TREAT-NMD SMA Core Dataset Appendix

This appendix corresponds with **Version 2** of the SMA Core Dataset

11 November 2020

Table of Contents

Introduction

This appendix has been prepared by the TREAT-NMD SMA Dataset project team on behalf of the TGDOC (TREAT-NMD Global Database Oversight Committee), as a guidance document to support TGDOC Member Registries with the collection of the TREAT-NMD SMA Core Dataset.

Any comments or questions about the dataset, or anticipated training or support requirements should be reported to joanne.bullivant@newcastle.ac.uk (Project Manager) or joanna.das@newcastle.ac.uk (Project Coordinator).

Advised additional data collection (not part of core dataset)

The following data will never be requested nor accepted by TREAT-NMD for central submission, and as such do not form part of the SMA Core Dataset. However, it is recommended that registries consider collecting the following information (where relevant) for their participants, for internal use only, to support local registry operations and communication with registry participants.

Identifiable data

First name / given name
Last name / surname / family name
Current address and zip/post code
Current active email address
Telephone number
Contact preferences

Local registry ID

As best practise, each participant should be assigned upon enrolment a local registry ID (identifier) by the registry (or the IT platform), to assist the registry with safe data management. This ID would be for local use only. To conform with GDPR guidance, this ID must not contain any potentially identifiable information such as initials, date of birth or medical record number. For more information about what constitutes personal or identifiable data, please refer to GDPR guidance (https://gdpr.eu/eu-gdpr-personal-data/) and/or the 18 HIPAA identifiers (https://www.hipaajournal.com/considered-phi-hipaa/)

Consent and ethics

It is the responsibility of each individual registry to ensure that valid, informed legal consent is in place before sharing any individual's data with TREAT-NMD. This should be checked and confirmed before each data submission, in case of consent withdrawal or a change in consent requirements (e.g. an individual reaching the legal age of consent in their country).

It is also the responsibility of each individual registry to ensure that they have obtained appropriate ethical approval from any relevant committees, before participating in the TGDOC Global Registry and/or sharing any data with TREAT-NMD.

Suggested wording

The wording in this section is provided to help registries describe their participation in the TREAT-NMD Global SMA Registry, and the implications for data usage. It may be useful for ethical applications, patient information materials, consent forms, and other similar documents. Please note, registries should ensure that the contents are adapted to suit their registry set-up, local ethical/consent requirements, and their patient cohorts.

Patient Information sheets

[Registry name] is part of an international network called the TREAT-NMD Global SMA Registry, which collects information from independent SMA registries across the world, including [registry name].

Being part of the Global SMA Registry ensures that patients can be contacted by their local registry team if their profile fits a clinical trial's eligibility criteria. In addition; third parties such as researchers, pharmaceutical companies and regulators can request de-identified global data reports to answer important research questions about SMA. The data provided in response to these research questions can help to monitor how well different treatments or interventions are working, and support other activities to improve patients' quality of life, such as the assessment of standards of care in different countries.

We will not share your data with the TREAT-NMD Global SMA Registry unless you give us explicit permission. Any data we do share will be de-identified and we will never transfer any of your personal identifiable details; if shared, your record will only be identifiable by an anonymous code. Researchers using the TREAT-NMD Global SMA Registry therefore cannot identify you personally from the information they have.

TREAT-NMD will manage all data shared with the Global Registry in compliance with relevant data protection regulations, including GDPR. When a third party requests a data report from the TREAT-NMD Global Registry, an independent governing committee votes on whether it is an appropriate use of the data. If approved, the Curator of each registry is asked to securely send their de-identified data to the TREAT-NMD Global Registry, and it is then combined with data from other registries and formed into a report. Sometimes this request may be for individual (but always de-identified) patient-level data, and sometimes it will be for aggregated total patient numbers against certain criteria. Sometimes the request may come from academic or clinical researchers, and sometimes it may come from industry (pharmaceutical or biotechnology companies).

Patient consent forms

Please note this does not represent a complete consent form – these suggestions only cover consent to include an individual's data in a data submission for a TREAT-NMD Global SMA Registry enquiry, and should be added into your existing/main consent forms as appropriate. *[1]

Preferably the participant should initial each individual statement to show clear, explicit and informed consent. As a minimum, each statement should at least have a tick-box to indicate that it has been read and understood (rather than just a single signature at the end of the form)

☐ *The TREAT-NMD Global SMA Registry has been explained to me and I have had the opportunity to ask questions and have them answered.*

I give permission for my data to be shared with the TREAT-NMD Global SMA Registry, for the purposes described in [Patient Information Sheet name and version number], in the following way (choose only 1):

☐ ***All levels*** *(de-identified and included at individual level* ***and*** *included in aggregated numbers)*
☐ *Included in* ***aggregated numbers*** *only*
☐ *No data to be shared with the TREAT-NMD Global SMA Registry*

(If permission is given for any level of sharing:)

Once my data has been shared with the TREAT-NMD Global SMA Registry, I give my permission for it to be used for (choose only 1):

☐ *All approved data enquiries*
☐ *Academic or clinical enquiries only (no industry enquiries)*

*[1] If your registry chooses to use the TREAT-NMD Universal Registry Platform (URP) to collect and store data before submitting to TREAT-NMD, the consent forms provided in the system will cover this.

Protocols / ethical applications

[Registry name] is part of an international network called the TREAT-NMD Global SMA Registry, which collects information from independent SMA registries across the world, including [registry name].
The Global SMA Registry can receive data enquiries from third parties (industry or academic). [Registry name] will only share de-identified data with relevant consent in place with the TREAT-NMD Global SMA Registry.

Third parties wishing to enquire into the data in the TREAT-NMD Global Registry must first have the approval of both the TREAT-NMD Global Database Oversight Committee (TGDOC) and the [registry name] Principle Investigator/Steering Committee/ Manager [delete/amend as needed according to registry governance]. If approval is granted, TREAT-NMD requests the relevant data from the registries in the network, and provides the third party with a report containing aggregated data.

Publications

TREAT-NMD and the TGDOC acknowledge that a great deal of hard work, resource and expertise goes into the collection of high quality patient data by its affiliated registries, and we are committed to ensuring that contributions towards the TREAT-NMD Global SMA Registry are appropriately acknowledged wherever and whenever relevant. To this end, TGDOC have formed a Publications Committee who have a priority task to develop and have ratified a TREAT-NMD Global Registries Publications Policy.

If you would like to find out more or get involved in the Publications Committee, please contact the Committee Chair, Dr Rasha El Sherif (dr.rashaelsherif@gmail.com).

Stakeholder feedback and dataset revisions

A formal SMA Dataset Revision Process has been developed to reflect TREAT-NMD's commitment to:

- ✓ ensure the core SMA dataset remains appropriate, feasible and relevant
- ✓ support collaboration and harmonisation with other data collection initiatives
- ✓ remain responsive to the needs of and consensus within the SMA community

- ✓ acknowledge and manage the burden placed on registries and their participants with each change to the dataset.

Any formal dataset review will involve stakeholder consultation. In addition to the periodic formal reviews, feedback or discussion on the SMA core dataset is welcome from stakeholders at any time.

More information on the revision process and how to provide feedback is available on the SMA Dataset webpage.

Contacts & Acknowledgements

Dataset Project Team:
Project Manager: Jo Bullivant (joanne.bullivant@newcastle.ac.uk)
Project Coordinator: Joanna Das (joanna.das@newcastle.ac.uk)

TGDOC Chairs:
Chair: Anna Ambrosini (AAmbrosini@telethon.it)
Chair-Elect: Michela Guglieri (michaela.guglieri@newcastle.ac.uk)
Outgoing Chair: Craig Campbell (Craig.Campbell@lhsc.on.ca)

TGDOC SMA Sub-Group Leads:
Victoria Hodgkinson (vhodgkin@ucalgary.ca)
Miriam Rodrigues (mrodrigues@adhb.govt.nz)

Expert Consultants:
Anna Mayhew (Consultant Research Physiotherapist)
Marcel Heidemann (IT and Dataset Modelling Consultant)

Acknowledgements:
Financial support for this work was provided by Biogen.