



## Engaging your organisation

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# Objectives

This capacity building sessions aims to:

- ✓ Present the rare disease grouping
- ✓ Identify for which group of RD there is already an application under development
- ✓ Explain how EURORDIS is structuring its membership per rare disease grouping and the democratic representation of patients groups in each ERN and will support the exchange of experience across the different ERN

# Rare Disease ERN Groupings

Fiodor - Autosomal recessive dopa-responsive dystonia

# 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: 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

**“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

## “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 **European Policy Action Group / Policy Advocacy 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' European Policy / Patient Advocacy Groups (EPAGs) 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 European Policy/Patient Advocacy Groups to support RD ERN applications and operational delivery (2016 and onwards)

# 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 (EPAGs)

# EUCERD Rare Disease Groupings

Rare immunological and auto-inflammatory diseases	Rare craniofacial anomalies and ENT (ear, nose and throat) disorders
Rare bone diseases	Rare Hepatic diseases
Rare cancers* and tumours	Rare hereditary metabolic disorders
Rare cardiac diseases	Rare multi-systemic vascular diseases
Rare connective tissue and musculoskeletal diseases	Rare neurological diseases
Rare malformations and developmental anomalies and rare intellectual disabilities	Rare neuromuscular diseases
Rare endocrine diseases	Rare pulmonary diseases
Rare eye diseases	Rare renal diseases
Rare gastrointestinal diseases	Rare skin disorders
Rare gynaecological and obstetric diseases	Rare urogenital diseases
Rare haematological diseases	

Source: [http://ec.europa.eu/health/rare\\_diseases/docs/20150610\\_erns\\_eucerdaddendum\\_en.pdf](http://ec.europa.eu/health/rare_diseases/docs/20150610_erns_eucerdaddendum_en.pdf)

03/11/2015

What applications are being developed?

What is the scope of rare diseases covered in these applications?



Alla - Myasthenia Gravis

**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 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

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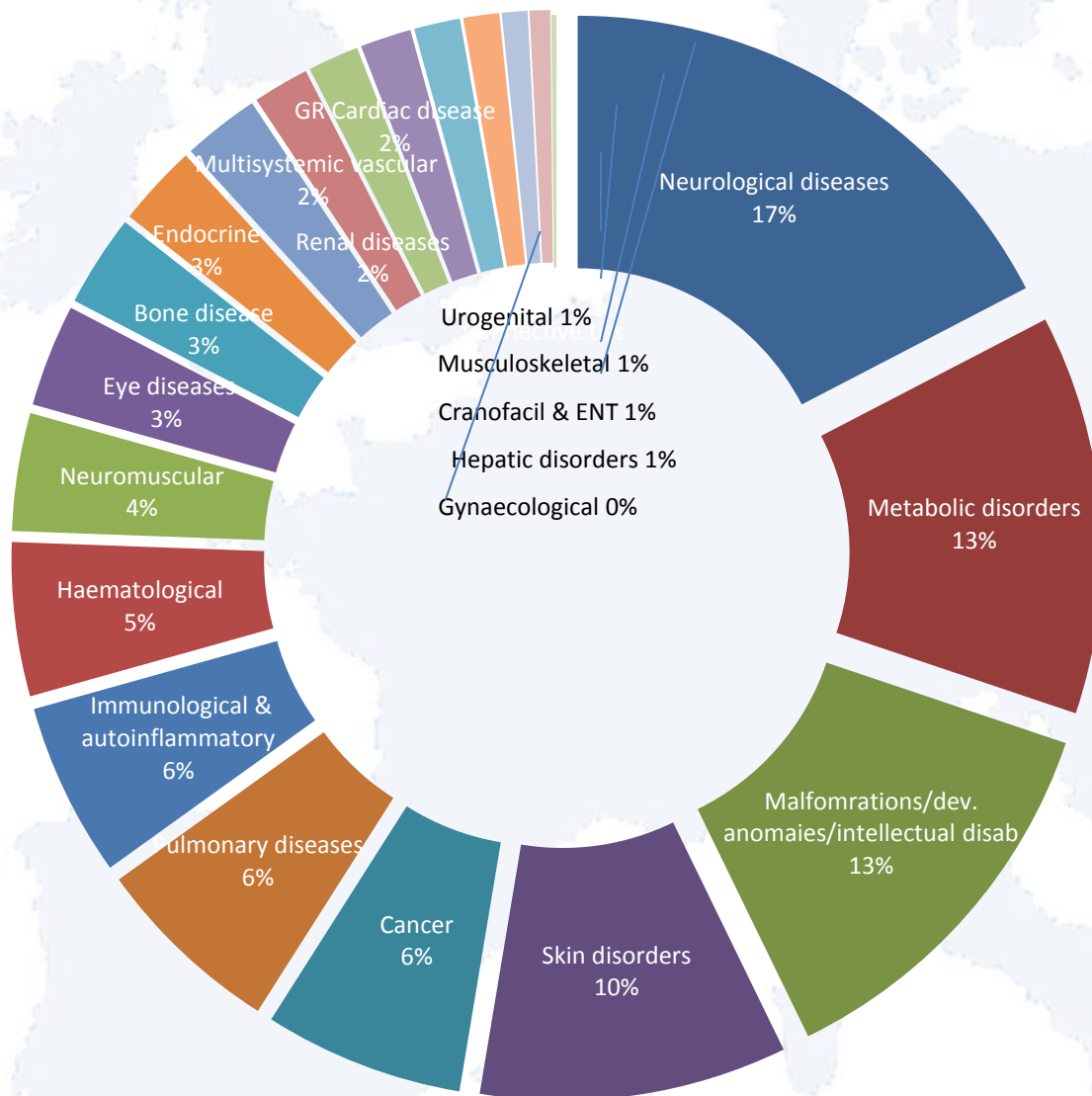
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# EURORDIS Member Organisations / ERN Groupings



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



# EURORDIS Member Organisations / ERN Groupings

ERN Group	Number of member organisations
GR Neurological diseases	106
GR Metabolic disorders	78
GR Malformations/dev. anomalies/intellectual disab	77
GR Skin disorders	60
GR Cancer	39
GR Pulmonary diseases	37
GR Immunological & autoinflammatory	34
GR Haematological	30
GR Neuromuscular	23
GR Eye diseases	20
GR Bone disease	18
GR Endocrine	16
GR Connective tissue & musculoskeletal	15
GR Multisystemic vascular	11
GR Cardiac disease	10
GR Renal diseases	10
GR Urogenital diseases	9
GR Hepatic disorders	7
GR Cranofacial & ENT	5
GR Gastrointestinal	4
GR Gynaecological & obstetric	1
<b>Grand Total</b>	<b>610</b>

*Plus 79 umbrella organisations*



## Comparative list of classification - ERN / Orphanet

ERN Grouping	Orphanet Classification
<b>Corresponding classification</b>	
Rare bone diseases	Rare Bone diseases
Rare endocrine diseases	Rare Endocrine disease
Rare eye diseases	Rare eye diseases
Rare gastrointestinal diseases	Rare gastroenterological diseases
Rare gynaecological and obstetric diseases	Rare gynecological & obstetric disease
Rare haematological diseases	Rare hematologic
Rare hepatic diseases	Rare Hepatic diseases
Rare hereditary metabolic disorders	Inborn errors of metabolism
Rare multisystemic vascular diseases	Rare Circulatory system diseases
Rare neurological diseases	Rare neurologic diseases
Rare pulmonary diseases	Rare respiratory disease
Rare renal diseases	Rare renal diseases
Rare skin disorders	Rare skin diseases
Rare urogenital diseases	Rare urogenital disease

### Partly corresponding classification

Rare cancers	Rare Neoplastic disease (benin and malignant tumors)
Rare cardiac diseases	Rare surgical cardiac diseases and rare cardia disease
Rare craniofacial anomalies and ENT (ear, nose and throat) disorders	Rare maxillo-facial surgical diseases
	Rare othorinolaryngologic diseases
Rare immunological & auto inflammatory diseases	Rare Immune & systemic & rheumatologic disease
Rare malformations / developmental anomalies/and rare intellectual disabilities	Rare Developmental defect during emrbyogenesis

### No directly corresponding classification

Rare neuromuscular diseases	Rare neurologic diseases
Rare connective tissue and musculoskeletal diseases	Rare systemic and rheumatologic disease
	Rare Infectious diseases
	Rare surgical thoracic diseases
	Rare Odontologic diseases
	Rare infertility disease
	Rare Allergic disease
	Rare Intoxication

Is this the best alignment of members  
with the RD groupings?



# 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

# Principles for Collaboration

The functioning of the European Reference Network should always reflect the following principles for collaboration:

- **Integrity** – any information presented and exchanged between network participants must be factual and presented in a balanced way
- **Independence** – the independence of all network participants should never be compromised
- **Respect** – the aims, objectives, priorities, and ways of working of all network participants should be agreed upon and respected
- **Equity** – contributions from all network participants should be considered to be of equal importance
- **Transparency** – the aims, objectives, priorities, and work done should be made available to all network participants as well as all patients' organisations concerned;
- **Mutual benefit** - the collaboration aims at making the best possible use of the existing knowledge coming from the perspective of both professionals and patients, who are experts by experience.

# ERN Timeline

**2014:** EC ERN Delegated Acts entered into force

**2014-15:** Development of the Assessment Manual

**2015-16:** Selection of Independent Assessment Body(s)

**February 2016:** First Call for Networks

**Q2 2016:** Assessment proposals

**Q3 2016:** Establish Networks

# Key steps

## October:

- Align members with EUCERD Grouping
- Feedback from Council of European Federations

## November:

- EURORDIS membership briefing
- Engage all members on proposed alignment of patient organisations with EUCERD groupings

## December:

- Communication of groupings results
- Identify potential leading organisations
- Adopt Terms of Reference for EPAGs

## January:

- Focus development of EPAGs for mature grouping ERN applications
- Call for EoI of candidates to form PAGs

## February-March: Election of EPAGs

## March-May: Engage EPAGs in application development of ERN identified

## April:

- Communicate on our membership grouping and EPAGs formed
- Communicate on new EURORDIS matrix organisation in healthcare

## May: EMM to consolidate outcome groupings of membership



# Thank you



Daniel -Sanfilippo syndrome