

EN
P-000982/2026
Answer given by Mr Várhelyi
on behalf of the European Commission
(19.5.2026)

The EU has a programme to support the development of medicines for rare diseases¹.

At present, one medicinal product has received authorisation in the EU for the treatment of Duchenne muscular dystrophy (DMD), while 34 others are undergoing development and possess active orphan designations within the EU^{2,3}. None of these 34 products has yet reached the stage of assessment by the European Medicines Agency (EMA) for marketing authorisation. The EMA provides monthly updates on the list of medicinal products currently under evaluation⁴.

In certain cases, patients who have a disease with no satisfactory authorised therapies or who cannot enter a clinical trial can receive unauthorised medicines. A 'named-patient basis' treatment' allows - if agreed by the national competent authorities and under the personal responsibility of the healthcare professional - the use of a non-authorised medicine for a specific individual patient, to fulfil special needs⁵.

Member States may also exceptionally allow under a tool referred to as 'compassionate use' the supply of a non-authorised medicine to patients⁶. Compassionate use programmes are generally intended for products under development. They are regulated by national legislation and the way they work varies from country to country⁷.

For medicines that have the potential to address unmet medical needs, the EMA provides early and enhanced advice to medicine developers to optimise development plans and speed up the evaluation so that these medicines can reach patients faster⁸. When the EMA accelerates its assessment, the Commission provides for an expedited decision-making process.

¹ <https://www.ema.europa.eu/en/news/development-medicines-rare-diseases>.

² <https://www.ema.europa.eu/en/human-regulatory-overview/orphan-designation-overview>.

³ https://ec.europa.eu/health/documents/community-register/html/reg_od_act.htm?sort=n.

⁴ <https://www.ema.europa.eu/en/medicines/medicines-human-use-under-evaluation>.

⁵ Article 5(1) of EU Directive 2001/83/EC.

⁶ Article 83 of Regulation (EC) No 726/2004.

⁷ <https://www.ema.europa.eu/en/human-regulatory-overview/research-development/compassionate-use>.

⁸ <https://www.ema.europa.eu/en/human-regulatory-overview/marketing-authorisation/accelerated-assessment>.