

# Don't Brexit on rare disease patients – the case of PIDs

## Recommendations of the International Patient Organisation for Primary Immunodeficiencies

March 2017

### Introduction

IPOPI is the International Patient Organisation for Primary Immunodeficiencies. As the association of national patient organisations, it is dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency (PID) patients worldwide.

Following the United Kingdom's (UK) vote in the Brexit referendum, both the EU and the UK are currently preparing for the formal negotiation process. The potential consequences of the UK leaving the EU are still unknown. However, patients with primary immunodeficiencies (PIDs), healthcare professionals working in the field and other stakeholders involved with rare diseases have expressed serious concerns about the consequences this political withdrawal from the EU will entail for patients, as well as healthcare professionals, researchers and other stakeholders.

IPOPI's recommendations aim to ensure that care for patients with PID and opportunities for healthcare professionals and researchers are not challenged following the Brexit referendum. The role of the European Union in the healthcare of patients was not a topic of debate during the referendum however it is clear that the impact of Brexit will be substantial on all healthcare stakeholders. **As such, this paper calls upon the UK government and EU negotiators to ensure that the health of all European citizens is on the agenda of negotiations seeking a positive collaborative future.**

## RECOMMENDATIONS

### The need of inclusion of rare diseases community in Brexit negotiation process

The UK has a long legacy of caring for rare disease patients and of researching in this field, a legacy that has been reinforced by the European Union. The UK's decision to exit the EU will have a substantial impact on many aspects for PID care and rare disease care in general. EU research funding and collaborative research networks, access to cross-border healthcare, and healthcare professionals mobility are all crucial factors in ensuring high quality PID care and its availability.

- 1. The expected timeline of two years' negotiation period for the UK's departure from the EU is a very short timeline, likely only able to accommodate the major topics around trade. Both negotiating parties must ensure that issues which will affect patients, researchers and healthcare professionals are not neglected. Given the strict cut-off point of 2-years, a transitional period for cross-border flows of patients might be useful in order to ensure there is no gap in patients' access to life-saving care.**

## Timely access of care for PID patients

At the instance of the European Union's Recommendations on Rare Diseases, the United Kingdom developed a national Strategy for Rare Diseases in 2013. The Strategy *"aims to ensure no one gets left behind just because they have a rare disease"*, putting *"the patients' needs first"*<sup>1</sup>. It is this conception of putting patients first that should govern the UK's future healthcare legislation and policy, once the exit from the European Union becomes a reality.

The European Union, when developing its policy on rare diseases, considered that these life-threatening or chronically debilitating diseases *"affect so few people that combined efforts are needed to:*

- *reduce the number of people contracting the diseases;*
- *prevent newborns and young children dying from them;*
- *preserve sufferers' quality of life and socio-economic potential<sup>2</sup>.*

- 2. The UK government should make sure that these objectives continue to be reflected in future rare disease policy and legislation and that combined efforts to care for PID patients are pursued. These European collaborative objectives should be the baseline for UK action on rare diseases, even after leaving the EU.**

For patients living with a rare disease, such as PIDs, where the expertise is scarce and fragmented across individual European countries, the EU and its Member States have recognised the importance of collaboration amongst professionals. The European Directive on the application of patients' rights in cross-border healthcare and the Regulation of coordination of social security systems have also promoted the possibility for patients wishing to receive the treatment outside their Member State due to the long waiting period to receive such treatment or because it is not available in theirs.

- 3. It should be guaranteed that patients with rare diseases can have access to treatment abroad in a timely manner. Access to specialist treatment (i.e. gene therapy) should remain available for UK Patients if needing to travel to an EU Member State. Vice versa, EU patients who may need to have their specialist treatment in the UK (i.e. SCID-ADA gene therapy) should be able to do so after Brexit.**

## Research

The UK Strategy for Rare Diseases aimed at promoting *"the UK as a first-choice location for research into rare diseases as a leader, partner and collaborator"*<sup>3</sup>. To this end, while welcoming the UK government's position of continuing to collaborate with European partners on major science, research and technology initiatives, IPOPI would like to see more detail on how this would work in

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<sup>1</sup> UK Department of Health: The UK Strategy for Rare Diseases. 2013, available at:

[https://www.gov.uk/government/uploads/system/uploads/attachment\\_data/file/260562/UK\\_Strategy\\_for\\_Rare\\_Diseases.pdf](https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/260562/UK_Strategy_for_Rare_Diseases.pdf) (accessed on 14 November 2016)

<sup>2</sup> European Commission: Policy. Rare diseases – what are they?". Available at: [http://ec.europa.eu/health/rare\\_diseases/policy/index\\_en.htm](http://ec.europa.eu/health/rare_diseases/policy/index_en.htm) (accessed on 15 November 2016)

<sup>3</sup> UK Strategy for Rare Diseases, available at:

[https://www.gov.uk/government/uploads/system/uploads/attachment\\_data/file/260562/UK\\_Strategy\\_for\\_Rare\\_Diseases.pdf](https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/260562/UK_Strategy_for_Rare_Diseases.pdf) (accessed on 15 November 2016)

practice. The leadership of the UK in the research area however depends on European collaboration and as such, the negotiations must ensure that UK researchers continue to have the opportunity to participate in European research projects.

- 4. Policies should be put in place to guarantee that once the UK has exited the European Union, the UK is still attractive for research on rare diseases.**
- 5. The UK government must seek to define a clear relationship allowing researchers from the UK to actively participate in EU research projects.**

By being part of the EU, the UK has been able to participate and influence EU decision-making in decisive dossiers such as the EU Clinical Trials Regulation or other legal provisions affecting research. The EU Clinical Trials Regulation, which will come into force in 2018, is considered a big improvement for the development of trials in Europe and the UK Government highlighted the considerable involvement of the Medicines and Healthcare Products Regulatory Agency (MHRA) in the development of this EU law.<sup>4</sup>

Developing medicines for PIDs and rare diseases in general requires the opening of a multicentre clinical trial involving several countries. A good example of this is the development of immunoglobulins, plasma-derived medicinal products, for treating patients with PIDs, that require the opening of multicentre trials in several EU countries to ensure that an adequate number of patients is involved. Having similar legislation on clinical trials research in the UK and EU Member States would greatly facilitate the maintenance and development of such trials, so vital for patients with PIDs and rare diseases in general.

- 6. The UK should implement the Clinical Trials Regulation and maintain it after it leaves the EU. The government should seek to ensure that the opening of multicentre clinical trials in the UK and the European Union is not made more difficult post-Brexit so as to provide UK patients with the opportunity to be enrolled in trials granting them a quicker access to potential new therapies. Ideally the UK government should seek to remain part of the EU regulatory framework for clinical trials, including the EU Clinical Trial Portal and Database.**

The UK is also a net beneficiary of EU health research funding. The UK has received €7 billion of funding through the 7<sup>th</sup> European Union Research Framework Programme representing 15% of all EU funding, and this percentage has been maintained in Horizon 2020.<sup>5</sup> While the Government's White Paper proposes to maintain any funding that is ongoing at the time that Britain leaves the EU, it appears unlikely that the UK will access the European research funds at the same level once the exit is completed. Alternative solutions and partnerships should be actively considered with the EU to maintain the advances led by UK researchers and academics.

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<sup>4</sup> Strategy for UK Life Sciences, available at:

[https://www.gov.uk/government/uploads/system/uploads/attachment\\_data/file/32457/11-1429-strategy-for-uk-life-sciences.pdf](https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/32457/11-1429-strategy-for-uk-life-sciences.pdf) (accessed on 15 November 2016)

<sup>5</sup> Statement made by Jane Ellison, Parliamentary Under-Secretary of State for Public Health, during an Oral evidence: Impact of membership of the EU on health policy in the UK at the Health Committee of the House of Commons, 26 April 2016, available at: <http://data.parliament.uk/writtenevidence/committeeevidence.svc/evidencedocument/health-committee/impact-of-membership-of-the-eu-on-health-policy-in-the-uk/oral/32509.html> (accessed on 15 November 2016)

- 7. UK investment in research and innovation should be maintained following Brexit and collaboration between UK and EU funded projects should be sought.**

### Healthcare professionals' expertise is key for PIDs

The UK currently benefits from the expertise and qualifications of healthcare professionals coming from many European countries. These professionals, representing over 9% of NHS doctors and over 6% of NHS nurses<sup>6</sup>, provide treatment and care for UK patients and patients travelling from other EU Member States to the UK. The first sign of change is already visible: according to the British Nursing and Midwifery Council, the number of European nurses registering to work in Britain in December 2016 has fallen by more than 90% in comparison to July 2016 (data for January 2017)<sup>7</sup>. With 24,000 nursing vacancies in the UK, it is challenging to fill them without EU nurses.

- 8. Given that the procedures to enter the working environment for non-EU and, after Brexit, non-UK citizens can be burdensome, the UK should ensure that, once it leaves the European Union, these highly skilled and qualified healthcare professionals are still able to contribute to the well-being of their patients, both from the UK and abroad.**

The EU has been fostering the exchange of students across the EU Member States, with the view to facilitate the training of healthcare professionals in different stages of their careers, encouraging transnational, inter-sectoral and interdisciplinary mobility. The UK has been a point of attraction for the students from all around the EU.

- 9. The government should ensure that once the exit is formally requested, agreements are reached with the EU to ensure that students and young healthcare professionals both from the UK and the rest of the EU can benefit from these exchanges, strengthening the capacity of future researchers and healthcare workers in PIDs and other rare diseases.**

### European Reference Networks

The European Commission defines a European Reference Network (ERN) as a network connecting healthcare providers and centres of expertise of highly specialised healthcare, for improving access to diagnosis, treatment, and the provision of high-quality healthcare for patients with conditions requiring a particular concentration of resources or expertise no matter where they are in Europe<sup>8</sup>.

In the case of PIDs, the European Reference Network on the Rare Immunodeficiency, Autoinflammatory and Autoimmune (RITA) is being led by the Newcastle upon Tyne Hospitals, in the United Kingdom, and counts with several other universities from the UK amongst the list of participating centres. The participation of UK centres in the European Reference Networks after the

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<sup>6</sup> Statement made by Jane Ellison, Parliamentary Under-Secretary of State for Public Health, during an Oral evidence: Impact of membership of the EU on health policy in the UK at the Health Committee of the House of Commons, 26 April 2016, available at: <http://data.parliament.uk/writtenevidence/committeeevidence.svc/evidencedocument/health-committee/impact-of-membership-of-the-eu-on-health-policy-in-the-uk/oral/32509.html> (accessed on 15 November 2016)

<sup>7</sup> British Nursing and Midwifery Council <https://www.nmc.org.uk/> (accessed on 2 February 2017)

<sup>8</sup> FAQs on ERNs, European Commission, available at:

[http://ec.europa.eu/health/sites/health/files/ern/docs/fag\\_establishing\\_ern\\_en.pdf](http://ec.europa.eu/health/sites/health/files/ern/docs/fag_establishing_ern_en.pdf) (accessed on 2 February 2017)

UK leaving the European Union is yet unknown and could be reviewed, based on the existing criteria. This potential revision is of great concern for patients with PIDs and rare diseases in general, as it may endanger access to specialised care and clinical expertise for UK patients.

**10. The interest and well-being of patients with PIDs and rare diseases in the UK and in the EU, should remain at the centre of the decision-making process for any potential change in the requirements for the European Reference Networks. Authorities in the UK and the European Union should seek to ensure that the UK remains part of the system of European reference networks so that patients with rare diseases continue to have access to expertise and the highly-specialised care they require.**

**11. The Brexit will also pose questions on sharing patient data, which would be detrimental for UK patients and researchers. Policy-makers should take into consideration this important aspect in negotiating the provisions on data flow to and from the UK.**

### **Development and approval of therapies for PIDs in the UK**

Currently, plasma-derived medicinal products, used by a large majority of patients with PIDs, and therapies used for treating rare diseases in general are centrally approved at European level. This common approval avoids duplication of national assessments of medicines, provides an immediate marketing authorisation throughout the European Union and facilitates the availability of these therapies to patients. This common assessment has benefited UK patients immensely and, after the UK exit of the EU becomes official, the Medicines & Healthcare Products Regulatory Agency (UK regulatory body for the approval of medicines) might be the sole responsible body for approving medicines in the UK.

**12. The UK should develop policies that ensure patients with PIDs and rare diseases in the UK are not disadvantaged in terms of availability of the medicines they need. These policies should consider the necessary resources to be able to assess new therapies, as well as guarantee exchanges with the European Medicines Agency to avoid a potential isolation of the UK in terms of access to safe and efficacious medicinal products that are life-saving, such as immunoglobulin replacement therapies for treating patients with PIDs.**



As of March 2017, the recommendations are endorsed by:

**Members of the European Parliament**

**Dame Glenis Willmott (S&D, UK)**

**Linda McAvan (S&D, UK)**

**Seb Dance (S&D, UK)**

**Carlos Zorrinho (S&D, Portugal)**

**Catherine Bearder (ALDE, UK)**

**Marian Harkin (ALDE, Ireland)**

**Patient representatives**



**Dr. Susan Walsh**  
**Director of Primary Immunodeficiency UK**



**Jose Drabwell**  
**Chair of IPOPI Board**

**Johan Prévot**  
**Executive Director, IPOPI**



**Norsk Immunsviktforening**

**Eva Brox**  
**President of the Norwegian**  
**Immunodeficiency Organisation**



**Dr. Susana Lopes da Silva**



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