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New results from Phase 3 EMBARK trial: Data three years after gene therapy treatment

Dear World Duchenne Organization and members,

Following your request for updates on our Duchenne programme, we share that topline results of **three-year data** from individuals treated in Part 1 of the global **Phase 3 EMBARK clinical trial** (NCT05096221) were announced today by our partner Sarepta Therapeutics (press release here).

The EMBARK trial investigated treatment with delandistrogene moxeparvovec (Elevidys™) in ambulatory boys with Duchenne, aged 4-7 years old. Following the trial's completion, participants were eligible to enroll in a follow-up study (EXPEDITION, NCT05967351) to observe long-term efficacy and safety. Consequently, three years of post-gene therapy treatment data are now available from the initial Part 1 treatment group (n=52) of EMBARK participants who are currently in EXPEDITION. Analysis of data for these boys show:

- **Positive Results Across All Endpoints:** Statistically significant and clinically meaningful efficacy was seen across all key functional outcomes - the North Star Ambulatory Assessment (NSAA), Time to Rise (TTR), and 10-meter walk/run (10MWR) - compared to a well-matched, external control group. Results confirm efficacy outcomes seen at the end of year 2.
- **Continued Treatment Effect:** The 3-year data show a slower progression of disease and a widening of the difference between boys treated with delandistrogene moxeparvovec and those in the external control group treated with corticosteroids.
- **Consistent Safety Profile:** No new safety signals were observed. The gene therapy's safety profile in the ambulatory setting remains consistent and manageable, based on data from more than 1,050 ambulatory people treated in clinical trial and real-world settings.

What happens now?

- **Data Sharing:** Full 3-year EMBARK data will be presented at an upcoming medical conference.
- **Regulatory Interactions:** Roche is collaborating with health and reimbursement authorities on the use of delandistrogene moxeparvovec in the ambulatory setting. These data will be shared as part of the growing body of evidence for the long-term efficacy of the gene therapy.
- **Continued Safety Monitoring:** Study participants continue to be followed up for at least five years after treatment via the EXPEDITION study.

We deeply appreciate the partnership and research commitment of the Duchenne community, trial families, and study sites to understanding long-term outcomes with delandistrogene moxeparvovec.

Sincerely on behalf of the Roche Duchenne team,

Mai-Lise Nguyen

Mai-Lise Nguyen
Global Patient Partnership Leader

About the EMBARK study
EMBARK is a global, phase 3, randomised, double-blind, crossover, placebo-controlled study assessing the safety and efficacy of delandistrogene moxeparvovec in ambulatory boys with a confirmed mutation in the *DMD* gene, aged 4-7 years old at the beginning of the trial (n=126). Participants received a single dose of delandistrogene moxeparvovec during either Part 1 or Part 2 of the study.

- In **Part 1**, participants were randomised to receive either delandistrogene moxeparvovec or placebo, with a follow-up period for one year.
    - At the completion of year 1, the trial did not meet its primary endpoint (a statistically significant difference in the NSAA total score one year after treatment). Clinically meaningful and nominal statistically significant improvements were observed in both key pre-specified secondary functional endpoints (TTR and 10MWR).
    - 1-year results of EMBARK were published in Nature Medicine in October 2024.

- In **Part 2**, participants crossed over, meaning that those who were previously treated with placebo in Part 1 received delandistrogene moxeparvovec and participants who were previously treated with delandistrogene moxeparvovec received placebo, with a follow-up period for one year. Due to the crossover study design and subsequent absence of a placebo group, EMBARK data at year 2 were compared with a well-matched, external control group.
    - At the completion of year 2, data from participants who received the gene therapy two years prior showed statistically significant and clinically meaningful improvements across all three key motor function measures of NSAA, TTR and 10MWR, when compared to external control.
    - 2-year results of EMBARK were published in Neurology & Therapy in January 2026.

The EMBARK study is complete, but continues to generate data (e.g., 3-year EMBARK data) thanks to the continued participation of trial families in the EXPEDITION long-term follow-up study.

About delandistrogene moxeparvovec
Delandistrogene moxeparvovec is the first approved disease-modifying gene therapy for Duchenne and is designed to address the underlying cause of the disease. Delandistrogene moxeparvovec is a one-time treatment administered through a single intravenous dose. It is contraindicated in individuals with any deletion in exons 8 and/or 9 in the *DMD* gene.

Delandistrogene moxeparvovec has been studied in the largest gene therapy clinical development program in Duchenne with the longest follow-up (up to six years). To date, more than 1,200 people have been treated with delandistrogene moxeparvovec in clinical and real-world settings, including more than 1,050 ambulatory people. Delandistrogene moxeparvovec is currently approved in nine countries. Roche is in a global collaboration agreement with Sarepta Therapeutics, Inc. to commercialise Elevidys in territories outside the U.S.

For more information or questions, Roche Medical Information can be contacted at medinfo.roche.com.