



Feedback on the EXPH opinion -

**Application of the ERN model in European cross- border healthcare cooperation
outside the rare diseases area**

European Alliance for Vision Research and Ophthalmology (EU EYE)

October 2018

ID in Transparency Register: 221589017973-83



European Innovation
Partnership on Active
and Healthy Ageing
PARTNER

The EU EYE applauds the concept of ERNs. Such concept has the potential to contribute to research in improving health outcomes in rare eye diseases particularly in transplant cases for children which are uncommon and with poor prognosis.

We believe that equity should be at the core of the ERNs. The ERNs are part of the complex legislative piece of the Cross Border Directive. There is therefore the need for clarity and transparency in any process whether for the enlargement of the ERNs or enrollment of patients to access this innovative way for diagnosis and treatment in rare diseases. The EU EYE would therefore kindly ask the EXPH to take into consideration the following points:

- Proactivity and clarity in informing the public (providers and citizens) and ‘advertising’ the services of the ERNs must be adopted widely, if the goal of equity is to be achieved. Only a proactive engagement with providers will improve access to the ERN tool and improve statistical validity at the same time as the number of participating patients will increase. Particular efforts should be made to reach small Member States with little resources. Otherwise there is a risk that the ERNs will not deliver what they set out to do and they will contribute to health inequities.
- Proactive reaching out should be monitored and evaluated regarding its effectiveness at all levels. Proactivity and opening up of the system will create a positive feedback loop in the ERN system particularly as demonstrating benefit at regional level will enlist bottom-up support among public providers. This will accelerate the integration of ERNs in the national health care systems as public providers have an influence on their national system particularly when they are involved in the commissioning of services. Currently how one can be involved with the ERNs (whether as provider or patient) is left to the discretion of the individual ERNs and the Member States
e.g. the [EpiCARE: a European Reference Network for rare and complex epilepsies](#) invites healthcare professionals who want to refer patient to them or patient and family member of patients to get in touch via their email or contact page; the website of the NHS UK ¹ provides clear guidance on eligibility of healthcare providers to join an ERN as set by each commissioning body (NHS England, NHS Scotland, NHS Wales and the Department of Health, Social Services and Public Safety in Northern Ireland).

The EU EYE believes that participating members of ERNs could proactively reach out to organisations in their own Member States by:

- targeting clinics and hospitals which are recognised by their national/regional health authorities as focal points for rare diseases (although not part of the ERNs). This includes hospitals which serve as official certification centres for rare diseases and are linked to social care.
- exploring synergies with existing national disease registries and also with Europe-wide registries currently sponsored either by EU match funding such as EUREQUO

¹ <https://www.england.nhs.uk/commissioning/spec-services/highly-spec-services/ern/>
<https://www.england.nhs.uk/commissioning/wp-content/uploads/sites/12/2016/01/ern-guidance-provider-application.pdf>
<http://www.eueye.org/>

(European Registry of Quality Outcomes for Cataract and Refractive Surgery) or through research funding such as ECCTR (European Cornea and Cell Transplantation Registry). National and European registries can easily pool out data concerning rare diseases e.g. child corneal transplants (rare cases with poor prognosis) or pediatric cataract. This will increase number of data but above all it will prevent fragmentation and duplication maximising at the same time the EU investment already in such registries.

The EU EYE believes that a number of issues need to be addressed early in the ERN development to facilitate uptake of guidelines, recommendations and care plans:

- The CPMS online platform for patient referral within ERNs meets all the advanced EU standards in terms of security and data protection. However it is not clear where the responsibility lies in case of 'medical errors' in diagnosis or treatment if knowledge travels to patient. Is it the ERN which generates the knowledge or the provider who will follow the advice and carry out the treatment. Although such issues may not be in the remit of this opinion of the EXPH, they may have an impact in the integration of the ERNs in the national referral schemes.
- Integrity of ERN products e.g. guidelines, recommendations etc must be addressed as early as possible in the ERN development process to ensure their uptake. In the case of rare diseases it may be quite difficult to establish a guideline evaluation group due to the small community of expertise and there are concerns how the biasing impact of financial conflicts will be managed in such virtual networks. This is of particular importance to rare diseases where expensive treatments may be introduced with no assessment regarding effectiveness. It is therefore important for the ERNs to select an appraisal process and instruments for any guidelines or recommendations that aim to change clinical practice such as the Appraisal of Guidelines Research and Evaluation (AGREE) Instrument (<http://www.agreecollaboration.org>) as the gold standard for guideline appraisal and endorsed by the World Health Organization, the Council of Europe, and the Guidelines International Network (<http://www.g-i-n.net>).

The EU EYE would also like to make the following additional point regarding the organising of the ERNs:

- There should be readily available lists of which rare diseases are considered by the ERNs to enable healthcare providers deciding whether to proceed for a patient referral to an ERNs. e.g. umbrella headings such as paediatric rare diseases do not give any indication regarding the existence of expertise for specific diseases.