



IPOPI 18th PID Forum

Cross-Border Healthcare Directive: Spotlight on curative treatments for PID patients

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13 July 2021

14:00-15:30 CEST

Cross-Border Healthcare Directive:
Spotlight on curative treatments for PID Patients

Co-hosted by Members
of the European Parliament

Radka Maxová | Tomislav
Alex Agius Saliba | Sokol.

Tuesday, 13 July 2021, 14:00 – 15:30 CEST

Co-hosted by MEPs Tomislav Sokol (EPP, Croatia); MEP Alex Agius Saliba (S&D, Malta); MEP Radka Maxová (S&D, Czech Republic)

Introduction

On 13th July 2021, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised its 18th PID Forum titled “**Cross-Border Healthcare Directive: Spotlight on curative treatments for PID patients**”. The online event was co-hosted by Members of the European Parliament (MEPs) **Tomislav Sokol (EPP, Croatia); Alex Agius Saliba (S&D, Malta); Radka Maxová (S&D, Czech Republic)**, and moderated by **Mr Johan Prevot**, Executive Director of IPOPI.

During the event, participants analysed the current status of curative treatments for primary immunodeficiency (PID) patients in the EU and whether the Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare (also known as the Cross-Border Healthcare Directive - CHBD), is an opportunity to make these treatments more accessible to patients. The outcomes of these discussions will be presented to the European Commission via their public consultation on “*Cross-border healthcare – evaluation of patients' rights*” open until 27th July 2021.

The event unfolded with MEP Tomislav Sokol (EPP, Croatia), MEP Alex Agius Saliba (S&D, Malta) and MEP Radka Maxová (S&D, Czech Republic) confirming their political support to the PID community when it comes to addressing their needs when seeking access to curative treatment.

Opening remarks



Opening the 18th PID Forum, MEP Tomislav Sokol (EPP, Croatia) welcomed all speakers and participants and expressed his delight to be able to continue to support the PID patient community. Mr Sokol emphasized that, even prior to his entry into the European Parliament, he had been particularly invested in the potential outcomes of cross-border healthcare as he recognised the importance of an efficient framework facilitating the mobility of patients. He stressed the

particular importance of a legal framework for rare disease patients, such as PID patients, by highlighting that the very nature of their conditions can lead to insufficient medical expertise or medical equipment necessary to treat a specific condition at a national level. These citizens, therefore, need to look for medical support beyond their national borders.

He discussed the particular point of rare diseases not being so rare after all given that an estimated 27 to 36 million European citizens are affected by one. Unfortunately, the specificity of these conditions mean that rare disease patients must often overcome a number of similar challenges, such as access to treatment and delays in diagnosis. He especially pointed to the differences in the implementation of the CBHD across the EU's Member States which have been acknowledged by the European Court of Justice as a problem regarding access to healthcare. Although the CBHD has made healthcare more accessible for rare disease patients, he noted that the upcoming evaluation of the Directive is a prime opportunity to focus on the specific situation of the PID patients and promote an individualised approach to their treatment.

Mr Sokol concluded by expressing his desire to continue contributing to improving the delivery of cross-border healthcare, promote awareness of rare diseases among all relevant stakeholders and help PID patients access curative treatments in a timely manner.

MEP Alex Agius Saliba (S&D, Malta) was the 18th PID Forum's second co-host to take the floor. He warmly welcomed all participants and said that he feels honoured to co-host such an event together with his colleagues. Mr Saliba focused his contribution on the benefits of the CBHD and the European Reference Networks (ERNs). He outlined that, despite these positive initiatives there is still a long way to go to fully achieve the objectives of the legislation, especially when it comes to smaller EU Member States.



MEP Saliba said building an effective framework that helps ensure rare disease patients across the EU get the treatment they need is a necessity because surveys on the topic have shown that a majority of rare disease patients are willing to travel to another country to receive treatment. Members of the European Parliament need the right information to truly understand the needs and hurdles of patients, especially when they try to receive curative treatments abroad.

Mr Saliba's conclusion saw him point out that PID patients represent a large demographic group which is constantly growing, so the experience of these patients who have received curative treatments in other countries would prove valuable for decision-makers such as himself who would look to support rare disease patients.



The event's third co-host, **MEP Radka Maxová (S&D, Czech Republic)** expressed her delight in collaborating with the PID community for the first time and stated she looked forward to learning more about more PID patients and their specific needs when looking to access curative treatments. Access to healthcare, she said, is a fundamental right which is often compromised by the obstacles of cross-border treatment.

MEP Maxová noted that several EU institutions were currently looking to identify and address the implementation gaps which need to be tackled so that rare disease patients can fully benefit from access to safe and high-quality healthcare in another Member State. She has personally tabled a parliamentary question on the topic, in collaboration with several colleagues from the European Parliament, to understand how the Commission intends to tackle gaps in the EU's cross-border framework; a framework which not only implicates the CBHD but also to the Regulation on coordination of social security systems. Ms Maxová said these two legislations are often critical to enable patient access to transplants, gene therapies and other advanced therapies medicinal products which might be curative. Despite this, she pointed to the complex reimbursement procedures and other financial hurdles as some of the primary barriers that need to be overcome when it comes to accessing curative treatments for rare genetic disorders.

Ms Maxová closed her intervention by expressing her wish to hear the thoughts and stories of the speakers gathered for the 18th PID Forum and underlined her commitment to ensuring the outcomes of the discussions will be used in her future political work on the cross-border healthcare framework.

Mr Johan Prevot, moderating the event, took the floor to remind the audience that, ten years after its entry into force, the CBHD was still a source of hope for Europe's rare disease patients, including citizens with PID. This pioneering legislation not only led to the establishment of the ERNs, it has also positively contributed to patients access to curative treatments by facilitating patient mobility. He expressed his hope that today's discussions will prove to be fertile ground for IPOPI's future work related to the CBHD.



Setting the scene: Uneven access to curative treatments across the EU



Martine Pergent

Ms Martine Pergent, President of IPOPI, began by thanking IPOPI's partners who made this event possible, CSL Behring, Grifols and Takeda. She provided some insights into the specificities of PIDs as conditions by explaining how it impacted a patient's immune system by causing it to not work adequately or at all. Fortunately, she explained, there are curative treatments available and most notably for children. Those usually involve biological/plasma therapies, anti-infectious therapies, hematopoietic stem cell transplantation, thymic transplant, or gene therapy.

She highlighted that the younger the patient, or the more severe their condition, the more urgent the treatment.

Curative treatments consist of adequate and accurate diagnosis, transplantation, and follow-up from transplantation. These operations require of expertise and resources that are often unavailable in all EU countries. However, the availability of a curative treatment does not mean that the patient can always be treated especially when taking the overall cost of these medical procedures and additional costs into account. She highlighted that major discrepancies in the healthcare systems of the EU's Member States and the insufficient awareness among the medical field, led to IPOPI's development the [PID Life Index](#), a tool developed to measure the status of the PID healthcare environment in a country.

Ms Pergent further stressed the importance of pursuing research in terms of curative treatments for the PID patients. EU funded research programmes are aiming at increasing the number of gene therapies and better access to the existing therapies.

Ms Pergent concluded by presenting IPOPI's suggestions on addressing the issues which encompass: the need to (1) increase awareness about the possibility of being treated abroad, (2) clarify the overarching procedure, reimbursement and payment clauses, (3) streamline the process and reduce bureaucratic complexity – especially in life-threatening cases, and (4) help and accompany the patients and their families through the process. Hopefully, these needs will be addressed by the revised CBHD.

Cross-Border Healthcare Directive and European Reference Networks



Jose Valverde Albacete

Dr José A. Valverde, Policy Officer for Unit B3 - Digital Health, European Reference Networks, at the Directorate-General for Health and Food Safety (DG SANTE), provided the audience with some insights into the European Commission's activities regarding the CBHD and the ERNs team role in support of the ERNs. He briefly explained that the ERNs enable virtual remote consultations and clinical data on patient cases exchange, advising and exchange of expertise on

diagnosis and treatment, generating knowledge, research on rare diseases, or education and professional training.

Dr Valverde provided the legal basis for the foundation of the ERNs and the network's achievements in terms of providing support to European rare disease patients through virtual consultations. He outlined

that knowledge generation had particularly increased through training, awareness-raising activities, and the implementation of clinical practice guidelines. Moreover, he also expressed the positive aspects surrounding the creation of the rare disease patient registries and the future inclusion on the European Health Data Space (EHDS). He explained that the next steps for the ERNs include managing the enlargement of the geographic scope and disease coverage while consolidating the system and ensuring long-term sustainability. Dr Valverde stated that he expected the enlargement of these networks to be completed later in 2021 and that a future Joint Action on ERNs shall look to ensure the integration of ERNs into national systems by 2022.

Dr Valverde closed his contribution by welcoming IPOPI's intent to participate in the Cross-Border Healthcare's public consultation and made a number of recommendations regarding the process in which they could do so.

A Patient's Journey through Cross-Border Treatment

The event continued with the contributions of two patient representatives: **Mr Colin Maher**, an Irish father of two boys who travelled internationally to secure treatment and **Ms Otilia Stanga**, a mother of a girl fighting a PID and a representative of the Romanian PID Patient Organisation, ARPID. They both shared their stories and provided recommendations based on the obstacles they had to face when seeking curative treatments for their children.



Mr Maher explained that their journey began with their son, Oliver, born in 2011. Despite a series of examinations, Oliver was not receiving a diagnosis from doctors until his referral to a specialised hospital in Newcastle. They then discovered that he had been suffering from a deficient immune system and that he needed a transplant.

Despite some delays in treatment and additional costs in terms of lodging and food, an unfortunate and significant financial burden, Mr Maher noted that they did not have to pay for the medical procedure. However, Mr Maher underlined the fact that they were often left in need of additional support and required more information regarding the ongoing process which added to the overall stress. Mr Maher explained that having someone to talk to, someone who would guide them through the process to explain what exactly is happening at a particular moment, would be an important improvement contributing to a better quality of the whole system.

Following Mr Maher's contribution, Mr Prevot gave the floor to **Ms Stanga**, who provided her personal perspective and experience from her country of Romania. Ms Stanga explained that despite the CBHD being implemented in 2014 there are still barriers and challenges patients face in terms of receiving the necessary information about possible administrative routes should a treatment abroad be needed. She noted that in her particular case, even with the help of Romania's sole National Contact Point, it was still difficult to obtain information.



Ms Stanga highlighted through her daughter's journey highlighted the difficulties that need to be overcome despite an understanding that cross-border healthcare was a necessity especially when it comes to the administrative burdens and the need for advance payments. She also explained that the lack of information in Romania spreads beyond patients into the domain of healthcare professionals and competent authorities which leads to crucial delays in decision-making and no clarity regarding possible reimbursement routes for the treatment.

She deplored the fact that these difficulties discourage PID patients from seeking curative treatments in other EU Member States. Because of this situation, the Romanian PID community has been advocating for a change in this area.. She expressed her hope that the further reinforcement of the Directive at the European level would improve the national implementation levels.

European Court of Auditors: Recommendations for the Cross-Border Healthcare Directive



Dr Nikolaos Milionis, Member of the European Court of Auditors (ECA), provided a recorded message wherein he explained the ECA's 2019 report that reviews the gaps in the implementation of the Cross-Border Healthcare Directive. Dr Milionis emphasised that although healthcare policy is managed and financed at the national level with the EU acting to complement it, it is important to take joint actions towards improving citizens' lives.

He noted that the CBHD offered possibilities to those seeking curative treatments, a particularly enticing prospect for the millions of European citizens suffering from rare diseases. However, he emphasised that the ECA recognised a number of limitations, which can be applied to PID patients, when it comes to the Directive's implementation. On the basis of their 2019 report, Dr Milionis highlighted the ECA's recommendations for the Directive's revision. Firstly, the ECA recommended that the Commission provide more support to the National Contact Points so they can help the patients with a broader scope of issues. Secondly, there should be a stronger focus on health data exchanges. The ECA identified severe delays in the deployment of the EU health infrastructure and poor flows in transfers of quality health data. Finally, the ECA recommended that access to cross-border treatment for rare disease patients should be significantly improved. Dr Milionis' statement concluded that the ECA will oversee the implementation of their recommendations into upcoming revisions of the Cross-Border Healthcare Directive so as to ensure that it benefits those who need it most: European patients.

Roundtable Session: Addressing Questions on Cross-Border Healthcare

Dr Milionis' statement was followed by an interactive discussion which included a number of patient representatives, healthcare professionals and stakeholders working in the field of cross-border healthcare. This fast-paced panel session offered a vivid exchange of views on the various problematics that needed to be overcome and a number of insights from its participants.



Dr Nizar Mahlaoui, Necker-Enfants Malades University Hospital in Paris (France), spoke about his experience in treating PID patients from other countries and provided some insights into the reasons why European patients would travel to France. Dr Mahlaoui explained that treating PID patients – both children and adults – and curing patients with some of the most severe PIDs has become a medical possibility and that awareness of these conditions is increasing. However, despite some

positive evolution, delays in treatment are still too common and he underlined the need for more access to information and the development of effective reimbursement schemes.

Dr Valverde took the floor once more to emphasise the aspects that needed reinforcement five years after the creation of ERNs and their representation within the EU4Health Programme. He stressed the need for cooperation across every level which needed to be supported by a strong national base. He called for more awareness within the patient and medical landscape of the opportunities they have to shape EU healthcare policy and added that the European Commission is working on making public consultation instruments more accessible to these stakeholders. He highlighted that the EU4Health Programme offers financial support for the ERNs' development within the first annual work programme adopted in June 2021.

Ms Joyce Loridan, a policy officer at Solidaris, a Belgian Mutual Health insurance fund, who also sits on the Board of Directors of the [International Association of Mutual Benefit Societies \(AIM\)](#) spoke of her involvement in a project on rare diseases and how to go from local and cross-border developments to European solutions: the [EMRaDi project](#). She stated that the project's foundation relied on the dedication of health insurance funds dedicated to learn about cross-border healthcare needs of rare diseases patients, and facilitate better access to better care for the patients and their families. The project concluded in 2020 with a number of recommendations on the need for a more holistic approach to rare diseases, the development of reimbursement mechanisms for telemedicine and a more structured approach to coordination across rare disease patient organisations at the European level. Ms Loridan stated that following the successful EMRaDI project, Solidaris continues its work with a focus on rare disease patients and prepares to answer the consultation on the Cross-Border Healthcare Directive.



Ms Otilia Stanga outlined the areas which could be improved upon during the revision of the CBHD so that PID patients can have access to cross-border treatment. She reinforced the need to reduce the burden on patients especially in terms of administration and upfront payments. She likewise spoke of the need for better referral processes so that diagnosis can be provided as early as possible. Finally, she explained that a patient advisor guiding them through the process would be a significant added value. Mr Maher stated that the families of PID patients likewise required support, especially when language barriers can make the process more complex.

Open Floor Discussion

This interactive section was followed by an open floor discussion moderated by Johan Prevot between the panellists and the attendees. **MEP Radka Maxová** was asked what steps the European Parliament can take to ensure the current framework regulating cross-border healthcare is improved upon when it comes to removing the burdens on rare disease patients. Ms Maxová stated that she views the lack of transparency of the pre-approval procedures at national level as a significant limitation of the Cross-Border Healthcare Directive. She promised that the MEPs will do their best to work for provisions which would eventually improve the implementation of the Directive at national level.

Dr José A. Valverde answered a question about the potential extension of the scope of the ERNs to diagnosis and knowledge-sharing in the context of the CBHD. He pointed that the European Commission seeks to balance its input with that of the Member States' internal rules. He also pinpointed further issues regarding inequalities in the level of development and integration of ERNs between the national systems. Dr Valverde likewise stated that the idea of the virtual consultations under the ERNs leans towards the promotion of fewer patient movement so as to ensure they can contact specialist anywhere in the EU.

Dr Nizar Mahlaoui responded to a question about the main social challenges when it comes to cross-border healthcare. He explained that many of his colleagues view additional costs (pertaining to the families, hence being a significant burden) – in addition to treatment cost – and discrepancies between patient rights across EU Member States should be addressed through the inclusion of a network of social workers working jointly with ERN representatives. He also proposed more training for administrative staff when it comes to ERNs, social security specifics and transitional care from paediatrics to adults. Also, this is a significant psychological burden for the patient and the caregivers.

Closing the session, Dr Valverde announced that there will be first evaluation of ERNs in 2022 and explained that this would be an opportunity for all stakeholders to raise awareness of their objectives and help choose the next steps for the ERNs' development.

Closing remarks

As the meeting ended, **MEP Radka Maxová** expressed her gratitude to all participants, her interest in learning more about the journeys of PID patients looking to access curative treatments and her commitment in pursuing this topic within the European Parliament. Her conclusion highlighted that the theoretical potential of the Cross-Border Healthcare Directive has not yet been fulfilled and that more needed to be done to improve upon the current framework. In addition to applauding IPOPI's efforts in organisation the event, she called for a bottom-up approach to the revision of the CBHD and a deeper cooperation with the National Contact Points to ensure access to quality healthcare for rare diseases patients can be guaranteed.

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 18th PID Forum. He added that IPOPI would waste no time in further developing their position on the cross-border healthcare



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directive's revision and revive the political debate on how to make curative treatments more accessible to everyone.