

(English version)

**Question for written answer E-000331/26
to the Commission
Alvise Pérez (NI)
(28 January 2026)**

Subject: Unequal access to gene therapies for rare diseases following EMA authorisation

The European Medicines Agency (EMA) has recently approved Vyjuvek, a pioneering topical gene therapy for treating dystrophic epidermolysis bullosa (butterfly skin), a rare, serious and highly incapacitating disease that often affects children.

However, access to the medicine following its authorisation is uneven across Member States, with some already administering it to patients while others have not yet incorporated it into their healthcare systems, unnecessarily prolonging the suffering of those affected.

In this context:

1. Is the Commission aware of the differences in access to Vyjuvek between Member States following its approval by the EMA?
2. Does the Commission consider it compatible with the objectives of EU health policy for a medicinal product authorised at EU level not to be available in certain Member States for extended periods of time, especially in the case of a serious rare disease without therapeutic alternatives?
3. What coordination measures, recommendations or tools can the Commission activate to ensure faster and more equitable access to innovative therapies for rare diseases, especially in paediatric cases of particular medical urgency?

Submitted: 28.1.2026

**Answer given by Mr Várhelyi on behalf of the European Commission
(30 March 2026)**

The European Medicines Agency (EMA) adopted a positive opinion in February 2025 recommending the granting of the marketing authorisation for Vyjuvek ⁽¹⁾.

On 24 April 2025, the Commission granted it an EU-wide marketing authorisation ⁽²⁾. It is the responsibility of the marketing authorisation holder to apply for pricing and reimbursement at Member State level and supply the medicine to patients.

The Commission is aware of the differences in access to medicines and especially orphan medicines at EU level. It recognises the importance of equal access to treatments and has launched initiatives to support it.

The EU pharmaceutical legislation promotes the development of treatments for rare diseases through regulatory incentives and free scientific advice from the EMA ⁽³⁾.

The revision of the legislation aims to accelerate patient access through a priority medicines scheme with enhanced scientific advice, rolling reviews for promising medicines for unmet medical need, faster assessments and new access provisions.

Under the latter, Member States may request marketing authorisation holders to ensure the availability and continuous supply of authorised medicines.

Marketing authorisation holders may be asked to comply with specific procedures, such as pricing applications, procurement procedures, and establishing roll-out plans.

If they fail to make the product available within three years, they risk losing the market protection for that medicine, allowing generics or biosimilars to enter the market earlier in that country.

In addition, the currently discussed Critical Medicines Act ⁽⁴⁾ will make it possible that such pharmaceuticals, as medicinal products of common interest, benefit from procurement collaboration between Member States.

⁽¹⁾ Vyjuvek: <https://www.ema.europa.eu/en/medicines/human/EPAR/vyjuvek>.

⁽²⁾ EU Register of medicinal products: <https://ec.europa.eu/health/documents/community-register/html/h1918.htm>.

⁽³⁾ Orphan medicinal products: https://health.ec.europa.eu/medicinal-products/orphan-medicinal-products_en.

⁽⁴⁾ COM(2025) 102 final.