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Gene therapy research update: New Phase III study to expand access

Dear Duchenne patient organisation leaders,

Following your request for updates on research efforts, we are pleased to share that Roche announced today it will initiate a new, global Phase III study for delandistrogene moxeparvovec (Elevidys™) to expand treatment access for ambulatory people living with Duchenne.

Delandistrogene moxeparvovec is currently approved for ambulatory boys with a confirmed *DMD* mutation in nine countries, based on the Phase III EMBARK study. We understand the urgency of families worldwide to access a drug that treats the underlying cause of Duchenne. The new study will generate additional placebo-controlled data to provide a pathway for regulatory submission with the European Medicines Agency (EMA) and enable regulatory and reimbursement submissions in additional countries. Our goal is to make this disease-modifying therapy accessible to ambulatory boys in Europe and around the world.

New registrational study
The study builds on six years of clinical evidence, including recent 3-year data from the Phase III EMBARK study, and feedback from the EMA and Duchenne community. We are diligently working to set up and start the study.

- **Study summary:** A double-blind study of the efficacy and safety of delandistrogene moxeparvovec compared to placebo over 72 weeks (1.5 years) in approximately 100 early ambulatory boys with Duchenne. Participants initially in the placebo group will be eligible to receive the gene therapy after the primary treatment period.
- **Study details and locations:** As study planning progresses, more information will be shared with you and on clinical trial registries, including eligibility criteria, participating sites and timing for enrolment.

Partnership with the community
The purpose and design of this study was shaped by conversations with the Duchenne research and family community. The study reflects the learnings and experience with delandistrogene moxeparvovec across seven clinical studies and real-world treatment, involving more than 1,050 ambulatory boys. We are incredibly grateful for the community partnership and all organisations and families committed to Duchenne drug development and research efforts.

Sincerely on behalf of the Roche Duchenne team,

Mai-Lise Nguyen

Mai-Lise Nguyen
Global Patient Partnership Leader

About delandistrogene moxeparvovec
Delandistrogene moxeparvovec is a one-time gene therapy administered through a single intravenous dose. It is designed to address the underlying cause of disease by delivering a micro-dystrophin protein to key skeletal, respiratory and cardiac muscles. Delandistrogene moxeparvovec is contraindicated in patients with any deletion in exons 8 and/or 9 in the *DMD* gene.

To date, more than 1200 individuals with DMD (more than 1050 of whom are ambulatory) have been treated across clinical and real-world settings. Delandistrogene moxeparvovec is approved in nine countries: the U.S., Japan, Brazil, Israel, United Arab Emirates, Oman, Bahrain, Qatar and Kuwait (specific country criteria apply).

The programme is under a multi-company partnership: Sarepta Therapeutics is responsible for regulatory approval and commercialisation of delandistrogene moxeparvovec in the U.S., as well as manufacturing. Roche is responsible for regulatory approvals and commercialisation in territories outside the U.S. Commercialisation of delandistrogene moxeparvovec in Japan is through Chugai Pharmaceutical, a member of the Roche Group.

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