = 14) ● DYNE-101 6.8 mg/kg Q8W (N = 6)

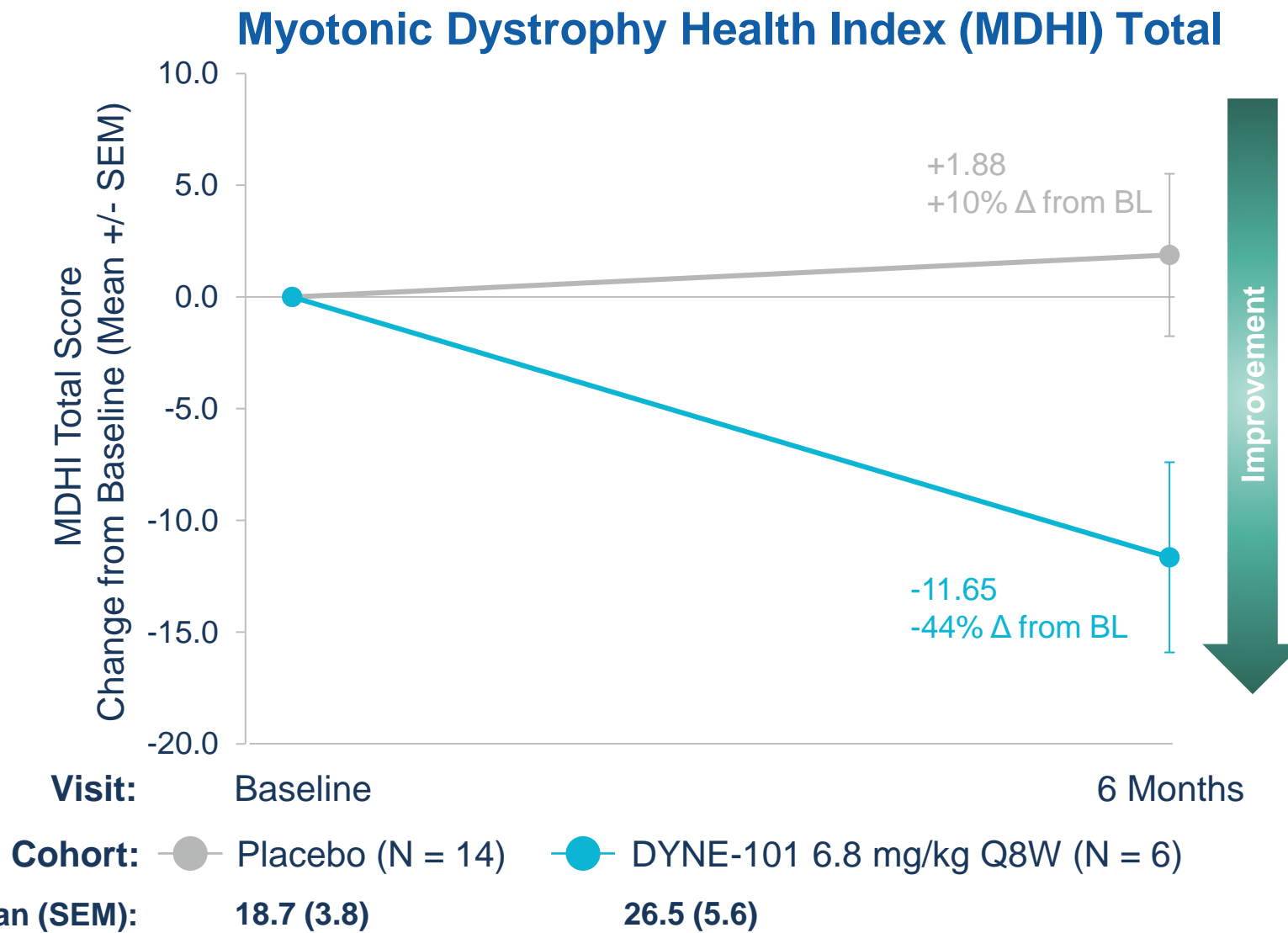
Baseline (sec), mean (SEM): 3.3 (0.1) 3.9 (0.6)

Baseline (sec), mean (SEM): 9.2 (0.5) 10.0 (1.4)

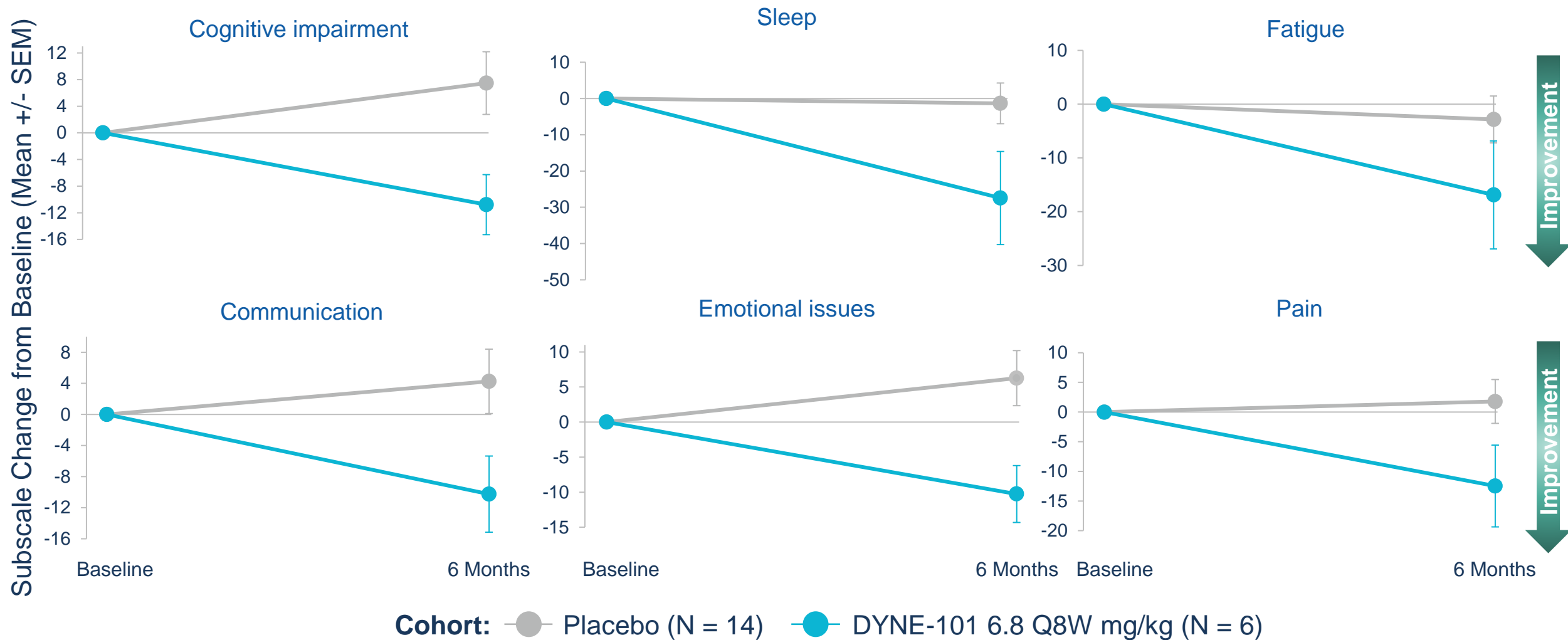


Notes: BL = baseline; 3 months = 85 days; 6 months = 169 days.

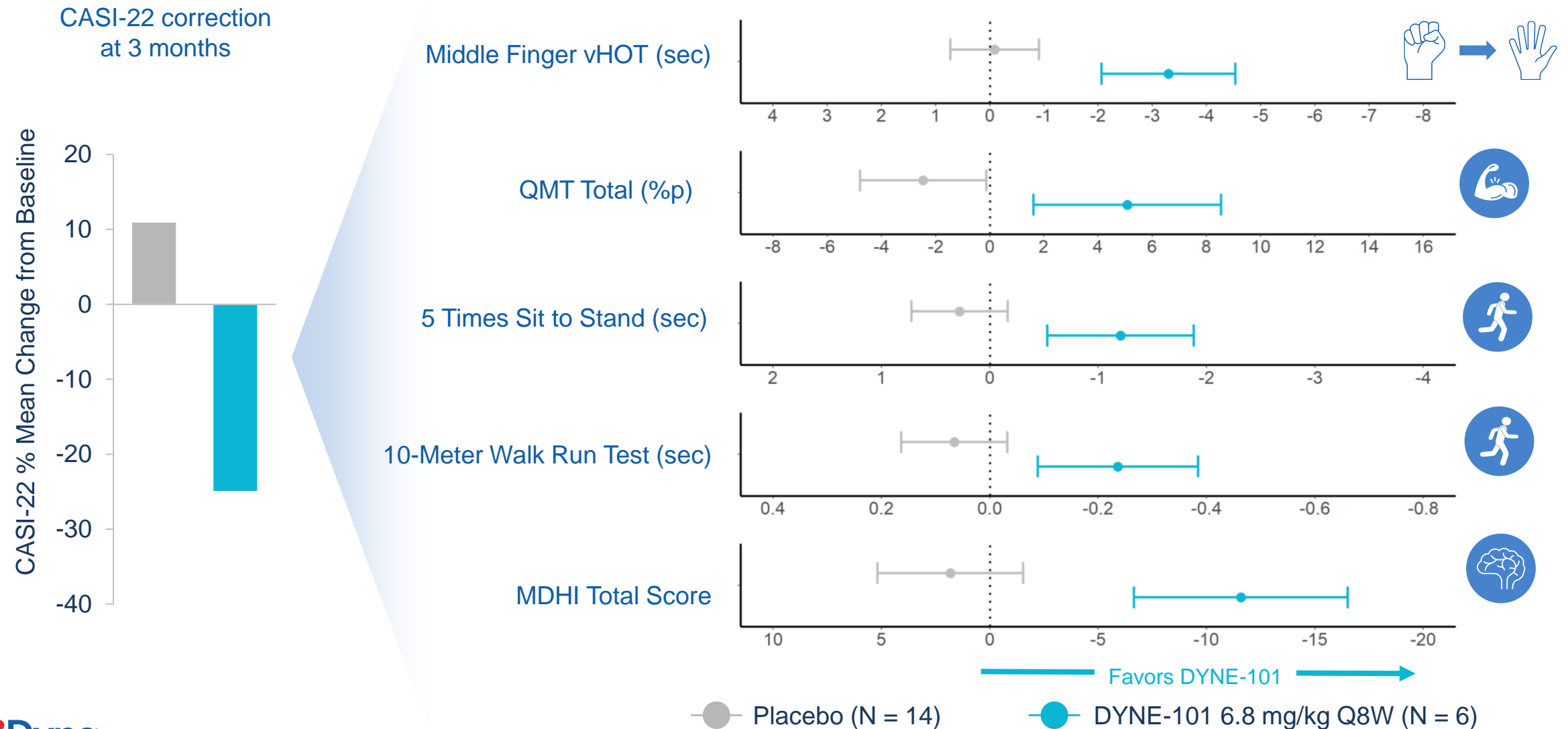
Improvement in MDHI Total Indicates Encouraging Patient Reported Outcome Trends



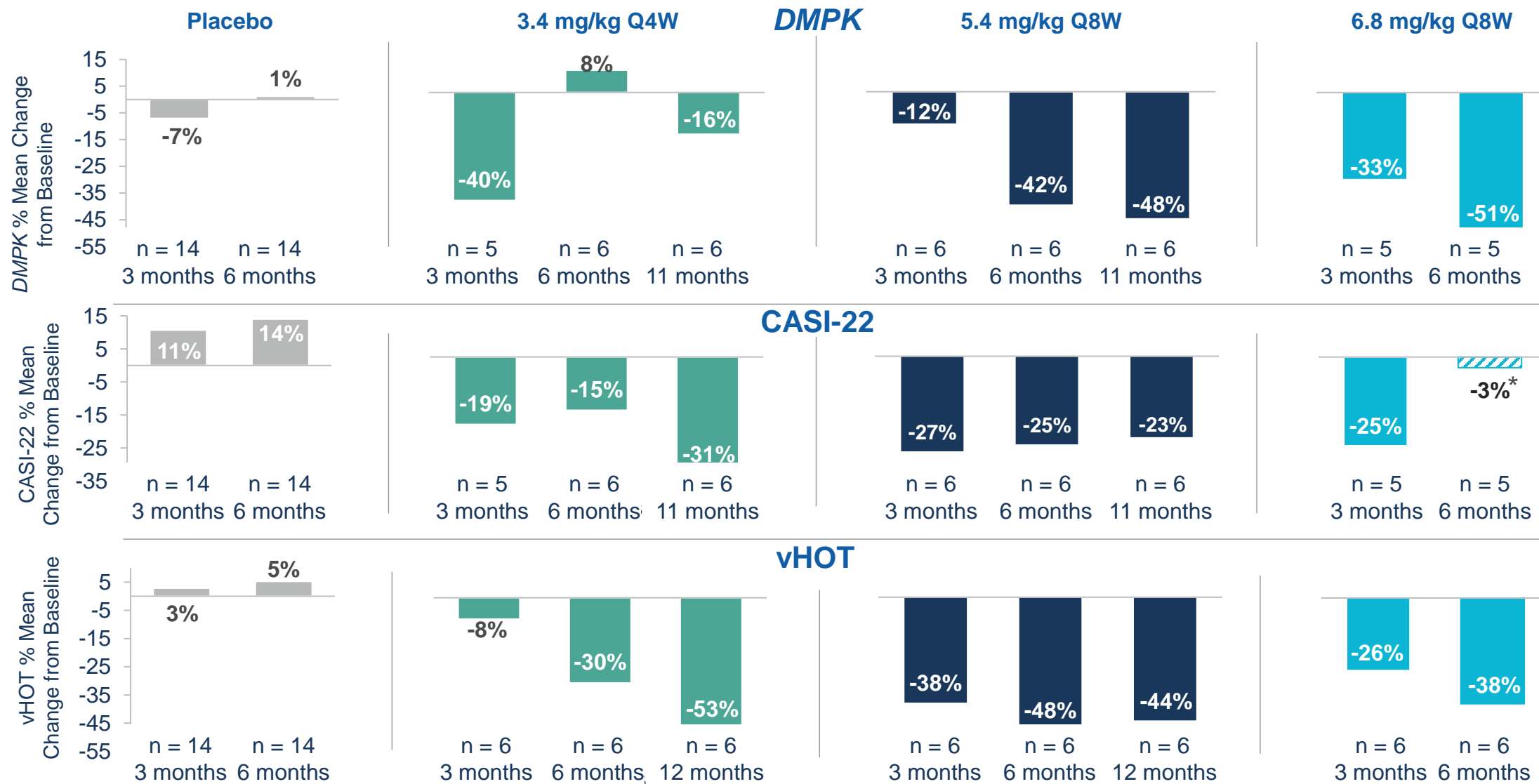
CNS-related MDHI Subscales Show Improvement at Registrational DYNE-101 Dose



Benefit at 6 Months Strengthened when Adjusting for Baseline Imbalances



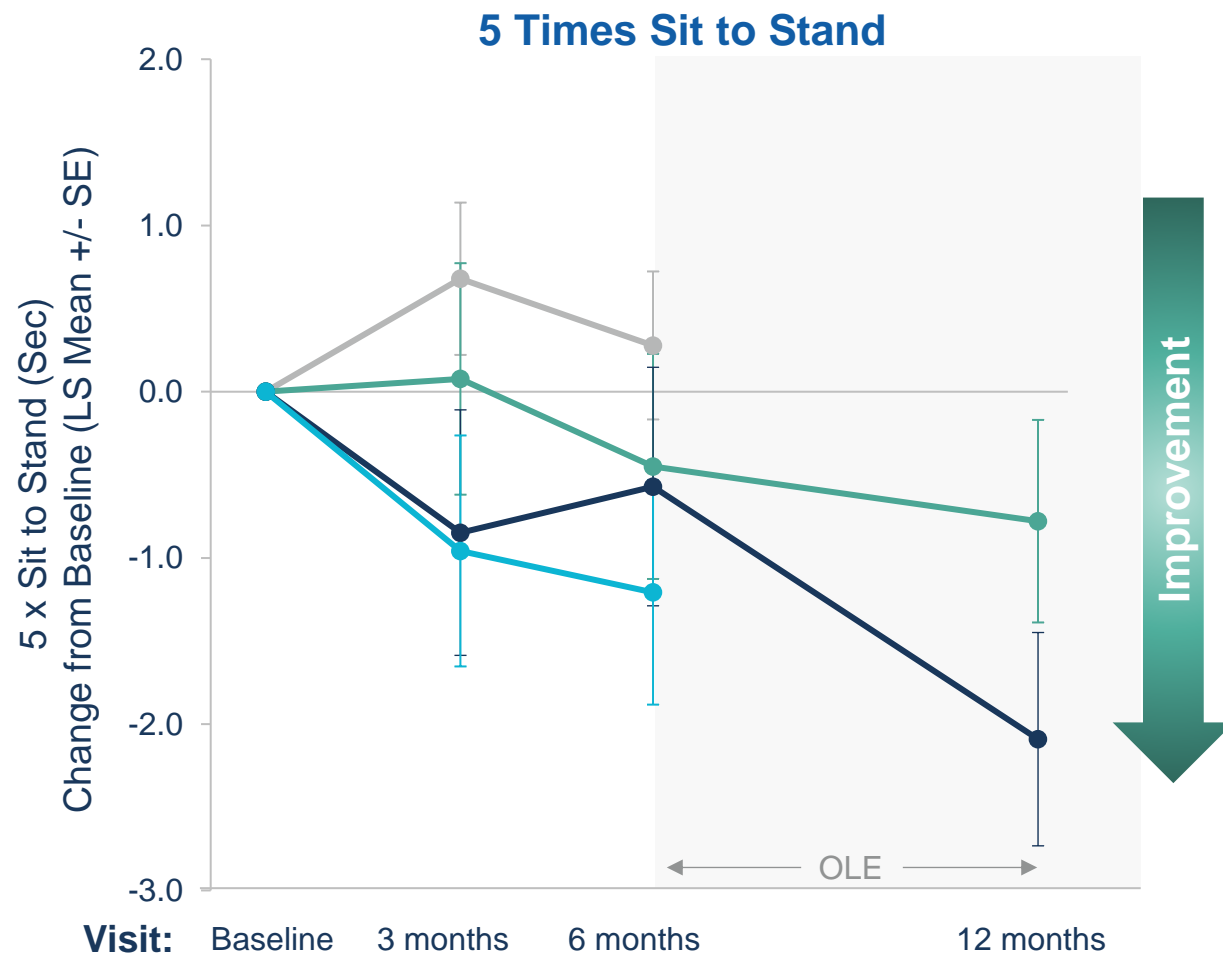
Additional Data: Robust *DMPK* KD, Splicing Correction, and vHOT



Note: One post-baseline sample in 3.4 mg/kg Q4W and one baseline sample in 6.8 mg/kg treatment groups not included within splicing assay as the sample did not meet QC criteria.

* Data confounded by missing baseline data and intra-patient sample variability. 3 months = 85 days; 6 months = 169 days; 11 months = 309 days; 12 months = 337 days.

Additional Data: Early, Sustained and Deepening Benefit on 5x Sit to Stand with Baseline Adjustment



Placebo (N = 14)
 DYNE-101 3.4 mg/kg Q4W (N = 6)
 DYNE-101 5.4 mg/kg Q8W (N = 6)
 DYNE-101 6.8 mg/kg Q8W (N = 6)

Myotonic Dystrophy Type 1 (DM1) Clinical Data Show Broad Functional Impact and Support Potential for Accelerated Approval



Initiating Registrational Expansion Cohort: primary endpoint of splicing correction at 3 months, supported by functional endpoints and PROs; full enrollment expected mid-2025



6.8 mg/kg Q8W dose showed robust splicing correction at 3 months and broad functional improvement, starting at 3 months and continuing at 6 months



Continued favorable safety profile¹; no serious related TEAEs

Building a Global DMD Franchise of Transformative Therapeutics



Overview

- Mutation in the *DMD* gene that encodes for dystrophin
- Onset in first few years of life
- Life expectancy ~30 years



Clinical Presentation

- Muscle weakness
- Progressive loss of function
- Loss of ambulation
- Respiratory/cardiac failure



Population

- ~12,000 - 15,000 (US)
- ~ 25,000 (Europe)



OUR APPROACH

Potential Best-in-class Targeted Exon Skipping


Increase dystrophin expression and enable less frequent dosing to potentially **stop or reverse disease progression**

Current Approved Exon 51 Therapies Only Increased Dystrophin Production

<1%

Registrational Expansion Cohort of DELIVER Rapidly Enrolling



Population	Primary Endpoints	Additional Endpoints	Stages of DELIVER
<ul style="list-style-type: none">• Male patients with DMD with mutations amenable to exon 51 skipping therapy• Ages 4 to 16 years• Ambulant and non-ambulant	<ul style="list-style-type: none">• Safety and tolerability• Change from baseline in dystrophin protein levels by Western Blot	<ul style="list-style-type: none">• Pharmacokinetics• Change from baseline of:<ul style="list-style-type: none">– Exon 51 skipping levels– Muscle tissue PDPF– Multiple assessments of muscle function, including NSAA score, SV95C and certain timed functional tests	<ul style="list-style-type: none">✓ Multiple Ascending Dose (MAD): 24 weeks• Open-Label Extension (OLE): 24 weeks• Long-Term Extension (LTE): 192 weeks <p style="text-align: center;"> Registrational Expansion Cohort</p>

DYNE-251 Safety Profile Is Favorable

Summary of Treatment Emergent Adverse Events (TEAEs)¹

TEAE Category	Participants with ≥1 TEAE – n (%)								
	0.7mg/kg Q4W N=6	1.4 mg/kg Q4W N=6	2.8 mg/kg Q4W N=6	5 mg/kg Q4W N=6	10 mg/kg Q4W N=8	20 mg/kg Q4W N=8	40 mg/kg Q8W N=8	40 mg/kg Q4W N=6	Overall ¹ N=54
Any TEAE	6 (100%)	6 (100%)	6 (100%)	6 (100%)	7 (88%)	8 (100%)	7 (88%)	5 (83%)	51 (94%)
Any related TEAE	3 (50%)	3 (50%)	0	6 (100%)	3 (38%)	4 (50%)	2 (25%)	3 (50%)	24 (44%)
Any serious TEAE	0	0	1 (17%)	0	0	1 (13%)	2 (25%)	2 (33%)	6 (11%)
Any serious related TEAE	0	0	0	0	0	0	0	2 (33%)	2 (4%)
Any TEAE leading to withdrawal from study	0	0	0	0	0	0	0	0	0
Any TEAE leading to death	0	0	0	0	0	0	0	0	0

Most TEAEs Were Mild or Moderate in Intensity¹

- 3 serious TEAEs potentially related to study drug in two participants
 - Acute kidney injury (1); thrombocytopenia (1)²
 - Pancytopenia (1)³
- 6 serious TEAEs unrelated to study drug
 - Dehydration due to gastroenteritis (1)
 - Femoral neck fracture (1); gastric volvulus (1)⁴
 - Tibia fracture (1)
 - Febrile convulsion (1); pyrexia (1)⁵
- Most common TEAEs (≥20% participant incidence)⁶
 - Pyrexia (44%)
 - Fall; vomiting (each 33%)
 - Headache (32%)
 - Nasopharyngitis (28%)
 - Cough; infusion-related reaction⁷ (each 20%)

Additional Safety Data

- Other than two participants with serious TEAEs in 40 mg/kg Q4W cohort:
 - No participants have demonstrated persistent related anemia or thrombocytopenia
 - No participants have demonstrated kidney injury
- No participants have demonstrated clinically meaningful changes in electrolytes, including magnesium

837 Doses Administered to Date Representing Over 65 Patient-Years of Follow-Up¹

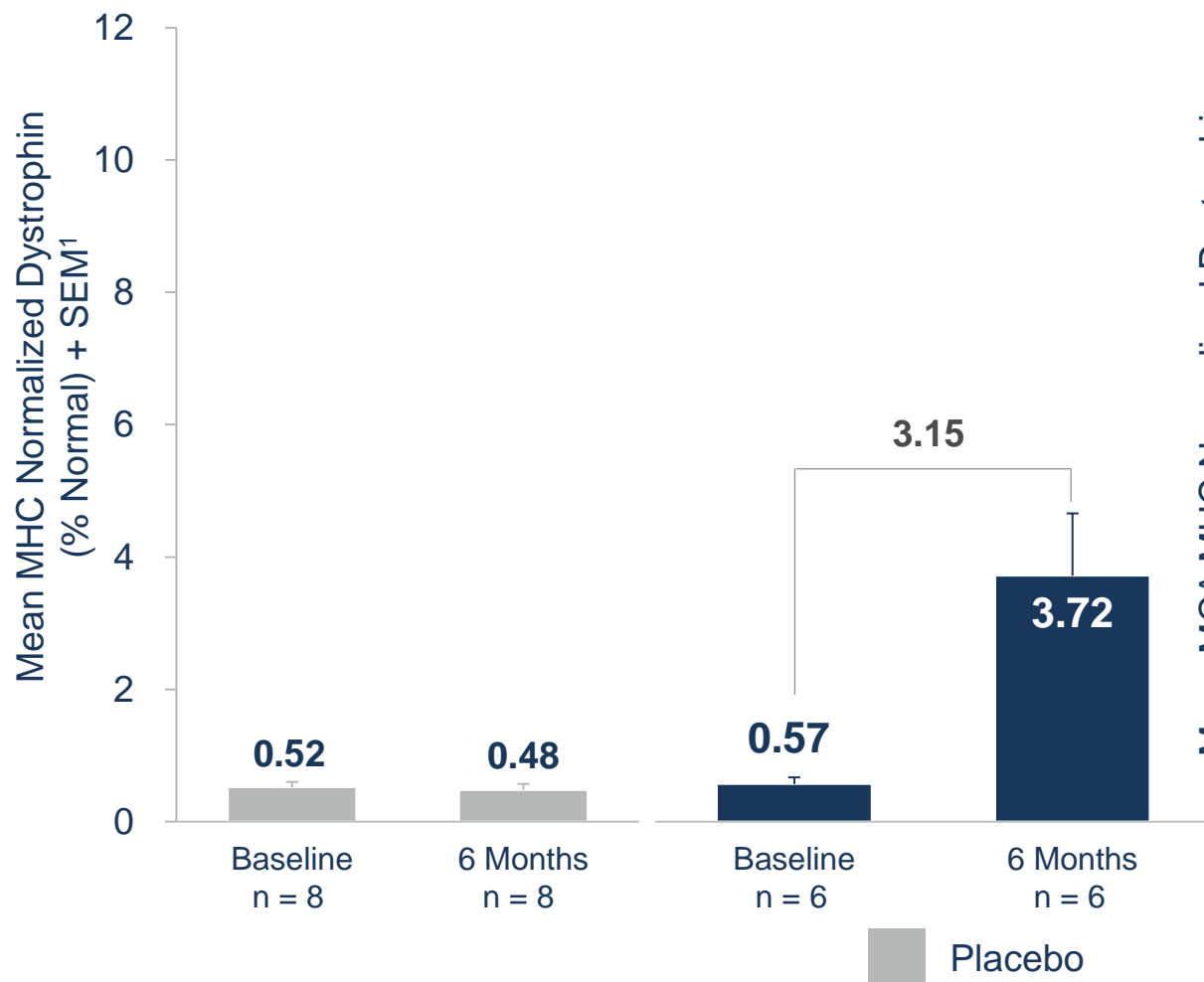
1. Data as of November 21, 2024; 2. Events have same day of onset in a single participant with a nonserious related TEAE of anemia in the context of fever, hemolysis, diarrhea and positive blood in stool; together these events are consistent with hemolytic uremic syndrome (HUS) with a possible infectious etiology; 3. Participant has a history of hemolytic anemia of unidentified etiology; presented with fever and tonsillitis; symptoms resolved without therapeutic intervention; 4. Events occurred in same participant at different times; 5. Events occurred in same participant at different times; 6. All cohorts combined; preferred terms are reported; 7. All infusion-related reactions have been mild or moderate in intensity; dosing has continued in all participants who experienced infusion-related reactions.

DYNE-251 Achieved Robust Dystrophin Expression

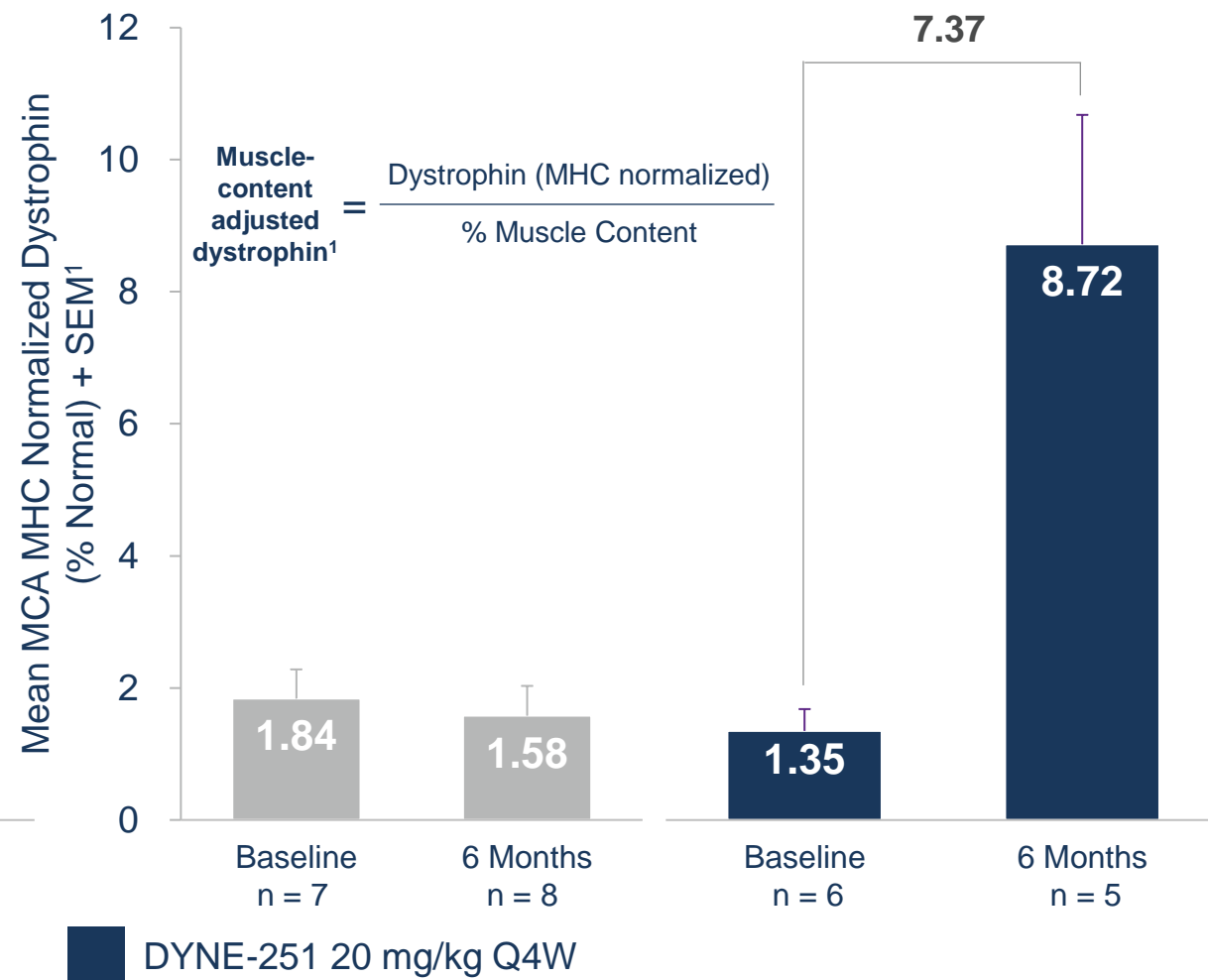
DYNE-251 Showed 3.7% Unadjusted and 8.7% Adjusted Dystrophin at 6 Months

Previously Presented Data

Unadjusted Dystrophin



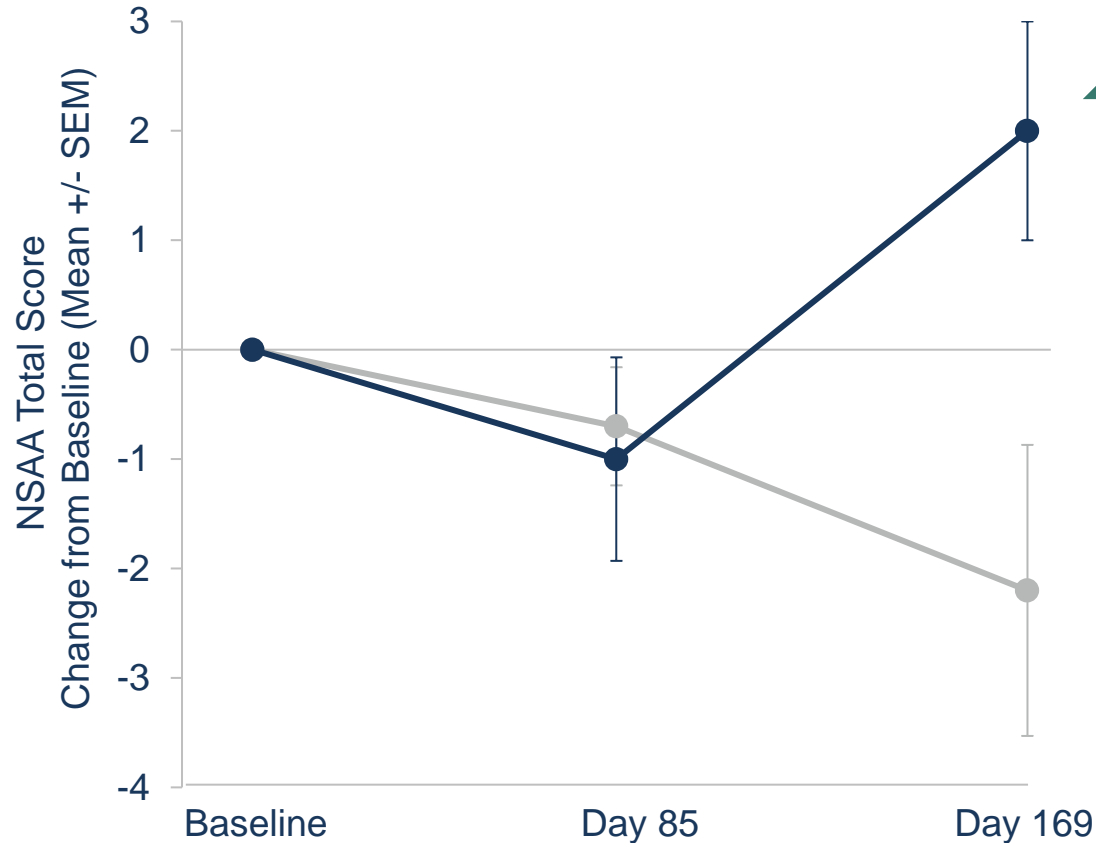
Muscle Content Adjusted Dystrophin



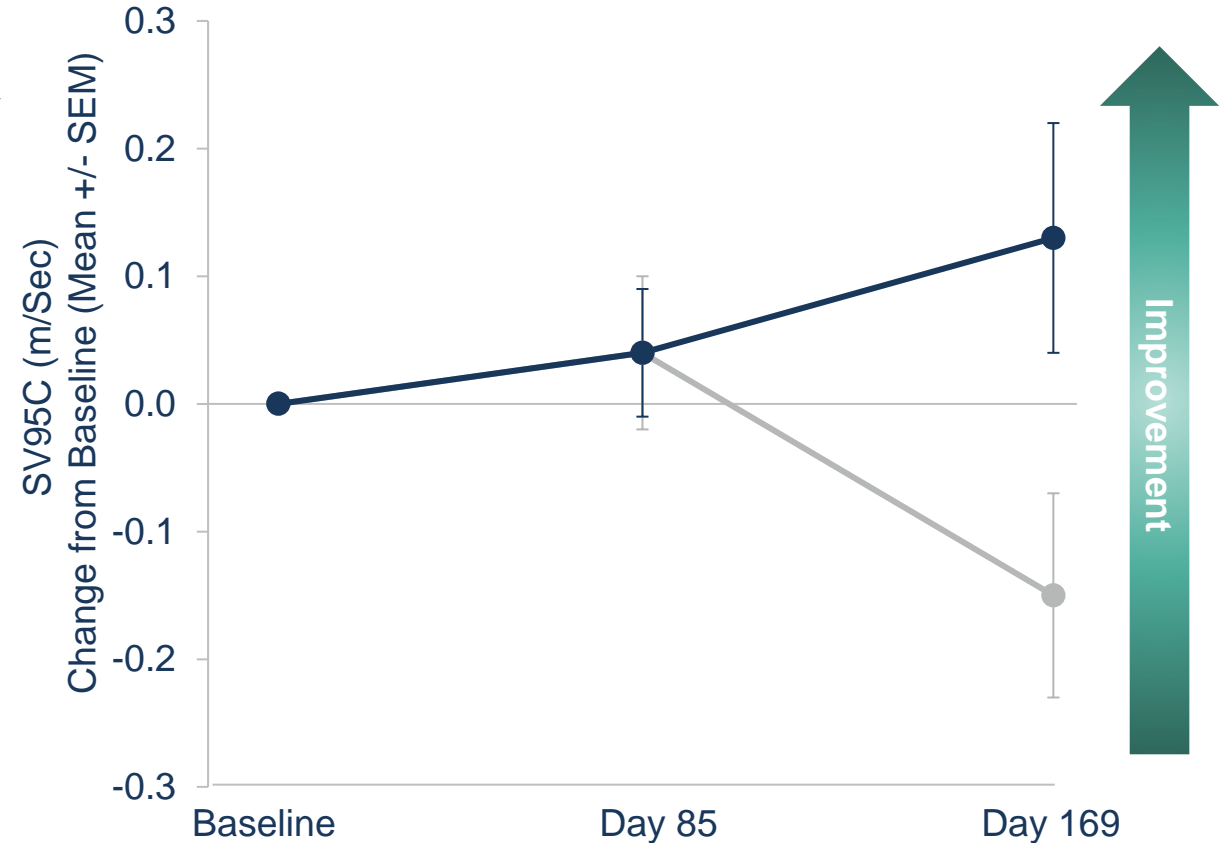
Unprecedented Clinically Meaningful Benefits Observed at Registrational Dose

Previously Presented Data

North Star Ambulatory Assessment (NSAA) Total Score



Stride Velocity 95th Centile (SV95C)

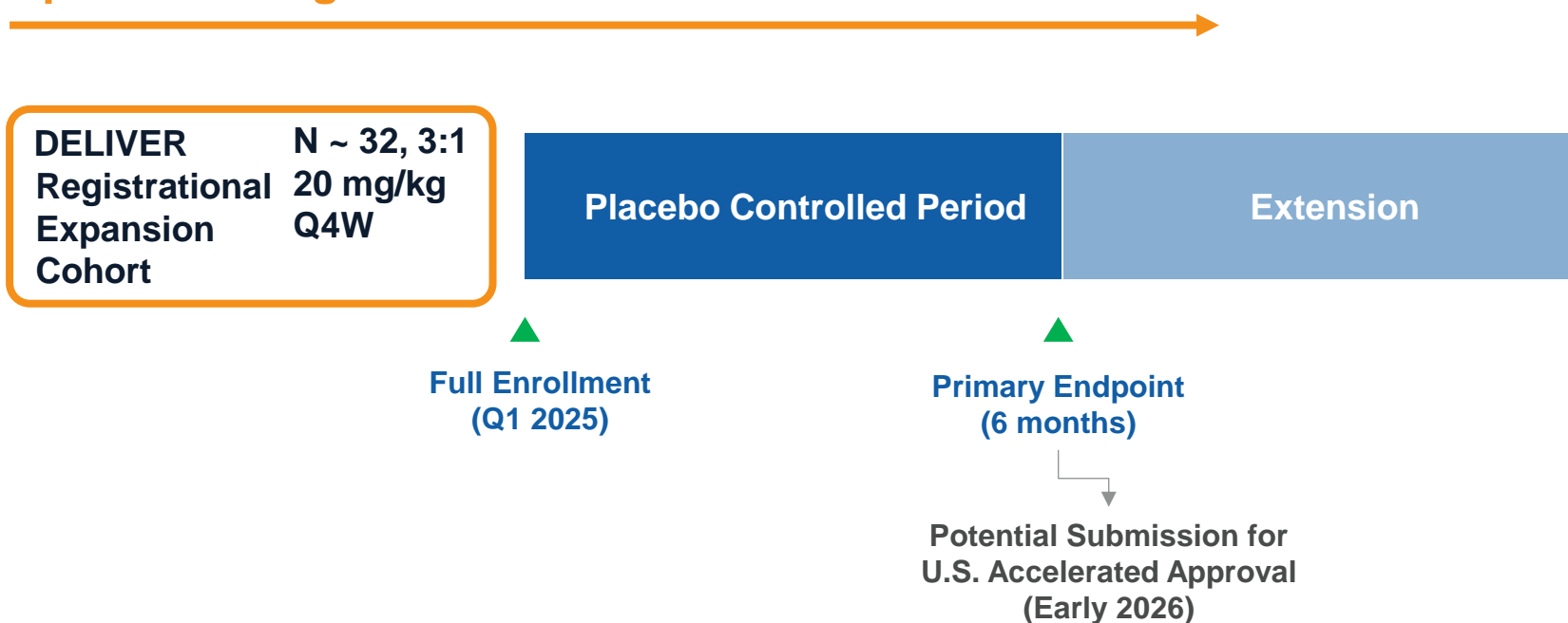


● Placebo (n = 6) for SV95C and (n = 14) for NSAA

● DYNE-251 20 mg/kg Q4W (n = 6 for both endpoints)

Registrational Expansion Cohort Timeline Enables Potential Submission for U.S. Accelerated Approval in Early 2026

Accelerated Approval Path Enables Speed to Filing



Potential Profile

Primary Endpoint (6 months)

- Change from baseline in dystrophin protein levels by Western Blot

Additional Endpoints

- Change from baseline of:
 - Exon 51 skipping levels
 - Muscle tissue PDPF
- NSAA
- SV95C

Program



Opening Remarks

John Cox, President & CEO



New Data from DYNE-101 ACHIEVE Trial in DM1 Update on DYNE-251 DELIVER Trial in DMD

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



Closing Remarks

John Cox, President & CEO

Robust Portfolio Focused on Neuromuscular Diseases with Opportunities to Expand by Leveraging FORCE Delivery

DISEASE	TARGET	DISCOVERY	PRECLINICAL	PHASE 1/2	ESTIMATED PATIENTS
Myotonic Dystrophy Type 1 (DM1)	DMPK	DYNE-101			US: >40,000 Europe: >74,000
Duchenne Muscular Dystrophy (DMD)	Exon 51	DYNE-251			US: ~12,000-15,000 Europe: ~25,000
	Exon 53				
	Exon 45				
	Exon 44				
	Other Exons				
Facioscapulohumeral Muscular Dystrophy (FSHD)	DUX4	DYNE-302			US: ~16,000-38,000 Europe: ~35,000
Pompe disease	GAA	DYNE-401			US: ~3,800 Europe: ~7,000

Pipeline expansion opportunities in CNS, rare skeletal, cardiac and metabolic

Building Momentum Toward Potential Launches in 2027

	2024	2025	2026
DYNE-101 for DM1	<ul style="list-style-type: none">✓ MAD Complete✓ Registrational dose selected	Fully enroll Registrational Expansion Cohort (mid-2025)	<p>Registrational Expansion Cohort readout (H1 2026)</p> <hr/> <p>Submission for U.S. Accelerated Approval (H1 2026)</p>
DYNE-251 for Exon 51 DMD	<ul style="list-style-type: none">✓ Registrational Expansion Cohort initiated	Registrational Expansion Cohort readout (late 2025)	Submission for U.S. Accelerated Approval (early 2026)

The background of the slide is decorated with several sets of light blue, wavy lines that flow horizontally across the page, creating a sense of movement and depth. These lines are composed of multiple parallel lines that vary in thickness and curvature, giving them a fluid, organic appearance.

Q&A



Appendix: Additional Study Results



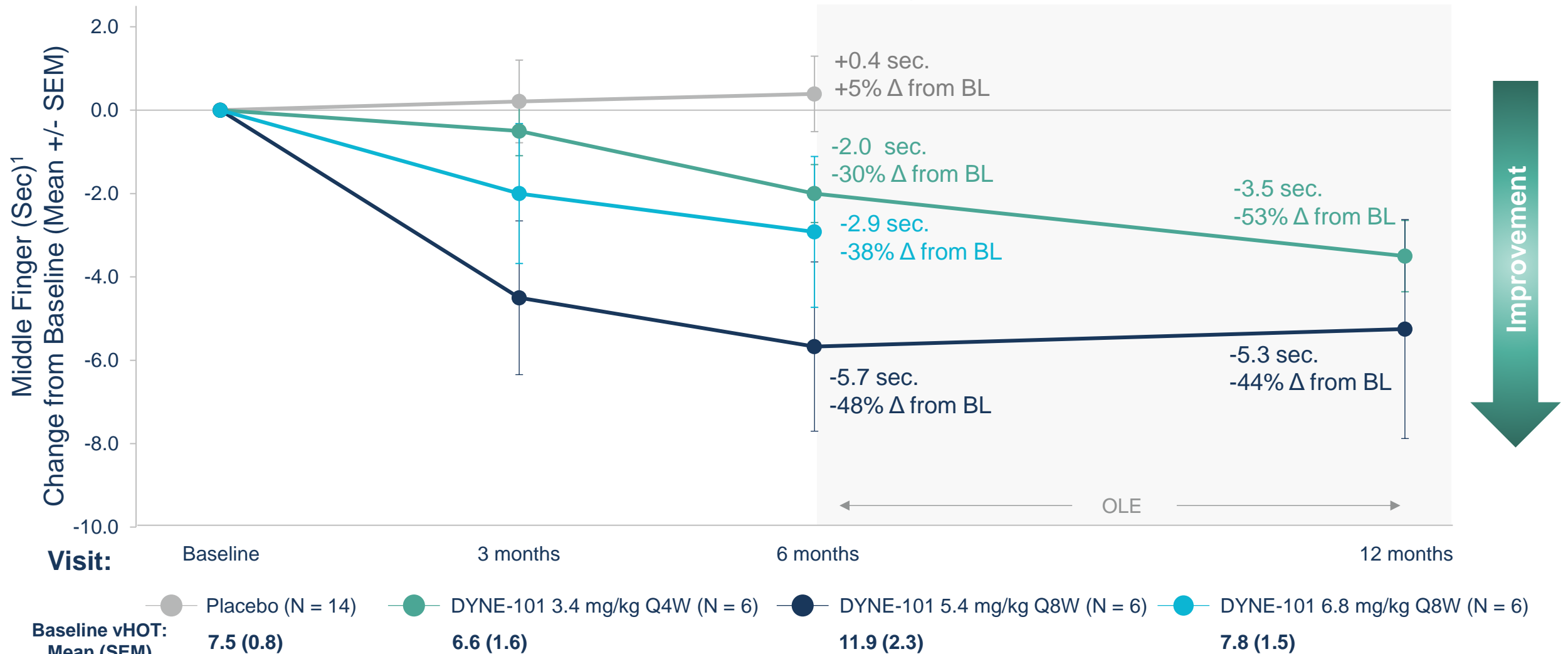
ACHIEVE Baseline Participant Characteristics: By Treatment

Mean (SD)	Placebo (N=14)	1.8 mg/kg Q4W (N=6)	3.4 mg/kg Q4W (N=6)	5.4 mg/kg Q8W (N=6)	6.8 mg/kg Q8W (N=6)
Age (years)	32.6 (9.6)	37.0 (10.5)	31.2 (4.4)	40.2 (6.5)	37.2 (9.7)
BMI (kg/m ²)	24.4 (4.7)	21.6 (5.8)	21.1 (1.8)	21.4 (2.5)	23.4 (5.6)
CASI-22	0.68 (0.20)	0.64 (0.25)	0.75 (0.12)	0.82 (0.16)	0.74 (0.25)
CTG Repeats	597 (246)	303 (163)	652 (258)	482 (236)	542 (191)
vHOT (sec) (middle finger)	7.5 (3.0)	11.3 (4.4)	6.6 (3.9)	11.9 (5.7)	7.8 (3.8)
QMT Total (% predicted)	51.5 (14.3)	48.1 (10.6)	42.0 (12.6)	46.6 (17.7)	51.3 (10.4)
10MWR (sec)	3.34 (0.48)	3.39 (0.55)	3.48 (0.67)	5.1 (2.40)	3.94 (1.56)
5 Times Sit to Stand (sec)	9.24 (2.03)	9.47 (2.04)	8.75 (1.88)	12.78 (6.79)	9.98 (3.33)
DM1-ACTIV ^c Total	47 (NA ^a)	46 (4.59)	38 (4.65)	44 (6.99)	43.4 (NA ^a)
MDHI Total	18.7 (13.8)	23.5 (23.2)	30.2 (23.2)	14.8 (7.4)	26.5 (13.7)

^a SDs for DM1-ACTIV^c are not reported to maintain blinding.

Early and Sustained Improvement in Functional Myotonia

vHOT Middle Finger



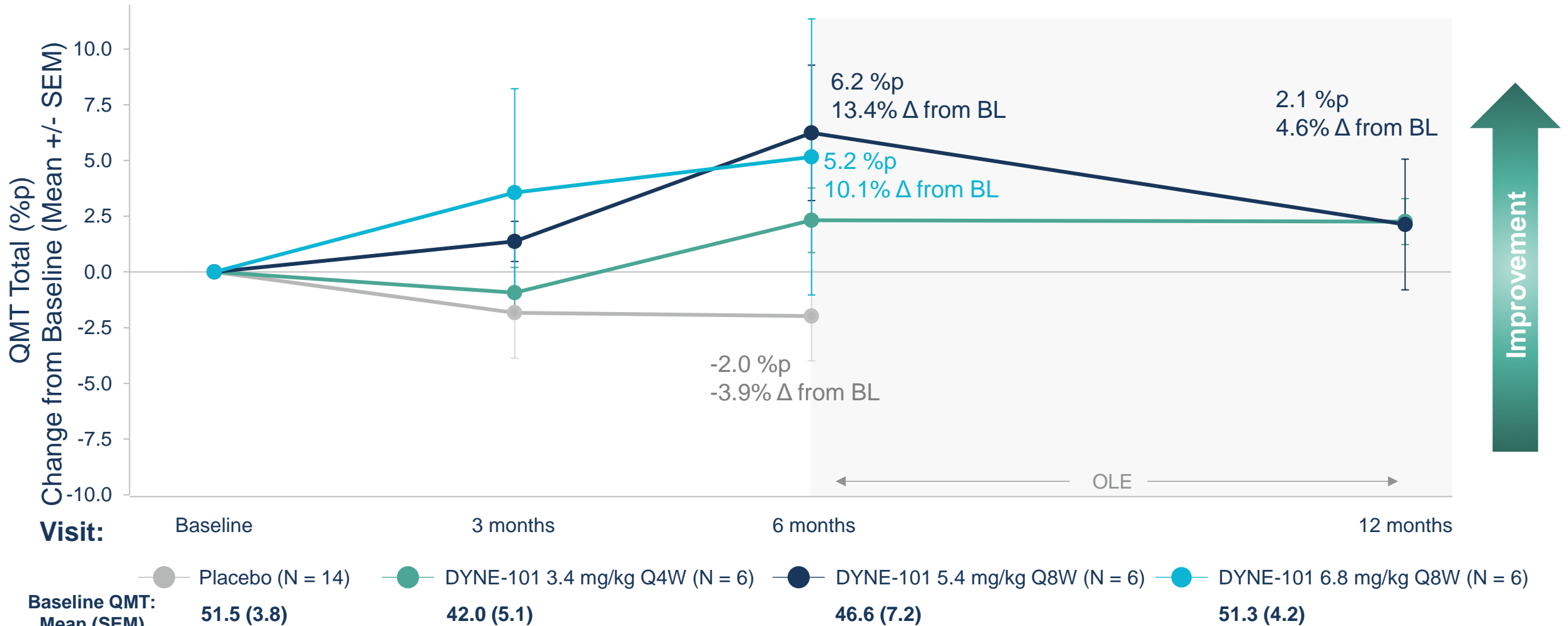
Baseline vHOT:
Mean (SEM)



Notes: 1. vHOT Middle Finger (sec) is the average of all myotonia trials for an individual participant in ACHIEVE; BL = baseline. 3 months = 85 days; 6 months = 169 days; 12 months = 337 days

Improvement in Muscle Strength at 6 and 12 Months

QMT Total Score



Baseline QMT:
Mean (SEM)

51.5 (3.8)

42.0 (5.1)

46.6 (7.2)

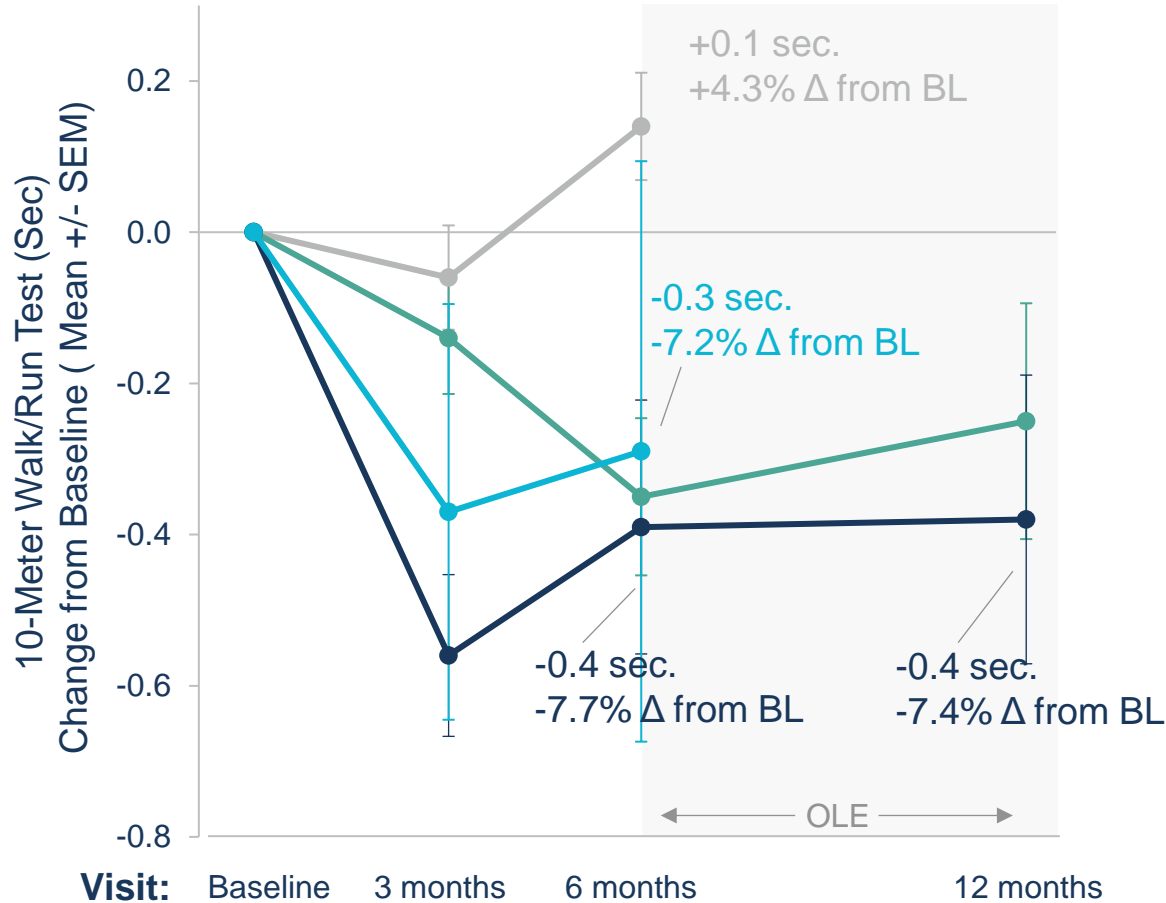
51.3 (4.2)



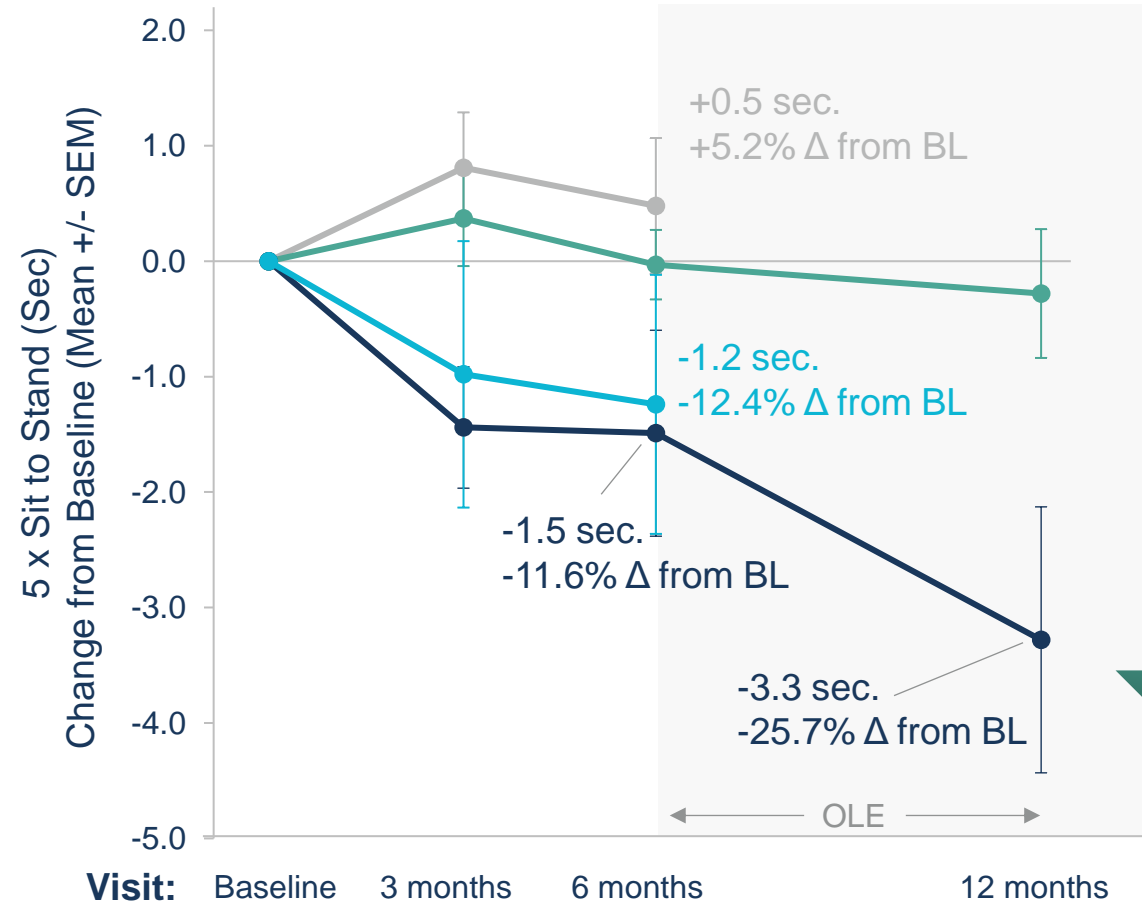
Notes: BL = baseline. 3 months = 85 days; 6 months = 169 days; 12 months = 337 days

Early and Sustained Benefit Across Measures of Daily Function

10-Meter Walk/Run Test



5 Times Sit to Stand



Improvement

Legend: Placebo (N = 14) (grey circle), DYNE-101 3.4 mg/kg Q4W (N = 6) (green circle), DYNE-101 5.4 mg/kg Q8W (N = 6) (dark blue circle), DYNE-101 6.8 mg/kg Q8W (N = 6) (light blue circle)

Baseline Mean (SEM) 10MWR: 3.3 (0.1) 3.5 (0.3) 5.1 (1.0) 3.9 (0.6)

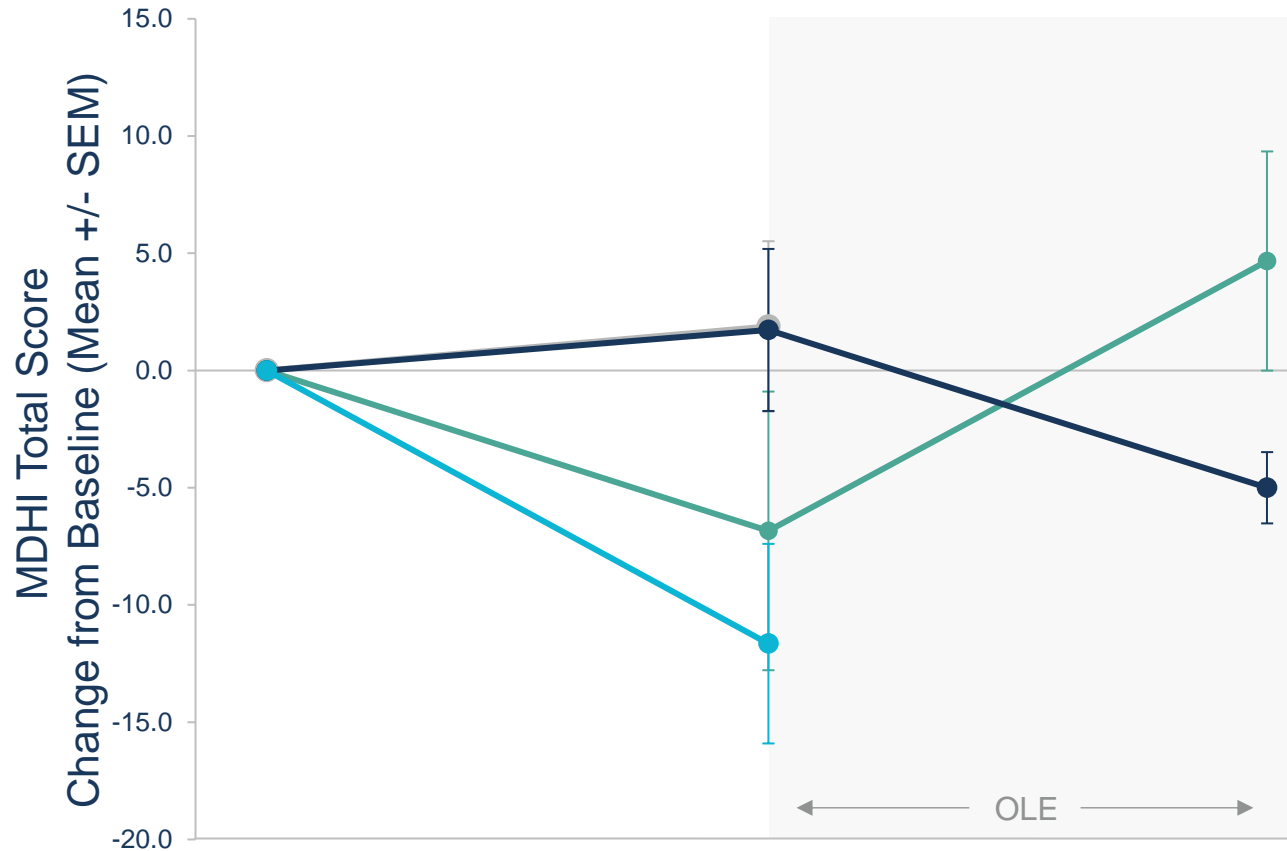
5xSTS: 9.2 (0.5) 8.8 (0.8) 12.8 (2.8) 10.0 (1.4)

Notes: BL = baseline. 3 months = 85 days; 6 months = 169 days; 12 months = 337 days



Encouraging Trends on MDHI Total PRO

MDHI Total Score



Visit:

Baseline

6 months

12 months

● Placebo (N = 14) ● 3.4 mg/kg Q4W (N = 6) ● 5.4 mg/kg Q8W (N = 6) ● 6.8 mg/kg Q8W (N = 6)

Baseline score, mean (SEM):

18.7 (3.8)

30.2 (9.5)

14.8 (3.0)

26.5 (5.6)

CNS-related MDHI Subscales Show Improvement at Registrational DYNE-101 Dose

Subscale Change from Baseline (Mean +/- SEM)

