



IPOPI 19th PID Forum Report **Digitalisation of healthcare:** Improving medical care for PID patients



19th PID Forum

Digitalisation of healthcare: Improving medical care for PID patients

2nd December 2021
14:00-15:30 CET

Co-hosted by MEPs Carlos Zorrinho (S&D, Portugal)
and Marina Kaljurand (S&D, Estonia)

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Introduction

On 2nd December 2021, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised its 19th PID Forum titled “**Digitalisation of healthcare: Improving medical care for PID patients**”. The online event was co-hosted by Members of the European Parliament (MEPs) **Carlos Zorrinho (S&D, Portugal)** and **Marina Kaljurand (S&D, Estonia)**, and moderated by **Mr Johan Prevot**, Executive Director, **together with Ms Martine Pergent**, President of the board of IPOPI.

During the event, participants analysed the current status of digitalisation of the healthcare for primary immunodeficiency (PID) patients in the EU and how the digital initiatives of the European Commission provide an opportunity to make treatments more accessible to patients, improve their everyday life with digital tools and how digitalisation could help both medical providers and research and innovation in the field of rare diseases. The expert speakers presented their views on the policy developments and provided recommendations on what needs to be done at EU level in order to tackle the existing policy challenges. **The outcome of these discussions will be used in the development of policy recommendations.**

The event was marked by MEPs Carlos Zorrinho (S&D, Portugal) and Marina Kaljurand (S&D, Estonia) confirming their political support to the PID community when it comes to promoting their needs regarding digitalisation.

Welcome & Opening Remarks



Opening the 19th PID Forum, MEP Carlos Zorrinho (S&D, Portugal) welcomed all speakers and participants and expressed his delight to be able to continue to support the PID patient community. Mr Zorrinho remarked that when he co-hosted the 6th PID Forum in 2015, which launched the [principles of care for primary immunodeficiencies](#), he especially endorsed the third principle: the need for trans-national and trans-institutional collaboration in scientific research. In the

field of healthcare, digitalisation has a big role to play in international collaboration, especially facing the reality of limited patient populations and data – which is the case for primary immunodeficiencies. He pointed out that the increasing discussions at EU level on digitalisation come rather timely and that the Europe fit for the digital age initiative and the upcoming EU Health Data Space proposal, which are expected in 2022, would be cornerstones in leveraging the benefits of digitalisation for rare disease patients.

He pointed out that digitalisation is not just about the large-scale collaborations – it's also about everyday life. Digital tools and health services can improve the daily life of patients through things like remote consultations, better communication with physicians, and easier symptom tracking.

Further, Mr Zorrinho linked the PID Principles of Care from 2015 to his following point: primary immune deficiencies are recognised as rare conditions and data is scarce, although many countries across the EU and worldwide have implemented registries for PIDs. He said he is aware that on one side exchange of information can lead to interdisciplinary research on rare diseases but on the other, there are several barriers – such as a lack of interoperability and fragmentation of health data. He concluded by stating that he recognises it is now the moment when the policymakers need to hear from the patients and healthcare professionals about the concrete benefits of digitalisation and understand how this can be reflected in concrete policies.

Mr Johan Prevot, moderating the event, took the floor to thank MEP Zorrinho for his longstanding support of the PID community and a number of previous PID Forums, as well as for his work with IPOPI on both the EU and national level.



Following Mr Zorrinho's note on interoperability of data, Mr Prevot reminded the audience that Screen4Rare – a multi-stakeholder initiative, led by IPOPI, the International Society for Neonatal Screening (ISNS) and the European Society for Immunodeficiencies (ESID), teamed up with ERN Rita and MetabERN to create a platform on new-born screening for rare diseases, and its third workstream will specifically look into registries and support interoperability of data. Mr. Prevot then introduced the current relevant EU initiatives: 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 IPOPI welcomes

this initiative, as PID patients belong among those who can fairly benefit from well digitalised healthcare system.

Mr Prevot emphasised that the nature of primary immunodeficiencies calls for novel tools and methods to facilitate everyday activities linked to patients' diagnosis & treatment, as well as increased research and development capabilities. Individually, digital tools can allow patients to easily keep track of their treatments, day-to-day symptoms, medication, contacts, and have quick access to their most important documentation. On a larger scale, the establishment of broad-scope registries and the effective exchange of relevant data can facilitate telemedicine and remote monitoring, as well as foster interdisciplinary research on rare diseases. As such, digitalisation is truly present at every step of PID care – from daily symptom tracking to global research initiatives.

Setting the scene: PIDs and digitalisation of healthcare

Ms Martine Pergent, President of IPOPI, began by thanking IPOPI's partners who made this event possible, CSL Behring, Grifols and Takeda. She provided insights into the specificities of PIDs, as diseases that occur when components of the immune system are not working properly. Fortunately, she explained that digitalisation of healthcare has the potential to improve patients' lives in many aspects. Globally, digital health is gaining momentum as it can improve access to healthcare, provide more personalised healthcare for patients, improve the quality of care, and optimise the healthcare systems.



Ms Pergent introduced the EU's ambition for 2030: to empower businesses and people in a human-centred, sustainable and more prosperous digital future via the EU Digital Strategy, as well put the Strategy in the context of one of the European Commission's 6 priorities for 2019-2024 – Europe fit for digital age. This agenda looks into strengthening EU's digital sovereignty and to set its own standards, rather than following those of others, with a clear focus on data, technology, and infrastructure. The first initiatives related to this include the creation of the EU Health Data Space.

She further pointed out that there are still inequalities in terms of access of citizens to the benefits of digitalisation across EU Member States. It is necessary for the experts to share their best practices internationally. A big role in the cooperation is played by data flow, which can be accelerated by the patients themselves when using different tool for tracking their fitness goals, monitoring ongoing conditions, learning about the care, searching for information, etc.

Finally, Ms Pergent highlighted the opportunities brought by the following EU initiatives: a proposal for a Regulation on EU Health Data Space that will come in early 2022, and the revision of the Cross-Border Healthcare Directive, including the provisions on the European Reference Networks, that both have a great potential to contribute to much needed international collaboration and provision of better care and daily life to the PID patients.

The potential of digitalisation...

The event continued with a series of contributions from three experts on digitalisation and rare diseases from across the world. Mr Prevot introduced a speakers' panel, whose objective was to examine the potential of digitalisation in various areas of research and cooperation. The panellists especially focused on data availability and highlighted the obstacles put in place by the existing data protection regulation and other policies.

...(1) for epidemiology and research

Professor Mikko Seppänen from Helsinki University Hospital spotlighted how epidemiology and research can be improved with digitalisation in terms of quality data flow – his main question was: “Is the General Data Protection Regulation (GDPR) protecting rare disease patients to death?”



Professor Seppänen first reminded the audience that healthcare data is always of a highly sensitive nature, as it overlaps with human rights to privacy and conflicts other rights related to health. He believes, however, that human rights are inseparable and thus no one right should be emphasised over others. The GDPR attempted to find a balance between these rights, but its principles are not optimal in the field of rare diseases. He sees the main problem in the lack of any special rights

for healthcare data collection, storage and analysis. Currently, the time and specified purposes for which patient data can be stored are very limited. Another issue is the strict requirements for patient consent that cannot always be met in the case of medical information.

He expressed his professional opinion that the GDPR needs special concessions in rules that govern data on rare occurrences in healthcare, while patients and patient organisations should have a pivotal role in designing safeguards against hurtful use of data.

...(2) for global cooperation

Professor Nicholas Rider from Texas Baylor College of Medicine and the Texas Children's Hospital followed with his views on the possibilities opened by global cooperation and the need to have appropriate data. One of the main goals of digitalisation is the creation of interoperable learning health systems serving patients. The lack of interoperability is, according to him, caused by the fact that the existing systems are not connected to one another, not enabled to utilise real-world data, and are not designed for analytics. He believes that other factors causing the challenges lie in culture and workflow – there are differences in referral practices, expertise, languages, available services and testing.



He stressed that the solution comes from standardisation. Datasets should include patient and phenotype descriptions, workflows, data governance, diagnostic evaluations, longitudinal monitoring, available resources, and lie in the front-end processing while analysed at back-end, as opposed to the

current scheme of back-end processing of unstructured information. Cooperation is essential – quality and comfortable healthcare can be delivered only when data is shared globally – for this purpose Professor Rider suggested the creation of a global registry. For that, terminology of relevance to PID patients must be refined, HER data capture improved, and resource sharing enabled.

...(3) for the use of semantic web and FAIRification in rare disease registries



Professor Mark Little from Trinity College Dublin completed the panel with his video message on the use of semantic web and data FAIRification in rare disease in the context of the European Reference Networks (ERNs). Professor Little first provided the example of the ERN Clinical Patient Management System, which is a mean of providing expert care to patients with rare disease across Europe and collecting patient data. However, the user interface is not very user-friendly, and

it has not yet met its goals of building a centralised data server for collaboration because of the variances in types of data.

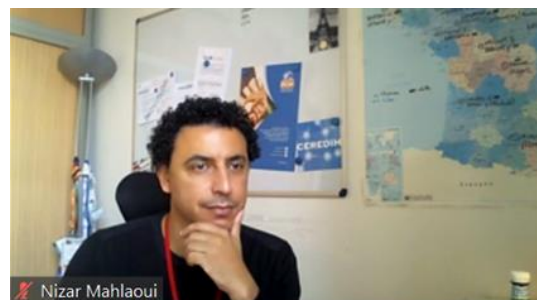
Most rare disease data already exists in small established regional registries. Accessing these at scale is an important step towards utilising the digitalisation of these healthcare records. The much-needed interoperability of data requires using harmonised terminology, data of same quality, etc. Semantic web has the potential to analyse all data on the internet based on a defined purpose.

His second example was the FAIRVASC registry, which was built as an interoperable registry aggregating the data of seven registries for vasculitis across Europe. Using FAIR (findable, accessible, interoperable, reusable) data at the aggregated level to protect patient privacy has so far provided initial success in researching and treating vasculitis. However, Professor Little ended by emphasising the acute need for sustainable funding. Currently, EU funding for FAIRVASC is set to end in two years, as is the case for many innovative projects such as this one. To be fully successful and ensure longevity, there needs to be long term investment from the EU.

Panel discussion on digitalisation and PID medical care

To expand upon the presentations, Ms Martine Pergent led a panel discussion with Dr Nizar Mahlaoui, Mr Friedolin Strauss, and Professor Isabelle Meyts.

Dr Mahlaoui (Unité d'Immuno-Hématologie & Rhumatologie pédiatrique, Hôpital Universitaire Necker-Enfants Malades, Assistance Publique-Hôpitaux de Paris (AP-HP)) described the changes in healthcare practice he has witnessed over the last two pandemic years. Indeed, the expedited transition to digital methods demonstrated how unprepared the system was to share data securely and transparently with patients. Unfortunately, this meant that patients and practitioners occasionally used methods of communication such as unsecured emails, leave them susceptible to



hacking and data leaks. He emphasised the need to find accessible and simple ways of communicating with patients.



Mr Friedolin Strauss, patient representative, furthered this sentiment by describing the difficulty patients have with digital tools from two angles: first, that the current digital tools are not intended for easy data access and sharing; and second, that privacy and security are currently difficult to understand and limit. Without having control over his own data, he has faced difficulties with knowing who needs to know what and how to ensure security. Additionally, he has attempted to “self-

digitalise” his data onto a USB drive to be able to better share and utilise it. However, the level of technological knowledge and time involved in this made it inaccessible to many – patients should not need expert knowledge to access their data – and the company no longer supports the device.

This difficulty – in giving fair access but also using data effectively for care and research, was expanded upon by **Professor Isabelle Meyts** (Professor in Faculty of Medicine KU Leuven, Head of Inborn Errors of Immunity Unit). Prof. Meyts highlighted the potential for digital tools to make a significant difference for patients with PIDs by identifying patients before they have irreversible damage. To create tools capable of this, data needs to be shared across borders and interoperable. She stated that, while the EU still has a long way to go in terms of achieving this fair access, the current momentum is significant, and she believes we could be on the right track to building infrastructure that utilises data in a way that creates and finds solutions for patients.



All of the speakers and panellists were then invited back to the virtual stage to discuss questions, again underlining the main concepts featured throughout the event: the need for **interoperable, reliable, and qualitative data** that is **transparent and patient-centred**. Indeed, questions revolved around how to leverage telehealth, improve global collaboration and intentionally use data.

Speakers' policy recommendations

At the end of their interventions experts provided one key policy recommendation to better harness the potential of digital for PIDs. These policy recommendations will guide further advocacy efforts and provide a baseline for MEPs to consider as they engage with the many upcoming relevant policies. They can be categorised into three main themes:

1. *Fund research and development*

Research and development are important for the PID community because they can increase the ability to give a timely diagnosis and avoid unnecessary, repeated procedures or preventable comorbidities, as well as identify best treatment pathways.

The European Commission is called to increase funding into the research into the **development** and **implementation** of easy and secure digital tools, including:

- o Utilising AI to enhance and expedite diagnosis
- o Utilising digital solutions for ad hoc treatment development
- o Incorporating analysis of electronic medical records into care practice

2. Increase the ability for cross-country knowledge sharing

Knowledge sharing is crucial for rare disease and PID patients due to the relatively limited amounts of data and research regarding PIDs. This exacerbates the need to collectively build knowledge. Data and experience must be shared to effectively innovate and find solutions.

EU Institutions should continue to support knowledge sharing and collaboration by:

- o Expanding support for - **methods for accessing regional data systems** to assist European Reference Network Clinical Patient Monitoring Systems' functioning and ability to conduct rare disease research
- o **Adapting the General Data Protection Regulation** to have special concessions and rules to govern rare occurrences in healthcare and research (such as indefinite storage and acceptance of broad consent)

3. Ensure patients are centred in decision-making and care

Including patients in the core of the legislative process is necessary to both build trust as well as optimal solutions. Involving patients in the legislative discussions will ensure the community's needs are well met.

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

- o **Incorporating patient representatives into registry steering committees** with decision-making roles
- o Mandating that the healthcare systems allow **patients to easily access their own health** and share it in a standard format
- o Being intentional in the use of digital tools for PIDs, ensuring that tools are not biased and **account for the risks and limitations** of data sharing

Closing Statements



MEP Marina Kaljurand began her closing remarks by describing how much she had learned from the experts at the Forum. Coming from Estonia, a country with a very strong e-health system, she described her high expectations for digital health because she has seen first-hand its success. Indeed, if there is interest, political motivation, cybersecurity and trust, she believes that unique digital ecosystem can be created that does successfully work for patients and providers. She was

thus excited for the recommendations and identifying how to build a stronger EU data and health system.

MEP Carlos Zorrinho then complemented Ms. Kaljurand's statement, as well as thanked her for coming out of her comfort zone. Indeed, he found the sharing of real, good practices at the Forum inspirational. He highlighted the importance of the PID Forums, during which he could also learn, be beside a colleague and identify routes for them as policy makers to pave the way for the experts to work.

Mr Johan Prevot provided the final remarks of the event and likewise thanked all speakers and participants, particularly those representing PID patients, for taking part in the 19th PID Forum. He added that IPOPI was excited to build such strong relationships with MEPs, both with long-term collaborators and new policymakers. With the overarching focus on digital in the EU, IPOPI looks forward to the potential gains within quality of care and research for patients with PIDs.

