21st August 2025

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.

As you may have heard, on the 25th July 2025 the European Medicines Agency's (EMA's) Committee for Human Medicinal Products (CHMP) unfortunately provided a negative opinion on the marketing authorisation for delandistrogene moxeparvovec in Duchenne Muscular Dystrophy (DMD) patients aged 3-7 years. While this is disappointing, Roche plans to continue its dialogue with the EMA to explore a potential path forward to make this gene therapy available to individuals living with DMD in the EU.

What does this mean for the UK?

In the UK we were planning on utilising the International Recognition Procedure (IRP), a regulatory route open to applicants that have already received an authorisation for the same product from one of the Medicines Healthcare Regulatory Agency's[1] (MHRA's) specified reference regulators (RRs). The EMA is one of the RRs and a CHMP positive opinion is a RR authorisation needed to apply for a licence in the UK using the IRP pathway. As the CHMP opinion was negative, this pathway is not open to us at this time.

Next steps

In the UK, we too continue to communicate with the MHRA, NICE, SMC, NHSE and other relevant entities. As the path with EMA in the EU becomes clearer, we will then assess the next steps for us in the UK. At this stage, we cannot commit to a date or timeline as the situation is a continuously evolving one.

Please let us know if you have any questions.

Sincerely,

Roberta Pace Balzan,
Medical Affairs Partner
On behalf of the Roche UK DMD team
Roche Products Ltd, UK

[1] *The MHRA is the health authority in the UK and is responsible for issuing Marketing Authorisations for medicines. It is worth noting that in the UK, regulatory approval is only the first step. Further assessments of clinical and cost-effectiveness by the National Institute for Health and Care Excellence (NICE) and the Scottish Medicines Consortium (SMC) must also be undertaken before a medicine is available to patients.*