Introduction:
The TREAT-NMD Alliance is a global, independent network governed by an Executive Committee of academics, clinicians and patient representatives facilitating collaborative research in neuromuscular disease (NMD).

Having developed a range of infrastructures, the network aims to accelerate drug development, provide new therapies to patients swiftly and improve standards of diagnosis and care. One such infrastructure is the Global Registry Network, a federated network of independent, national (or regional) patient registries that together collect data on 9,758 Spinal Muscular Atrophy (SMA) patients from 35 countries worldwide.

Utilising the Global Registry Network to capture real world evidence (RWE) to support Post-Marketing Surveillance (PMS) studies is of increasing interest to stakeholders.

Results:
To facilitate collection of longitudinal data suitable for PMS, a working group was established that included clinicians, patient representatives, and registry curators to produce a TREAT-NMD Expanded Core SMA Dataset.

Dataset items and a data dictionary were agreed through stakeholder engagement and reviewed to evaluate the feasibility of data collection and implementation into registry platforms.

The initial expanded dataset included 167 data items. A review generated 700 feedback points from registries, clinicians, and other stakeholders. This feedback, coupled with a FAIR data analysis and an IT and dataset modelling exercise, led to the finalised TREAT-NMD Expanded Core SMA Dataset released in October 2020.

This reduced the total data items to 154, increasing standardisation and harmonisation with international standards, and also defined permitted values and structure for data items. The finalised dataset is FAIR-compatible, and has an increased focus on RWE and Patient-Reported Outcome Measures to support future PMS studies.

15 registries within the Global Registries Network have fully adopted the expanded dataset and 16 are working toward adoption. Registries report improved data quality and utilisation; however, adoption of the expanded dataset was challenging for many registries. A formalised, dataset review process has now been established to minimise future disruption. This will combine continuous feedback collection with regular biannual review to ensure that changes to the dataset can be easily adopted by registries, while supplying the RWE needed to support PMS studies and other enquiries.

Conclusions:
Provision of RWE by patient registries is key for PMS studies. The Expanded Core SMA Dataset and Global Registry Network ensure that TREAT-NMD is well-placed to provide this data. A key criteria for joining the Global Registry Network is collection of all mandatory data items. Recognising the challenges of adopting an expanded dataset, TREAT-NMD offers registry bursaries and the option of using a freely available Global Registry Platform including the expanded dataset.

The dataset review process must provide a clearly defined pathway to future dataset updates. This will provide relevant and useable data while limiting the burden on registries. Since identical data items appear across TREAT-NMD datasets, a standardised revision approach will minimise impact on registries collecting data across NMDs.

TREAT-NMD Core Dataset for SMA: A Consensus-Driven Approach to Support Post-Marketing Surveillance

The TREAT-NMD Expanded Core Dataset for SMA includes a list of fields, data dictionary and example questions) is freely available to any organisation/system collecting data on SMA and is suitable for patient- or clinician-reported registries and compatible with Privacy Preserving Record Linkage (PPRL) tools. The dataset’s content and example screenshots are shown on the right.

TREAT-NMD SMA Dataset

The TREAT-NMD Expanded Core Dataset for SMA (which includes a list of fields, data dictionary and example questions) is freely available to any organisation/system collecting data on SMA patients.

The dataset is suitable for patient- or clinician-reported registries and compatible with Privacy Preserving Record Linkage (PPRL) tools. The dataset’s content and example screenshots are shown on the right.

### SMA Dataset Contents

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Scan to download full details of the dataset

Figure 1

A. The TREAT-NMD SMA Global Registry Network includes 15 registries (dark blue shows registry country across 4 continents. Countries shaded light blue show prospective members. Image: mapchart.net. B. Breakdown of FSHD member registries by data collection model, and type of organisation responsible for registries.