6 November 2026

Gene therapy programme update: Non-ambulatory Phase 3 study & planned ambulatory Phase 3 study

Dear World Duchenne Organization and leaders,

Following your request for timely information on our research efforts for Duchenne muscular dystrophy, we are writing to share updates regarding the development programme for delandistrogene moxeparvovec (also called Elevidys*). Below is information on the global Phase 3 ENVISION study of non-ambulatory and late-ambulatory individuals, as well as the ongoing progress to open a new Phase 3 study for ambulatory children.

Phase 3 ENVISION study data to be analysed
Roche and our partner Sarepta Therapeutics have decided to close recruitment for the ENVISION study (Study 303; NCT05881408) and proceed directly to analysing the blinded data from Part 1 of the study. The study has enrolled a total of 90 participants (64 non-ambulatory and 26 late-ambulatory) in 14 countries: Australia, Belgium, Canada, Germany, Hong Kong, Israel, Italy, Japan, South Korea, Spain, Sweden, Taiwan, UK, and the USA.

Since 2025, the study has been on a global clinical hold (a worldwide pause on a clinical trial) following two tragic, fatal cases of acute liver failure in non-ambulatory people treated with delandistrogene moxeparvovec. Following these cases, our companies initiated research into risk mitigation approaches for non-ambulatory treatment, including studying a preventative immunosuppressive regimen in an ongoing study (cohort 8 of the ENDEAVOR trial; NCT04626674).

Roche and Sarepta have discussed next steps for the ENVISION study. If supported by data from ENDEAVOR cohort 8, resuming enrollment would require administering blinded sirolimus alongside corticosteroids prior to dosing. This would create a mixed population (patients treated with and without sirolimus) materially altering the original study design. Because continuing under a modified design is not practical, the decision was made to close recruitment and proceed to data analysis.

What this means for ENVISION participants
Trial sites are notifying participants to discuss next steps and transition plans; participating families should speak with their study teams. Once the study database is closed, information on individual treatment assignments will be provided. Gene therapy recipients will be invited to transition into the EXPEDITION long-term follow-up study (Study 305; NCT05967351). It is still being evaluated whether participants who received a placebo will have the opportunity to enter an open-label crossover. This possibility depends on ensuring a positive benefit-risk profile supported by ENVISION Part 1 efficacy and ENDEAVOR Cohort 8 safety data, and remains subject to Data Monitoring Committee and regulatory approval to lift the clinical hold.

New Phase 3 study for young ambulatory boys - study set up continues
Roche continues to progress planning and setup for a new, global Phase 3 study for ambulatory boys, where the benefit-risk profile is positive (supported by data from earlier studies). As previously announced, the study intends to generate additional placebo-controlled data for regulatory submission and access in Europe and other countries. The study will enroll approximately 100 early ambulatory boys with Duchenne. When the study is close to opening recruitment, information will be shared on clinical trial registries, including eligibility criteria, participating sites, and timing for enrolment.

With sincere gratitude
We extend our deepest gratitude to the Duchenne community and its continued commitment to research, especially to the boys, young men, and families participating in the ENVISION study, as well as the clinicians and patient organizations supporting them. Your partnership and commitment continues to shape the future of Duchenne research.

Sincerely,

Carlos Mayer

Carlos Mayer
Global Patient Partnership Leader, Neuromuscular Diseases

***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 1500 individuals with DMD (more than 1200 of whom are ambulatory) have been treated across clinical and real-world settings. Delandistrogene moxeparvovec is approved as Elevidys in eight countries: the U.S., Japan, 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.

M-XX-00025517