



6th PID Forum

Principles of Care for Primary Immunodeficiencies

7th December 2015

European Parliament, Brussels

Hosted by MEPs Carlos Zorrinho and José Inácio Faria

Report



INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

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1. Introduction

On Monday 7 December 2015, MEPs Carlos Zorrinho (S&D, Portugal) and José Inácio Faria (ALDE, Portugal) hosted the 6th Primary Immunodeficiency Forum in the European Parliament dedicated to the launch of the new Principles of Care for Primary Immunodeficiencies (PID). The meeting was organised in collaboration with the International Patient Organisation for Primary Immunodeficiencies (IPOPI) with the aim to implement the PID Principles of Care across the European Union member states, emphasizing ways of action to promote enhanced international collaboration in research, care and treatment. Furthermore, the conference focused on the key role of patient organisations and the need for newborn screening, an essential tool to accelerate access to curative treatment for severe combined immunodeficiencies.

The forum provided an opportunity to highlight good practices in Europe between healthcare professionals, patient organisations, representatives from the European Parliament and industry. Importantly, the Forum set forth the elements that should be put into place to reach 'gold standard' treatment for PID patients. It also highlighted the crucial role of patient organisations and the need for further specific policies on immunodeficiencies at both national and EU levels. All participants agreed on the value of further collaboration between EU institutions, patient organisations and healthcare professionals for better policy-making.

2. Summary of discussions



Mr. Faria MEP pointed out in his opening speech the fast-growing number of rare and chronic immunological disorders that prevent the immune system from working properly or not at all and which can cause serious organ damages and endanger patients' lives if left untreated. He welcomed this 6th PID Forum for bringing together policy makers, patient organisations and healthcare experts to cover a wide range of subjects impacting the lives of PID patients. He then called for further action on PIDs in the European Union. He went on to say that the EU shall encourage Member States to adopt and implement standards of care on immunodeficiencies in health and social policies. Mr. Faria emphasised that patients should be at the forefront of health policies and as such endorsed the Principles of Care.



Mrs. Jose Drabwell, chairperson of the IPOPI Board of Directors, provided an overview of the Principles of Care for primary immunodeficiencies, referencing the article published in *Frontiers in Immunology* in December 2014¹. Mrs. Drabwell emphasised the need for specialised centres to raise awareness in the medical community and connect all layers of medical disciplines and research. She stressed the importance of international registries which provide crucial tools for both research and policy-making and she welcomed the recent trend worldwide for the establishment of international registries of PID patients. Mrs. Drabwell noted that those registries should foster international collaboration to increase research potential for treatment and better disease understanding. The PID Principles of Care identify patient groups as key stakeholders in collecting data on patients' health and providing policy-makers with information on ways of action. With a unique focus on the health of PID patients in their countries, patient organisations should be at the forefront of decision making. Mrs. Drabwell also elaborated on the issue of fast and reliable access to PID treatment which requires availability of specialised medical staff as well as affordable access to immunoglobulin products and haematopoietic stem cell transplant (HSCT). She finally stressed the importance of maintaining a strong level of care internationally and continuously exchanging information on best practices so as to ensure equal levels of diagnosis and care across the European Union.



Mr. Carlos Zorrinho MEP commenced his speech by highlighting the importance of PIDs as a priority health policy topic both at national and European level. Mr. Zorrinho reminded that support for enhanced cooperation is needed to be able to turn the PID Principles of Care into concrete action. He further noted that a multi-stakeholder approach could provide the essential critical mass needed to develop actions that truly address the challenges of PIDs. He stated that the S&D group will develop a special working group on access to medicines and further proposed that they should examine the unique issues facing access to high quality medicines for PID patients. Mr. Zorrinho particularly endorsed the third principle of PID on international collaboration for scientific research by stressing how vital increased PID Research is for the future of PID patients. Finally, Mr. Zorrinho recommended new actions on newborn screening for Severe Combined Immunodeficiency (SCID) and welcomed a further discussion with Ricardo Pereira, the president of the Portuguese patient association.



¹ Chapel H. et al (2014) *Primary immune deficiencies – principles of care*, *Frontiers in Immunology* Volume 5, Article 627



Mr. Johan Prévot, IPOPI Executive Director, provided the audience with an international perspective on the PID Principles of care. He described the scarcity of global knowledge at medical, political and societal levels and explained how this leads to disparities in screening, treatment and care worldwide. A specific emphasis was placed on disparities in the European Union as he elaborated particularly on the example of newborn screening practices and immunoglobulin treatment levels in Member States. Mr. Prévot listed tools that EU policy-makers should mobilize to support people with PIDs to establish standards of care. He highlighted EU health policies on rare diseases, blood

and tissues, cross-border healthcare, advanced therapies and pharmaceuticals. He also discussed the importance of EU research policies and public spending monitoring as opportunities to address challenges surrounding PIDs. Mr. Prévot then described actions that IPOPI is supporting at the EU level including a Horizon 2020-funded research programme on the development of gene therapy, gaining policy support from the EU on newborn screening, working towards developing a European Reference Network and conducting a survey on the burden of treatment to disseminate patients' views in relation to current discussions on HTA and reimbursement policies.



Prof. Martin Van Hagen, Professor at the Erasmus MC University Medical Centre in Rotterdam, spoke from his academic perspective on the role of specialised centres (Principle of care no. 1) and the importance of registries (Principle of care no. 2). He noted that national PID expert centres are the mainstay in the regional diagnosis and treatment of PIDs and also highlighted that rare diseases need registration especially in the case where there are multiple symptoms, comorbidities and limited treatment. Prof. Van Hagen elaborated that national registries are important tools for disease prevalence monitoring and detection of areas of low-diagnostic rate. In addition, he emphasised the need for high quality products that can

provide patients with a wide-variety of treatments. Finally, Prof. Van Hagen agreed with Mr. Zorrinho MEP that international collaboration is a key element to address rare diseases.



Prof. Bobby Gaspar, from the Institute for Child Health at the University College of London, described the potential benefits of enhanced international collaboration for patients (Principle of care no. 3). Sharing data and knowledge would improve the level of awareness on PIDs and increase accuracy of clinical studies. Given the diversity of immunodeficiencies, Prof. Gaspar noted the necessity of collecting and sharing data at the international level. Furthermore, he



stressed that patients' quality of life may be improved by increasing the opportunities for cross-border treatments. Prof. Gaspar then spoke about PID management and treatment options (Principle of care no. 5). The Principles of Care recommend three treatments to be made available worldwide: immunoglobulin replacement therapy, haematopoietic stem cell transplant (HSCT) and gene therapy. He highlighted that Ig products are essential medicine as recognized by the World Health Organisation and recommended that access to those products should be at the forefront of discussions during engagement with policy-makers. While HSCT treatments can be applied to patients born with severe forms of PID and can have tremendous results, he went on to say gene therapies offer safer conditions and are increasingly seen as a very good therapeutic option.



Mr. Ricardo Pereira, President of the Portuguese Association of Primary Immunodeficiencies (APDIP), provided an insight into the importance of patient groups and shared his experience of living with a PID. Mr. Pereira spoke of the role of patient organisations in providing guidance, support and awareness, Collaborating with healthcare professionals in order to give PID patients a voice and allow them to bring their perspective to policy makers was also mentioned. Throughout his presentation, he indicated that PID patients encounter numerous obstacles due to their medical

condition in the work place and during their daily routines. To address these issues, APDIP has been focusing on the recognition of the status and conditions of patients with PIDs both nationally and globally. The organisation has been raising awareness in Portugal through national TV programmes, organising events, social media, and a future Youtube channel.



Dr. Susana da Silva, immuno-allergologist at the Hospital Santa Maria in Lisbon, showcased challenges and good practices in Portugal for patients with PIDs. Even though Portugal has had a national registry for some years, the country suffers from a lack of awareness which leads to late diagnosis, inadequate treatments and care and inaccurate policies. Dr. da Silva joined Mr. Zorrinho MEP and Mr. Prévot in stressing the importance of new policies implementing PID screening for newborns. She also highlighted that universal BCG vaccination at birth is particularly harmful for newborns with PIDs. She went on to say that people in Portugal benefit from a good access to Ig products which are mostly reimbursed but

she also noticed that improvements on access to HSCT are needed in the years to come. Dr. da Silva presented the opportunity for Portuguese medical and research staff on immunology to work with colleagues, to get trained in reference centres and establish connections to discuss difficult cases and develop clinical research. She concluded her presentation by welcoming the 2018 ESID Meeting in Lisbon as a good opportunity to increase national awareness and to improve the national policy on BCG for newborns.

Prof. Cedric Hermans, Head of the Haematology Department at the University Clinic of Saint-Luc, Belgium elaborated on the Principles of Care for Haemophilia published in 2008. He gave valuable feedback on the implementation and dissemination of the Haemophilia principles, which may serve as a good example for the PID Principles of care. Prof. Hermans noted that the principles of haemophilia care are generally applied throughout Europe and all patients have access to adequate therapies for haemophilia. However, even in the haemophilia field national registries are not used everywhere and not all moderate and mild haemophilia patients are treated in designated centres. The activities of the European Haemophilia Network (EUHANET) were also briefly described. This project aims at establishing a network of haemophilia centres to work towards improving the care of European citizens with inherited bleeding disorders. This initiative could serve as an inspiration for the future development of better frameworks of care for PID patients. Prof. Hermans concluded with the announcement of the 9th annual congress of the European Association for haemophilia and allied disorders which will take place in Malmo in February 2016.





3. Conclusions

Mrs. Drabwell drew several important conclusions from the discussions that took place during the event, notably the continuing need for strong patient groups in all countries, and the ever-present need for policy-makers to reconsider newborn screening issues. Furthermore, she noted that the Principles of Care are an important tool to make sure policy makers are aware of the issues surrounding the care of PID patients. She indicated that there will be follow-up actions with both Mr. Faria and Mr. Zorrinho MEPs offices to put forward some actions to spread the message in the European Parliament to their colleagues working on health policies. Parliamentary Questions to the European Commission should also be considered to outline the lack of action implementing the Commission's recommendations on newborn screening. The meeting was concluded by stressing the major value of the publication on PID Principles of Care for the future of strong PID frameworks of care and all participants were encouraged to make reference to this document in all meetings on PIDs in the future.



List of participants

Mr. Carlos Zorrinho, Member of the European Parliament, host of the PID Forum

Mr. Paulo Couto-Ferreira, Parliamentary Assistant to José Inácio Faria MEP

Mr. Silvino Monteiro Cardita Gomes da Silva, Parliamentary Assistant to Carlos Zorrinho MEP

Ms. Joana Benzinho Santos, Parliamentary Assistant to Carlos Zorrinho MEP

Mr. Abdelhakim Lattef, Parliamentary Assistant to Gilles Pargneaux MEP

Mrs. Jose Drabwell, IPOPI

Mr. Johan Prévot, IPOPI

Ms. Magda Lourenço, IPOPI

Ms. Edith Klapwijk, BOPPI

Mr. Ricardo Santos Pereira, APDIP

Prof. Bobby Gaspar, University College London – Institute of Child Health

Dr. Susana Lopes da Silva, Centro de Imunodeficiências Primárias, Lisboa

Prof. Cédric Hermans, Cliniques universitaires de St Luc, Bruxelles

Prof. Martin Van Hagen, Erasmus MC University Medical Centre Rotterdam

Ms. Lena Bera, CSL Behring

Ms. Françoise Rossi, IPFA

Mr. Karl Petrovsky, PPTA

Ms. Melina Raso, Health First Europe

Mr. Cristian Luțan, Rohde Public Policy

Mr. Christopher Greenop, Rohde Public Policy

Mr. Yordan Aleksandrov, Rohde Public Policy

Mr. Pierre Baty, Rohde Public Policy