WAYS TO A Rare NMD ERN
Networks criteria and capacities (From the Delegating and Implementing acts):

- Knowledge and expertise to diagnose, follow up and manage patients
- Evidence of good outcomes
- Multi-disciplinary approach
- Capacity to produce and implement: good practice guidelines, outcome measures and quality control
- Research, teaching and training
- Collaborate with other centres of expertise and networks

How to prove this?
Identify:

- The expertise (Paediatrics and Adults + multidisciplinary approach)
- The coordination of the network (who will deal with the planning of the network)
- How to make the ERN visible to the wider public (interaction with patients, other healthcare providers and regulatory agencies)

10 Members in at Least 8 Countries

Most healthcare providers are involved in the care of all groups of NMD, adding to that a super-specialized area of expertise / research
Aims of the workshop:

1. exchange of knowledge and expertise in processes for the delivery of NMD care
2. assessment of existing resources both at national and international level
3. identification of gaps which need to be addressed
4. decide on a guideline document for the implementation of a ERN in the NMD field
Current status of specialized neuromuscular centres in Europe

• **Experience in the neuromuscular field on networking activities and Biobanks:**
  - European Neuromuscular Centre (ENMC)
  - TREAT-NMD Alliance
  - Telethon Network of Genetic Biobanks (TNGB) and/or the EuroBioBank (EBB)
  - RD-Connect

• **The role of the learned societies in an ERN:**
  - Current resources, such as e-learning, teaching courses and guidelines should be integrated into a future ERN.
  - Contribute to the establishment of a European NM curriculum and to the structure of the European Board Examination.

• **e-health:**
  - E-learning programmes are in place through the scientific societies, can be adjusted to different needs.
  - Other resources are being assembled through projects like the cross border EU project SIGN (telegenetics system to perform genetic counselling and clinical genetics consultations)
Total of 360 members

100 organizations – 40 countries

260 individuals – 42 countries

Members in every continent apart from Africa 😞
Areas of interest – How to map different participants?

**Mapping**

**Coordination**
- Muscle diseases
- ALS/MND
- NMJ defects
- Peripheral Neuropathies

**Action Points**
- Contact 1 or more experts in the different areas, cascade the information through those experts
- Establish the connection between the different centres
- Involve patient organizations
- Establish the aims, structure, governance, services to be offered, integration of existing networks (most of them research based networks)

**Countries Engaged**
- UK
- Italy
- Netherlands
- Germany
- Cyprus
- Spain
- France
- Belgium
- Hungary
- Sweden
Common purpose

• Improve quality and equity of healthcare for patients with NMDs
  - Equity in diagnostic
  - Uniform care standards

• Enable exchange of knowledge (teaching and training)

• Help with translational research: the development of new drugs and the recruitment into clinical trials – link to research
Structure

Hub-and-Spoke Structure

This is the model proposed by the EU.

Multi-Tiered Hub Structure

Country level
ISSUES:

✓ Where are **patients representatives** going to be represented? At a country level? At an European Level?

✓ How many HC providers are there going to be in the ERN? Depending on this number; the Board of the ERN could become non governable.

✓ How is the Coordinator going to be nominated?
Main functions of the ERN

• Promote and sustain good practice
• Organise and manage all relevant information/data
• Help to diffuse valid information to patients, other healthcare providers and the public
• Teleconsultation/Tele expertise
• Training and teaching
Still under discussion at the EC level, it is likely that the themes will include:

- healthcare in a network environment,
- clinical guidelines development,
- training
- provision of a better environment for clinical research including clinical trials
What Services should we offer?

- **Clinical**
  
  Direct: teleconsultation, ?traditional clinical appt?

  Support to healthcare providers: e-Health
  (Exchange, gather and disseminate knowledge)

- **Non Clinical**
  
  Clinical guidelines / patient pathways
  (Implement outcome and performance indicators)

  Epidemiological surveillance, registries

  Training and continuous education programmes

  Dissemination of information

- **Trials**
  
  Selection of patients (registries)

  Training of professionals in assessment protocols
Possible working Groups that could feed into the Board of the ERN

- Care guidelines
- Imaging
- Genetics
- Social services
- Pathology
- IT
- Registries
- Therapeutics
- Teaching

...
Board of the ERN should be supported by:

- Input about the teaching priorities
- Evaluation of the initiatives
- Input on common research priorities
- Screening of research projects
- Grant applications for research
- Among others
  - Grant Search for sustainability
  - Contacts with Industry

External evaluation body

Ethics Committee

Secretariat

Educational Board

Research Board

Input about the teaching priorities
Input on common research priorities
Care and Trial Site Registry – CTSR
A Powerful Tool for Clinical Research and Networking in Rare Diseases

Jan Kirschner
Dept. of Neuropaediatrics and Muscle Disorders
Universitätsklinikum Freiburg, Germany
Background

• Established in 2007 in the scope of the TREAT-NMD project.

• In September 2013 the CTSR expanded to cover the field of rare neurodegenerative diseases as a branch of NeurOmics (FP7, 2012-2017) and now encompasses 32 rare diseases subdivided into two groups.
Number of sites since 2008
Patient numbers since 2008
Potential role for ERN

• Motivate all centres interested to participate in ERN to register or to update information in the CTSR

• Use the content of the database for the application, e.g. infrastructure of existing centres and networks, identify gaps for patient care in different European countries
The ERN needs to take into consideration:

- Cross-country payments
- IT platform maintenance
- Technical support
- Administrative work
- Network meetings
- Dissemination costs
- Care coordination
ERN IMPLEMENTATION: the way forward

Cross-sectorial cooperation and funding sources

✓ Public health program 2014-2020: studies & project grants to approved ERN
✓ RTD horizon 2020: 2016 research on networks organizational models
✓ Connecting European Facilities (CEF): the eHealth dimension
✓ Structural funds (cross border cooperation)
✓ Social funds (training and better skills)
Preparatory and strategic activities
From Enrique Terol presentation

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

- Liaison with MS authorities

- Define the services of the Network
- Agree on the specific criteria for each area of expertise
- Self-assessment exercise (Network and members): decision of participation as members or as Associated National Centres
- Define Pathways models, referral criteria, clinical decision tools
- Information system/indicators
ERN tentative timeline & milestones

- **May 2014**: Entry into force of legal acts
- **July 2014**: Call for Assessment Manual
- **II quarter 2015**: Call for selection of independent body(ies)
- **IV quarter 2015**: Call for Networks
- **II quarter 2016**: Establishment of Networks