TREAT-NMD SMA Registries Core Dataset

31 August 2018

Version 1

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1. Introduction

TREAT-NMD is a network for the neuromuscular field that provides an infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. Since its launch in January 2007 the network's focus has been on the development of tools that industry, clinicians and scientists need to bring novel therapeutic approaches through preclinical development and into the clinic, and on establishing best-practice care for neuromuscular patients worldwide.

The TREAT-NMD global network of SMA Registries feed into a central hub called the TREAT-NMD Global SMA Registry, which can receive requests from third parties in order to answer research questions. National registries that are part of the Global Registry must collect as a minimum, a standardized core dataset from their patients.

The TREAT-NMD Global SMA Registry is governed by a Charter* and by the TREAT-NMD Global Database Oversight Committee (TGDOC). The TGDOC is responsible for reviewing the requests for data from the Global Registry and it votes on whether the request is in line with the Charter and is in patients' best interests.

In May 2017, TGDOC initiated a project to review and expand the core dataset for the TREAT-NMD network of SMA Registries, and this document describes the resulting expanded core dataset.

Part of the strength of the TREAT-NMD SMA Registries lies in their diversity; however, this also means that the expanded dataset will present more of a challenge to some registries than to others. The network strives for inclusivity and if registries are not able to implement the full expanded dataset immediately, the TGDOC would encourage discussion on feasible implementation plans and any identified support requirements.

** The TGDOC Charter is currently under review*

2. Data Submissions

Schedule

As a minimum, national SMA registries will be asked to provide an annual submission of their dataset to the central registry. Ad-hoc enquiries into the Global Registry may also result in one-off data submission requests.

The timing for the annual submissions is yet to be decided.

Method

Registries using their own platform will be asked to provide their data submissions in a pre-agreed file format, e.g. Excel or CSV.

Registries who choose to use the TREAT-NMD Universal Registry Platform to host their registry will be able to securely submit their data online via the platform.

3. The Dataset

Please read these important notes before reading or using this dataset:

- This is a detailed description of the TREAT-NMD SMA Registries Core Dataset. It is supported by, and should be used in the context of, the following documents:
  - "**TREAT-NMD SMA Registries Core Dataset: Overview**": Gives a high-level overview of the dataset.
  - "**TREAT-NMD SMA Registries Core Dataset Manual**": Contains full definitions and guidance on data collection.

- **Toolkits** are in development to support affiliated registries in the collection of motor measures and patient-reported outcomes.

- Any anticipated **training or support requirements** should be reported to joanne.bullivant@newcastle.ac.uk.

PLEASE NOTE:

- For the mandatory data items: TREAT-NMD SMA Registries are required to include these items in their case report forms, and make every effort to collect them (or agree actions to work towards their collection).
- However, the minimum data needed for an individual record to be accepted as valid for a global registry enquiry submission will be defined on a case-by-case basis.

- Registries should ensure that all data entries and updates are date-stamped (and time-stamped if possible)

KEY
Items in black text are mandatory
Items in blue are highly encouraged
CR = mandatory only for clinician-reported registries
PR = mandatory only for patient-reported registries (**an adapted dataset with different wording will be developed for patient-reported registries**)

Section 1: ENROLMENT

Item no.	Data item description	Coding	Baseline	Follow-up
1.00	Date of enrolment	DD-MM-YYYY	X	
1.01	Date of consent (if different from date of enrolment)	DD-MM-YYYY	X	
1.02	Date of any re-consents	DD-MM-YYYY (Can add multiple)	X	X
1.03	Patient has also consented to pseudonymised data being shared with the TREAT-NMD Global SMA Registry?	Yes; No	X	
1.04	Local registry ID		X	

Section 2: DEMOGRAPHICS

Items 2.00-2.16: Registries are encouraged to collect the demographic items in blue for internal use, but only the mandatory items (in black) would ever be requested for central submission.

^ *= items which support PPRL functionality (Privacy Preserving Record Linkage)*

Item no.	Data item description	Coding	Baseline	Follow-up
2.00	Date of birth	DD-MM-YYYY	X	
2.01	First name	[Free text]	X	X
2.02	First name given at birth (if different)^	[Free text]	X	
2.03	Last name	[Free text]	X	X
2.04	Last name given at birth (if different)^	[Free text]	X	
2.05	Sex	Male; Female; Unspecified	X	X
2.06	Sex assigned at birth (if different)^	Male; Female; Unspecified	X	
2.10	Address	[Free text]	X	X
2.11	Zip/post code	[Free text]	X	X
2.12	Country of residence	[ISO 3 Standards]	X	X
2.13	Country of birth^	[ISO 3 Standards]	X	
2.14	City/town of birth^	[Registry own picklist]	X	

2.15	Email address	[Free text]	X	X
2.16	Telephone number	[Numerical value]	X	X
2.20	Does the patient have any other known family member diagnosed with SMA?	Yes; No	X	X
2.21	If Yes to 2.20; state kinship (select all that apply)	Mother; Father; Daughter; Son; Brother; Half Brother; Sister; Half Sister; Niece; Nephew; Maternal Uncle; Paternal Uncle; Maternal Aunt; Paternal Aunt; Maternal Cousin; Paternal Cousin; Maternal Grandfather; Paternal Grandfather; Maternal Grandmother; Paternal Grandmother; Granddaughter; Grandson (Can add multiple)	X	X

Section 3: LIVING STATUS

Item no.	Data item description	Coding	Baseline	Follow-up
3.00	Is the patient alive?	Yes; No; Loss of follow-up	X	X
3.01	If 'No' to 3.00: Date of death	DD-MM-YYYY		
3.02	If 'No' to 3.00: Cause of death	[Full ICD-10 Classification] *Or:* Certain infectious and parasitic diseases; Neoplasms; Diseases of the blood and blood-forming organs and certain disorders involving the immune mechanism; Endocrine, nutritional and metabolic diseases; Mental, Behavioural		

		and Neurodevelopmental disorders; Diseases of the nervous system; Diseases of the eye and adnexa; Diseases of the ear and mastoid process; Diseases of the circulatory system; Diseases of the respiratory system; Diseases of the digestive system; Diseases of the skin and subcutaneous tissue; Diseases of the musculoskeletal system and connective tissue; Diseases of the genitourinary system; Pregnancy, childbirth and the puerperium; Certain conditions originating in the perinatal period; Congenital malformations, deformations and chromosomal abnormalities; Other - Symptoms, signs and abnormal clinical and laboratory findings, not elsewhere classified (Specify, free text)		

Section 4: GENETIC DIAGNOSIS

◊ = in patient-reported registries, items marked with ◊ should be reported by the Registry Curator, following review of the patient's genetic report.

Item no.	Data item description	Coding	Baseline	Follow-up
4.00	Has the patient had genetic confirmation of SMA?	Yes; No	X	X
4.01	◊ If yes to 4.00, was it through screening?	Unknown; No; Yes, Family Screening; Yes, Newborn Screening Programme; Yes, Prenatal Screening	X	X
4.02	If 'Yes' to 4.00: Send/upload copy of genetic report (PR)	[File upload option]	X	X

4.03	If 'Yes' to 4.00: Name/location of genetic testing centre	[Free text]	X	X
4.04	◊ If 'Yes' to 4.00: Date of genetic diagnosis	DD-MM-YYYY	X	X
4.05	◊ If 'Yes' to 4.00: Mutation name in SMN1 gene	SMN1 homozygous deletion of exon 7 (&8); SMN1 heterozygous deletion of exon 7 (&8) and compound heterozygous to a point mutation in SMN1; Compound heterozygous (or homozygous) for two (or one) point mutation(s) in SMN1)	X	X
4.06	◊ If 'Yes' to 4.00: Method of SMN1 testing	RFLP (Restriction Fragment Length Polymorphism); HRM (High Resolution Melting); MLPA (Multiplex Ligation-dependent Probe Amplification); Luminex Genotyping / DNA Sequencing; qrtPCR (Quantitative Real-Time PCR); ddPCR (Droplet Digital PCR); Other (specify); Unknown		
4.07	◊ If 'Yes' to 4.00: Was SMN2 Copy number tested?	Yes; No	X	X
4.08	◊ If 'Yes' to 4.07: Method of SMN2 testing	RFLP (Restriction Fragment Length Polymorphism); HRM (High Resolution Melting); MLPA (Multiplex Ligation-dependent Probe Amplification); Luminex Genotyping / DNA Sequencing; qrtPCR (Quantitative Real-Time PCR); ddPCR (Droplet Digital PCR); Other (specify); Unknown	X	X
4.09	◊ If 'Yes' to 4.07: SMN2 copy number	1; 2; 3; 4+	X	X

Section 5: CLINICAL OBSERVATIONS

Item no.	Data item description	Coding	Baseline	Follow-up
5.00	Age of symptom onset (At what age was it suspected that something might be different?)	Prenatal; at birth; age YY-MM (from 0.5M)	X	
5.01	Spinal Muscular Atrophy type	0; 1; 2; 3; 4	X	
5.02	Height/length (cm)	[Numerical value]	X	X
5.03	Method of height measurement	Standing height; Recumbent length; Arm span; Ulnar length; Other (specify, free text)	X	X
5.04	Weight	[Numerical value] kg	X	X
5.05	Head circumference (for infants <24 months old)	[Numerical value] cm	X	X
5.06	Chest circumference at full expiration (for infants <24 months old)	[Numerical value] cm	X	X
5.07	Chest circumference at full inspiration (for infants <24 months old)	[Numerical value] cm	X	X
5.08	Does the patient suffer from shoulder contractures?	Yes; No	X	X
5.09	Does the patient suffer from elbow contractures?	Yes; No	X	X
5.10	Does the patient suffer from wrist contractures?	Yes; No	X	X
5.11	Does the patient suffer from finger contractures?	Yes; No	X	X
5.12	Does the patient suffer from hip contractures?	Yes; No	X	X
5.13	Does the patient suffer from knee contractures?	Yes; No	X	X
5.14	Does the patient suffer from ankle contractures?	Yes; No	X	X
5.20	[PR] Name of the neuromuscular specialist or main doctor in charge of your care	[Free text]	X	X
5.21	[PR] Name/location of your main healthcare centre	[Free text]	X	X

Section 6: SCOLIOSIS

Item no.	Data item description	Coding	Baseline	Follow-up
6.00	Has the patient been diagnosed with scoliosis?	Yes; No; Unknown	X	X
6.01	If 'Yes' to 6.00: Cobb angle according to radiology results	[Numerical value] degrees	X	X
6.02	If 'Yes' to 6.00: has the patient had surgery for the scoliosis?	Yes; No	X	X
6.03	If 'Yes' to 6.02: Surgery technique	Arthrodesis; Growing Rods; Other (specify); Unknown	X	X

6.04	If 'Yes' to 6.02; date (month & year) of first surgery	MM-YYYY	X	X

Section 7: MOTOR FUNCTION

WHO = *indicates that this item is used to derive the WHO motor milestone score*

Please refer to the dataset manual for definition of each motor function

Item no.	Data item description	Coding	Baseline	Follow-up
7.00	Holding head up without support	*For each motor function item, specify:* Never able; Gained (age YY-MM); Gained & lost (age gained YY-MM & age lost YY-MM) *And:* Observed in clinic; Reported by Patient/Caregiver	X	X
7.01	Rolling onto side		X	X
7.02	Sitting without support WHO		X	X
7.03	Crawling on hands and knees WHO		X	X
7.04	Standing with assistance WHO		X	X
7.05	Standing alone (without assistance) WHO		X	X
7.06	Walking with assistance WHO		X	X
7.07	Walking alone (without assistance) WHO		X	X
7.08	Able to walk 10 metres unaided		X	X
7.09	Climbing stairs		X	X
7.10	Useful function of hands		X	X
7.11	Reaching overhead in a sitting position		X	X
7.12	Raising hands to mouth in a sitting position		X	X

Section 8: WHEELCHAIR USE

Item no.	Data item description	Coding	Baseline	Follow-up
8.00	Does the patient use a wheelchair? (For patients ≥ 2 years old)	No (able to walk independently); Part-time (age began YY-MM); Full-time (age began YY-MM)	X	X

Section 9: NUTRITION

Item no.	Data item description	Coding	Baseline	Follow-up
9.00	Has the patient ever used a gastric or nasal feeding tube? (Select all that apply)	Never; Previously exclusively fed by tube (start and end date MM-YYYY); Previously supplementary e.g. for fluids (start and end date MM-YYYY); Currently exclusively fed by tube (start date MM-YYYY); Currently supplementary e.g. for fluids (start date MM-YYYY); Unknown	X	X

Section 10: PULMONARY FUNCTION

Item no.	Data item description	Coding	Baseline	Follow-up
10.00	Has the patient ever used invasive ventilation?	Never; Previously (start and end date MM-YYYY); Currently (start date MM-YYYY); Unknown	X	X
10.01	If 'Yes' to 10.00: Frequency of invasive ventilation	Full-time; part-time; unknown	X	X
10.02	If 'Yes' to 10.00: Invasive ventilation start date (month and year)	MM-YYYY	X	X
10.03	Has the patient ever used non-invasive ventilation?	Never; Previously (start and end date MM-YYYY); Currently (start date MM-YYYY); Unknown	X	X
10.04	If 'Yes' to 10.03: Frequency of non-invasive ventilation	Full-time; part-time; unknown	X	X
10.05	If 'Yes' to 10.03: Non-invasive ventilation start date (month and year)	MM-YYYY	X	X
10.06	Does the patient need assistance in airway clearance and/or secretion mobilisation?	Yes; No	X	X
	If 'Yes' to 10.06; Type of assistance (select all that apply)			
10.07	Suction	Daily; Weekly; Occasionally	X	X

10.08	Chest percussion	Daily; Weekly; Occasionally	X	X
10.09	Cough Assist device	Daily; Weekly; Occasionally	X	X
10.10	IPPV (Intermittent positive-pressure ventilation)	Daily; Weekly; Occasionally	X	X
10.11	Other (Please Specify)	Daily; Weekly; Occasionally	X	X
10.12	Has the patient had a Forced Vital Capacity (FVC) test?	Yes; No; Unknown	X	X
10.13	If 'Yes' to 10.12: Date of most recent FVC test, if known	DD-MM-YYYY	X	X
10.14	[CR] If 'Yes' to 10.12: FVC litre	[Numerical value]	X	X
10.15	[CR] If 'Yes' to 10.12: FVC predicted %	[0-150] %	X	X

Section 11: THERAPIES AND MEDICATIONS

Item no.	Data item description	Coding	Baseline	Follow-up
11.00	[CR] Has the patient ever received a disease-modifying therapy for SMA?	Currently; Previously; Never; Don't know	X	X
11.01	[PR] Are you receiving Spinraza? (*Question will be modified in future as needed)*	Currently; Previously; Never	X	X
11.02	[CR] If 'Currently' or 'Previously' to 11.00: Name of drug(s)	Spinraza; Other (specify, free text) (Can add multiple)	X	X
11.03	[CR] For each drug named in 11.02: Start date	[DD-MM-YYYY]	X	X
11.04	[CR] For each drug named in 11.02: Stop date if not ongoing	[DD-MM-YYYY]	X	X
11.05	[CR] If stop date given in 11.04: Reason for stopping	Insurance coverage/funding; Side effects from the procedure; Side effects from the drug; Lack of apparent benefit; Elective choice of other treatment	X	X
11.06	[CR] For each drug named in 11.02: Dosage given	Numerical value	X	X
11.07	[CR] For each drug named in 11.02: Frequency of dosage	Every [numerical value] day(s); week(s); month(s); year(s)	X	X
11.08	[CR] For each drug named in 11.02: Route of administration	Intrathecal injection; Other (specify, free text)	X	X
11.09	[CR] For each drug named in 11.02: Is the patient following the current recommended dosing schedule?	Yes; No; Don't know; Not applicable	X	X
11.10	[CR] If 'No' to 11.09: Reason for not following dosing schedule	Illness; Access problem; Scoliosis surgery; Other (specify, free text)	X	X

11.11	Has the patient taken any prescribed allopathic drugs in the last 12 months (*baseline*) / since the last registry update (*follow-up*)?	Yes; No; Don't know	X	X
11.12	If 'Yes' to 11.11: Name of drug ** Please note; the inclusion of a supplement in this list does not necessarily indicate TREAT-NMD endorsement.*	Bone health: Vitamin D; Calcium; Biphosphonate; Gastro intestinal system: Drugs for gastroesophageal reflux; Drugs for constipation; Respiratory system: Antibiotics; Anticholinergic drugs; Immunisations: Annual influenza immunizations; Annual pneumococcal immunizations; Supplements*: Creatine; Acetyl-L-carnitine; Phenylbutyrate; Gabapentin; Thyrotropin-releasing hormone; Hydroxyurea; Valproate; Albuterol; Other (specify, free text) (Can add multiple)	X	X
11.13	For each drug named in 11.12: Start date (month & year)	[MM-YYYY]	X	X
11.14	For each drug named in 11.12: Stop date (month & year) if not ongoing	[MM-YYYY]	X	X
11.20	Which of the following therapeutic interventions has this patient received in the last 12 months (*baseline*) / since the last registry update (*follow-up*)? (Select all that apply)	Physiotherapy sessions (e.g. stretches); Respiratory physiotherapy sessions; Massage; Home programme (e.g. stretches/exercises); Hydrotherapy/water-based activity; Management of contractures using orthotics (e.g. ankle foot orthoses); Brace; Occupational therapy sessions /	X	X

		input for home or equipment; Speech and language therapy sessions; Other (specify, free text)		

Section 12: HOSPITALISATIONS AND COMORBIDITIES

Item no.	Data item description	Coding	Baseline	Follow-up
12.00	Has the patient been hospitalised in the last 12 months (*baseline*) / since the last registry update (*follow-up*)?	Yes; No; Don't know	X	X
12.01	If 'Yes' to 12.00: Type of initial hospitalisation	Planned; Acute (Can add multiple)	X	X
12.02	For each hospitalisation in 12.01: Admission date (month & year)	[MM-YYYY]	X	X
12.03	For each hospitalisation in 12.01: Number of days in hospital	[Numerical value]	X	X
12.04	[CR] For each acute hospitalisation: Reason for hospitalisation	[MedDRA Coding]	X	X
12.05	[CR] For each planned hospitalisation: Reason for hospitalisation	Placement of g-tube; Sleep study; Scoliosis fusion; Hip surgery; Other orthopaedic surgery (specify, free text); Administration of Spinraza; Administration of other disease-modifying treatment for SMA (specify, free text); Other reason (specify, free text)	X	X
12.06	[CR] For each Acute hospitalisation: was this also reported as an SAE?	Yes; No	X	X
12.07	[CR] If 'Yes' to 12.06: in relation to which medication?	Spinraza; Other (specify, free text)	X	X
12.10	In addition to the hospitalisations already reported, has the patient been diagnosed with any other co-morbidities in the last 12 months (*baseline*) / since the last registry update (*follow-up*)?	Yes; No; Don't know	X	X
12.11	If 'Yes' to 12.10: Comorbidity details	[Full ICD-10 Classification] *Or:* Certain infectious and parasitic diseases; Neoplasms; Diseases of the blood and blood-forming	X	X

		organs and certain disorders involving the immune mechanism; Endocrine, nutritional and metabolic diseases; Mental, Behavioural and Neurodevelopmental disorders; Diseases of the nervous system; Diseases of the eye and adnexa; Diseases of the ear and mastoid process; Diseases of the circulatory system; Diseases of the respiratory system; Diseases of the digestive system; Diseases of the skin and subcutaneous tissue; Diseases of the musculoskeletal system and connective tissue; Diseases of the genitourinary system; Pregnancy, childbirth and the puerperium; Certain conditions originating in the perinatal period; Congenital malformations, deformations and chromosomal abnormalities; Other - Symptoms, signs and abnormal clinical and laboratory findings, not elsewhere classified (specify, free text) (Can add multiple)		
12.12	For each Comorbidity: Comorbidity start date (month & year)	[MM-YYYY]	X	X
12.13	For each Comorbidity: Comorbidity end date if not ongoing	[MM-YYYY]	X	X
12.14	[CR] For each Comorbidity: Comorbidity also reported as SAE?	Yes; No	X	X
12.15	[CR] If 'Yes' to 12.14: in relation to which medication?	Spinraza; Other (specify, free text)	X	X

12.20	[CR] In addition to the hospitalisations, co-morbidities or death already recorded: any other SAEs reported?	Yes; No		
12.21	[CR] If 'Yes' to 12.20: in relation to which medication?	Spinraza; Other (specify, free text)	X	X

Section 13: CLINICAL RESEARCH

Item no.	Data item description	Coding	Baseline	Follow-up
13.00	Has the patient ever participated in a clinical trial?	Currently; Previously; Never; Don't know	X	X
13.01	If 'Currently' or 'Previously' to 13.00: Name of trial(s)	[Free text] (Can add multiple)	X	X
13.02	For each trial named in 13.01: Name of drug	[Free text]	X	X
13.10	Is the patient currently part of another registry and/or natural history study?	Yes; No; Don't know	X	X
13.11	If 'Yes' to 13.10: Name of registry or natural history study	[Free text]	X	X

Section 14: MOTOR MEASURES

*Clinician-reported registries only. Registries are required to collect a minimum of **1 validated motor measure**, in addition to the mandatory motor function question in Section 7.*

*Selection of appropriate motor measure(s) is at the discretion of the clinician and/or preference of the patient. Where there is no pre-existing preference, the measures marked with * are suggested by TREAT-NMD, based on current Standards of Care and prior use in Clinical Trials.*

Item no.	Data item description	Coding	Baseline	Follow-up
14.00	[CR] Was a validated motor measure taken for this patient at this visit?	Yes; No	X	X
14.01	[CR] If 'No' to 14.00: give reason	Unable to attain start position/disease progression; Injury/acute injury/illness (specify, free text); Inability to follow or understand directions; Refusal/ attention/behaviour issue; Fatigue	X	X

		Pain/Muscle cramp; Equipment/software issue; Other (specify, free text)		
	[CR] If 'Yes' to 14.00: provide relevant details:			
	Infantile onset SMA:			
14.10	* CHOP-INTEND score	[Numerical value]	X	X
14.11	* CHOP-INTEND date	DD-MM-YYYY	X	X
	(Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders)			
14.12	* HFMS score	[Numerical value]	X	X
14.13	* HFMS date	DD-MM-YYYY	X	X
	(Hammersmith Functional Motor Scale)			
14.14	* HFMS-E score	[Numerical value]	X	X
14.15	* HFMS-E date	DD-MM-YYYY	X	X
	(Hammersmith Functional Motor Scale - Expanded)			
14.16	HINE Section 2 score	[Numerical value]	X	X
14.17	HINE Section 2 date	DD-MM-YYYY	X	X
	(Hammersmith Infant Neurological Exam. Section 2 = motor milestones)			
14.18	Observed WHO score	[Numerical value]	X	X
14.19	Observed WHO date	DD-MM-YYYY	X	X
	(World Health Organisation motor development milestones)			
14.20	Other validated measure for infantile onset SMA (specify)	[Free text] (Can add multiple)	X	X
14.21	For each measure named in 14.20: score	[Numerical value]	X	X
14.22	For each measure named in 14.20: date	DD-MM-YYYY	X	X
	Later onset SMA:			

14.30	* HFMS-E score	[Numerical value]	X	X
14.31	* HFMS-E date	DD-MM-YYYY	X	X
	(Hammersmith Functional Motor Scale - Expanded)			
14.32	* RULM score	[Numerical value]	X	X
14.33	* RULM date	DD-MM-YYYY	X	X
	(Revised Upper Limb Module)			
14.34	Brooke score	[Numerical value]	X	X
14.35	Brooke date	DD-MM-YYYY	X	X
	(Brooke Scale of upper extremity function)			
14.36	Revised Brooke score	[Numerical value]	X	X
14.37	Revised Brooke date	DD-MM-YYYY	X	X
	(Revised Brooke Scale of upper extremity function)			
14.38	MFM score	[Numerical value]	X	X
14.39	MFM date	DD-MM-YYYY	X	X
	(Motor Function Measure)			
14.40	6MWT score	[Numerical value]	X	X
14.41	6MWT date	DD-MM-YYYY	X	X
	(Six-Minute Walk Test)			
14.42	10MWT score	[Numerical value]	X	X
14.43	10MWT date	DD-MM-YYYY	X	X
	(Ten-Metre Walk test)			
14.44	TUG score	[Numerical value]	X	X
14.45	TUG date	DD-MM-YYYY	X	X
	(Timed Up & Go Test)			

14.46	EK2 score	[Numerical value]	X	X
14.47	EK2 date	DD-MM-YYYY	X	X
	(Egen Klassification)			
14.48	Observed WHO score	[Numerical value]	X	X
14.49	Observed WHO date	DD-MM-YYYY	X	X
	(World Health Organisation motor development milestones)			
14.50	CHOP-ATEND score	[Numerical value]	X	X
14.51	CHOP-ATEND date	DD-MM-YYYY	X	X
	(Children's Hospital of Philadelphia Adult Test of Neuromuscular Disorders)			
14.52	Other validated measure for adult-onset SMA (specify)	[Free text] (Can add multiple)	X	X
14.53	For each measure named in 14.52: score	[Numerical value]	X	X
14.54	For each measure named in 14.52: date	DD-MM-YYYY	X	X

Section 15: PATIENT-REPORTED OUTCOMES (PRO)

Item no.	Data item description	Coding	Baseline	Follow-up
15.00	[CR] CGI-S (Clinical Global Impression of Severity) – Baseline only *Clinician's rating of this patient's current severity of illness; based on the clinician's total clinical experience with the relevant population*	1=Normal, not at all ill; 2=Borderline ill; 3=Mildly ill; 4=Moderately ill; 5=Markedly ill; 6=Severely ill; 7=Among the most extremely ill patients.	X	
15.01	Date of Clinician CGI-S score given in 15.00	DD-MM-YYYY		
15.02	Total Global Impression (TGI) <u>according to patient or parent</u>: *How does the patient/parent feel that the patient's condition has changed in the last 6 months?*	1=Very much improved; 2=Much improved; 3=Minimally improved; 4=No change; 5=Minimally worse; 6=Much worse; 7=Very much worse	X	X
15.03	Date of Patient TGI score given in 15.02	DD-MM-YYYY	X	X

15.04	[CR] Total Global Impression (TGI) according to clinician – follow-up only *How does the clinician feel that the patient's condition has changed in the last 6 months?*	1=Very much improved; 2=Much improved; 3=Minimally improved; 4=No change; 5=Minimally worse; 6=Much worse; 7=Very much worse		X
15.05	[CR] Date of Clinician TGI score given in 15.04	DD-MM-YYYY		X
15.10	[CR] Was any other validated PRO taken for this patient at this visit?	Yes; No	X	X
	If 'Yes' to 15.10: Provide relevant details:			
15.11	PedsQL (NM & fatigue scales) score	[Numerical value]	X	X
15.12	PedsQL (NM & fatigue scales) date	DD-MM-YYYY	X	X
	(Paediatric Quality of Life Inventory)			
15.13	PEDI-CAT score	[Numerical value]	X	X
15.14	PEDI-CAT date	DD-MM-YYYY	X	X
	(Pediatric Evaluation of Disability Inventory - Computer Adaptive Test)			
15.15	SMA FRS score	[Numerical value]	X	X
15.16	SMA FRS date	DD-MM-YYYY	X	X
	(Spinal Muscular Atrophy Functional Rating Scale)			
15.17	ACEND score	[Numerical value]	X	X
15.18	ACEND date	DD-MM-YYYY	X	X
	(Assessment of Caregiver Experience with Neuromuscular Disease)			
15.19	ACTIVLIM score	[Numerical value]	X	X
15.20	ACTIVLIM date	DD-MM-YYYY	X	X
	(ACTIVLIM measure of activity limitations)			
15.21	DISABKIDS score	[Numerical value]	X	X

15.22	DISABKIDS date	DD-MM-YYYY	X	X
	(DISABKIDS measurement of quality of life and level of distress)			
15.23	Other validated PRO (specify)	[Free text]	X	X
15.24	For each PRO named in 15.23: score	[Numerical value]	X	X
15.25	For each PRO named in 15.23: date	DD-MM-YYYY	X	X

Section 16: ELECTROPHYSIOLOGY AND BIOMARKERS

Item no.	Data item description	Coding	Baseline	Follow-up
16.00	Has the patient had a CMAP (Compound Muscle Action Potential) scan?	Yes; No; Don't know	X	X
16.01	Has the patient had a DEXA (Dual Energy X-ray Absorptiometry) scan?	Yes; No; Don't know	X	X
16.02	Has the patient had any muscle imaging undertaken?	Yes; No; Don't know	X	X

4. Feedback, Harmonisation & Revisions

The TREAT-NMD Global SMA Registry is one of several notable data collection initiatives in operation across the world. Harmonisation and comparability across these different data repositories is vital to ensure that the collected data meets the current and future needs of the SMA community.

Considerable work has gone into ensuring that the data collected by the TREAT-NMD registries, through this first iteration of the expanded core dataset, will be comparable with the data collected by other initiatives. However, many of these initiatives are still in development, and in addition to this, global consensus on the most appropriate and relevant data to collect is expected to continue to evolve over the coming years.

The TGDOC are committed to the continued harmonisation of the TREAT-NMD SMA Core Dataset with other data collection initiatives, and to the evolution of the dataset in response to the needs of the SMA community. Therefore, an **annual revision plan** is proposed, which will allow suggested revisions to be accepted throughout the year, and considered and implemented (if appropriate) on an annual basis.

The first annual revision will be planned for September 2019 and further details will be circulated as soon as they are available. Please note, we do not anticipate that these changes will be significant, and we are equally keen to limit the burden on the registries who will need to implement any changes made.

If you have any feedback on the dataset, please contact joanne.bullivant@newcastle.ac.uk.

5. Data Sharing & Publications

Data Sharing

Third parties may request a report on data held in the TREAT-NMD Global SMA Registry, by submitting a global enquiry to the TREAT-NMD Secretariat. Only de-identified, aggregate data is included in enquiry reports.

If an enquiry is approved (via vote) by the TGDOC, the relevant SMA registries are asked to provide the relevant data. The data that the registries provide is de-identified. When the national registries submit data to the central TREAT-NMD secretariat team, it is cleaned and compiled into a report for the third party.

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. All contributions from our partners towards the TREAT-NMD Global SMA Registry should be appropriately acknowledged wherever and whenever relevant. To this end, TGDOC are forming a Publications Committee and have initiated an audit of all previous publications using data from the Global SMA Registry. The first priority task for the Publications Committee will be to develop and have ratified a TREAT-NMD Global Registries Publications Policy.

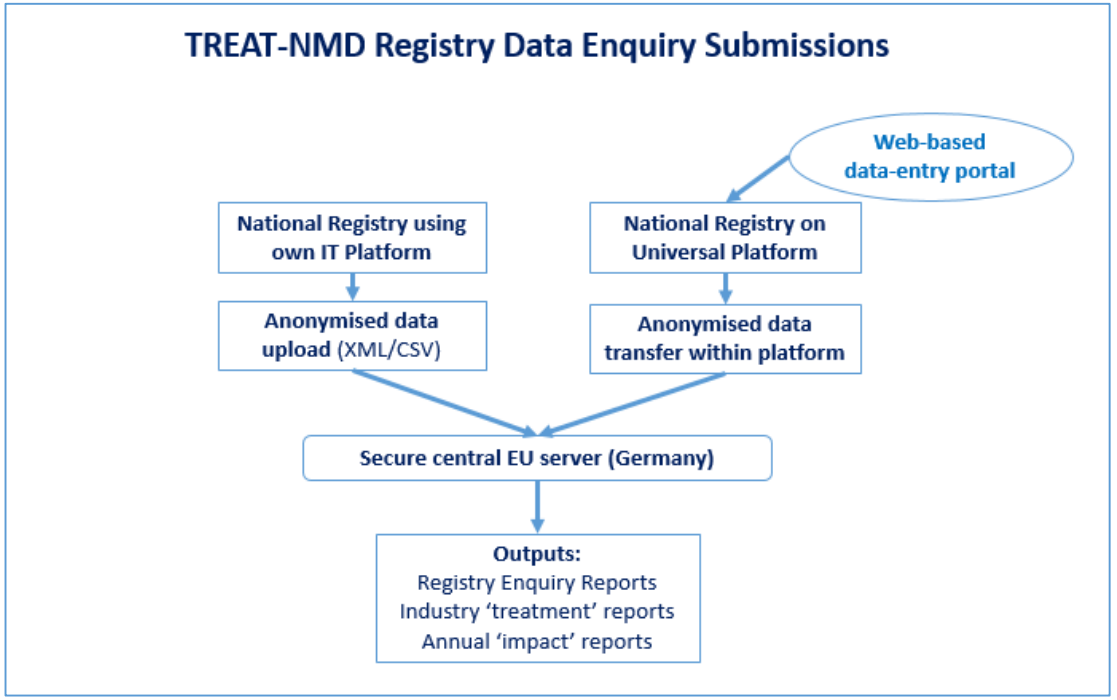
If you would like to be involved in the Publications Committee, please contact the TGDOC Chair-elect, Craig.Campbell@lhsc.on.ca.

6. TREAT-NMD Universal Registry Platform

A significant difficulty for many TREAT-NMD Registries in the adoption of the expanded SMA Dataset is the lack of a suitable IT platform. Therefore, in parallel to the SMA Dataset Expansion, a custom-built IT platform (the TREAT-NMD Universal Registry Platform) is under development, which is provided by healthcare software specialists OpenApp.

The expanded TREAT-NMD SMA Dataset will be pre-built into the Universal Platform, so for SMA registries who wish to use it to host their registry, it will support the capture, management, and reporting of high quality, harmonised patient data. For registries who do not wish to use the Universal Platform to host their registry, they may still submit their data for global enquiries by uploading it in a pre-agreed format, in a commonly used file type, e.g. Excel or CSV.

The Universal Platform will be independently owned and operated by TREAT-NMD. Further information on the different options available to registries is under careful development, alongside clear policies and agreements on governance and data protection. This information will be made available as soon as it is finalised.



7. Contacts & Acknowledgements

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The International SMA Consortium (iSMAC) who have collaborated to support data collection harmonisation by sharing their work.

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