

IPOPI 19th PID Forum

Harnessing the potential of digitalisation for patients with primary immunodeficiencies

Policy recommendations

A “Europe fit for the digital age” is one of the six political priorities of the European Commission for the 2019-2024 mandate, and this has led digitalisation of healthcare to be increasingly discussed by policymakers. The overarching eHealth Strategy has arisen from this discussion and the International Patient Organisation for Primary Immunodeficiencies (IPOPI) welcomes this initiative, **as patients with primary immunodeficiencies (PIDs) belong among those who would benefit from a digitalised healthcare system.**

The nature of PIDs calls for novel tools and methods to facilitate everyday activities linked to patients’ diagnosis and treatment, bolstering epidemiological knowledge as well as increased research and development capabilities. Digitalisation is truly present at every step of PID care – from daily symptom tracking to global research initiatives.

However, there are currently a multitude of limitations and restrictions preventing the potential of digitalisation from being fully harnessed for PID and other rare disease patients. **The following recommendations provide a list of goals and guidelines for policymakers to focus on in the upcoming digital legislations.**

These recommendations are the result of the 19th PID Forum, organised by IPOPI on 2 December 2021. The event brought together experts, politicians and patients to discuss the impact of digitalisation on PID care. Experts present included Prof. Mikko Seppänen,¹ Prof. Isabelle Meyts,² Mr. Friedolin Strauss,³ Prof. Nicholas Rider,⁴ Prof. Mark Little⁵ and Dr. Nizar Mahlaoui.⁶

In the weeks following the Forum, the incoming French Presidency of the European Union Council unveiled its priorities. Among them was the aim to “transform Europe into a digital power.” This further exemplifies the need to highlight the benefits of digitalisation for PID patients and how these benefits can be effectively supported through policy.



Ensuring EU digital and health policy supports the needs of patients with Primary Immunodeficiencies (PIDs)

IPOPI, in collaboration with the experts present at the 19th PID Forum, calls on the EU to:

1. Fund research and development so that PIDs can benefit from digital technology outcomes

Research and development of technologies, such as e-Health symptom tracking tools and health related artificial intelligence applications, can highly benefit patients with rare diseases and more specifically the PID community. It can help ensure timely diagnosis and avoid unnecessary, repeated procedures, as well as help prevent or even predict risks in the development of a disease or the outcome of a treatment. Digital technology can also be used to identify the most appropriate personalised treatment pathways that exist for patients' conditions. Tailoring existing technologies to PIDs would allow for example to detect crucial patterns necessary for accurate machine learning decision-making regarding diagnosis, treatment and management of PIDs, and encourage routine data collection and usage.

The European Commission is called to increase funding into the research focusing on the **development and implementation** of easy and secure digital technologies that are tailored to the needs and specificities of PID patients, including:

Utilising digital technologies and health-related AI applications:

- to enhance and expedite diagnosis based on PID-specific datasets
- to develop ad hoc treatment development and management practices based on PID-specific expressions and systems analysis
- to incorporate analysis of data sets from electronic medical records into routine so as to predict condition evolution, provide the best management and prevent major risks.

2. Increase the ability for cross-country knowledge and case sharing of PID digital tools and data to broaden the scope of EU-led research possibilities for PID patients (including in cooperation with countries outside the EU)

Knowledge and experience sharing is crucial for rare diseases, including patients with PID, due to the relatively limited amounts of cases and research available. When knowledge is shared, it can improve patient outcomes by decreasing time to diagnosis, providing additional perspectives, allowing for greater influx of new ideas and treatment options as well as connecting patients and providers to identify best practices. It also shows them as public health issues where solutions exist and where national health systems have a role to play. The difficulty in sharing knowledge, particularly through digital data, exacerbates the discrepancies between countries and the disproportionately poor outcomes often faced by PID patients. Data and experience must be shared to effectively enhance cooperation between health care providers, innovate and find solutions. Therefore, the right balance between the right to privacy, and the right to access treatments and conduct research that leads to a better understanding of PIDs and potential innovative treatments, must be carefully considered.



EU Institutions should continue to encourage and support knowledge sharing and collaboration of PID digital tools and data by:

- Expanding support **to facilitate data and case sharing, and to make** the European Reference Network Clinical Patient Monitoring System fit for purpose as an international registry,
- Expanding support to conduct research into rare disease **data sharing mechanisms and practices**, particularly supporting the interoperability of PID data and other data from conditions with low prevalence.
- Optimising such research by **lifting barriers to data collection and sharing by adapting the General Data Protection Regulation** to have special exemptions and rules for handling rare disease and PID data in healthcare and research (such as indefinite storage and acceptance of broad consent, which would prevent the deletion of crucial, rare data regarding PIDs as well as increase the ability to conduct research and analysis on PID data). This also applies to EU cooperation with other countries including the developing world.

3. Ensure PID patient-centred decision-making

Including patients representatives in the core of the legislative process is necessary to both build trust as well as optimal solutions targeted towards patients. This is particularly important for digital health data, which is both very private and often difficult to understand. Involving patients in the legislative discussions will ensure the community's needs are well met and that they support, understand and agree with the ways in which they can utilise digital tools – and the ways in which digital tools utilise their data.

EU institutions must ensure PID patients play a central role in health digitalisation by:

- Ensuring that patients are heard and that their perspectives are included in any committee aimed at developing diagnosis, treatment or research purposes through digitalisation.
- Encouraging national healthcare systems to allow **PID patients to easily access their own health data** and share it in a standard format. PID patients may often be in positions where practitioners do not know or recognise their case and require as much background information as possible.
- Ensure that digital tools are not biased and **account for the risks and limitations** specific to PID data, such as relatively small cohorts and unique characteristics.