



European
Reference
Network

for rare or low prevalence
complex diseases



Network
Neuromuscular
Diseases (ERN EURO-NMD)

7th ERN EURO-NMD ANNUAL MEETING

Inter-ERN working group Gene Therapy

21st February 2024

Kleopas A. KLEOPA, MD, PhD

The Cyprus Institute of Neurology and Genetics



Funded by
the European Union



AIMS AND SCOPE

This is a joint action between ERN Euro-NMD, ERN-RND and ERN EpiCARE with following aims:

- Exchange ideas for **actions addressing Gene Therapy (GT) aspects relevant to all 3 'neuro' ERNs**
- Strengthen the **role of ERNs in gene therapy development**, clinical trials, and policy around marketed treatments in Europe
- Promote preparation of **position statements, information exchange and integration** in the GT field relevant to the participating ERNs
- **Education, updates**, organization of **webinars** around GT
- Discuss and promote **clinical research for GT** in rare diseases
- Gather information about **approval processes** (risk-benefit evaluation) and **access to GTs across Europe**
- **Evaluate current pricing and reimbursement issues** across Europe, as well as criteria for access to gene therapy, in order to provide uniform **guidelines**
- **Streamline organizational aspects of administering GTs** (in specialized centers, qualification criteria, specialty)
- **Post-marketing surveillance of GTs** (long-term follow up, safety, registries...)
- ***GT WG encompasses everything that relates to modifying genes or genetic information (from gene modification, to RNA therapies and all the way to protein translation)***



Structure of the WG Gene Therapy

- **Project Manager:** [Houda ALI, ERN EURO-NMD](#)
- **18 members** in total comprising (in majority) a mix of adult and pediatric neurologists, some of whom are affiliated with Healthcare providers that participate in all three ERNs
- Participation of **ERN Leaders** (Teresinha Evangelista for EURO-NMD and Holm Graessner for RND)
- The group has not been active between Nov 2021 and May 2023
- Re-started regular **3-4 monthly meetings since May 2023** (May, October, December)
- Kleopas A. Kleopa (The Cyprus Institute of Neurology & Genetics) appointed as WG Chair in May 2023, taking over from Profs Jan Kirschner and Davide Pareyson who were previously co-chairs

WG Gene Therapy Members

EpiCARE	Gaetan Lesca	FR	CHU de Lyon	Medical geneticist
	Davide Mei	IT	University of Florence	Neurogenetics lab technician
ERN-RND (also EURO-NMD)	Holm Graessner	DE	Universitätsklinikum Tübingen	Healthcare & research manager
	Caroline Sevin	FR	Kremlin Bicêtre hospital	Pediatric neurologist
	Günter Höglinger	DE	Hannover Medical School	Clinical neurologist
	David Gomez	ES	University Hospital Vall d'Hebron	Child neurologist
	Bart van de Warrenburg	NL	Radboud university medical center	Clinical neurologist
	Ludger Schoels	DE	University of Tübingen	Clinical neurologist
	Samuel Gröschel	DE	University Children's Hospital, Tuebingen	Child neurologist
EURO-NMD	Kleopas A. Kleopa (also ERN-RND)	CY	The Cyprus Institute of Neurology & Genetics	Neurologist, Researcher
	Davide Pareyson	IT	Istituto Nazionale Neurologico Carlo Besta	Clinical neurologist
	Jan Kirschner	DE	University Medical Centre Freiburg	Pediatric neurologist
	Francina Munell	ES	University Hospital Vall d'Hebron	Pediatric neurologist
	Hanns Lochmuller	CA	The Ottawa Hospital	Neurologist
	Madelon Kroneman	NL	Netherlands Institute for Health Services Research	Researcher, Patient representative
	Houda Ali	FR	Hopital Pitié Salpêtrière	Project manager
	Annemieke Aartsma-Rus	NL	Leiden University Medical Center	Translational genetics- expertise in DMD
Juan Francisco Vázquez Costa	ES	Universidad de Valencia	Neurologist - genetics- expertise in ALS	

Update WG GT activities 2023: SURVEY

Survey on current practices regarding genetic therapies in Europe

- ▶ *Proposed and developed by Holm Graessner with the objectives:*
- To investigate the current landscape of GTs in Europe
 - ✓ access conditions
 - ✓ organizational aspects
 - ✓ clinical decision-making processes currently in use in Europe when gene therapy is considered for treating a patient
- To improve and harmonize access conditions and practices
- To potentially lead to developing linked activities, such as establishment of treatment eligibility boards for GTs for difficult cases, with focus on different diseases groups



Update WG GT activities 2023: SURVEY

Input on the Survey Structure was received by WG Members between Jun-Sep 2023

- ▶ **Focus:** identifying and comparing practices between different countries/centers in regards to treatment availability, reimbursement policies, implementation of clinical trials
- ▶ **Other ERNs were invited to participate**, bringing the total to 5 ERNs:

EURO-NMD

ERN-EYE

ERN-RND

MetabERN

EpiCARE



Update WG GT activities 2023: SURVEY

Methodology

- Web-based questionnaire
- **Each ERN coordination office to send the survey to their HCP representatives by email**
- When: [early March 2024](#)
- The HCP representative will be responsible of completing the survey and collecting more specific information from appropriate specialists within their center

What if my center belongs to more than one participating ERN?

→ Your center will receive the survey from each coordination office and will be kindly asked to complete 1 survey separately for each ERN



Update WG GT activities 2023: WEBINAR SERIES

- ▶ **10 episodes scheduled between early April and end of June 2024**
- ▶ **Led by EURO-NMD, co-organized with EpiCARE**
- ▶ **Focus on:**
 - Advanced gene therapy programs towards clinical translation (IND)
 - Ongoing clinical trials (Phase 1-3)
 - Updates from approved therapies (SMA, MLD, DMD)
- ▶ Cover diseases of interest: **Neuromuscular** (DMD, LGMD, MND), **Epilepsy**

Scope: Lessons learned and practical implications that are relevant for all diseases of interest to the participating ERNs (rather than go too much into details on specific products)



Update WG GT activities 2023: WEBINAR SERIES

► Dates and speakers to be disseminated soon



1	1a. General overview of gene therapy developments for clinicians 1b. Gene therapy in clinical practice- when and how
2	Basic gene therapy technologies and challenges in translation
3	3a. Lessons learned across different disorders and challenges in the development of gene therapies 3b. The regulatory approach to evaluating and approving gene therapies for neurological and other rare disorders
4	n-of-1 and personalized therapies
5	Safety issues in gene therapies and how to address them
6	Genetic therapies and therapy developments for ALS
7	Genetic therapies and therapy developments for epileptic disorders
8	Genetic therapies and therapy developments for muscular dystrophies (DMD, LGMD)
9	Health technology assessment (HTA) of genetic therapies
10	Genetic therapies and therapy developments for SMA

Update WG GT activities 2023: WEBINAR SERIES



Gene Therapy *Webinar Series*



SUBSCRIBE TO RECEIVE ALL INFORMATION ON THE GENE THERAPY WEBINAR SERIES!

Update WG GT activities 2023: Collaboration with Industry

General context: ERNs are encouraged to collaborate with the industry

Key points:

- So far, discussions have mainly focused on development of **educational/scientific activities**: e.g. in-person meeting, workshop, panel discussion...
 - Other areas of collaborations could be discussed later
 - Company to provide financial support (sponsorship?)
 - Diversity of private partners (avoid collaborating with only 1 company)
 - A collaborative project may involve more than one ERN
- Collect ideas on educational activities that we could submit for support (NB: on behalf of all ERNs involved)



7th ERN EURO-NMD ANNUAL MEETING

21st – 23rd February 2024

THANK YOU!

