



16 April 2026

Gene therapy research update: New Phase III study

Dear UK Duchenne community organisations,

Following your request for updates about Roche's Duchenne muscular dystrophy (DMD) programme, we are writing to inform you of the latest update. Roche announced today that it will initiate a new, global Phase III study for delandistrogene moxeparvovec (Elevidys™) with the intention 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. The new study will generate additional placebo-controlled data to potentially support regulatory submission with the Medicines and Healthcare products Regulatory Agency (MHRA) and the European Medicines Agency (EMA).

New registrational study

The study design builds on 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. We are incredibly grateful for the community partnership and all organisations and families committed to Duchenne drug development and research efforts.

Sincerely,

The Roche UK DMD team
Roche Products Ltd, UK

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.

Delandistrogene moxeparvovec 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.uk@roche.com.