Partnering & Collaborating with Industry

www.treat-nmd.org/industry
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Amyotrophic Lateral Sclerosis
Becker Muscular Dystrophy
Charcot Marie Tooth
Congenital Muscular Dystrophy
Congenital Myasthenic Syndromes
Duchenne Muscular Dystrophy
Facioscapulohumeral Muscular Dystrophy
GNE Myopathy
Limb Girdle Muscular Dystrophy
Myotonic Dystrophy
Myotubular Myopathy & Centronuclear Myopathy
Pompe Disease
Other Neuromuscular Diseases
How we can help

TREAT-NMD is a global network of world-class experts 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, industry, academic 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 registry management, clinical trial assistance and post marketing services - to advance novel treatments for neuromuscular conditions.
Supporting Therapy Development at Every Stage

Connecting our partners to essential ‘go-to’ resources in the neuromuscular field

REGISTRIES

TREAT-NMD Global Registry Enquiries
National and international coverage
Patient self and/or clinician reported
Compliance with information governance regulations and ethical bodies’ (REC/IRB) requirements
Data curation and validation
Communication with stakeholders: patient, academic and commercial parties

RESEARCH

Standardized experimental protocols
Development and assessment of Standards of Care (SoC)
Phenotype/genotype correlation studies
Patient reported outcomes expertise, including working towards validation of new outcomes
Socio-economic studies on burden of illness
Access to EuroBioBank
TREAT-NMD Advisory Committee for Therapeutics (TACT)

info@treat-nmd.org
TREAT-NMD offers a range of services for investigators and the pharmaceutical industry. By providing important resources and strong expertise, TREAT-NMD continues to help streamline the translational research process, whilst supporting collaborative relationship interactions.

Feasibility study in population of interest
Care and Trial Site Registry (CTSR):
  - Trial site identification
  - Identification of patient population
Patient recruitment via TREAT-NMD global registries
Dissemination of information about your study
TREAT-NMD Advisory Committee for Therapeutics (TACT)
Good Clinical Practice (GCP) and trial design training

TREAT-NMD Universal Registries Platform
Advice on post marketing surveillance (PMS) development
Support with PMS monitoring and outcomes of research
Identification of patient population
Delivery of communication strategy

Educational masterclasses and training workshops
Conferences and networking
Collaboration opportunities (public-private partnerships)
Development and translation of medical writing
EURO-NMD and TREAT-NMD summer school

If you would like to discuss any of the above opportunities or any additional opportunities, please contact us: info@treat-nmd.org Tel: +44 (0)191 241 8839
TREAT-NMD has established an experienced global network of more than 80 patient registries, in over 40 countries. All patients have a genetic diagnosis.

TREAT-NMD patient registries and the Care and Trial Sites Registry (CTSR) are unique and important tools in the clinical trial feasibility process. Collaboration with TREAT-NMD has enabled the commencement of an extensive number of clinical studies.

The data provided through the global patient registry network and CTSR dramatically accelerates trial planning and removes the barriers to getting a trial established.
The TREAT-NMD global database network assists academic investigators and pharmaceutical industry partners with trial feasibility and recruitment strategy and projections.

TREAT-NMD can also 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. Please see the TREAT-NMD website for further information, including our mandatory core datasets for DMD, SMA, DM1 and FSHD.

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<th>From the Global Patient Registry Network:</th>
<th>From the Care and Trial Site Registry:</th>
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<td>Numbers of patients with specific mutations</td>
<td>The CTSR contains over 360 centres for NMDs and/or NDDs in 57 countries</td>
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<td>Aggregated numbers of patients in categories, such as patients by age range, treatment regimen and ambulation status</td>
<td>Information on trial sites including equipment and staffing, muscle biopsy experience and clinical trial experience</td>
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<tr>
<td>Total numbers of patient per country meeting specific inclusion criteria from over 40 countries worldwide</td>
<td>Details of site diagnostic capabilities e.g. availability of specific genetic tests</td>
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What is TACT?

TACT is an advisory panel of leading, international experts in all aspects of neuromuscular therapy development. The TACT committee is comprised of over 70 expert members including clinicians, regulatory experts, pre-clinical academics, patients, statisticians, toxicologists, 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 twice a year to review therapy development plans from early preclinical through to phase III trials from industry or academic-led groups applying for advice. Bespoke panels drawn from the wider committee offer guidance on the translational and development pathway in all genetic NMDs. Since its establishment in 2009, TACT has reviewed almost 60 applications for advice in disease areas including, DMD, SMA, MTM, LGMD, FSHD, DM, Pompe and congenital myopathies.

The confidential and comprehensive review provides recommendations and advice on areas including regulatory considerations, trial design, selection of endpoints and go/no-go decisions, enrolment and dosing.
What industry and academics have to say about TACT

“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

“TACT is a very valuable instrument to help clinicians and scientists to design sensible clinical trials in muscular dystrophy”

Dr Emilio Clementi & Dr Grazia D’Angelo, Sacco University Hospital, Italy

“The discussion and varied perspectives of the diverse group that TACT convenes has been invaluable in moving towards the clinical testing of our compound in DMD”

Dr George Mulligan, Mitobridge

“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 unchartered territory as the first company working in this disease (CMD)”

Jodi Wolff, Santhera

“TACT comments highlighted relevant issues of the proposal and will surely help us to strengthen the final version of the project”

Professor Giuseppe Vita & Dr Sonia Messina, University of Messina, Italy

“It is a special privilege to be involved in TACT, as it is a unique advisory board based on independence, excellence, and multidisciplinary expertise and with no direct interest, other than to provide honest and constructive advice on the de-risking of therapy development in rare neuromuscular diseases.” Prof. Annamaria De Luca, TACT Chair
Since 2015 TREAT-NMD has been designing and delivering expert masterclasses, 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.

Further information about all of our events is available on our website: https://treat-nmd.org/engagement/

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: info@treat-nmd.org
What the delegates said:

“The masterclass was an excellent learning event.”

“Each session featured excellent speakers who shared their knowledge and expertise in a very friendly and engaging way.”

“An amazing event. Very well-organised with excellent speakers.”

“All of the topics were very interesting and enlightening, especially the presentation on emerging therapies. The event also provided a great platform for networking.”

“Excellent organization and the opportunity to meet the DMD specialists from different countries.”
Post marketing surveillance (PMS) strategies are becoming a necessary part of an orphan drug manufacturer’s responsibilities.

PMS is part of the drug development process (also known as Phase IV) and is mandated by the pharmaceutical regulators (FDA, EMA and other national bodies). This is to allow for the collection of safety and efficacy data once a drug has received (conditional) marketing authorization and to assess how it performs in clinical practice.

As well as helping to extend the evidence base for the therapeutic treatment in clinical practice, the capture of PMS data also enables companies to use this evidence to extend the indications for use of the therapeutic treatment in a further submission for market authorization.

Within the TREAT-NMD network we have a team who have extensive experience supporting the PMS requirements of pharmaceutical companies.
To assist companies during the PMS phase we offer a range of services including:

- Strategic and operational input throughout the PMS process
- Health economic models
- Burden/cost-of-illness studies
- Access to the global patient registry network and the Care and Trial Sites Registry to assist with recruitment strategy and projections
- Development and management of communications strategy
- Development and delivery of educational/training workshops (e.g. training on study specifics and outcome measures for clinicians)
- Expansion of mandatory core datasets to include data items required for PMS

To find out more about our complete PMS service and our current work on the development of a PMS platform, please contact us - info@treat-nmd.org
“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

Here at TREAT-NMD our ambition is to continue accelerating therapeutic development and improve standards of diagnosis and care for those living with neuromuscular disease around the world.
Want to find out more about how we can help you?

Contact us

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