

PID FORUM

NAVIGATING THE COMPLEXITIES OF THE PHARMACEUTICAL LEGISLATION

An IPOPI Event

Hosted by MEPs Billy Kelleher (Renew),
Cyrus Engerer (S&D) & Tomislav Sokol (EPP)



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14.30 - 16.00

ASP1E3



European Parliament

EVENT REPORT



On 7 June 2023, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised its latest PID Forum, entitled *“Navigating the Complexities of the Pharmaceutical Legislation”*. The event took place in the European Parliament and was co-hosted by Members of the European Parliament (MEPs) Billy Kelleher (Renew, Ireland), Cyrus Engerer (S&D, Malta) and Tomislav Sokol (EPP, Croatia) and was moderated by Martine Pergent, President of IPOPI.

Opening the Forum, **Martine Pergent** thanked the co-hosting MEPs, as well as IPOPI’s sponsors, for their support in helping organise the event. She noted that the European Commission’s overhaul of the pharmaceutical framework would include changes that could be of vital importance to PID patients, but also acknowledged the scale of the challenges that needed to be overcome. Finally, she expressed her hope that the event would foster a better understanding of the Commission’s proposals and provide different perspectives that would shape IPOPI’s Call to Action.

Welcome Address



MEP Billy Kelleher began his speech by welcoming the PID Forum’s attendees. He emphasised the significance of the recently published pharmaceutical strategy and its potential consequences for innovation and access to medicines. In his contribution, Mr Kelleher highlighted that the existing gaps in patient access, high prices for treatments and medicine shortages within the European Union (EU) demonstrated the urgent need for legislative and funding frameworks that ensure a timely provision of transformative therapies, especially for rare diseases.

Citing the importance of a competitive yet profitable pharmaceutical industry, he also acknowledged the delicate balance between affordability and the financial viability of

pharmaceutical companies and called for more rapid regulatory pathways and incentivisation for companies to develop medicines for rare diseases.

MEP Cyrus Engerer likewise expressed his delight in co-hosting the PID Forum. His contribution focused on his deep concern about the availability of medicines, particularly critical ones, for all EU citizens. He emphasised the need for access to innovative medicines, especially for patients with rare diseases, and highlighted the challenges and inequities faced by different EU Member States.



Mr Engerer cited statistics that showed low availability of approved pharmaceutical products in Malta, his home country, and noted the country's lack of access to two-thirds of the 168 new medical products approved in the EU since 2018. He underlined the need for a proactive approach to prevent shortages and market withdrawals through early warnings and prevention plans. He also called for improved coordination with the European Medicines Agency (EMA) and patient involvement in decision-making processes. Mr Engerer concluded his statement by highlighting the importance of safeguarding the health and well-being of citizens, irrespective of where they come from or their disease type.



MEP Tomislav Sokol noted his delight in taking part in a patient-driven event as one of the key rapporteurs on the pharmaceutical regulation.

He emphasised his interest in amplifying the concerns of patient communities and his interest in IPOPI's upcoming Call to Action. More importantly, he stressed the need to address the availability of medicines and bring healthcare investments and innovation to Europe. He echoed Mr Engerer's sentiments and noted the existence of a "postcode lottery" when it comes to accessing key therapies and care. However, he acknowledged that the proposed legislation would not be a silver bullet but a tool that could be used to provide incentives for the development of innovative products, particularly for rare diseases.

Mr Sokol also called for amending the outdated Directive relating to the transparency of measures regulating the prices of medicinal products for human use and their inclusion in the scope of national health insurance systems (89/105) to ensure more transparent pricing and reimbursement procedures, and called on the European Commission to develop a proposal on a dual transparency directive to help resolve this matter.

Setting The Scene



Leire Solis, Health Policy and Advocacy Senior Manager at IPOPI, provided an overview of the key areas in which the proposed legislation could play a big role, and analysed the potential impact of the legislation as proposed by the European Commission on the treatment and care of patients with

PIDs. Ms Solis also discussed a number of issues affecting the patient community that the pharmaceutical legislation could seek to overcome:

- Ensuring quicker access to new treatments and Advanced Therapy Medicinal Products (ATMPs) in a sustained manner;
- Tackling and preventing shortages through the notification system in a way that respects the specificities of the therapies;
- Smooth interplay between the European Reference Networks and existing legislation, such as the Directive on the application of patients' rights in cross-border healthcare (2011/24), the Regulation on the coordination of social security systems (883/2004), and of future legislation such as the proposal for a Regulation on standards of quality and safety for substances of human origin intended for human application (SoHO);
- Increased patient representation and meaningful involvement at the European Medicines Agency level and with the development of the List of Critical Medicines and the definition of "unmet medical need".

What The Pharmaceutical Legislation Brings to Patients

Julia Schmitz, Policy Officer, DG SANTE (Unit D1 Medicines: policy, authorisation and monitoring) at the European Commission, gave an overview of the political objectives and key elements of the proposed reform of the pharmaceutical legislation. The Commission proposals focus in particular on overcoming issues linked to access, availability and affordability of medicines, while ensuring a competitive EU regulatory framework that promotes innovation.

Of particular relevance to patients, Ms Schmitz noted more targeted incentives for medicines addressing unmet medical needs, measures to accelerate marketing authorisations, enhance patient access to and availability of medicines, patient

representation within the EMA's Committee for Medicinal Products for Human Use (CHMP) and other opportunities for patient involvement in strengthened dialogues between public authorities responsible for medicines.

Panel Discussion: The Pharmaceutical Legislation's Impact on Rare Disease and PID Communities

Following her contribution, Ms Schmitz was joined by three other speakers for a panel discussion on the pharmaceutical legislation's impact on rare disease and PID communities.



Luisa Antunes, Policy Analyst, Directorate-General for Parliamentary Research Services at the European Parliament (EPRS), highlighted the different types of studies conducted in her department. She mentioned two studies requested by MEPs from the Panel for the Future of Science and Technology (STOA) that went beyond analysing current

proposals and instead examined what was lacking in the current research landscape. These studies identified a fragmented research landscape in the EU and a disconnect between industry and public interests. To address these issues, the proposal suggested the establishment of a new European public health body, separate from existing agencies, that would define research priorities, particularly in the areas of unmet medical needs. The body would also aim to ensure strategic autonomy, transparency, and fair pricing policies, with potential collaboration between the public and private sectors.

Otilia Stanga, President of the Romanian PID patient organisation (ARPID), shared her personal experience with the severe immunoglobulin shortage in Romania in 2017-2018, which resulted in her daughter and other patients not receiving life-saving treatment for over a year. Discussing the complications that arose from this situation, such as social isolation and educational setbacks, Ms Stanga highlighted the financial and logistical challenges they faced when seeking to obtain treatment



from neighbouring countries. Ms Stanga cited her ongoing concerns about the potential for future shortages and access to basic antibiotics like penicillin in Romania. As such, she called for improvements in the European Union healthcare system to ensure that patients, particularly those with limited resources or smaller voices, no longer face such challenges

and can access the treatments they need chronically without constant fear and uncertainty of not being able to access them.



Juan García-Burgos, Co-Chair of the Patients' and Consumers' Working Party at the European Medicines Agency (EMA), highlighted the success of patient engagement since the EMA's establishment in 1995. He acknowledged the positive transformation brought about by patients' involvement in regulatory discussions and their contribution to the assessment and development of

medicines. He noted that patient engagement has been valuable in areas, such as unmet medical needs and safety monitoring, and that online engagement during the COVID-19 pandemic, was critical in shaping regulatory procedures for new vaccines and technologies. He added that transparency and trust are essential in a sound regulatory system and that patient dialogue needs to extend beyond the European level, with patients playing a crucial role in communicating outcomes to national authorities.

Julia Schmitz noted that tackling complex issues like addressing unmet medical needs, access and availability of medicines requires a combination of legislative and non-legislative measures, both at European and national level. Ms Schmitz highlighted the significance of the Commission legislative proposals, in particular the more targeted regulatory protection incentives for addressing unmet medical needs and promoting patient access. Regulatory support like scientific advice to medicine developers will also be strengthened. Ms Schmitz also mentioned that the proposed reform includes possibilities for the involvement of patients and other stakeholders in discussions between public authorities related to unmet medical needs.

Open Floor Discussion



The panel discussion was followed by an open floor discussion during which members of the audience could ask questions to the event's speakers. **Carlo Martuscelli**, journalist from Politico, asked about the connection between a country's Gross Domestic Product (GDP) and the subsequent availability of medicines. In response, Julia Schmitz explained how the proposed reform

of the pharmaceutical legislation, with its regulatory protection incentive for market launch in all EU Member States, can improve patient access across the EU.

Luisa Antunes added that two studies from STOA propose shifting incentives towards access and reducing the influence of money in politics by moving away from industry-driven incentives and prioritising the public interest. By creating a new structure, they believe it is possible to solve problems of access, transparency and the interchangeability of different assets.

Representatives of national patient organisations, **David Jiménez Gonzalez**, representative of the Spanish PID patient organisation (AEDIP), **José Verstegen** and **Janine Smith**, representing the Dutch PID patient organisation (SAS) and **Kersti Urbala**, representative of the Estonian PID patient organisation underscored the importance of the pharmaceutical legislation in ensuring the development of innovative medicines, while also underscoring issues facing PID patients.

Several members of the audience asked questions that could not be answered due to a lack of time:

- **Jose Drabwell (IPOPI)** asked about the Substances of Human Origin (SoHo) regulation, noting that it was unclear whether companies would need to register with national authorities or on an EU-wide platform.
- **Aoife Gallagher (Eli Lilly)** raised concerns regarding the limited definition of unmet medical needs provided by the European Commission. She further pointed out that while the accelerated pathway of approval by the EMA is a step forward, it still lags behind the FDA's process, prompting her to inquire about the possibility of further accelerating it.
- **Sofia Ramos Paiva (TRANSFORM Alliance)**, asked how EU institutions can finance the participation of patients during the lifecycle of medicines while also providing patients with educational opportunities to fully take part in the process. She also asked if the Commission can incentivise early-multi stakeholder dialogue to identify which Member States possess the necessary infrastructure (e.g. Centres of Excellence) to deliver Advanced Therapy Medicinal Products (ATMPs) to patients, and if it could support the development of such infrastructure in other Member States across the EU.
- **Eveline Kozubovska (The Plasma Protein Therapeutics Association)** emphasised the importance of aligning the pharmaceutical legislation with other regulations, particularly regarding inspections of both active pharmaceutical ingredient (API) manufacturing sites and collection sites.

Call To Action: Ensuring the Voice of Patients in the EU Pharmaceutical Legislation



Following the conclusion of the open floor discussion, Leire Solis thanked all participants and speakers for their contributions throughout the event. She noted that IPOPI had gathered substantial input during the discussions and interventions and would be developing a Call to Action on the pharmaceutical legislation focussing on the most relevant aspects for patients with PIDs.

From ensuring equal access to medicine and alignment with legislation such as the European Health Data Space (EHDS) and the SoHo proposal, to ensuring the EU's ability to overcome potential medicine shortages and the participation of patient representatives during political discussions, she expressed her hope that the Call to Action would be taken into account during political negotiations and she called on European policymakers to ensure the voice of patients is taken into account during the upcoming regulatory process.

Closing remarks

In his closing statement, MEP Cyrus Engerer thanked participants for providing their insights on the pharmaceutical legislation. He noted the important role that the European Parliament plays in listening to and representing the voices of EU citizens. He emphasised the importance of listening to those affected by the legislation and aligning it with other regulatory proposals, such as the European Health Data Space and the SoHo regulation. He also acknowledged the complexity of the topic and the role of parliament in the upcoming process. He assured his support for IPOPI – and expressed his interest in amplifying their Call to Action – over the coming months and underscored the importance of achieving a patient-centred healthcare system. Mr Engerer also noted that through active participation, patient representatives can help shape policies that prioritise patient well-being and improve access to essential medicines for all EU citizens.

Closing the PID Forum, Martine Pergent thanked attendees, including panellists, members, sponsors, and represented bodies for their participation. She emphasised the importance of collective efforts and stakeholder collaboration and how IPOPI hoped to build on the momentum generated during the event and translate these discussions into actionable initiatives through the Call to Action.