European Reference Networks (ERNs)

Q&A for Patient Advocates

European Reference Networks (ERNs) have been the focus of much discussion at EURORDIS events, including the EURORDIS Membership Meeting 2015 and EURORDIS member webinars.

The below is a compilation of questions asked during these discussions, divided into topics:

For further information:

EURORDIS web section on ERNs:
www.eurordis.org/european-reference-networks

European Commission webpage:
http://ec.europa.eu/health/ern/implementation/faq_en.htm

This Q&A will be developed based on your feedback and policy developments.

Please contact Matt Johnson, EURORDIS Healthcare & Research Director, with any questions: matt.johnson@eurordis.org.

1. Timing

- **What is the schedule for the first call for ERNs?**

The European Commission ERN Delegated Acts require the first call to have been launched by 27 May 2016. It is expected that **first call will be in spring 2016, possibly in March.** There will be 3 months to develop an application.

2. Patient involvement in ERNs

- **Can patient organisations be involved before the formation of ERNs, and if so, how?**

Patient organisations can engage with clinicians who are preparing an application to help shape the scope of rare disease ERNs and potential services provided by successful ERNs (but this is not a formal requirement in the legislation). The European Commission and Member States strongly encourage patient involvement in ERNs, as set out in the addendum to the EU Committee of Experts on Rare Diseases Recommendations on ERNs. EURORDIS has strongly advocated for full engagement of patient representatives in the ERN application process, governance and activities of ERNs.

- **What is the role of patients and patient organisations in each ERN?**

ERNs have to demonstrate they are patient-centred and empower patients as defined in the European Commission Delegated Decision. Patients and patient organisations will play a critical role in rare disease ERNs due to their expertise in their rare disease. The Commission Expert Group on Rare Diseases Addendum gives a clear strategic message that ERNs should involve patient representatives to play an active role in the governance structures of ERNs. This includes:

- To advise on planning, assessment and evaluation of Centres of Expertise and European Reference Networks based on their experience, with a consistent approach
- To ensure transparency of quality of care, safety standards, clinical outcomes and treatment options
- To promote and encourage a patient-centric approach in both delivery of clinical care, service improvement and strategic development and decision-making
- To ensure all ethical issues and concerns for patients are addressed, balancing patients' and clinical needs appropriately
- To ensure care is patient-centred and respects patients' rights and choice
- To ensure the application of personal data protection rules, compliance of informed consent and management of complaints
- To ensure feedback on patient experience and the active evaluation of patient experience

This Addendum has been approved by members of the Commission Expert Group on Rare Diseases including Member States, the European Commission and the Joint Action partners, therefore providing a strong strategic direction for ERNs to act on.

In order to enhance patient representation and empower the patient representatives in each ERN, EURORDIS wants to facilitate and support patient organisations to participate in the ERN decision-making processes and activities through a structured and democratic approach.

EURORDIS is working with its members to establish a *EURORDIS Patient Advocacy Group* (EPAG) for each of the 21 ERN groupings. This initiative stems from the EURORDIS General Assembly 2015 in Madrid and was

further developed by the EURORDIS Council of European Federations in October and approved by the EURORDIS Board of Directors in November 2015. All EURORDIS members are being consulted during the first quarter of 2015 to discuss which ERN grouping they belong to and elections of representatives to the EPAGs will take place in due course.

EURORDIS will gather elected patient representatives who will ensure that the patient's voice is heard in the successful development of ERNs. Through these EPAGs, patient representatives will help organise and exchange information, contribute to the decision-making process within the ERN, take action to collect feedback from patient groups at local levels and from patients and families, and participate in the creation or maintenance of registries, best practices guidelines etc.

- **In the implementing decision there is mention of collaborative/ associated members of ERNs – what are these and can patient organisations get involved?**

Collaborative and associated members are centres which can also be part of an ERN. There is no definition of these centres in the European Commission ERN Delegated acts and they are not assessed as part of an ERN application. Only HCP that have been identified as expert in the Member State legislation can formally sit on the Network's Board and be assessed against this EC legislation. It is the responsibility of Member States to identify and define the role and function of collaborative and associated centres in an ERN.

3. Groupings of diseases

- **How do multi-disciplinary diseases fit into one ERN?**

It is expected that multi-system rare diseases will be supported in a number of relevant ERNs, with these networks working together to meet the needs of rare disease patients. The important aspect is to ensure that, by working with ERN applicants, the scope of an ERN application includes these diseases. Different ERNs are expected to work together for the benefit of patients living with a multi-system condition or disease.

It is unfeasible to create a separate ERN for every one of the over 6000 rare diseases that exist; ERNs will therefore be organised according to disease groupings. This grouping of diseases does not prevent a patient from being able to go to a disease-specific centre of expertise, nor from benefiting from the expertise of several ERNs.

4. Funding

- **Is there funding available to support ERNs?**

Presently there is no confirmed funding for ERNs, however it is expected that there will be grants available to support the ERNs over the first five years. EURORDIS is strongly advocating for at least a minimum resource or "seed money" to support the coordination of ERNs. In addition, it is considered that ERNs will be in a strong position to secure research grants and other funding due to the fact that they will have been successfully awarded a European Commission ERN status following a formal assessment process.

5. Quality of expertise

- **One concern is that the quality of Centres of Expertise (CoE) is so broad. Before entering into ERNs, we need to establish these criteria to make sure all CoEs meet standards. Who is it that decides these standards and how?**

It is each Member State's responsibility to assess the quality of their CoE and healthcare providers (HCP) and endorse their participation in an ERN application, according to their respective national legislation. Member States are at various stages of developing legislation to designate or assess their CoE and HCP ready for the first call.

As part of the assessment process for ERN applications, the European Commission will assess the quality of each CoE and HCP in an application against the Delegated Decision's general and specific conditions/ criteria. This will ensure that CoE and HCP participating in an application meet clear and robust quality criteria both at a national and EU level.

In addition, during the application process, the ERN itself will define the threshold or required level of disease-specific expertise or competency that CoE and HCP will need to meet to be a member of an ERN. The ERN has to validate their application by providing evidence that the required level of expertise is met.

The final level of assurance is that each CoE within a network are required to verify that each centre continues to meets the required competency as defined by the legislation, throughout the 5 years that networks have been approved for operations.

If one or more HCP or CoE, at any point in the five years, ceases to comply with the conditions and criteria set out in the Delegated Decision, they are required under the legislation to highlight this to the Network's Board, whose members should then report this to the Board of Member States.

- **How will compliance to the requirements of the ERNS be regulated to ensure that standards are maintained? What sanctions will there be for CoE which do not continue to meet the required standards?**

ERN applications will need to demonstrate compliance with the conditions and criteria in the Delegated Decision. If a network application does not meet some of these conditions and criteria it will need to include a plan on what it will do to achieve compliance in the first five years. This will be monitored by the Network's Board. At the end of the five years, these networks will be evaluated by the European Commission for a renewal of their network as an ERN.

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- **Which bodies will endorse an ERN in Member States?**

Each Member State has a named representative on the Board of Member States (BoMS). The BoMS oversees and approves ERN applications, following an assessment by the European Commission's Independent Assessment Bodies. These representatives are working with their respective Member States to ensure that the endorsement process in established in their Member State.

6. *Clarification of impact of ERNs*

- **Is there a risk that through ERNs you might not be able to go to your normal hospital?**

ERNs create a clear governance structure for knowledge sharing and care coordination across the EU. They are networks of centres of expertise, healthcare providers and laboratories that are organised across borders.

Patients will continue to visit their local hospital and not see any changes in how their care and treatment is given, but they will see improvements to the outcome of their treatment; the changes will be seen in how, because of ERNs, clinicians will be able to liaise through a wider clinical network spanning Europe, connecting up with experts in specific rare diseases and getting advice or sharing knowledge of complex and rare cases that will ultimately improve the outcome of the care provided.

Patients have the right to choose where they receive care and need to liaise with their own national healthcare system to make an informed choice. ERNs support local provision of care to the patient where possible, and encourage experts to share their expertise and knowledge with national, regional and local healthcare systems. **ERNs promote the sharing and mobility of expertise, rather than the movement of patients themselves across borders.**

- **Does involvement in an ERN make European patient umbrella organisations redundant?**

European Reference Networks are clinical networks that are being established to create a clear governance structure for knowledge sharing and care coordination across the EU. They are networks of centres of expertise, healthcare providers and laboratories that are organised across borders and **not patient organisations networks**.

7. Incentive for clinicians

- **How do we convince professionals to make these networks? They already have a lot of work and are often not interested in rare diseases.**

ERN applications require strong cooperation between clinicians as this will reflect their ability to provide a functional and operational ERN if an application is successful. The strongest applications in the first call will be those where there is a well-developed network of clinicians who can demonstrate their cooperation to making their network functional.

There are significant benefits and opportunities in the creation of an ERN, which clinicians will respond positively too, including:

- Connecting up scattered expertise to increase understanding of rare diseases, natural history and increase in diagnosis and outcome to treatment
- Increased critical mass for research and ability to successfully secure research grants
- Improve access to high quality diagnosis and healthcare and reduce inequalities in care
- Share learnings and eHealth / IT platforms support
- Reduce ineffective treatment and inappropriate use of scarce resources
- Maximise integration and interoperability of EU and national strategic projects
- Provide a clear interface for industry, attracting investment opportunities and economic growth

Clinicians do not have to develop an application in the first round of call for applications in Spring 2016 as it is expected that there will be other future calls for ERN applications. The timing of these future calls has not yet been published.

There is significant interest from the clinical community to take part in these networks and an **ERN application has a minimum requirement of 10 healthcare providers from 8 Member States**. It is important to reach out to clinicians in your Member State and across the EU to work together to ensure that this minimum requirement is met and that HCP are endorsed by their respective Member State.

There will be different infrastructures to support successful ERN applications, which will support the sustainably of networks in the long term. It is likely that ERNs will be able to apply for other EU project and research grants to support the improvement to diagnosis and improved quality of healthcare.

For the rare disease community, sharing of knowledge and expertise and connecting up the clinical community will enable a levelling up of the knowledge of healthcare practitioners in the ERN, as well as to national, regional and local healthcare systems, bringing this expertise to the patient. This will see improvement to the outcome of diagnosis and treatments available in Member States.

8. Existing legislation

- **How does national and EU legislation align?**

The mapping report of the current state of the art on national assessment systems, completed in the development of the European Commission's Assessment Manual and Technical Toolbox for the assessment of ERN applications, shows that whilst there is variation in the processes MS employ to assess their respective HCP and CoE, the themes they assess these centres by are in line with the European Commission's Delegated Decision.
This European Commission legislation outlines the conditions and criteria a network and its HCPs are required to meet to be awarded ERN status.