

## 5<sup>th</sup> PID Forum

# A comprehensive framework of care and policy for PIDs

4<sup>th</sup> December 2014

European Parliament, Brussels

Hosted by MEPs Dr. Cristian Silviu Buşoi and Dr. Bolesław Piecha

## Report



*From left to right: dr. Artermiza Bâldea, dr. Nizar Mahlaoui, dr. Cristian Buşoi MEP, Mr. Johan Prévot, dr. Bolesław Piecha MEP, Ms. Jose Drabwell*

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INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

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## Conclusions of the 5<sup>th</sup> PID Forum

- Primary Immunodeficiencies (PIDs) are a group of around 250 genetic rare disorders in which the immune system does not work adequately or at all.
- PIDs are a unique and complex group of rare diseases that involve a large spectrum of scientific and care disciplines.
- Living with a PID or having a family member with a PID is challenging in many countries due to the difficulties in having access to timely and adequate screening, diagnosis, treatment and care.
- Support is needed to keep on launching and developing initiatives that promote improving treatment and care of patients with PIDs.
- Patient participation is essential for the development of policies that adequately support patients with PIDs at national, European and international level.
- PIDs Principles of Care aim at providing high-level standards and disseminate them across PID patients, healthcare specialists and policy makers.
- We need a comprehensive approach on PIDs that allows for a swift combination of policy making and care delivery.
- This comprehensive approach can only come from the continued and sustained collaboration of patients, healthcare professionals, industry and policy makers.
- Only with collaboration we can achieve a comprehensive framework for PIDs from a care and a policy side!
- IPOPI looks forward continuing collaborating with the European Parliament, European Commission and WHO to ensure that patients with PIDs and their families can lead normal lives.

## 1. Introduction

On Thursday 4 December 2014, MEPs Dr. Cristian Silviu Buşoi (EPP, Romania) and Dr. Bolesław Piecha (ECR, Poland) hosted the 5th Primary Immunodeficiency Forum in the European Parliament entitled: “A comprehensive framework of care and policy for PIDs”. The meeting was organised in collaboration with the International Patient Organisation for Primary Immunodeficiencies (IPOPI) and placed a specific emphasis the methods by which to deal with Primary Immunodeficiencies (PIDs), from a patient or a physician perspective.

The event provided ample opportunity for patients, health professionals, and patient organisations, representatives from the European Parliament and industry to convene to discuss the challenges facing PID patients in Europe today. All participants in the meeting agreed that the gap between policy and care must be bridged by patients and healthcare professionals so as to ensure those patients and their families are afforded the ability to lead a normal life.



## 2. Summary of discussions

Dr. Buşoi commenced the meeting by highlighting the pertinence of the meeting topic as the European Union is at an important juncture with the commencement of a new term of the European Parliament and the European Commission. Dr. Buşoi went on to say that the ultimate goal of the meeting would be to showcase challenges and good examples of how legislation or policy and care do not have to be in two different silos but rather should go hand-in-hand. The MEP stressed that a comprehensive framework for both care and policy can be achieved through an enhanced collaboration across these two areas. Furthermore, the MEP referenced his own dual experience of having been President of the National Health Insurance House of Romania and an MEP in order to highlight the

importance of EU action in ensuring that EU institutions and Member States work together in the field of health and, more specifically, in rare diseases. Dr. Buşoi also mentioned the level of inefficacy which can be attributed to rare diseases across the 28 Member States and, as such, stressed that much needed mobilisation of resources can only be efficiently achieved through a coordinated European approach.

Dr. Boleslaw Piecha MEP underlined the complexities of Primary Immunodeficiencies which can be attributed to the mismanagement and mis-diagnosis of this group of disorders. In this regard he remarked that this complexity in care translates into policy with numerous policy dossiers and policy areas being of direct relevance. Dr. Piecha also mentioned some of the



dossiers currently being discussed that are of direct relevance for PID patients and patients with rare diseases in general, including: The Transparency Directive and the European Reference Networks. Lastly the MEP indicated that many other political dossiers that have been adopted in the past decade might be subject to revision or update during the present mandate, including: the Blood Directive, the Regulation on Advanced Therapy Medicinal Products, the Paediatric Regulation and the Health Technology Assessment.



Mr. Johan Prévot, Executive Director of IPOPI, briefly described how patients affected with PIDs often live for many years with their condition before being diagnosed, being treated for the symptoms of their disease, rather than for the disease itself. This is due to the lack of awareness and knowledge about this group of around 250 different diseases. Mr. Prévot reiterated the importance of ensuring that PIDs are well understood so that policy encompasses care needs, and that patients with PIDs receive the diagnosis, treatment and care they need, and are not impaired by political/administrative hurdles and can effectively live a normal life

Mr Prévot also elaborated on past successful examples of advocacy campaigns aimed at bridging the gap between policy and care, including: advocacy campaign at EU level to promote SCID newborn screening, national initiatives in EU Member States calling for pilot projects or supporting the existing pilot projects on SCID (Severe Combined Immunodeficiency) Newborn screening, and the re-instatement of Immunoglobulin in the WHO Essential Medicines List. Mr Prévot concluded that current challenges facing PID patients can only be overcome through taking a comprehensive approach which should encompass a swift combination of policy making and care delivery. This comprehensive approach will only be borne from a collaboration of patients, healthcare professionals, industry and policy makers. He emphasised the fact that a lack of dialogue leads to unexpected hurdles for patients and their families

Dr. Nizar Mahlaoui, Manager of the French Centre de Référence Déficiences Immunitaires Héritaires (CEREDIH), spoke from his perspective as a medical practitioner regarding diagnosis, treatment and care. He further emphasised the recommendations of the previous speakers regarding the need for a comprehensive tailored framework at both national and EU levels so as to ensure that PID patients receive a quick and accurate diagnosis. Throughout his presentation, Dr. Mahlaoui discussed how patients with missed or a delayed diagnosis of PIDs would undergo unnecessary suffering and depend heavily on healthcare resources. Dr. Mahlaoui recommended the establishment of PID medical centres catering to both paediatric and adult patients as a method of addressing under-diagnosis, a problem which is exacerbated by the diversity of PID manifestations (autoimmunity/inflammation, granuloma, and infections). He also highlighted the proven benefits of familial screening through centres of reference as an example of how best to provide patients with access to treatment, training for physicians to identify and diagnose PIDs and support



spreading best practices regarding the treatment of the conditions. Lastly Dr. Mahlaoui espoused the benefits of the application of a prophylactic regimen as a method of preventing infections for PID patients.

Dr. Lisa Bâldea offered insight into the current obstacles facing PID patients from a national perspective and spoke about her involvement with ARPID, the national PID organization from Romania. The objective of ARPID is to obtain access to early diagnosis and adequate treatment and care for PID patients. Dr. Bâldea shed light on the numerous obstacles encountered by PID patients in Romania, including, but not limited to, the absence of a SCID screening program for newborns and delayed diagnosis. Additionally, access to treatment for new patients is difficult owing to restrictions in the public budget. In keeping with the overall theme of the forum, Dr. Bâldea further highlighted the importance of creating a comprehensive framework so as to bring patients, doctors and policy makers together to work for a better future for patients with PIDs & their families.



Ms. Jose Drabwell, President of IPOPI, provided an overview of the current landscape and perception of patients in relation to their circumstances, outlooks and treatment needs with PID from a global perspective. In this regard, Ms. Drabwell referenced the 2012 IPOPI PID Patient Needs and Outlooks Survey. Ms. Drabwell informed the attendees that this survey illuminated the reality of living with a PID in 21 different countries (5 continents; patients & carers). The survey also revealed the importance of providing tailored treatment options and modes of administration to ensure individual patient needs are best met. Given the evident complexities of PIDs, Ms. Drabwell placed particular emphasis on the need to put into writing golden standards for treating PIDs, which was the aim of the PIDs Principles of Care<sup>1</sup>.

Finally, Ms. Drabwell discussed the need for enhanced collaboration with and policy makers if we are to achieve high-level standards for PID patients and healthcare specialists.

After the presentations there was a lively discussion on several pertinent topics. Mr. Adrian Goreki, representing the Polish PID Patients association (Immunoprotect), contributing by highlighting the problem faced by patients in Poland regarding access to immunoglobulins. Authorities undertaking tenders to acquire the treatment, consider these plasma-derived



<sup>1</sup>Primary immune deficiencies – principles of care was published by the journal *Frontiers in Immunology* on 15<sup>th</sup> December 2014 and can be accessed here: <http://journal.frontiersin.org/Journal/10.3389/fimmu.2014.00627/abstract>.

products as generics, which creates an administrative barrier to access. On the subject of future initiatives, Ms. Amanda Bok, Chief Executive of the European Haemophilia Consortium (EHC) emphasised the, which is essential for therapeutic medicinal products for PIDs. Ms. Bok also reiterated that enhanced involvement of PID patients in policy development is imperative. On a similar note, Dr. Paul Strengers (PPTA) stated that the forthcoming review of the Blood Directive (2002/98/EC) must be balanced so as to not hamper the collection of plasma in Member States. Dr. Buşoi intervened and expressed his willingness to work together to ensure that the final parliamentary report provides an adequate framework for PID patients and their families.

Mr. Ruediger Gatermann (CSL Behring) underlined that a concrete approach is needed to address the national situation of patients with PIDs. Mr. Prévot, responding, agreed and emphasized that once the Principles of Care for PIDs would be published, ways of implementing them at a national level would have to be pursued.

Access to medicines and reimbursement were also amongst the topics discussed- Ms. Edith Klapwijk, Chairperson of the Belgian Organisation for Patients with Immunodeficiencies (BOPPI) spoke about her adult children's challenges in accessing medication and stressed that the intentions of Governments must be to keep the cost of medications down.

The MEPs in their concluding remarks once again stated their commitment towards ensuring that in upcoming policy or legislative proposals, the voices of PID patients are heard.

Dr. Cristian Silviu Buşoi  
Member of the European Parliament



Dr. Boleslaw Piecha  
Member of the European Parliament



### 3. List of participants

Dr. Cristian Buşoi, Member of the European Parliament, co-host of the PID Forum  
Dr. Boleslaw Piecha, Member of the European Parliament, co- host of the PID Forum  
Mr. Jakub Lecki, Parliamentary Assistant to Boleslaw Piecha  
Ms. Elisabeta-Ana Covaci, Parliamentary Assistant to Cristian Buşoi  
Ms. Elena Cătălina Radu, Parliamentary Assistant to Cristian Buşoi  
Ms. Jose Drabwell, IPOPI  
Mr. Johan Prévot, IPOPI  
Dr. Nizar Mahlaoui, CEREDIH  
Dr. Artemiza Bâldea, ARPID  
Dr. Mihaela Bătăneanţ, ARPID  
Mr. Adrian Gorecki, Polish PID Association  
Ms. Edith Klapwijk, BOPPI  
Ms. Laura Savini, EHC  
Ms. Amanda Bok, EHC  
Ms. Leire Solis Garate, IPOPI  
Dr. Andrea Silenzi, World Health Organisation  
Ms. Melina Raso, Health First Europe  
Ms. Lena Bera, CSL Behring  
Mr. Ruediger Gatermann, CSL Behring  
Ms. Sonia Florian, Hill & Knowlton  
Dr. Paul Strengers, IPFA  
Mr. Karl Petrovsky, PPTA  
Mr. Bruno Santoni, PPTA  
Mr. Charles Waller, Rohde Public Policy  
Mr. Cristian Luţan, Rohde Public Policy  
Ms. Ailish Tierney, Rohde Public Policy  
Mr. Christopher Greenop, Rohde Public Policy