Dyne THERAPEUTICS

DELIVER

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Zeleciment Rostudirsen Significantly Increased Dystrophin Protein Levels and Led to Functional Improvement in Clinical Measures in the DELIVER Trial

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Baseline characteristics well balanced after propensity score weighting: Ambulant z-rostudirsen vs untreated control

ActiLiège Next study:[1] Contemporaneous, ongoing natural history study collecting digital and clinic-based outcome measures

Inclusion criteria:

- Age 4–16 years
- Ambulant
- Corticosteroid treatment for ≥12 weeks
- DMD excluding mutations amenable to exon 44 skipping (ActiLiège)

BL covariates included in propensity score:[2]

- Age, height, NSAA total score, 10MWR velocity, TTR velocity, and SV95C

	Untreated control[5,6] N=60 Mean (SD) or n (%)	z-rostudirsen[6] (REC) N=21[7] Mean (SD) or n (%)
Age (years)	7.4 (2.8)	6.7 (5.4)
Height (cm)	117.5 (11.9)	117.4 (26.0)
Time since first symptom (years)	4.9 (2.8)	4.9 (5.2)
Receiving ongoing corticosteroid treatment	60 (100)	21 (100)
Duration of corticosteroid treatment (years)[3]	2.7 (2.3)	1.7 (3.4)
TTR velocity (rise/sec)[4]	0.22 (0.14)	0.23 (0.17)
10MWR velocity (m/sec)[4]	1.9 (0.6)	1.9 (0.7)
NSAA total score (points)[4]	23.1 (7.3)	21.8 (7.6)
SV95C (m/sec)[4]	1.6 (0.5)	1.6 (0.6)

1. ActiLiège Next study (ClinicalTrials.gov: NCT05982119). 2. 1/PS for z-rostudirsen and 1/(1-PS) for control were used as weight in analysis. 3. Cumulative duration of previous and most recent corticosteroid treatment at the time of randomization. 4. Ambulant participants; out-of-threshold values imputed. 5. Untreated control cohort includes a prespecified analysis of 55 ambulant participants from ActiLiège and five ambulant participants from DELIVER REC placebo. 6. Data shown are after propensity score weighting. 7. Three non-ambulant participants from the z-rostudirsen REC treatment cohort removed from analyses.

10MWR, 10-meter walk/run; BL, baseline; cm, centimeter; DMD, Duchenne muscular dystrophy; m, meters; MAD, multiple ascending dose; NH, natural history; NSAA, North Star Ambulatory Assessment; REC, registrational expansion cohort; SD, standard deviation; sec, second; SV95C, stride velocity 95th centile; TTR, time to rise.

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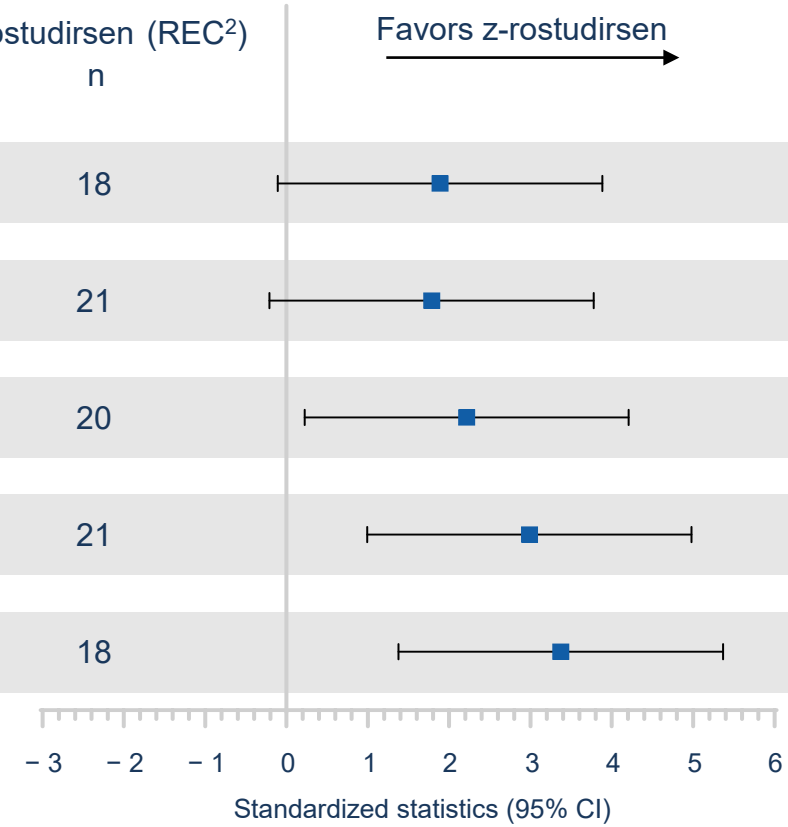
Z-rostudirsen showed functional improvement at 6 months compared to a large untreated cohort across multiple clinical measures

Change from baseline at Month 6

	Untreated control[1] n	Z-rostudirsen (REC[2]) n	LSM change difference (95% CI)	p-value
TTR velocity	56	18	**0.03 rise/sec** (−0.00 to 0.05)	0.0632
10MWR velocity	59	21	**0.11 m/sec** (−0.01 to 0.23)	0.0784
SV95C	60	20	**0.09 m/sec** (0.01 to 0.18)	0.0298
NSAA	59	21	**2.0 points** (0.7 to 3.4)	0.0038
Composite score[3]	56	18	**0.32** (0.13 to 0.50)	0.0012



1. Untreated control cohort includes 55 ambulant participants from ActiLiège who met analysis inclusion criteria and five ambulant participants from DELIVER REC placebo,
2. Ambulant participants treated with z-rostudirsen in the REC. 3. Composite score: a global statistics test derived as the average of SV95C, NSAA, TTR velocity, and 10MWR velocity after standardization for each participant at each visit.
10MWR, 10-meter walk/run; CI, confidence interval; LSM, least-squares mean; m, meters; NSAA, North Star Ambulatory Assessment; REC, registrational expansion cohort; sec, second; SV95C, stride velocity 95th centile; TTR, time to rise.

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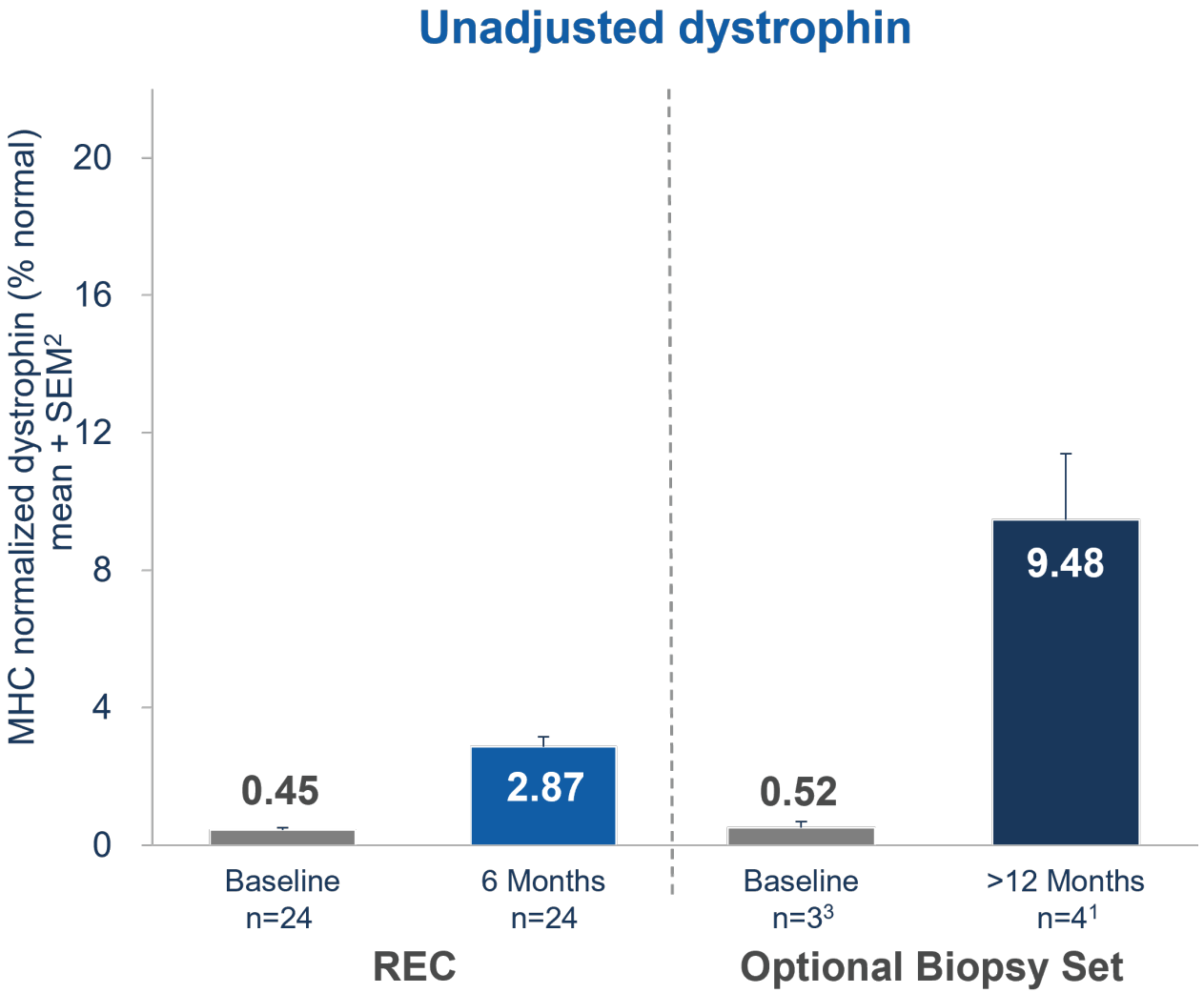
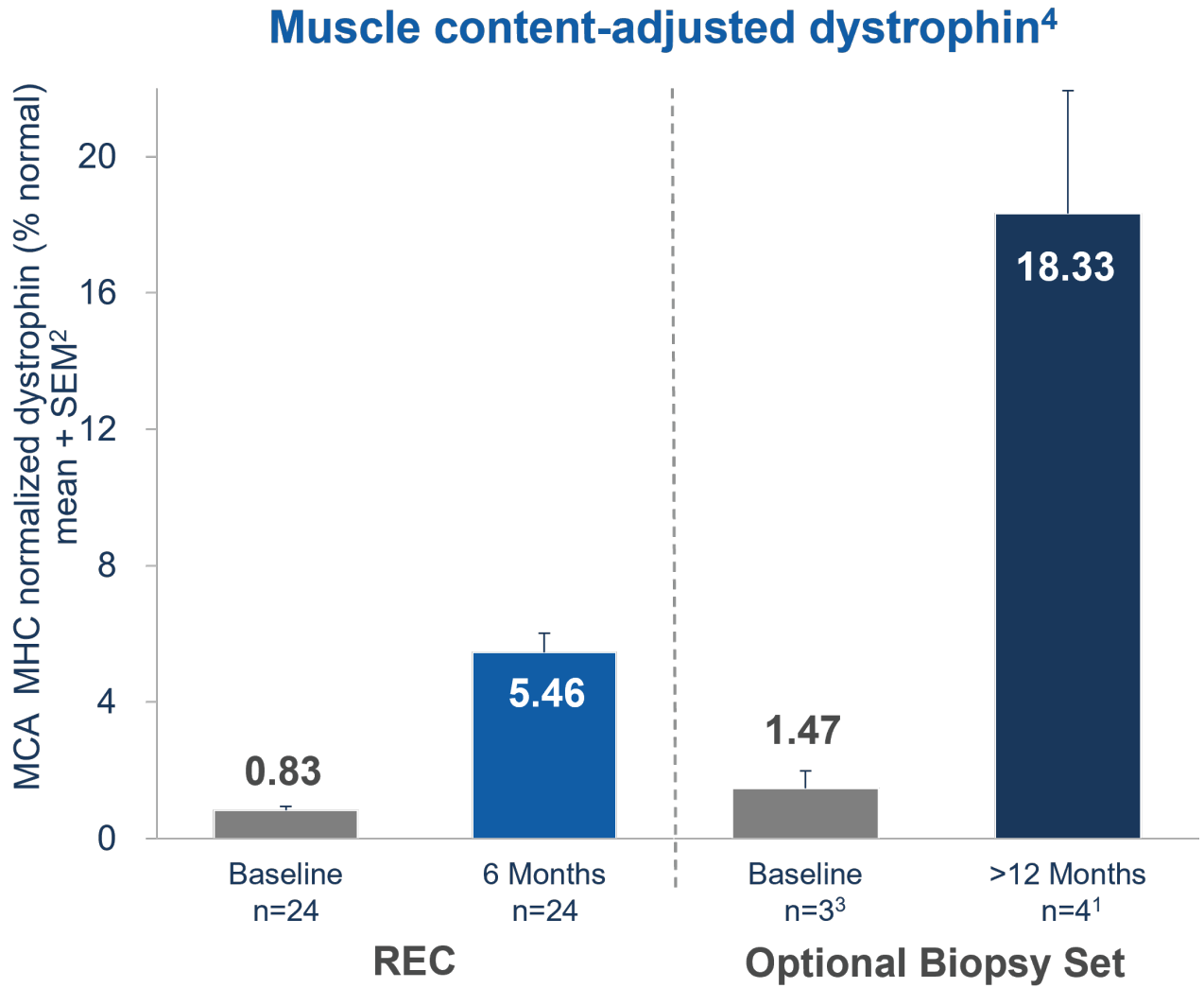
Dystrophin levels observed in optional biopsies from participants treated with 20 mg/kg Q4W z-rostudirsen for >12M[1]





1. >12 month data reflect 4 participants who were dosed with 20 mg/kg Q4W for 67-104 weeks at the time of biopsy. This biopsy was optional per trial protocol in participants who received at least 48 weeks of 20 mg/kg Q4W z-rostudirsen treatment. The sample reported here reflects all optional biopsies collected. 2. Biopsies in REC taken approximately 28 days after most recent dose; optional biopsies taken after at least 48 weeks of 20 mg/kg Q4W z-rostudirsen treatment. 3. Baseline biopsies are pre-treatment biopsies for 3 participants with >12M optional biopsies. One participant with a >12M optional biopsy did not have a baseline biopsy. 4. Muscle content-adjusted dystrophin = MHC normalized dystrophin / % muscle content. 6 months = Week 25 for DELIVER; > 12M = Greater than 48 weeks. REC, registrational expansion cohort; MCA, muscle content-adjusted; MHC, myosin heavy chain; Q4W, every 4 weeks; SEM, standard error of the mean.

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