



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Executive Director

Letter of support for TREAT-NMD Core Dataset for Spinal Muscular Atrophy (SMA)

On 07/03/2022, the Applicant Treat-NMD requested qualification advice on their Core Data registry for Spinal Muscular Atrophy for regulatory purposes.

During its meeting held on 4 -7 April SAWP agreed on a discussion meeting held during the SAWP on 7-10 June, whereafter the SAWP agreed on the advice to be given to the Applicant on 1 September 2022. During its meeting held on 12-15 September 2022, the CHMP adopted the advice to be given to the Applicant. This letter of support is issued based on the review of the qualification advice.

The TREAT-NMD Core Dataset for SMA is a registry which is admitting data from local or smaller SMA registries to be selectively combined and used in aiding drug development regarding pre- and post-authorisation clinical studies, besides natural history studies and other real-world data/evidence. The TREAT-NMD offers existing registries an interface to collect data in SMA in a systematic and standardized manner which is stored in an overarching Core Data Warehouse (CDW).

Analysis of these real-world data from the CDW is considered valuable for academic and pharmaceutical research, trial feasibility and planning, and for informing regulatory decision-making. It may support an improved understanding of disease progression in treated and untreated patients, allow long-term evaluation of safety and efficacy for existing or new treatments, inform clinical trial design and outcome measure selection, facilitate and disseminate better standards of care in SMA as well as inform pre- and post-authorisation regulatory decisions.

The development of the CDW is ongoing and the overarching registry is being filled with data. There are 25 core member registries collecting the SMA core dataset and sharing data with TREAT-NMD for enquiries, and a further 18 registries are working towards this. Currently, the data in the registry is limited to four SMA registries who are sharing data in the CDW encompassing data from circa 250 subjects that have been entered. It is however anticipated that, across all member SMA registries involved, there will be over 8,000 patients with genetically confirmed SMA, with a further 2,500 subjects with a clinical diagnosis of SMA awaiting genetic confirmation.

The concept and infrastructure of the TREAT-NMD-SMA data framework is clear and has a high potential to allow retrieving data that may address many potential questions, not least given the anticipated magnitude of the SMA registry. In the absence of concrete data, or concrete questions currently being addressed, it is difficult to assess at this stage whether the CDW databank potentially



could address the intended contexts of use mentioned. The Applicant is invited to seek follow-up qualification advice once a specific study protocol addressing specific contexts of use and once real-world evidence data is available. This would allow an assessment whether the data in the CDW in principle can meet its anticipated contexts of use i.e., monitoring disease progression in treated and untreated patients, long term evaluation of safety and efficacy of existing or new treatments, facilitation of clinical trial design and clinical outcome selection, improvement of standards of care and support to pre- and post-authorisation regulatory decisions.

In conclusion, the EMA acknowledges the efforts of the Applicant in establishing an overarching disease-based registry in SMA and has issued this Letter of Support to encourage the further development and validation of the TREAT-NMD Core Dataset for Spinal Muscular Atrophy. Whilst it is considered that a lack of concrete examples in relation to any particular context of use (including regulatory) precludes a qualification opinion at this stage, EMA is prepared to consider a future submission with a concrete feasible plan supported by sufficiently detailed protocols and procedures for a well-defined context of use.

Yours sincerely,

Emer Cooke
Executive Director