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1. DISCLOSURE AND CONFLICT OF INTEREST

This workshop was part of the TREAT-NMD SMA Dataset Implementation Project supported by Biogen; a pharmaceutical company with an approved therapy (Spinraza) for spinal muscular atrophy (SMA). This report provides an overview of the discussions and recommendations made during the workshop; it does not necessarily represent the full perspectives of any individual attendees, Biogen, or TREAT-NMD.

This report has been prepared by Joanna Das (SMA Dataset Project Coordinator) and Jo Bullivan (SMA Dataset Project Manager) with review and input from the Chairs of the TREAT-NMD Global Database Oversight Committee (TGDOC).

TGDOC Chairs:
- Craig Campbell (Chair)
- Nathalie Goemans (outgoing Chair)
- Anna Ambrosini (incoming Chair)
2. EXECUTIVE SUMMARY

TREAT-NMD is an international neuromuscular network, which coordinates a global network of SMA registries who all collect a common core dataset. The TREAT-NMD SMA Dataset Project is funded by Biogen and led by Project Manager Jo Bullivant at the John Walton Muscular Dystrophy Research Centre, Newcastle University, United Kingdom.

A three year implementation project is supporting SMA registries in the TREAT-NMD network to collect an agreed expanded dataset for SMA patients. On Friday 13th December 2019 a workshop was held at Leiden University in the Netherlands to provide information, support and discussion for Registry Curators and other stakeholders on the implementation of the dataset to date. This workshop report provides a summary of the key discussion points, questions raised and agreed next steps.
3. ABOUT THE WORKSHOP

3a. Project context
The SMA core dataset allows central enquiries into the data within all registries in the network. This model has been in place for 10 years and the dataset was originally quite small (enquiries were predominantly for clinical trial feasibility studies and recruitment). However, with therapies now coming to market TREAT-NMD has expanded the core dataset with a view to providing real-world data from patient registries to support postmarketing surveillance.

SMA registries in the TREAT-NMD network are now being asked to increase their data collection activities in order to comply with the new dataset.

3b. Workshop aims and objectives
The target audience for this workshop was Curators of registries taking part in the SMA Dataset Implementation Project, although it was open to any other stakeholders with an interest in this work.

Workshop Objectives:
- Provide information and guidance to curators on implementation of the new dataset.
- Provide information about support available.
- Update on this project and related projects.
- Discuss progress and resolve issues.
- Share solutions and best practice between registries.

3c. Workshop agenda and structure
The workshop aimed to provide accurate and clear information and to ensure that all participants had opportunities to:
- voice their opinion and ask questions;
- participate in discussions and share their experience;
- learn from other registries facing similar challenges.

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Item</th>
<th>Who*</th>
</tr>
</thead>
<tbody>
<tr>
<td>08:00</td>
<td>Registration &amp; coffee</td>
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<td></td>
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<tr>
<td>08:30</td>
<td>1 Setting the scene</td>
<td>Welcome &amp; Introductions (5 mins)</td>
<td>CC</td>
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<tr>
<td></td>
<td></td>
<td>Project overview and context: Why we are doing this? (15 mins)</td>
<td>CC</td>
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<tr>
<td></td>
<td></td>
<td>Workshop scope and purpose (10 mins)</td>
<td>JB</td>
</tr>
<tr>
<td>09:00</td>
<td>2 The Universal Platform</td>
<td>Update on the Universal Platform (15 mins)</td>
<td>JB</td>
</tr>
<tr>
<td>09:15</td>
<td>3 The expanded dataset</td>
<td>The Expanded Dataset (30 mins)</td>
<td>JB</td>
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<tr>
<td></td>
<td></td>
<td>Clarity on motor measures &amp; PROMs (15 mins)</td>
<td>JB</td>
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<tr>
<td></td>
<td></td>
<td>Annual revision plan (15 mins)</td>
<td>JD</td>
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<tr>
<td>10:30</td>
<td>Coffee break</td>
<td></td>
<td></td>
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<tr>
<td>10:45</td>
<td>4 Dataset implementation:</td>
<td>Results of internal survey (30 mins)</td>
<td>MR</td>
</tr>
<tr>
<td></td>
<td>progress so far</td>
<td>Feedback from registries who have already implemented the dataset (40 mins)</td>
<td>Group 1</td>
</tr>
<tr>
<td>Time</td>
<td>Session</td>
<td>Details</td>
<td>Participants</td>
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<tr>
<td>12:00</td>
<td>Lunch</td>
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<tr>
<td>12:45</td>
<td>Support</td>
<td>Bursaries (10 mins)</td>
<td>JD, JB</td>
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<tr>
<td></td>
<td></td>
<td>Dataset Manual (1 hour, interactive session)</td>
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<tr>
<td>14:00</td>
<td>Q&amp;A / Trouble-shooting</td>
<td>Panel Q&amp;A with:</td>
<td>All</td>
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<td>TGDOC Chairs (Craig Campbell, Nathalie Goemans, Anna Ambrosini)</td>
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<td>SMA Subgroup Lead: Miriam Rodrigues</td>
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<td></td>
<td></td>
<td>Project Manager: Jo Bullivant</td>
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<tr>
<td></td>
<td></td>
<td>Group1*</td>
<td></td>
</tr>
<tr>
<td>14:45</td>
<td>What next?</td>
<td>Project Plan, milestones and deliverables</td>
<td>JB</td>
</tr>
<tr>
<td>14:55</td>
<td>Close of workshop</td>
<td></td>
<td>CC</td>
</tr>
</tbody>
</table>

Participants were reminded of the scope of the workshop does not include the Universal Registry Platform.
3d. Workshop participants

**Chairs / Organisers:**
1. Nathalie Goemans, TGDOC Chair outgoing
2. Craig Campbell, TGDOC Chair
3. Anna Ambrosini, TGDOC Chair Elect
4. Jo Bullivant, Project Manager
5. Joanna Das, Project Coordinator

**Registry Curators**
6. Nina Barisić (Croatia)
7. Ria Broekgaarden (Netherlands)
8. Marján Cosyns (Belgium)
9. Hanneke Deenen (Netherlands – non SMA)
10. Rasha El Sherif (Egypt)
11. Robin Forbes (Australia)
12. Olga Germanenko (Russia)
13. Nathalie Goemans (Belgium)
14. Jana Haberlová (Czech Republic)
15. Sahar Hassanain (Egypt)
16. Marcel Heidemann (Germany Munich)
17. Kristine Hovhannesyan (Armenia)
18. Marlène Jagut (Belgium)
19. Ayşe Karaduman (Turkey Ankara)
20. Veronika Karcagi (Hungary)
21. Michelle Kruinshka (Switzerland)
22. Anna Lusakowska (Poland)
23. Vitaliy Matyushenko (Ukraine)
24. Said M’Dahoma (Canada)
25. Beatrix Palmafý (Hungary)
26. Miriam Rodrigues (New Zealand)
27. Sureshkumar Sankaran (India)
28. Nana Nino Tatishvili (Georgia)
29. Simone Thiele (Germany, Munich)
30. Sonia Segouie (Spain)
31. Ludo van der Pol (Netherlands)

**TREAT-NMD Secretariat**
32. Nicole O’Connor, TREAT-NMD SMA Education Coordinator
33. Cathy Turner, DMD Programme Coordinator
34. Helen Walker, TREAT-NMD Reg/Post Marketing Coordinator
35. Becca Leary, Connect4Children (C4C) Project Manager

**Biogen**
36. Aisha Rashid

**Apologies**
37. Damjan Osredkar (Slovenia)
38. Aya Ahmed Hanafy (Egypt)
39. Ipek Gürbüz (Turkey)
40. Lindsay Murphy (UK/Ireland)
41. Andrew Corbett (Biogen)
42. Sue Hall (Biogen)
43. Vedrana Milic Rasic (Serbia)
44. Huub van Rijswijck (SMA Europe)

**Key**
**Bold:** Speakers
4. WORKSHOP DISCUSSIONS (all slides available)

4a. Session 1: Setting the scene
Craig Campbell welcomed everyone and led a brief round of introductions. He summarised the context and drivers for the expansion of the SMA Dataset, which was initiated in response to the development of novel therapies for SMA. Following a successful pilot project with 12 registries, roll-out (implementation) of the dataset to other SMA registries commenced in May 2019.

Jo Bullivant presented the scope and purpose of the workshop and reminded participants that their feedback on the dataset itself was very important so would be recorded throughout the day as it came up. However, this was not the primary purpose so there would be limited time to discuss.

4b. Session 2: The Universal Platform
Not in scope for this workshop, although related to these discussions, is the Universal Registries Platform (URP). The URP is a dual-purpose web based IT solution, under development by TREAT-NMD. Firstly, it will help TREAT-NMD collect data in a more secure and efficient manner. Secondly, it will provide registries with an IT platform to host, collect a dataset to other SMA registries commenced in May 2019.

Jo Bullivant recapped how the URP will work and that registries will be able to either use it as their own registry platform, or continue to use their own platform and upload data into it. Key points included:

- The central URP server will likely be located in Germany. Some participants asked if there were servers available in other locations. **ACTION POINT: Jo Bullivant to investigate this further.**
- Registries will maintain independent ownership and governance of their data unless they choose to release it into the central database for the purposes of an enquiry, at which point the centralised data would come under TREAT-NMD governance. Appropriate consent must be in place for each patient before this can happen.
- Currently only aggregate data is requested/received from registries. Future PMS activities may require more stringent quality checks and agile analysis, which may lead TREAT-NMD to request anonymised but individual-level data from registries. This would be a significant change from previous processes, which needs careful thought and consultation. Submission of individual-level data would never be universally mandated by TREAT-NMD, and the option to submit aggregate data will always be retained.
- Development of the platform is paused due to removal of funding, however the SMA Module is estimated 70% complete and TREAT-NMD is trying to secure alternative funding. If funding is secured the development of both the DMD and SMA modules is planned for 2020, with piloting from Q3 2020.

**Questions:**

**Q:** In year one, will registries be able to send their data electronically?
TREAT-NMD confirmed that is the plan

**Q:** What is the relationship between the EURO-NMD dataset project and this project?
Jo Bullivant is in contact with François Lamy and understands that EURO-NMD project is still in the very early stages (funding not yet approved). Communications are open between the two groups and TREAT-NMD aims to make sure we are clear about how the projects sit together, how they differ, and how they can collaborate/learn from each other.

**Q:** Post Brexit how can we ensure GDPR rules?
This is an international project and UK Data Protection regulations are among the strongest in the world. TREAT-NMD will of course ensure that all activities are GDPR compliant and Brexit should not be an issue for this project.

**Q:** What is the position from Roche and AveXis?
There have been ongoing discussions with both, and both have shown interest in making use of the expanded dataset. AveXis have started a registry in the US and feel that the model will work well elsewhere, however the feeling is that their approach would not work in the EU. There have been calls to discuss how we could work and share data etc. with each other.
Q: What about feedback to patients about how their data is being used?

One of the outputs of the TGDOC Publications Committee is to track the impact and reach of publications using the registries, which will become an annual process. A newsletter item will be included in the next TGDOC newsletter to encourage registries to pass this information (as well as other feedback) to their participants.

4c. Session 3: The expanded dataset

The following documents (all version 1) are available:

- **SMA Dataset Overview** (high level overview of data items only)
- **SMA Dataset** (data items, response options, baseline/follow-up)
- **SMA Dataset Manual** (definitions, guidance on collection and submission, standardised text)
- **SMA Dataset Patient-reported Wording** (open for consultation)

There will be an additional two documents to support the SMA Dataset collections, these are:

- **Dataset Revision Process** (outlining versioning approach and mechanisms for stakeholder feedback)
- **Outcome Measure Toolkit** (guidance on appropriate outcome measures and signposting to relevant resources)

Key points about the dataset:

- **There is no typical timescale** on how long it will take to implement the expanded dataset. Curators are advised to get in touch with the project team for support if needed.
- **Section 2: Demographics contains the identifiable data** of the patient, this is good practice to collect locally, however curators will never be asked to release this to TREAT-NMD.
- The data submission process for global enquiries has not changed; the only difference is the dataset has been expanded. This process may change in the future.
- **Mandatory items** must be included in registry questionnaires/case report forms; however, some are subject to conditional logic.
- **Highly Encouraged items** are optional; all registries are encouraged to collect as best practice.
- Individual registries can collect additional items if they wish.

Clarity on motor measures & POMs

During the pilot project, a lack of global consensus on the most appropriate Outcome Measures (OM) for SMA patients became apparent. Different countries, registries and individuals have different opinions and preferences, and TREAT-NMD felt it was neither appropriate nor feasible to mandate a particular set of OMs for all registries.

However, collection of reliable outcome measures is critical to monitor both the natural history of SMA and the ongoing efficacy of treatments. So the agreed approach was to mandate that clinician-reported registries should collect a minimum of one validated OM for each participant (regardless of treatment status).

Key points raised by attendees included:

- Anna Ambrosini advised different companies might want to use specific measures over others. Need to find a way to address this.
- Becca Leary advised when the platform is used for PMS, the OM for a particular study will dictate which OM the participating registries must report.
- Sahar Hassanein suggested it might be difficult for data to be compared if everyone is using different OMs.

Although reluctant to mandate any particular OMs, it is important for TREAT-NDM to give guidance to registries who need it. The OM toolkit (in development) will help give more guidance in the future. Aisha Rashid commented that as long as the scale is validated, comparisons can be made, and that Biogen is not worried about the data being unusable. OMs in the TREAT-NMD dataset are currently split by early/late onset. The OM toolkit will be available online and will provide further details on each OM, such as suitable cohorts, conditions for use, and training resources.
Dataset revision process
Joanna Das introduced a proposed dataset revision process to ensure the SMA Dataset remains up-to-date, fit for purpose, in line with other initiatives and can evolve over time as the needs of the community changes. Discussion on the proposed process was encouraged during this session, and the document will also be circulated for feedback.

The process allows TREAT-NMD to both accept continuous feedback as it comes in, and to periodically seek feedback from all stakeholder groups. It aims to manage the burden of changes on curators, clinicians and patients while allowing appropriate updates in response to feedback and changing requirements. The first formal revision is planned for March-June 2020 and types of feedback received so far include:

- changes to wording
- additional response options
- consistency suggestions
- formatting changes

**Figure 2: The Proposed Revision Process Cycle**

**Persons Responsible:**
- Stakeholders
- Project Team
- TGDOC Chairs

**Questions:**
Q: What about data items that take time to implement or changes that need to go to ethics.
We can see that would be an issue and can consider a biennial (every 2 years) revision, however during the first couple of years we feel it is important to do this annually due to rapid changes in the SMA landscape. We also need to consider the needs of companies.

The group discussion revealed a strong consensus that annual revisions would be too frequent, however there was an acknowledgement that this might be necessary for the first few years while there are so many new therapies coming to market.
4d. Session 4: Dataset implementation: progress so far

Miriam Rodrigues presented a poster (Appendix 1) which she had presented at the TREAT-NMD Conference earlier that week titled ‘Collaborative data collection by TREAT-NMD Registries to support post-marketing surveillance in Spinal Muscular Atrophy’.

**Question:**
What is the reason behind insufficient human resourcing for registries?
TREAT-NMD’s understanding is that it is down to funding resources available to pay for time. Many curators are performing their curation duties in addition to existing fulltime jobs.

Feedback from registries who have already implemented the dataset

*Jana Haberlová, Czech Republic and Slovak Registries.*

This clinician-reported registry is for Czech and Slovak SMA patients. Most participants are paediatric with SMA Type 1, 2 or 3. It became clear that more data was needed so from April 2019, the registry has been collecting the expanded dataset. The registry has secured a 5 year grant to pay staff to upload the completed forms done by clinicians.

The main positive of being involved in the project is the data is now of a high quality. Only validated data is paid for by the grant (70% of data entered is validated). There is currently a push to try to get adult access to Spinraza which is hoped to happen next year.

Conclusion: now is the time to collect data on not only treated patients but also untreated.

*Said M’Dahoma, Canada*

This clinician-reported registry currently has 4400 NMD patients across 10 provinces and covers ALS, DMD, DM, LGMD and SMA, collected through 31 clinics. When implementing the SMA expanded dataset, every clinic had to update ethical approval before they could start. It also required support time for staff to collect the data, and physiotherapist capacity. All clinics with ethical approval are collecting data using the Redcap platform. Approximately half of patients have longitudinal data.

Said provided an overview of the challenges and successes experienced in implementing the expanded dataset by the Canadian Registry and outlined some of the ongoing and future SMA projects planned.

*Sureshkumar Sankeran, India*

This clinician-reported registry has close to 280 children (Comprising of all types of Muscle disease) registered. However, it is estimated there are around 79800 to 133000 people with SMA in India (Based in the international Statistics of SMA Incidence). Suresh outlined the main outcome measures used in clinics in India, the equipment required, and the issues faced.

He also summarised the registry protocol and update schedule. The Indian Council for Medical Research has recently launched the National Registry for NMD which will have four main centres with 49 nodal centres all participating, giving a much wider coverage across India.

*Marcel Heidemann, Munich, Germany*

This patient-reported registry was launched in 2008 and has approximately 900 SMA patients. Registry is not yet collecting the expanded dataset but the platform update is almost complete. The registry uses a web-based system. Key challenges include managing the patient burden, clarity of wording to avoid false data, and migration/validity of historic data.

4e. Session 5: Support

**Bursaries**

There is a significant impact on time/resource needed by registries to collect this expanded dataset. Financial bursaries are available for registries not receiving direct funding from Biogen for their registry or related data.
collection activities. The bursary can be used to offset any costs related to this work, such as updating IT platforms or the cost of staff time. Joanna Das summarised the process and timescales for claiming the bursaries.

**Questions:**

**Q: What kind of invoice is needed?**

Jo Bullivant confirmed the project team can provide a template if needed.

**Q: What can we spend it on?**

It is up to the individual registry to use as they see fit, however it is important that we know how it is spent.

**Dataset Manual**

Jo Bullivant provided an overview of the sections included in the dataset manual. The room was divided into groups and each group took two sections of the manual to review and provide feedback. Comments were recorded on flipchart paper (Appendix 2) and have been recorded for use during the first dataset revision (Mar-Jun 2020).

**4f. Session 6: Panel Q&A/ Trouble-Shooting**

**Q: to Jana Haberlová: Which data is not validated and why:**

Only expanded the dataset in April and only collecting since 1st September 2019. In the process of going back to clinicians to get more information to validate.

**Q: to Jana Haberlová: What is meant by validated?**

Data is checked/searched by a computer to make sure no data is missing, completed fully.

**Q: to Jana Haberlová: what is your plan to expand your registry to include adults?**

No plan yet, at the moment, in the Czech Republic they can only treat paediatric patients but expect adults to be included at some point next year. JH feels positive that centre will use the registry to give data for payers/regulated.

**Q: to All: The distribution of the types of SMA, why are we missing some, e.g. SMA4 and what can be done to capture this better?**

The majority of patients are paediatric, one way to increase adult data capture is to offer more treatments. Adults will be more willing to attend clinics, which means data may be easier to collect. Craig Campbell believes Canada has one of the best adult coverages possibly covered through the ALS clinics. It is difficult to establish age of onset for SMA4.

Miriam Rodrigues: New Zealand has set up an SMA clinic for adults with all adults in the country invited to attend.

**Q: to Miriam Rodrigues: How do you support patients to attend this clinic?**

Offers financial support for travel.

**Q: to All: When updating data how do you not lose the existing data?**

For previous visits the data is still available.

**Q: Do you collect only 5QSMA data?**

In Belgium, it is normal to collect 5QSMA data on the NMD registry. May be worth flagging in the dataset? For non 5QSMA patients who are still allowed to join, the purpose of the registry should be made clear and expectations clarified for what they can/can’t expect.
4g. Session 7: What Next?

The deliverables for the project are:

Year 1
1. Dataset manual
2. Financial bursaries for Y1 registries
3. Establish Annual Dataset Revision Process
4. Year 1 workshop for Curators
5. Outcome Measure Toolkit
6. Year 1 Project Report

Year 2
7. Financial bursaries for Y2 registries
8. Year 2 workshop for Curators
9. Year 2 Project Report

Year 3
10. Financial bursaries for Y3 registries
11. Year 3 workshop for Curators
12. Final Project Report

Jo Bullivant clarified that no data will be shared with Biogen as part of this project; to access data they would need to submit a TGDOC enquiry in the same way as any other third party.

5. PRESENTATIONS

We would like to thank everyone who presented at this workshop. A PDF document containing all the slides is available on the project webpage on the TREAT-NMD website.

6. FEEDBACK AND NEXT STEPS

6a. Feedback
Following the workshop, a short feedback survey (Appendix 3) was circulated to attendees, asking the following questions:

- What did you find useful at the SMA Dataset Workshop on the 13th December 2019?
- What could we improve to make the next SMA Dataset Workshop even better?
- Any other comments

To date there have been eight responses to the survey and further feedback has been received by email. Feedback has been positive as shown below:

- “Every aspect of the workshop was useful in some way.”
- “Very practical approach, clear explanations and instructions.”
- Participants thought the workshop was “well organised and enjoyable.”

Attendees found the presentations from the four registries on their experience of implementing the dataset to be particularly beneficial.

Areas for improvement included:

- Length of meeting: “another day would help have better communications, more detailed discussions and questions”
- Venue: “easy access to outside and fresh air would be good”
- Meeting Prep: “having better overview of material for meeting would help people preparer and be able to offer solutions.”

Other Feedback

- It would be great if the whole group could now share how they are implementing or have implemented the new dataset
- Consider using semantic versioning for the dataset to better identify type and impact of change.
6b. Next steps
Registries were called to continue their excellent work and forge ahead with implementing the expanded dataset. Curators were reminded that the project team and the TGDOC Chairs are here to support in whatever way possible, and that feedback or questions are always welcome. The following next steps were identified:

- Project team to record all feedback gathered during and after the meeting, and consider/implement during relevant document reviews.
- Project team to amend the revision process to reflect the discussions about revision frequency.
- Jo Bullivant to circulate all slides and documents from the workshop; including a list of all participants with email addresses.
- Participants to review relevant documents and send feedback before March 2020.
- The first formal revision process will start in March 2020.
Appendix 1: M. Rodrigues & V. Hodgkinson Poster ‘Collaborative data collection by TREAT-NMD Registries to support post-marketing surveillance in Spinal Muscular Atrophy’.

Background

The TREAT-NMD Global Networks of Sema Registers (n=127) collate a common core dataset and are governed by the TREAT-NMD Global Data Governance Committee (TGDC). Research into and identity can impact on untapped and aggregated data through the registries, offering a single point of access to the diverse and fragmented datasets that TREAT-NMD has identified as a need.

The TREAT-NMD SMA core dataset containing 21 data items was established in 2018 for clinical trials and recruitment. In the current SMA landscape there is a need for more widespread longitudinal data collection to support future research and post-marketing surveillance (PMS) requirements for emerging therapies. With this in mind TREAT-NMD reviewed and expanded the core dataset for their SMA Registry.

Workshops were held in May 2019 and June 2019 evening clinicians, healthcare providers, patient representatives, industry representatives, and other stakeholders from across the world. These led to the development of an expanded SMA dataset for TREAT-NMD registries, containing 398 data items.

Methods

Based on an initial pilot study of 32 TREAT-NMD Sema registers (both clinical and patient reported), a mapping and mapping framework was across all Sema registries in order to implement the expanded data set into the existing TREAT-NMD Sema registries.

The expanded dataset framework is laid out in a single data sheet. The data is structured as a single table with columns corresponding to the data items. The framework provides a clear and consistent format for the data. The data sheet allows for easy data collection and analysis.

The expanded dataset framework is laid out in a single data sheet. The data is structured as a single table with columns corresponding to the data items. The framework provides a clear and consistent format for the data. The data sheet allows for easy data collection and analysis.

Results

The expanded dataset highlights the power of the global registries, with substantial numbers of registered patients across the globe. To date, registries in 60 countries with specific data items including morbidity and mortality data. The expanded dataset highlights the power of the global registries, with substantial numbers of registered patients across the globe. To date, registries in 60 countries with specific data items including morbidity and mortality data.
Appendix 2: Dataset Manual Feedback Flip Chart images:
Sections 1-7:

1. **DATE FORMAT**
   ISO 8601

1.03 **CONSENT - SPECIFY AGE/PLD?**

2.16 **Specify inc. format.**

2.11 **Postcode - current res. address**

2.20 **Specify genetically related/biological**

4.02 Both CR+PR
   More than 1 test - dates of all
   **SMA ncopy - sometimes diff. date**

4.05 - **Minimum**

5.00 - **FIRST SYMPTOMS**

5.01 - **CURATOR VERIFIED - Type 0 in table**
   - **ADD PRESYMPTOMATIC**

6.03 'Other' should incl. more

6.01 **Cobb angle technique**

7.04 'Support' not assistance - consistency
Sections 8-12

8. OTHER DEVICES - Scooters etc.
9. Clammy - for feeding
10. Define part-time vent. use
    - Define cut-off & temp use, eg 1 weak what if only during hosp. stay
10.06 - Include examples + explain types
    - How/why started vent.

11.00 - May need multiple 'routines' - not spiroza
11.05 - Expand options
    - START/STOP = PR also
    - SIDE EFFECTS not SAEs?
    - AEs AS WELL AS SAEs
    - COMORB = a SIDE EFFECT of MEDS? WHICH ONES?
12. - Scoliosis surgery = planned hospital stay
12. - not patient-centric - not mandatory
12. - also could include PR in some 12.04 - 12.10
    - PATIENT VOICE

Sections 13-15 and standard text

13.02 - poss. identifying info?
    - just type of therapy = gen even step
    - don't know

14.11. Standard text misleading
    - GR 'Taking over' local reg
    - Clarify who does what
    - Age/PLD. Not all data
    - What's in it 4 patient?
    - Always a s.c.?
    - Consent → academic pharma
    - 'i understand' both

15. - SMA Europe already have toolkit, should map 1st then harmonise
15.02 - Age - patient/parent? + specify?
    - + what about reaching age of consent.
## Appendix 3: Feedback Survey Responses:

<table>
<thead>
<tr>
<th>What did you find useful at the SMA Dataset Workshop on the 13th December 2019?</th>
<th>What could we improve to make the next SMA Dataset Workshop even better?</th>
<th>Any other comments:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Every aspect of the workshop was useful in some way, in particular the range of presentations from individual registries. Perhaps most useful was the opportunity to meet in person the curators of the various registries from around the world.</td>
<td>Easy access to the outside for fresh air is always a good thing.</td>
<td>The workshop was very useful - not just because of the scheduled programme but the chance to meet and share information re funding etc. with other Registry coordinators</td>
</tr>
<tr>
<td>Very practical approach, clear explanations and instructions Interactive change of experience in registries Timelines clearly explained</td>
<td>Nothing important Keep going on as you are already use to Event. to check if the data required for some sections are maybe to huge /or unnecessary in practical sense</td>
<td>Congratulations for efficiency and organization of the workshop</td>
</tr>
<tr>
<td>New clinical trials, standardised data base</td>
<td>I think everything was quite well organised and interesting</td>
<td></td>
</tr>
<tr>
<td>Information on actual situation regarding expanded SMA Dataset project E.g. how many Registries are involved, on which level, what most common problems they meet. Possibility to know many new curators, exchange the experiences between the curators Common work on data set at the meeting</td>
<td>Presentation of updated situation in chosen Registries Verification of data set after some months of activities</td>
<td>The meeting was excellent, very well atmosphere, congratulations!</td>
</tr>
<tr>
<td>Work which have been done previously and future plans</td>
<td>One more day for better communication and questions</td>
<td>Hope to have another meeting soon</td>
</tr>
<tr>
<td>The most useful part as for me to see real experiences from different registries on expanded datasets implementation and current work, as well as working review and commenting on different aspects of it.</td>
<td>More interactive on purpose discussions</td>
<td>To try to have investigate differences of patient reported and clinicians reported registries and challenges (and then best practices how to work on it), make sure that any data gathering is patient centric and included PROMs</td>
</tr>
<tr>
<td>Extremely useful</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Yes</td>
<td>Brain storming topics before the meeting to be prepared with more suggestions</td>
<td></td>
</tr>
</tbody>
</table>

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