

Event Report



PID Forum – Exploring the impact of medicine shortages on PID patients

5 November 2024

On 5 November 2024, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised a PID Forum titled “*Exploring the Impact of medicine shortages on PID Patients*”. The event took place in the European Parliament in Brussels and was co-hosted by Members of the European Parliament (MEPs) **Vytenis Andriukaitis** (S&D, Lithuania) and **Tomislav Sokol** (EPP, Croatia).

Moderator Johan Prévot, IPOPI’s executive director, opened the event by highlighting medicine shortages as an all-too-common reality in Europe that have a significant impact on patients with primary immunodeficiencies (PIDs). He underscored the need for EU-level action and welcomed initiatives such as the proposed Critical Medicines Act (CMA), the European Rare Disease Action Plan, and the formation of the Critical Medicines Alliance. Mr Prévot stated that long-term solutions were critical to improve health outcomes for people living with rare diseases.

Welcome address

In his opening statement, **MEP Tomislav Sokol** (EPP, Croatia) underlined his positive long-term collaboration with IPOPI. He emphasised the need for both MEPs and stakeholders to mobilise in order to keep health high on the political EU agenda, noting that his political group is in favour of setting up a fully-fledged Health Committee at the European Parliament, so as to better focus and discuss on health-related policies. Turning to rare diseases, Mr Sokol highlighted the importance of accessing cross-border treatments, particularly for smaller EU Member States, like Croatia and Lithuania, where access to rare disease treatments can be limited and hindered by the lack of reimbursement or prior authorisation. He outlined the EU’s efforts to improve treatment availability, referencing his role as shadow rapporteur in the revision of the pharmaceutical legislation and also mentioning future EU initiatives such as the Critical Medicines Alliance and the strategies for cardiovascular and rare diseases.

MEP Vytenis Andriukaitis (S&D, Lithuania) expressed regret that health often receives insufficient political attention, stressing that the issue requires consolidation and a collective effort from all political groups. Mr Andriukaitis agreed with Mr Sokol on the importance of cross-border treatments and the creation of an independent Health Committee but also lamented the lack of cooperation



between EU Member States to facilitate such treatments. He emphasised the need for pan-European healthcare mechanisms to ensure the delivery of critical medicines and treatments to patients in need. Mr Andriukaitis expressed his support for treaty changes that would give the EU greater flexibility to act on health-related matters.

Setting the scene

Leire Solis, health policy and advocacy senior manager at IPOPI introduced the audience to IPOPI's work and reviewed the findings of a survey the organisation conducted on medicine shortages among its national member organisations. The survey, which gathered input from 21 European national patient organisations between 15 July and 6 September 2024, provided some important insights into the impact of medicine shortages on European PID patients.

It revealed that 9 out of 21 national organisations had experienced shortages in the past 12 months with intravenous immunoglobulin (IVIg) drugs being the most likely to be affected by shortages, followed by subcutaneous immunoglobulin (SCIg) therapy and facilitated subcutaneous immunoglobulin (fSCIg) therapy. The survey also found that PID patients experiencing shortages can result in irregular and sporadic treatment, increased distress and anxiety, and higher infection rates. Ms Solis stated that the reasons for immunoglobulin (Ig) shortages are multifaceted but that contributing factors include tender systems not being fit for purpose, supply chain problems, and a greater demand for Ig than planned. In most cases, patient organisations were not able to point to one reason for the shortage. One particular point of concern that Ms Solis highlighted as a necessary area of improvement was the fact that only 4 of the 21 PID patient organisations were contacted by public authorities or regulators regarding the Ig shortages.



How to overcome medicine shortages in the EU?

Klaus Kruttwig, medicines and medical devices shortages specialist at the European Medicines Agency (EMA), provided an overview of shortage management in the EU. He highlighted the role of the European Medicines Regulatory Network (EMRN) in improving the availability of authorised medicines and emphasised that regulatory authorities - within and outside Europe - are increasingly working together to prevent shortages and to limit their impact whenever they occur. The work of the Medicine Shortages Steering Group (MSSG) and the Medicine Shortages Single Point of Contact



(SPOC) Working Party in monitoring shortages and issuing recommendations to EU Member States, industry and the European Commission. Mr Kruttwig concluded his presentation by stressing the need to focus on preventive actions when managing medicine supply to avoid a disruption of patient care. He underscored the importance of the revised EU pharmaceutical legislation and the Communication on Addressing Medicine Shortages in the EU, including the Critical Medicines Act to strengthen the security of supply for critical medicines and prevent shortages.

Panel: the impact of medicine shortages on rare disease patients

Otilia Stanga, president of the Romanian Association for Patients with Primary Immunodeficiencies (ARPID) and IPOPI treasurer, shared her experiences with medicine shortages in Romania, pointing to a lack of understanding among policymakers regarding the specificities of Ig therapies and of primary immunodeficiencies. She explained how individuals with PIDs have had to seek treatment abroad during shortages of antibiotics and highlighted the challenges her daughter, who also suffers from a PID, faced in accessing support in other EU Member States. Ms Stanga welcomed initiatives such as the European Shortages Monitoring Platform (ESMP), expressing hope that these efforts will facilitate easier access to necessary patient treatments.



Lúcia Mamede, Medical Affairs Project Manager at IPOPI, shared insights on behalf of **Dr Nizar Mahlaoui**, a paediatrician at Necker-Enfants Malades University Hospital and chair of IPOPI Medical Advisory Panel, regarding the impact of medical shortages in hospitals. She highlighted the frequent shortages of plasma-derived medicinal products (PDMPs), crucial therapies for over 300,000 patients across the EU. She also emphasised the challenges posed to the quality of life and treatment adherence when paediatric formulations of therapies were in shortage and children could not cope with the adult formulations.



Karlien Claes, Director of education at the International Nursing Group for Immunodeficiencies (INGID) and Nurse specialist for children with PIDs and rheumatic diseases at the University Hospital Ghent (Belgium), shared her experiences supporting patients with immunodeficiencies in Belgium. She highlighted the substantial challenges that medicine shortages, particularly Ig therapies shortages, posed to both patients and nurses. Drawing on her personal experience during an Ig shortage in 2021-2022, nurse Claes emphasised the impact on patients and healthcare professionals alike. Patients identified the shortage themselves, leaving healthcare providers uninformed and unable to take timely measures or adequately inform patients, pharmacists and home care services. In the acute phase, patient communication demands became so overwhelming that essential nursing tasks were sidelined, increasing the overall workload due to shortage

management. Medication adherence was also disrupted: with temporary dose adjustments and extended intervals, patient adherence became inconsistent, making it challenging to re-establish



correct therapeutic routines once the shortage was resolved. In addition, newly diagnosed patients could not be started on the necessary Ig therapy. Ms Claes emphasised the urgent need for improved support and communication from authorities to manage such situations more effectively in the future and, ideally, to prevent them altogether.

The panel's final speaker, **Klaus Kruttwig**, provided further information on the shortages catalogue entry for normal human immunoglobulins, published in June 2024. He informed the audience that the EMA updates the catalogue regularly to ensure the information is kept up to date to ensure that healthcare professionals and patients are informed. He also highlighted that a reference to the national shortage register is included.

Open Floor Discussion

The panel discussion was followed by an open floor discussion which saw several patient representatives from across Europe take the floor to highlight their concerns and challenges. **Janine Smith**, board member of the Dutch PID patient organisation, Stichting voor Afweerstoornissen (SAS), emphasised the importance of securing sufficient reserves of medicines, noting that shortages lead to anxiety, stress, and insecurity among patients.

David Jiménez González, board member of the Spanish PID patient organisation, Asociación Española de Déficits Inmunitarios Primarios (AEDIP), highlighted the need for tailored treatments, sharing several cases of PIDs, including his personal experience.

Meanwhile, **Kersti Urbala**, president of the Estonian Patient Society for Primary Immunodeficiencies, emphasised the importance of advocating for continued access for patients who rely on antibiotic treatments in a chronic manner.

Maria Linnea Löfving Sonesson, president of the Swedish Primary Immunodeficiency Organization (PIO), expressed satisfaction with the steps taken by the Swedish Medicines Agency



to meet with PIO and discuss how to improve and ensure access to critical medicines, like Igs.

Finally, **Ellen Desmet**, board member of the Belgian Patient Organisation for Primary Immunodeficiencies (BePOPI), described how in 2021, PID patients were prioritised in access to Ig therapies but only if it was received in registered hospitals, which caused

many challenges for the daily lives of some patients which lived far away from the hospitals. This was especially the case in the event of Ig shortages, where patients would be required to travel several additional times to get the needed therapies. Ms Desmet stated that BePOPI supports a European solution to prevent and mitigate future shortages.

Closing Statements

In his closing remarks, **MEP Vytenis Andriukaitis** (S&D, Lithuania) reiterated his call to amend the Lisbon Treaty to make healthcare a European competence, thereby providing stronger EU-level instruments in the fight against rare diseases. He added that they embody the added value of a European approach to public health. Finally, he thanked all participants for having joined an important discussion and emphasised his commitment to supporting rare disease patient organisations during the 2024-2029 mandate.

Closing the PID Forum, **Mr Prévot** thanked attendees, panellists, patient representatives, sponsors, and EU representatives for their participation. He noted that the event was an ideal setting to establish the current state of play on medicine shortages in Europe through the lens of PID patients and highlighted the need to shift from a reactive management approach to a proactive one to ensure a reliable supply of medicines.