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
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
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