Call to protect and strengthen meaningful patient involvement in EMA decision-making

in the context of the revision of the EU pharmaceutical legislation

In the context of the revision of EU pharmaceutical legislation, the European Patients' Forum (EPF) and EURORDIS - Rare Diseases Europe (EURORDIS) **urge EU Member State representatives to maintain strong and meaningful patient involvement in the European Medicines Agency's (EMA) regulatory decision-making.**

We are concerned that as part of the current Council negotiations, key provisions from the European Commission's proposal, in particular the inclusion of **four patient representatives with voting rights on the Committee for Medicinal Products for Human Use (CHMP) and in the Pharmacovigilance and Risk Assessment Committee (PRAC), are being discarded or significantly weakened.**

We urge EU member states to uphold the commitment to meaningful patient involvement during the upcoming negotiations. It is critical that patient representation is embedded in the legislation and especially that the voting rights for patient representatives are maintained in Articles 148 and 149 of the Regulation proposal.

With the restructuring of the EMA and its committees that the future revision of the EU pharmaceutical legislation will bring about, **patients' expertise, input, and voting rights should be preserved and strengthened in the regulatory lifecycle of medicines for the interest of public health**.

Removing the voting rights of patient representatives in EMA Committees diminishes both their expertise and the meaningful influence they should have on regulatory decisions that will directly impact the health, quality of life, and needs of the communities they represent. Scrapping this crucial role of patients and reducing them to mere observers will lead to losing the invaluable knowledge and added value they bring to discussions and disregarding the significant time commitment and efforts required to serve on such important EMA committees.

In the past 25 years, increased patient engagement in regulatory decision-making has proven highly beneficial to reinforce trust in the regulatory system, improve health outcomes, and ensure that medicines developed and approved in Europe truly reflect the needs of the patients they are intended to serve.

This revision represents a once-in-a-generation opportunity to make the EU regulatory framework more patient-centred by promoting patient involvement in the regulatory process, improving access to safe, effective, and high-quality medicines, and developing new medicines that better address unmet medical needs.

1. Why it is crucial to uphold patient voting rights in CHMP and PRAC

- **The EU should lead the way in patient involvement and embed it throughout the regulatory process for medicines**. The attractiveness of the European continent should not only be based on its capacity for innovation but also on its rigorous involvement of patients in the regulatory process for medicines and consistently throughout the lifecycle of medicines**.** The EU pharmaceutical legislation represents a unique opportunity to do so and strengthen it further, while also aligning with other European procedures that foster meaningful and impactful patient involvement.

- **The EU cannot afford to lose the invaluable expertise gained over 25 years of patient involvement** in the committees that are set to disappear under the revised legislation. This should be continued in the CHMP and PRAC, and patients' participation in the scientific working groups to be created by the CHMP must be systematic
- **Those outcomes can be achieved thanks to the EMA's strong policy on handling competing interests of scientific committee members and experts**. The EMA's rigorous conflict of interest policy effectively limits the involvement of experts with potential competing interests in the EMA's work, while maintaining its ability to access the best available expertise. A recent comprehensive review has further strengthened the EMA policy to remove any possible doubts as to the objective impartiality of the EMA's assessments[6].

2. What is the added value of patient engagement in regulatory decision-making

Regulatory decisions informed by the patient's perspective are crucial:

- **To enhance the quality of opinions** provided by the Scientific Committees and Working Parties[4] and foster greater transparency and trust in the regulatory process[5].
- To gain a **deeper understanding of the patient's perspective** as patient representatives provide expertise based on their knowledge of specific disease areas, on patients' lived experience, and a practical understanding of new medicines' research and development. Patients have a thorough and often unique understanding of their disease or condition, the benefits and side effects of treatment, and its impact on their daily lives.
- Ultimately, **to strengthen the EU's public health system and lead to better health outcomes for all.** Only through consistent patient engagement throughout products' lifecycle can we ensure that the voice of the patient community is heard and embedded in decisions that will directly impact their health, quality of life, and needs.
- Patient organisations also play a vital role in communication, as they help disseminate information about medicines within their communities and among carers, ensuring that patients receive clear, accessible, and accurate information about their treatment options.

Beyond regulatory processes, meaningful and active involvement is also recognised for instance in the Regulation on Health Technology Assessment (EU) 2021/2282, which fosters the participation of patients as experts in joint work.

3. Patient involvement in EMA's activities: a success story

The European Medicines Agency (EMA) has long recognised the vital role patients play in the regulatory lifecycle of medicines and its engagement with patients and their representatives has deepened over time.

In 2000, patients were first appointed as members of the Committee for Orphan Medicinal Products (COMP), then of the Paediatric Committee (PDCO), the Committee for Advanced Therapies (CAT), and the Pharmacovigilance and Risk Assessment Committee (PRAC). In 2006, the Patient and Consumer Working Party (PCWP) was established as a platform for exchange of information and discussion of issues of common interest between EMA, patient, and consumer representatives. Patients have been contributing for many years to Protocol Assistance, Scientific Advice, Scientific Advisory Groups and more recently, patients were involved in the EMA's Emergency Task Force, which handles regulatory activities in preparation for and during a public-health emergency, and in early dialogues with the CHMP.

This evolution reflects the added value of patients' contributions to enhance regulatory decision-making and promote the research, development, and approval of products that better address patients' and public health needs.

At present, patients and patient organisations are actively involved in:

- **EMA Management Board:** 2 patient organisation representatives.
- **EMA's Scientific Committees**: 3 patient representatives in COMP, 3 (+3 alternates) in PDCO, 2 (+2 alternates) in CAT, and 1 (+1 alternate) in PRAC. These committees discuss various aspects of medicine regulation, such as orphan designation, Paediatric Investigation Plans, safety, efficacy, and risk management. In all committees,

patients' members have full voting rights in line with the committee's terms of reference, placing them on an equal footing with other stakeholders, including national competent authorities. They participate in discussions, contribute to evaluations and guidelines. All committee members:

  - Participate in accordance with the committee's rules of procedure and defined tasks.
  - Maintain confidentiality, declare any conflict of interest, and abide by the EMA code of conduct.
  - Take part in committee decisions and have equal voting capacity. Alternates may attend all committee meetings and contribute to the work and discussions within the committees with equal rights.

- **The Patients' and Consumers' Working Party (PCWP)**, which facilitates dissemination of information about EMA's initiatives across the board and helps integrating the patient perspective as EMA develops and implements new activities.
- **Scientific Advisory Groups (SAGs) and ad-hoc expert groups** convened to answer questions raised during a medicine's assessment, in which patients are invited by the EMA to share their real-life perspective and experience in relation to a particular medicine in their disease area. This can help medicine developers and regulators understand the real-life experience of living with a condition and treatment and identify issues they had not previously considered. A recent EMA study demonstrated that patient contributions led to additional reflection by EMA procedure coordinators in more than half of the cases[2].

In addition, patients provide their unique insights and experiences during the drafting of a **medicine's overview**, when EMA can consult individual patients with experience of the condition to obtain additional information; or in the review of **written information on medicines** prepared by the Agency to ensure they are more accessible and adapted to a lay audience, in particular, patient leaflets and European public assessment reports (EPAR).

The EMA is currently drafting a reflection paper on how to integrate **patient experience data (PED)** throughout the regulatory assessment of medicines. These data are collected to describe patients' experience of their health status, symptoms, disease course, treatment preferences, quality of life and impact of health care. Promoting the inclusion of PED at all stages of medicine development and regulatory decision-making ensures that new medicines address the outcomes and preferences that matter to patients.

In 2021-2022, the EMA launched a pilot of early contacts between patient representatives and the CHMP to inform the early stages of medicines' assessments. Following this pilot and based on the positive experience, the CHMP's engagement methodology was adopted as a core methodology and extended beyond orphan medicines, adding to other existing methods for gathering patient input into medicines development and evaluation.

4. Conclusions and Call to Action

We urge EU institutions to build on EMA's longstanding experience in patient involvement and maintain meaningful patient involvement across the regulatory lifecycle, including patient participation in CHMP and PRAC on an equal footing with other committee members and in scientific working groups.

In addition, further improvements of the legislation could be achieved by:

- Embedding the EMA definition of "patient organisation" in the legislation to formally recognise and strengthen the important role of patients in accordance with high governance standards;
- Consulting patients in drawing up the Union's list of critical shortages and critical medicines;
- Enabling patient representatives to contribute to muti-national assessment teams (MNAT) in the evaluation of marketing authorisation applications;
- Including patient representatives in the Coordination Group for Mutual Recognition and Decentralised Procedures for human medicines (CMDh);
- Providing adequate training and remuneration from the Agency's budget to enable them to make a meaningful contribution, especially when undertaking extensive and very time-consuming tasks.

About European Patients' Forum (EPF)

The European Patients' Forum (EPF) is an independent non-profit, non-governmental umbrella organisation of patient organisations across Europe and across disease areas. Our 80 members include disease-specific patient groups active at EU level and national coalitions of patients. To read about our vision, mission, and strategy, visit: www.eu-patient.eu

About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 1,000 rare disease organisations from 74 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe. By connecting people, families, and rare disease groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies, and services. Find out more: http://www.eurordis.org/