2nd EURORDIS member webinar on European Reference Networks – 13/10/15

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European Reference Networks

Implementation process: timeline and milestones

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How are things going?

- More than **50 interested groups** or pilot networks identified
- **Dynamic interaction** of stakeholders with MS and Commission
- **Preparatory work** of Network proposals ongoing
- **Interaction among professionals** of the same thematic diseases groups
- Active implication of **EU patients and professional/scientific societies**
- **Strong support** of the Commission advisory groups (in particular the RD expert group)
- Close **cooperation between Board of MS and Commission**
Challenges

- Attract and approve the right ERNs
- Effective Network model & useful platforms and tools
- Commitment of Member States and sustainability
- Avoid fragmentation and overlapping
- Develop & use standardised tools (clinical guidelines, registries, patient pathways, interoperable IT systems)

To involve healthcare professionals, health authorities, managers and patients in a **common project** of ERNs.
Network proposals: Preparatory and strategic activities

✓ Strengthening the network value and capacities:
  - Multidisciplinarity
  - Avoid fragmentation: Grouping of diseases
  - Identify mature and clear EU added value type of diseases
  - Discuss and Identify other players, partners and members

✓ Liaison with MS authorities (contact with ERN board representative at national level and discuss !!)
Network proposals: Preparatory and strategic activities

- Agree on the **specific criteria for each area of expertise**
- Define the services of the Network
- **Self-assessment exercise** *(Network and members)*
- Define **Pathways** models, referral criteria, clinical decision tools
- **Plan an Information** system
EURORDIS
Thematic Groups – Rare Disease European Reference Networks

Arseniy - Mucopolysaccharidosis I
Voicing Rare Disease patients’ expectations

Fiodor - Autosomal recessive dopa-responsive dystonia
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
**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 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.
Key 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
Readiness for the involvement of patient groups into RD ERNs
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 a preparing RD patient groups for the disease grouping to enable their full engagement through democratic representation, support, and quality contribution** - European Policy Advocacy Groups (EPAGs - XX) will bring together the European / International federations, the relevant patient groups, the web-based patient communities.
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)
EURORDIS Member Organisations / ERN Groupings

Critical areas in structuring patient engagement:
- High and low volume disease grouping networks to ensure equitable coverage and representation
- Align RD PAG and expansion plans of disease coverage for thematic grouped RD ERN
Mutual Benefit and Opportunities

Clear mutual clinical and patient benefits to a structured patient engagement approach for RD ERN

- **All diseases can be represented**

- **Unified patient voice with equitable representation** of all patient representatives for each thematic RD ERN and the Network Board

- **Optimal engagement** through highly developed capacity and capabilities of PAG representatives

- **360° patient centric view** – all strategic areas of public health, healthcare, research, social, human and patient rights

- **Good flow of information** to support all networking functions including: governance and clinical operational delivery

- **Clear compliance in meeting legal requirements** and CERD recommendations of patient empowerment in application and operational delivery
Daniel -Sanfilippo syndrome
Thank you!

Q&A