29 November 2024

European Economic and Social Committee

# Leaving No One Behind : European Commitment to Tackling Rare Diseases


## French Rare Disease National Plan - Model and Impact

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ASSOCIATIONS UNIES POUR VAINCRE

RD National plans are supported by

MINISTÈRE DE LA SANTÉ ET DE L'ACCÈS AUX SOINS
*Liberté Égalité Fraternité*

MINISTÈRE DE L'ENSEIGNEMENT SUPÉRIEUR ET DE LA RECHERCHE

maladies rares

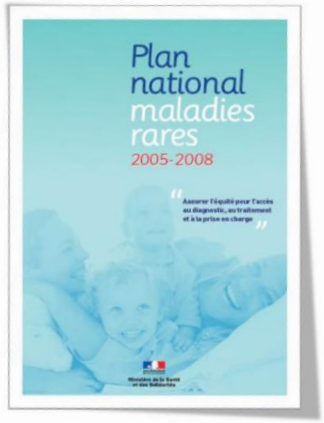
# Nothing about us without us !

**Strong engagement** from patient associations

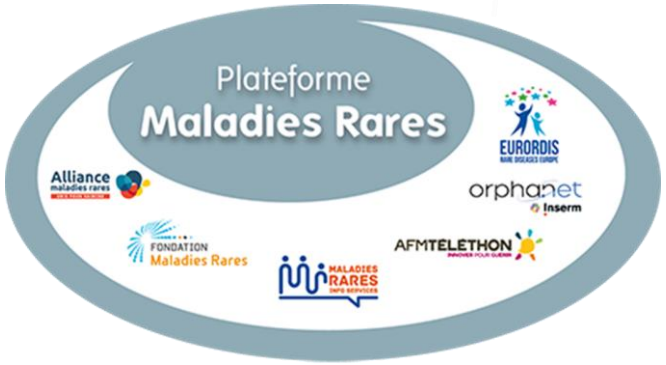
**Major political involvement** from the Parliament and the French Republic Presidents

**A global approach to the rare diseases cause,** tackling all issues simultaneously and in a coordinated way, regardless of any institutional silos

**Strong building of public policies with all stakeholders,** in particular patient associations, through numerous consultations and working groups.

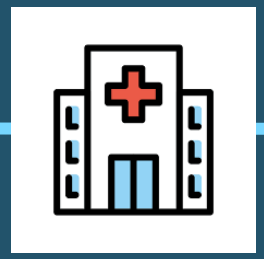




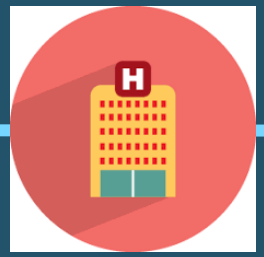
# A rare diseases care ecosystem


The French model



603 Reference Centers

1708 Support expertise centers



23 National rare diseases networks

23 Local rare diseases networks

maladies rares

**Patient Organisations**

Governance involvement

# Reference centers at the heart of patient care

- 70% of patients say they found it easy to contact their center.
- Over 60% waited an average of less than three months for an appointment.
- Only 6% waited over a year.
- 77% were satisfied with the quality of care offered, and 68% with the speed of delivery of test results and hospitalization reports. 71% of respondents were satisfied with the services offered by expert centers.

**TO IMPROVE**

- Coordination between GP and expert center.
- Regarding the “child-adult” transition, 54% of associations said they were satisfied with the support offered between a pediatric service and an adult service.
- Finally, only 33% of centers offer psychological support, 20% the services of a social worker and 30% full information on transport costs, long-term conditions and other more technical aspects.

Survey Alliance/VIAVOICE Septembre 2022

# Rare diseases health networks: For a change of scale

- 80% of patient organizations said they are "satisfied" with the Rare Diseases health networks.

This high level of overall satisfaction is due to a systematic collaboration that works, from the point of view of both the associations and the medical professional networks.

Major successes of the national reference networks includes : decisive support in creating guidelines, monitoring the re-labeling of expert centers, and help in designing and monitoring patient health education programs.

**TO IMPROVE**

Supporting human and social sciences programs, financial support for associations, legal and psychological support, telehealth and raising awareness of rare diseases among general practitioners.

**Raising awareness of rare diseases among professionals appears to be a priority issue for all stakeholders.**

Survey Alliance/VIAVOICE, April 2023

# Concrete example

Filnemus

Filière Neuromusculaire

European Reference Network

for rare or low prevalence complex diseases

Network Neuromuscular Diseases (ERN EURO-NMD)

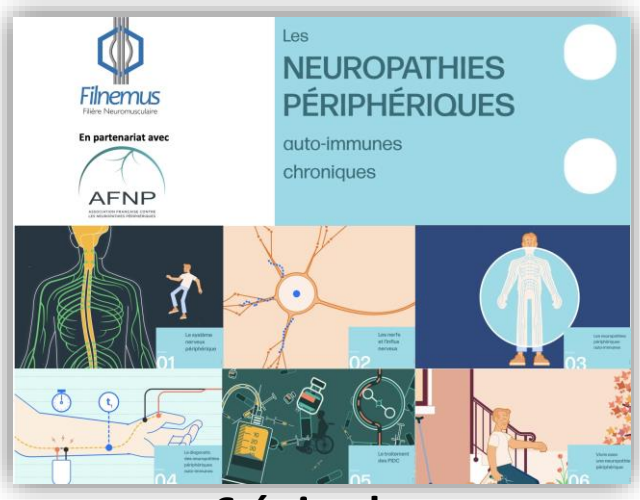
# Education / Information

Ex. Dysimmune and inflammatory neuropathies

.National call for projects, dedicated to patient organizations
.Specific WG « Improve patient journey» / Treatment observatories
.Patient therapeutic education program : to support patient autonomy

. Supporting relevant projects for patients / Education; information…
. In line with the missions of the national reference networks ( improving care - research - education, training and information - Europe and international).

**Videos**




**6 épisodes**


**Interactive information sheets online**


**11 episodes**

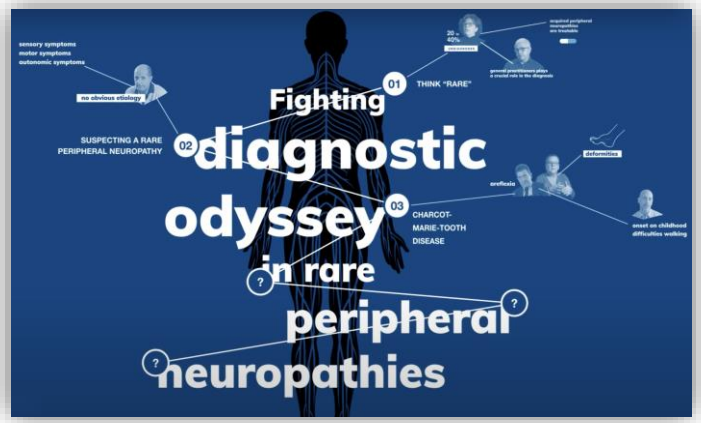
European Reference Network
for rare or low prevalence complex diseases
Network Neuromuscular Diseases (ERN EURO-NMD)

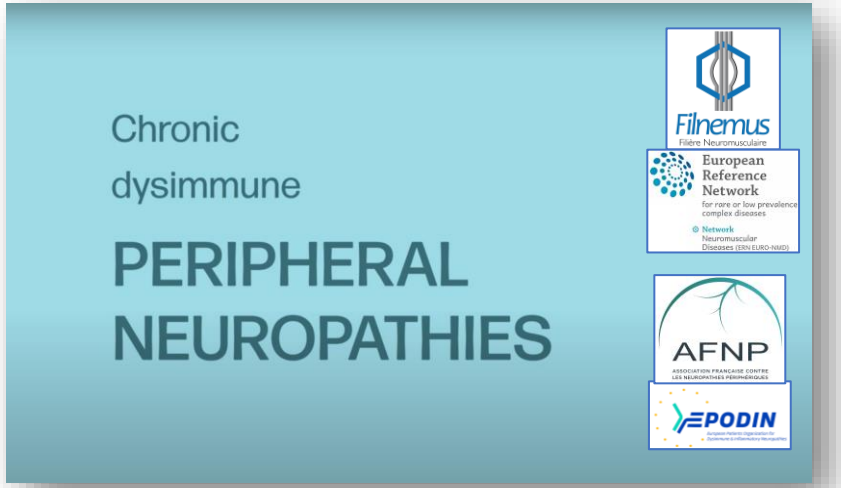
# Education / Training

https://ern-euro-nmd.eu

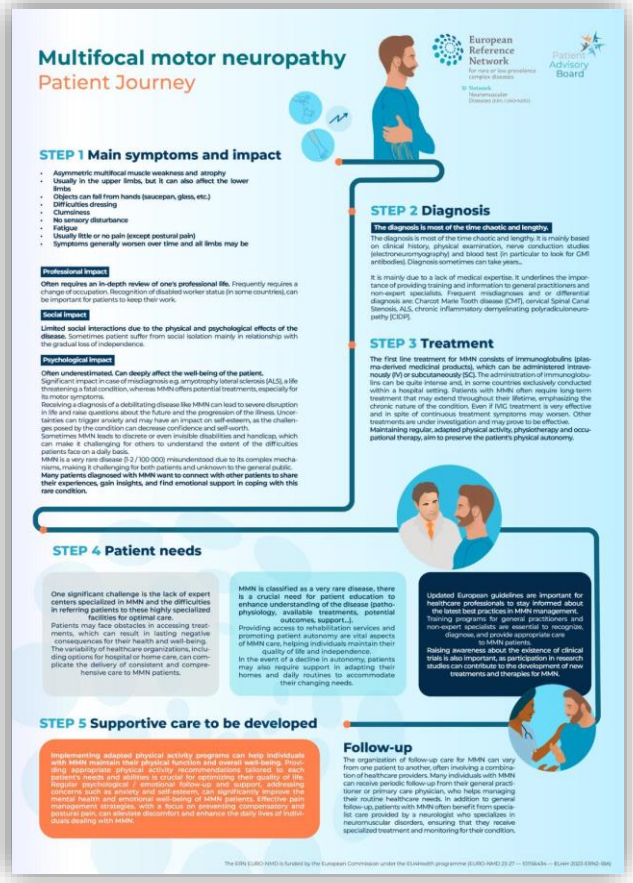
**COLLABORATION ERNs – NRNs +++**
**Supported by POs involvement**






**6 Videos**
**6 Episodes**




**Patient Journeys program (2023-2024)**

. Multifocal Motor Neuropathies
. Myasthenia gravis
. Myofibrillar Myopathy


« But then says Alice, if the world makes absolutely no sense, who's stopping us from inventing one? »

Quotation attributed to
**Lewis Carroll**

**MERCI**

**THANK YOU**