

IPOPI's World PI Week 2016 Celebration

Tuesday 26 April 2016

European Parliament, Brussels

Co-hosted by MEPs José Inácio Faria and Carlos Zorrinho

Report



Introduction

On Tuesday 26 April 2016, MEPs José Inácio Faria and Carlos Zorrinho co-hosted the events marking the World Primary Immunodeficiency Week (WPIW) 2016 in the European Parliament. The meeting, organised by the International Patient Organisation for Primary Immunodeficiencies (IPOPI) focused on raising awareness of Primary Immunodeficiencies (PID) and the challenges experienced in both European and International settings on the occasion of the WPIW. It was an opportunity to amplify and promote the WPIW call to action 'Test, Diagnose, Treat'.

During the conference, representatives from the European Parliament, including Dr. Bolesław Piecha MEP, engaged with patients and healthcare professionals to learn and exchange good practices for PID care. This exchange of experience and understanding was appreciated by all participants as a crucial step in effectively developing and implementing appropriate policies for rare disease patients. All participants agreed that patient organisations, European institutions and healthcare professionals need to continue their work together in order to address the burden of PIDs.

The event earmarked WPIW and was an opportunity to reflect on the work IPOPI has undertaken in the European Parliament over the last 5 years and to re-launch the WPIW call to action "Test. Diagnose. Treat." calling on governments to understand, appropriately diagnose and manage PIDs by developing national strategies. The call to action was signed by all participants from the meeting and members of the European Parliament committed to sharing this further with their colleagues.

Summary of discussions

First session: World PI Week from an international perspective



Mr. José Inácio Faria MEP opened the meeting by reaffirming his support for the World Primary Immunodeficiency Week (WPIW) call to action entitled "Test. Diagnose. Treat." and emphasised the need of such initiatives to strengthen PID discussions in the European Parliament. As one of the MEPs who has supported IPOPI's work in the Parliament over recent years, particularly in the launch of the PIDs Principles of Care, Mr. Faria MEP stressed the need to engage patients, physicians, scientists, industry and politicians in discussions surrounding the necessity of global standards on PID care, ensuring an exemplary guidance to

governments worldwide. As discussions will certainly continue around PID care in the coming years, Mr. Faria MEP was very hopeful for the implementation of the PIDs Principles of Care and for the further collaboration between IPOPI and the European Parliament.

Ms. Martine Pergent, IPOPI Vice-Chair, welcomed the participants on behalf of IPOPI and thanked Mr. Faria MEP and Mr. Zorrinho MEP for their continued support to patients with PIDs. Ms Pergent emphasized the importance of collaboration in the field of Primary Immunodeficiencies and initiatives like World Primary Immunodeficiency Week were the perfect example of how efforts could be joined to improve the lives of people with PIDs. Global campaigns like WPIW showcase the importance of raising awareness about this large group of rare and chronic diseases and highlight the importance of pulling together all the existing expertise and efforts. The lack of knowledge can lead to a lack of access to care, as patients with PIDs are often mis-diagnosed and treated inappropriately for their symptoms rather than for the disease itself. The Principles of Care for PIDs, an IPOPI-led initiative with the contribution of a multi-disciplinary team of specialists, sets out comprehensive principles of care for PIDs. The principles can now be used by all stakeholders, including payers to improve care around the globe and they could crucially improve the awareness around PIDs. Ms. Pergent noted that only by implementing adequate health and social policies that facilitate a timely and accurate diagnosis with access to correct treatment, will it be possible for patients to positively contribute to society. She emphasised that IPOPI, through the collaboration with European institutions, Member States, patient organisations and other stakeholders, is constantly working to ensure that people with PIDs are diagnosed as early as possible and have appropriate access to safe, affordable and efficient life-saving treatments. Together, the vast gaps in knowledge of PIDs between regions, countries, and even areas can be addressed.



Dr. Piecha MEP commenced his speech noting the work undertaken with Dr. Buşoi MEP in 2014 at the 5th PID Forum which focused on developing a comprehensive framework of care for PIDs. He congratulated IPOPI on the PID Principles of Care which have brought to reality a strong framework for care that can be implemented not only in the EU but also across the world. This emphasised the importance of continuing to build on momentum generated through the PID Forum's in increasing political awareness and support. Dr. Piecha MEP emphasised the need to ensure that the document resonates not only with the European Commission but also in Member States. He continued stressing the importance of patient involvement in policy making encouraging patient organisations and stakeholders to bring to the legislators' attention the important policy issues where care can be improved for PID

patients. For both of the events Dr. Piecha MEP has been involved in at the European Parliament, he has had the opportunity to exchange views with Adrian Górecki, President of the Polish PID patient organisation 'Immunprotect'. To this extent he emphasised the need to create contact between patients and their European Parliament representatives to enhance national as well as European understanding of the burden of PIDs.



Ms. Gabrielle Rosario from the Primary Immunodeficiency Network of South Africa (PiNSA) provided the audience with the regional perspective on the African priorities on PID. She announced that there are currently more than 200 PiNSA members and continent-wide awareness initiatives. Ms. Rosario stressed the need of continuing to raise awareness amongst healthcare professionals and within the public domain. She presented the success of PiNSA in alignment with allergology community and the Rare Disease South Africa organisation

reminding participants of the benefits of collaboration. Unfortunately, despite the success led by IPOPI and the WHO, PID patients in South Africa are still at the mercy of insurers because PIDs are not on the list of chronic diseases, and immunoglobulin therapy is not on the list of the essential medicines yet. She announced the 18th International Conference on human Genetic Disorders and Diseases that will take place in November 2016 in Cape Town and will aim to bring together leading academic scientists, researchers and research scholars to exchange and share their experiences and research results about all aspects of human genetic disorders and diseases.

Prof. Aziz Bousfiha from the Hajar Association in Morocco, joined the conference digitally from his office in Morocco. Through a teleconference, he presented the priorities, developments and challenges of PID patients in North Africa and the Middle East. While presenting the developments in North Africa and the Middle East, Prof. Bousfiha encouraged governments to give more importance to PIDs. Importantly he called for MEPs to write a letter to the leaders of North African countries calling for improved care for PIDs and better prioritisation. He announced several crucial PID development tools, such as the national centres and registries, PID references in population language and awareness, and patients' education documents. Last, but not least, Prof. Bousfiha noted the 5 challenges before 2025 in North Africa and the Middle East. These challenges include diagnosing 30% of the patients, describing the genetic bases of the main clinical phenotypes, providing allograft to 30% of those in need, providing immunoglobulins for all patients, and developing newborn screening in the Middle East.



Ms. Dragana Koruga from 'Supporting Persons with Primary Immunodeficiencies' in Serbia spoke from her perspective about the Balkan countries' priorities. From her intervention it became clear that for about 33 million citizens living in 8 Balkan countries there is an insufficient supply of life saving plasma-derived products despite only having around 600 PID patients. Ms. Koruga presented the crucial difference of European Union membership in its impact on the lives of PID patients. In non-EU member countries, the access to diagnostic procedure is limited due to lack of

resources. In spite of numerous challenges, certain improvement has been made in the Balkan

region. In all countries, safe immunoglobulin therapy is applied and only high quality plasma derivatives are present on the market. Furthermore, thanks to the continuous education and networking among doctors and patients from Balkan countries, the number of diagnosed PID patients has been steadily increasing. According to Ms. Koruga, the voice of patients has become much stronger in the last 10 years, but there is still a need for increased access to diagnostics and treatment.

Second session: World PI Week from a European Perspective

Mr. Carlos Zorrinho MEP opened the second session by focusing on the work done with IPOPI in the last few months. As the co-host of the 6th PID Forum on the Principles of Care for PIDs, Mr. Zorrinho MEP promoted these principles to the European Commission. He strongly believes that the European Union has a clear competence to facilitate the sharing of best practices, expertise and guidelines to address the PID challenges. In order to raise more awareness on the topic, Mr. Zorrinho MEP together with Mr. Faria MEP tabled two questions to the European Commission on the principles of care for PIDs and on screening for Severe Combined Immunodeficiency (SCID). Mr Zorrinho MEP reinforced the strong work of IPOPI in bringing patients and policy makers together and welcomed further work together with IPOPI to further enforce the view of patients in the European Parliament. Joining his two previous colleagues from the European Parliament in signing the WPIW call to action, Mr Zorrinho confirmed the need to tackle this issue at European and national level and was further glad to have had the opportunity to meet with patients and doctors from Portugal. This exchange of views is crucial in enhancing patient representation across all forms of government.



Mr. Johan Prévot, IPOPI Executive Director provided the audience with a European public health perspective on PIDs noting the different EU tools available to improve PID care. Mr. Prévot's presentation focused on the call for action of the World Primary Immunodeficiency Week. He elaborated on the SCID newborn screening status in Europe and the PIDs Principles of Care as a scarce knowledge at medical, political and societal level about PIDs. Mr. Prévot noted that not all of Europe benefits from international scientific collaboration, from facilities that would allow patients to get diagnosed, treated and cared for. He also announced that in 2016, IPOPI facilitated initial discussions of top physicians to develop a rare immunological and auto-inflammatory diseases European Reference Network. Mr. Prévot called on the

participants to work towards ensuring that future legislation revisions take into consideration the needs of the patients receiving therapies, i.e. the blood legislation.

This intervention was followed by World Primary Immunodeficiency Week ‘learning from each other’ section, where best practices were shared from National Members Organisations, regarding their activities during World PI Week including the national association for patients with immunodeficiencies from Belgium (BOPPI), Italy (AIP), PID UK, the Netherlands (Stichting voor Afweerstoornissen), Poland (Immunoprotect), Serbia (POSPID), Spain (AEDIP), Sweden (PIO). Dr Laura Marques and Dr Stephan Borte joined the discussions and provided their views on newborn screening for severe forms of PIDs.



Prof. Klaus Warnatz, from the University of Freiburg, continued the session with his presentation on World Primary Immunodeficiency Week in 2020. Professor Warnatz made a call for a Europe-wide new born screening while at the same time highlighting the importance of the ERNs and the need to strengthen this cooperation. According to him, all patients need to be registered in order to create a European scale and address the challenges of PIDs. Prof. Warnatz stressed that every European PID patient deserves access to genetic diagnosis and that we need a European call for translational medicine in PIDs. Moreover, as early diagnosis is essential to improve health outcome, newborn screening is absolutely crucial for children with severe combined immunodeficiency.

Ms. Martine Pergent went on to draw some conclusions from the lively discussions. She indicated the importance to the call for action “Test. Diagnose. Treat.” during the two sessions and was glad to welcome the wide political support it received. The call invites stakeholders around the world to join the WPIW global call for action, asking governments to understand, appropriately diagnose and manage PIDs diseases in their respective countries and to develop national strategies that address the diseases. Bringing the conference to an end, Ms. Pergent asked participants to show their support by signing the signature list of the call for action. This was warmly received and IPOPI believes it is crucial that policy makers go on to spread awareness about this call to action to improve care for PIDs.



List of participants

- Mr. José Inácio Faria, Member of the European Parliament
- Mr. Carlos Zorrinho, Member of the European Parliament
- Dr. Bolesław Piecha, Member of the European Parliament
- Ms. Gabrielle Rosario, Primary Immunodeficiency Network of South Africa (PiNSA)
- Professor Klaus Felix Warnatz, University of Freiburg
- Ms. Dragana Koruga, Supporting Persons with Primary Immunodeficiencies (Serbia)
- Dr. Laura Marques, Maria Pia Hospital, Porto
- Professor Ahmed Aziz Bousfiha, Hajar Association, Morocco (teleconference)
- Mr. Johan Prévot, IPOPI
- Ms. Martine Pergent, IPOPI
- Ms. Leire Solis Garate, IPOPI
- Ms. Edith Klapwijk, BOPPI
- Ms. Susan Walsh, PID UK
- Mr. Adrian Górecki, Polish PID
- Mr. Andrea Gresani, AIP
- Ms. Jose Verstegen, Stichting voor Afweerstoornissen
- Dr. Teresa Español, AEDIP
- Dr. Stephen Michael Borte, Karolinska Institut
- Mr. Toon Digneffe, Baxalta
- Mr. Bruno Santoni, PPTA
- Mr. Karl Petrovsky, PPTA
- Mr. Cristian Lutan, Rohde Public Policy
- Mr. Christopher Greenop, Rohde Public Policy
- Mr. Yordan Aleksandrov, Rohde Public Policy
- Ms. Sarah Harvey-Kelly, Rohde Public Policy