Webinar on Rare Disease ERNs, 21 July 2015
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Content

• What are European Reference Networks?

• How will European Reference Networks be grouped?

• When is the call for applications from networks of Centres of Expertise / healthcare providers in EU Member States that want to become European Reference Networks?

• What are the key actions a patient organisation like yours can take to get involved?
What are European Reference Networks?
Their purpose ... 

European Reference Networks should ...

“improve access to diagnosis, treatment and the provision of high-quality healthcare to patients who have conditions requiring a particular concentration of resources or expertise, and ... could also be the focal points for medical training and research, information dissemination and evaluation, especially for rare diseases.”

*Commission Delegated Decision (2014/286/EU)
Healthcare and conditions covered:

Commission Delegated Decision (Article 2):

• ‘Highly specialised healthcare’ means healthcare that involves high complexity of a particular disease or condition in its diagnosis or treatment or management and high cost of the treatment and resources involved;

• ‘Complex disease or condition’ a particular disease or disorder which combines a number of factors, symptoms, or signs that requires a multidisciplinary approach and well-planned organisation of services over time because it implies one or several of the following circumstances:
  • — a large number of possible diagnoses or management options and co-morbidity,
  • — difficult interpretation of clinical and diagnostic tests data,
  • — a high risk of complications, morbidity, or mortality related to either the problem, the diagnostic procedure or the management
What will one look like?

Networks must have:

- Minimum of 10 Healthcare providers (Centres of Expert) from a minimum of 8 Member States
- No maximum number of healthcare providers in a network

Can include:

- Associated National Centres (focus on provision of healthcare)
- Collaborative National Centres (focus on production of knowledge, tools, improve quality of care)

European Reference Networks will have:

- Virtual networks of expert centres cross Europe with different models of Centres of Expertise (from multi-disease to single diseases centres)
- ERN Board with a nominate Coordinating Centre
- Governance structures for the safe and legal handling of clinical information and coordination of care
- Annual call for new members of existing ERNs
Possible functions and services offered:

• Principle that the expertise will travel to the patient, where possible. In some cases, patient maybe referred for treatment in one of the ERN centres.

• Networks activities – governance meetings, development of clinical pathways and therapeutic policies, best practice guidelines, registries & data collection, review of clinical outcomes, discussion of difficult cases or rarer cases, exchange and disseminate knowledge on clinical care through training and education programmes.

• Possible services offered - clinical or tumour boards, virtual multidisciplinary review, virtual consultation, second opinions, confirm diagnosis, recommend treatment / care plan, telemedicine.
### Study of services project

The study of services to be provided by the European Reference Networks and its Members

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**WP1** – Review current models, typologies and methodologies of services provided by healthcare providers, Centres of Expertise in networking conditions, mapping existing practices and experience of already established networks for highly specialised healthcare in the EU.

**WP2** – Develop a catalogue of possible healthcare services that could be provided by the future European Reference Networks.

**WP3** – Identify the cost drivers, develop simulation models and estimate costs for the possible healthcare services to be provided by future European Reference Networks.

**WP4** – Produce an integrated report and catalogue of healthcare services and cost estimations of the Healthcare Services to be provided by the future European Reference Networks.

**WP5** – Coordinate management and engagement activities through a team of experts in the field of health economics and delivery of highly specialised healthcare services.

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WP5: Expert Group (x2) engagement through a series of three workshops with national authorities, clinical network (including patient members each)
How will European Reference Networks be grouped?
Shared Vision

POSITION:
• EURORDIS has advocated strongly that RD ERNs should not be created for every single RD, on the grounds that this would mean creating hundreds of ERNs (impossible) and leaving many patients without an ‘umbrella’ ERN, a ‘home ERN’ (unethical and inefficient)

RATIONALE:
• European Commission’s Delegated Acts require Networks to have at least 10 members from 8 Member States
• Impossible to create 6-8000 individual ERNs, not even hundreds
• Specific needs of rare disease patients require an integrated strategic approach due to low prevalence, isolated cases, fragmented care, scarce expertise and high cost interventions
“Organic approach” → would mean leaving it to ad-hoc network applications

Risks linked to this approach would turn against interest of PLWRDs & professionals:
- Several applications competing for each rare disease area
- Greater fragmentation, loss opportunity of both verticality and horizontality
- Increased isolation of most vulnerable population amongst PLWRDs
- High proportion of rare diseases not covered by a RD ERN

“Shared strategic structured approach for RDs”:

Opportunities linked to this approach:
- Maximise inclusion of all PLWRD
- Improve access and reduce inequalities
- Establish a structure for expansion of RDs covered, expertise and functions of ERNs
- Share learnings and tools
- Improve dissemination of scarce knowledge and “level-up” expertise
- 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
Our Model for Rare Disease ERNs

• One RD ERN per thematic disease group

• Operational RD ERN constituted of several Clinical RD ERN with a diversity of CoE models, mixing single-disease CoE and broader overarching CoE

• Flexibility of model and membership to reflect the diversity of needs, functions and tools even though some common core competency are needed and expected, flexible evolution of members

• Dynamic stepwise approach to the expansion of disease coverage within a grouped RD ERN – a real life & bottom up approach – as well as a process of progression of the activities over years

• Patient organisations as integral members of Network Boards, to demonstrate RD ERNs ensure patient’s involvement as equal actors

• Flexible and smooth management of care both between CoE in a RD ERN, and between RD ERN – patients’ pathway
The Addendum to EUCERD Recommendation on ERN / RD Grouping and Patient Engagement, has been adopted at CERD Meeting 10-11 June
EUCERD - Grouping Rare Diseases in Thematic Networks (adopted)

- Rare immunological and auto-inflammatory diseases
- Rare bone diseases
- Rare cancers* and tumours
- Rare cardiac diseases
- Rare connective tissue and musculoskeletal diseases
- Rare malformations and developmental anomalies and rare intellectual disabilities
- Rare endocrine diseases
- Rare eye diseases
- Rare gastrointestinal diseases
- Rare gynaecological and obstetric diseases
- Group on Cancer Control

- Rare haematological diseases
- Rare craniofacial anomalies and ENT (ear, nose and throat) disorders
- Rare hepatic diseases
- Rare hereditary metabolic disorders
- Rare multi-systemic vascular diseases
- Rare neurological diseases
- Rare neuromuscular diseases
- Rare pulmonary diseases
- Rare renal diseases
- Rare skin disorders
- Rare urogenital diseases

*Note: The networking of rare cancers is currently under discussion in EC Expert
Expected benefits

Rare Disease ERNs are not isolated they are part of a (new) ecosystem on data and innovation:

- To further the quality of treatments based on expanded and more structured clinical expertise
- To create a structured approach to better and more data collection
- Enhance attractiveness of Europe for clinical trials, and hence scientific recognition of our experts as well as earlier access to innovation
- ERN status will be linked to access to funding from different EU programmes like CEF, digital & ehealth, most probably support from DG SANTE, potentially eligibility criteria for some calls in H2020, recognition and easier engagement in some public-private partnership eg IMI, long term access to structural funds
Call for ERN applications – the when and the how
Progress in Establishment of European Reference Networks

Since 2006

2006-2009
EU Commission pilots 10 ERNs

2007 – 2010
EURORDIS

RAPSODY project
Collects patient feedback on Centres of Expertise through qualitative workshops and quantitative EURORDISCARE survey

2008
EURORDIS Membership Meeting – ‘Centres of Expertise for Rare Diseases: How can we make it happen?'

May 2008
EURORDIS

EURORDIS members adopt a Declaration of Common Principles on Centres of Expertise and European Reference Networks

June 2009
European Council adopted the Council Recommendation on an Action in the Field of Rare Diseases.

2009
EURORDIS

POLKA project further explores added-value of ERNs and patient representative preferences in involvement based on experience of pilots

2011-2013
EURORDIS

Directive 2011/24/EU clarifies rules on patients’ rights in cross-border healthcare. EU countries have until 25 October 2013 to pass their own laws implementing the Directive which requires the Commission to support the development of ERNs (article 12).

2011
EURORDIS

July 2015
Webinar

EURORDIS ERN Webinar: dedicated to: What is an ERN? Grouping of RD ERN, the WHEN and HOW, deliberating best how patient organisations can preparation for the 1st Call for Application

2014
EURORDIS

EU Commission publishes a delegated decision listing the criteria/conditions that healthcare providers and ERNs should fulfil and an implementing decision containing criteria for establishing/evaluating ERNs.

2015
EURORDIS

May 2015
MADRID

EURORDIS Membership Meeting: session dedicated to deliberating best ERN grouping model to ensure a “home” for all RD patients within the European healthcare system

2015-2016
EURORDIS

2016
EC & BoMS
Approve Successful ERN

June 2016

End of 2015 / January 2016

First call for applications from networks of CEs wanting to become ERNs

Today

2015
EURORDIS

EURORDIS leading EU Commission Tender to develop a technical proposal for:
- A manual including operational criteria for the assessment of all applications
- A toolbox provide guidelines for network applicants and independent assessment bodies to facilitate the application process

2016

WEBNAR

EURORDIS ERN WEBNAR: dedicated to: What is an ERN? Grouping of RD ERN, the WHEN and HOW, deliberating best how patient organisations can preparation for the 1st Call for Application

To 2025

End of 2025
ERNS will be established and developed

2006
EURORDIS

April 2006
EURORDIS

EURORDIS Membership Meeting – ‘Centres of Expertise for Rare Diseases: How can we make it happen?’

November 2008
EURORDIS

EU Commission adopted the Communication Rare Diseases: Europe’s Challenges along with a proposal for a European Council Recommendation

May 2008
EURORDIS

EURORDIS members adopt a Declaration of Common Principles on Centres of Expertise and European Reference Networks

2007

2008

2009

2011

2013

2015

2016

2017

2018

2019

2020

2021

2022

2023

2024

2025


European Reference Networks will be legal bodies, created by …

- The HOW - Implementation Decision (2014/287/EU) outlined the role of the ERN Board of Member States, assessment process and assessment by Independent Assessment Bodies.

- The WHAT - Delegated Decision (2014/286/EU) sets out the criteria and conditions a Network should fulfill (x6), and the Healthcare Provider (x7)

- MS Endorsement and approval by National Authorities of HCP to be a member of a Network application
ERN application process for a network

The Implementation Decision outlined the assessment process will include:

- The call for interest
- Member States’ endorsement of applications of their healthcare providers
- Submission of applications to the Commission
- Verification of the completeness of applications
- Technical assessment by an independent assessment body(s) of applications fulfill the criteria, possibly through self-assessment, desk-top review and on-site audit to validate evidence
- Communication of the assessment outcomes
- Approval of the Networks and their membership by Member States
- Publication of the list of established Networks and of their Members
Ambitious Roadmap

- EUCERD (CERD) Recommendations
- Adoption legal acts
- Awareness campaign launched
- Call for Assessment Manual and toolkit
- Identifying clusters of diseases workshop feedback to CERD
- Selected consortium to lead the study of support services for ERNs
- Selection of Independent Assessment body(ies), and networks guidelines and technical documents
- Call for Networks – Complete individual’s proposal and self-assessment
- Technical Assessment (criteria and conditions)
- Only positive assessment will be reviewed / approved by the Board of Member States to be established as an ERN network
- Establish of Networks

- Oct 2011
- March 2014
- 23 June 2014
- July-Sept 2014
- October 2014
- Jan-Apr 2015
- Q2 2015
- Jan 2016
- Spring 2016
- Summer 2016
Key actions for patient organisations to get involved
Necessity of a patient-centred approach to RD ERNs:

Patients and patient representatives should play an integral role in the decision and opinion making process in RD ERNs and be involved in all networking activities.

It is recommended that RD ERNs demonstrate meaningful patient involvement, patient-centeredness and empowerment through recognition of the role of patients, as experts by experience and co-producers of knowledge, in all ERN activities and in line with the legal requirements in the Delegated Acts.
EUCERD Addendum (adopted)

Patients’ involvement depends on the scope of ERNs in the following dimensions:

• 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 to 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
Readiness of Patient Groups

Involvement of patient organisations across Europe to support an integrated grouped model:

• **Patient organisations membership of RD ERN Boards** – essential that RD ERNs demonstrate meaningful patient involvement, patient centricity and empowerment to prove they meet the legal requirements in the Delegated Acts.

• **EURORDIS is preparing RD patient groups for the disease grouping to enable their full engagement through democratic representation, support, and quality contribution** - European Policy Action Groups (EPAGs - XX) will bring together the European / International federations, the relevant patient groups, the web-based patient communities.
Paradigm shift: combining unity & diversity

- EURORDIS wants to combine unity and diversity; EURORDIS will structure its membership base in **Policy Action Groups by rare disease groupings** so to enable patient engagement in European Reference Networks, Registries & Data Collection, European Research projects, R&D and Assessment of therapies, Screening & Genetic testing, Disease Management and Good Diagnostic & Care Practices, and associated ethical issues.

- EURORDIS’ Policy Action Groups per rare disease grouping will empower our members while being inclusive of and supportive to even rarest diseases; EURORDIS enables acting at national, European, International levels, partnering with all stakeholders, and in all strategic areas of public health, healthcare, research, social, human and patient rights, so to have a patient-centric 360° view.
Thank you