

(English version)

**Question for written answer E-002494/24
to the Commission
Georgiana Teodorescu (ECR)
(11 November 2024)**

Subject: Withdrawal of marketing authorisation for muscular dystrophy medicine Translarna by the EMA

In September 2023, the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) decided not to renew the marketing authorisation for Translarna (ataluren), a medicine used for treating patients with Duchenne muscular dystrophy, a rare genetic disease.

Representatives of some patients suffering from the disease claim that Translarna had beneficial effects and they say that they are now in a vulnerable situation because they do not have access to this medicine. As I was reading through the communication posted on the EMA's website and dated 18 October 2024, I noticed that while a lack of effectiveness is cited as the reason for the medicine's withdrawal, the potential adverse effects mentioned do not justify this decision, especially as the treatment did work in some cases.

In view of the above, can the Commission answer the following:

1. At what stage is the European Medication Agency's re-authorisation procedure for the medicine given the manufacturer's request in July 2024 for another review to be carried out by the CHMP and when will a decision be issued?
2. If the EMA stands by its decision not to renew the marketing authorisation for Translarna, what alternative treatments does it propose in order to comply with the principle of competitiveness?

Submitted: 11.11.2024

**Question for written answer E-002616/24
to the Commission
Maria Grapini (S&D)
(21 November 2024)**

Subject: Withdrawal of Ataluren for patients with Duchenne muscular dystrophy

Romanians and other Europeans suffering from Duchenne muscular dystrophy and being treated with Ataluren are in a desperate situation. Many EU citizens with Duchenne muscular dystrophy have written to me saying that Ataluren should not be withdrawn from the European medicines market. Despite the fact that the Commission rejected the recommendation by the European Medicines Agency (EMA) to withdraw the medicine from the market and explicitly requested that all measures and evaluations undertaken so far by the EMA/CHMP be cancelled, on 17 October 2024 the EMA/CHMP decided to renew the recommendation to withdraw the medicine from the market.

It should be stressed that Ataluren is the ONLY possible effective and risk-free treatment for such patients with a nonsense genetic mutation, who number over 800 in the European Community. Without this treatment, all those patients are condemned to die!

What will the Commission do to ensure that all the clearly positive aspects of this treatment, along with the Commission's requests, are taken into due consideration by the EMA/CHMP?

Submitted: 21.11.2024

**Joint answer given by Mr Várhelyi on behalf of the European Commission
(19 February 2025)**

In September 2023, the European Medicines Agency (EMA) Committee for Medicinal Products for Human Use (CHMP) recommended to refuse the annual renewal of the conditional marketing authorisation for Translarna. The opinion was confirmed in January 2024 following a re-examination.

In May 2024 the Commission asked the CHMP to consider further data brought to its attention. The CHMP reviewed its assessment and confirmed its recommendation in June 2024 and, after a re-examination, on 17 October 2024.

The benefit-risk was re-assessed based on available data including that from the time of the conditional approval, that generated as per the specific obligations under the conditional approval (two clinical trials), that from the patient registry STRIDE, additional real-world data and from two paediatric studies. These data failed to confirm a positive benefit-risk balance.

In line with the legislation ⁽¹⁾, the Commission shall prepare a draft decision based on the CHMP opinion and submit it to the Standing Committee on Medicinal Products for Human Use.

In a Committee meeting on 12 December 2024, Member States considered the CHMP assessment robust but recognised the unmet medical need of Duchenne muscular dystrophy (DMD) patients and the very specific circumstances of this case.

Member States discussed the need to manage therapeutic continuity for patients already under treatment given the absence of alternative treatments authorised in the EU.

The Commission is considering how these aspects can be integrated into the final Decision which Member States will vote on early in 2025.

There are currently 32 treatments for DMD under development in the EU. EMA is currently reviewing 2 such medicines: givinostat and the gene therapy delandistrogene moxeparvovec.

⁽¹⁾ https://health.ec.europa.eu/medicinal-products/legal-framework-governing-medicinal-products-human-use-eu_en