

(English version)

**Question for written answer E-002280/24
to the Commission
Nicolás González Casares (S&D)
(28 October 2024)**

Subject: Involvement of patients and healthcare professionals in the evaluation, authorisation and monitoring of medicines in the EU

Patient experience plays a key role in the evaluation, authorisation and monitoring of medicines. For this reason, the European Medicines Agency (EMA) enables patients and their carers, including clinical health professionals, to directly participate in these regulatory stages, as well as in the early stages of development of new treatments.

However, in some cases the authorisation of medicines is denied or withdrawn, against the wishes of patients and the healthcare professionals who care for them on a daily basis and who notice significant improvements thanks to their use.

1. To what extent are patients and the healthcare professionals prescribing these medicines really and effectively involved in the decision-making of the EMA and the European Commission?
2. In which situations do other criteria take precedence over the reported improvement of the patients undergoing these treatments?

Submitted: 28.10.2024

**Answer given by Mr Várhelyi on behalf of the European Commission
(13 January 2025)**

Patients and healthcare professionals (HCPs) organisations are involved in many activities of the European Medicines Agency (EMA), including as members of the EMA Management Board and scientific committees, being consulted on disease-specific requests by scientific committees and working parties, taking part in discussions on medicines assessment and in EMA's conferences and workshops ⁽¹⁾, reviewing written information on medicines prepared by EMA, being involved in the preparation of guidelines.

Besides these activities, patients and HCPs contribute to promoting the generation, collection, and use of evidence-based patient experience data for benefit-risk decision-making.

Data from patients' registries are taken into account during the assessment, though they cannot overrule results from placebo-controlled clinical trials, which are designed by default to produce the most valid results.

The Commission's decisions concerning the authorisation of medicines are based on EMA opinions ⁽²⁾, which conclude on the benefit risk balance for a medicine, after having assessed all the evidence available on the efficacy, safety and quality of the medicine ⁽³⁾.

In the Commission proposal for the revision of the pharmaceutical legislation ⁽⁴⁾, representation of patients and HCPs with expertise in all areas, including rare and paediatric diseases, are increased at the committee for medicinal products for human use (CHMP) and at the pharmacovigilance risk assessment committee (PRAC).

Additionally, there would be dedicated working parties that represent patients and HCPs. This proposal builds on the good experience with the participation of patients and healthcare professionals in some of EMA's committees and in EMA's activities overall.

⁽¹⁾ <https://www.ema.europa.eu/en/partners-networks/patients-consumers>

⁽²⁾ In accordance with Article 10(1) of Regulation (EC) 726/2004 <http://data.europa.eu/eli/reg/2004/726/oj>

⁽³⁾ Benefit-risk methodology; European Medicines Agency (EMA) <https://www.ema.europa.eu/en/about-us/what-we-do/regulatory-science-research/benefit-risk-methodology>

⁽⁴⁾ https://health.ec.europa.eu/medicinal-products/pharmaceutical-strategy-europe/reform-eu-pharmaceutical-legislation_en