An overview of EURO-NMD
European Reference Network for Rare Neuromuscular Diseases

Teresinha Evangelista
The process behind ERN-NMD

- **RDTF (2004-2009)** - WG on Networks of Reference
  
  **(2005-2006)** Options for a procedure for identification and development of European reference networks

- **March 2011:** European Directive on the application of patient’s rights in cross-border healthcare

- **EUCERD (2010 – 2013)**
  
  **2013** – EUCERD Recommendations on RD ERNs

- **2014** – Implementing and Delegated decisions
The process behind ERN-NMD

- **2015** – EUCERD Joint Action (now RD-ACTION) led drive to find a ‘model’ to group diseases

- **March 2016** - Call for ERNs

- **24th January 2017**
The process behind ERN-NMD

The work for EURO-NMD has started in 2013

Ongoing
What is an ERN?

• A network of health care providers/centres providing highly specialised healthcare, with the purpose of improving access to diagnosis, treatment and care for patients across Europe

24 ERNs
900 HCP
300 Hospitals
26 Countries
Building bridges and breaking barriers in rare neuromuscular diseases
Building bridges and breaking barriers in rare neuromuscular diseases
AOU policlinico "G. Martino" Messina - Prof Antonio Toscano
AOU- Second University of Naples (SUN) - Prof Luisa Politano
ASST degli Spedali Civili di Brescia - Dr Massimiliano Filosto
Azienda Ospedaliera di Padova (AOP) - Prof Elena Pegoraro
Azienda Ospedaliera Universitaria Pisana - Prof Gabriele Siciliano
Azienda Ospedaliera-Universitaria Ferrara - Prof Alessandra Ferlini
Azienda Ospedaliero Universitaria di Torino - Prof Tiziana Mongini
Fondazione IRCCS CA'Granda Ospedale Maggiore Policlinico di Milano - Prof Maurizio Moggio
Fondazione Policlinico Universitario A Gemelli Rome - Prof Eugenio Mercuri
Istituto Giannina Gaslini - Prof Carlo Minetti
Istituto Nazionale Neurologico Carlo Besta - Prof Davide Pareyson
Nemo clinical Center (Neuromuscular Omnicomprehensive) - Dr Valeria Sansone
Ospedale Pediatrico Bambino Gesù Rome - Prof Enrico Bertini
Rare Neuromuscular Diseases (NMDs) (ORPHA68381)

- Muscle Diseases
  - Coordination: Marianne de Visser
  - Acquired
    - Inherited

- Peripheral Nerve diseases
  - Coordination: Mary Reilly
  - Acquired
    - Inherited

- Motor Neuron Diseases
  - Coordination: Leonard van den Berg
  - ALS and related disorders
  - SMA and related disorders

- Neuromuscular Junction Defects
  - Coordination: Hanns Lochmüller
  - Myasthenia Gravis & Other autoimmune NMJ defects
  - Congenital Myasthenic Syndromes

- Mitochondrial diseases
  - Coordination: Rita Horvath
  - Mitochondrial encephalomyopathies caused by mtDNA mutations
  - Nuclear gene defects causing Mitochondrial encephalomyopathies, myopathy or neuropathy

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Coordination Grant Application

- **WP1**: Coordination, Management and Evaluation
- **WP2**: Development, Implementation and Quality Assurance of Guidelines and Outcomes Measures
- **WP3**: Knowledge Base and E-Health Tools
- **WP4**: Training and Continuous Education Programmes
- **WP5**: Promoting clinical research and facilitating access for research participation
- **WP6**: Dissemination, Outreach and Communication
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Board of the ERN

• **Chairperson** (Coordinator of the ERN)

• **Composition:**
  1 representative from each HCP
  PAB members
Executive Committee

ACTIONS

• Provides policy and strategic direction
• Delegates responsibility for day-to-day operations to the Activity Groups

COMPOSITION (8-15 members)

PAB; Ethics Board; Research and Education Boards
CTSR and the Secretariat; Specialist Groups; Diagnostic Tool Groups.
Activity Groups

Composition

1 chairperson; (Co-Chairs)
Members from the HCPs
Patient representatives nominated by the PAB

Objectives

• Provide expert consultations,
• Produce and review guidelines for diagnostic methods and care,
• Participate in actions for training, research, among others.
Participants to the activity groups

All centres, members of the network, were asked for:

- Their experts in the different areas, interested to participate and actively support the objectives of the network
EURO-NMD SERVICES

• Develop and implement standards of care, care guidelines/pathways
• Create a European NMD education network
• E-Health tools and remote access healthcare
• Catalyse research
• Regulator, policymaker and payer interactions
• Interactions with commercial third parties
“Patient clinical pathway“ in the network

• **Clinical pathway**: from the moment a patient is referred to the ERN by the treating physician until a discharge report is issued by the ERN
Diagram 1 – Care Pathways

EURO-NMD Health Care Provider requests e-consultation after discussion with patient.

Steering group reviews referral and decides on diagnostic pathway. Referral or response Within 1 week.

Health Outcomes
Test result. Diagnosis. Care. Treatment

NMD patient specific response within 8 weeks

Additional information is requested from referral HCP

e-consultation by specialist board

Diagnostic Tools Board

Face to Face consultation

HCP directed to online resources and guidelines EURO-NMD website

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Clinical Governance Group

**Coordination:** Patient and HCP Liaison (Clinical Lead)

**Constitution:** One or more elements appointed by the different Activity Groups.

**RESPONSABILITIES**

- Screening of referrals to the network
- Implementing the clinical pathways
- Assuring that the times for replies are kept
E-Health

- Virtual multidisciplinary meetings

- The leads of the Specialist Groups will be responsible for the organisation of multidisciplinary teams needed for each particular case

- The multidisciplinary meetings/consultations will be registered with a record of cases discussed, professionals involved, and decisions made.

A written report, addressed to the referring clinician and to the CGG, will be issued at the meeting.
Overview of the functionalities of the platform

- **Restricted access**: Access restricted to group members, Access managed by the coordinator of the network
- **Homepage with latest news**: Latest activities of the group are published on the homepage, New/updated events, publications, discussions, documents...
- **Meeting management**: Agenda, plan meetings, attach documents, a poll...to the meeting invitation, Conduct virtual meetings (at a later stage)
- **Interactive collaboration**: Start a discussion (forum), comment, endorse...
- **Document library**: Share a document with the group, upload document, update and consult previous versions
- **User guide**: Interactive user guide embedded in the system (and PDF)
Website

Information from 31 healthcare providers ~50%

Thanks to those who have submitted their information 😊

We will send a reminder email to everyone soon please ensure you have sent us your basic information to avoid having a blank entry about your healthcare provider on the website.

Contact us through

info@ern-euro-nmd.eu
European Reference Network
for rare or low prevalence complex diseases

Network
Neuromuscular Diseases (ERN EURO-NMD)

Coordinator
The Newcastle upon Tyne Hospitals NHS Foundation Trust — United Kingdom

29- 30 November

We are delighted to announce that we are organising our 5th international conference focussing on translational medicine in inherited neuromuscular diseases.

The aim of this international conference is to share progress and lessons learned in the area of translational medicine in inherited neuromuscular diseases and plan for the delivery of future therapies to patients.

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**ERN Meeting details**

**Wednesday 29th 5:30-7pm Drinks reception for all ERN network**

**Thursday 30th November ERN Meeting:**

<table>
<thead>
<tr>
<th>Time</th>
<th>Details</th>
<th>No of participants</th>
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<tbody>
<tr>
<td>8:00-9:00</td>
<td>Poster set up</td>
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<tr>
<td>9:00-11:00</td>
<td>Session 1 of ERN conference</td>
<td>Max 120</td>
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<tr>
<td>11:00-11:15</td>
<td>Coffee</td>
<td>Max 120</td>
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<tr>
<td>11:15-13:15</td>
<td>Session 2 of ERN Conference</td>
<td>Max 120</td>
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<tr>
<td>13:15-14:15</td>
<td>Networking lunch</td>
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<td>2:15-4:15</td>
<td>ERN Board Meeting</td>
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<tr>
<td>16:15-16:30</td>
<td>Coffee</td>
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<tr>
<td>16:30-18:00</td>
<td>Executive committee meeting</td>
<td>16</td>
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Thanks goes to the following people...

Kate Bushby

Teresinha Evangelista

Becca Leary

Michael Hails