Core SMA Dataset for TREAT-NMD affiliated Registries

First Dataset Review (March to June 2020): 10 minute briefing

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Core SMA Dataset: high level view

Expanded Mandatory Items

- Enrolment & consent
- Genetic diagnosis
- SMN2 copies
- Scoliosis surgery
- FVC results if done
- Hospitalisations & co-morbidities
- PRO: Clinical/Total Global Impression
- Family history
- TGI according to clinician
- Screening programme & method of testing
- Demographics incl. PPRL fields
- Airway clearance / secretion mobilisation
- Electrophysiology & biomarkers taken (Y/N)
- Date & cause of death
- Clinical observations incl. contractures

Expanded Highly Encouraged Items

- DOB, Sex, Country
- SMA type & onset age
- Best & current motor function extended
- Medications & disease modifying therapies
- ≥ 1 validated motor outcome measure
- Therapeutic interventions
- Living status
- HCP details
- Wheelchair use
- Feeding tube use
- IV & NIV use
- Clinical trial participation
- Participation in other registries or NH studies
Global Registry Enquiries

- Enquiry received from 3rd party (e.g. industry, academia)
- TREAT-NMD requests Data
- National registry returns Data
- TGDOC Vote
- National SMA/DMD registry
- National SMA/DMD registry
- National SMA/DMD registry
- National registry returns Data
- Clinician Entered
- Or a combination
- Patient Entered

Global Registry

- Enquiry received from 3rd party (e.g. industry, academia)
Core SMA Dataset: the expansion

- TREAT-NMD expanded the core SMA dataset in September 2018; significantly increasing the number of data items and therefore the work required to collect it.

- Phased and long-term implementation of the dataset across the network.

- 4.5 year project in 2 phases:
  - 18 month Pilot Project (April 2017 to September 2018) – 12 registries
  - 3 year Implementation Project (May 2019 to May 2022) – remaining registries

- To support the expanded dataset:
  - Dataset manual (dictionary, wording for patient-facing registries, standardised text).
  - Information and guidance on outcome measures
  - Financial bursaries and 3 x annual workshops for Curators.

- Dataset Revision Process
Why do we need to make revisions?

- Fast-moving field; accommodate new treatment options or advances in standards of care
- Gather ongoing stakeholder feedback
- Harmonise with other initiatives
- Support continuous improvement

Why do we need a formal process?

- Manage burden on registries
- Protect longitudinal validity of dataset wherever possible
- Inform all stakeholders of any compatibility issues
- Clarity for stakeholders about when and how they can give feedback (and to proactively seek this feedback)
- Close the feedback loop
About the Formal Revision Process

• Major revisions will be kept to a minimum wherever possible.

• Urgent but minor revisions (which do not affect the longitudinal validity of the data) may be made at any time, if deemed necessary by the TGDOC Chairs.

• Major revisions (which may affect the longitudinal validity of the data) will be withheld until the next Formal Revision Process. This is planned annually for the first 2 years (Mar-Jun 2020 and Mar-Jun 2021) and every 2 years thereafter.

• A Version Control Table on the first page of each new version will track and categorise all changes made.

• A Dataset Feedback Log on the project webpage will record all feedback received from all stakeholders, and the outcome of each as soon as available (including explanation if needed).
About the Formal Revision Process

1. Launch Formal Revision Process
2. TGDOC Chairs Review
3. TGDOC Chairs Review
4. Collate and apply feedback
5. Update new version
6. 2nd Stakeholder Review
7. TGDOC Chairs Review
8. Confirm & disseminate new version
9. Gather continuous feedback
10. Refresh Stakeholder Map
11. 1st Stakeholder Review
12. TGDOC Chairs Review
13. Stakeholders
14. Project team
15. TGDOC Chairs
# About the Formal Revision Process

<table>
<thead>
<tr>
<th>Activity</th>
<th>Week commencing:</th>
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<tbody>
<tr>
<td>1. Project team: launch stakeholder consultation</td>
<td>09-Mar 0</td>
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<tr>
<td>2. Stakeholders: review dataset v1</td>
<td>16-Mar 1</td>
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<td>3. Project team: analyse feedback</td>
<td>23-Mar 2</td>
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<td>4. TGDOC Chairs: review feedback analysis</td>
<td>30-Mar 3</td>
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<td>5. Project team: prepare dataset v2 draft and circulate</td>
<td>06-Apr 4</td>
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<td>6. Stakeholders: review dataset v2 draft</td>
<td>13-Apr 5</td>
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<td>7. Project team: analyse further feedback</td>
<td>20-Apr 6</td>
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<td>8. Project team: prepare dataset v2 final</td>
<td>27-Apr 7</td>
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<td>9. TGDOC Chairs: final review/approval of dataset v2</td>
<td>04-May 8</td>
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<td>10. Project team: prepare final dataset v2 documents</td>
<td>11-May 9</td>
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<td>11. Project team: update website and circulate dataset v2</td>
<td>18-May 10</td>
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<td>22-Jun 15</td>
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What will a new version mean for registries?

- If your registry *has not* yet implemented the 2018 expanded dataset:
  - Please disregard previous versions and work on applying the newest version.
  - If you are not already in contact with us about support available, please get in touch.

- If your registry *has already* implemented the 2018 expanded dataset:
  - Please apply all relevant changes in your data collection forms as soon as possible, but within a maximum of 6 months from the revision date.
  - Please notify the project team when you have done so.
What should you do now?

We are grateful for all comments and suggestions to make the dataset the best it can be. All documents are available on the project webpage*:

- Review the current (v1) dataset alongside the dataset manual
- Review other documents if you have an interest:
  - Suggested wording for patient-facing registries
  - The dataset revision process itself
- Review the feedback we have already received since Sept 2018
- Send any additional thoughts via the feedback template, by 27 March.

Before you go: Key points to remember

- Intended for **all** registries collecting data on SMA (clinician/patient/dual reported)
- However some items are only relevant for certain types of registry and are marked accordingly.

- Personal identifying information on patients will **never** be requested by a TREAT-NMD enquiry. This is included in the dataset as recommended best practise but would be for registries’ internal use only.

- **Mandatory items**: registries are required to include in their data collection forms if applicable – but could be either mandatory or conditional (dependent on response to previous question) fields.
- **Highly encouraged items**: registries are encouraged to include in their data collection forms if applicable, but this is optional. If included, registry decides if mandatory/optional field.
Thank you for your engagement!

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