Background

The TREAT-NMD Global Network of SMA Registries (n=52+) collect a common core dataset and are governed by the TREAT-NMD Global Database Oversight Committee (TGDOC). Researchers and industry can request anonymized and aggregated data via the committee, offering a single point of access to this diverse and extensive dataset.

The TREAT-NMD SMA core dataset containing 23 data items was established in 2008 for clinical trial readiness and is now used for regulatory submissions, clinical trials, observational studies, and routine clinical care for SMA. The expanded dataset highlights the power of the global registries, with substantial numbers of registered patients across the globe. To date, registries note concerns with specific data items including feasibility and standardized collection of motor outcome assessments, particularly for those not on therapy, as well as hospitalizations, and SAE’s. 27 out of 52 registries reported a TREAT-NMD internal survey request.

TREAT-NMD Network SMA Patient Characteristics

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Results

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Methods

Based on an initial pilot study of 12 TREAT-NMD SMA registries (both clinician- and patient-reported), and a mapping and scoping exercise across all SMA registries a plan was developed to implement the expanded SMA dataset across all 52 TREAT-NMD SMA registries over a 3 year time frame. The phased implementation allows for feedback on feasibility, helping develop strategies to mitigate issues and concerns for future implementation.

Currently, year 1 of implementation includes 8 national registries in addition to the pilot 12 who are launching and collecting the expanded dataset. Feedback throughout the process highlights individualized needs for each participating nation, including platform requirements, ethics and consent for an amended dataset, and standardization across clinical centres.

Conclusion

The expansion of the SMA dataset for safety and effectiveness highlights the extensive collaborative work across the community. The roll-out of the expanded data set across all 52 TREAT-NMD SMA registries is taking place over a 3 year project. In order to support data collection of the expanded SMA dataset, TREAT-NMD is developing an IT platform for affiliated registries to use either directly, or to upload data from their own platform. Annual dataset reviews will be undertaken to assess the continued feasibility and relevance of data items, and additional support required across registries to ensure high quality data collection, while limiting increased burden on clinicians, research staff, or patients.

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Example Data Output from Expanded Dataset

TREAT-NMD SMA Registries Expanded Dataset Collection

Motor measures collected in SMA

Half of registries have implemented collection of motor measures, however the majority of registries are yet to implement collection of patient reported outcome measures (PROMs).