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EPF's recommendations for the ongoing Trilogues on the revision of the EU pharmaceutical legislation

Introduction

The European Patients' Forum (EPF) has been actively engaged in the revision of the EU Pharmaceutical Legislation since the publication of the proposal in 2023, as the revision will have profound implications for the patient community.

This revision is a once in a generation opportunity to update the current regulatory framework for medicinal products and ensure it responds to current public health challenges, from antimicrobial resistance to medicines shortages and crisis preparedness. In addition, it reflects the pressing need for stronger EU action to address growing inequalities in access to medicines across EU member states. It should also provide a strengthened framework for patient engagement in regulatory processes, reflecting over 20 years of EMA engagement and progress in this area. Ultimately, as the revision transforms the regulatory environment in the EU, **it is crucial to ensure it meets the needs of those who rely on safe, effective, and high-quality medicines: the patients.**

As the EU institutions engage in trilateral discussions, **it is crucial for EPF to convey the patient voice and re-state our key priorities. This is particularly relevant as EPF is concerned about some aspects of the Council's position, which weaken critical provisions on patient involvement throughout the product lifecycle.**

As trilogue negotiations progress, EPF stands ready to work constructively with the European Commission, the Council, and the European Parliament to ensure that the final legislative text truly places patients at the heart of the EU's pharmaceutical system.

1 – Ensuring meaningful patient involvement in EMA decision-making

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 respond to patients' needs.

The EU should therefore 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 ecosystem for innovation, but also on its inclusive, evidence-based regulatory decision-making through rigorous involvement of patients**.** The EU pharmaceutical legislation represents a unique opportunity to do so and strengthen it further.

However, the Council's position on the EU pharmaceutical legislation represents a notable regression **in terms of patient involvement compared to the European Commission's proposal and European Parliament's report.** Key issues include the removal of voting rights for patient and healthcare representatives in the Committee for Medicinal Products for Human Use (CHMP), reducing the number of representatives in the Pharmacovigilance Risk Assessment Committee (PRAC), and failing to mandate patient involvement in scientific working parties and patient consultation in guideline development, including for unmet medical needs (UMNs).

Without institutionalising and systematising the patient's perspective in regulatory processes, the legislation misses an opportunity to improve patients' access to medicines that truly address their needs.

EPF calls for the following during the Trilogues:

- ➔ **Include a definition of patient organisations,** based on the EMA definition.
- ➔ **Safeguard adequate representation and patients' voting rights** in the CHMP (Art 148).
- ➔ Maintain the number of patient representatives **in the PRAC as per the Commission's proposal** (Art 149).
- ➔ Mandate **the creation of scientific working groups** to retain current committee members' expertise and maintain separate fora for discussions of specific issues**, especially on paediatric medicinal products** (Art 150).
- ➔ **Guarantee overall a meaningful and strong involvement of patients throughout the regulatory lifecycle of medicines, including involvement in the definition of UMNs**, participating in scientific working groups set up by the CHMP, and serving as co-rapporteurs in the evaluation of marketing authorisation application (Art 152 - Art 162).
- ➔ Ensure that patient representatives actively participating in EMA processes **receive adequate remuneration through the Agency's budget and training opportunities to make meaningful contributions.** This aspect should be duly addressed in the context of the next Multiannual Financial Framework (MFF) 2028–2034.

2 – Ensuring availability and security of supply of medicinal products

Shortages of medicines, regardless of the cause, can have serious consequences for public health. Evidence of medicine shortages in Europe has been growing in recent years, involving both highly specialised and commonly used medicines. Impacts on patients range from deaths, side effects, toxicities, and medical errors due to the dispensing of substitute medicines, resulting in poorer treatment and poorer health outcomes.

EPF strongly supports the focus on shortages in the revision and the Commission's proposed measures, including earlier notification of shortages and withdrawals, mandatory manufacturers' shortage prevention plans for all medicines, and stronger EU coordination mechanisms. EPF also welcomes the European Parliament's amendment requiring consultation of patients and healthcare professionals as EMA develops further guidance on the shortage prevention plans.

However, **additional steps are needed for a comprehensive, multistakeholder approach** to understanding the root causes of shortages and finding appropriate long-term solutions. We therefore welcome the European Parliament's additional provisions related **to the involvement of patients and their representatives in the reporting and management of shortages.** Patient organisations play a key role in liaising with competent authorities, disseminating information about ongoing shortages and alternatives to their communities, and finding practical solutions by engaging with all

stakeholders. **They collect invaluable information about ongoing shortages and their societal impacts** and ensure that public policies respond to patients' needs.

EPF calls for the following during the Trilogues:

- ➔ **Strengthen patient involvement in the reporting and management of shortages**, in particular the consultation of the Patient and Consumer Working Party (PCWP) in drawing up the methodology for the Union's lists of critical shortages and critical medicines, and the establishment of a system for patient organisations to report shortages at national level (Art 117 – Art 121 - Art 131).
- ➔ **Clear, user-friendly public platforms** for patients to access shortage updates, including information about causes, start and end dates, alternatives, and ongoing mitigating measures (Art 121).
- ➔ Allow member states to adopt **additional measures to protect patients against shortages as appropriate** while avoiding fragmentation, promoting uptake of best practices, and ensuring national measures do not create any negative impacts in other member states (Art 134).
- ➔ **Strengthen solidarity** between member states by supporting the Voluntary Solidarity Mechanism as suggested by the European Parliament (Art 132).
- ➔ **Ensure that the pharmaceutical legislation and the Critical Medicines Act (CMA) do not overlap or conflict** as several (common) provisions might also be included in the CMA.

3 – Improving access to medicines that better address patients' needs

A. Establishing a mechanism that truly improves patient access

EPF sees this review as a unique opportunity to improve patients' access to the medicines they need. Unacceptable inequalities persist across the EU, as new medicines become available with significant delay in some member states compared to others. While many issues can only be resolved at national level, we welcome increased focus on access at EU level.

Achieving a true single market that benefits patients requires stronger coordination. During the trilogues, it will be crucial to agree on effective mechanisms **that can be implemented in practice and improve the situation for patients on the ground**.

- ➔ *Of note, the EU pharmaceutical legislation must work in conjunction with other EU laws to effectively improve patient access to medicines, including the Health Technology Assessment Regulation, the Transparency Directive, etc. Other mechanisms included in new and future pieces of legislation, such as the Critical Medicines Act (CMA), should also be explored and complement coordinated action at EU level to improve patient access. This is the case for joint procurement and improved cross border access to treatments when treatments at home are not possible.*

EPF supports the mechanism proposed by the European Parliament and the Council, introducing the obligation for companies to file for pricing and reimbursement at the request of member states. However, for this mechanism to be successfully implemented, **clear and enforceable deadlines are**

needed, which should effectively accelerate equitable patient access to medicines across the EU. In addition, well-defined obligations accompanied by sanctions in cases of non-compliance must be put in place. Further guidance is needed to ensure that this mechanism is fit for purpose.

We also strongly support the European Parliament proposal **to develop indicators to measure access to medicines in the EU** to improve transparency and inform future policy measures.

B. Adapting to innovation

We further support provisions that aim to accelerate the EMA's processes while safeguarding the highest standards for regulatory assessments. In particular, we continue to support adaptive pathways, including conditional marketing authorisations and rolling reviews, as they have the potential to accelerate patient access to potentially life-saving treatments. At the same time, ensuring strong EMA oversight and close monitoring are essential for patient safety.

In addition, **we support the introduction of regulatory sandboxes** to provide the EMA with the right tools and flexibility to assess new and emerging products and ensure they are safe and effective for patients.

However, it is essential that in all cases, the EMA mandates and reviews the **collection of post-market data** to inform the regulatory assessment throughout the product lifecycle.

C. Better incentives to ensure new medicines better address patients' and public health needs

EPF calls on the co-legislators to ensure a fair balance between incentivising R&D of new medicines that address patients' needs while ensuring timely patient access. Excessively long exclusivity periods can delay the entry of generics and biosimilars, thereby limiting competition and ultimately hindering patient access. In addition, increased conditionality of incentives can help steer pharmaceutical research towards serving public health priorities. This includes rewarding the development of medicines that address unmet patients' needs and those backed by significant EU-based R&D, which can also improve patient access to clinical trials within Europe. However, this should not lead to an extension of existing exclusivity periods.

Regarding antimicrobial resistance, the regulatory framework should create the conditions for faster approval of new antimicrobials. It should further promote proportionate incentives that de-link sales revenues from sales volumes and do not negatively affect patients' access to other medicines. **We support the proposal of the European Parliament which introduced milestone payment and market entry reward schemes for 'priority' antimicrobials**. These schemes provide early-stage financial support for the achievement of specific R&D milestones before market authorisation. They are complemented by a voluntary joint procurement scheme based on a subscription model to encourage investment in antimicrobial development.

Regarding the **transferable exclusivity voucher (TEV)**, its effectiveness and cost to national healthcare systems raise questions, as well as its impacts on patient access to other products. However, since the European Parliament and the Council have retained the concept of TEVs, it is likely that this mechanism will feature in the final text. We therefore ask for additional requirements and limitations on the granting, use, and transfer of TEVs, such as ensuring supply of the final products, improving predictability, restricting the accumulation of multiple extensions, clarifying eligibility criteria according to public health needs, as well as including an "anti-blockbuster" criterion. Without such

safeguards, TEVs risk placing a heavy financial burden on health systems and unfairly delaying access to generics and biosimilars.

D. Stimulate research and development of medicines in areas of unmet medical need (UMN), as defined by patients

By only considering morbidity and mortality as indicators of UMN, **the EU would ignore other important life-changing indicators for which significant therapeutic improvements or even breakthroughs may be needed from a patient perspective**.

The pharmaceutical legislation should outline a framework for defining UMN, which should be inclusive and cover the appropriateness of existing treatments or impacts on quality of life. EPF supports therefore the inclusion in the legislation of a broad definition, as suggested by the European Parliament. As a second step, assessing whether a medicine fulfils a UMN will require **case-by-case assessments and additional guidance by the EMA** to specify key criteria. In view of their unique expertise and experience of their condition and needs, we call for meaningful patient involvement in this process, including patient participation in decision making, as well as consultation of patient representatives in each specific disease area.

In this context, EPF recalls the importance of including patient experience data (PED), beyond "traditional" clinical endpoints, at all stages of medicines' development and regulatory decision-making. PED are data collected to describe patients' experience of their health status, symptoms, disease course, treatment preferences, quality of life and impact of health care. There is consensus among regulatory authorities and other healthcare stakeholders on the value of PED to inform the benefit/risk assessment and drive medicines' development. PED can play a crucial role in assessing whether and how a product addresses patients' unmet needs.

➔ *Of note, the EMA is currently developing a reflection paper on Patient Experience Data, to be published in 2026.*

EPF calls for the following during the Trilogues:

- ➔ The access mechanism to be adopted must be assessed **based on its real-world impact on improving patient access in every EU member state, without creating further disparities** (Chapter IV).
- ➔ If the obligation to file for pricing and reimbursement, at the request of member states, is selected as the access mechanism, **ensure the establishment of clear and enforceable deadlines, accompanied by well-defined and timely sanctions** (Chapter IV).
- ➔ **Establish a robust and transparent conciliation mechanism** to resolve access-related disputes swiftly and fairly, in the interest of patients (Chapter IV).
- ➔ **Support the EP proposal for the EU Access to Medicines Notification System**, to enhance transparency, oversight, and accountability across the Union, with adequate safeguards in place related to timing, transparency, and strict limits on derogations (Chapter IV).
- ➔ Support the introduction of **regulatory sandboxes while guaranteeing patient safety and close monitoring** (Art 115).

- ➔ **Strike the right balance between incentivising R&D of new products with real added value and ensuring access to new therapies for all patients across the EU** through selected modular incentives and proportionate exclusivity periods (Articles 80 – 85).
- ➔ Promote proportionate incentives for AMR **that de-link sales revenues from sales volumes** and do not negatively affect patients' access to other medicines (Art 39).
- ➔ If the concept of TEVs is retained, **adopt strong safeguards** (e.g. ensuring supply of the final products, predictability, restricting the accumulation of multiple extensions, including an "anti-blockbuster" criterion) to avoid a heavy financial burden on healthcare systems (Chapter III).
- ➔ **The definition of UMN should be inclusive and include important life-changing indicators from a patient perspective**, such as the appropriateness of existing treatments or impacts on quality of life. Patients must be involved in the development of the definition (Art 83).
- ➔ **Pursue EMA's efforts to systematise the collection and use of Patient Experience Data** to support the development of medicines that truly meet patients' needs.

4 – Improving patients' access to objective and reliable information

The revision should also move towards more objective, reliable, relevant and user-friendly information on medicines. For that, it is essential **that paper leaflets remain available, alongside electronic formats**, as not all patients have access to the Internet or are digitally literate.

Information on medicines **should be better** tailored to the needs of patients and include a **key information section** in the package leaflet, information on how to dispose of the product and, where possible, its environmental footprint to support choice of less polluting alternatives when available.

The involvement of patients in the drafting of materials intended for them (e.g. AMR Awareness Card) is crucial to ensure that these meet their needs.

It is also essential to strengthen EMA's and other stakeholders' ability to fight against misinformation and disinformation. Patient organisations play a key role in disseminating objective and reliable information to their communities and should be supported in this regard, including by clarifying misinterpretations on the provisions related to advertising that affect POs' dissemination activities.

EPF calls for the following during the Trilogues:

- ➔ **Paper leaflets should remain available, alongside electronic formats**, as not all patients have access to the Internet or are digitally literate (Art 63 – Art 64).
- ➔ Information on medicines should be better tailored to the needs of patients **and include a key information section in the package leaflet** (Art 64).
- ➔ **The involvement of patients in the drafting of materials** intended for them is crucial to ensure that these meet their needs (Art 64 – Art 69).

➔ **Clarify that patients' organisations can disseminate factual and balanced information on authorised medicines to patients**, in compliance with the applicable legislation (Art 175).

FOR FURTHER READING

- Call to protect and strengthen meaningful patient involvement in EMA decision-making in the context of the revision of the EU pharmaceutical legislation
- EPF statement on informing and empowering patients through accessible and clear information on medicines
- EPF proposal for a patient-centred framework for defining Unmet Medical Needs
- EPF statement on ensuring consistent and coherent application of EU rules on medicines' advertising
- EPF advocacy toolkit on the revision of the EU Pharmaceutical Legislation
- EPF reaction to the European Parliament position on the revision of the EU Pharmaceutical Legislation
- EPF statement on advancing patients' access to medicines in the pharmaceutical legislation
- EPF statement on supporting patient-centred innovation: the value of patient experience data

ABOUT 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 82 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

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