



## Call to Action Ensuring the Voice of Patients in the EU Pharmaceutical Legislation

For the first time in 20 years, the European Union (EU) is looking to comprehensively overhaul its regulatory framework for pharmaceuticals. While the overall regulatory framework has steadily improved and underpinned strong progress in medical treatment, there are many areas in need of improvement to improve European patients' access to safe and affordable medicinal products and innovation, especially in areas where the medical needs of patients are not met.

As the voice of primary immunodeficiencies (PIDs) across the world, IPOPI would like to outline the following points of concern that should be addressed in the pharmaceutical package, to ensure that it results in a truly patient-centred legislation.

1. Ensuring faster and sustainable access to new treatments & advanced therapy medicinal products (ATMPs)
2. Tackling and preventing medicine shortages
3. Ensuring a smooth interplay between existing & future legislation
4. Meaningfully increase patient representation and involvement in decision-making processes

We call on European policymakers from the 2019-2024 mandate – and those who will enter into function after the 2024 European elections – to ensure the following points guide political discussions over the next months:

### Access to medicines

*Patient access to medicines is not a straightforward process. Securing marketing authorisation for any given medicine is only one step of the process, the healthcare system's sustainability and resilience, the manufacturer's decision to market the medicine in a given country as well as the coverage and/or reimbursement of a medicine within national or regional schemes are factors that must be taken into consideration.*

1. Support the reduced timelines suggested for the European Medicines Agency's (EMA) approval of medicines.
2. Work towards ensuring equity of access to new and innovative medicines – with special attention to antibiotics – with marketing authorisation for all European patients irrespective of their Member State or the rarity of their condition.
3. Recognise that highly specialised knowledge, infrastructure and follow-up are needed for complex therapies and that a pragmatic approach may be needed when it comes to the interesting proposal to launch new therapies simultaneously across all Member States. Equity of access could instead be achieved by utilising already existing infrastructures such as European Reference Networks (ERNs).

4. Seek pathways to overcome systemic challenges linked to incentives that lead to limited availability, delayed access to medicines and inadequate research and development of orphan medicines as well as potential market failures.
5. Implement coordinated measures across national and European levels to address the rising costs of medications to promote transparency in pricing and negotiation processes and explore innovative reimbursement models.
6. Empower the ERNs to develop EU recommendations on newborn screening for treatable diseases that can lead to early diagnosis and treatment of life-threatening diseases through gene therapy.

### **Medicines shortages**

*Medicine shortages are becoming increasingly concerning for PID patients. The Commission's approach to overcoming supply and availability challenges is welcome given that they recognise the problems patients face in the EU, but should be further complemented with additional measures.*

1. Develop prevention plans, early warning systems and contingency plans that can support the EU's ability to overcome potential medicine shortages in a timely fashion and across all diseases with specific regard for therapies, such as immunoglobulin replacement therapies, where the creation of contingency stocks may negatively impact under-pressure markets.
2. Take steps to efficiently assess and address the multifactorial causes of medicine shortages which can be directly caused by the scarcity of their source material. The production of immunoglobulin replacement therapies, for instance, is only possible thanks to the availability of plasma and blood donations.
3. Address systemic shortages and supply chain challenges that lead to medicine shortages through the coordinated efforts of European and national regulatory authorities, healthcare professionals, patient organisations, and industry stakeholders.
4. Ensure relevant information on medicines shortages is up-to-date, digitally accessible and translatable.
5. Clarify definitions proposed in the pharmaceutical package, such as 'high unmet needs' and 'critical shortage'.

### **Ensuring a smooth interplay between existing and future legislation**

*The pharmaceutical package is one of the primary legislative efforts envisaged by the European health policy ecosystem. Harmonising provisions across legislative dossiers and initiatives already in force – or that are being negotiated – is highly desirable.*

1. Empower ERNs to become a platform that can support access to advanced therapies if the patient's country lacks the expertise, technology or infrastructure for the delivery of the therapy and/or the follow-up that is required.
2. Ensure that patients have clarity on diagnostics and treatment options they can access abroad by further exploring and defining interplay between the Directive on the application of

patients' rights in cross-border healthcare (2011/24) and the Regulation on the coordination of social security systems (883/2004)).

3. Optimise the EU's public health ecosystem by ensuring regulatory clarity for medicines derived from substances of human origin, such as immunoglobulin replacement therapies impacted by the future Regulation on standards of quality and safety for substances of human origin intended for human application (SoHO) and the future pharmaceutical package.
4. Work towards the development of an EU Action Plan on Rare Diseases that provides a holistic vision and comprehensive approach that supports people living with rare diseases.

### **Involvement of patients**

*The EU's positive approach to patient participation has led to increased rates of representation across many European institutions such as ERNS and the European Medicines Agency (EMA) Committee for Human Medicines (CHMP). European decision-makers must ensure the participation of patient representatives during upcoming political discussions and public forums so they may bring their insights and experiences to the table.*

1. Facilitate the involvement of patient organisations at the European Medicines Agency (EMA) level including the EMA working parties.
2. Include patient representatives in the development of the List of Critical Medicines, the definition of "unmet medical need" and upcoming processes such as the setting up of a regulatory sandbox.
3. Develop formalised pathways, either via the EMA or national competent authorities, for patient organisations to easily report a shortage of a given medicinal product marketed in a Member State.
4. Engage with patients to ensure that no provisions negatively impact certain patients or lead to the de-prioritisation of certain diseases or therapies.