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- ENDEAVOR (NCT04626674) is an ongoing multi-cohort, open-label, Phase 1b clinical trial; Cohort 1 enrolled ambulatory patients aged ≥ 4 to < 8 years, Cohort 2 enrolled ambulatory patients aged ≥ 8 to < 18 years, and Cohort 5A enrolled ambulatory patients aged ≥ 4 to < 9 years¹²
- EMBARK (NCT05096221) is a multinational, randomized, double-blind, placebo-controlled, two-part, Phase 3 clinical trial that enrolled ambulatory patients aged ≥ 4 to < 8 years¹³
- Key additional eligibility criteria included study-specific eligible *DMD* mutations, rAAVrh74 antibody titres $\leq 1:400$, and stable corticosteroid use for ≥ 12 weeks before screening^{12,13}
- Patients aged 8–12 years treated with a single IV infusion of delandistrogene moxeparvovec were pooled from ENDEAVOR Cohort 1 (8 years),* Cohort 2 (8–12 years) and Cohort 5A (8–9 years), and EMBARK Part 2 (8–9 years) (Figure 1)
- Changes in functional outcomes at 1 and 2 years post-infusion were compared with a well-matched EC cohort
 - Functional outcomes included NSAA total score, RFF velocity and 10MWR velocity; velocity-based measures were selected rather than timed function tests because they are more responsive to functional changes in this older age group
- Entry criteria for the EC cohort included age ≥ 8 years, NSAA total score ≥ 17 and ≤ 31 points, TTR ≤ 9.7 seconds, 10MWR ≤ 7.1 seconds, stable dose of oral corticosteroids for ≥ 12 weeks, ≥ 1 post-baseline visit, and 2 years of follow-up
- Propensity-score weighting was based on baseline age, NSAA total score, TTR, 10MWR, height, weight, and body mass index
- MMRM methods account for missing data; *P*-values are nominal

- To characterize the efficacy and safety of delandistrogene moxeparvovec in older ambulatory patients (aged 8–12 years) from ENDEAVOR Cohorts 1, 2, and 5A and EMBARK Part 2 compared with a well-matched propensity-score weighted EC cohort

The diagram illustrates the study timeline from Day -31 to Week 260. It is divided into four main phases: **Screening and baseline** (Day -31 to Day 0), **Part 1 (52 weeks)** (Day 0 to Week 52), **Part 2 (52 weeks)** (Week 52 to Week 104), and **Open-label extension (Study 305; up to 5 years)** (Week 104 to Week 260). Key events include the infusion of Delandistrogene moxeparovoc or placebo at Day 0 and Week 52, and the start of additional corticosteroids at Day -1, continuing through the study.

Mean follow-up was 104.3 weeks in ENDEAVOR Cohorts 1, 2, and 5A, and 101.1 weeks (SD, 12.37) in EMBARK Part 2 (safety data cutoff: October 13, 2025)

- There were no treatment-related deaths or study discontinuations due to AEs
- The mean (SD) time to first occurrence of most TR-TEAEs was 16.0 (55.21) days post-infusion (**Table 2**)

*Inverse probability of treatment weighting. An SMD of 0.25 (absolute value) is generally considered to indicate an adequate balance between the two groups.

- TR-TEAEs were mostly mild or moderate in severity
- Most TR-TEAEs and TR-SAEs appeared within the first 60 days post-infusion and resolved spontaneously or with appropriate intervention ([Table 3](#))
- There were no IMM events observed for the patients included in this analysis

*All 25 patients treated with delandistrogene moxeparvovec and all 99 patients in the EC cohort were included in the analyses. MMRM methods account for missing data in these analyses. All *P*-values are nominal and have not been adjusted for multiple comparisons. †LSMs (of change from baseline) and CIs were standardized in the forest plot by dividing by the SE.

The figure consists of three vertically stacked bar charts, each representing a different clinical outcome. Each chart compares two groups: Delandistrogene moxeparovec (represented by purple bars) and the EC cohort (represented by grey bars). The data is presented for two time points: Year 1 and Year 2. For each time point, the mean change from baseline is shown on the x-axis, with error bars representing the standard error (SE). The difference between the two groups at each time point is indicated by a bracket and a p-value.

Top Chart: NSAA total score at Year 1 and Year 2*

Group	Year 1	Year 2
Delandistrogene moxeparovec	0.52	-1.75
EC cohort	-2.91	-5.07
Δ (P-value)	3.43; $P < 0.0001$	3.32; $P = 0.0022$

Middle Chart: RFF velocity at Year 1 and Year 2*

Group	Year 1	Year 2
Delandistrogene moxeparovec	-0.010	-0.064
EC cohort	-0.075	-0.105
Δ (P-value)	0.065; $P < 0.0001$	0.041; $P = 0.0019$

Bottom Chart: 10MWR velocity at Year 1 and Year 2*

Group	Year 1	Year 2
Delandistrogene moxeparovec	-0.060	-0.311
EC cohort	-0.206	-0.506
Δ (P-value)	0.146; $P = 0.0113$	0.195; $P = 0.0114$

Legend: ■ Delandistrogene moxeparovec, N=25 ■ EC cohort, N=99

*All 25 patients treated with delandistrogene moxeparovec and all 99 patients in the EC cohort were included in the analyses. MMRM methods account for missing data in these analyses. All P-values are nominal and have not been adjusted for multiple comparisons.

- In older ambulatory patients with DMD aged 8–12 years at dosing, treatment with delandistrogene moxeparovec demonstrated durable and clinically meaningful functional benefits over 2 years versus a well-matched EC cohort, supporting a sustained treatment effect and slowing of disease progression, and providing clinically relevant evidence for the efficacy of delandistrogene moxeparovec in this patient population
- Safety in older ambulatory patients was manageable and consistent with the known safety profile of delandistrogene moxeparovec
- These findings help to address an important evidence gap regarding gene therapy for DMD in older ambulatory patients and support the use of delandistrogene moxeparovec in ambulatory patients with DMD aged 8–12 years

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