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

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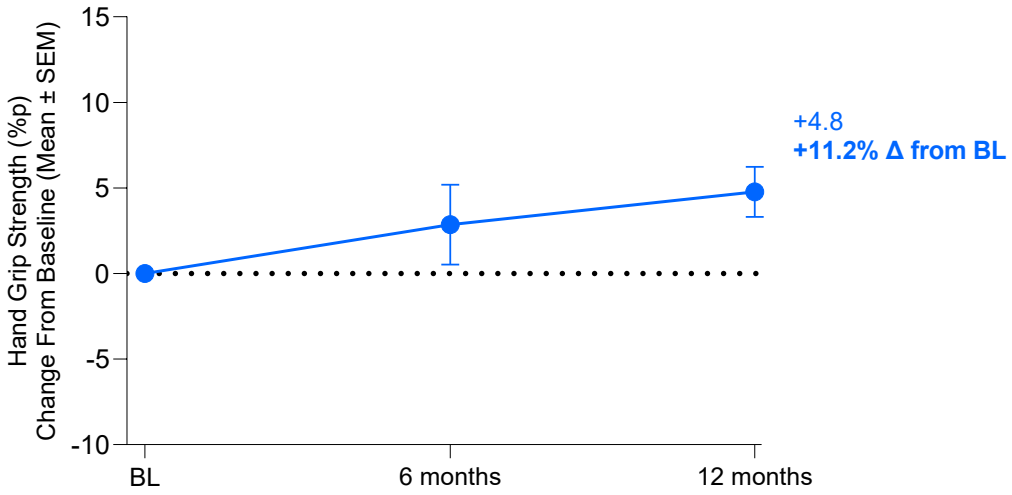
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Improvement in Strength Is Sustained at 12 Months, Including Hand Grip, as Measured by QMT (%p)

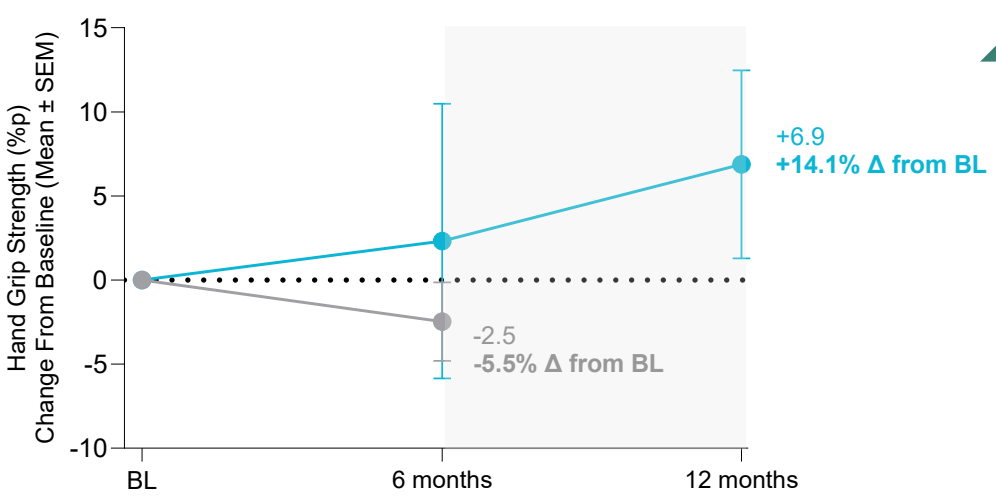
Quantitative Muscle Testing (QMT) Hand Grip Strength

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



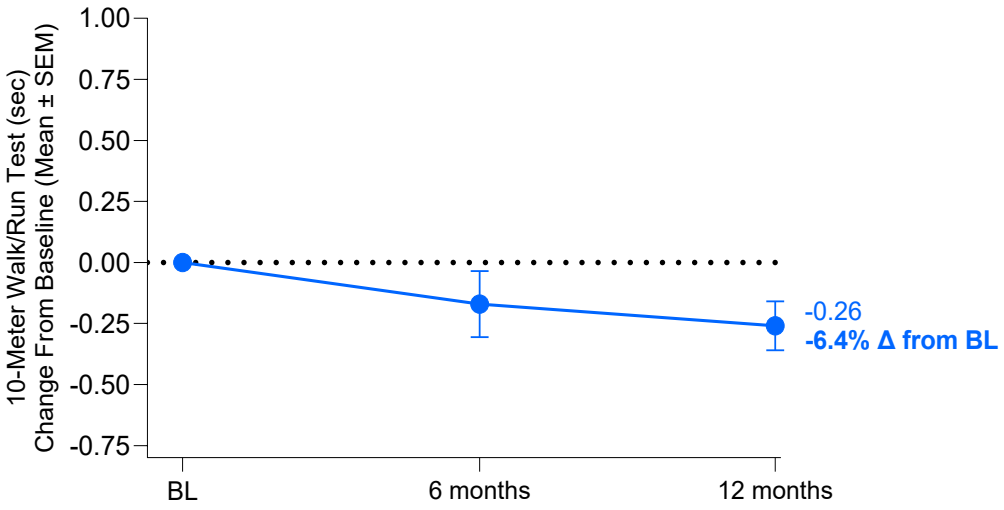
	Baseline Mean (SEM)	Patients, N		
		BL	6 months	12 months
Placebo	44.8 (4.5)	14	14	-
6.8 mg/kg	49.0 (5.3)	6	6	6

Pooled Dose Group includes participants from the 6.8 mg/kg Q8W, 5.4 mg/kg Q8W, and 3.4 mg/kg Q4W cohorts, including participants who initially received placebo within those cohorts and subsequently received active treatment. The participants who initially received placebo were re-baselined to the visit prior to their first active dose. Only 8/26 participants in the Pooled Dose Group received the registrational dose for the full 12 months analyzed, and most did not receive the registrational dose during this timeframe.
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

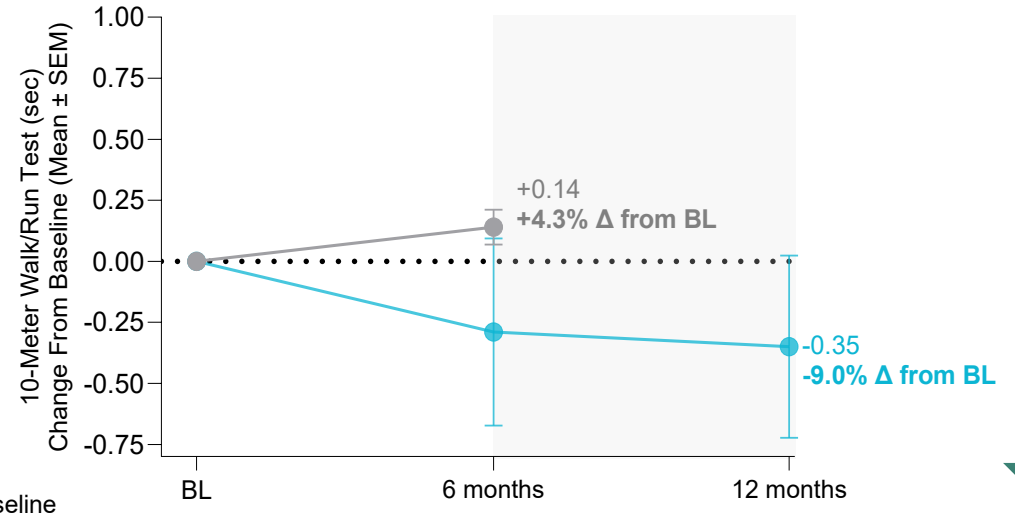
10-Meter Walk/Run Test

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

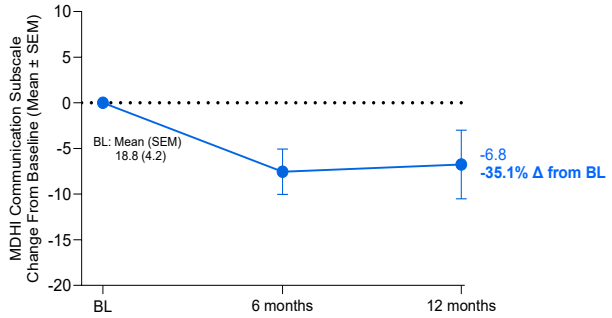


For information on the Pooled Dose Group, see footnotes on slide 2.
Percent mean change from baseline = (mean change from baseline/baseline mean) x 100. 6 months = 169 days; 12 months = 337 days.
10MWR, 10-Meter Walk/Run; BL, baseline; Q4W, every 4 weeks; Q8W, every 8 weeks; sec, seconds; SEM, standard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

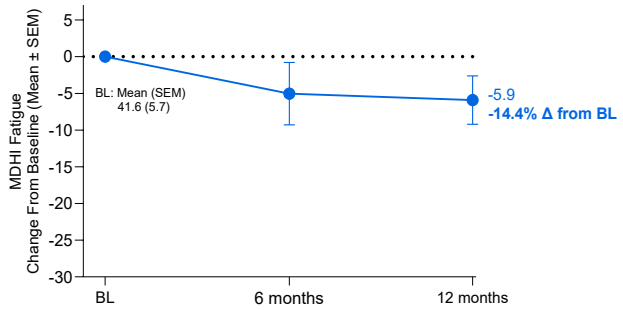
Improvement Is Observed Across CNS-Related MDHI Subscales Through 12 Months

Pooled Dose Group (N=26)

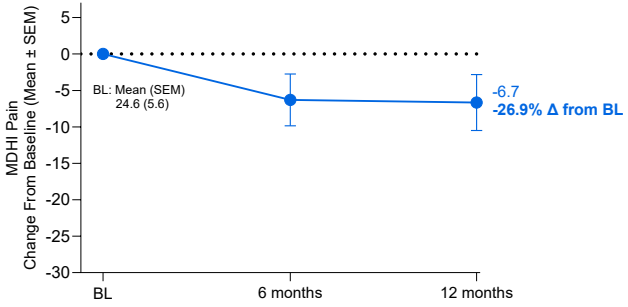
Communication



Fatigue



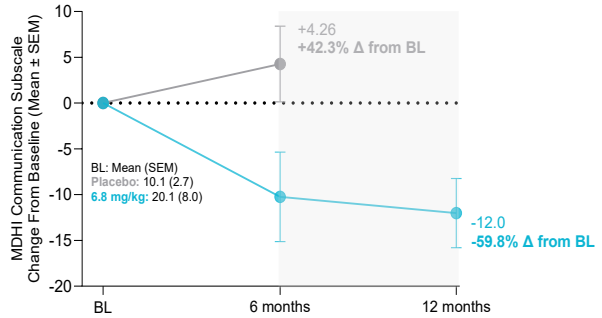
Pain



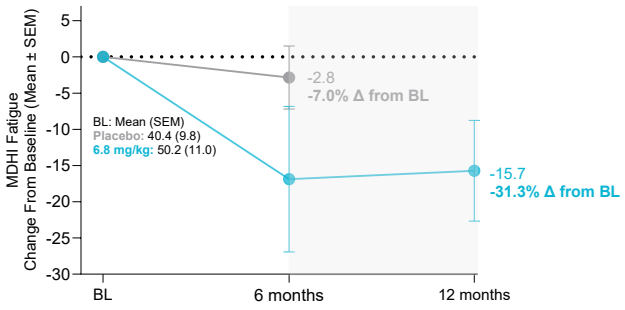
Patients, N: 26 (BL), 26 (6 months), 25 (12 months)

6.8 mg/kg Cohort

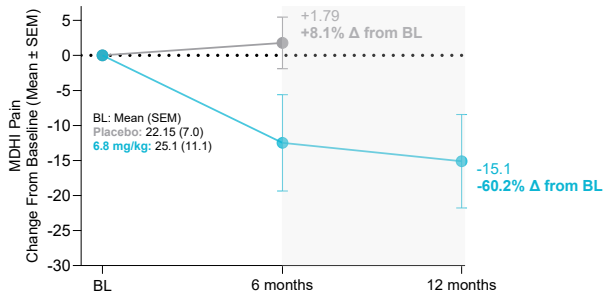
Communication



Fatigue



Pain



Patients, N
Placebo: 13 (BL), 13 (6 months), - (12 months)
6.8 mg/kg: 6 (BL), 6 (6 months), 6 (12 months)



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.

ACHIEVE

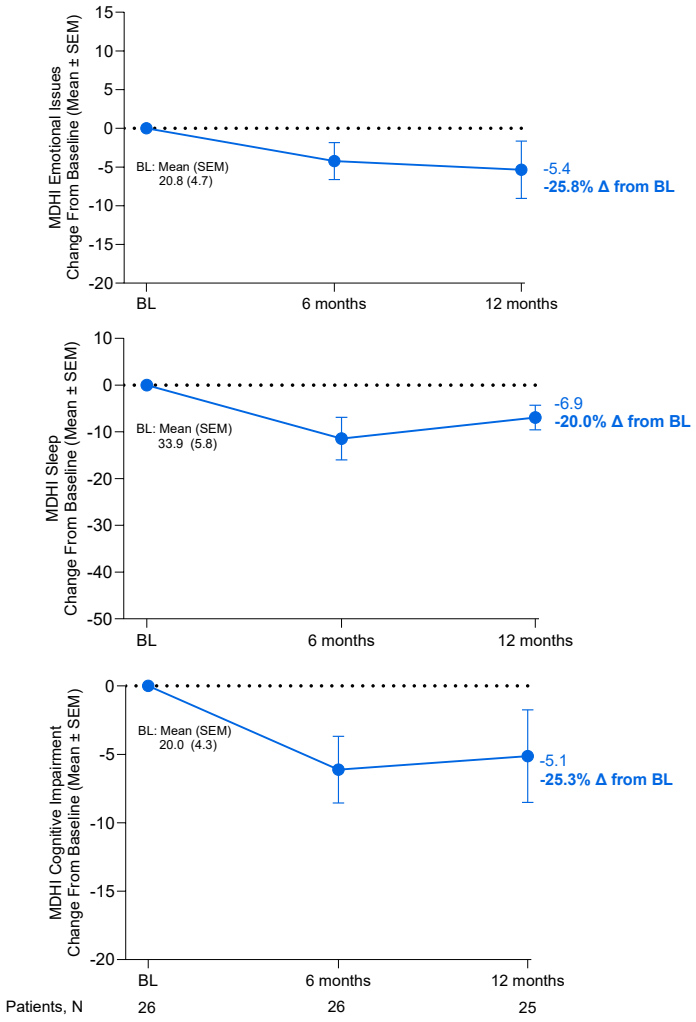
Improvement Is Observed Across CNS-Related MDHI Subscales Through 12 Months

Emotional Issues

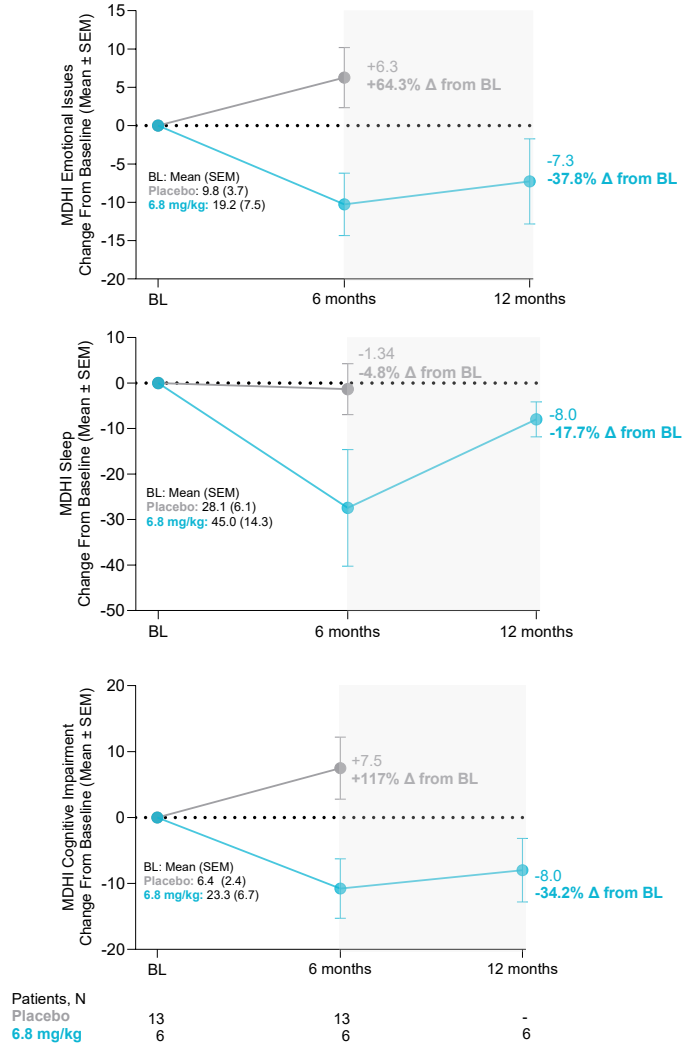
Sleep

Cognitive Impairment

Pooled Dose Group (N=26)



6.8 mg/kg Cohort



Improvement

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.

ACHIEVE

END-DM1[1] Assesses Natural DM1 Progression

END-DM1 is an ongoing, international, prospective DM1 natural history study designed to expand understanding of DM1 disease progression and to establish biomarkers and endpoints appropriate for interventional studies[1,2]

Propensity-Matched Baseline Characteristics

	END-DM1 Matched Cohort[3] Mean (SD)	ACHIEVE Pooled Dose Group Mean (SD)
N	49	26
Age at diagnosis (years)	27.5 (11.9)	23.7 (8.3)
Male (%)	19 (38.8)	14 (53.8)
Age at baseline (years)	36.1 (10.7)	35.6 (8.0)
BMI (kg/m^2)	23.3 (4.8)	22.7 (3.9)
vHOT (sec)	7.7 (4.6)	8.2 (4.4)
QMT Total (%p)	53.9 (17.6)	48.0 (14.3)
QMT hand grip (%p)	43.2 (22.8)	41.5 (16.3)
10MWR (sec)	4.19 (1.91)	3.99 (1.49)

Parameters for propensity matching included age, sex, and measures of strength and function

Dyne Therapeutics is one of multiple companies funding the END-DM1 study, along with the US Food and Drug Administration (FDA) and the Myotonic Dystrophy Foundation (MDF). The Myotonic Dystrophy Clinical Research Network (DMCRN), consisting of END-DM1–participating centers, has extended permission to present these analyses. END-DM1 data transfer July 2026. Note: END-DM1 is an ongoing natural history study, and data comparisons are subject to change. For information on the Pooled Dose Group, see footnotes on slide 2.

[1]ClinicalTrials.gov NCT03981575. https://clinicaltrials.gov/study/NCT03981575. Accessed September 14, 2026. [2]Mul K, et al. *PLoS One*. 2025;20(12):e0331163.[3]The 5xSTS assessment was not included in the original END-DM1 protocol.
%p, percent predicted; 10MWR, 10-Meter Walk/Run; BMI, body mass index; DM1, myotonic dystrophy type 1; QMT, quantitative muscle testing; SD, standard deviation; sec, seconds; vHOT, video hand opening time.