



The Patient View - Network organisational challenges and experiences

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Voicing Rare Disease patient's expectations

Fiodor - Autosomal recessive dopa-responsive dystonia

Voicing Rare Disease patients' expectations

People living with a rare disease have been central in the development of the policy, legislation and now implementation of ERNs:

- Started in 2004 with EurordisCare surveys (The Voice of 12 000 patients) and EU High Level Group Health Services and Medical Care & EURORDIS Membership Meeting in 2006
- Declaration of “Common Principles on Centres of Expertise and European Reference Networks for Rare Diseases” Copenhagen, 2008
- Advocacy toward EU Dir Patients’ Right to CBHC, 2008-2009
- Position Paper on “European Reference Networks for Rare Diseases”, May 2012 (post Directive)
- EUCERD Recommendations on ERN, 2013
- EURORDIS-EPF Comments to European Commission on ERN, August 2013
- Council of European Federations, Council of National Alliances, EURORDIS Board of Directors and EURORDIS Public Affairs Committee, October 2014
- EUCERD Workshop RD ERNs, 2014 & Recommendation Addendum 2015
- EURORDIS Membership Meeting Madrid, May 2015
- Advocacy to Member States and MEPs, 2015 - 2016

Delegated Decision and Implementation Decision (2014/286/EU and 2014/287/EU)

1. Horizontal criteria and conditions - Mandatory criteria that are **broad in nature for all ERNs**

- **Networks are required by law to demonstrate patient-centric care and to allow for patient empowerment**
 - **Patient Organisations are not specifically included** in governance, assessment and evaluation of networks.
 - However, **legislation does not exclude that networks include Patient Organisations in their governance**

2. Specific criteria and conditions - Specificities of rare diseases not included in the Implementing Decision, yet they can be included in its implementation.

Our Ambition To address patient challenges



Bryce - Sanfilippo syndrome

Our Ambition: Implementation of ERN to improve Patient Health Outcomes

- Defining **patient healthcare pathways**
- Identifying the **experts** and the existing **expertise** in rare diseases
- **Connecting scarce expertise and 'levelling up' knowledge**
- Creating the **critical mass of rare disease data**
- Pushing the **pace** of rare disease research & daily care practice
- Fostering **translational research and therapeutic products** into the market
- Engaging **patient advocates as actors** in healthcare offering

Our ambition is to:

- **Improve access to quality care, clinical excellence and patient health outcomes**
- **Drive greater integration and interoperability for rare diseases in healthcare within Member States and across Europe**

RD ERNs need to address 2 main issues:

1. Every person with a rare disease has a home, a pathway
2. Every person with a rare disease deserves quality of care



**Our Vision
for
strategically
conceived
RD ERNs
and
step-wise
progressive
approach**

Arseniy - Mucopolysaccharidosis I

Steering an optimal course

1) “Organic approach” → would mean leaving it to ad-hoc network applications

Risks linked to this approach would turn against interest of people living with rare diseases (PLWRDs) & professionals:

- Several applications competing for each rare disease area
- Greater fragmentation, loss opportunity both verticality and horizontality
- Increased isolation of most vulnerable population amongst PLWRDs
- High proportion of rare diseases not covered by a RD ERN

Steering an optimal course

2) “Shared strategic structured approach” for RDs

Opportunities linked to this approach:

- Maximise inclusion of all PLWRD
- Improve access and reduce inequalities
- Establish a structure with potential for expansion to more RDs covered, expertise and ERN functions
- 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 Vision: a Structured Approach

- **Grouping diseases into ‘families’** which can then be addressed by a comprehensive RD ERN
- This grouping has to be **meaningful for patients, experts, healthcare managers**
- RD ERN should be developed in a **step-wise approach, building on** and enhancing capacities of **already existing or most advanced disease specific networks**
- RD ERN are “**Operational networks**”, each composed of several “**Clinical Networks**”
- In the **long term**, each RD ERNs would ensure **every RD patient finds a ‘home’** in the thematic grouping for his/her disease

Our Vision: a Stepwise Approach

A **stepwise strategy** is needed to shape thematic RD ERN and this needs to be reflected in the application process:

- **Inform the Call for RD ERN applications** based on the vision and specificities captured in EUCERD guidance
- **Restrict to eligibility of one application only for each thematic RD ERN grouping**, not to be open to several applications
- **Start building RD ERNs on existing or more advanced / mature clinical networks**: formal or informal network of clinical experts, CoEs well identified, registries in place, active network of patient groups, products and clinical trials
- Require that applications **include 'progressive expansion plans' of disease coverage over multi-year periods: a dynamic approach**

Our Vision: a new eco-system

Rare Disease ERNs are not isolated

They are part of a new ecosystem on data and therapeutic innovation

- To further the **quality of treatments** based on **expanded and more structured clinical expertise**, including **good clinical practices**
- To create a structured approach for **better and more data collection**, using **new opportunities from eHealth and IT solutions**, based on **shared standards and principles**
- To enhance attractiveness of Europe for **clinical trials**, hence scientific recognition of our experts; **earlier access to innovation (diagnostic tools, therapeutic interventions)** and **evidence generation all along life cycle of medicines**

Combining unity & diversity

- EURORDIS wants to combine unity and diversity: EURORDIS will structure its membership base in **Policy Action Group / Patient Advocates Groups by rare disease groupings** so to enable **patient engagement in European Reference Networks** and relevant activities: 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 Advocacy Groups per rare disease grouping will empower our patient groups** while being **inclusive of and supportive to even rarest diseases**; EURORDIS supportive platform **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**

Readiness of patient groups

THIS STRATEGIC AND STEP WISE APPROACH IS ADOPTED

- EURORDIS position paper on ERN (May 2012)
- EUCERD Recommendations on ERN (January 2013)
- Council of European Federations, Council of National Alliances, EURORDIS Board of Directors and EURORDIS Public Affairs Committee (October 2014)

IMPLEMENTATION BY PATIENT ADVOCATES IS ONGOING:

- Consultation of members at the EURORDIS Membership Meeting (Madrid, May 2015)
- Forming the RD grouping of members and advising on where each rare disease best fits in a thematic grouping for RD ERNs, from diagnosis through to treatment, (2015, ongoing)
- Election of Policy/Patient Advocacy Groups to support RD ERN applications and operational delivery (2016 and onwards)

Take Home Messages

European networking has a very high EU-wide added value to overcome rarity, treat complex diseases and handle costly interventions

Main features of the proposed model:

- Integrated, structured approach with clear shared arrangement between levels of care (local, CoE, ERN) into pathways
- Focus on patient health outcomes, clinical excellence
- Grouping diseases into thematic families to be addressed by a comprehensive ERN: every patient has to have a home
- Stepwise approach, flexibility, dynamic expansion & improvement
- Patient Organisations integrated into governance of ERN to support ERN “patient centric care” and “empowerment” (legal requirements)
- EURORDIS provide the platform for patient groups engagement in ERN through European Policy / Patient Advocacy Groups by rare disease groupings

Thank you

*To Volunteers on Eurordis Public Affairs Committee and
To families for their photos*



Daniel -Sanfilippo syndrome