# 7<sup>th</sup> ERN EURO-NMD ANNUAL MEETING

#### PAB updates

21<sup>st</sup> – 23<sup>rd</sup> February 2024

François Lamy PAB chair



for rare or low prevalence complex diseases

Network Neuromuscular Diseases (ERN EURO-NMD)



The ERN EURO-NMD is funded by the European Commission under the EU4Health programme (EURO-NMD 23-27 — 101156434 — EU4H-2023-ERN2-IBA)



# Agenda

1. Achievements

### 2. Ambition for 2024

3. Patients Journey

The ERN EURO-NMD is funded by the European Commission under the EU4Health programme (EURO-NMD 23-27 — 101156434 — EU4H-2023-ERN2-IBA)



Funded by the European Union

### Achievements

- EURO-NMD ERN first cycle evaluation
- Registry Hub (ad-hoc working group)
- ENMC workshop on bone health (muscle group)
- PROMs on pain and fatigue (Work Package)
- Patients Journey (first wave)



Funded by the European Union

## Ambition for 2024

Pursue ongoing activities

- Registry Hub project
- PROMs on pain and fatigue

- Build more Patients Journey (second wave)
- Communicate outside the network (POs)

...



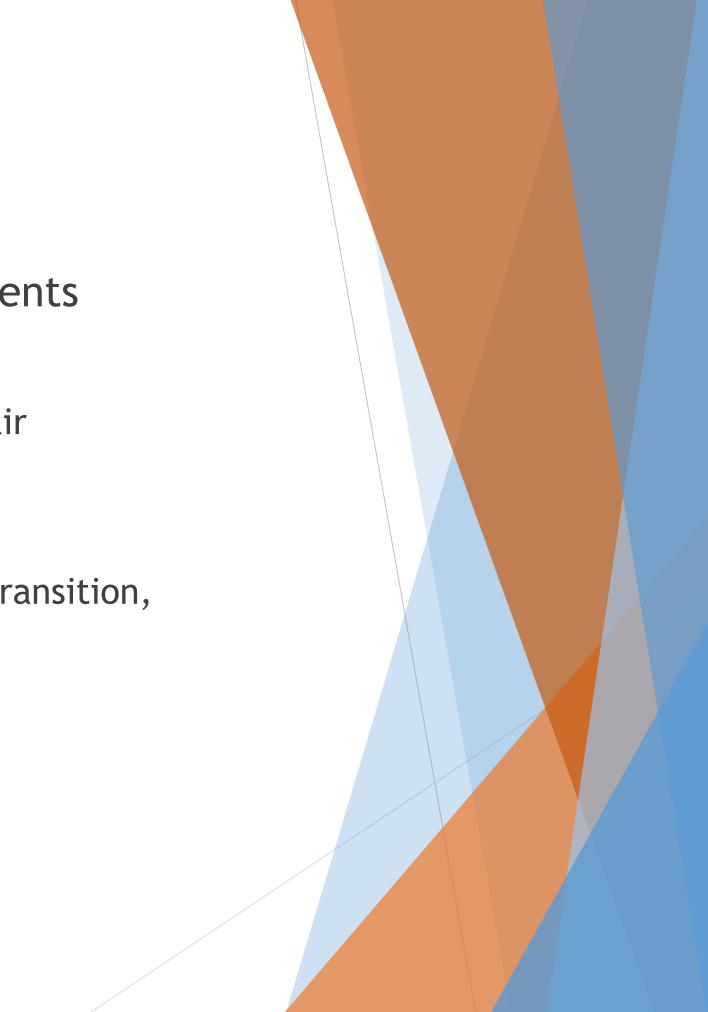
Funded by the European Union

## Ambition for 2024

Build educational material (webinars) for patients

- Registries
- Disability technical support (robotic arm, wheelchair positionning, wheelchair standing)
- Innovative drugs access
- Care (emergency, care quality, child to adulthood transition, virtual consultation)
- Very rare diseases specific information
- European Regulatory affairs

#### To be prioritized /ammended





### Patients Journey - what is a patient journey?

A patient journey is the chain of events people experience when they face a health issue.

Patients with rare and complex conditions often embark on a life changing odyssey including many aspects medical, social, psychological ... that are incompletely known.

The aim of the Patients' Journey Program is to **improve the knowledge** of neuromuscular diseases by developing a **description of the diseases through patients' lenses.** 

With this material our ambition is to lay the foundation of improved and standardized care for neuromuscular diseases in Europe.



### Patients Journey - method

#### Conception

- Perform a mapping exercise of the needs of the disease they represent across the different phases of the disease
- Perform a literature review of existing guidelines for the disease(s) of choice

#### Construction

- Divide the journey in different stages
- Build a graphic representation of the patients' journey
- Revision of the final Journey by patients and experts

#### Diffusion through Euro-NMD and patient organizations

• Newsletter, webinar, conference, poster, ...



### Patients Journey - organization

- Disease specific working group
  - 1 group leader
  - 3 to 4 patient representatives
  - 1 clinical contact point
  - 3 to 4 clinicians/experts
- Independent working groups
- Monthly workshops
- Coordination insured by a "support team" (Tamara / Elizabeth)
- Regular reports to the PAB
  - Synchronize with other groups work
  - Get external comments / advice



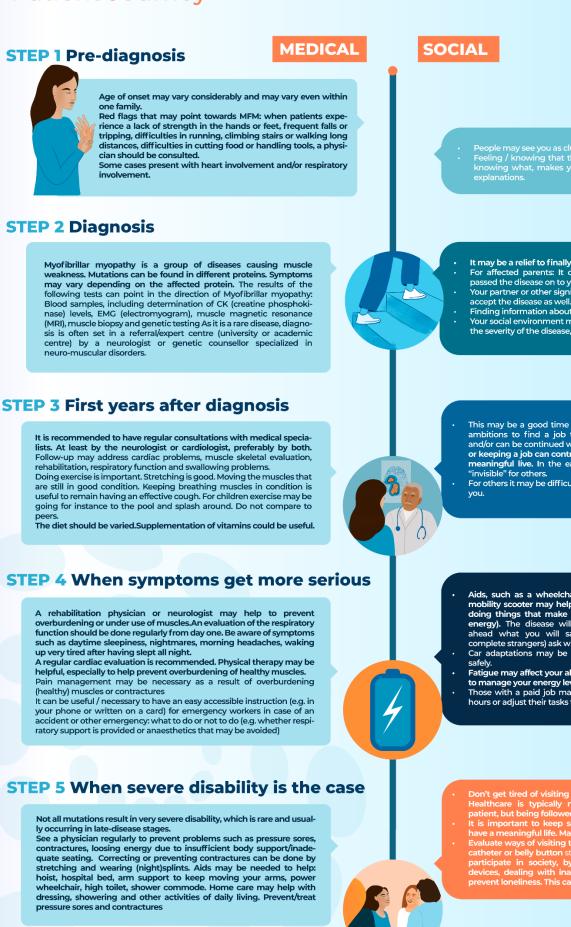


Funded by the European Union

#### **Patients Journey** achievements

- Multifocal Motor Neuropathy
- Myathenia Gravis
- Myofibrillar Myopathy

#### Myofibrillar myopathy **Patient Journey**





Board

European **Reference** Network for rare or low prevale complex diseases

Network Neuromuscular Diseases (ERN EURO-NMD)

ple may see you as clumsy or lazy. ling / knowing that there is something wrong, not wing what, makes you feel uncertain, looking for

It may be a relief to finally get the correct diagnosis. For affected parents: It can be difficult to see that you passed the disease on to your child

Your partner or other significant persons may need time to

Finding information about this rare disease is often difficult. Your social environment might have difficulties to estimate the severity of the disease, because it is unknown.

This may be a good time to reconsider your professional ambitions to find a job that is possibly less strenuous and/or can be continued when the body weakens. Having or keeping a job can contribute to the feeling of having a meaningful live. In the early stage, the disease often is

For others it may be difficult to understand what it does to

Aids, such as a wheelchair, crutches, walking stick or mobility scooter may help to manage fatigue and keep doing things that make you happy (and thus provide energy). The disease will become more visible. Think ahead what you will say when people (sometimes complete strangers) ask what is wrong with you. Car adaptations may be necessary to continue driving

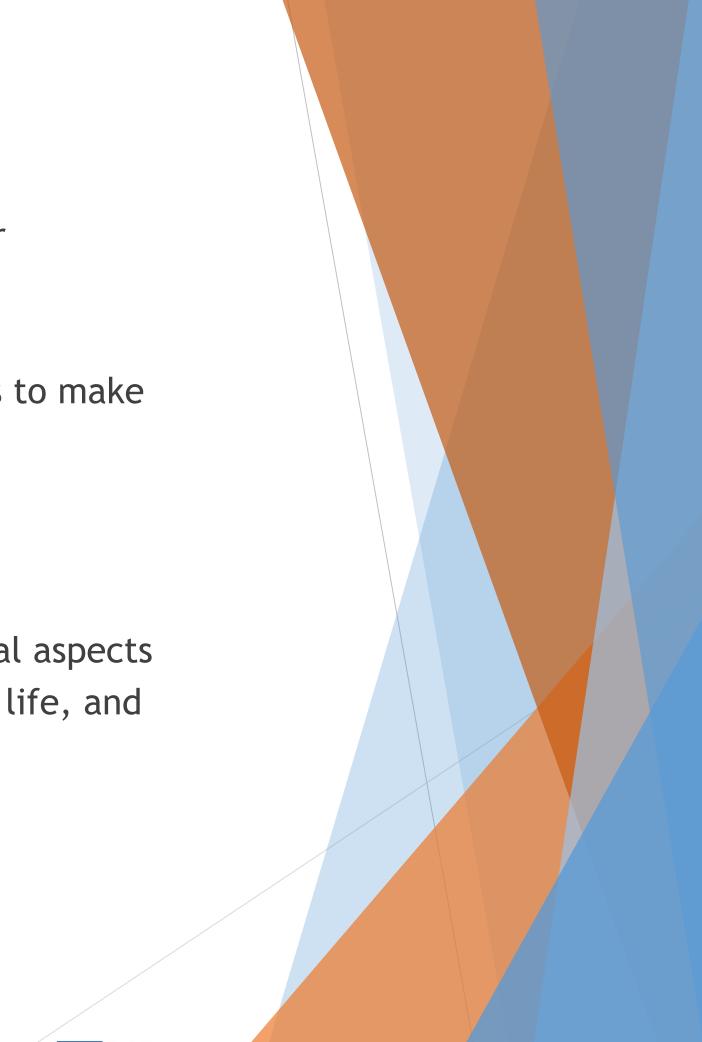
Fatigue may affect your ability to keep positive. Learning to manage your energy levels is important. Those with a paid job may need to adapt their working hours or adjust their tasks to less strenuous activities

Don't get tired of visiting all different medical specialists. Healthcare is typically not yet organized around the patient, but being followed up medically is important. It is important to keep socially active and to somehow have a meaningful life. Make good use of your better days. Evaluate ways of visiting the toilet, such as a (suprapubic) catheter or belly button stoma. Make sure you manage to participate in society, by obtaining the right assistive devices, dealing with inaccessibility issues in society to prevent loneliness. This can be strenuous.



#### Patients Journey - lessons learned

- The final version must be a maximum of 2 pages (a larger document can be accessed through a QR code)
- It is better to use full sentences rather than bullet points to make it as clear as possible.
- Allow more space on the schedule for the design
- The patient journey must identify the medical and clinical aspects of the disease, as well as its impact on the patient's real life, and must direct patients to the best experts.



Funded by the European Unio

### Patients Journey - next steps

Diffusion through Euro-NMD and patient organizations

- Conference
- Poster
- Newsletter
- More patient journeys in preparation for 2024
  - Myositis
  - Amyotrophic Lateral Sclerosis (ALS)



Funded by the European Union