TREAT-NMD Spinal Muscular Atrophy (SMA) Core Dataset

Dataset Revision Process

Foreword
This document describes the Revision Process for the core dataset for SMA Registries in the TREAT-NMD network. Feedback on this process or document is welcome and can be submitted by sending an email (or an annotated version of the document) to the Project Coordinator Joanna Das: joanna.das@newcastle.ac.uk.

Current versions of all documents are available on the project web page.

Introduction
The TREAT-NMD Global SMA Registry is one of several notable data collection initiatives in operation for Spinal Muscular Atrophy across the world. The model is based on participating registries collecting a common core dataset into which enquiries can be made. The core dataset for SMA registries was expanded in 2017, and following a pilot study the new core dataset was confirmed in September 2018.

Considerable work went into ensuring the data collected by the TREAT-NMD registries, through the first iteration of the expanded core dataset, will be comparable with data collected by other initiatives. However, many of these initiatives are still in development or will also be making periodic revisions to their datasets. In addition to this, new therapies are approaching the market and global consensus on the most appropriate and feasible data to collect will continue to evolve over the coming years.

This Dataset Revision Process (hereafter the Revision Process) has been developed to reflect TREAT-NMD’s commitment to ensuring its core SMA Dataset remains appropriate, feasible, collaborative, harmonised with other initiatives, and responsive to the needs of the whole SMA community.

This Revision Process will remain a working document for the duration of the project and will itself be subject to version control. This document describes:

1. The objectives of the Revision Process - what we want to accomplish.
2. The version control approach for the dataset and related documents.
3. The stakeholder groups to whom our activities are relevant.
4. Process and timelines - how we will accomplish our objectives.
5. The version control approach for this document.
6. Contact details for the SMA Dataset Project Team.

1. Objectives of the Revision Process:
   - Allow the dataset to be responsive to the needs of the SMA community
   - Manage and streamline the burden of dataset changes on Curators, Clinicians and Patients
   - Promote harmonisation across relevant initiatives globally
   - Drive and respond to global consensus on outcome measures
   - Respond to feedback from all stakeholders
   - Ensure and demonstrate feedback is being considered and acted upon where appropriate
   - Facilitate continuous improvement
2. Version control for the Dataset

To support the Revision Process, a semantic versioning approach¹ will be applied to the following dataset documents:

1. The Dataset
2. The Dataset Manual
3. The Dataset Overview

This approach is explained below and will allow small improvements and ‘fixes’ to the dataset to be made as needed, without waiting for the next one/two-yearly formal revision. It also enables each change to be categorised according to the impact it will have on ongoing data validity.

All previous versions of the dataset will be available on the project web page for reference. Each time a new version is released, a tracked changes copy of the document will be stored electronically and may be requested from the Project Team for reference if needed.

Semantic Versioning¹

A version number MAJOR.MINOR.PATCH will be assigned to each iteration of the dataset:

- A PATCH increment will be applied when a small change is made in a backwards compatible* way; for example, wording is clarified or a typo is corrected. When a PATCH increment is made it does not affect the MAJOR or MINOR increments, e.g. from v1.5.2 to v1.5.3.

- A MINOR increment will be applied when a more significant, but still backwards compatible* change is made; for example, adding a new response option to a data item. When a MINOR increment is made, the PATCH increment reverts to zero, e.g. from v1.5.2 to v1.6.0.

- A MAJOR increment will be applied when a change is made which is not backwards compatible*; for example, adding a new data item or splitting a response option into two sub-options. When a MAJOR increment is made, both the MINOR and PATCH increments revert to zero, e.g. from v1.5.2 to v2.0.0.

*Backwards compatibility¹

A change to a data item is backwards compatible if a data record conforming to the previous version would still be valid in the new version. For example:

<table>
<thead>
<tr>
<th>Version 1</th>
<th>Version 2</th>
</tr>
</thead>
<tbody>
<tr>
<td>Does the patient use a wheelchair?</td>
<td>Does the patient use a wheelchair?</td>
</tr>
<tr>
<td>1. Yes</td>
<td>1. Yes</td>
</tr>
<tr>
<td>2. No</td>
<td>2. No</td>
</tr>
<tr>
<td></td>
<td>3. Don’t know</td>
</tr>
</tbody>
</table>

☑ Version 2 of this data item is backwards compatible because a data value collected for v1 (e.g. ‘Yes’) would still be a valid response in v2.
A change to a data item is not backwards compatible if a data record conforming to the previous version would no longer be valid in the new version. For example:

Table 2: Example of a backwards incompatible change (for illustration purposes only)

<table>
<thead>
<tr>
<th>Data Item Number</th>
<th>Details of Change</th>
<th>Type of Change</th>
<th>Backwards compatible?</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Does the patient use a wheelchair?</td>
<td>Does the patient use a wheelchair?</td>
<td>MAJOR/MINOR/PATCH</td>
</tr>
<tr>
<td>1. Yes</td>
<td>1. Yes – more than 16 hours per day</td>
<td>2. Yes – less than 16 hours per day</td>
<td>Yes / No</td>
</tr>
<tr>
<td>2. No</td>
<td>3. No</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

※ Version 2 of this data item is backwards incompatible because a data value collected for v1 (e.g. ‘Yes’) would be an invalid response in v2.

Version Control Table

A Version Control Table on the first page of each new version will track and categorise all changes made within that iteration of the dataset. Information will be displayed as follows:

Table3: Version control table example

Dataset Feedback Log

In between each revision, a Dataset Feedback Log will record all feedback and suggestions received from all stakeholders, and the outcome of each when available (including an explanation if relevant). This will be made publically available as a live document via the project webpage.

1 https://semver.org/

3. Stakeholder Groups

This Revision Process is based on the principle of collaboration and inclusivity and therefore effective, transparent and sustained communication and engagement with both the SMA and wider neuromuscular and rare disease communities is required.

The SMA Dataset project team will identify and engage with the following stakeholder groups for the purposes of the Revision Process:

- SMA patients and their families
- SMA Patient Advocacy groups and organisations
- Pharmaceutical industry
- Regulators and Payers
- SMA Registry Curators and owners
- Healthcare professionals (clinicians, physiotherapists, geneticists)
- The wider TREAT-NMD and TGDOC community
- Other academic groups or registry initiatives
- Expert consultants as needed: for example FAIR data experts, registry software providers, bioinformaticians
4. Formal Revision Process and Timelines

This Revision Process allows stakeholders to provide feedback and suggestions continuously, as well as being proactively consulted at the start of each **Formal** Revision Process described below.

The SMA Dataset project team will make every effort to align the timings of each Formal Revision Process with those of other known initiatives, to maintain dataset harmonisation. Each Formal Revision Process will be initiated by the sharing of a short pre-recorded webinar to:

- Explain the context of the SMA Dataset.
- Recap the dataset itself.
- Provide guidance on the Revision Process.

The Formal Revision Process will then be undertaken in an open and transparent way; seeking feedback from relevant stakeholders at the stages outlined below.

**Figure 1: Formal Revision Process cycle**

**Persons Responsible:**

- Stakeholders
- Project Team
- TGDOC Chairs

**Timelines**

All revisions will be kept to a minimum wherever possible.

PATCH or MINOR (backwards compatible*) revisions may be made at any time if deemed necessary; although every effort will be made to minimise burden on registries and participants.

MAJOR revisions will be withheld until the next Formal Revision Process as described above. This is planned to take place annually for the first 2 years (Mar-Jun 2020 and Mar-Jun 2021), and every 2 years thereafter (may be subject to change, stakeholders would be notified).

TREAT-NMD SMA Dataset Revision Process_v1.0
28 January 2020
Each Formal Revision Process will take 15 weeks, as described below:

**Table 4: Formal Revision Process Timeline**

<table>
<thead>
<tr>
<th>Activity</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Short webinar to launch revision process</td>
</tr>
<tr>
<td>2. Circulate current dataset</td>
</tr>
<tr>
<td>3. Collate/analyse/review feedback</td>
</tr>
<tr>
<td>4. TGDOC Chairs review feedback analysis</td>
</tr>
<tr>
<td>5. Meet with TGDOC Chairs to agree responses to feedback</td>
</tr>
<tr>
<td>6. Prepare draft version for circulation</td>
</tr>
<tr>
<td>7. Circulate draft to stakeholders for feedback</td>
</tr>
<tr>
<td>8. Collate/analyse/review any further feedback</td>
</tr>
<tr>
<td>9. Prepare revised documents</td>
</tr>
<tr>
<td>10. Final review/approval by TGDOC Chairs</td>
</tr>
<tr>
<td>11. Publish new version of documents</td>
</tr>
<tr>
<td>12. Circulate to stakeholders and update website</td>
</tr>
</tbody>
</table>

For all revisions (MAJOR/MINOR/PATCH), registries will be asked to:

- Implement applicable revisions as soon as possible, but within a maximum of 6 months from the revision date.
- Notify the project team as soon as the revision has been implemented.

### 5. Version control for this document

This document itself will be subject to version control under the following naming convention:

- TREAT-NMD SMA Dataset Revision Process_v1.0
  - TREAT-NMD SMA Dataset Revision Process_draft_v1.1
  - TREAT-NMD SMA Dataset Revision Process_draft_v1.2
- TREAT-NMD SMA Dataset Revision Process_v2.0

**Minor Revisions:** for drafts. Each new draft version of the document will be reflected in an increase of the decimal number, e.g. v1.0 to draft_v1.1, draft_v1.2 and so on.

**Major Revisions:** will take effect when a new version of the dataset has been approved by the TGDOC Chairs and will be reflected in an increase in the whole number by 1, e.g. draft_v1.3 to final_v2.0.

Feedback received on this document or the Revision Process itself will be considered and implemented for subsequent revisions if appropriate, and the outcome communicated to the individual who provided the feedback.

### 6. Project Contacts

Any questions or feedback about this process or the SMA Dataset itself can be directed to:

- Joanna Das (Project Coordinator) at joanna.das@newcastle.ac.uk.
- Jo Bullivant (Project Manager) at joanne.bullivant@newcastle.ac.uk.