Question for written answer E-002494/2024
to the Commission
Rule 144
Georgiana Teodorescu (ECR)

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?

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