



European Reference Networks for RDs: The patient's perspective

Flaminia Macchia

EURORDIS - European Public Affairs Director

ERNs in the CBHC Directive – April 2011

Finally (7 years after process started in 2004 with the HLG on Health Services and Medical Care → WG on ERNs), the CBHC gives a **legal basis** to ERN of HC providers & CoE – in particular in the area of rare diseases - based on voluntary participation.

→ Help realise potential of EU cooperation for highly specialised healthcare exploiting innovation in medical science & health technologies.

→ Improve diagnosis & delivery of high quality HC for patients with medical condition requiring a particular concentration of expertise.

→ The Commission shall support MSs in the development of ERNs: both EU and national business!



European Reference Networks for **RDs**

*“The main added value of the European Reference Networks and of the Centres of Expertise is the **improvement of access to both diagnosis and high-quality, accessible and cost-effective healthcare** for patients who have a medical condition requiring a particular concentration of expertise or resources, particularly in medical domains where expertise is rare”*

Summary report of the replies on the PUBLIC CONSULTATION ON THE IMPLEMENTATION OF EUROPEAN REFERENCE NETWORKS (ERN), European Commission

European Reference Networks for RDs

- There are over 6000 diseases which are rare.
- The challenges of rarity :
Patients are rare + Experts & expertise are rare
- CoE (nationally designated) = **nodes of the ERNs** but ERNs will also include HC providers, labs, patient groups and individual experts.
- CoE reveal where **expertise** lies and gather the existing experience to improve patient care.
- To organise the healthcare pathways at national and EU levels, it is necessary to build **networks**.
- CoE can have very different structures → ERNs need to be **flexible** to integrate these differences.



European Reference Networks for **RDs**

- Development of ERNs is encouraged explicitly in the **Council Recommendation** on RDs (2009)
- But legally established in the Directive on the application of patients' rights in **Cross Border Healthcare** (Dir. 2011/24/EU)
- ERNs have a key role in facilitating **patient mobility** as provided for in the CBHC Directive.
- **MS** are encouraged to foster participation of CoE & HC providers in the ERNs.
- **National Plans for RDs** also need to integrate the dimension of ERN → appropriate resources should be allocated.

Core tools & activities?

1. **Disease registries**: international terminology to support interoperability as part of global data-sharing effort.
2. ERNs should promote the use of lab testing facilities which participate in **Quality assurance programmes** (EuroGentest)
3. ERNs should develop a mechanism for **sharing good practice guidelines** for diagnosis and care between MSs
4. Training and education tools to raise standards of care
5. **Evaluation** of ERNs: multi-stakeholder (incl. POs) with indicators covering processes, outcomes and impact (PROs)
6. **Communications infrastructures** to ensure visibility of the ERNs, their processes & accessibility (own website & Orphanet)
7. **Cross-border referral** mechanisms to help operate the CBHC & Reg. on the coordination of SSS
8. In all of these areas, **Telemedicine** is core to support tele-consultation, training & education



European Reference Networks for RDs

Now, focus on implementation – what should be envisaged at national level (including National Plan):

- how to integrate different structures
- how to find adequate funding
- how to ensure real patient involvement
- how to provide comprehensive care
- how to promote research

Minimum common denominator: a ERN should gather a critical mass of patients to support research and be able to develop best practices.



(Rare disease) patients' vision

- **All RDs** covered by at least one ERN which focus on groups of diseases such as rare hematologic diseases, genodermatoses, rare pulmonary diseases, etc.
- ERNs should deliver & disseminate structured **healthcare pathways** through a high level of **integrated expertise** to improve diagnosis and care to the best European standards.
- **Future:** 20 to 30 RD ERNs to be established 'based on the concept of medical specialties and body systems: diagnostic and therapeutic areas can be identified each covering a wide range of rare diseases'

Rare disease patients' **vision**

- RD ERNs must be **multidisciplinary** to address multisystem disorders and include social care: network between Centre of Expertise, healthcare providers, social workers, patient organisations, genetic testing labs, research groups...



Patient involvement

- Patient representatives involved in the management of a ERN in a **meaningful way** – membership of steering committees/Board/project groups.
- Patient groups should be involved at **all levels** of activity, including governance and evaluation.
- ERN should promote **networking** of the patient groups representing the conditions covered (→ Federation).
- Participation of patient organisations should be a **prerequisite** for an ERN to receive funding.
- Budget of the ERNs should include **funding for patient organisations** to allow full participation.

Rome was not built in one day

- **Implementation will be stepwise and progressive**, starting from the most advanced & organised groups; ERNs will not spring up fully formed;
- Pilot projects led by the EC
- Better to **identify short, medium and long term priorities** and do them well rather than try to do everything at once;
- **Patients' priorities include:**
 - Best practice guidelines for diagnostic and care
 - Networking between HCPs & patient groups
 - Clinical research, registries, clinical trials
 - Social care

ERNs still in the making

- 2014: In the framework of the CBCH the EC will adopt:
 - List of criteria that **ERNs** must fulfill
 - List of criteria that **HC providers** must fulfill to join an ERN
 - Criteria for **evaluating** ERNs

→ MS should be able to implement those measures, e.g. identify CoE on the basis of adopted criteria.

- 2014: EC to launch a **call for candidatures of ERNs.**

→ MS, CoE and existing Networks should be prepared to participate. Are they in your country?

- In 2015, call Horizon 2020, the EC will fund a **pilot to define validated models of organisation of ERNs**

THANK YOU!



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