Partnering with Industry

"Connecting and collaborating on translational research and clinical innovation"

treat-nmd.eu/industry
<table>
<thead>
<tr>
<th>Neuromuscular Disorders</th>
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<tr>
<td>Becker Muscular Dystrophy</td>
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<td>Charcot Marie Tooth</td>
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<td>Congenital Muscular Dystrophy</td>
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<td>Congenital Myasthenic Syndromes</td>
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<td>Duchenne Muscular Dystrophy</td>
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<td>Facioscapulohumeral Muscular Dystrophy</td>
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<td>GNE Myopathy</td>
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<td>Limb Girdle Muscular Dystrophies</td>
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<td>Myotonic Dystrophy</td>
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<td>Myotubular Myopathy &amp; Centronuclear Myopathy</td>
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<td>Spinal Muscular Atrophy</td>
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We support these neuromuscular disorders.
What can we do for you?

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 (NMD). 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.

TREAT-NMD is highly recognized for playing a key role in successfully addressing some of the major issues that face therapeutic development. 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**

Set-up and management
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 SERVICES**

Disease prevalence and epidemiological studies
Standardized experimental protocols
Development and assessment of Standards of Care (SOC)
Phenotype/genotype correlation studies
Patient reported outcomes expertise, including validation of new outcomes
Quality of life assessment
Socio-economic studies on burden of illness
EuroBioBank
TREAT-NMD Advisory Committee for Therapeutics (TACT)

business@treat-nmd.eu
“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 of 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

Advice on post marketing surveillance (PMS) development
Support with PMS monitoring and outcomes of research
Identification of patient population
Delivery of communication strategy
Preparation of education material and training

Educational masterclasses and training workshops
Conferences and sponsorship opportunities
Collaboration opportunities (public-private partnerships)

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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 registries and CTSR dramatically accelerates trial planning and removes the barriers to getting a trial established to test a potential therapy.

At the recruitment stage, eligible patients can be contacted through the TREAT-NMD global patient registry in parallel with clinical trial site-based recruitment efforts.
“Global databases assisting academic investigators and the pharmaceutical industry with trial feasibility and recruitment strategy and projections”

All patients have a genetic diagnosis

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<tr>
<th>From the Global Patient Registry:</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 330 centres for NMDs and/or NDDs in 49 countries</td>
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<tr>
<td>Numbers of patients in categories such as patients by age range, corticosteroid use and ambulation status</td>
<td>Information on trial sites including equipment and staffing, muscle biopsy experience, clinical trial experience</td>
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<tr>
<td>Total numbers of patients 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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TACT is internationally recognized for its multi-disciplinary panel of experts with representatives from academia, industry, patient foundations and governmental scientific research centres.

The committee meets twice a year to review promising drug candidates and provide guidance on the translation and development pathway in NMD and amyotrophic lateral sclerosis (ALS).

The confidential and comprehensive review provides recommendations including go/no-go milestones, is independent of any funding stream, however it may enable subsequent funding.
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

“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
TREAT-NMD has designed and delivered comprehensive educational training workshops and conferences. These programmes have proven successful with patients, clinicians, researchers, patient advocacy groups and industry, and have acted as a catalyst for greater engagement with partners and fostered greater knowledge exchange.

In 2015 and 2016, TREAT-NMD delivered a DMD Masterclass*, which offered presentations and interactive workshops led by internationally recognized experts in the field. The 2-day workshop provided a high-quality scientific meeting for clinicians and other specialist health professionals involved in diagnosing and/or managing the care of patients with DMD.

If you are interested in supporting an expert masterclass or other types of educational workshops, in any neuromuscular disease, or issue relating to the field, please contact TREAT-NMD - business@treat-nmd.eu

* Made possible by an unrestricted educational grant from PTC Therapeutics
What delegates said about this workshop

“I commend the organizers and the faculty for optimizing time / discussions / forums throughout the conference”

“The meeting was successful, informative and educational”

“Excellent presentations by experts”

“Well organized, good networking. Excellent”

“I found the masterclass to be immensely useful. I would like to attend similar educational opportunities in the future. I appreciate your excellent logistical support”
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.
“TREAT-NMD supports late phase PMS (IV) studies, by providing expertise to help support the implementation of systematic processes and solutions, that help you address regulatory demands and achieve your PMS goals”

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 global patient registry 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)

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