



## REPORT

### **IPOPI's 9<sup>th</sup> EU Primary Immunodeficiency Forum “Growing Older with PIDs: Issues of Transitional Care and Ageing” Thursday 22 June 2017 European Parliament, Brussels (Belgium)**



## Introduction

On Thursday 22 June 2017, IPOPI organised its 9<sup>th</sup> EU PID Forum on Growing Older with Primary Immunodeficiencies at the European Parliament in Brussels (Belgium). Chaired by MEP Dr Elena Gentile (S&D, IT) and supported by Carlos Zorrinho (S&D, PT), the meeting represented an opportunity for the European Commission, the European Economic and Social Committee (EESC), Members of the European Parliament (MEPs), patients, academics and healthcare professionals to discuss the omitted topic of transition of care and ageing of people with Primary Immunodeficiencies (PIDs).

Transition from paediatric PID care to adult care has many kinds of implications for people with PIDs, such as physical, social, educational, and domestic among others. In PID management, transitional care is often inadequately planned ignoring individual needs of the patient, even though it is a cost-effective way of ensuring compliance with treatment. Furthermore, new advances in diagnosis and treatment have led to an increased number of people with PID reaching old age. To this end, stakeholders were brought together to elaborate on both adolescent and elderly PID patients' needs and their ability to independently manage their own condition.

MEPs and participants were invited to work with IPOPI and crystallise the main points of the discussions in a set of recommendations that can be used by patients and patient groups, healthcare professionals and decision makers in their efforts of improving the quality of life of PID patients.

## Opening remarks



**Dr Elena Gentile MEP**, opened the 9<sup>th</sup> EU PID Forum by thanking IPOPI for co-organising this event and Mr. Carlos Zorrinho for supporting it. Dr Gentile stressed the enormous inequalities in access to diagnosis, especially for PID patients. She emphasised the importance of the European Parliament and the European Commission in dealing with these growing health inequalities within and between EU healthcare systems. In her opinion, transition of care from youth to adult should be addressed with a vision of reducing these inequalities. Although often overlooked, this process

is also a cost-effective way of ensuring compliance with treatment of the individual.

**Leire Solis from IPOPI**, thanked Dr Elena Gentile and Carlos Zorrinho for their dedicated support of the PID community and all participants and experts who joined the discussion in the European Parliament. She further set the scene of the 9<sup>th</sup> PID Forum by providing a comprehensive overview of the challenges faced by these rare disease patients. With clinical immunology being a relatively young discipline, there are increasing number of patients diagnosed and registered. Discrepancies amongst national health systems and the stigma of PIDs being perceived as a paediatric issue, force the need to turn the attention to specific stages in life where support is key to ensure the well-being of the patient. To this end, identifying best practices for the transition of care and elderly care of persons with a PID have a central role in improving patients' quality of life. For this reason, concrete recommendations on the topic would be of utmost importance.







**Dr Jaroslaw Waligora from the European Commission's Directorate General on Health and Food Safety**, outlined the Commission's activities in the EU rare disease policy (e.g. investment in this area through European research funding programmes such as the 7<sup>th</sup> Framework Programme for Research and Technological Development and Horizon2020). He noted the importance of effectiveness and efficiency in this area of highly specific and limited number of patients. After elaborating on the main policy dossiers in rare diseases, he stated that as of 2016, 23 European Member State have adopted national rare disease plans. Dr Waligora also elaborated on the Commission's efforts in supporting the interoperability of existing rare disease registries through the EU platform on rare disease registration. In relation with transition of care for rare disease patients, he expressed his strong feeling that the European Reference Networks (ERNs) should

ensure continuity of care for paediatric, adolescent and adult patients within each overarching thematic network. Dr Waligora informed the participants that the Commission's rare disease action is focused on (1) continuing the implementation of the policy priorities and support the work of the Commission Expert Group on Rare Diseases<sup>1</sup>, (2) contributing to solutions to ensure an appropriate codification of rare diseases in health information systems, and (3) supporting the further development and sustainability of the Orphanet database on rare diseases.

**Alain Coheur, Director of European and international affairs for Solidaris Mutual Health Insurance (Belgium) & member of the European Economic and Social Committee (EESC)**, presented the

field analysis of the rare disease patient pathways within the EMRaDi project. The Euroregion Meuse-Rhine (EMR) Rare disease (RaDi) project is a cross-border and cross sectoral initiative comprising 16 partners and running from 2016 to 2019. The initiative, which focuses on 8 rare diseases from 4 categories (neurological, haematological, syndromal and metabolic) was presented as a useful best practice for the PID community by looking at improving patient pathways, building networks and facilitating diagnosis and treatment. The project has a triple objective – (1) increase the transparency of needs and availability of services in the field of rare diseases in the EMR, (2) develop EMR models of rare disease patient pathways to elaborate patient-orientated recommendations in synergy with national and European developments, and (3) improve the network of health care providers, health insurance providers and patient organisations. With both IPOPI and the EMRaDi initiative having similar long-term aim of improving the quality of life of rare disease patients, the presentation was well received by the participants. More information on the initiative can be consulted [here](#).



<sup>1</sup> The European Commission Expert Group on Rare Diseases was established in 2013 to support the development of legal and policy instruments, advise on potential European actions and improvements in this field, monitor, evaluate and disseminate the results of the measures taken at Union and national level, exchange best practices, amongst others. More information is available [here](#).

## Transition of care for primary immunodeficiency patients – from youth to adult

The IPOPI 9th PID Forum was divided in two main panels. The first panel focused on the specific challenges of transition of care from paediatric to adult care. Participants elaborated on the issues faced by patients in terms of changing doctors, hospitals and healthcare professionals.



**Andrea Gressani from the Associazione Immunodeficienze Primitive Onlus (AIP)**, presented a patient perspective on transition of care for PID patients in Italy. Mr. Gressani noted that transition from youth to adult is already problematic for healthy people. For PID patients it is even more complicated with changing doctors/caregivers and hospitals among others. One of the main challenges of Italian patients is the limited amount of healthcare specialists with experience in patient specific PIDs. Mr. Gressani invited patients to collect as much data as possible about their specific PID history in order to share it with their new doctors/caregivers. In addition, paediatric

physicians should spend more time in exchanging knowledge and information with the potential adult physician, aiming at passing the trust of the patients in the new doctors. Last but not least, the role of caregivers should not be omitted, as they have the crucial objective of making patients feel better.

**Dr Nizar Mahlaoui from the Necker-Enfants Malades University Hospital in Paris**, elaborated on the transitional care for adolescents and young adults with PIDs from a physician's perspective. Dr Mahlaoui stated that health care providers, researchers, insurance companies, and authorities lack essential knowledge of rare diseases. Thus, further training of medical doctors in terms of symptom awareness is a crucial point for ensuring an adequate transition of care. Dr Mahlaoui noted the UK Department of Health definition of transition of care – “A purposeful, planned



process that addresses the medical, psychosocial and educational/vocational needs of adolescents and young adults with chronic physical and medical conditions as they move from child centred to adult oriented health care systems”<sup>2</sup>. He further stated that usually transition should take place around the age of 18 but with flexibility that takes into consideration the maturity of the patient. An important aspect to be ensured is that transition between specialist centres coincides with transition in local hospitals. With a scarce reliable data on epidemiology, treatments, quality of life, patients' preferences and healthcare system and organisation available, a key aspect in the transition process is the networking and exchange of knowledge between PID specialists, general practitioners, nurses, physiotherapists, pulmonologists, and social workers.

<sup>2</sup> Blum RW, Garell D, Hodgman CH, et al. Transition from child-centered to adult health-care systems for adolescents with chronic conditions. A position paper of the Society for Adolescent Medicine. J Adolesc Health. 1993;14:570–6.

## Ageing with a primary immunodeficiency

The second panel focused on ageing with a PID, with interventions addressing the challenges of growing older with a PID as well as living as an elderly PID patient.



**Janika Sundström**, Chair of the Immunodeficiency Patient Organisation in Finland, gave a patient's perspective on what it means to live with a PID. According to PID patients in Finland, once diagnosed with a PID, it is important to accept the condition. She noted that early diagnosis is crucial to avoid complications in older age. Ms. Sundström informed participants that in Finland there is no official treatment pathway established. To this end, it is essential to create a pathway that ensures early diagnosis but also a healthcare and social security system which can support it. The pathway should also take into consideration all phases of life. For elderly PID patients for example, a support with doctor visits and the healthcare system is also something to be addressed, with a possibility for elderly PID patients to have their own doctors. Furthermore, Ms. Sundström confirmed what has

been stated earlier on networking and sharing knowledge and experiences between doctors and care givers on specific PID conditions.

**Prof Martin van Hagen**, from Erasmus MC, made an intervention from an academic perspective on ageing with a PID. With the strength of the immune responses declining with age, Prof van Hagen emphasised the need of more attention to recurring infections such as respiratory and ENT (ear, nose, throat) infections; opportunistic infections, as well as infections of the skin, organs, skeletons. The choice of therapy in the elderly PID patients is often complicated and many factors need to be considered such as infusion type (by patient or by a professional), control of compliance, comorbidities, skin conditions, body composition etc. In addition, primary disease manifestations or complications of treatment in this group of patients may yet develop, thus follow-up throughout the life of these patients is extremely important.



Elderly patients do not respond to immune challenges as robustly as the young. To this end, elderly persons with PIDs require a disciplinary approach with patient support programmes and home care. Personalised medicine in these patients should be considered as the process requires considerations on the individual characteristics, needs and preferences of a patient during all stages of care.





**Dr Renate Heinisch**, from the Federal Association of German Senior Citizens' Organisations and member of the EESC, elaborated on the social perspective of ageing with a PID. She noted that due to technological advances there are more and more elderly PID patients whose specific needs should be addressed through targeted measures with a comprehensive strategy with actions on individual, organisational and societal levels.

To this end, reliable health care for elderly PIDs entails medical care (especially geriatric) and rehabilitation, adequate care services, advice, information and education on patient rights, formal and informal social support and technical support systems. Dr Heinisch believes that the

digital evolution should also be considered, when tackling the problems of future elderly patients. In 20 years, there will be much more computer literate elderly patients, which will entail eventually better digitalization facilities.

Health promotion is a cost-effective way to prevent further illness development in elderly PIDs patients that lead to longer hospital stays, bacterial infections and increased risks of cancer. Dr Heinisch noted that further provisions on specialised PID elderly patient care are needed in national rare disease plans.

## Discussion

During the discussion section key issues of transitional care in Estonia and Belgium were presented. In Estonia, lack of experience and expertise in PID treatment and care are one of the main issues in addition to limited treatment opportunities and no possibility for home care. On the same topic, it was noted that in Belgium, the situation with transition of care in larger hospitals is good. However, the process is still problematic in small hospitals. Dr Mahlaoui expressed his opinion that the ERNs might be the solution for countries experiencing similar challenges.

Dr Mahlaoui emphasised once again the importance of networking and exchanging information to ensure an adequate transition of care for PID patients. In this regard, Prof van Hagen, informed participants that within the Erasmus MC and Eye Hospital Rotterdam, monthly discussions between patients and physicians are being organised in order to better determine the specific patients' needs.

Dr Waligora from the European Commission noted that within the ERNs, a workshop is planned for December 2017, where he is optimistic that guidelines on transition of care for not only one ERN would be discussed as well as other overarching ERN activities. On the same topic, Dr Mahlaoui confirmed that in the Necker-Enfants Malades University Hospital, he is currently running a transition programme of 4000 rare diseases proving the feasibility of the activity.

## Concluding remarks

Carlos Zorrinho MEP concluded the meeting by noting that the conversation should echo within the European Parliament, the European Commission and the European Economic and Social Committee. He also informed the participants that he is looking forward to concrete recommendations from the discussions to be shared with patient organisations, healthcare professionals and national and European institutions. He summarised the main points of action aired during the presentations and the contributions to the unexplored topic of transition of care and ageing for PIDs.



- There is a need for a defined and coordinated pathway guiding young patients toward adult services. This will help preventing adolescent patient being lost in the healthcare system, which will lead to poor compliance to treatment, potential irreversible organ damage, lower life expectancy, and reduced quality of life.
- Planned transitional care for PID patients is often overlooked but it is a cost-effective way of preventing poor compliance.
- Collaboration is a vital point in ensuring easy access to essential healthcare and support during transitional period.
- Elderly PID patients should have access to a holistic treatment and care.
- Elderly healthcare should be included into national rare disease plans. In this sense, the establishment of a framework of accessible and sustainable home care and long-term care services for elderly PID patients would play an essential role.
- Targeted measures should be taken with regards to infrastructure, the supply of adequate health care and care services, as well as participation in economic, political and social life.
- Clinical guidelines should also be developed to ensure that care provided to elderly patients with PIDs is adequate to this population's needs.

## List of participants

### *European Parliament*

- Dr. Elena Gentile (S&D, Italy)
- Carlos Zorrinho (S&D, Portugal)
- Sirpa Pietikäinen (EPP, Finland)
- Silvino Da Silva, Office of Mr. Carlos Zorrinho MEP
- Daniela Giordano, Office of Dr. Elena Gentile MEP
- Francesco Losappio, Office of Dr. Elena Gentile MEP
- Marguerite Overbeek, Office of Marian Harkin MEP

### *External Participants*

- Yordan Aleksandrov, Rohde Public Policy
- Alain Coheur, Solidaris Mutual Health Insurance (Belgium) & member of the EESC
- Claudine Deckers, BOPPI
- Kit Greenop, Rohde Public Policy
- Andrea Gressani, Associazione Immunodeficienze Primitive Onlus
- Prof Martin van Hagen, Erasmus MC and Eye Hospital Rotterdam
- Dr. Renate Heinisch, Federal Association of German Senior Citizens' Organisations and member of the EESC
- Tea Jardas, Rohde Public Policy
- Joelle Khraiche, CSL Behring
- Saara Kiema, IPOPI
- Joyce Loridan, Solidaris Mutual Health Insurance (Belgium)
- Dr Nizar Mahlaoui, the Necker-Enfants Malades University Hospital, Paris
- Jelena Malinina, Rohde Public Policy
- Karl Petrovsky, PPTA
- Leire Solis, IPOPI
- Janika Sundström, Finnish Immunodeficiency Patient Organisation
- Kersti Urbala, Estonian Patient Society for Primary Immunodeficiencies
- Dr Jaroslaw Waligora from the European Commission's DG Health and Food Safety