TREAT-NMD Neuromuscular Network

SMA Core Dataset Workshop 2022

7 December 2022
Fairmont Waterfront Hotel, Vancouver

Workshop Agenda

Time	Session Description	Presenter
13.15	Welcome and meeting objectives	Julie / Jess
13.30	Ice – Breaker and curator introductions	Jess
14.00	TREAT-NMD 2022 update	Neil
14.15	Year 3 Annual Report - highlights	Julie
14.30	Registry Case Study – UK Patient Registry PROMS	Lindsay
14.40	Core Dataset – success and lessons learnt	All
15.30	Coffee Break	
15.45	Group Activities – Group A, B ,C D (20 min)	Julie / Jess
16.05	Feedback session (10 mins per group)	All
16.45	Looking Ahead … priorities for 2023	Julie / Jess
17.10	What are the Take-Home Messages	Julie / Jess
17.30	Close Session	

Welcome & Introductions

Julie Bohill
Project Manager

Jess Page
Project Coordinator

Victoria Hodgkinson
SMA Sub-group Lead,
Executive Director of NMD registry

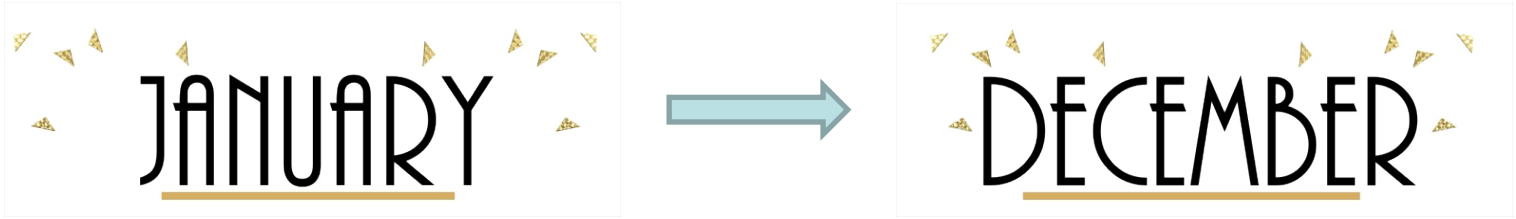
Miriam Rodrigues
SMA Sub-group Lead,
Neurogenetic Research Lead,
New Zealand

Marcel Heidemann
IT Consultant

Our Aims ….

- Update on TREAT-NMD activities
- Highlights from Year 3 Annual Curator survey
- Review what's going well with data collection
- Discuss the key challenges with dataset roll-out
- Share experiences and best practice
- Agree priorities for 2023 and next steps

Ice-breaker



- Please order yourself in line by birth month !
- Now introduce yourself to the person on either side of you

TREAT-NMD Neuromuscular Network

Registry Introductions

Please stand up and state:

- Your name
- Country
- Are you representing Clinician / Patient /Dual reported registry?
- SMA specific or general NMD Registry?
- What is your population? (i.e. paediatric, adult)
- What you hope to get out of today?

TREAT-NMD & Global Registry Network Update

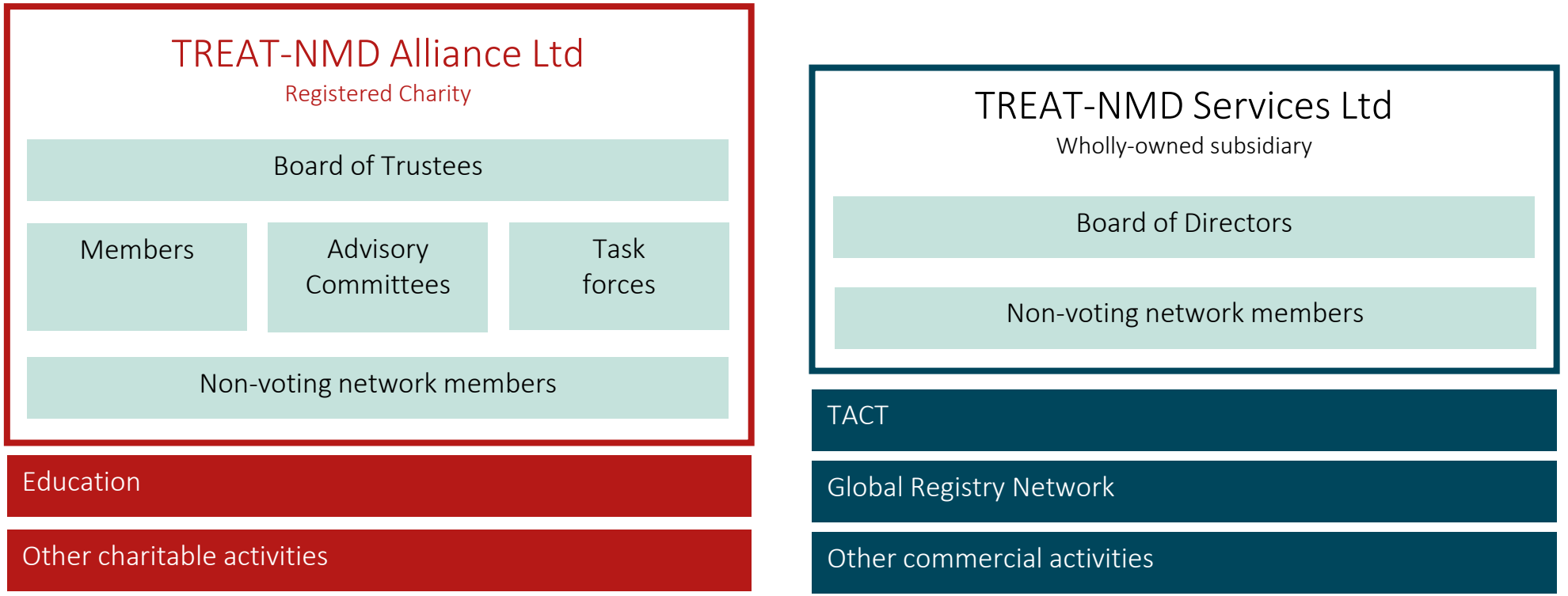
NEIL BENNETT

TREAT-NMD GLOBAL REGISTRIES MANAGER

TREAT-NMD
Neuromuscular Network

Consolidating TREAT-NMD's not for profit status

TREAT-NMD Alliance Ltd is a registered charity with a wholly-owned business arm called TREAT-NMD Service Ltd. The companies are owned by the network, and work to drive forward the network's aims.



TREAT-NMD Neuromuscular Network

Our vision

To accelerate the development of effective treatments and to establish best practice diagnosis and care for neuromuscular patients worldwide.

Our mission

To operate a collaborative, inclusive global network and organisational infrastructure that will overcome fragmentation, providing support services, information and data to advance treatment, diagnosis and care for neuromuscular patients globally.

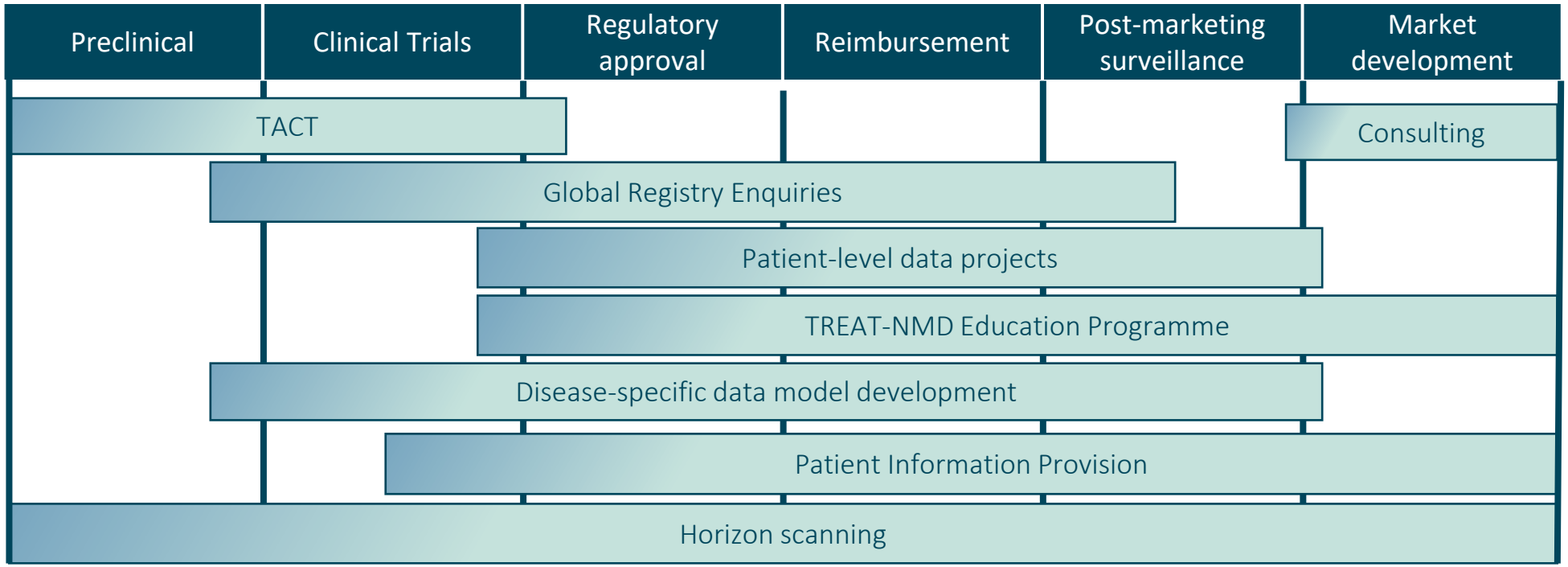
Our goals

- Leverage and expand our global reach
- Provide the ‘go to’ tools and services to support each stage of translational research
- Provide educational tools to improve diagnosis, treatment and care
- Facilitate agreement and adoption of standardised care guidelines, pre-clinical models, outcome measures and disease-specific datasets
- Further de-risk and accelerate the development of therapies by extending our advisory committees and enquiries processes
- Raise our profile and that of the neuromuscular disease areas we serve
- Facilitate best practice in data collection and become the ‘go to’ provider of NMD data to support evaluation, approval and post authorisation requirements of new treatments

TREAT-NMD Neuromuscular Network

De-risking and accelerating drug development

TREAT-NMD has several complimentary work streams that support drug development and bring new treatments to patients as quickly as possible



TREAT-NMD Neuromuscular Network

Key registry contacts at TREAT-NMD

TREAT-NMD has several complimentary work streams that support drug development and bring new treatments to patients as quickly as possible

Registries Team

Neil Bennett

Registries Manager

Hsin Chieh Chua

Registries Coordinator

Farjana Ali

Datasets & GRP Coordinator

Projects Team

Emma Watson

Seung Lee

John McKenna

CEO

David Allison

TREAT-NMD Neuromuscular Network

Any questions?

TREAT-NMD
Neuromuscular Network

This year’s highlights – supporting registries

- Refreshed registry enquiries costing matrix
- Supported conference abstract writing and poster production
- Changed enquiry contract to better represent registries

This year’s highlights – registry activity

- Completed one SMA Registry Enquiry
- Agreed contract for first post-authorisation study
- Started one disease landscaping study
- Working on a hypothesis generation study for a post-authorisation study

TREAT-NMD Neuromuscular Network

This year’s highlights - behind the scenes

- Totally rebuilding the TREAT-NMD website
- Totally rebuilding the Monday.com boards
- Compliance, documentation and staff training etc

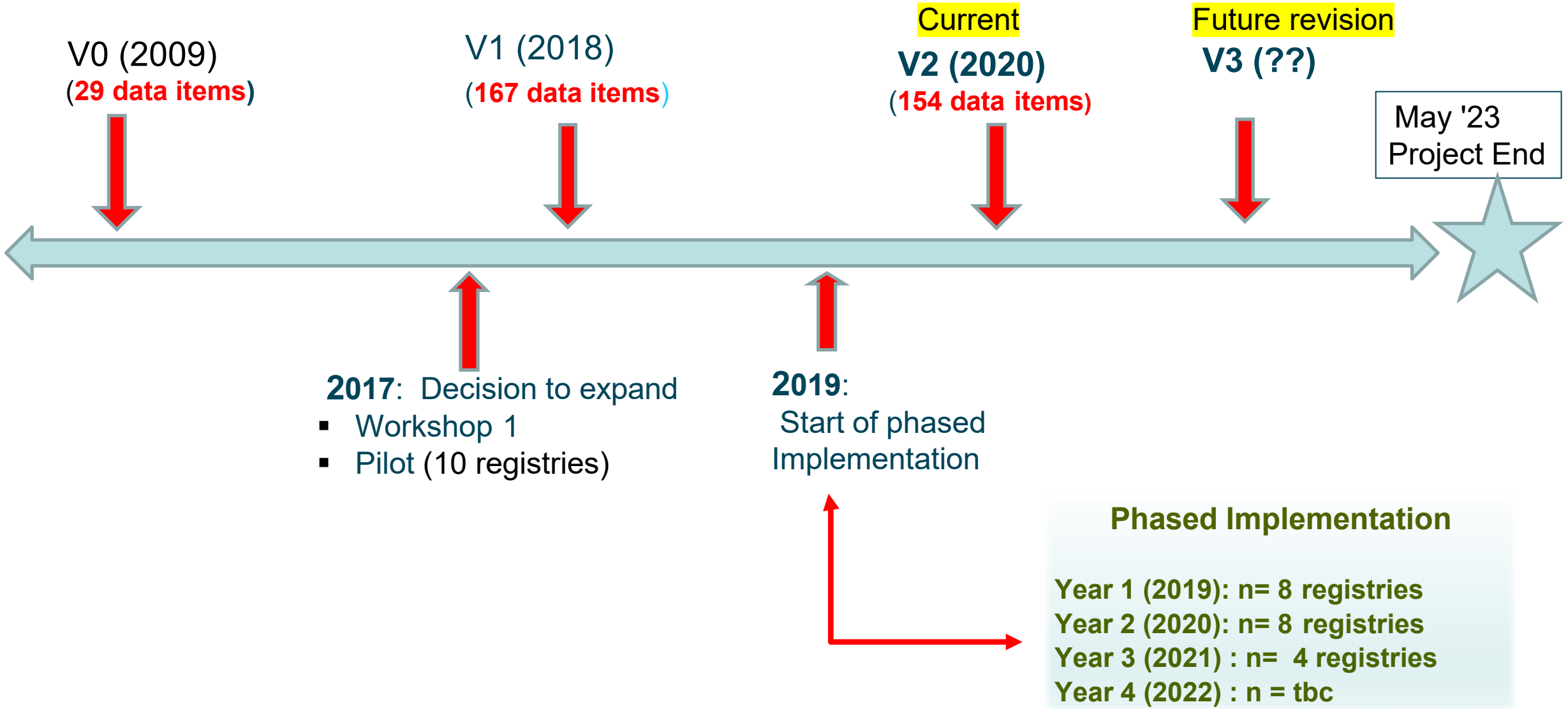
TREAT-NMD Neuromuscular Network

Next year…

- Registry summary publication
- Working with subgroups to address research questions
- Reviewing registry reimbursement
- Taking global registry platform to the next level

Any questions?

SMA dataset journey



SMA Core Dataset (V2)

TREAT-NMD Neuromuscular Network

154 Data Items …
Mandatory : 117 (CR), 91 (PR)
Non-Mandator : 37

Mandatory Items

Non-mandatory Items

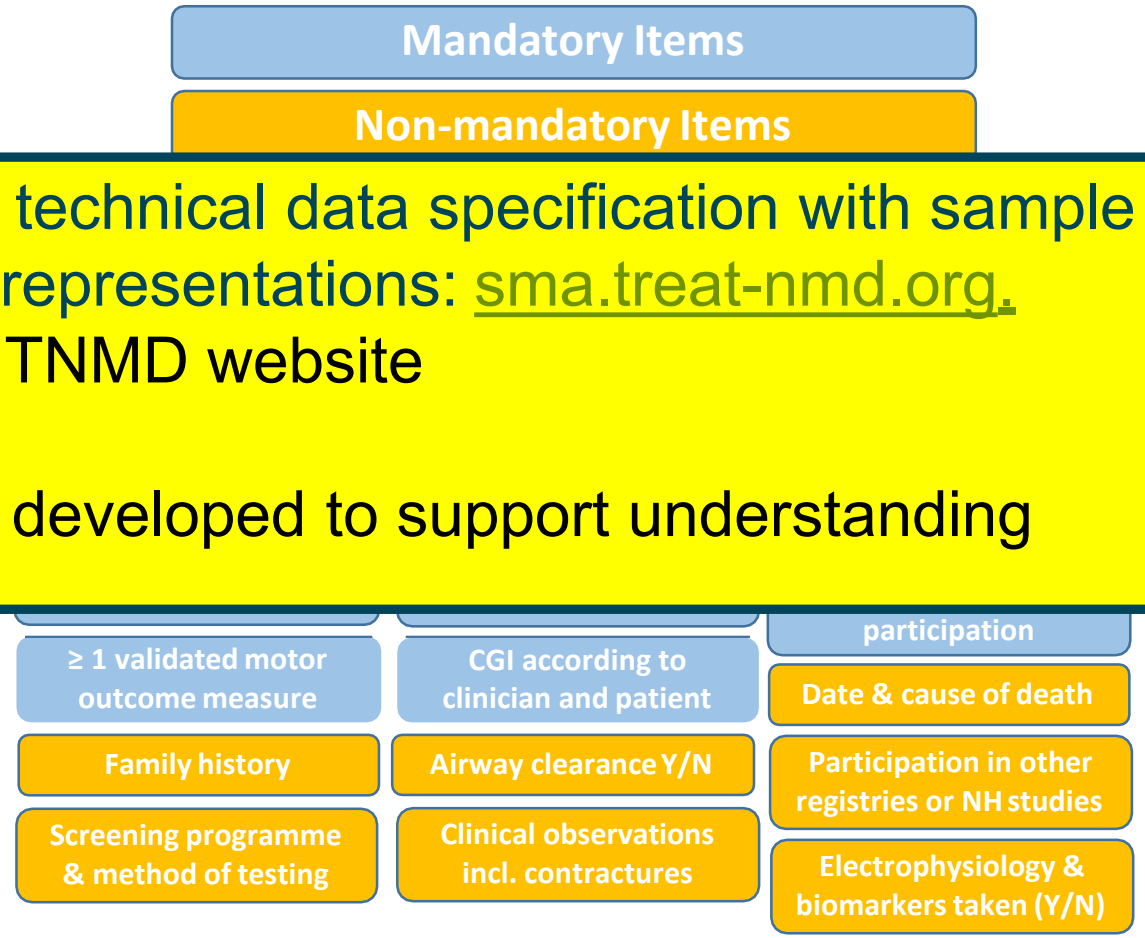
PPRL items	DOB, Sex, Country	Living status
Genetic diagnosis	SMA type & onset age	Wheelchair use
SMN2 copies	Best & current motor function extended	Feeding tube use
Scoliosis / surgery	Disease-modifying therapies	IV & NIV use
FVC results if done	Medication and rehabilitation	Therapeutic interventions
Hospitalisations & co-morbidities	CGI according to clinician and patient	Clinical trial participation
≥ 1 validated motor outcome measure	Airway clearance Y/N	Date & cause of death
Family history	Clinical observations incl. contractures	Participation in other registries or NH studies
Screening programme & method of testing		Electrophysiology & biomarkers taken (Y/N)

- ☑ DMT CR PR
- ☑ DMT status CR PR
- ☑ DMT single administration date CR PR
- ☑ DMT stopping reason CR
- ☑ DMT dosage value CR
- ☑ DMT dosage unit CR
- ☑ DMT administration route CR
- ☑ DMT administration intervals

CR items are *mandatory* for clinician-reported registries

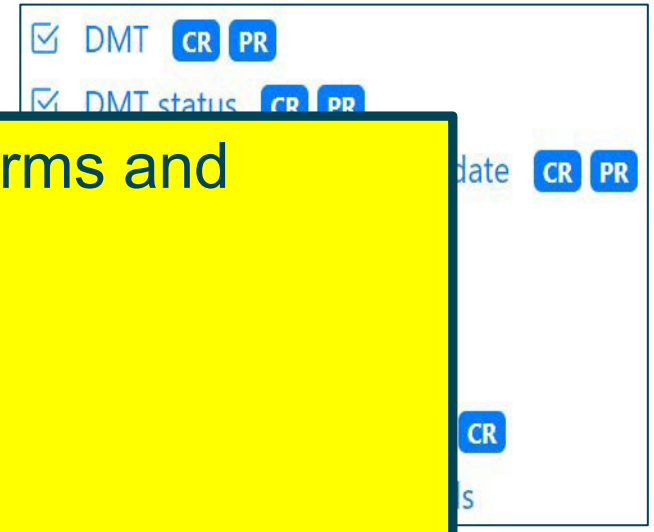
PR items are *mandatory* for patient-reported registries

SMA Core Dataset (V2)



Available as a technical data specification with sample forms and example data representations: sma.treat-nmd.org.
hosted on the TNMD website

10 min videos developed to support understanding



CR mandatory for clinician reported registry
PR – mandatory for patient reported

sma.treat-nmd.org

Any questions?

Year 3 Annual Curators Report

Key Highlights

Participating Registries

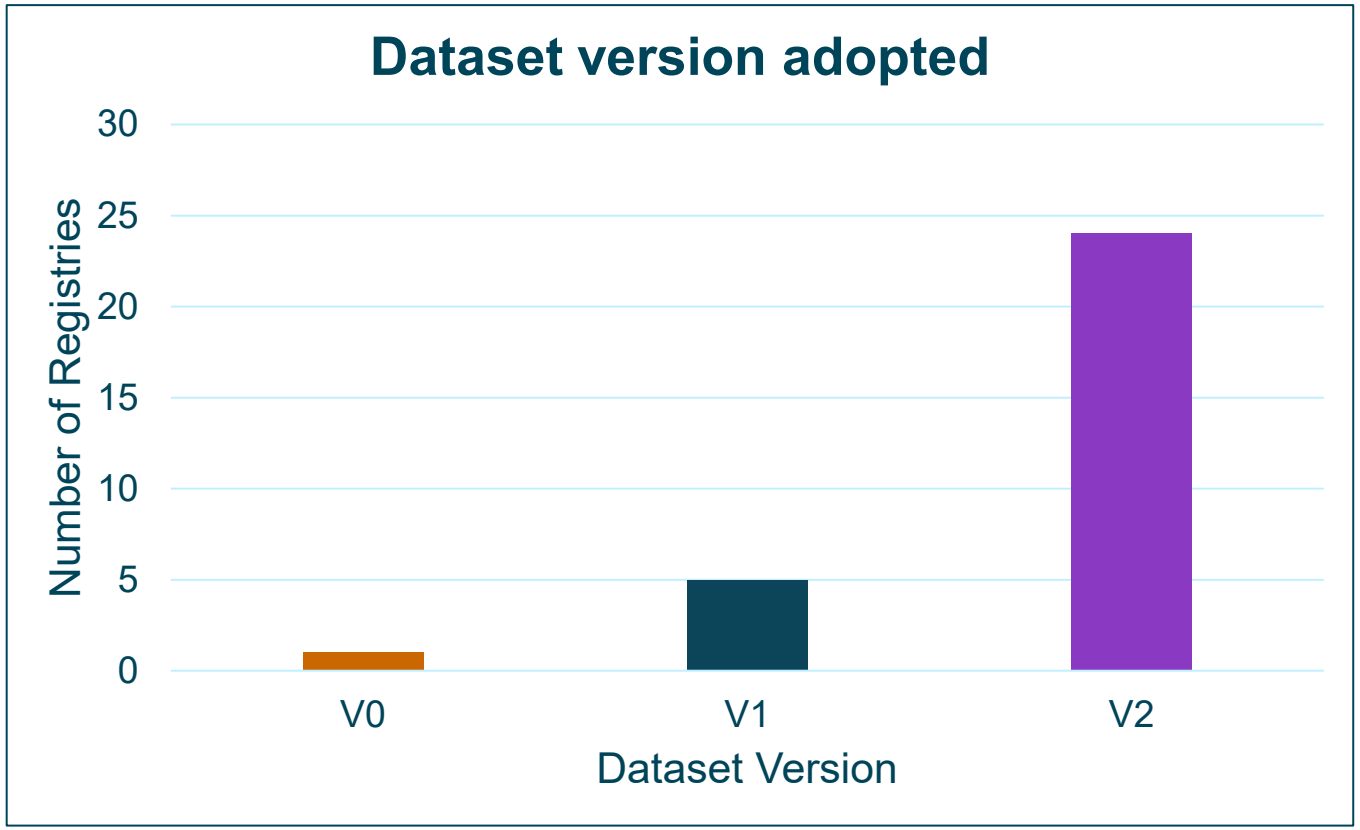
TREAT-NMD Neuromuscular Network

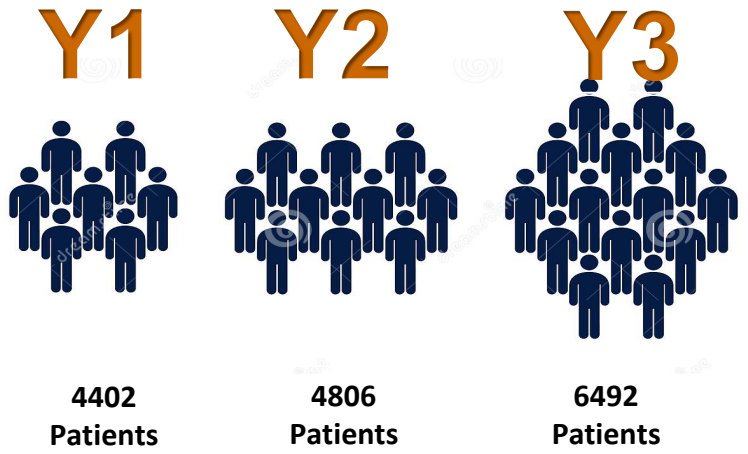
SMA Dataset Project - Participating Registries (by year)

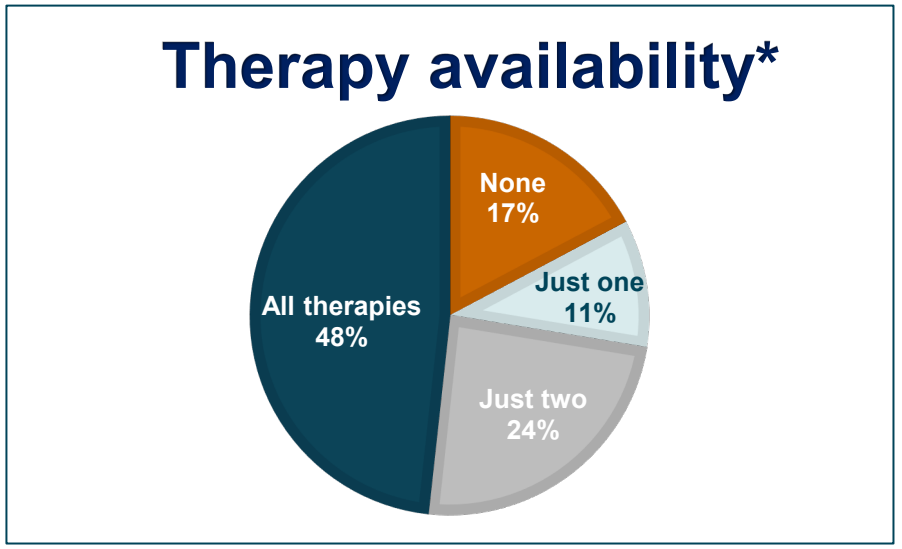
Pilot Year (2018) n=10	Year 1 (2019) n=8	Year 2 (2020) n=8	Year 3 (2021) n=4	Year 4 (2022) n= tbc
Australia	Czech Republic*	Armenia	China	Argentina tbc
Belgium	Hungary*	Bulgaria*	Lebanon*	Chile tbc
Canada	Latvia*	Columbia	South Africa*	Mexico*
Egypt NMD	Poland	Croatia*	Iran	Turkey (LUKAM)
Germany (Munich)	Serbia	Egypt (PED NMD)		
India	Spain	Georgia		
New Zealand	Switzerland	Malaysia		
Slovenia *	Turkey (KUKAS)	Sweden		
UK & Ireland				
Ukraine				

Key	
Clinician Reported	19
Patient Reported	9
Dual Reported	4
GRP users*	9
Total Participating Registries	32

Y3 Annual Report Update



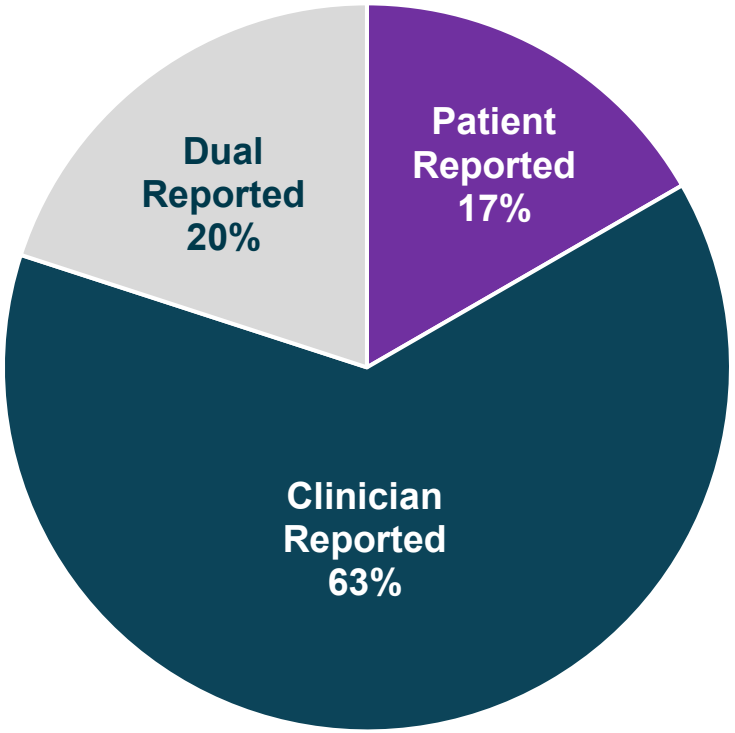


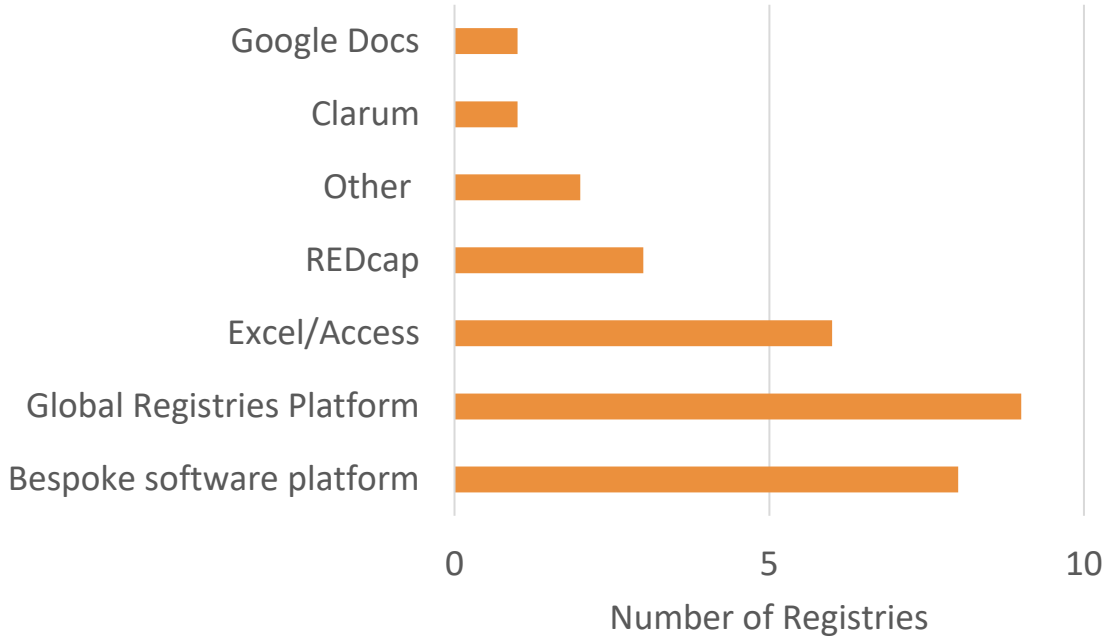


*n=30, Data presented is from a curator survey carried out in May 2022 and reflects what registry curators have reported regarding therapy access in their own country. Access is defined as any DMT availability at all irrespective of reimbursement restrictions or route of access.

Registry Type & Data collection methods

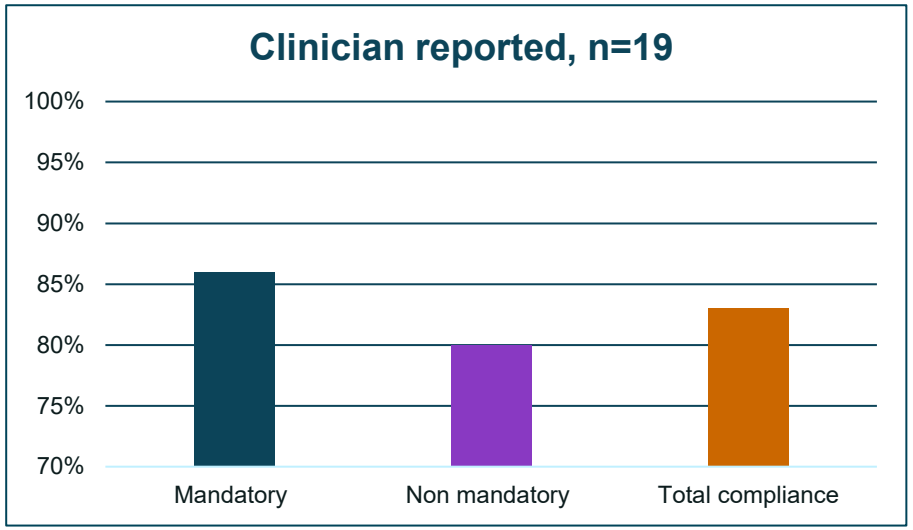
Registry Types

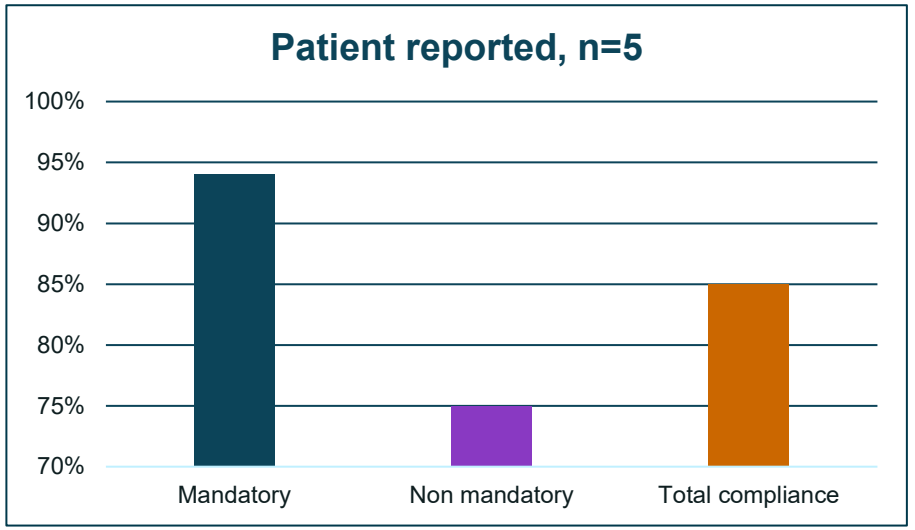


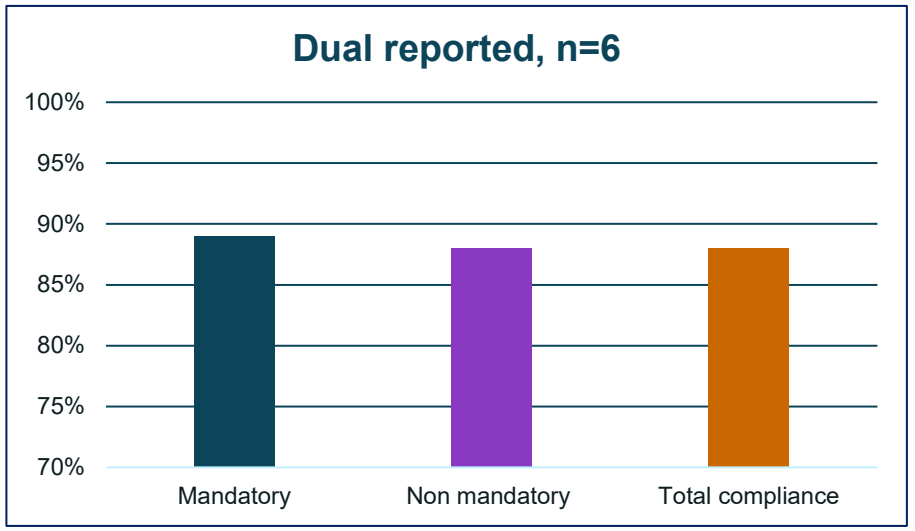


Bursary Payments		
	Part A	Part B
Number of Registries	24	9

Dataset Compliance Levels – Year 3







TREAT-NMD Neuromuscular Network

Most common items reported NOT collected

Mandatory	
Item	**Number of Registries NOT collecting**
Patient global impression of severity (CR) (PR), n=30	17 (57%)
Patient global impression of improvement (CR) (PR), n=30	17 (57%)
Clinical global impression of improvement (CR), n=19	9 (47%)
Clinical global impression of severity (CR), n=19	8 (42%)
Peak cough flow (CR), n=19	8 (42%)
DMT corticosteroid administration duration (CR), n=19	8 (42%)
DMT corticosteroid drug (CR), n=19	7 (37%)
Rehabilitative interventions usage (CR) (PR), n=30	6 (20%)
DMT dosage unit (CR), n=19	6 (32%)
Motor ability observed in clinic (CR) (PR), n=30	6 (20%)
SMN1 variant HGVS (CR) (PR), n=30	6 (20%)

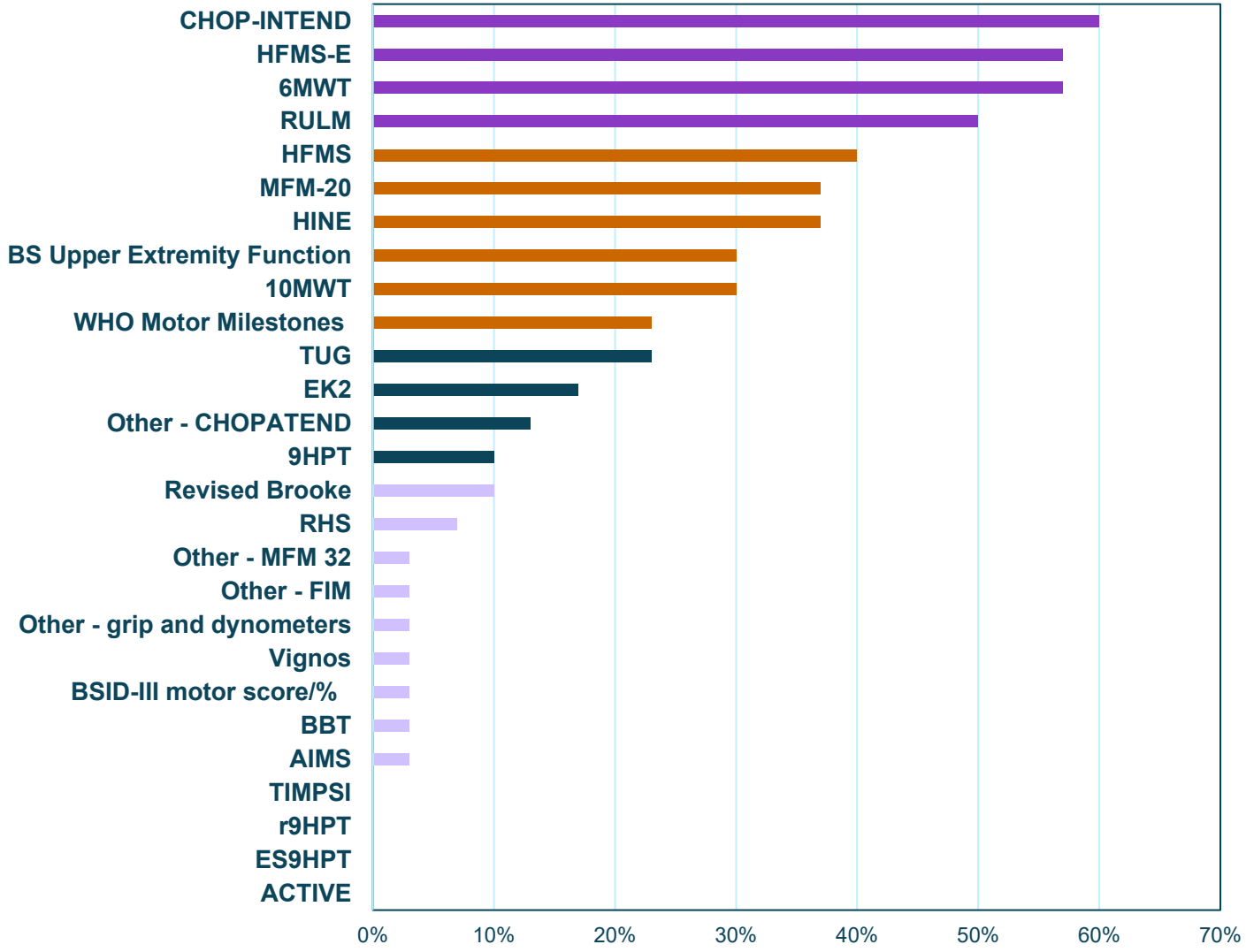
Non-Mandatory	
Item	**Number of Registries NOT collecting**
SMN2 variant c859GtoC testing method (n=30)	19 (63%)
SMN2 variant c859GtoC (n=30)	18 (60%)
Anti-AAVp antibody test days before administration (n=30)	16 (53%)
Anti-AAV9 Antibody test result (n=30)	15 (50%)
Cause of death (n=30)	11 (37%)
Cause of death classification (n=30)	11 (37%)
Head circumference (n=30)	10 (33%)

Motor Outcome Measures

Increased OM collection… building consensus !
What are the reasons for this?

- Increased therapy availability
- PMS studies
- Requirement for regulators & payers

Scan to download the SMA Outcome Measures Library



Any questions?

UK SMA Patient Registry
PROMs data collection & sharing

TREAT-NMD SMA Dataset Workshop

7th December 2022

Registry manager: Lindsay Murphy
lindsay.murphy@newcastle.ac.uk

Date collection in the UK

These three data collection studies work collaboratively to form UK SMA network.

Paediatric — SMA REACH UK

Adult — ADULT SMA REACH

SMA population — UK SMA PATIENT REGISTRY

UK SMA PATIENT REGISTRY

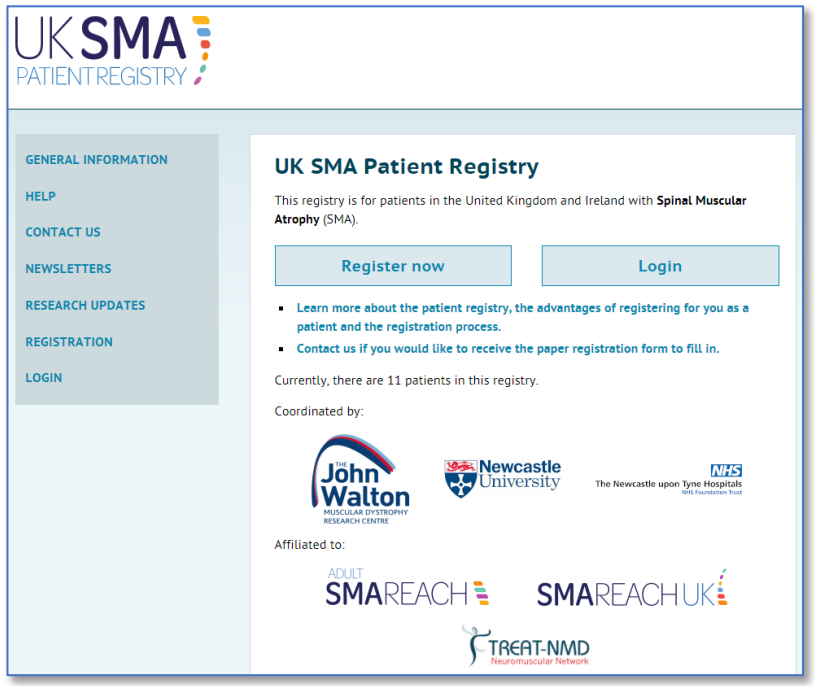
SMA REACH UK ADULT & SMA REACH

- Registration is patient-initiated.
- Collects *patient-reported* data.
- Active patient choice to participate and control use of their data; enables the patient voice to be captured.
- Available to all SMA patients in the UK & Ireland, independent of clinic.
- Affiliated to the TREAT-NMD SMA Global Registries Network; collects the TREAT-NMD SMA Expanded Core Dataset.
- Communication tool & ideal for collection of Patient Reported Outcome Measures (PROMs).

- Clinical databases for natural history.
- Collect *clinician-reported* data.
- To participate, patients must attend a Neuromuscular Clinic that is part of the SMA REACH networks.
- Apart from consent, patient participation is passive and occurs through their routine clinic visits.
- Networks are actively expanding, however cover is incomplete – therefore participation is not available to all UK SMA patients.

PROMs

- Capture the patient's perspective on their condition & any changes, their quality of life and activities of daily living
- Inform the revision and improvement of Standards of Care
- Help improve understanding of the impact of SMA therapy, supplementing Clinical Data and Functional Assessments
- PROMs collection and reporting is a requirement for Nusinersen and Risdiplam Managed Access Agreements - inform and assist regulatory authorities determine the efficacy of treatments



- Collected via the UK SMA Patient Registry, implemented in April 2022
- Ask consent for the sharing of data with SMA REACH
- All SMA patients in the UK & Ireland are welcome to register and to complete PROMs questionnaires

www.sma-registry.org.uk/

PROMs Pilot Study

- Collection of PROMs for the Nusinersen & Risdiplam Managed Access Agreements:

SMA REACH UK

50 paediatric Nusinersen patients & 50 adult Nusinersen patients

50 paediatric Risdiplam patients & 50 adult Risdiplam patients

100 patients via SMA REACH UK

100 patients via Adult SMA REACH

ADULT SMA REACH

- PROMs must coincide with six monthly clinic visits & therefore with clinical data collected by SMA REACH
- PROMs Pilot Study was introduced in the first instance to enable coordination between SMA REACH sites & patient registry
- Clinics in PROMs Pilot Study will receive individual, patient PROMs reports
- SMA REACH coordination teams will align PROMs with clinic data, anonymise & report to Regulatory Authorities

PROMs… benefit to *all* SMA REACH clinics

PROMs data from the patient registry is shared with clinics in **grouped patient reports**:

- Sharing of identifiable, patient-level data with each patient's own neuromuscular clinic to inform patient care (SMA REACH & patient registry consent in place), every ~6 months
- To enable this, sites first inform patient registry of patient ID
- Patient registry will provide all PROMs available for identified patients

PROMs collected

- Quality of Life EQ-5D
- Patient Global Impression of Change (Severity, Improvement)
- SMA Independence Scale (SMAIS) – non-ambulant
- Free-text box

Patient-reported & caregiver-reported versions

PROMs communications

- Regular & frequent communications with Pilot Study sites
- Increased frequency of patient registry automated reminder email from annual to every six months
- Emphasise the importance of reporting of PROMs at the same time as clinical data
- Patient registry PROMs promotional material
- Postcard & business card to be distributed to all sites
- Short animation posted to websites & shared with patient organisations

PROMs – UK SMA Patient Registry case study

https://www.youtube.com/watch?v=9EsZsBb5wb8&t=5s

Registry thoughts on the UK PROMS case study

- Do you think this is a good initiative?
- Would this be useful for your registry?
- Have you delivered any similar communications to patients / clinicians?
- Sharing best practice initiatives – how can improve on this in the future?

SMA Expanded Core Dataset

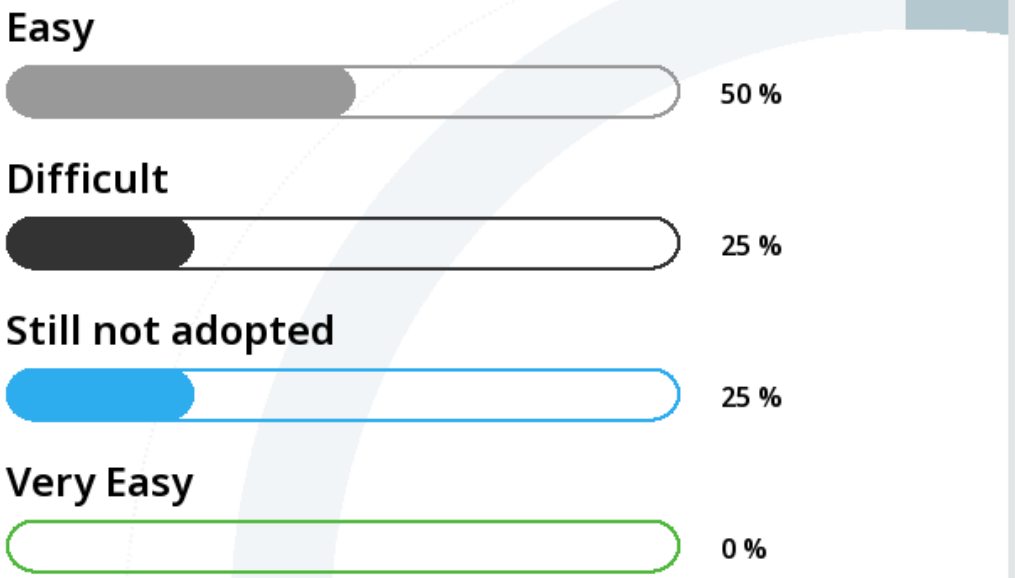
Group Work (50 mins)

Whova Poll – Results

Poll question

Please rate how easy/difficult your registry found it to adopt V2 of the SMA Dataset?

My response



Only displaying the top results. There were 1 other poll options

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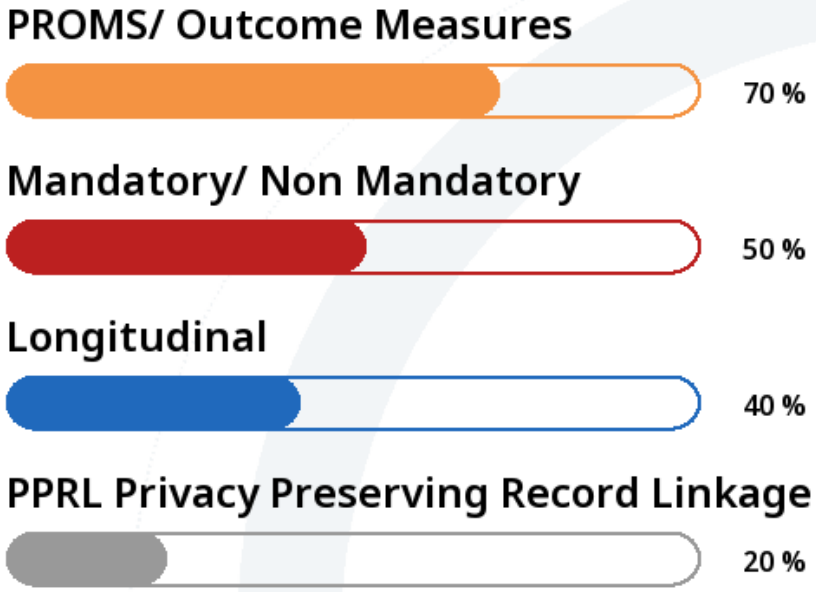
Whova Poll – Results

Poll question

Which elements of the Dataset Specification would you like further clarification/training on?

My response



Only displaying the top results. There were 2 other poll options

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Main dataset challenges

..... feedback from Annual Survey

TREAT-NMD Neuromuscular Network

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Cause of death (n=30)	11 (37%)
Cause of death classification (n=30)	11 (37%)
Head circumference (n=30)	10 (33%)

Feedback from groups

- Identify reasons why specific data items are proving difficult to collect?
- Should some items be re-classified as non-mandatory?
- How can we learn from one another?

What are the benefits/advantages of adopting the V2 dataset (word cloud - Whova poll)

Group Breakout Session

TREAT-NMD Neuromuscular Network

Group A

What are the key research questions the SMA community want answers to ?

How can this lead to publications / poster?

Group B

How can the global SMA registry network work better together?

Group C

Working with industry/ regulators - share registry experience of what has worked well and not so well

Group D

Sharing best practice on patient recruitment strategies and how to make registries more patient centric

Break-out Group feedback

Next Steps (Dec 22 – May 23)

Project End Date

SMA Project Timeline

15 May 2023

Dec ‘22

- SMA workshop
- Annual Curators Meeting
- Analyse workshop feedback

Jan ‘23

- Prepare & circulate workshop Report
- Monthly drop-in Sessions
- Continue supporting new/ existing registries
- Review TNMD dataset revision process

Feb ‘23

- Prepare & Issue Curator Surveys
- Confirm adoption of V2 by remaining registries
- Bursary processing
- Support new TNMD registries
- Circulate TNMD revision process for comment to TGDOC

Mar - April 23

- Analyse survey responses
- Registry 1-2-1 calls
- Process Final Bursary payments
- Start final report preparation

May ‘23

- Early May deliver report to Biogen.
- Upload to website
- Close project !

TREAT-NMD Dataset Revision Process

- **Need a single co-ordinated strategy for all NMD datasets – implement ‘23**

- **Dataset harmonisation across NMD's datasets**

✓ Who should be involved?
✓ When should it take place ?
✓ How long will registries be given to adopt the dataset to retain membership status ?

- **Does the One size fit’s all model work ?**

- **Multi stakeholder involvement in discussions**

Key priorities for 2023

- Outline what support TREAT-NMD can provide to assist with registry data collection
- What future networking events do SMA registry curators want
- Identify the key research questions that need to be asked
- Support for publications / posters for conferences
- Sub-Group Lead Role – seeking nominations (2023)

Whova Poll – Results

Poll question

What support could we provide which would be of most benefit to registries/curators?

My response

Only displaying the top results. There were 2 other poll options

Powered by Whova

Take-home messages!

- ❑ Collection of robust, standardised data is essential to building real world evidence to support PMS studies
- ❑ Need to balance needs of various stakeholders alongside registry maintenance
- ❑ How do we educate patients and clinicians on the value data collection
- ❑ Improve networking going forward - work collaboratively improve knowledge sharing and best practice
- ❑ Keep a patient focus to registry data collection. Patients focus on patient outcomes – registries should focus on the clinical need AND the patient outcomes and QoL

Please scan to complete the survey!

Thanks for the valuable support from our project sponsor