Event report
PID Forum: Shaping the Future of Research: Patients as Equal Partners

MEP Romana Jerković and MEP Nicolás González Casares with Martine Pergent and Johan Prévot of IPOPI

On 14 May 2025, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised a PID Forum titled 'Shaping the Future of Research: Patients as Equal Partners'. The event took place in Brussels (Belgium) in the European Parliament. It was co-hosted by Members of the European Parliament (**MEP) Romana Jerković** (S&D, Croatia) and **Nicolás González Casares** (S&D, Spain).

Martine Pergent, President of IPOPI, moderated the Forum which aimed at discussing how EU research could benefit from the inclusion and recognition of patient organisations as equal partners. Martine began by recognising the significant progress made in supporting rare disease research and collaboration and emphasised how crucial it is to continue supporting patient organisations with specific expertise in current and future research funding schemes.

Welcome address

In her opening statement, **MEP Romana Jerković** (S&D, Croatia), a medical doctor and researcher, highlighted the importance of patients in advancing health research. She argued that patients should be treated as equal partners in health research and that the EU should design health policy with them in mind. This is because patient experiences are crucial in understanding rare diseases and advancing research, but also because they often become experts in navigating complex care pathways. MEP Jerković informed the audience that she would advocate for more EU budget for health and an EU Action plan for Rare Diseases. She expressed that she was looking forward to building on the results of the SANT Committee's public consultation on rare diseases.

The PID Forum's second co-host, **MEP Nicolás González Casares** (S&D, Spain), also drew from his experience as a healthcare professional. He emphasised that millions of Europeans with unique situations are affected by rare diseases, and their participation is crucial to gathering data to advance rare disease research. While he echoed MEP Jerković's call for an expanded EU budget for health and an EU Action Plan on Rare Diseases, MEP González Casares also highlighted the value of newborn screening as a tool to overcome rare diseases. He concluded by expressing his concerns about the challenges that may occur due to possible U.S. tariffs on pharmaceutical products.

Setting the scene

Johan Prévot, Executive Director of IPOPI, shared insights on primary immunodeficiencies (PIDs), a fast-evolving field that requires constant scientific research to better understand conditions, but also diagnosis, treatments and care for the patients. He explained that studies in the PID field and other rare diseases are crucial for identifying cellular and molecular pathways associated with the development of other more common diseases. Johan also highlighted how supranational federations representing specific rare diseases, such as IPOPI, are key players in research and innovative projects. For instance, they ensure that research is fit for purpose and can effectively tackle current unmet needs. He called for the EU to enable a meaningful participation of patient organisations and appropriately recognise the contributions of different types of rare disease patient federations.

EU Rare Disease Research: ERDERA perspective

Dr. Daria Julkowska, Coordinator at the European Rare Diseases Research Alliance (ERDERA), provided insights into the current European rare diseases research ecosystem and programmes launched to support rare disease patients. During her overview of ERDERA's work and structure, she emphasised the importance of patient involvement in rare disease research and how the organisation is involving patients and patient organisations.

Despite the positive work achieved, Dr. Julkowska listed a number of challenges that still need to be addressed such as overlapping goals, the potential dispersion of scattered funds and the need for bi-directional (research-patient) training. Crucially, she emphasised the need to review the definition of a research partner to ensure that patient organisations are considered at the same level as other research partners. As such, she proposed to review the eligibility criteria and definitions used by national funding bodies.

Panel discussion – Perspectives in patient involvement in EU research

Prof. Dr. Frank Staal, Coordinator of H2020 programme RECOMB, working at Leiden University Medical Center in the Netherlands, spoke about the value of collaborating with patient organisations for EU funded research projects and spoke about his work with IPOPI on a series of research projects in the field of PIDs.
He emphasised that patient organisations excelled at co-creating clinical trials, disseminating information and creating awareness among the patient community, which is essential in improving diagnostic rates and patient involvement in research and clinical trials. Prof. Dr. Staal concluded by highlighting five cured patients – in part thanks to IPOPI's involvement – and advocated for introducing a paediatric voucher programme in the EU that is similar to the soon to be discontinued U.S. programme.

Prof. Dr. Filomeen Haerynck, Paediatric Immunologist at UZ Gent in Belgium, commented on the growing impact of patient organisations' involvement in research over the years. At a patient level, she emphasised the fundamental importance of their involvement in translational research as a motivational factor for health researchers, but also in having them share information and become co-creators of the research. To emphasise the importance of patient organisations, she shared an example of a patient survey launched by IPOPI in 40 countries during the COVID-19 pandemic. Faced with patient concerns about the safety of the COVID-19 vaccine, IPOPI took the initiative of raising awareness of this issue with clinical researchers who understood that there was a need to develop guidelines ensuring PID patients receive the vaccine after seeing the results of the survey.

Johan Prévot, Executive Director at IPOPI, made a case for involving patient organisations even those partially funded by industry players. He explained that the current eligibility criteria used in determining eligibility for access to funds for health research excludes federations of patient organisations, such as IPOPI. He proposed that organisations like ERDERA and the European Commission use the eligibility criteria of the European Medicines Agency (EMA) for assessing the profile of patient organisations. Johan also raised the issue of how to define 'independence', as this is a highly subjective term that can easily be abused. He concluded that this issue had been flagged at the European Reference Network (ERN) level as well and that they recognise that organisations like IPOPI hold essential expertise in clinical trial design, patient recruitment and dissemination and should be involved in research programmes.

Dr Christina Kyriakopoulou, Scientific Policy Officer of Health Research programmes, at the European Directorate-General for Research and Innovation, DG RTD.D.2, discussed the work of DG Research on rare diseases research under Horizon Europe and its Health Cluster. She

spoke of the European Commission's long-standing commitment to rare disease research, with more than €5 billion allocated in the past 25 years. She posited that the EU's strengthened focus on competitiveness should lead to more 'outside-the-box' and disruptive ideas for collaboration and innovation. Despite tremendous work happening at the national level, she also emphasised that no European country could solve the issue of rare diseases alone and that's why the European Commission via its Horizon Europe programme set-up strategic partnerships such as ERDERA. She explained that patients are key players as they are experts in their disease and provide a real-world experience to enable co-design of more effective clinical studies. She echoed the comments of other panellists and concluded that thinking out of the box, flexibility and cooperation between researchers, innovators and patient communities were crucial to deliver breakthrough research on rare diseases.

Dr. Daria Julkowska, coordinator at ERDERA, acknowledged that the independence and involvement of patient organisations in research had to be discussed. She suggested that redefining the criteria surrounding patient involvement would require a gathering of all types of patient organisations, from umbrella groups to ultra-rare diseases before making a decision. She explained that the definition of research partners was a particularly challenging one to overcome as many institutions do not recognise dissemination and other patient organisation activities as research activities. She discussed different options of how ERDERA could boost the involvement of patient organisations as she recognised that they were essential to advancing rare disease research and responded that the EMA definition could be used as a reference point for patient involvement. She committed to engaging with IPOPI and the wider patient organisation community to solve these issues.

Open Floor Discussion

The panel discussion was followed by an open floor discussion which saw several panellists further discuss the topics laid out during the panel discussion. Following this, patient representatives from across Europe take the floor to highlight their concerns and challenges.

David Jiménez González, board member of the Spanish PID patient organisation AEDIP, agreed with the panellists that patients were a net positive to rare disease research outcomes. He emphasised that patients want to help and that the PID Forum is a clear sign of their power and expertise to advance health research through collaborative discussions.

Janine Bastiaans-Smith, board member of the Dutch PID patient organisation SAS, called on researchers and policymakers to ensure patient input is involved from the start. She emphasised the strong relationships created between patient organisations and researchers from ERN RITA (Rare Immunodeficiency, Autoinflammatory and Autoimmune Diseases

Network), but that they should not be punished for relying on transparent industry funding that helps them maintain a sustainable organisation.

Anne-Sophie Henry-Eude, board member of the French PID patient organisation IRIS, welcomed the statements made by the various speakers of the PID Forum and re-emphasised why IPOPI's presence is vital for patient training, awareness and knowledge-building. She emphasised the importance of an equitable distribution of funding and that data collection facilitated by patient organisations is a fundamental part of health research.

Closing statements

In her closing remarks, Martine Pergent expressed her gratitude to all participants for their contributions to the discussion on **shaping the future of research from a patient's perspective**. She welcomed the consensus generated during the discussions and thanked all panellists for sharing their insights and expertise on rare disease research. She highlighted the openness of ERDERA and the European Commission in discussing patients' involvement in future EU health research. Concluding the event, she stated that funding ceilings and defining what is a conflict of interest should be at the heart of future discussions on patient research.