



IPOPI's 7th PID Forum

Tackling PID Care Disparities in the EU

Wednesday 12 October 2016
European Parliament, Brussels (Belgium)

Co-Chaired by MEPs Dr Anna Záborská, Dr Elena Gentile and Ms. Karin Kadenbach

Report



Introduction

On Wednesday 12 October 2016, Members of the European Parliament (MEPs) Dr Anna Záborská, Dr Elena Gentile and Ms. Karin Kadenbach co-hosted the 7th IPOPI Primary Immunodeficiencies (PID) Forum on “Tackling PID Care Disparities in the EU” at the European Parliament in Brussels (Belgium). The meeting, co-organised by the International Patient Organisation on Primary Immunodeficiencies (IPOPI) aimed to address the current challenges of care disparities within the primary immunodeficiency’s community.

During the Forum, representatives from the European Parliament engaged with patient representatives and healthcare professionals from around the European Union and familiarized themselves with the issues of PID patients, while discussing the best practices in the field. The discussion was appreciated by all participants and Members of the European Parliament (MEPs) agreed to assist the community with further actions. Their political support was highly appreciated, as the European Parliament and all relevant stakeholders need to continue their collaboration with the aim of improving the quality of life of PID patients.

The event addressed PID care challenges and practices in Italy, Slovakia, Romania and Austria and was an opportunity for patient representatives to meet with their respective MEPs to discuss the most appropriate way in improving the treatment of PID patients. Bringing different perspectives to the issue was a corner stone of the Forum, which updated the MEPs with the latest developments in the field, including severe combined immunodeficiency (SCID) newborn screening which occurs in three Italian regions and will be implemented in the Spanish region of Catalonia as of 2017.

Summary of the interventions



Dr Anna Záborská MEP opened the meeting by elaborating on the need of addressing the challenges of PID patients. She confirmed her political support to patients and highlighted the need of assistance from policy-makers within the European Parliament. Dr Záborská stressed the need of further collaboration with EU institutions, healthcare professionals and patients. She was very pleased about the wide range of stakeholders gathered at the Forum and expressed her willingness to continue supporting the PID community.

Dr Elena Gentile MEP welcomed participants and confirmed her political commitment to the cause of improving treatments for PID patients. She was very passionate about the issues faced by the disparities of care within the European Union and highlighted the key role of the European Parliament in providing a platform for raising further awareness on these conditions. As a policy-maker, she emphasised the need of following up on political actions, while emphasizing the activities which need to be undertaken in addressing the discrimination of PID patients in Europe. Dr Gentile elaborated on the cost of healthcare services, further stating that prevention, early diagnosis and care are an investment rather than an expense. She further



committed to write a letter to the Italian Minister of Health informing him about the platforms created through the IPOPI Forums and bringing to his attention the urgent need of action in the field of the PIDs. These initiatives are the perfect framework at the EU level for discussing these challenges and policy-makers need to start working on the suggestions brought to them by the patient community.



Ms. Jose Drabwell, IPOPI's Chair, presented her personal experience as a patient in the impact of diagnosis and treatment. Since early age, as many PID patients, she was facing the numerous conditions, such as polio, chronic kidney problems and severe chicken pox. As years pass by without receiving adequate treatment, PID patients' health deteriorates and can experience several severe health issues, such as gastro-intestinal issues, bronchitis, extreme tiredness and asthma, amongst others. If not diagnosed early, PID patients lose many years through ill health, continue to face unnecessary medication and operations while constituting a tremendous costs of the healthcare system. However, early diagnosed patients can enjoy normal day-to-day life. To this end, it is imperative to raise awareness for PID worldwide in cooperation with the European

Parliament and all PID stakeholders. Working for accurate and faster diagnosis for children and adults should be a top priority for both politicians and healthcare professionals in order to ensure an adequate quality of life for these patients, while integrating them at the fullest in the community.

Mr. Johan Prévot, IPOPI Executive Director, provided the audience with insights on the importance of availability and choice of treatments for PID patients. He elaborated on the treatment options for this community, including immunoglobulin replacement therapy, bone marrow transplantation, antibiotics, gamma interferon, and gene therapy, while providing a general overview of the treatments. Mr. Prévot noted that PID patients require availability of all therapies as to ensure that they receive the most appropriate care for them and that each patient requires an individualised health plan adapted to his/her PID condition. To this end, and in order to treat a patient, earlier diagnosis is of utmost importance. He then discussed the disparities in newborn screening in the EU, while giving an overview graph of the current situation. It was furthermore stated that stakeholders need to ensure that patients have access to a wide range of products so as to receive the most suitable for them. The reality is that treatment leads to improvement in patients' health, but also have some associated burdens such as travel, social, emotional and personal. Thus, understanding the burden associated with the different immunoglobulin route scan help patients in collaboration with their doctors, to make an informed choice on their respective therapies.





Dr Eleonora Gambineri, from Anna Meyer Children's Hospital in Florence (Italy), presented the healthcare professionals' perspective on diagnosis, treatment and care disparities in Italy. Following an overview of the PID diseases and their severity, Dr Gambineri elaborated on the link between early and accurate diagnosis and better therapeutic options. She noted that the encouragement of early diagnosis drastically improves the quality of life and survival rate of patients, before presenting the main points of the PID Principles of Care (e.g. the role for specialised centres; the importance of registries; the need for multinational research, the role of patient organisations, management and treatment options, and important considerations for developing countries and suggestions for implementation). She highlighted that at the international level coordination and networking is the way forward in addressing the issues of PID patients. Dr Gambineri also presented the

criteria for regional specialist PID centres for adults/children emphasising the importance of effective integrated care with primary and secondary healthcare services; the commitment to training and professional development for sustainability; the PID research; and the primary responsibility for clinical and observational trials in PIDs. She continued by discussing the European Reference Networks and the missing common definition of what a centre of reference is among the Member States. She also presented an Italian overview of PID care, where there is an established network to share knowledge regarding diagnosis, patient care and management in PIDs (e.g. IPInet). Through this system, diagnosis and treatment is generally available all over Italy. However, there are still too many centres which do not fully comply with internationally agreed standards of care. She recommended that there should be a selection on two national PID specialised centres where high standard of care/research are reached, to provide advice to other centres. Furthermore, with the support of the AIP, the representatives of IPInet's centres have joined common forces to formulate and propose common recommendations for diagnosis and treatment of PIDs to be applied at the national level.

Dr Katarina Fedorova, from the Slovak Association of Patients with Primary Immunodeficiency, made an intervention on the challenges facing the new EU Member States from a patient's perspective. She elaborated on the need of early diagnosis, reimbursement policies, patient information and addressing the discrimination barriers. Dr Fedorova discussed the urgent need of raising further awareness among general practitioners (GPs) in order to avoid undiagnosed and misdiagnosed patients. In addition, at this stage legal healthcare provisions are quite vague and patients are not aware of their rights. She discussed the current situation for the reimbursement of medicines and the challenges for some patients in Slovakia who are reimbursed if only the medicines figure on the list of categorized medicines, which can be changed up to 12 times per year. Dr Fedorova informed the group that the Slovak Ministry of Health is currently preparing a change in the legislation in regards with the medicines reimbursement provisions and the list of categorized medicines. However, there is still a lot of uncertainty about the final version of the legislation. The proposal for amending the law will be brought up during the November 2016 session of the Slovak National Council. In order to ensure the correct lines of these provisions, Dr Fedorova urged



for a concrete dialogue with members of the National Parliament and the Ministries of Health and Social Affairs.

Dr Baldea, from the Romanian Association for Patients with Primary Immunodeficiencies (ARPID), made an intervention on the challenges experienced by the Romanian patients. Dr Baldea presented ARPID, which currently includes 60 patients, children and adults, who are supported only by few doctors and medical centres in Romania. She emphasised the need of having access to timely and adequate screening, diagnosis, treatment and care. She noted as well the current challenging situation in Romania with delayed diagnosis, absence of SCID screening programme for newborns and absence of a national registry for PIDs. She elaborated on the benefits of early diagnosis as cost saving method in the long-term. Furthermore, access to treatment for new patients in Romania is still a problem due to the limited public budget, although improvements have been made in the recent years. All treatments are only provided in hospitals which means higher costs for the society and time spent by patients away from work or from their families. Despite the challenges, ARPID continues its support to PID patients. Dr Baldea informed the participants that in 2014 in Tg. Mures the first national meeting for the Romanian patients was organised, followed by a National Conference for PIDs. From 2013, the association has started to produce informative booklets about PIDs and as of this moment, they have nine new booklets ready for patients, parents, doctors and schools. Foreseen actions of APRID include promotion of early and correct diagnosis, raising PID awareness, and the encouragement of establishment of national PID registry.



Dr Peter Ciznar, from the Children's University Hospital in Bratislava (Slovakia), provided a physician's perspective on the challenges faced by the new EU Member States in relation to PID care. He discussed presence of the PID centres, availability of newborn screening, genetic testing and drug reimbursement policies in Slovakia. Dr Ciznar noted that there are three Slovak PID centres. He informed the Members of the European Parliament that the only transplantation unit for children in Slovakia was established in 1995 at the Children University Hospital in Bratislava. The current challenges related to PID care in Slovakia include the lack of national registries, absence of SCID newborn screening, limited genetic testing availability, absence of good reimbursement policy, low research resources. He urged for a world wide access to adequate care, networks, training, empowerment of

patients 'associations, development of targeted therapies and gene therapies, long term assessment of patients' sequelae, a need to pursue further research.

Discussion

Following the presentations from participants and the Members of the European Parliament, the Forum's Chair, Jose Drabwell, opened the floor for discussions. During this session, participants elaborated on the political support provided in their respective countries to patient organisations. In Romania, ARPID is encouraging the exchange of information between patients and doctors, while raising awareness on the PID conditions. Overall,



IPOPI's national member organisations are working towards the establishment of a firm collaboration with the local governments aimed at raising further awareness on the topic while educating healthcare professionals. To this end, more needs to be done, but it is clear that these are the steps in the right direction. Mr. Prévot expressed his satisfaction with the direction that the European Reference Networks have taken. However, even at the European level, there is still room for improvement, such as the revision of the Blood Directive, which is still unclear when it would take place, or the implementation of SCID newborn screening in the European Union. One thing is certain – that there is need to focus and consultation of PID patients for any piece of legislation and policy that affects them.

Participants also highlighted the need of more PID data to ensure that the right decisions at political level are taken. The importance of bringing the European Commission to the discussions was also noted.

Concluding remarks

Dr Ida Aringer, from the office of MEP Karin Kadenbach, made a concluding intervention on Ms. Kadenbach's behalf. She emphasised the excellent example of the Forum on bringing various stakeholders to the table, as diverse opinions are a crucial step in understanding and addressing the challenges experienced by PID patients. Dr Aringer informed the participants that Ms Kadenbach will bring the points discussed at the Forum to her colleagues within the Interest Group on Maternal and Neonatal Health and was very pleased with the platform's ability to disseminate the information among numerous stakeholders.

Dr Anna Zaborksa MEP closed the event by summarizing the key point discussed:

- We need to speak more about PIDs, as the awareness of the disease remains low, also among the health professionals
- Treating a patient, means having diagnosed him/her earlier previously. Knowing about the disease is the first and foremost step in effective management of the disease
- Each patient with PID requires an individual health plan adapted to his/her PID, which may involve many different treatments, to ensure adequate quality of life
- Patients across Europe should have much better access to treatment, which should be fairly reimbursed
- Each country should be aimed at having specialised centres and PID registries.

List of participants

- Dr Anna Záborská, Member of the European Parliament
- Dr Elena Gentile, Member of the European Parliament
- Dr Ida Aringer, Assistant to Ms. Karin Kadenbach, European Parliament
- Mr. Toni Kapelina, office of Ms. Biljana Borzan, European Parliament
- Mr. Francesco Losappio, office of Dr Elena Gentile, European Parliament
- Mr. Claudio Vitale, office of Dr Elena Gentile, European Parliament
- Mr. Peter Stach, office of Dr Anna Zaborska, European Parliament
- Ms. Jose Drabwell, IPOPI
- Mr. Johan Prévot, IPOPI
- Dr Eleonora Gambineri, "Anna Meyer" Children's Hospital Florence (Italy)
- Dr Katarina Fedorova, Slovakian Association of Patients with Primary Immunodeficiency
- Dr Artemiza Baldea, Romanian Association for Patients with Primary Immunodeficiencies (ARPID)
- Dr Peter Ciznar, Children's University Hospital Bratislava
- Mr. Peter Jandus, European Society for Immunodeficiencies (ESID)
- Ms. Edith Klapwijk, BOPPI
- Ms. Martine Pergent, IPOPI
- Mr. Andrea Gressani, AIP and IPOPI
- Ms. Karin Modl, OSPID
- Ms. Leire Solis, IPOPI
- Mr. Ruediger Gatermann, CSL Behring
- Mr. Toon Digneffe, Shire
- Mr. Sandor Toth, PPTA Europe
- Mr. Charles Waller, Rohde Public Policy
- Mr. Kit Greenop, Rohde Public Policy
- Ms. Jelena Malinina, Rohde Public Policy
- Mr. Yordan Aleksandrov, Rohde Public Policy