



# Collaborating with Partners



**Advancing diagnosis, care and  
treatment for those living with  
neuromuscular diseases**

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# What We Can Do For You

## How we can help

TREAT-NMD is a global network of key opinion leaders within the neuromuscular community whose mission is to support all stages of therapy development, in order to improve the health and quality of life of people around the world with neuromuscular diseases (NMDs). TREAT-NMD is committed to promoting collaborations among patients, clinicians, industry, academic and research institutions, advocacy groups and regulatory agencies, recognizing the advantages of strong interactions in accelerating cutting-edge therapies for otherwise unmet medical needs.

## What we do

TREAT-NMD is internationally recognized for its key role in successfully addressing some of the major issues that face therapeutic development in NMDs. We have a strong track record of advising the scientific and medical community, as well as providing the pharmaceutical industry with essential go-to resources - including education masterclasses, clinical trial assistance and global data provision - to advance novel treatments for neuromuscular conditions.



# TREAT-NMD Advisory Committee for Therapeutics (TACT)

TACT is an advisory panel of leading, international experts in all aspects of neuromuscular therapy development.

## What is TACT?

The TACT committee is comprised of over 70 expert members including clinicians, regulatory experts, pre-clinical academics, patients, statisticians, toxicologists, medicinal chemists, physiotherapists and clinical trialists. TACT provides a unique resource and educational tool to the neuromuscular community, which helps to bridge the gap between promising preclinical data and successful clinical trials.

## What does TACT do?

TACT meets to review therapy development plans from early preclinical through to phase III trials from industry or academic-led groups seeking advice. Bespoke panels drawn from the wider committee offer guidance on the translational and development pathway in all genetic neuromuscular diseases (NMDs). Since its establishment in 2009, TACT has reviewed over 70 applications for advice in disease areas including, DMD, SMA, MTM, LGMD, FSHD, DM, Pompe and congenital myopathies.

The confidential and comprehensive independent review provides recommendations and advice on areas including regulatory considerations, trial design, selection of endpoints and go/no-go decisions, enrolment and dosing.



I feel very privileged to be involved in TACT, which combines multidisciplinary expertise with a constructive attitude. The goal is not to discredit therapeutic approaches, but to make sure preclinical and clinical studies are designed and conducted optimally because as a field we owe this to the patients. The multidisciplinary teamwork for each application is a unique, motivating and educational experience for everyone involved. Each expert tries to improve an approach based on their own background and knowledge to provide the applicant with very comprehensive feedback for future development."

**Annemieke Aartsma-Rus, TACT Chair**



"We found the TACT report to be concise and well-organized. The committee put together a very thoughtful review and we intend to use the recommendations to augment our development plan"

**Dr Deborah Ramsdell, Valerion Therapeutics, LLC**

"The expertise TACT assembled in one room was unparalleled and greatly appreciated. The review provided us with an outside consensus regarding how to proceed in this uncharted territory as the first company working in this disease (CMD)"

**Jodi Wolff, Santhera**

# TREAT-NMD Education & Training

Since 2015 TREAT-NMD has been designing and delivering expert masterclasses, seminars, workshops and conferences on a range of neuromuscular diseases.

These educational events have proven successful with patients, clinicians, researchers, patient advocacy groups and industry. Such events are a great platform for knowledge exchange as well as being a catalyst for greater engagement with partners and an excellent opportunity to network.

TREAT-NMD masterclasses feature presentations led by internationally recognised experts in the field of neuromuscular disease. The programmes for the events are comprised of high-quality scientific lectures and interactive workshops for clinicians and other health professionals involved in diagnosing and/or managing the care of patients.

*It has been a very well organised event. Learnt a lot! Great platform to interact on. Congratulations from India!*

Vineeth Jaison  
Christian Medical College,  
India

*It has been a tremendous opportunity to meet other experts around the world.*

Doug Levine  
Cure Duchenne, USA

*Thank you so much. Great opportunity to learn so much and feel part of something much bigger.*

Paula Fenty NHS Trust  
Nottingham, UK

*Thanks for a fantastic Masterclass. It's truly amazing work.*

Fatima Vido-Vecchio  
Children's Hospital,  
Canada

## Masterclasses

Since 2015 TREAT-NMD has delivered...

Over 10 successful educational masterclasses reaching over 35 different countries around the world

Events to more than 750 delegates

Masterclasses and seminars in four different disease areas both virtually and face to face

These educational events are made possible by unrestricted grants from industry. If you are interested in supporting an expert masterclass or another type of educational event in any neuromuscular disease or an issue relating to the field of neuromuscular disease, please contact: [education@treat-nmd.org](mailto:education@treat-nmd.org)

Further information about all of our events is available on our website:  
[treat-nmd.org/engagement/](http://treat-nmd.org/engagement/)

# TREAT-NMD Conference

The TREAT-NMD Conference is a networking and knowledge exchange event. Delegates include academics, patients and carers, patient advocacy organisations, clinical specialists and representatives from industry.

The aim of this international conference is to share progress and lessons learned in the field of translational medicine in inherited neuromuscular diseases and plan for the delivery of future therapies to patients.

The first TREAT-NMD Conference was held in 2009 and with each event it has gone from strength to strength with delegates travelling from across the globe to attend.



### Delegates have the opportunity to:

- Network with partners on key issues and challenges in neuromuscular disease
- Engage with panels of international experts who will inform, stimulate and challenge debate
- Acquire the latest updates on standards of care, outcome measures, patient reviews, and new developments
- Be a part of the global network of TREAT-NMD

The TREAT-NMD Conference provides a fantastic opportunity for all stakeholders, including patients, academics, clinicians, registry curators and industry representatives, to get together to network, learn and exchange ideas about translational research.

To find out about sponsorship opportunities please get in touch:  
[info@treat-nmd.com](mailto:info@treat-nmd.com)

# TREAT-NMD Event Support

The team at TREAT-NMD has extensive experience in running virtual and in person events such as neuromuscular conferences, seminars and educational events.

Are you a society, group or organisation working in the neuromuscular field? Do you want to run a conference, an event or an educational seminar? Need help to do so?

If you have a neuromuscular conference or event in the pipeline and need our expertise, we can assist with:

- Creating a programme
- Approaching expert speakers
- Gathering feedback
- Marketing your event
- Setting up an online event platform
- Running the event

Get in touch to find out how we can help with your next event: [info@treat-nmd.com](mailto:info@treat-nmd.com)



# TREAT-NMD Global Registry Network 'TGDOC'

TREAT-NMD operates an established and experienced global network of more than 80 independent neuro-muscular patient registries in over 40 countries. These registries make up the TREAT-NMD Global Registry Network, (TGDOC).



To find out more please get in touch: [info@treat-nmd.com](mailto:info@treat-nmd.com)

TGDOC aims to support the registries in several areas including the expansion of existing disease specific datasets to include data items around therapies and medications, as well as the establishment of new disease datasets to increase the possible scope and range of Global Registry Enquiries.

## Global Registry Enquiries

Global Registry Enquiries can be requested by industry, contract research organisations, regulators, patient organisations, clinicians or academia into the network in order to support clinical trial feasibility, academic research, trial recruitment, or other research needs.

The income from Global Registry Enquiries is reinvested into the Global Registry Network by funding the Annual Curators Meeting, Disease Subgroup Meetings, Registry Bursaries, and subsidising academic enquiries where appropriate.

TREAT-NMD patient registries and our strategic partner Care and Trial Sites Registry (CTSR) are important tools in the clinical trial feasibility process.

TREAT-NMD can direct and coordinate enquiries into the Care and Trial Site Registry (CTSR) for neuromuscular and neurodegenerative diseases (NDDs). This valuable resource can provide information to assist with selection of trial sites and identification of potential partners for upcoming research projects.

## Data Governance

Registries are supported to collect the established TREAT-NMD Core Minimum Datasets for Duchenne muscular dystrophy (DMD), spinal muscular atrophy (SMA), myotonic dystrophy type 1 (DM1), facioscapulohumeral muscular dystrophy (FSHD) and limb girdle muscular dystrophy (LGMD) wherever possible.

The network includes a variety of data entry types, including clinician-reported, patient-reported and various levels of data verification by curators or geneticists.

The TGDOC Membership Process ensures a high level of data quality and compliance with information governance regulations and ethical bodies' (REC/IRB) requirements.

The Global Registry Network and the Registry Enquiry Process are governed by a Charter and the TGDOC Executive Board, whilst supported by Disease Subgroup Leads.



# TREAT-NMD Core Dataset Projects

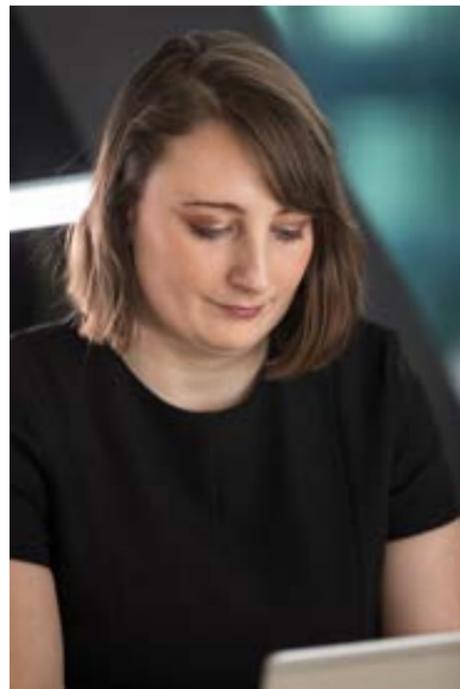
Comprehensive, disease specific datasets in Spinal Muscular Atrophy, Duchenne Muscular Dystrophy, Limb Girdle Muscular Dystrophy to better inform on the natural history of neuromuscular diseases are a cornerstone of TREAT-NMD's work.

These datasets capture high quality data to support post-marketing surveillance (safety and effectiveness) for new treatments.

Working with industry, regulators and key clinical & academic opinion leaders, we use collaborative workshops to develop new and expand existing datasets which are validated by pilot registries from across our global network.

To ensure the successful adoption of the datasets by registries, our in-house project management team collaborate with curators to support implementation. These efforts are supported by an in-depth online dataset specification to assure high quality data capture.

Collaborating with its global alliance of registries, leading clinicians and other partners, TREAT-NMD is ideally positioned to advance the expansion of rare disease datasets, to enable registries to be a reliable source of high quality data in the collection of real-world evidence and to capture valuable information for regulatory purposes and ultimately post marketing activities.



# TREAT-NMD Global Registry Platform

The TREAT-NMD Global Registries Platform is a cloud-based platform which is offered to our registry network.

## What we offer:

- Patient Reported Portals
- Clinician Reported Portals
- Dual Reported Patient Led Portals
- Dual Reported Clinician Led Portals.

The latest TREAT-NMD core datasets for SMA, DMD and LGMD are implemented into the platform. We are expanding the Global Registries Platform to include additional datasets and have exciting plans for FSHD and DM1.

As part of TREAT-NMD Global Registries Platform, the Central Data Warehouse has been developed to provide a secure data sharing mechanism which will support natural history study and provide anonymised patient-level data and aggregate data for research purposes.

At TREAT-NMD we are embarking on the development of a Post Marketing Surveillance module to further support this activity.

To find out more get in touch:  
[info@treat.nmd.com](mailto:info@treat.nmd.com)

*"TREAT-NMD has a global reputation for successfully delivering world-class translational research tools, educational/training programmes and advancing clinical innovation throughout the neuromuscular community.*

*Since 2007, the breadth and expertise within our network has enabled us to develop the highest quality resources and tools to address the major challenges faced within our community, in collaboration with our stakeholders – patients, clinicians, advocacy groups, industry and regulatory agencies."*

Professor Kevin Flanigan, former TREAT-NMD Executive Committee Chair



At TREAT-NMD our vision is to accelerate the development of effective treatments and to establish best practice diagnosis and care for neuromuscular patients



Get in Touch

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**TREAT-NMD**

Neuromuscular Network

[www.treat-nmd.org](http://www.treat-nmd.org)