



EUCERD Recommendations on Rare Disease European Reference Networks (RD ERNs)

EUROPEAN REFERENCE NETWORKS FOR RARE DISEASES IN EUROPE: THE CONTEXT

The need for European Reference Networks (ERNs) for rare diseases

- There are over 6'000 rare diseases
- The challenge of rarity :
 - Patients are rare
 - Experts are rare
- Centres of expertise are a means of:
 - Revealing where expertise lies
 - Gathering experience to improve knowledge and care

Previous EUCERD recommendation on centres of expertise (2011)

- To help MS in their reflections/ policy developments concerning national plans and strategies for RD
 - Specifically in addressing the organisation of healthcare pathways at national and European level
 - Necessary steps to build up ERNs

European context

- Development of ERNs in the field of RD encouraged explicitly in:
 - Council Recommendation on an Action in the Field of RD (2009/C 151/02) (8 June 2009)
 - Directive on the application of patients' rights in cross-border healthcare (2011/24/EU) (9 March 2011)

ERNs in the Cross-Border Healthcare Directive

Directive on the application of patients' rights in cross-border healthcare (2011/24/EU) (9 March 2011)

- The Commission shall support Member States in the development of European reference networks between healthcare providers and centres of expertise in the Member States, in particular in the area of rare diseases
- Member States are encouraged to facilitate the development of the European reference networks:
 - (a) by connecting appropriate healthcare providers and centres of expertise throughout their national territory and ensuring the dissemination of information towards appropriate healthcare providers and centres of expertise throughout their national territory;
 - (b) by fostering the participation of healthcare providers and centres of expertise in the European reference networks.

ERNs in the Council Recommendation

Council Recommendation on an Action in the Field of RD (2009/C 151/02) (8 June 2009) encourages MS to:

- Foster the participation of centres of expertise in European reference networks respecting the national competences and rules with regard to their authorisation or recognition

EUCERD RECOMMENDATIONS



Elaboration of the Recommendations

- Concepts defined by the High Level Group on Health Care & Medical Services (HLG) and by the Rare Disease Task Force (RDTF)
- Initial discussion at EUCERD in January 2012
- Informal workshop and discussion at EUCERD plenary in June 2012
- Workshop in September 2012 to work on draft recommendations
- Draft sent to EUCERD MS representatives for review prior to November 2012 meeting & rediscussed by EUCERD
- Final draft elaborated and sent to EUCERD for adoption
- Recommendations unanimously adopted at EUCERD meeting 31 January 2013

Aim of EUCERD recommendations

- Inform the Commission services and expert groups working on criteria for the creation and designation of ERNs in the context of the Cross-Border Healthcare Directive on specificities of RD
- Inform Member States who are developing their healthcare pathways at both the national and EU levels in the field of rare diseases – above all in the context of national plans/strategies for rare diseases (recommended by end of 2013)

Structure of the Document

- Introduction
 - Background to the Recommendations
 - Scope of these Recommendations
 - Target groups for these Recommendations
- Recommendations to the MS and the EC
 - Mission, Vision and Scope
 - Governance
 - Composition of RD ERNs
 - Funding and Evaluation
 - Designation of RD ERNs
- Appendix

Target Groups for the Recommendations

- Member States
- European Commission
- Other EC initiatives (e.g. other projects and Joint Actions, Cross-Border Healthcare Expert Group, EUnetHTA, EPAAC)
- Centres of expertise in the field of RD
- Healthcare providers
- RD experts and existing RD network co-ordinators and partners
- Patient Organisations

Mission, scope and vision

- Mission of the ERNs
- Role of CEs
- Core tools and activities
- Mechanisms to ensure healthcare pathways for CEs and patients, including those with unclear diagnosis

Mission, Vision and Scope of RD ERNs

- ERNs provide framework for healthcare pathways for RD patients
- CEs are core participants in ERNs
- ERN to be flexible to work with different national CE structures

Mission, Vision and Scope of RD ERNs

- ERN to cover core tools and activities
 - Registries
 - quality assurance mechanisms for laboratory testing
 - mechanism for information flow for good practice guidelines
 - training/education tools
 - mechanisms for evaluation/indicators of performance
 - communications infrastructure
 - cross-border referral mechanisms
 - telemedicine core

Mission, Vision and Scope of RD ERNs

- ERNs provide guidance, definitions and mechanisms to ensure transparent/seamless healthcare pathways
 - Models to provide such a mechanism for patients without a clear diagnosis should be explored
- ERNs should have capacity to follow patients with unclear diagnosis and manage their care according to medical need

Governance

- ERNs should have robust and clearly defined governance with oversight structures and comparable methods for evaluation
- ERNs require strong leadership: coordinating site should show proven ability to coordinate network and shared tools

Composition of RD ERNs

- RD ERNs to deliver added value in at least 3 of the objectives of Cross-Border Healthcare Directive, Art.12
- Different forms of affiliation to ERNs to allow inclusivity

→ Sharing knowledge: An overarching goal

Composition of RD ERNs

- Composed of existing CEs and future ones to enhance collaboration
- Due to the complexity of rare diseases, ERNs will link CEs and stakeholders involved in the care management of the patients:
 - specialised health care providers
 - specialised social care providers
 - patient groups
 - diagnostic laboratories
 - relevant research groups

Funding and evaluation

- Financial system of CEs/ affiliated centres is a MS competence
- Funding for ERNs to support coordination and networking activities:
 - needs to be adequate, long term: at least 5 years
 - To be proportional to number of target patients, number of integrated centres, number of diseases covered
 - specific costs for networking should be part of a sustainable funding mechanism from EC funds

Designation

- Before ERN designation, the development of shared platforms across RD ERNs should be considered
- Clear RD ERN designation process
- Stepwise-strategy for ERN designation so all RD patients will have access to appropriate ERN in a defined period of time

Designation

- As there will only be a limited number of RD ERNs, priority given to those:
 - With existing formal/informal networks of experts
 - With patient organisation cooperation
 - With existing patient organisation networks
 - With sufficient existing activities of research output

Designation

- Based around the concept of medical specialties and body systems, diagnostic and therapeutic areas can be identified, covering a wide range of diseases – approximately 20-30 ERNs

→ Goal: to be established by the end of the Health For Growth Programme in 2020

Designation

- Formal system for networking across all RD ERNs and sharing expertise should be defined and implemented
- Working groups to be established as necessary to oversee implementation of RD ERNs, acting ideally under EUCERD auspices

Next steps

- Presented to the Expert Group on Cross Border Healthcare in February 2013 for use in their work on ERNs
- EUCERD ready to collaborate further in the field of ERNs for RD in the context of the implementation of the Cross-Border Healthcare Directive

Thank you for your attention!

The text of the recommendation can be found on the EUCERD's website

www.eucerd.eu

Key Documents (part 1)

- **Directive (EC 2011/24/EU) of the European Parliament and of the Council on the application of patients' rights in cross-border health care**
- **EUCERD Recommendations on Quality Criteria for Centres of Expertise for Rare Diseases in Member States (24 October 2012)**
- **EURORDIS Position Paper – European Reference Networks for Rare Diseases (May 2012)**
- **EUCERD Report: Preliminary analysis of the experiences and outcomes of ERNs for rare diseases (May 2011)**
- **Commission Communication, Rare Diseases Europe's Challenge**
- **Council Recommendation (2009/C 151/02) of 8 June on an action in the field of rare diseases**
- **Work of the High Level Group on Health Services and Medical Care during 2005**

Key Documents (part 2)

- **RDTF Report: Overview of Current Centres of Reference on rare diseases in the EU (September 2005)**
- **RDTF Report: Centres of Reference for Rare Diseases in Europe – State-of-the-art in 2006 and Recommendations of the Rare Diseases Task Force (September 2006)**
- **RDTF Report: European Reference Networks in the field of Rare Diseases: State of the art and Future Directions (July 2008)**
- **EUCERD Workshop Report: Centres of expertise and European Reference Networks for Rare Diseases (8-9/12/2010)**
- **EUCERD Workshop Report: National centres of expertise for rare diseases and networking between centres of expertise for rare diseases (21-22/03/2011)**
- **Eurordis Declaration of Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases, 15 November 2008**