

# REPORT

**IPOPI's 8<sup>th</sup> PID Forum**

**Tuesday 24 January 2017  
European Parliament, Brussels**

**DON'T BREXIT ON  
RARE DISEASE PATIENTS –**  
the case of primary immunodeficiencies



**Co-Chaired by MEPs Seb Dance and Carlos Zorrinho  
Supported by MEP Catherine Bearder**

## Introduction

On Tuesday 24 January 2017, Members of the European Parliament (MEPs) Seb Dance and Carlos Zorrinho co-hosted IPOPI's 8th Primary Immunodeficiencies (PIDs) Forum "Don't Brexit on Rare Diseases Patients: the Case of Primary Immunodeficiencies" at the European Parliament.

The meeting, conducted with the support of Catherine Bearder MEP and co-organised by the International Patient Organisation for Primary Immunodeficiencies (IPOPI) was aimed at addressing the potential implications of the United Kingdom's (UK) departure from the European Union (EU) for rare disease patients, with a specific attention to the case of primary immunodeficiencies.

The Forum brought together European and British patients, healthcare professionals, researchers, and policy-makers to share their views on Brexit's effects on the rare diseases community.

The event attracted a great attention from the European Parliament: nine MEP offices were present during the event. These included: Seb Dance (S&D, UK), Catherine Bearder (ALDE, UK), Linda McAvan (S&D, UK), Mairead McGuinness (EPP, Ireland), Marian Harkin (ALDE, Ireland), Carlos Zorrinho MEP (S&D, Portugal) and the offices of Dame Glenis Willmott (S&D, UK), Kay Swinburne (ECR, UK) and Danuta Jazłowiecka (EPP, Poland). All MEPs committed to supporting the rare diseases and PID patient communities and researchers in their efforts to ensure a good future after Brexit.

The discussions which took place at the event will be the basis of a recommendations paper.

## Introductory remarks

**Mr. Seb Dance MEP** opened the meeting by welcoming participants and emphasising the need for discussions such as this one in the context of the ongoing Brexit negotiations to ensure the best possible outcome for all British and EU citizens, especially those, affected by rare diseases such as PIDs. Mr. Dance noted that now is the right time to raise the voice of patients, healthcare professionals and researchers to guarantee that the benefits of the EU research funding regimes and developed cross-border health collaboration are not undermined by the Brexit. It is crucial to ensure that the UK remains a hub for medical research and that both UK and EU patients can benefit from treatment abroad. He emphasised that networks between the UK and the EU must be maintained and strengthened.



**Seb Dance**  
**S&D UK**



**Carlos Zorrinho**  
S&D Portugal

**Mr. Carlos Zorrinho MEP** expressed his hope that the Forum would leave a mark in the upcoming complex negotiation procedures following the Brexit vote. He also thanked IPOPI for their continuous work towards ensuring that the voices of patients with PIDs are well heard and considered. He noted that in these times of political turmoil around the world, it is paramount to continue cross-country collaboration on research and sharing best practices in addressing rare diseases patients' needs. Mr. Zorrinho expressed his optimism that when Brexit happens, the UK and the EU can develop a relationship allowing European patients with PIDs to have the best possible access to care and that European researchers will still have the opportunity to thrive.

## Current EU Framework for Rare Diseases

**Mr. Johan Prévot** presented the current EU framework on rare diseases, and how PIDs have benefitted from EU support in terms of policy, legislation and funding. Some of the examples of the EU influence included Council's Communication in Rare Diseases (2009) which encouraged the adoption of national plans on rare diseases. The Commission also facilitated the establishment of the European Reference Networks (ERNs), foreseen in the Directive on the application of patients' rights in cross-border healthcare. In addition, the EU Regulation on Orphan Medicines established a centralised procedure for the designation of orphan medicinal products and put in place incentives for research, marketing and development of medicines for rare diseases. Furthermore, the EU has been providing funding to numerous projects on rare diseases, including projects for PIDs such as SCIDnet. Mr. Prévot emphasised that the key goal of the Forum is to ensure that PID care and rare diseases care in general should not suffer from the political decisions during Brexit process.



**Johan Prévot**  
Executive Director IPOPI

## Policy-maker's perspective



**Catherine Bearder**  
ALDE, UK

**Ms. Catherine Bearder MEP** noted that current discussions on Brexit are mainly about trade, however, the trade works on certain rules in the same way as medical research works on rules, and these rules need to be discussed thoroughly. Ms. Bearder expressed her concerns that due to Brexit, the UK might lose its position as the team leader in EU research projects. Furthermore, given the current U.S. President's approach to research, the EU will become a global leader in research, and the UK would lose many opportunities to participate in cross-country science developments. In addition, she noted that after Brexit, the UK might lose such benefits of the EU such as the centralised medicines authorisation procedure and cross-border healthcare, which will result in more costs for the UK. Ms. Bearder noted, that in order to ensure the



smooth transition of the UK, the process of departure should take longer than two years. This would allow to include a thoughtful consideration of many aspects, including all the issues concerning the rare diseases community.

## **Brexit: what do we have to gain & lose? The British Perspective**

**Dr. Siobhan Burns** presented the University College London (UCL) Centre for Immunodeficiencies, specialising in the expert care, development of novel therapies and diagnostic tools and research of causes in PIDs.

Dr. Burns noted that for the past 15 years, gene therapy in the UK has been supported by EU grant funding, which resulted in new curative treatments for patients with PIDs across Europe. What is more, the EU funded initiatives have enabled research into the causes of PIDs.

Dr. Burns indicated that Brexit might have significant implications for PIDs, resulting in restricted possibilities for patients in terms of care and clinical trials, disruption of the current academic collaborations, lost opportunities for training doctors and scientists, and negative impact on recruitment for research and clinical positions. Dr. Burns stressed the importance to preserve a cross-border agreement for healthcare for rare disease patients, retain movement of doctors and researchers, as well as to protect UK participation in EU grant funded consortia during the Brexit negotiations.



**Dr. Siobhan Burns**  
University College London



**Sarita Workman**  
Specialist Sister in  
Immunology Research

**Ms. Sarita Workman** presented the Royal Free London NHS Foundation Trust and the services of the Department of Clinical Immunology. The Department is used for the European referrals of patients for diagnosis and treatment including access for bone marrow transplant and gene therapy that may not be available in their own country. In addition, the department participates in multi-research studies based in the EU. Ms. Workman highlighted that due to Brexit there is a risk of devaluation of pound, which consequently will lead to the increased cost of medication. Furthermore, Brexit might negatively affect collaboration in clinical research and employment of EU nationals by the UK NHS. According to Ms. Workman, in the context of Brexit, maintaining the best patient care, including through cross border healthcare agreement, is key. There is also a need to encourage new and future research and clinical collaborations between the UK and the EU. Ms. Workman also stressed that it is paramount to protect staff recruitment processes for the UK and EU nationals.

## Brexit: what do we have to gain & lose? The European perspective

**Ms. Martha Gouldsbury**, from the Irish Primary Immunodeficiency Association (IPIA) presented the Irish PID patients' perspective on Brexit. She noted that Ireland currently relies on the UK labs for rare testing. At present Ireland is granted with UK NHS prices for these services, however this courtesy might be discounted after Brexit, which would result in a need of a bigger budget for testing. Furthermore, after Brexit, Ireland would need greater support to reduce the big waiting list for Intravenous Immunoglobulin (IVIG). In addition, the UK might leave the E112 scheme, allowing cross-border health treatment, thus Irish patients may not be able to access treatment in the UK. She also emphasised the importance of Irish government and the EU to help PIDs community upscale paediatric Hematopoietic cell transplantation services to allow Irish patients to receive it at home. Ms. Gouldsbury expressed her hope to collaboration with the immunology community in the UK after the Brexit.



**Martha Gouldsbury**  
Irish Patient  
Organisation (IPIA)



**Prof. Martin Van Hagen**  
Erasmus MC

**Prof. Martin Van Hagen** from the Erasmus MC, the largest university medical centre in the Netherlands, noted that currently patient referral to the UK is very rare, as the centre has all facilities for PID treatment and care. However, increasingly European and worldwide collaboration will increasingly be needed (e.g. for gene editing). He noted that although the Erasmus MC Center would not be affected by Brexit, others centres and member states may be, and it is therefore paramount to maintain good links with British researchers. Also UK researchers should continue to have the possibility to participate in EU research. Prof. Van Hagen expressed his concerns regarding the potential detrimental effect on the pharmaceutical industry and innovation in the EU, as many of them are UK-based.

**Dr. Susana Lopes Da Silva**, Centro de Imunodeficiências Primárias Lisboa, highlighted that international cooperation in PIDs is needed to share experiences, improve diagnosis and increase awareness. Portugal has referred Portuguese patients with PIDs to the UK for some specific diagnostic tests, BMT treatment and some continuous follow-ups. Furthermore, Portuguese healthcare professionals participate in international multicentral projects and do fellowships in well-recognised PID Centres (e.g. London, Oxford, Newcastle centres). Dr. Lopes Da Silva noted that Brexit might challenge the existing education and networking possibilities for the Portuguese PID community.



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

## The Norwegian Way



**Eva Brox**  
**President of PID**  
**Organisation Norway**

**Ms. Eva Brox**, President of the Norwegian Immunodeficiency Organisation, shared the experiences of Norway in rare diseases management as a non-EU country but a Member of the European Economic Area (EEA). Ms. Brox highlighted that Norwegian PID care specialists benefit a lot from the European cooperation by acquiring training and sharing experiences with other countries through various exchange programmes. Norway can also benefit from EU funding in research, however, it has no vote, when it comes to decision-making. Additionally, Norway implemented the EU Cross Border Healthcare Directive, thus Norwegian patients can now benefit from treatment in other EEA countries, and then be reimbursed. It is particularly advantageous for rare diseases patients. There is also a number of Norwegian PID patients being referred to other countries, including the UK, Sweden, Germany. The reasons for referral include unknown diagnosis, gene therapy, transplantation and severe rare complicated PIDs, the success rate of all referrals was very high.

## Impact for the Rare Disease Community at large

**Ms. Amanda Bok** from the European Haemophilia Consortium presented a perspective on how Brexit is going to affect haemophilia patients and other rare disease patients. She noted that the true impact is difficult to predict, as current national standard of haemophilia treatment and care are very high in the UK, thus patients could be protected by the strong national system and low prices for treatment. However, it might be challenged by market and currency fluctuations, which could result in higher medicines pricing, thus reduced access for patients. Ms. Bok also highlighted that the UK's departure from the EU might result in changes in medicines assessment and labelling, as well as significantly impact the research, training and exchange of expertise in rare diseases. She noted that it is important to maintain and reinforce solidarity, safeguard ties and linkages where possible. In addition to that, the rare diseases community with other stakeholders need to understand new rules of engagement and seize the opportunity to co-shape, co-define new rules, and to bring the patient expertise to shape the new agenda.



**Amanda Bok**  
**CEO of the European**  
**Haemophilia Consortium**

## Discussion

### Development of IPOPI Recommendations Paper

Linda McAvan MEP suggested IPOPI to develop the recommendations paper highlighting the needs of the rare disease community, including PIDs. Johan Prévot (IPOPI) confirmed that IPOPI will release the recommendations after the meeting and will disseminate it among all participants.

Amanda Bok (EHC) proposed to make the recommendations available for signature for other rare disease patient organisations.

### UK rare disease national plan is at risk?

Jose Drabwell (IPOPI) raised a question on the UK national rare disease plan, she expressed her concerns, as this plan was not yet implemented, whether UK's departure from the EU might negatively affect it. This message was echoed about files such as the Clinical Trials regulation. Linda McAvan MEP noted that much EU legislation has already become national law and will hence remain, however, after Brexit, it might become a subject of change or removal by the national authority which was hereto impossible.

### Brexit timeline

Marian Harkin MEP emphasised that a foreseen timeline of two years transition period for the UK's departure from the EU is realistic only in terms of large pieces of legislation and big issues. All smaller (but not less significant) issues which will affect patients, researchers and healthcare professionals require much more time a transitional period might be useful in order to agree on all necessary details and arrangements.

### Concluding remarks

Carlos Zorrinho MEP thanked Forum participants for the debate and noted that it is crucial to communicate about needs of rare disease community and to highlight all the benefits of the cross-border health collaboration in the upcoming Brexit negotiations.

Seb Dance MEP stressed that networks between the UK and EU are essential and they should be preserved and developed further on. MEPs Seb Dance, Catherine Bearder and Linda McAvan expressed their commitment to deliver the recommendations from the Forum to their colleagues at both European and British Parliaments



**Johan Prevot (IPOPI), Jose Drabwell (IPOPI), Seb Dance MEP, Linda McAvan MEP, Martine Pergent (IRIS), Carlos Zorrinho MEP, Marian Harkin MEP**



## List of participants

### *European Parliament*

- Mr. Seb Dance, Member of the European Parliament
- Mr. Carlos Zorrinho, Member of the European Parliament
- Ms. Catherine Bearder, Member of the European Parliament
- Ms. Linda McAvan, Member of the European Parliament
- Ms. Mairead McGuinness, Member of the European Parliament
- Ms. Marian Harkin, Member of the European Parliament
- Ms. Margarida Matias, Office of Carlos Zorrinho, European Parliament
- Ms. Sara Silva, Office of Mr. Carlos Zorrinho, European Parliament
- Ms. Jessica Clayton, Office of Ms. Catherine Bearder, European Parliament
- Mr. Alexander Keynes, Office of Mr. Seb Dance, European Parliament
- Ms. Emily Hunter, Office of Dame Glenis Willmott, European Parliament
- Mr. Argos Girling, Office of Ms. Kay Swinburne, European Parliament
- Ms. Isabela Moskwa, Office of Ms. Danuta Jazłowiecka, European Parliament
- Ms. Marguerite Overbeek, Office of Ms. Marian Harkin, European Parliament
- Ms. Katherin Power, Office of Ms. Mairead McGuinness, European Parliament
- Ms. Charlotte Butterick, Office of Ms. Linda McAvan, European Parliament

### *External Participants*

- Mr. Yordan Aleksandrov, Rohde Public Policy
- Ms. Amanda Bok, European Haemophilia Consortium
- Ms. Eva Brox, Norwegian Immunodeficiency Organisation
- Dr. Siobhan Burns, University College London
- Ms. Laura Cigolor, Health First Europe
- Mr. Robert Delis, British Medical Association
- Ms. Jose Drabwell, IPOPI
- Mr. Ruediger Gatermann, CSL Behring
- Mr. Kit Greenop, Rohde Public Policy
- Ms. Martha Gouldsbury, IPIA
- Prof. Martin van Hagen, Erasmus MC Rotterdam
- Ms. Saara Kiema, IPOPI
- Dr. Susanna Lopes da Silva, Centro de Imunodeficiências Primárias, Lisboa
- Ms. Jelena Malinina, Rohde Public Policy
- Ms. Irina Odnoletkova, PPTA
- Ms. Martine Pergent, IPOPI & IRIS
- Mr. Karl Petrovsky, PPTA
- Mr. Johan Prévot, IPOPI
- Ms. Maria Rodriguez Sanchez, Shire
- Mr. Sebastian Rohde, Rohde Public Policy
- Dr. Françoise Rossi, IPFA
- Ms. Laura Savini, European Haemophilia Consortium
- Ms. Leire Solis, IPOPI
- Ms. Undis Veisten, Norwegian Immunodeficiency Organisation
- Dr. Frank Willersinn, Alpha-1 Global
- Ms. Sarita Workman, Royal Free Hospital UK