

Position statement from the European Reference Networks

(ERNs) Network Coordinators

28 February RARE DISEASE DAY!

As Coordinators of the 24 European Reference Networks (ERNs), we stand together and united with our 900 strong members (healthcare providers) and the patient community, in the fight against rare diseases. Rare diseases are indiscriminate in nature and do not recognise borders. Individually no one country can successfully address the specific challenges rare diseases presents. Rare diseases clearly evidence the need for continued and enhanced EU-wide collaboration. ERNs bring together the collective knowledge and expertise of clinicians and researchers from across Europe, to take meaningful steps in tackling the impact of these rare diseases, demonstrating EU-added value.

We believe that European Union and its members should remain steadfast in their resolve to tackle rare diseases and complex conditions requiring highly specialised healthcare together, taking steps to safeguard our activity to help the complex needs of this vulnerable patient population. We want to highlight in the strongest possible terms the importance of maintaining our current membership of the ERNs. **We call upon the European Commission and the UK government to ensure that a solution is found so that the UK healthcare providers can continue to participate in the ERNs and all our patients can benefit from our collaboration.**

Rare in presentation, these diseases are devastating for the approximately 30 million EU citizens living with a rare disease. Rare diseases often mean a lifetime pursuit to find an accurate diagnosis, an increasing burden of needs and a significantly reduced quality of life. Patients and experts are few, geographically scattered and often isolated. Reliable data and information is scarce and research is fragmented, often resulting in limited effective treatments and therapies. This leads to poor outcomes for patients and, in some cases, reduced life expectancy.

ERNs represent a paradigm shift in how we work together, breaking the isolation of patients, connecting information and data and fostering collaboration between experts and researchers. ERNs will revolutionise access to care and be the hot-bed for innovation and driving force accelerating the development of research and new therapies. The ERNs have only recently been formally established and are at a crucial stage in their genesis, and we

believe that the loss of the active input of the UK groups would be disadvantageous for the UK and the EU for the reasons listed below. The UK National Health Service (NHS) is involved in 23 of the 24 networks (approximately 40 NHS hospitals), with NHS Trusts leading a quarter (6)¹ of these networks and many further lead on delivery of outcomes from within their respective ERNs. It is of the utmost importance to secure the continued and lasting involvement and fruitful collaboration of UK colleagues, especially in the formative years of the ERNs.

1. Pooling of scarce expertise

Rare diseases are rare, experts are rarer. The success of the ERNs will be measured by their ability to draw on and harness the collective (and often limited) expertise and knowledge across Europe. 70% of healthcare provider ERN members come from Western Europe - France, Italy, Germany, the Netherlands and the UK. We, as a community, cannot afford to reduce the pool of scarce experts working together in the ERNs, as this would result in fragmenting the already scarce expertise across the needs of all 28 Member States. This would risk our ability to deliver our mandate and ambition to provide highly specialized advice and recommendations for treatment, drive research and innovation and deliver cross-border training and education.

The legal basis for the ERNs is the EU law on reciprocal healthcare (the EU Directive on Patients' Rights in Cross-Border Healthcare). Ending reciprocal healthcare arrangements with the EU post-Brexit would jeopardise the UK's participation in the ERNs. This would have the effect both of depriving UK patients' access to the best specialised care for their specific condition and of depriving EU patients outside the UK of the benefits of UK specialists' knowledge and expertise.

2. Improving access to high quality healthcare

ERNs are an equalising force for patients to develop and share best practice, offering both the EU and UK patients' quicker access to expert advice, diagnosis and treatment

¹ UK led ERNs

ERN for rare hepatological diseases, Prof. David Jones; Newcastle Upon Tyne Hospitals NHS Foundation Trust (NUTH)

ERN for rare immunodeficiency, autoinflammatory and autoimmune diseases, Prof. Andrew Cant; NUTH

ERN for rare neuromuscular diseases, Prof. Kate Bushby; NUTH

ERN for rare urogenital diseases and complex conditions, Prof. Chris Chapple; Sheffield Teaching Hospitals NHS Foundation Trust

ERN for rare epilepsies; Prof. Helen Cross; Great Ormond Street NHS Foundation Trust

ERN for rare congenital malformations and rare intellectual disabilities, Prof. Jill Clayton-Smith; Central Manchester University NHS Foundation Trust

recommendations, drawing on the experiences and knowledge of the ERN members through the new Clinical Patient Management System (CPMS) and to direct care locally.

CPMS is an innovative web-based platform provided by the European Commission that allows healthcare providers in our Networks to provide and receive advice on challenging cases by using an unprecedented Europe-wide consultation technology, responding to the most advanced EU standards in terms of security and data protection. We are now within a critical period as we are all working together to implement the CPMS. Using the CPMS, our ERN healthcare providers are setting up 'virtual' advisory panels of medical specialists across different disciplines and discussing cases in real time. This is an incredibly exciting time for us as we now have the capability to spread our knowledge and expertise without the need for patients to travel. Our collaborative digital health efforts are also addressing the problem of equity of access of specialist services and overcoming financial obstacles to better care. By safeguarding the continued membership of UK experts in ERNs, this will enable ERNs to harness the rich and diverse experience of rare and complex presentations of patients, to guide prescribing high-cost treatment and optimise patient outcomes and quality of life. Ultimately, this will reduce ineffective tests and treatments.

3. The EU and the UK as a world-leader market for innovation and research

Action which makes it more difficult for the UK and the EU to collaborate will diminish the attractiveness of industry investment in the EU and the UK. Clinical research, including clinical trials, has benefited from UK and EU researchers working together, especially for rarer and paediatric diseases where the UK has the highest number of trials. Because the number of patients with individual rare conditions in each country is low, it is only possible to locate and recruit enough patients for clinical trials by collaborating across countries. A situation whereby UK trials were no longer able to recruit European patients, or UK patients were no longer able to participate in European trials would lessen the benefits for patients in the UK and across the whole of Europe, as well as risking damage to the UK and European scientific reputation.

ERNs are the vehicle to drive forward research and innovation. Bringing together a pan-European population allows the critical mass of expertise, patients and their data in 'ready-made communities'. ERNs are set to revolutionise how research is delivered, with healthcare as research and research as healthcare. We are dedicated to creating an integrated community with our patients that will optimise research and clinical trials,

attracting investment in the EU and the UK as a world-leading market for innovation and research.

4. Safe and sustainable supra-specialised workforce

ERNs are ideally placed to collectively develop the safe and sustainable supra-specialised workforce through ERN cross-border training and educational activities. As new technologies, especially in the fields of genomics, imaging and biomarker studies increase diagnosis of rare diseases, the number of patients needing specialised input is expected to increase. We need to build the capacity in the countries currently without expert centres to meet all the needs of their population and train the experts of the future now.

In conclusion, we ask that our patients are put at the heart of the forthcoming EU negotiations with the UK under Article 50. We want to aim for **the optimum possible outcomes for our patients**. We believe that the ERNs are therefore essential in providing us with the ability to work together, to conduct joint research, to educate our colleagues and in particular to train the next generation. There is no doubt that the ERNs are a landmark opportunity for interdisciplinary collaboration between healthcare professionals working in close collaboration with patient groups.