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Refusal of the marketing authorisation for Elevidys (delandistrogene moxeparvovec)

The European Medicines Agency has recommended the refusal of the marketing authorisation for Elevidys, a medicine intended for the treatment of Duchenne muscular dystrophy.

The Agency issued its opinion on 24 July 2025. The company that applied for authorisation, Roche Registration GmbH, may ask for re-examination of the opinion within 15 days of receiving the opinion.

What is Elevidys and what was it intended to be used for?

Elevidys was developed as a medicine for treating Duchenne muscular dystrophy, a genetic disease that causes increasing weakness and atrophy (wasting) of muscles. It was intended to be used in children aged 3 to 7 years who are able to walk.

Elevidys contains the active substance delandistrogene moxeparvovec and was to be given as a single infusion (drip) into a vein.

Elevidys was designated an 'orphan medicine' (a medicine used in rare diseases) on 28 February 2020 for Duchenne muscular dystrophy. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/en/medicines/human/orphan-designations/eu-3-20-2250.

How does Elevidys work?

Patients with Duchenne muscular dystrophy lack normal dystrophin, a protein found primarily in skeletal muscles (muscles used for movement) and cardiac (heart) muscle cells. Because this protein also helps to protect muscles from injury as muscles contract and relax, in patients with the disease the muscles become progressively weaker and eventually stop working.

The active substance in Elevidys, delandistrogene moxeparvovec, is made of a virus that contains genetic material for producing a truncated (shorter) version of dystrophin. The medicine was designed to introduce the genetic material into skeletal muscles and the heart. A single infusion was intended to enable the patient to produce a shortened form of dystrophin and so slow down progression of the disease.

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What did the company present to support its application?

The company presented data from a main study in 125 children aged between 4 and 7 years with Duchenne muscular dystrophy who were able to walk. They received one infusion of either Elevidys or placebo (a dummy treatment). The main measure of effectiveness was an effect on movement abilities over 12 months, assessed using a standard scale called North Star Ambulatory Assessment (NSAA). The scale ranges from 0 to 34, with higher scores indicating better movement abilities.

What were the main reasons for refusing the marketing authorisation?

The study failed to show that Elevidys had an effect on movement abilities after 12 months. Improvements in NSAA scores were observed both in patients who received Elevidys and in those who received placebo. The difference in the change in NSAA scores between the two groups was 0.65 on a 34-point scale and was not statistically significant, meaning that it may be due to chance. In addition, although many patients treated with Elevidys were shown to produce a shorter form of the dystrophin protein, the levels of dystrophin could not be linked to an improvement in movement abilities.

The company also presented data for a sub-group of patients who seemed to respond better to Elevidys; however, even in this group, effectiveness of treatment was not demonstrated.

The company had applied for a conditional marketing authorisation; as the Agency's opinion was that the benefits of Elevidys have not been demonstrated, it recommended refusing conditional marketing authorisation.

Does this refusal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials. Currently, all clinical trials with Elevidys are temporarily paused; no patients are being treated with Elevidys. Patients who have previously been treated with Elevidys in a clinical trial continue to be monitored.

If you are in a clinical trial and need more information about your treatment, speak with your clinical trial doctor.