15 June 2025

Dear members of the World Duchenne Organization,

We are sharing a community letter in response to your request for updates about our Duchenne programme.

On March 18 we shared sad news about a death from acute liver failure following treatment with delandistrogene moxeparvovec. Last week we learned about another tragic death also caused by acute liver failure following treatment with this gene therapy. We are deeply saddened by the loss of these young lives.

Patient safety and well-being are our top priority. These two fatal liver failure cases occurred in non-ambulatory individuals, out of a total of about 140 non-ambulatory individuals treated to date as part of and outside of clinical trials. These events led to a company reassessment of the benefit-risk profile of delandistrogene moxeparvovec which has resulted in immediate action around dosing, as announced today (link to Roche press release).

We recommend halting treatment with delandistrogene moxeparvovec in non-ambulatory individuals with Duchenne regardless of age. Health authorities, investigators and physicians are being informed. This decision does not impact the treatment of ambulatory boys with Duchenne of any age, where the benefit-risk ratio remains positive and unchanged.

Following the first case of fatal liver failure, and as we previously shared, European regulators requested that Roche and Sarepta temporarily halt clinical studies 104 (NCT06241950), 302 (ENVOL, NCT06128564) and 303 (ENVISION Study 303, NCT05310071). You can find additional information about each of these studies below. The temporary halts are still in effect. Outside of Europe, dosing will be paused, effective immediately, for ENVISION.

We remain committed to ongoing monitoring and evaluation of the benefit-risk profile of delandistrogene moxeparvovec for members of the Duchenne community.

We will continue to provide relevant updates to the community. Please let us know if you have any questions.

Sincerely,

Sandra Blum, on behalf of the Roche Global DMD team
Global Patient Partnership Leader

- Study 104 (NCT06241950), a Sarepta-sponsored Phase I open-label, systemic gene delivery study to evaluate the safety, tolerability and expression of Elevidys in association with imlifidase in individuals aged 4 to 9 years with pre-existing antibodies to recombinant adeno-associated virus serotype, rAAVrh74.
- ENVOL (Study 302, NCT06128564), a Roche-sponsored Phase II study evaluating the safety of Elevidys and expression of Elevidys micro-dystrophin protein in young children, including babies and newborns.
- ENVISION (Study 303, NCT05881408), a Sarepta-sponsored global Phase III study investigating the safety and efficacy of Elevidys in participants who are ambulatory (aged 8 to <18 years old) and non-ambulatory (no age limitation).