

# 2-6 July Newcastle 2018 United Kingdom

This summer school is a joint venture between the European Reference Network for Neuromuscular Diseases - EURO-NMD and TREAT-NMD.



EURO-NMD is one of 24 European Reference Networks (ERNs) approved by the Board of Member States. The ERNs are supported by the European Commission. For more information about the ERNs and the EU health strategy, please visit <https://ec.europa.eu/health/ern>

# Neuromuscular Translational Summer School

## 2-6 July 2018

### Programme Committee

Annemieke Aartsma-Rus  
Leiden University Medical Center - Netherlands  
& Newcastle University - UK

Teresinha Evangelista  
Newcastle University - UK

Hanns Lochmüller  
Newcastle University - UK

Silvere van der Maarel  
Leiden University Medical Center - Netherlands

**L** **U**  
**M** **C** Leids Universitair  
Medisch Centrum



International Centre for Life  
Institute of Genetic Medicine  
Newcastle University  
Central Parkway  
Newcastle-upon-Tyne  
NE1 3BZ - United Kingdom



European  
Reference  
Network

for rare or low prevalence  
complex diseases

Network  
Neuromuscular  
Diseases (ERN EURO-NMD)



# Neuromuscular Translational Summer School

2-6 July 2018

This five-day summer school has been developed for researchers and clinicians interested in translational research.

This course will address the following aspects as it travels along this established pathway.

- Bench to bedside research
- Regulatory system
- Clinical trials
- Outcome measures
- Patient communication
- Registries and biobanks
- Biomarkers and –omics.

Approximately 24 participants will be based at the Institute for Genetic Medicine, part of Newcastle University in the UK for the duration of the summer school.

During their stay, they will discover how networks such as the European Reference Network EURO-NMD and TREAT-NMD work and collaborate with patients and regulators to facilitate therapy development. Aspects such as standards of care, outcome measure development, biomarkers and others will be developed during the week. Attendees will also learn about the various interactions that take place between key stakeholders.



Our summer school will consist of plenary lectures, interactive panel sessions, workshops and participatory developmental sessions.

Attendees will learn how the regulatory system works, take part in a TACT style review and discuss ethical issues in clinical trials.

In addition, attendees will find out about various outcome measures and gain an appreciation of the need for standardisation and training in this area. They will participate in a clinical trial practicality forum as well as a patient communication workshop to demonstrate the results of both good and poor communication.

This comprehensive course will serve as a superb foundation for those wanting to steer their medical or research career in the direction of neuromuscular diseases whilst contacts made as a result of attending will, no doubt, prove to be invaluable.

Attendees who complete this course will not only be furnished with knowledge of the many different aspects that are involved in translational research but will also gain a deeper understanding and compassion for those involved in the whole trial process from researcher to patient.

## Draft Programme

**Monday 2 July**  
**Translational lifecycle**  
Bench to bedside research  
Preclinical research tools  
Regulatory system  
TACT style therapeutics review

**Tuesday 3 July**  
**Clinical trials**  
Ethical aspects of clinical trials  
Outcome measures  
Developments in outcome measures  
TREAT-NMD clinical trial tools

**Wednesday 4 July**  
**Biomarkers**  
Validating MRI as biomarker  
Patient reported outcome measures  
Clinical trial practicality forum

**Thursday 5 July**  
**Patient Communication Workshop**  
Importance of patient communication  
Communication workshop by behavioural scientists  
How patients can help with research

**Friday 6 July**  
Participant presentations  
General discussions  
Feedback

Are you already working in the neuromuscular field?  
Are you a researcher or a clinician interested in discovering more about this exciting field?

Or, are you interested in sponsoring this summer school?

For further details contact us  
info@ern-euro-nmd.eu

**ern-euro-nmd.eu**