Long-Term Functional Improvement With Zeleciment Basivarsen in the Phase 1/2 ACHIEVE Trial

Karlien Mul, MD, PhD[1], Guillaume Bassez[2], Jordi Diaz-Manera[3], James Lilleker[4], Marika Pane[5], Richard Roxburgh[6], Benedikt Schoser[7], Christopher Turner[8], Allison Kreuzer[9], Alexandra Lovett[9], Soma Ray[9], Huaihou Chen[9], Shauna Andersson[9], Douglas Kerr[9], Valeria Sansone[10]

[1]Radboud University Medical Center, Nijmegen, the Netherlands; [2]Institut de Myologie, Paris, France; [3]John Walton Muscular Dystrophy Research Centre, Newcastle University, Newcastle-Upon-Tyne, UK; [4]Muscle Disease Unit, Northern Care Alliance NHS Foundation Trust, Manchester Academic Health Science Centre, Manchester, UK; [5]Fondazione Policlinico Universitario A. Gemelli, Rome, Italy; [6]Neurogenetics Clinic Centre for Brain Research, University of Auckland, Auckland, NZ; [7]Friedrich-Baur-Institute, Dep. of Neurology LMU Clinics, Ludwig-Maximilians University, Munich, Germany; [8]University College London Hospitals, London, UK; [9]Dyne Therapeutics, Inc., Waltham, MA, USA; [10]Centro Clinico NeMO, University of Milan, Milan, Italy

WMS2026 HIROSHIMA
The 31st Annual Congress of the World Muscle Society
29 September - 3 October 2026, Hiroshima, Japan

To view an electronic version of this presentation and the related appendix, scan the QR codes below:

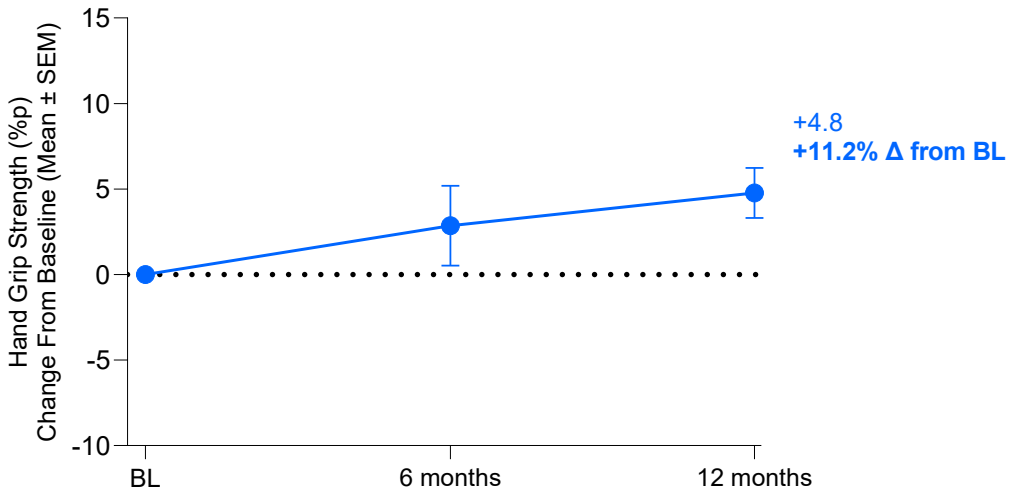
Presentation

Appendix

Improvement in Strength Is Sustained at 12 Months, Including Hand Grip, as Measured by QMT (%p)

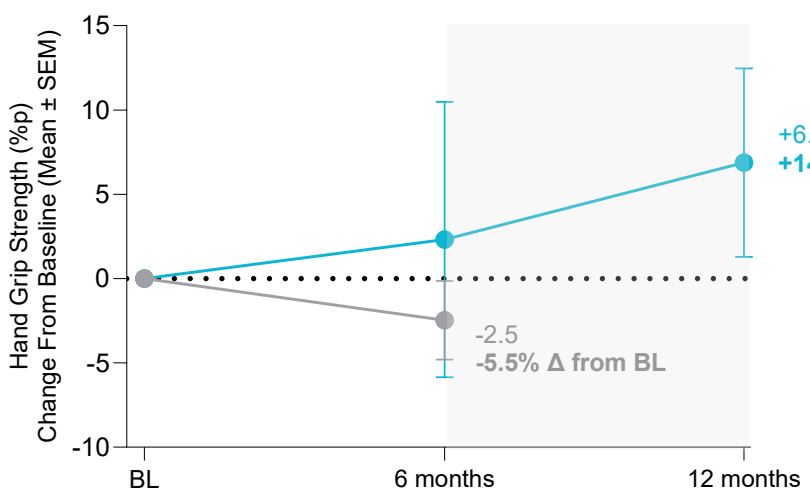
Quantitative Muscle Testing (QMT) Hand Grip Strength

Pooled Dose Group (N=26)



Baseline Mean (SEM)	Patients, N		
	BL	6 months	12 months
41.5 (3.2)	26	26	25

6.8 mg/kg Cohort



	Baseline Mean (SEM)	Patients, N		
		BL	6 months	12 months
Placebo	44.8 (4.5)	14	14	-
6.8 mg/kg	49.0 (5.3)	6	6	6

Improvement

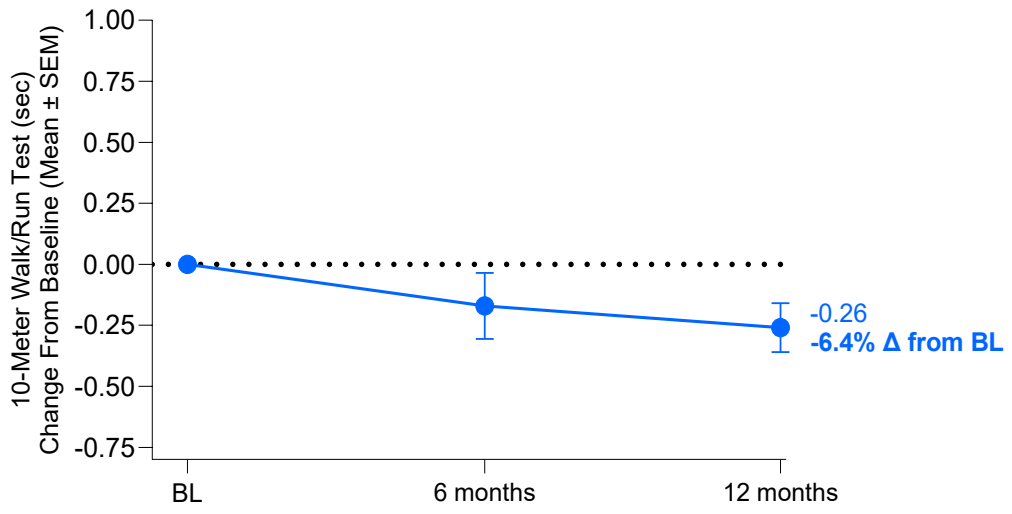
ACHIEVE

For information on the Pooled Dose Group, see footnotes on slide 2.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
%p, percent predicted; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; QMT, quantitative muscle testing; SEM, standard error of the mean.

Improvement in Motor Function Is Sustained at 12 Months, as Measured by 10MWR Timed Function Test

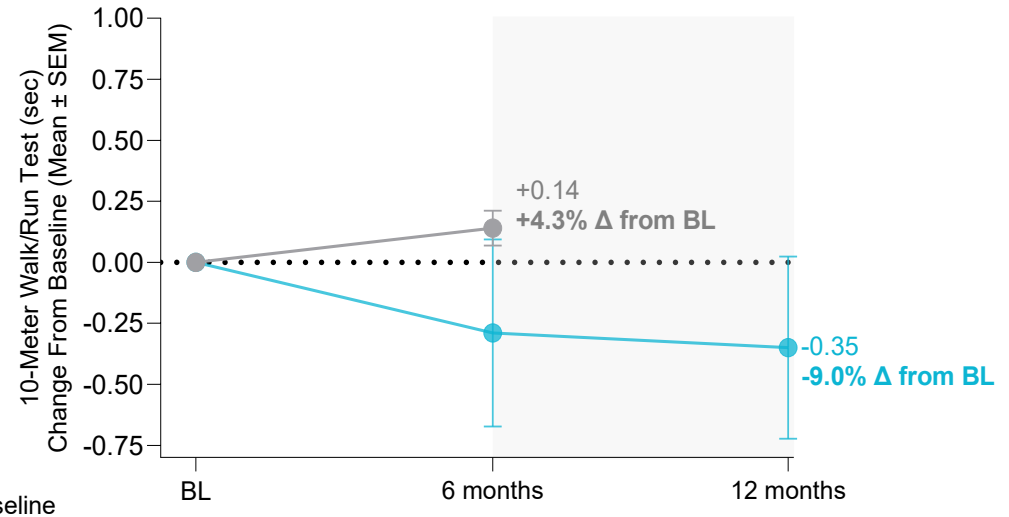
10-Meter Walk/Run Test

Pooled Dose Group (N=26)



Baseline Mean (SEM)	Patients, N		
	BL	6 months	12 months
4.0 (0.3)	26	26	26

6.8 mg/kg Cohort



	Baseline Mean (SEM)	Patients, N		
		BL	6 months	12 months
Placebo	3.3 (0.1)	14	14	-
6.8 mg/kg	3.9 (0.6)	6	6	6

Improvement

ACHIEVE

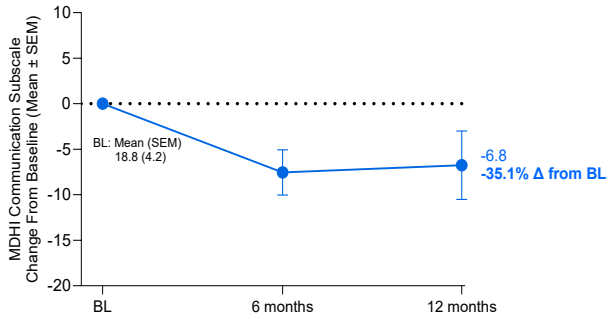
For information on the Pooled Dose Group, see footnotes on slide 2.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
10MWR, 10-Meter Walk/Run; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; sec, seconds; SEM, standard error of the mean.

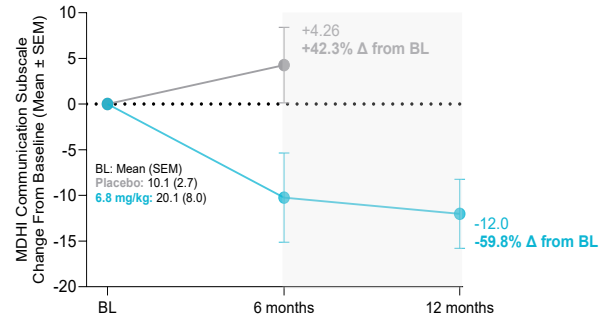
Improvement Is Observed Across CNS-Related MDHI Subscales Through 12 Months

Pooled Dose Group (N=26)

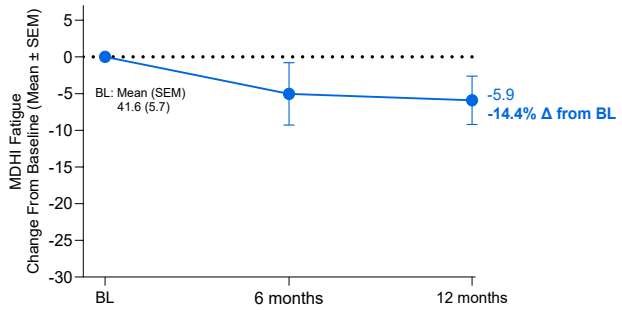
6.8 mg/kg Cohort

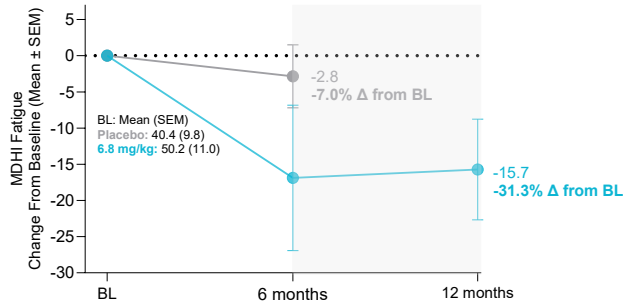
Communication



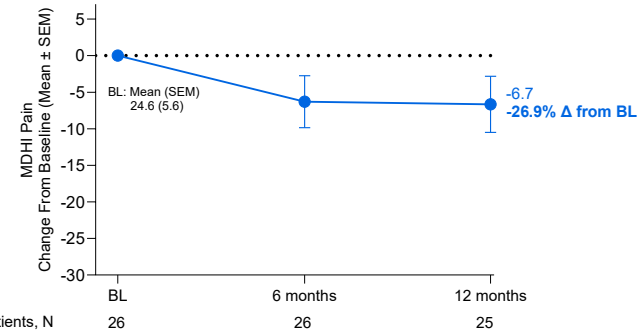


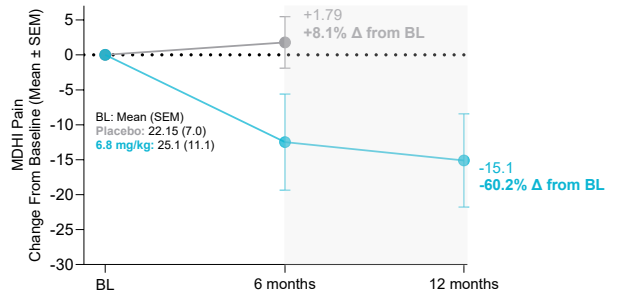
Fatigue





Pain







Patients, N			
	26	26	25
Placebo	13	13	-
6.8 mg/kg	6	6	6

ACHIEVE

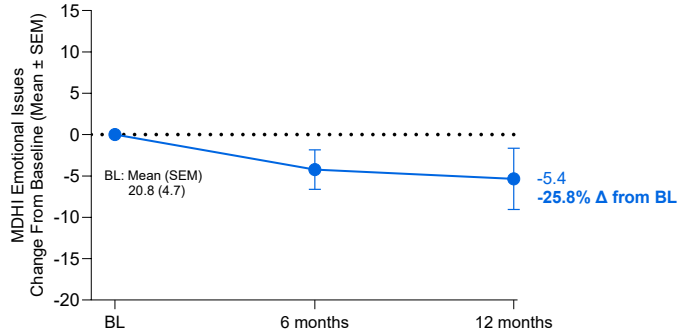
For information on the Pooled Dose Group, see footnotes on slide 2.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days. BL, baseline; CNS, central nervous system; MDHI, Myotonic Dystrophy Health Index; SEM, standard error of the mean.

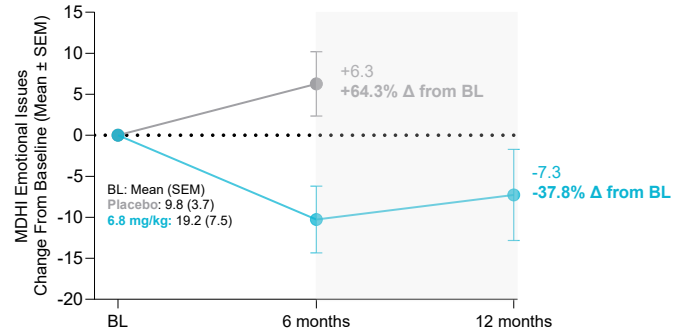
Improvement Is Observed Across CNS-Related MDHI Subscales Through 12 Months

Pooled Dose Group (N=26)

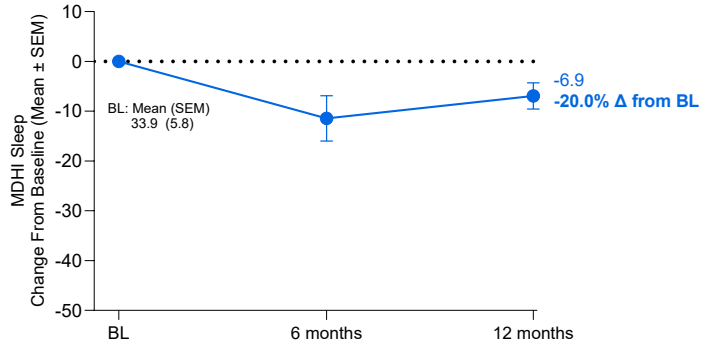
6.8 mg/kg Cohort

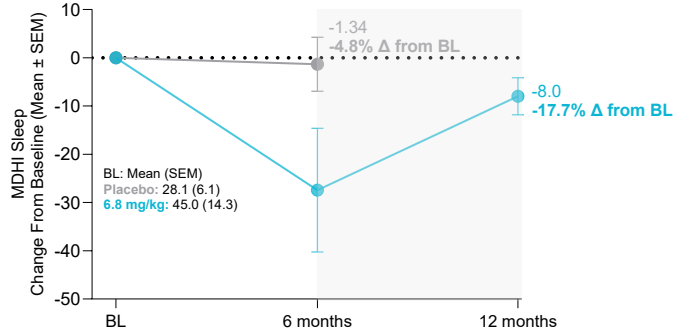
Emotional Issues



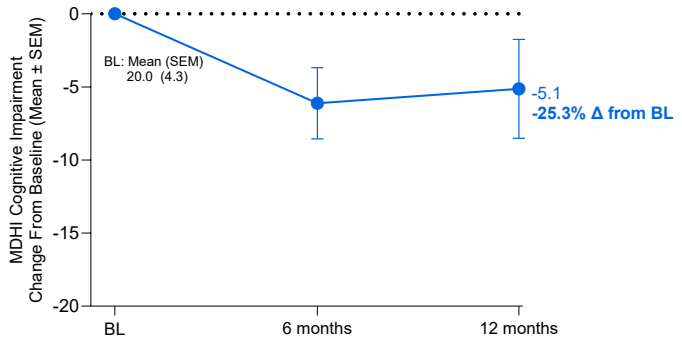


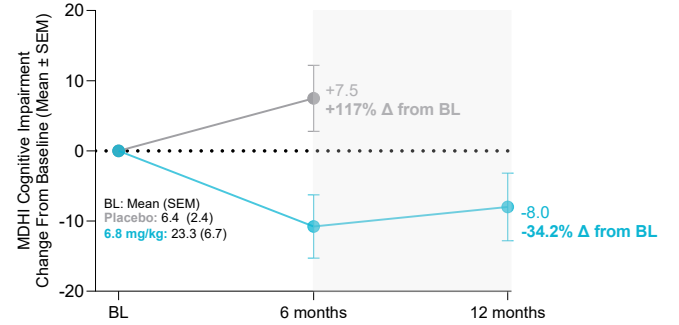
Sleep





Cognitive Impairment







Patients, N			
	26	26	25
Placebo	13	13	-
6.8 mg/kg	6	6	6

ACHIEVE

For information on the Pooled Dose Group, see footnotes on slide 2. Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days. BL, baseline; CNS, central nervous system; MDHI, Myotonic Dystrophy Health Index; SEM, standard error of the mean.