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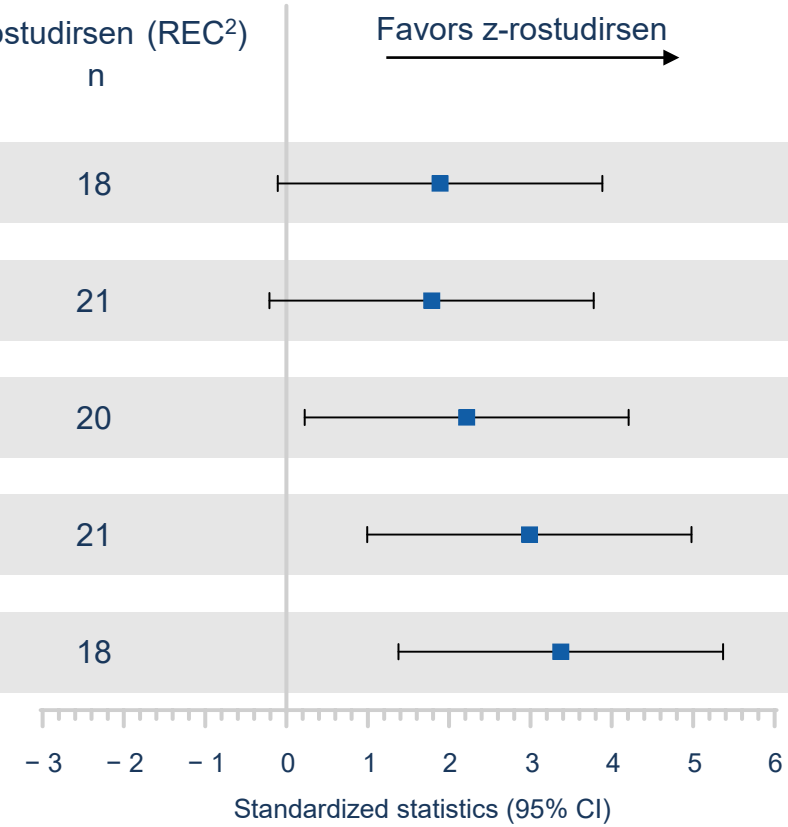
Z-rostudirsen showed functional improvement at 6 months compared to a large untreated cohort across multiple clinical measures

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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