



## Charter for the TREAT-NMD Global Data systems Oversight Committee (TGDOC)

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## 1) Background

Inherited neuromuscular disorders (NMDs) form a large group of diseases, each of which is individually rare (prevalence < 5/10,000). Scientific advancement has led to substantial changes on how to approach the treatment of NMDs. New therapeutic strategies are being developed, many of which target specific genetic defects and there are a small number of novel therapeutics approved for a few diseases. The majority of NMDs, however, are orphan diseases and there is still considerable ground to cover to help translate basic science to impactful patient care. Given the rarity of these diseases, the implementation of clinical trials requires large, multi-centered studies. It is very important that patients who meet the right criteria are identified and contacted within a short period of time. Equally, natural history and other forms of disease data are invaluable to academics, patient groups and biotech/pharma companies, working together to develop safe and effective therapies and cures. A new era of regulatory approved and commercially available therapeutics for NMD is upon us and this requires a comprehensive and academic approach to generating real world evidence related to new therapies to inform regulatory decisions and Post Marketing Surveillance.

Since its launch in January 2007 TREAT-NMD's focus has been on the development of tools that industry, clinicians and scientists need to bring novel therapeutic approaches through preclinical development, into the clinic and ultimately to patients, as well as establishing best-practice care for rare neuromuscular disease patients worldwide. More information on TREAT-NMD can be found in the [TREAT-NMD Alliance Charter](#). One of the subgroups within TREAT-NMD that is crucial in supporting TREAT-NMD's goals, is the TREAT-NMD Global Data systems Oversight Committee (TGDOC).

TREAT-NMD believes it can provide a key role in supporting the NMD community, particularly through close working and collaboration with patient registries, many of which are already working together in a federated manner as part of TGDOC, contributing anonymized patient data from their datasets.

The aim of having TGDOC Member Registries is to provide a greater level of engagement and harmonization of our efforts and approach, to capturing and collecting unified disease specific data, to support registry development, and to ensure high quality global data can be made available for effective research and analysis. As such, TGDOC Member Registries represent a core component of the TREAT-NMD community, to which they can contribute through:

- The provision of disease specific data for academic research, public health studies, or clinical trial feasibility studies,
- The identification and contacting of patients whose profile fits the inclusion criteria of a clinical trial or other scientific study,
- The provision of data for long term longitudinal drug-specific studies, such as those required for Post Marketing Surveillance (PMS) once therapies have received regulatory approval.

All data requests and data handling will be in compliance with ethical guidelines for research and scientific studies involving human subjects as well as regional and international legal requirements for data governance for their handling, transfer & storage, and sharing of data.

In 2016, the TREAT-NMD global network of registries received the label of IRDiRC Recognised Resource (<https://irdirc.org/research/irdirc-recognized-resources/current-irdirc-recognized-resources/>; <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5255942/>). IRDiRC (International Rare Diseases Research Consortium; <https://irdirc.org/>) is a world-wide consortium of research funding bodies that promote diagnosis and development of therapies in the field of rare diseases. With the support of its Task Forces and Scientific Committees, it has developed a number of recommendations and guidelines on good practices in preclinical and clinical research on rare diseases, including those related to sharing of patients' data.

TREAT-NMD is proud of its recognition in the field of patient registries and strives to maintain this IRDiRC recognition with the support of all participating Registries.

## 2) Definitions

- **The TREAT-NMD Membership Alliance** [TREAT-NMD Alliance] is an independent, unincorporated global network consortium, whose aim is to work together with the network and other stakeholders, to support therapy delivery and accelerate translational research, through the advancement of diagnosis, care and treatment for those living with rare NMD around the world, using the most efficient and effective means possible.
- **TREAT-NMD Services Ltd** [the TREAT-NMD Enterprise] is the not-for-profit corporate and legal entity, set up by the TREAT-NMD Alliance, to manage its legal and commercial affairs and, as such, will be the entity that will be involved in legal agreements, operational management and financial transactions relating to TGDOC matters.
- **TREAT-NMD** will be the term used to refer to both TREAT-NMD Alliance and TREAT-NMD Enterprise collectively.
- **TGDOC** is the **TREAT-NMD Global Data systems Oversight Committee**.
- **TGDOC Executive Board** is comprised of the TGDOC Chair troika (i.e., the Chair, the Chair-elect, and the outgoing Chair), the TREAT-NMD Services CEO and the TREAT-NMD Global Registry Enquiries Manager as well as others that the TGDOC Chairs may see fit to appoint as additional Executive Board members.
- **Patient Registries** are databases (or data repositories) containing structured and searchable data, collected from individuals with a shared characteristic such as a disease or a gene defect. These may be regional, national or international registries that aim to enlist the majority of patients in a given region, country or several countries.

- **TGDOC Core Member Registries** are individual regional, national or international Patient Registries that must meet the TGDOC Core Membership criteria detailed in section 4 of this Charter. Core Member Registries are expected to share anonymous, aggregate patient data for use in TGDOC Registry Enquiries whenever possible.
- **TGDOC Affiliated Member Registries** are individual regional, national or international Patient Registries that meet the Affiliated Member criteria in section 4 of this Charter. These registries are part of the TGDOC Community but do not, or are not able to currently meet the TGDOC Core Membership criteria, required to take part in Registry Enquiries.
- **TGDOC Member Registries** means the TGDOC Core Member Registries and TGDOC Affiliated Member Registries, collectively.
- The **Curator** of a TGDOC Member Registry is the individual responsible for the curation and coordination of the registry. This person is the main point of contact for TGDOC matters. It is advisable for each registry to have a primary and a secondary curator if possible.
- **TGDOC Subgroup Leads** are individuals who assist the TGDOC Executive Board with coordinating the TGDOC disease-specific Subgroups comprised of the Core and Affiliated member registries who collect data on the relevant disease. See the *TGDOC Subgroup Lead Terms of Reference* for further information.
- **Patient Representatives** are individuals that represent the interests of disease-specific patient communities on the TGDOC. Each TGDOC Subgroup will have at least one Patient Representative. Please refer to the *TGDOC Patient Representative Terms of Reference* for more information.
- A **Data Enquiry** is a formal written request to TREAT-NMD by a third party for non-exclusive access to aggregated, de-identified patient data within the TREAT-NMD Global Data systems by means of the TGDOC Core Member Registries.
- A **Recruitment Enquiry** is a formal written request to TREAT-NMD by a third party to support patient recruitment and enrolment in one or more clinical trials through patient outreach by one or more disease-relevant TGDOC Core Member Registries.
- A **Registry Enquiry** is either a Data Enquiry or a Recruitment Enquiry involving Core Member Registries.
- An **Enquiry Vote** is a vote to approve or reject an Enquiry as described in Section 6(a) of this Charter and in the *TGDOC Voting and Enquiries SOP*.
- A **Survey** or **informal query** is where a third party requests information from the entire TGDOC Member network where collection of specific Core Dataset items is either not required, or not yet possible. In such situations, TGDOC Affiliated Member Registries will also be asked to participate in these queries. **Recruitment support activities** may also fall under this category if Core Dataset items are not required within the recruitment inclusion criteria. See section 6 of the *TGDOC Voting and Enquiries SOP* for further details.

### 3) Purpose of the Charter

This Charter shall:

- a. Define the basic requirements for Patient Registries to become and remain a TGDOC Core Member Registry or a TGDOC Affiliated Member Registry.
- b. Define the relationship between the TGDOC Core and Affiliated Member Registries with TREAT-NMD.
- c. Define the association between the TGDOC Core Member Registries and TGDOC, and the data that they provide.
- d. Define the relationship and the use/transfer of information from TGDOC with third parties (such as industry and academic partners) requesting use of data that is shared through the Core Member Registries, for research or clinical trials.
- e. Be an integral part of agreements and contracts between TREAT-NMD and institutions/associations in charge of regional, national or international Patient Registries, and between TREAT-NMD and third parties.
- f. Be a publicly available guidance document for clarity of the stakeholder community.

### 4) TGDOC Membership Criteria

See chart in **appendix b** for a summary of the essential and highly encouraged TGDOC membership criteria for Core and Affiliated registries.

#### Core Member Registries

The following **essential criteria** MUST be met for a Patient Registry to become a **TGDOC Core Member Registry**:

- Pledge to adhere to this TGDOC Charter, the TGDOC Membership and Voting SOP and the TREAT-NMD Alliance Charter.
- Collect all **mandatory items** defined in the TREAT-NMD Core Minimum Dataset(s) for the relevant disease areas, and as many of the **highly encouraged** items as possible
- Agree to complete an initial Registries Review Survey, and to update this annually.
- Sign a Confidential Disclosure Agreement (CDA) to allow TREAT-NMD to share requests, which may be of a confidential nature, from registry enquirers, and a Disclosure of Interests (DoI) form for internal review only.

- Agree to adhere to and abide by all applicable regional / national data protection regulations.
- Agree to inform TREAT-NMD of any important operational updates regarding their Patient Registry (i.e. change of curator, new Core dataset collection etc.).
- Agree to inform the TREAT-NMD Publications Committee of any upcoming publications where TREAT-NMD is mentioned (prior to publication), and consult with them when required.
- Allow their registry contact information on the TREAT-NMD website.
- At least one Curator, or member of staff from each Patient Registry should aim to attend the Annual TGDOC Curators' Meeting (reimbursements are offered to support travel and accommodation).
- Participation in disease-specific TGDOC Subgroups, as required.
- Sign up to receive the TREAT-NMD monthly, and the TGDOC quarterly newsletters.

The following **strongly preferred** criteria should also be met whenever possible:

- Participation in Surveys / Non-Core dataset Queries

#### Affiliated Member Registries

The following **essential criteria** MUST be met for a registry to become a **TGDOC Affiliated Member Registry**:

- Pledge to adhere to this TGDOC Charter and the TREAT-NMD Alliance Charter.
- Agree to complete an initial Registries Review Survey, and to update this survey annually.
- Agree to adhere to and abide by all applicable regional / national data protection regulations.
- Agree to inform TREAT-NMD of any important operational updates regarding their Patient Registry.
- Allow their registry contact information on the TREAT-NMD website.
- Agree to inform the TREAT-NMD Publications Committee of any upcoming publications where TREAT-NMD is mentioned (prior to publication), and to consult with them when required.
- Sign up to receive the TREAT-NMD monthly, and the TGDOC quarterly newsletters.

The following **strongly preferred** criteria should also be met whenever possible:

- Aim to collect all mandatory items defined in the TREAT-NMD Core Minimum Dataset(s) for the relevant disease areas, and as many of the highly encouraged items

as possible.

- Sign a Confidential Disclosure Agreement (CDA) to allow TREAT-NMD to share requests, which may be of a confidential nature, from registry enquirers and a Disclosure of Interests (DoI) form for internal review only.
- At least one Curator, or member of staff from each Patient Registry should aim to attend the Annual TGDOC Curators' Meeting (reimbursements are offered to support travel and accommodation).
- Participation in disease-specific TGDOC Subgroups, as required.
- Participation in Surveys / Non-Core dataset Queries.

### All TGDOC Member Registries

The following **statements** apply to all TGDOC Member Registries:

- a. Industry shall not hold member status within TGDOC.
- b. All TGDOC Member Registries remain owner, maker or author of the data included in their registries and are free to grant access, re-utilization or permit extraction or grant any right in accordance to this ownership, without the approval of TREAT-NMD.
- c. All TGDOC Member Registries should strongly support and promote the use of the TREAT-NMD Global Data systems as an available single point of entry for data access by third parties related to multinational clinical trials and studies.
- d. The TREAT-NMD website will display information about TGDOC activities and inform patients and the public about all TGDOC Core and Affiliated Member Registries.
- e. All TGDOC Member Registries may display the TREAT-NMD logo on their websites. TREAT-NMD may display the name, contact information and logo of TGDOC Member Registries on the TREAT-NMD website.

## **5) Relationship of TGDOC Core Member Registries with TREAT-NMD**

- a. TGDOC recognizes the reality that each registry is an independent entity that will function within TGDOC in accordance with their internal governance.
- b. TGDOC Core Member Registries will provide de-identified data as described below in Section 5c to TREAT-NMD for non-exclusive use consistent with its mission as described herein. TREAT-NMD may provide such de-identified data to third parties, under agreement, who have engaged with TREAT-NMD in Enquiries requiring data from the TREAT-NMD Global Data systems for specific disease areas, but only following the Enquiry Voting Process. This includes participation in Data Enquiries and Recruitment Enquiries. Individual TGDOC Core Member Registries are expected to submit an Enquiry Vote regardless of their capacity to provide enquiry data.

- c. Data to be collected by TGDOC Core Member Registries will include a standardized and harmonized set of core mandatory items, as specified in the [TREAT-NMD Disease Specific 'Core Datasets'](#). TGDOC Core Member Registries will collect all of the Mandatory Core Dataset items, as a minimum requirement, and as many of the 'Highly Encouraged' data items as possible. Mutations will be annotated according to the guidelines of the [Human Genome Variation Society](#).
- d. As the number, frequency and complexity of Global Registry Enquiries has increased in recent years a new enquiry costing model has been adopted to include reimbursement payments to registries that participate in the data enquiries into the DMD and SMA datasets, in line with the current payment model for recruitment enquiries. Academic and not-for-profit enquiries will continue to be charged at a cost-recovery price and will not include enquiry payments to ensure these remain as affordable as possible for those without easily available funding. Enquiry payments are paid in recognition of the work of the registry curators and will never be linked directly to the data provided in an enquiry; three enquiry payment brackets have been established based on the total number of genetically confirmed patients in an individual registry for the disease in question.
- e. TREAT-NMD Services Ltd. will sign a Confidential Disclosure Agreement with each TGDOC Member Registry with respect to any patient-related data submitted for use in the TREAT-NMD Global Data systems to ensure that the use and disclosure of any such data is limited to the specific purposes set forth in this Charter and the purpose for which that data has been provided to TREAT-NMD.
- f. Where TREAT-NMD have gone on to establish an agreed 'Expanded Dataset' for a particular disease, TGDOC Core Member Registries will collect all of the Mandatory Core Dataset items in the Expanded Dataset as a minimum requirement within a year of the Expanded Dataset being issued. Any issuing of revisions to Mandatory Core Datasets, must be implemented within 6 months of the revision being issued.
- g. TGDOC Core Member Registries that are unable to collect all Mandatory items from a Core Dataset within the specified timeframes must inform TGDOC Executive Board as soon as possible. Such Registry may temporarily move into the 'Affiliated' membership status until the essential criteria are met to resume their 'Core' membership status.
- h. TREAT-NMD will not request or receive patient identifiable data from any TGDOC Member Registries and shall not have direct access to patients.
- i. When a Recruitment Enquiry is approved, patients meeting eligibility criteria for a clinical trial/study will either be contacted directly by their TGDOC Core Member Registry Curator, other registry staff member or their physician, depending on the Registry. All patient contact will take place directly via the applicable TGDOC Core Member Registry.
- j. TGDOC Core Member Registries will collect and process data according to the national or international laws and best practices that apply to each of them respectively (in particular, accuracy and minimization of data; informed consent concerning use of the data for research; right of the patients to withdraw consent etc).
- k. TGDOC Core Member Registries must ensure the patient data that they collect pursuant to this Charter is updated at least every 12 months. If for any reason such patient data has not been updated in over 12 months, and is rendered out of date as a result, the Registry Curator should bring it to the attention of the TREAT-NMD Global Registry Enquiries Manager, and any out of date data should not be included in TGDOC Enquiries



(unless otherwise indicated in the Scope of Work).

- l. In accordance with the ethical principles of sharing information derived from patient data, the Executive Board of TGDOC will share with TGDOC Member Registries details of any publications, projects or trials, or other academic product that may benefit the NMD community that arise from Data or Recruitment Enquiries.
- m. A summary of active and completed Enquiries will be included in the quarterly TGDOC newsletters, and a detailed summary of all Enquiries will be presented at the Annual Curators' Meeting. In addition, full enquiry reports detailing the results of the enquiry will be shared with TGDOC Core Member Registries pursuant to the terms of the applicable confidential disclosure agreements.
- n. TGDOC Core Member Registries will refer enquiry requests by third parties for accessing data to the Executive Board where multiple TGDOC Core Member Registries are relevant to the request. This principle does not apply to enquiries that are related to a single regional, national or international registry only. Upon referral of any such request, the Executive Board shall treat it as an Enquiry received from a third party.
- o. The fee structure for charging third parties for TREAT-NMD registry enquiries/services will be developed by TREAT-NMD Services Ltd, approved by the TGDOC Chairs and reviewed on at least an annual basis. Fees paid by third parties for access to de-identified data collected from the TGDOC Core Member Registries by TREAT-NMD pursuant to this Charter, and for any services of TREAT-NMD on behalf of the TREAT-NMD Global Data systems, will be shared between TREAT-NMD and TGDOC Core Member Registries, within an annualised fee sharing structure, to be determined by the TGDOC Executive Board. Where appropriate/necessary, TREAT-NMD Services Ltd will negotiate on behalf of the TGDOC Core Member Registries with third parties.
- p. Registry compensation payments are pre-agreed financial contribution payments made by commercial third parties, to TGDOC Member Registries (indirectly via TREAT-NMD) as compensation for using their services (e.g. for the recruitment of patients into clinical trials). All such payments will be shared fairly among the applicable TGDOC Registry Members to compensate for the time commitments in providing such outreach and recruitment services.
- q. A contract or service agreement shall be established between TREAT-NMD Services Ltd and any third party whilst following the Enquiry Voting Process, the agreed Scope of Work and any other relevant principles outlined in the Charter. Please see the TGDOC Voting and Enquiries SOP for further information.
- r. The TGDOC [Publications Committee](#) must review any publications that use collective data from the TREAT-NMD Global Data systems and approve any use of the TREAT-NMD logo on any publications from any TGDOC Member Registries. Registries that have participated in an enquiry which results in a publication should be given the right to view a draft of the publication in advance of publishing. For the avoidance of doubt, any TGDOC Member Registry shall be free to publish, without any such review or approval, any data otherwise in its possession or control even if also in the TREAT-NMD Global Data systems, however use of the TREAT-NMD logo on such a publication would still require approval.
- s. All TGDOC Core Member Registries within the applicable disease area will be required to participate in Enquiry Votes on third party Data Enquires requesting data from the TREAT-

NMD Global Data systems.

- t. Specified TGDOC Core Member Registries within the applicable disease area will also be required to participate in Enquiry Votes on Recruitment Enquiries that require the identification and contact of patients from individual TGDOC Core Member Registries in a specific disease area.
- u. Any TGDOC Core Member Registry who does not fulfil their Enquiry Voting obligations for 2 consecutive Enquiries (i.e. fail to vote) will be invited to participate in a teleconference with one of the TGDOC Chairs to discuss voting issues and help overcome any obstacles to full participation. Persistent non-voting may result in TGDOC Core Membership being reviewed by the TGDOC Chairs. Further details are in the *TGDOC Voting and Enquiries SOP*.

## 6) Relationship of third parties with TREAT-NMD and TGDOC Core Member Registries

- a. TREAT-NMD will grant access to aggregated, de-identified data to third parties only under the following conditions:
  - Third parties provide full disclosure on the purpose of their Enquiry, how the data will be used, and appropriate ethics approval has been granted (e.g. by an institutional review board),
  - The third party is not in conflict with TREAT-NMD goals,
  - The Scope of Work (SOW) of the Enquiry is approved by an Enquiry Vote. Please see the *TGDOC Voting and Enquiries SOP* for further information.
- b. A contract (which includes confidentiality clauses) will be signed between TREAT-NMD Services Ltd and the third party.
- c. Services provided in connection to commercial organizations will be charged at commercial rates which will vary depending on the Scope of Work (i.e. extent and complexity).
- d. Services provided in connection to academic institutions and Patient Organisations shall be provided at a cost-recovery rate when necessary. Researchers are encouraged to include funding for Enquiries in grant proposals. Any research publications derived from the use of the data obtained through TREAT-NMD must acknowledge support by TREAT-NMD and by the TGDOC Core Member Registries that contributed the data to the research, and the Publications Committee should be informed within the specified timeframes.
- e. Services provided by TGDOC Core Member Registries for time and effort spent contacting patients eligible for a trial or scientific study during a Recruitment Enquiry from commercial third parties shall be reimbursed at a standard rate, to be agreed in the Scope of Work for the Enquiry.
- f. A statement detailing the total annual service fees generated by use of the TREAT-NMD

Global Data systems shall be shared with all TGDOC Member Registries and with the TREAT-NMD Alliance Executive Committee at their respective Annual Meetings.

- g. Third parties will not be given direct access to patients or patient identifiable data.
- h. All parties agree that data derived from the TREAT-NMD Global Data systems may be used for registering medicinal products through the FDA, EMA and other regulatory bodies.
- i. All parties agree with the ethical principle of benefit sharing, which requires that where possible, details of any publications, projects or trials, or other academic product resulting from any scientific research and its applications should be shared, especially with the persons and groups that have taken part in the research.
- j. For the avoidance of doubt, all parties agreeing to work together within the terms of this Charter will not be held liable by each other or to each other for unintentional acts or omissions whilst undertaking their responsibilities, as covered under this Charter.

## **7) TREAT-NMD Global Data systems Oversight Committee (TGDOC)**

- a. TGDOC is the governing structure with oversight of the TGDOC Member Registries, and the TREAT-NMD Global Data systems comprised of TGDOC Core Member Registries, on behalf of TREAT-NMD.
- b. The TGDOC is composed of all TGDOC Member Registries, supported by representatives of the TREAT-NMD Alliance (Care and Trial Site Registry, Project Ethics Council), Subgroup Leads and Patient Representatives for each disease area.
- c. The TGDOC is governed by the TGDOC Executive Board, with the Chair being elected every two years. The Chairs are responsible for the management of TGDOC and provide strategic guidance and governance decisions on the work of TGDOC, its activities and its interactions with the TGDOC Subgroups, TGDOC Member Registries and other stakeholders. The Chairs will hold regular fortnightly conference calls, supported by the TREAT-NMD Global Registry Enquiries Manager and other TREAT-NMD Secretariat representative(s) and, where appropriate, TGDOC Subgroup Leads.
- d. If no suitable incoming Chair is found, or if it is deemed operationally appropriate by the troika, the terms of the Chairs can be extended for an additional year by a two-thirds vote of all TGDOC Members.
- e. The incoming Chair position will be advertised to all TGDOC Members and via other appropriate TREAT-NMD communication channels, including the TREAT-NMD and TGDOC newsletters, website and twitter account. Relevant person criteria will be agreed by the sitting TGDOC Chairs and should aim to fill any knowledge or experience gaps that may exist when the outgoing Chair will rotate off. Nominations will be sought, then a shortlist will be drawn up by the current TGDOC Executive Board and all TGDOC Members will vote to elect the new incoming Chair. The nominee who receives the majority of votes will be elected.
- f. TGDOC Subgroup Leads will support the TGDOC Executive Board and assist with the

management and coordination of the applicable disease-specific TGDOC Subgroups.

- g. The TGDOC Subgroups consist of the TGDOC Core Member Registries for a specific disease, and any Affiliated Member Registry who also collects data on that disease.
- h. TGDOC Subgroup Leads will be selected from each TGDOC subgroup of Member Registry curators and will join the TGDOC Executive Board on a conference call (with the TREAT-NMD Global Registry Enquiries Manager) on a quarterly basis.
- i. Each TGDOC Subgroup for which a disease Core Dataset exists should have two Subgroup Leads, as they will be required to coordinate both TGDOC Core and Affiliated Member Registries. Please see the *TGDOC Subgroup Leads Terms of Reference* for further information.
- j. All TGDOC Member Registries that are required to complete a CDA must also disclose any potential conflicts of interest in a Disclosure of Interest (DoI) form for internal review only. TGDOC Core Member Registries should also disclose any conflicts during specific enquiry votes.
- k. All TGDOC Core Member Registries and Advisory Voting Members are required to sign a Confidential Disclosure Agreement (CDA) with TREAT-NMD. TGDOC Affiliate Member Registries are also encouraged to sign a CDA in order to participate in a third party survey or informal enquiry, as specified in the Section 6 of the *TGDOC Voting and Enquiries SOP*.
- l. The TGDOC should meet in person at least annually, and by teleconference or by e-communication upon request.
- m. The TGDOC Executive Board will report to the TREAT-NMD Alliance and/or Services Ltd Executive Board and to the TGDOC Member Registries annually.
- n. The TGDOC Executive Board reviews all Enquiries of third parties into the TREAT-NMD Global Data systems. The TGDOC Chairs will have initial oversight of an Enquiry and will instruct the TREAT-NMD Global Registry Enquiries Manager to agree a Scope of Work (SOW) with the third-party enquirer. The SOW will then be reviewed by the Subgroup Leads and Patient Representative for the disease area in question, the PEC representative and the CTSR representative (if appropriate). The TGDOC Executive Board will agree on the fee to be charged, and the Scope of Work will be sent to the TGDOC Core Member Registries for the relevant disease area to complete an Enquiry Vote for final approval or rejection pursuant to Section 6(a) of this Charter. The TREAT-NMD Global Registry Enquiries Manager will then report the decision of the Enquiry Vote in writing to the third party. For the avoidance of doubt, any TGDOC Member Registry shall have the right to submit a Data Enquiry to the TGDOC Executive Board, which will then be reviewed and subject to the usual Enquiry Voting process. Additionally, any TGDOC Member Registry is free to sharing data with any other TGDOC Member Registry outside of the scope of this Charter.
- o. All Enquiry Votes require a minimum of two-thirds of the TGDOC Core Member Registries for the relevant disease area to vote to reach quorate, and of those votes received a minimum of two-thirds must be in favor for the Enquiry to be approved. If a decision cannot be reached, the Enquiry shall be rejected. In the case of a rejection, the TGDOC Chairs may set a time frame for reconsideration if appropriate, and the TREAT-NMD Global Registry Enquiries Manager will report this, and the reason for rejection to the

third party.

- p. TGDOC Member Registries will be consulted for co-authorship on any publications resulting from use of their data in accordance with the International Committee of Medical Journal Editors (ICMJE) criteria.
- q. The TGDOC Budget shall be managed by TREAT-NMD Services Ltd., via the TREAT-NMD Global Registry Enquiries Manager and any substantial spending decisions shall be approved by the TGDOC Executive Board.
- r. TGDOC recognizes the reality that each registry is an independent entity that will function within TGDOC in accordance with their internal governance.

## **8) Validity of the Charter**

- a. TGDOC Members shall review and provide comment on any substantial revisions of the Charter and its enclosures. The TGDOC Executive Board will discuss feedback received and implement any further changes deemed appropriate. The TREAT-NMD Alliance Executive Committee will then review the final draft and take advice from any TREAT-NMD advisory committees (e.g. Project Ethics Council), before approving and ratifying the Charter.
- b. The Charter shall be reviewed on an annual basis.

## **9) Ethical and legal principles**

- a. TREAT-NMD Services Ltd will ensure that all data received from TGDOC Member Registries is handled, processed, stored and transferred only in accordance with the terms of this Charter and in line with the General Data Protection Regulations (GDPR 2016/EU) and will abide by this and any other relevant laws and regulations relating to EU law on data protection and privacy in the European Union and the European Economic Area.
- b. TGDOC Member Registries should ensure that the data they hold on the patients within their individual registries is handled, processed, stored and transferred in line with the relevant data protection laws pertaining to the country and jurisdiction that they fall within, whether inside or outside of the European Union and the European Economic Area.
- c. TREAT-NMD Services Ltd., TGDOC, and the TGDOC Member Registries should abide by all relevant ethical guidelines in relation to the data, the origin of the data (including the data source) and will, as required by local policy, consult and obtain approval from local ethics committees and ethics boards, for the collection storage, use and transfer of such data, for the purpose in which it was intended and with the appropriate consent of patients.
- d. To the extent applicable to a party's activities under this Charter, ethical guidelines to adhere to will cover, but will not be limited to:
  - Biomedical Research
  - Human Genetic Data

- Bioethics and Human Rights
  - Data Protection
- e. TGDOC Member Registries should also abide by applicable local and universally recognized standards where data is collected, transferred and used for academic research, scientific study and any resulting publications.
- f. It is inherent within this Charter that there is an understanding that all reasonable measures will be taken to ensure that the data transferred and shared, will only be used for the purpose of which it was intended and will not be misused, misrepresented or sold on to additional parties beyond the scope of the original agreements.
- g. Unless otherwise specified herein, the terms “party” and “member” mean an individual or entity that is specified as being subject to the terms of the Charter, but shall not suggest any other legal or contractual relationship to each other. The term “third party” shall mean any individual or entity who is not subject to the terms of this Charter. None of the parties makes any representations or extends any warranties of any kind, either express or implied, with respect to the data provided hereunder. The parties are independent contractors acting on their own behalf, and nothing contained in the Charter places the parties in the relationship of partners, principals and agents, or joint venturers. No party has any right, power or authority to bind or obligate any other party, nor shall any party hold itself out as having such right, power or authority.

## Revision history

Revised draft by Hanns Lochmüller (July 22nd, 2007), revised by Stephen Lynn (July 23rd, 2007), sent to TREAT-NMD partners and national registries for consultation (August-October 2007), legal comments by Christopher Roy-Toole (August-October 2007). Revised version by Hanns Lochmüller (October 19th, 2007), revised by Stephen Lynn (October 23rd, 2007).

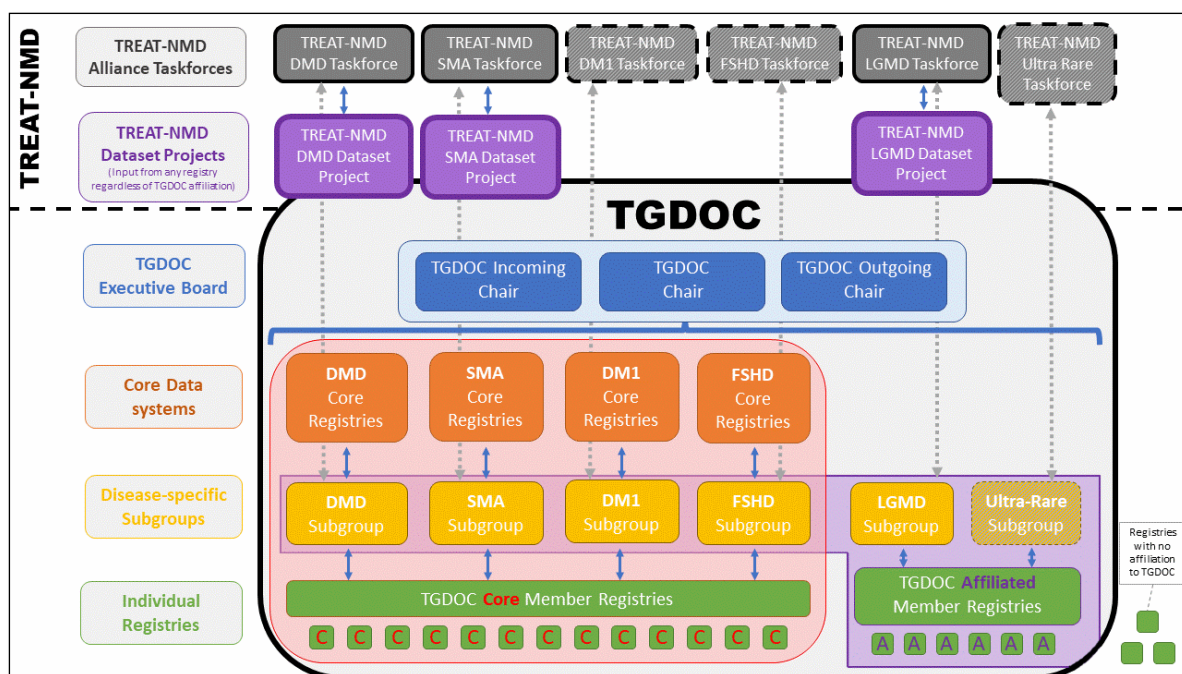
Revised version by Serge Braun (December 26th, 2007), revised by Christophe Bérout (December 26th, 2007). Final version by Hanns Lochmüller (December 29th, 2007) and reviewed by Stephen Lynn (January 6th, 2008). Charter approved by the TREAT-NMD Governing Board (January 15th, 2008). Discussed at the TREAT-NMD Oversight Committee Meeting in Geneva (November 12th, 2011), revised accordingly by Karen Rafferty and Hanns Lochmüller (November 25th, 2011). Charter approved by the TGDOC (March 9th, 2012).

Charter revised by Karen Rafferty (October 26th, 2012). Charter approved by the TGDOC (November 14th, 2012). Discussed at the TREAT-NMD Oversight Committee meeting in Newcastle (October 31st, 2013), revised accordingly by Agata Robertson and Jan Verschuuren (April 1st, 2014). Charter approved by TGDOC (September 20th, 2016).

Charter revised by TGDOC Executive Board (July 2020). *Revisions approved by TGDOC Members (August 2020) Discussed at TREAT-NMD Executive Committee Meeting (18 Sept 2020) Charter approved by TGDOC and TREAT-NMD Executive Committee (25 Sept 2020). Minor revision Oct 2021 approved by TGDOC Chairs (13 Oct 2021)*

## Appendixes

### Appendix a – Example of current TGDOC Structure and relationship with Core and Affiliated Member Registries



### Appendix b – TGDOC Membership criteria

Affiliation Criteria	TGDOC		Non-TGDOC
	Core Member Registry	Affiliated Member Registry	Unaffiliated Registry
Allow registry info on TREAT-NMD website	Essential	Essential	Strongly Preferred
Abide by Charters & data protection regulations	Essential	Essential	Strongly Preferred
Must complete annual Registries Review Survey	Essential	Essential	Strongly Preferred
Collect <b>all</b> mandatory items in Core Dataset(s), and HE if possible	Essential	Strongly Preferred	Strongly Preferred
Participate in dataset revision process	Essential	Strongly Preferred	Strongly Preferred
Participate in disease subgroups	Essential	Strongly Preferred	n/a

Attend TGDOC Annual Curators' meeting	Essential	Strongly Preferred	n/a
Engage with TGDOC Publications Committee	Essential	Essential	n/a
CDA in place and DoI submitted	Essential	Strongly Preferred	n/a
Participate in Surveys / Non-Core dataset Queries	Strongly Preferred	Strongly Preferred	n/a
Participate in Enquiries	Essential	<i>Only relevant for Core Members</i>	<i>Only relevant for Core Members</i>
Abide by Voting SOP	Essential		

