



2nd CONFERENCE ON



European
Reference
Networks

Etat de la question

8-9 OCTOBER
2015, LISBON



GOVERNO DE
PORTUGAL

MINISTÈRE DA SAÚDE

Presidency of
the Council of the
European Union

GRAND DUCHÉ DE
LUXEMBOURG

Health



Filnemus

Filière Neuromusculaire

2^{ÈME} JOURNÉE DE FILNEMUS
6 NOVEMBRE 2015

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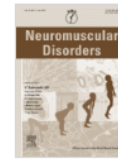
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Éléments de contexte

- dernier étage de la fusée européenne Maladies Rares
- projet ancien, cadre légal datant de 2014
- projet porté par la Commission Européenne (UE)
- et s'inscrivant dans les directives soins transfrontaliers
- importants travaux préparatoires (EUCERD)
- objectif **d'accès à des soins d'excellence**
- dans un contexte **d'équité**
- mais marche en ordre dispersé
 - entre structures très abouties (PNMR, CRMR, etc.)
 - et des pays où il n'y a rien ou presque
- pour les MNM, on ne part pas de rien



Workshop report

200th ENMC International Workshop “European Reference Networks: Recommendations and Criteria in the Neuromuscular field”, 18–20 October 2013, Naalden, the Netherlands

Teresinha Evangelista^a, , Baziél van Engelen^b, Kate Bushby^a

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

EUROPEAN
NEURO
MUSCULAR
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Agenda 2015

- webinar ERN (organisé par Newcastle)
- Colloque de Lisbonne (7-8 octobre 2015)
 - grand-messe organisée par Commission Européenne
 - toutes pathologies rares confondues
 - tous types d'acteurs représentés
- travaux de la commission 'Réseaux' FILNEMUS

Agenda 2016

- workshop fin janvier 2016 à Newcastle
 - afin de préparer une application commune
 - à soumettre à l'appel d'offres ERN T1 ou T2 / 2016



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Les certitudes

- un appel d'offres ERN aura bien lieu (S1-2016)
- regroupement effectif des pathologies rares (21).
- MNM et SLA réunies dans le même groupe
- le mode d'évaluation a été précisé (double processus)
- Unité élémentaire constitutive : Health Care Provider
- chaque HCP devra candidater à titre individuel
- filtre / évaluation préalable
 - par les autorités nationales (chez nous, DGOS)
 - assistée (?) par les Filières (quand elles existent)
- en parallèle, l'ERN devra candidater en tant que tel
- Newcastle se positionne comme leader naturel et légitime
- les ERN auront une existence juridique (legal entity)

How can you get involved in building a Network?

As healthcare professionals and providers, experts, patients and authorities, you should reflect on which diseases or conditions in your area of expertise would match the criteria of rarity or low prevalence and complexity, as set out in the legislation governing ERNs. And focus on the grouping of diseases and the types of services to be offered.

Then you can use your experience to examine the technical and organisational feasibility of the Network. You can bring in existing networks, specialised centres, or scientific associations to do this. The ERNs are based on a co-operative, multidisciplinary approach, so you should also look to develop the right forum to move the project forward.

Who can join a Network?

All the members in a Network should have in common the same area of expertise and focus on the same health condition(s).

All Networks should demonstrate that they are strong in:

- patient empowerment and patient-centred care;
- organisational, management and business continuity of the centre of expertise;
- research and training capacity;
- exchange of expertise, information systems and e-health tools; and
- good practice, quality, patient safety and evaluation.

For more information on the application process and requirements, please visit ec.europa.eu/join-ern



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Share. Care. Cure

What are European Reference Networks?

Imagine if the **best specialists** from across Europe could join their efforts to tackle **complex or rare medical conditions** that require **highly specialised healthcare** and a concentration of knowledge and resources.

That's the purpose of **the European Reference Networks** and it's becoming a reality.

Share and enhance your expertise. Talk with your colleagues. **Be ready to prepare a proposal and join a network.** The European Commission will launch a **call for Networks in 2016.**

European Reference Networks (ERNs), which are expected to be operational by 2016, will bring together highly specialised healthcare providers from different EU Member States in areas where expertise is rare.

By pooling knowledge and expertise across the EU, they will facilitate access to diagnosis, treatment and provision of affordable, high-quality and cost-effective healthcare in these areas. ERNs will also serve as focal points for medical training and research, information dissemination and healthcare evaluation.

The intention is not to create new providers, but rather to link existing highly specialised providers.

CHALLENGES

- To engage, attract, identify the right Networks and healthcare providers
- To establish a Network model with useful platforms and tools
- To foster commitment by Member States and ensure sustainability of established Networks
- To avoid fragmentation and/or duplication of efforts
- To develop and use standardised tools (clinical guidelines, registries, patient pathways, interoperable IT systems)
- To involve healthcare professionals, health authorities, managers and patients in a common project for the implementation of ERNs

Why are European Reference Networks needed?

It is often difficult for the 28 health systems of the EU to provide patients with rare or low prevalence complex diseases or conditions with the treatment they need. Therefore the European institutions and the Member States, have decided to implement ERNs to help address this difficulty.

The aim is to benefit patients by improving high-quality specialised care, through better co-operation and greater economies of scale.

BENEFITS

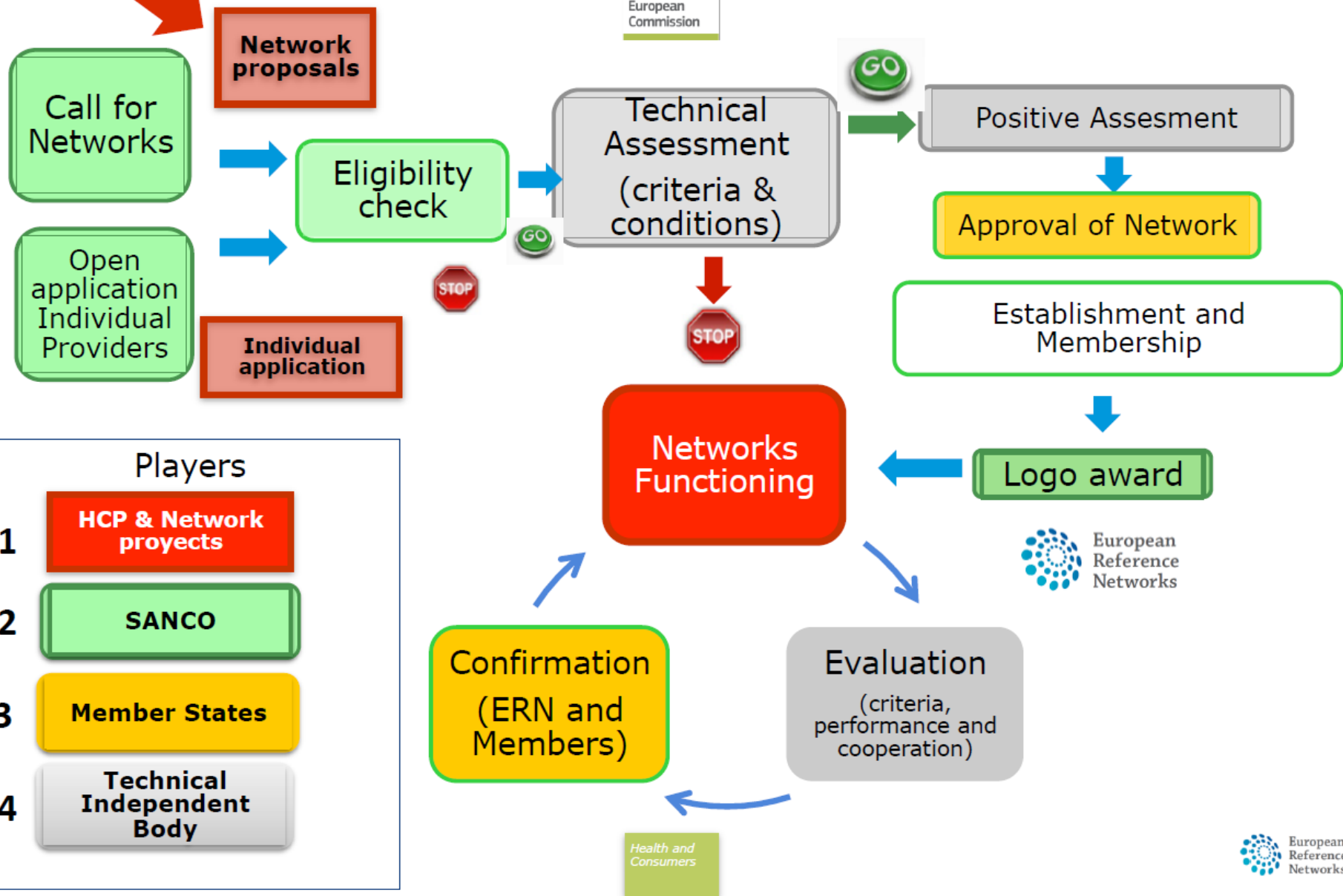
- Improve high-quality specialised care, for patients with rare or low prevalence complex diseases or conditions
- Improve European cooperation and economy of scale on highly specialised healthcare
- Pool knowledge on certain diseases or treatments
- Improve diagnosis and treatment capacities where expertise is rare by developing and sharing clinical guidelines and patient pathways
- Help Member States enhance the provision of highly specialised care
- Maximise the speed and scale of incorporating innovation in medical science and health technologies
- Serve as focal points for medical training and research

There are also obvious benefits to health systems, in improving and optimising their ability to provide such highly specialised care.

There will be other benefits as well. The pooling of knowledge and expertise will allow for the development of more evidence-based clinical tools and treatment. Networks should help maximise the speed and scale at which innovation in medical science and health technologies is incorporated into healthcare provision. They will serve as focal points for medical training and research and for the dissemination of information to other parts of the health system. And they will offer capacity for translational research, clinical trials, Health Technology Assessments, and more.

The providers involved in the Networks will obviously benefit from this pooling of knowledge with other specialists and will be recognised as leaders in their area of expertise.

ERN Scenario



A model for the purposes of grouping RD thematically

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

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.



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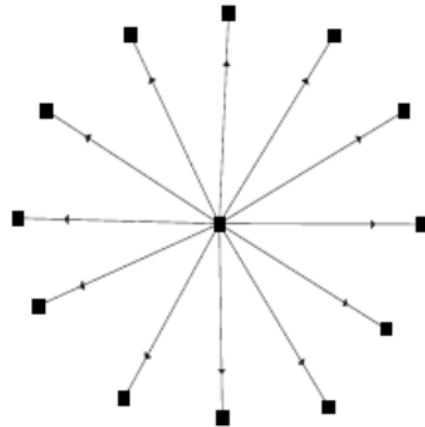
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Les incertitudes

- modalités de la gouvernance / désignation du coordinateur
- taille minimale connue (8 pays – 10 centres) quid de taille optimale réseau ?
- critères objectifs pour être HCP éligible
 - où mettra-t-on le curseur ? qui le mettra ?
 - réseau avec 1 ou 2 centres experts de taille critique et multivalent vs. modèle FSMR
- financement des actions
 - uniquement pour de la coordination ?
- place des Filières (ici, Filnemus)
 - acteur collatéral ou à part entière ?
- calendrier de mise en place
- services et valeur ajoutée attendus
 - orientation soins +++
 - mais lien aussi avec recherche translationnelle

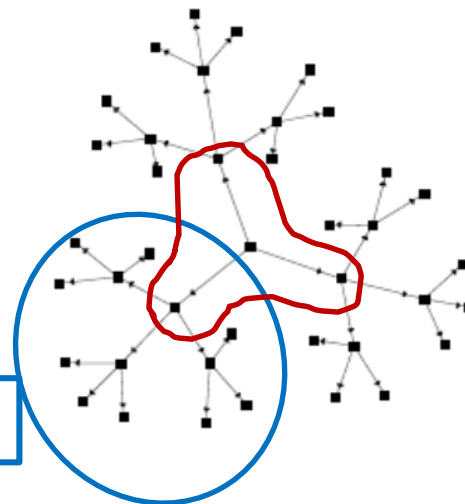
Structure

Hub-and-Spoke Structure



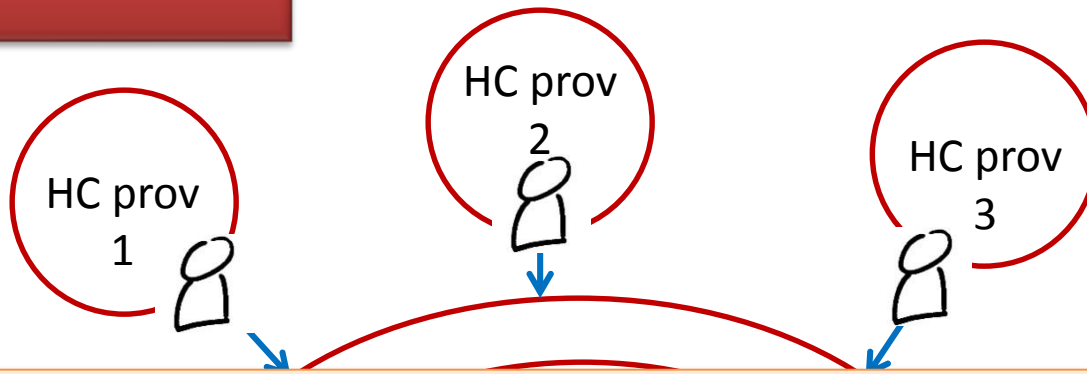
This is the model
proposed by the EU.

Multi-Tiered Hub Structure



Country level

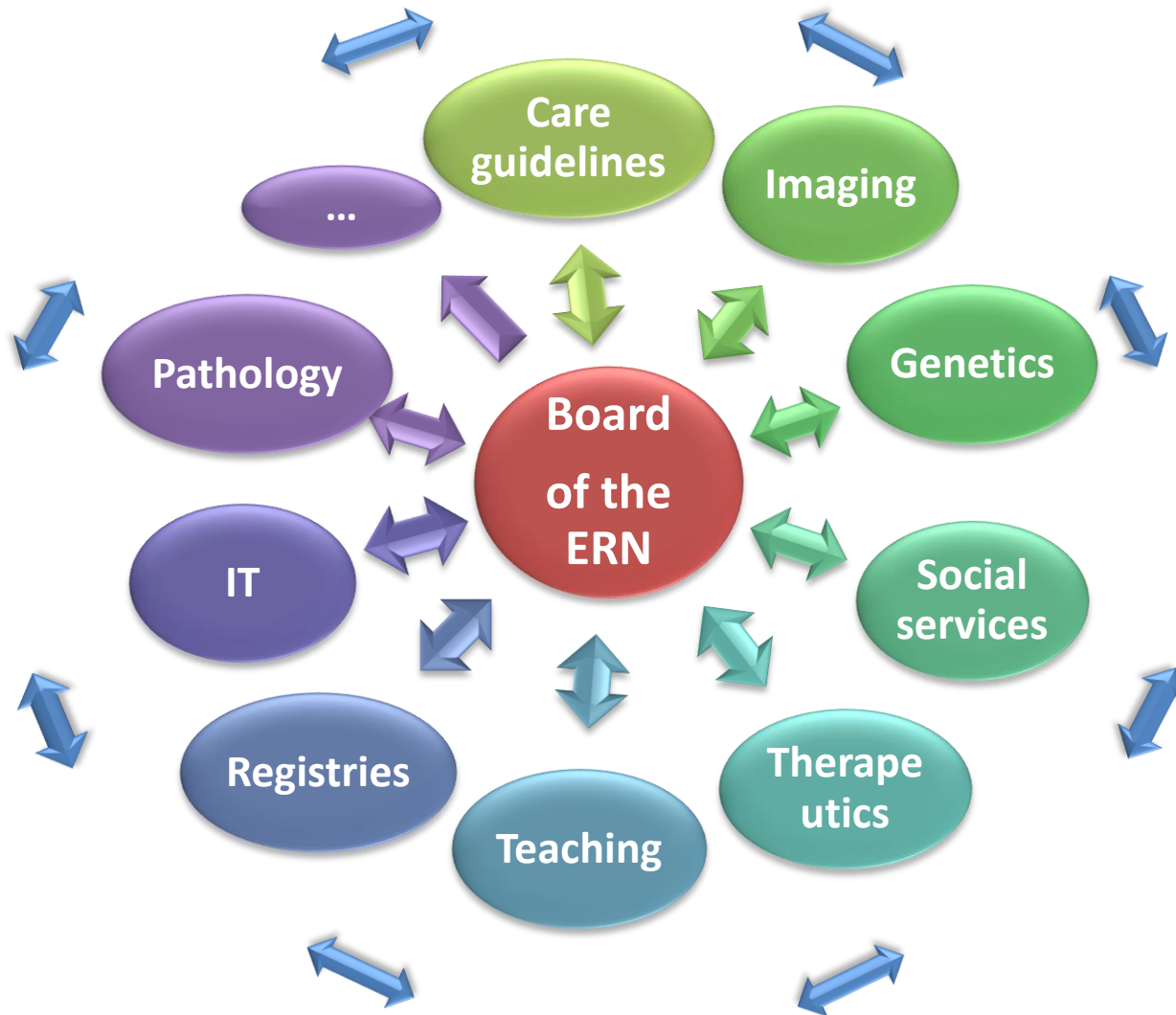
Governance



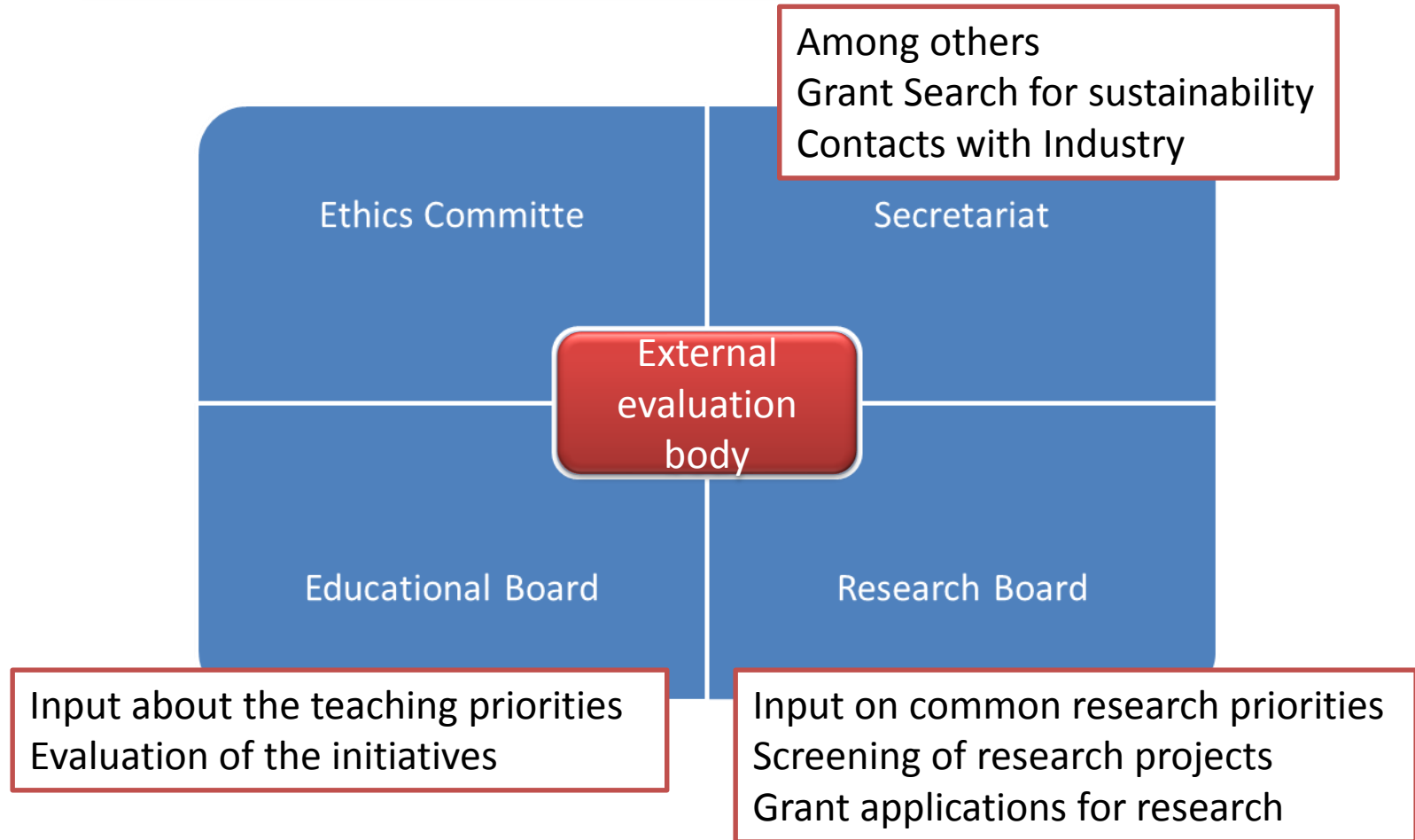
ISSUES:

- ✓ 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?

Possible working Groups that could feed into the Board of the ERN



Board of the ERN should be supported by:



ERN tentative timeline & milestones



May
2014



**Entry into force
legal acts**

July
2014



**Call for
Assessment
Manual**

II quarter
2015



**Call for selection
independent
body(ies)**

IV quarter
2015



**Call for
Networks**

II quarter
2016



**Establishment
of Networks**






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Points d'attention

- leadership de Newcastle
 - légitimité indiscutable
 - workshop fondateur ENMC de 2013
 - le bilan du TREAT-NMD
 - l'implication dans le comité EUCERD
- Nécessité de peser dans la future gouvernance de l'ERN
 - soit au travers d'un représentant national (à désigner par FILNEMUS)
 - soit en prenant le lead sur des thématiques / WP
 - formation – enseignement
 - accompagnement
 - essais thérapeutiques
 - imagerie...
- que restera-t-il du Treat-NMD ?
- Quid si brexit ?
- la place des 'petits' Etats-Membres



Care

Outcome measures



Training

Training and education



Research

Clinical Trial Coordination Centre



Trials/ Research Registries

Global patient registries



Trials/ Research

Communication infrastructure



Regulatory information




Trials/ Research

TACT

Advisory committee for therapeutics



Care and trials

CTSR

Care and trial site registry



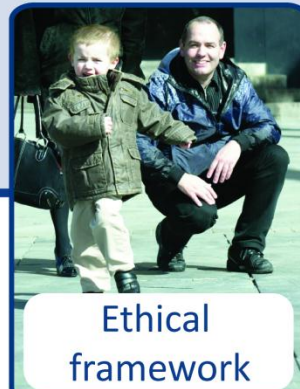
Research

Standards for animal assessment



Care

Care standards



Care

Ethical framework



Research

EuroBioBank



www.treat-nmd.eu

2007-2011

EU funded Network

2012 onwards

Alliance funded through multiple streams with global partners & membership

Governance

Chair – Annemieke Aartsma-Rus

Vice Chair – Eric Hoffman

Executive Committee

Supported by academic advisory board (“task force”) of NMD leaders

Total of 360 members

100 organizations – 40 countries

260 individuals – 42 countries

Members in every continent apart from Africa ☹️



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A FAIRE

- poursuivre la réflexion de manière collégiale
- y compris avec nos propres administrations hospitalières
- l'enrichir de celles des autres FSMR
- la partager avec les collègues européens
 - NL, D, Dk, Espagne, et autres
- identifier les expertises françaises
 - dans les réseaux par maladies
 - Steinert, Laminopathies, Pompe...et bien d'autres
 - par thématique (plus facile, cf. commissions FILNEMUS)
- but : arriver en position de force au workshop de Newcastle
- être force de propositions (alternatives)

Rare Neuromuscular Diseases ERN

Areas of interest – How to map different participants?

Mapping

Coordination

Muscle diseases

Genetic

Inflam
matory

Metab
olic

Mitoch
ondrial

ALS/MND

NMJ defects

CMS

Autoimmune

Peripheral Neuropathies

Genetic

Inflam
matory

COUNTRIES ENGAGED

UK

Italy

Netherlands

Germany

Cyprus

Spain

France

Belgium

Hungary

Sweden

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)

annexes

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?

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)

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**

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

Rare Neuromuscular Diseases ERN

Services To Be Offered

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

INCOME AND NON-MONETARY RESOURCES

- 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**