Rare disease research likely to lose out from Brexit

Written by Glenis Willmott on 21 February 2017 in Opinion

EU cooperation has been hugely important in advancing research into rare diseases, but Brexit could put an end to this, warns Glenis Willmott.

Around 30 million people in the EU suffer from a rare disease. A disease is classed as rare if it affects less than one in 2000 people and the majority are little known, except by the people who suffer from them.

Roughly 8000 rare diseases have been identified and include dystonia - a rare brain disorder that causes pain and involuntary muscle contractions; the blood-clotting disorder Von Willebrand disease; and severe combined immune deficiency (SCID) - a condition in which immune system abnormalities leave patients highly susceptible to infection. While they may be uncommon, the impact on patients can be debilitating and in some cases - like with SCID - even fatal.
The need for coordinated EU-level action on rare diseases is clear. While millions of people suffer from a rare disease, the number of patients with a particular disease in each country is so small that expertise and treatments are not always available. Working together across the EU has allowed scarce resources to be pooled and patients, doctors and experts to link up.

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Thanks to EU reference networks, we no longer have isolated centres of expertise but networks of experts that cross borders. Free movement and the cross-border healthcare directive have facilitated mobility of patients and doctors, meaning that patients can access treatment in another EU country that isn't available in their own, while doctors have the opportunity to connect with peers and share expertise and training.

Nevertheless, many rare disease patients still wait too long for a correct diagnosis and access to treatment. The EU must do more to raise awareness of rare diseases and their symptoms, as well as the opportunities for cross-border treatment. EU research funding is also hugely important.

Under the FP7 programme, the EU invested over €620m in rare disease research and this level of investment is being continued under Horizon 2020.

However, pharmaceutical companies are still reluctant to prioritise research into rare diseases, as the small number of patients means treatments are unlikely to be profitable. As a result, there are still no effective treatments for many rare diseases. The EU has adopted legislation to incentivise the development of treatments for rare diseases - so called 'orphan medicines'.

Yet, while the legislation has undoubtedly had some success, there are concerns that it's also been used as a way to continuously extend the patents of medicines that are not truly 'orphan' and then charge higher prices for them.

The legislation is clearly necessary, but we need to be sure that it is the patients who really benefit. While EU action has clearly made a difference to rare disease patients, many in the rare disease community are now deeply concerned about the potential impact of Brexit.

The UK is a global leader in medical research and British centres currently receive 50 per cent of rare disease funding under Horizon 2020. UK researchers are naturally concerned about where future funding will come from - and although the UK government has promised to underwrite EU funding until 2020, very little has been said beyond this.

But it isn't just about money and it isn't just Britain that stands to lose out. Brexit also calls into question the future of research collaboration, patient mobility and reference networks.

EU-wide collaboration helps to drive innovation by allowing researchers to share their knowledge, expertise and resources - if it becomes harder for UK-based academics to collaborate with their EU counterparts or for EU researchers to access the world class institutions in the UK, we all stand to lose
out.

Take the example of clinical trials: Great Ormond Street hospital in London is the only centre in Europe running trials on SCID, but many of the trials there are multi-centre trials, meaning they involve patients in hospitals across Europe. Such cross-border trials are vital for rare diseases because there aren't normally enough patients to make a trial in one country viable.

The UK is a centre for rare disease research, but if Brexit makes it harder to organise cross-border trials there's a risk that fewer trials will go ahead, meaning patients both in the UK and the EU won't have access to potentially life-saving trials.

Similarly, in terms of access to healthcare, some smaller countries like Ireland, Portugal and Norway currently send some specialist rare disease tests to the UK for diagnosis and likewise patients are often sent to Britain for bone marrow transplants. It's not clear whether this could continue once the UK leaves the EU.

Rare diseases are a great example of the importance of EU cooperation, but Brexit is causing uncertainty for doctors, academics and patients. It's clear that on both sides of the Channel we stand to lose out if the future UK-EU relationship makes this collaboration harder.

About the author

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