

# TREAT-NMD

Impact Review 2022



## A history of TREAT-NMD

TREAT-NMD is a global network for the neuromuscular field, providing an infrastructure to ensure that the most promising new therapies reach patients as quickly as possible and that best practices are shared.

Established in 2007 with European funding, the network brings together clinicians, researchers, patients and their representatives, and the pharmaceutical industry. Since 2007, the network has developed infrastructure to support drug development and access in the rare, genetic neuromuscular diseases space.

As the Network's capabilities improved, it was clear that the network needed a legal entity to allow it to enter contracts and employ staff to further the Network's aims. In 2019, TREAT-NMD Alliance Ltd and wholly owned subsidiary were set up. These companies are owned by the TREAT-NMD Network and reinvest any surplus funds into the driving forward the aims of the Network.



## Our vision

To accelerate the development of effective treatments and to establish best practice diagnosis and care for neuromuscular patients worldwide.

## Our mission

To operate a collaborative, inclusive global network and organisational infrastructure that will overcome fragmentation, providing support services, information and data to advance treatment, diagnosis and care for neuromuscular patients globally.

## Our aims

- Leverage and expand our global reach
- Provide the 'go to' tools and services to support each stage of translational research
- Provide educational tools to improve diagnosis, treatment and care
- Facilitate agreement and adoption of standardised care guidelines, pre-clinical models, outcome measures and disease-specific datasets.
- Further de-risk and accelerate the development of therapies by extending our advisory committees and enquiries processes.
- Raise our profile and that of the neuromuscular disease areas we serve
- Facilitate best practice in data collection and become the 'go to' provider of NMD data to support evaluation, approval and post authorisation requirements of new treatments.

# Consolidating our not-for-profit status

A key achievement of 2022 was the consolidation of the not-for-profit status of TREAT-NMD Alliance when the company was registered with the UK Charity Commission.

This registration will bring immediate impact to TREAT-NMD. It makes new funding streams available and is tax efficient, allowing us to bring more funding to the NMD field and drive forward the networks aims.

To maximise the benefits to the Network, each service that TREAT-NMD offers has now been mapped to either TREAT-NMD Alliance Ltd or TREAT-NMD Services Ltd - a wholly owned business arm that allows for commercial work to be undertaken to further the Charity's aims.

# Mapping the neuromuscular disease

One of the aims of TREAT-NMD is to prevent fragmentation in the NMD field. It is vital to understand the areas where other organisations are investing and working – either in case we can add value to their work, or to minimise duplication and allow our resources to be invested elsewhere.

In 2022 we completed landscaping reviews in facioscapulohumeral muscular dystrophy and myotonic dystrophy. We are now working with the strategic partners identified in those reviews to offer the support of TREAT-NMD in their work.

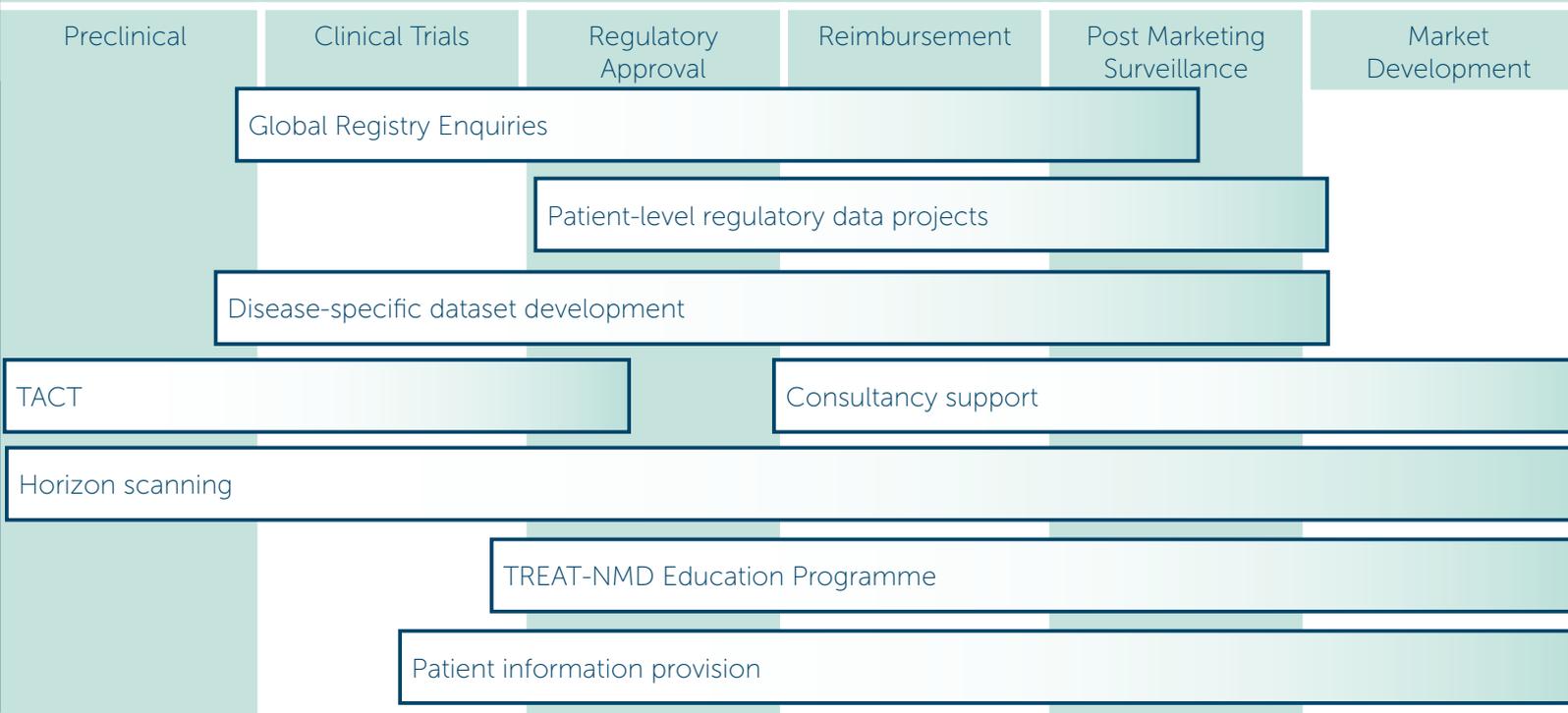
In future these landscaping reviews will form part of the Charity's strategic decision-making process, with a new Neuromuscular Disease Advisory Committee providing quarterly updates and recommendations to the Board of Trustees of TREAT-NMD Alliance Ltd on the landscape and latest developments in NMD research, clinical trials and patient care; specific areas of unmet need within the NMD field; the establishment of a task force to address a specific unmet need; and work areas and projects where TREAT-NMD could add value.

# How does TREAT-NMD impact the NMD field?

The power behind TREAT-NMD is the membership, with more than 1,000 members across the world who drive the agenda and aims of TREAT-NMD Alliance Ltd. TREAT-NMD Alliance Ltd offers direct and indirect support to network members, providing skills and experience in a broad range of support functions, from event management to contract negotiation, to data analysis.

Both TREAT-NMD Alliance Ltd and Services Ltd conduct projects that are identified as important by the Network members. These projects cover areas of unmet need across the rare, genetic neuromuscular space, from educational masterclasses suggested by the TREAT-NMD Educational Committee, to supporting members of the TREAT-NMD Global Registry Network to take part in enquiries and regulatory studies.

In short, TREAT-NMD Alliance Ltd aims to support the NMD field wherever our members identify an unmet need and we believe TREAT-NMD can add value.



# What is TACT?

The TREAT-NMD Advisory Committee for Therapeutics (TACT) is a multi-disciplinary group of internationally recognized experts that review and provide guidance on the translation and development of therapeutics programmes in rare NMDs.

TACT can address issues of trial design, formulation, toxicology, regulatory and marketing considerations, and make recommendations on go-no-go milestones.

Established in 2009, TACT has held 25 review meetings in Europe, the US, and remotely and reviewed 70 applications across 10 diseases; 80% were from industry and 20% from academics.

Following each review, TACT sends a confidential, advisory report to the applicant. A TACT review is NOT an endorsement by TACT or by TREAT-NMD, and while TACT does not share the report with anyone other than the original applicant but interested parties and potential funders are encouraged to contact the researcher directly to request a copy of TACT reports.

8

reviews in 2022

2

sets of meetings

70

reviews completed

80%

reviews from industry

# De-risking drug development

This year TACT completed eight reviews of drug development programmes. The de-risking and expert input a TACT review provides is held in high esteem across established pharmaceutical companies in the NMD space, and this year we made changes to maximise benefits of TACT and to guarantee its successful future.

The scope of TACT has been widened to allow companies to apply for a review even before a lead compound has been selected. As well as allowing more experts to take part in TACT reviews, this extension will start the vital de-risking process as early as possible in the development process.

TACT has benefitted significantly from our new horizon scanning activities. We now proactively approach companies entering the NMD space to offer the services of TACT. This year also saw a new meetings schedule, with one annual and one virtual meeting each year; a change that will reduce workload on reviewers, and allow more flexibility for applicants. Excitingly, we've also seen the TACT model being used in other conditions, with an "ACT" being established as part of the European Connect 4 Children initiative.

This is an extremely collegial and helpful environment and I really found the engagement of TACT to be exceptional. The panels backgrounds were really helpful and the questions already prior to getting the final report were very instructive and will help us in our clinical design as well as enhancing our preclinical story

Pliant Therapeutics

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

Valerion Therapeutics, LLC

## The TREAT-NMD Global Registry Network

The TREAT-NMD Global Registry Network is a federated network of 67 individual, independent, national (or regional) patient registries that collect data on neuromuscular patients from more than 61 countries worldwide.

The network includes registries collecting data from clinicians, patients or both, with some patient-reported registries employing varying levels of verification by curators, clinicians, or geneticists.

Our membership process ensures a high level of data quality and compliance with information governance regulations and ethical bodies' (REC/IRB) requirements.

Bringing eligible patients and relevant clinical trials together is a key challenge in rare disease research. The Global Registry Network enquiries process allows industry, researchers, regulators, patient organisations, clinicians or academics to request access to data held within the registries of the Global Registry Network.

# 61

Member registries

# 12+

Neuromuscular diseases

# 31

Countries

# 78,183

Patients

# 50,223

Patients with a genetic diagnosis

# Supporting regulatory data provision

This year, we supported members of the TREAT-NMD Global SMA Registry Network to take part in a multi-year post-authorisation study required by the European Medicines Agency. Eight registries collecting data on SMA patients are taking part in the project, with TREAT-NMD Services Ltd providing central services that cover project management, contracting, indemnity, data quality control and assurance.

Pharmaceutical companies awarded marketing authorisations in the rare disease space are often required by regulators to commit to long-term post-authorisation studies. These studies allow drug developers to gain real-world insight into the treatment's safety and efficacy and often provide regulators with the data required to grant a full approval to a treatment. This is a vital step to give patients access to treatments.

The complex requirements of sponsors can make this kind of study inaccessible to registries with limited resources. By dealing with these complexities centrally, TREAT-NMD can offer registries the chance to take part in the study, gaining valuable experience and longer-term funding. As more drugs are given marketing approvals, TREAT-NMD hopes to offer more registries in our network the opportunity to take part in this kind of study.

## Towards a sustainable registry network

This year we rolled out a new registry funding model, and have started to routinely reimbursing individual registries for the time and effort they put into responding to enquiries. We've also formalised our registries' development fund that allows us to reinvest in the Global Registry Network. The first bursaries to registries have been issued (a total of £17,000), and the fund has supported our annual curator's meeting and training and support for registries.

TREAT-NMD has also provided resources to support publications from the registry network. Nineteen poster abstracts were accepted at leading conferences, with one being awarded a flash presentation at the World Muscle Society Congress.

By supporting the registries within the Global Registry Network we hope to improve data collection and build a thriving community, where registries collaborate closely to collect good quality data and address research questions that matter to registry participants.

# Data bringing together patients and clinical trials

TREAT-NMD provides a single point of contact for academics, clinicians and pharmaceutical companies to request aggregate data from across the TREAT-NMD Global Registry Network. In 2022, 4 enquiries were completed, including clinical trial feasibility, recruitment and real-world evidence studies.

This year we have started to be proactively reach out to companies who may benefit from registry enquiries. Relying on our horizon scanning information and relationships maintained with companies attending TACT, we plan to increase the number of enquiries undertaken in the coming years, with discussions on at least 6 future enquiries already underway.

To enable us to support the increasing number of enquiries, we have also invested in our Registries team. We've brought different internal workstreams together into a single team and this allows more enquiries to be dealt with, both in total and in parallel. As well as providing income to participating registries, Enquiries also support bursaries and meeting for the wider Global Registry Network.

## Driving harmonisation with defined datasets

Registries working in a specific disease area collect data using a harmonised, disease-specific dataset developed using a consensus-based approach that involves key opinion leaders, registries, clinicians and patients.

To promote harmonisation across the NMD field, TREAT-NMD makes the dataset specification (including list of data items, data dictionary and suggested questions) freely available online. Data collectors interested in the dataset can benefit from free support for detailed gap analyses as well as financial support in adopting the dataset.

This year TREAT-NMD provided Registry Bursaries worth more than £12,000 to assist registries in adopting one of the TREAT-NMD datasets. We have also secured funding that will allow us to offer bursaries to eight registries adopting the limb girdle dataset in 2023.



DMD Expanded dataset



LGMD Expanded dataset



SMA Expanded dataset

## Supporting registries: the TREAT-NMD Global Registry Platform

The TREAT-NMD Global Registry Platform is provided to registries free of charge and is designed to provide an off-the-shelf experience for registries, through a software-as-a-service agreement. Ten registries are currently using the platform.

TREAT-NMD Services Ltd customises the platform for each user (for example, choosing whether clinicians or patients report data) and provides training and support. The platform can currently store data on patients living with Duchenne and Becker muscular dystrophy, limb girdle muscular dystrophy or spinal muscular atrophy.

TREAT-NMD ensures the platform remains compliant with data protection and other relevant regulations, allowing registries to focus on collecting and curating their data. Each registry maintains ownership of their data, and only users approved by registry curators can access the data.

# About the TREAT-NMD Education Programme

The TREAT-NMD Education Programme brings together world-leading experts in neuromuscular diseases with those keen to learn more, allowing clinicians to take their professional development into their own hands and learn the latest in diagnosis, standards of care and emerging therapies in these diseases.

TREAT-NMD has been designing and delivering masterclasses and workshops on a range of neuromuscular diseases since 2015. The format has proved popular with patients and clinicians, with programmes of high-quality scientific lectures and interactive workshops presented by internationally recognised experts in the field of neuromuscular disease.

In 2022 we organised masterclasses on congenital myopathies and limb girdle muscular dystrophy masterclass. We also took our successful format further to deliver an "expert" spinal muscular atrophy masterclass and a Duchenne muscular dystrophy early diagnosis seminar aimed at non-NMD specialist clinicians.

# 4

Masterclasses in 2022

# 280+

total attendees

# 40+

Attendee Countries



Upcoming events

# Online learning with Talent LMS

In September 2022 TREAT-NMD launched an online education and training portal, using TalentLMS. The platform aims to educate and train clinicians and provides for testing, progress monitoring and reporting.

Online content is developed by leading clinicians, academics and expert educators around the world and we aim to host from the OpenTACT physiotherapy educational resource set up by Muscular Dystrophy UK.

The learning content can be accessed by professionals to learn wherever, whenever. The platform is available at any time of day and night, across multiple devices, so users can learn around their other commitments for free.

As well as providing a standalone functionality, TalentLMS will increase the impact of TREAT-NMD masterclasses, making content available to more viewers over longer periods of time. Importantly, it will reduce the time leading clinicians are asked to devote to teaching the same content freeing time to create content in other areas.

## Family guides

Family care guides play a key role in translating the complex standards of care in NMDs into family-friendly language.

To date, our family guides have focussed on translating and simplifying complex care guidelines, but there is an increasing requirement for guides covering complex clinical trials and treatments to be made available in countries with less active patient advocacy groups. This year we have established working groups and secured initial funding to produce a guide to gene therapy, and will start this work in 2023.

## Supporting network members

TREAT-NMD Alliance Ltd is owned by the members of TREAT-NMD and can offer support and services to members who are undertaking work that will help to further our charitable aims. This support we offer comes in different forms, and we're always happy to discuss how different types of support could help to accelerate the developments of treatments and improve care.

## Event support

The education team at TREAT-NMD has extensive experience in running virtual and in person events such as neuromuscular conferences, seminars and educational events for societies, groups or organisations working in the neuromuscular field.

We can assist with programme and speaker management, marketing, running and collecting feedback for your event.

## TREAT-NMD Website

In 2022 we have invested heavily in the TREAT-NMD website – rebuilding the underlying architecture to make it fit-for-purpose. The new website will be launched in 2023 and will make it easier to find the right content, and allow content on individual pages to be tailored to different audiences.

As well as impacting people looking for content, the website will have a huge impact on how we work as an organisation, streamlining the process for TACT applications and registry enquiries, and allowing us more control over event sign ups.

## Consultancy services

The staff at TREAT-NMD Services Ltd have a broad range of experience, which can be offered on a consultancy basis to organisations working on projects that further the aims of TREAT-NMD.

In 2022, we supported a company investigating outcomes-based reimbursement for an NMD drug in the UK. All too often, a market approval is seen as the successful endpoint in developing a new treatment and reimbursement addressed as an after thought. One of our key aims is to bring treatments to patients as quickly as possible and supporting novel solutions brings this goal closer.

# TREAT-NMD newsletter

The TREAT-NMD newsletter is sent to more than 3,000 people interested in rare neuromuscular diseases every month.

As well as keeping readers up to date with what is happening at TREAT-NMD, the newsletter can have direct impact. This year it has enabled companies interested in developing treatments for NMD diseases to contact patient organisations, to gain input at the very earliest stages.

The newsletter also allowed us to offer support to non-specialist clinicians in countries around Ukraine who were treating NMD patients fleeing the war. Within 1 week of an appeal in the TREAT-NMD newsletter we had built up a database of more than 100 clinicians (neurologists, physiotherapists, occupational health specialists, cardiologists) offering to answer questions from colleagues in Eastern Europe. We'd like to thank everybody who took part in this effort, especially those who answered questions.

## Horizon Scanning

Our horizon scanning service was started in 2022, initially to help TREAT-NMD Services Ltd to become more proactive in reaching out to biotech and pharmaceutical companies entering the rare neuromuscular disease field for the first time, and to existing companies in the space starting new drug development programmes.

The service has already strengthened our TACT And Registry Enquiries pipelines, and has helped us to offer drug developers a seamless journey between the services we provide. However, we also appreciated that providing the horizon scanning information externally could be of benefit to member organisations of TREAT-NMD - supporting investment decisions and providing an overview of the NMD, or disease-specific landscape.

In 2022 we worked with two patient organisations in DMD to provide quarterly updates about the latest therapeutic advances and drug development news in their disease area. We're currently working with them to develop a more in-depth offering that will help them to maximise the impact of our horizon scanning service.



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