



# How to build thematic and patient-centred rare disease networks

## Workshop 4

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## 4. How to build thematic and patient-centred rare disease networks

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# 4. How to build thematic and patient-centred rare disease networks

## Programme

- **Opening**
- **Developing thematic ERNs for rare diseases**
  - Developing thematic ERNs for rare diseases: potential and challenges - Kate Bushby and Victoria Hedley
  - Group discussion 1
  - Feed-back 1
- **Patient focus and involvement**
  - Patient involvement in thematic ERNs for rare diseases: potential and challenges - Yann Le Cam
  - Group discussion 2
  - Feed-back 2
- **Conclusion**



# 4. How to build thematic and patient-centred rare disease networks

## 1.1 Developing thematic ERNs for rare diseases

- Think of how you would formulate and develop a mission statement and strategic plan for your network.
  - How would you describe and demonstrate the added value for networks and centres specialised in specific rare disease of working together in broader thematic networks?
  - How could the specificity of individual rare diseases be maintained within the context of thematic networks?
  - How to select a coordinating member and network coordinator for the thematic network?
  - How would you agree on the scope of services/activities that thematic networks could develop to the benefit of all members specialised in various rare diseases covered?
  - How could collaboration between different thematic networks be promoted to facilitate cross-cutting linkages between rare diseases stratified in distinct groups?
  - What are the challenges of turning existing European pilot networks into an ERN?



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## 1.2 Patient focus and involvement

- Design a patient-centredness agenda of your network as part of its strategic plan
  - How should the network and its members demonstrate and document their patient-centred approach and commitment to patient empowerment?
  - How would the input and experience of patients, families and patients' association be used in the development of guidelines and pathways, quality and safety framework, outcome and performance indicators.
  - What role and responsibilities could patient organisations take in ERNs? How would you select them? How would they fit in the ERN governance model? How can they be involved in the application process?

# A model for the purposes of grouping RD thematically

|  |
|--|
| <b>Rare immunological and auto-inflammatory diseases</b>                                 |
| <b>Rare bone diseases</b>  |
| <b>Rare cancers* and tumours</b>   |
| <b>Rare cardiac diseases</b>   |
| <b>Rare connective tissue and musculoskeletal diseases</b>                               |
| <b>Rare malformations and developmental anomalies and rare intellectual disabilities</b> |
| <b>Rare endocrine diseases</b>   |
| <b>Rare eye diseases</b>   |
| <b>Rare gastrointestinal diseases</b>  |
| <b>Rare gynaecological and obstetric diseases</b>  |
| <b>Rare haematological diseases</b>  |

**Rare craniofacial anomalies and ENT disorders**

**Rare hepatic diseases**

**Rare hereditary metabolic disorders**

**Rare multi-systemic vascular diseases**

**Rare neurological diseases**

**Rare neuromuscular diseases**

**Rare pulmonary diseases**

**Rare renal diseases**

**Rare skin disorders**

**Rare urogenital diseases**

\*Note: The networking of rare cancers is currently under discussion in EC Expert Group on Cancer Control.