



# TRANSFORMING CARE FOR RARE DISEASES

## Our vision for a mature ERN system

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An illustration depicting a medical professional in a red coat and a light blue surgical mask examining a patient lying in a hospital bed. The patient is wearing a teal hospital gown. In the background, there is a brown medical cabinet with various items on it. The scene is set in a hospital room with a white wall and a green wall on the left.

**Our vision is for a mature ERN system that leaves no person living with a rare disease in uncertainty regarding their diagnosis, care and treatment**

How should the ERN system look like in 10 years time?

1. Collaborative activities: Care, Research, Knowledge sharing, Training
2. Common cross-ERNs services
3. Scope and Structure
4. Funding and Governance
5. What needs to be in place at national level?

## CARE

- **Health outcomes** are used to facilitate continuous learning and improve quality of care
- Clinical governance principles + reimbursement model for online specialist advice and remote consultations
- **Referral pathways** published for all diseases under ERNs operational scope

## K.S

- Annual work plan to develop **CDSTs tools for all diseases** under ERN operational scope
- Systematic collection of outcome measures for all diseases under ERN
- Annual clinical summits to present outcomes and best practice
- **Data** is used to identify emerging best practice & new surgical concepts

## Research

- Research **compliant with regulatory & HTA standards**
- Patient-centered outcomes used on clinical research and patients' advice is used to identify unmet needs
- Development of testing capacities in research settings
- Minimum 2 **natural history studies/year**
- Regular collaboration with RD research infrastructures, EMA, EJP, IRDiRC and industry

## Training

- Annual training programme includes a Clinical and a Leadership track
- ERN clinicians do short-term placements to develop **supra-specialised expertise**
- Formal twinning of Affiliated Partners + full Members to develop their expertise
- Target trainings for medical students to identify RD **red flag signs earlier**

## 2. COMMON SERVICES

1. **Clinical decision support tools Competence Centre** to support on selected functions such as literature review and methodological training
2. **Data management Centre** IT services/architecture; data collection protocols; data curation services, data mgmt. tools, data analytics tools/services, governance
3. **Virtual and e-learning platform**
4. **Framework for patient engagement in the ERNs** to fully legitimise and recognise their role of patient representatives in the ERNs
5. **Common research support structure covering 4 domains:** clinical research, data management, engagement and dissemination and administrative support
6. **Suite of digital tools and services to support ERNs specific needs**

### 3. Scope and Structure

- **Operational disease expansion is completed** and the ERNs covers all diseases under its thematic groupings (comprehensive operational coverage).
- **ERNs have achieved full geographic coverage** through full members, affiliated partners & partnerships with HCPs not endorsed/designated nationally
- **New ERNs to cover gaps:** rare infectious diseases; rare and complex gynecological and obstetrical conditions
- **Clinical leads & patient representatives work in inter-ERN WGs** for multi-system rare diseases
- **ERNs integrate health professionals from other disciplines** on a regular basis
- ERN members work with their hospital managers and national health authorities to **connect with hospitals within their country, extending the ERNs locally.**

## 4. Funding and governance

- **Public funding is granted through a 'cost and volume' model.** There is also a **mechanism to receive funds from other sources** (industry, donations, etc.) and a governance mechanism in place for public-private partnerships.
- **Membership is a dynamic process based on a benchmark model** that incentivises HCPs to improve health outcomes and quality of care year-on-year.
- **Patients' role is fully recognised in all ERNs** and funding is allocated to enable meaningful patient engagement activities.
- **ERNs have formal agreements with global or regional clinical networks** and can show tangible achievements from this cooperation.

## 5. WHAT NEEDS TO BE IN PLACE AT NATIONAL LEVEL

- All MS have a process to identify and designate national centres of expertise.
- All MS have an annual work plan to strengthen their RD centres of expertise **competencies and capacities** to fulfil the vision of the EUCERD Recommendations on Quality of CoE.
- **There is a common guidance regarding endorsement** criteria and process to join the ERNs and all MS conform to it.
- **All MS have implemented their roadmap to integrate the ERNs into their NHS, overcoming the fragmentation between the EU and national levels**
  - National policy and/or legal framework updated
  - Clear RD pathways easy to navigate for clinicians and patients
  - Clear referral procedures to all ERNs
  - Communication strategy to disseminate information about ERNs
  - National networks of rare disease centres of expertise
  - Mechanisms to facilitate uptake of the ERNs knowledge assets

The background features a white central area. On the left side, there are two overlapping shapes: a blue shape in the upper-left corner and a green shape in the lower-left corner. Both shapes have rounded corners and extend towards the center of the page.

**Thank you**