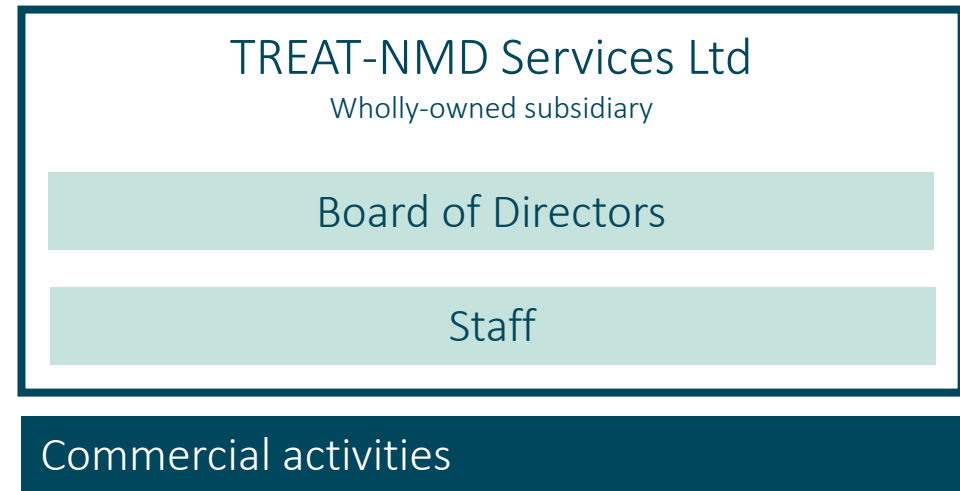
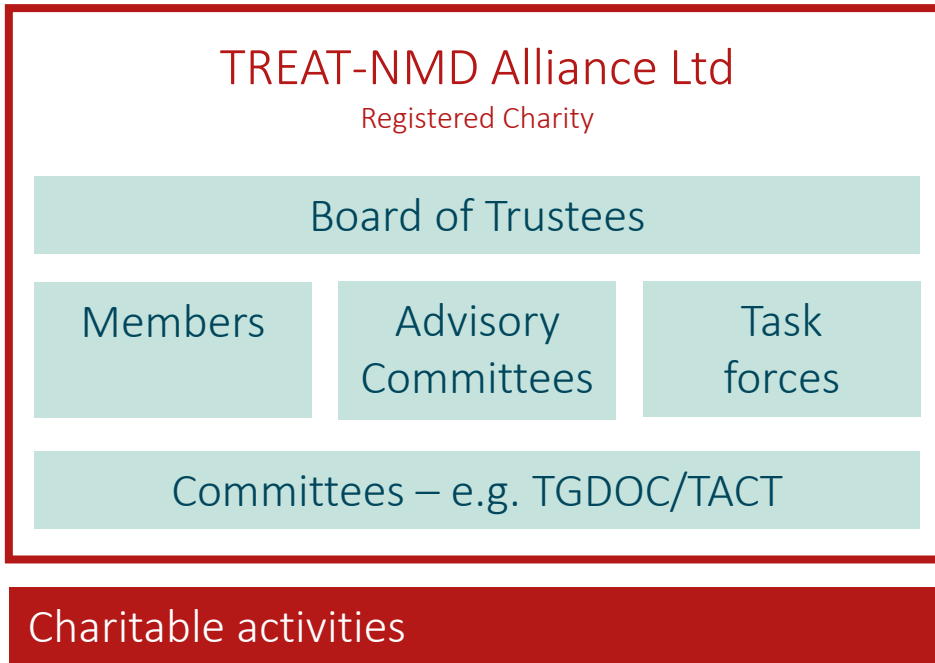


TREAT-NMD and EMA Registry Qualification

TREAT-NMD

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



Our vision

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

Our mission

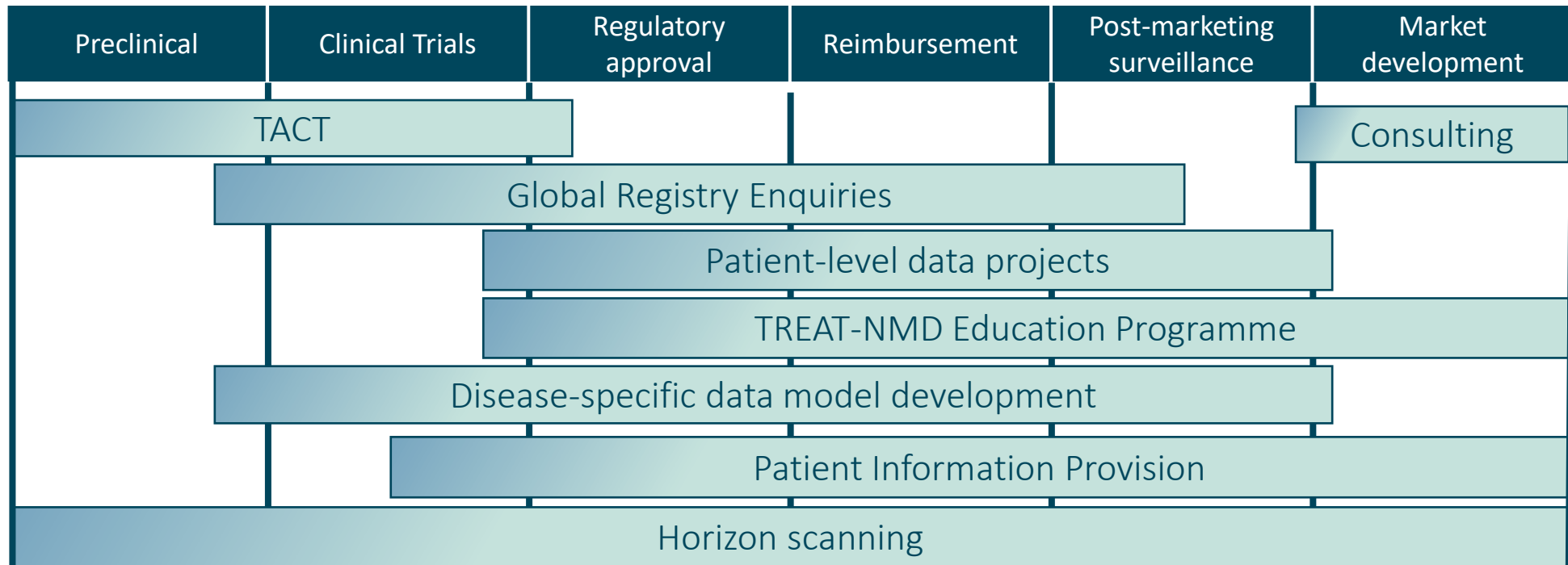
To operate a collaborative, inclusive global network and organisational infrastructure that will overcome fragmentation, providing support services, information and data to advance treatment, diagnosis and care for neuromuscular patients globally.

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

De-risking and accelerating drug development

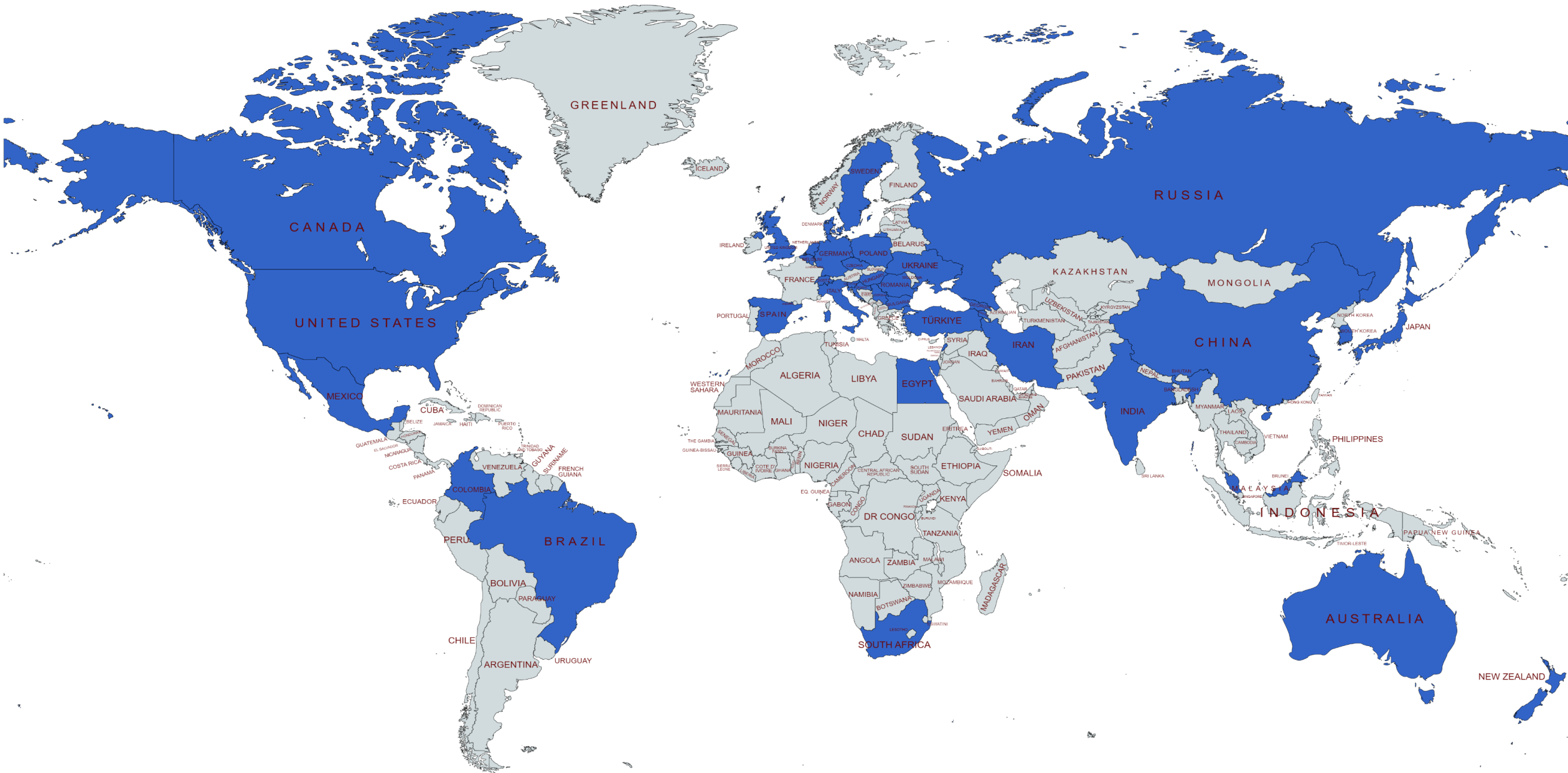
TREAT-NMD has several complimentary work streams that support drug development and bring new treatments to patients as quickly as possible



TREAT-NMD Global Registry Network...

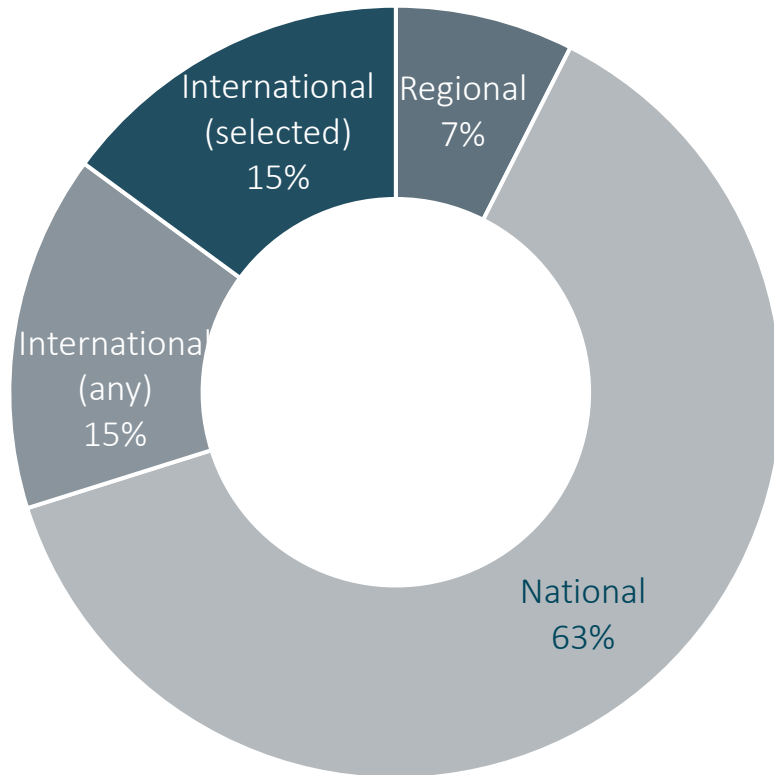


The network

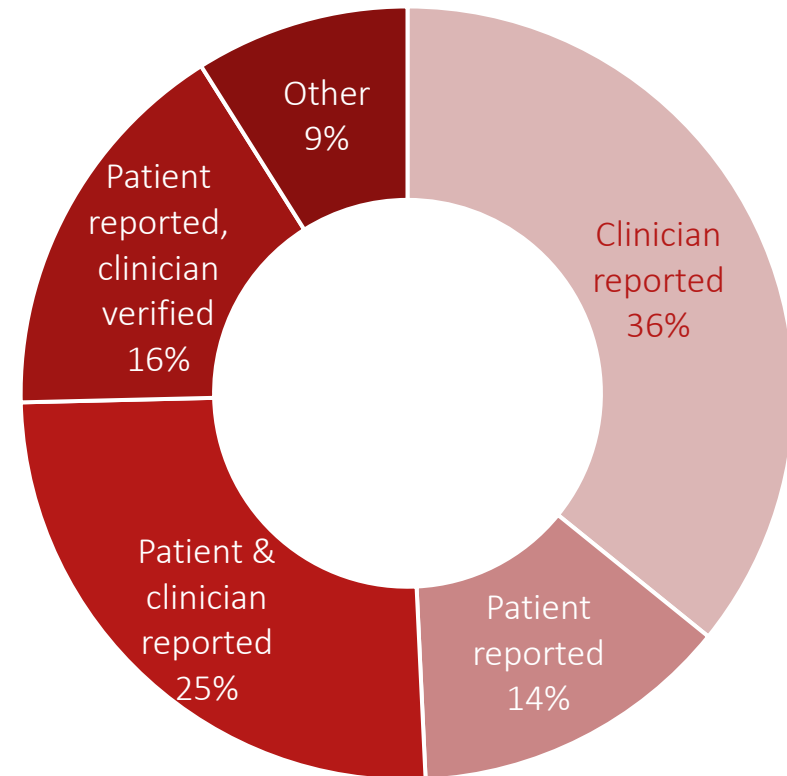


Registries within the network

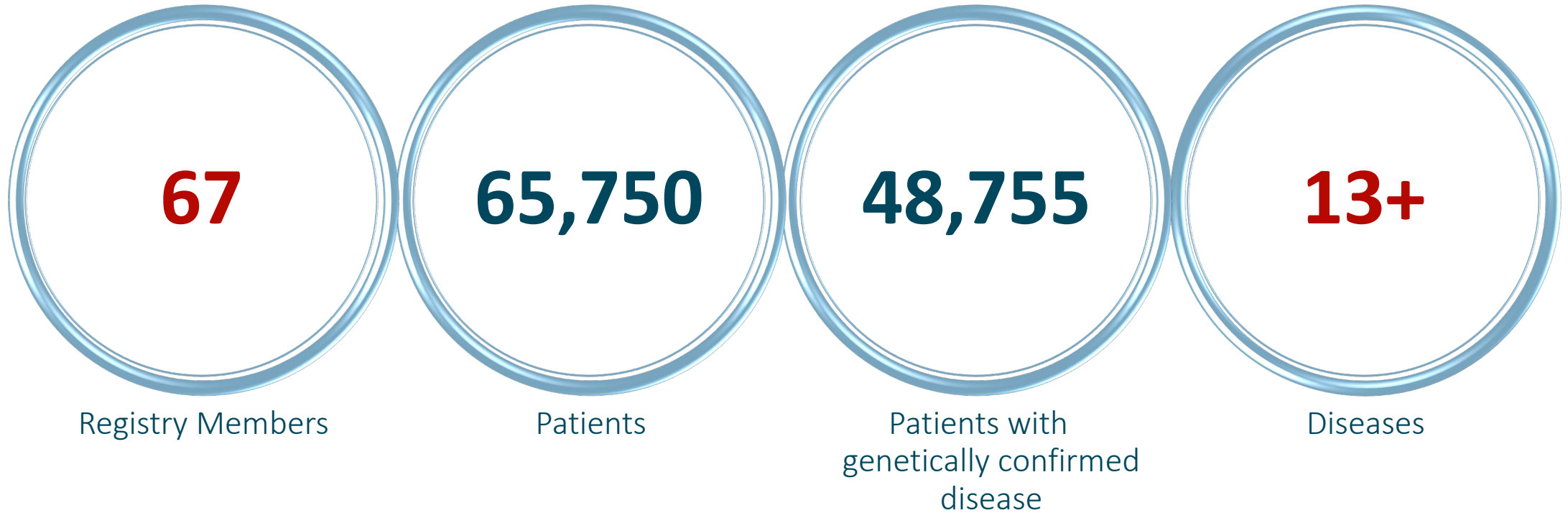
Geographic Reporting Boundaries



Registry Reporting Type



The network in numbers



Our qualification journey...

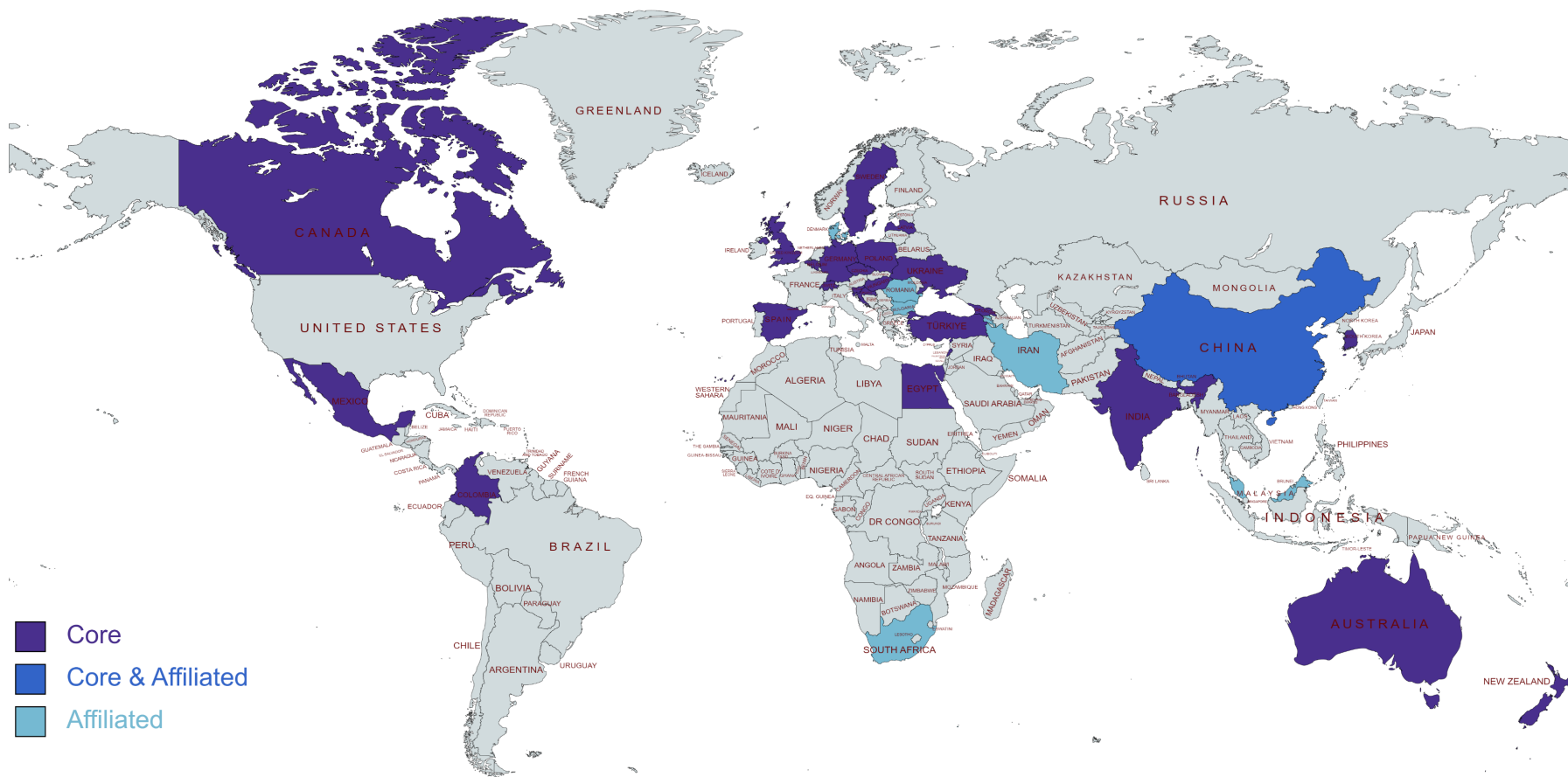
What to apply for?

A single disease: SMA

- Most mature expanded core dataset
- Treatments available, so most data requirements
- TREAT-NMD infrastructure most advanced

Infrastructure, data and processes

The network in numbers



37

Registries

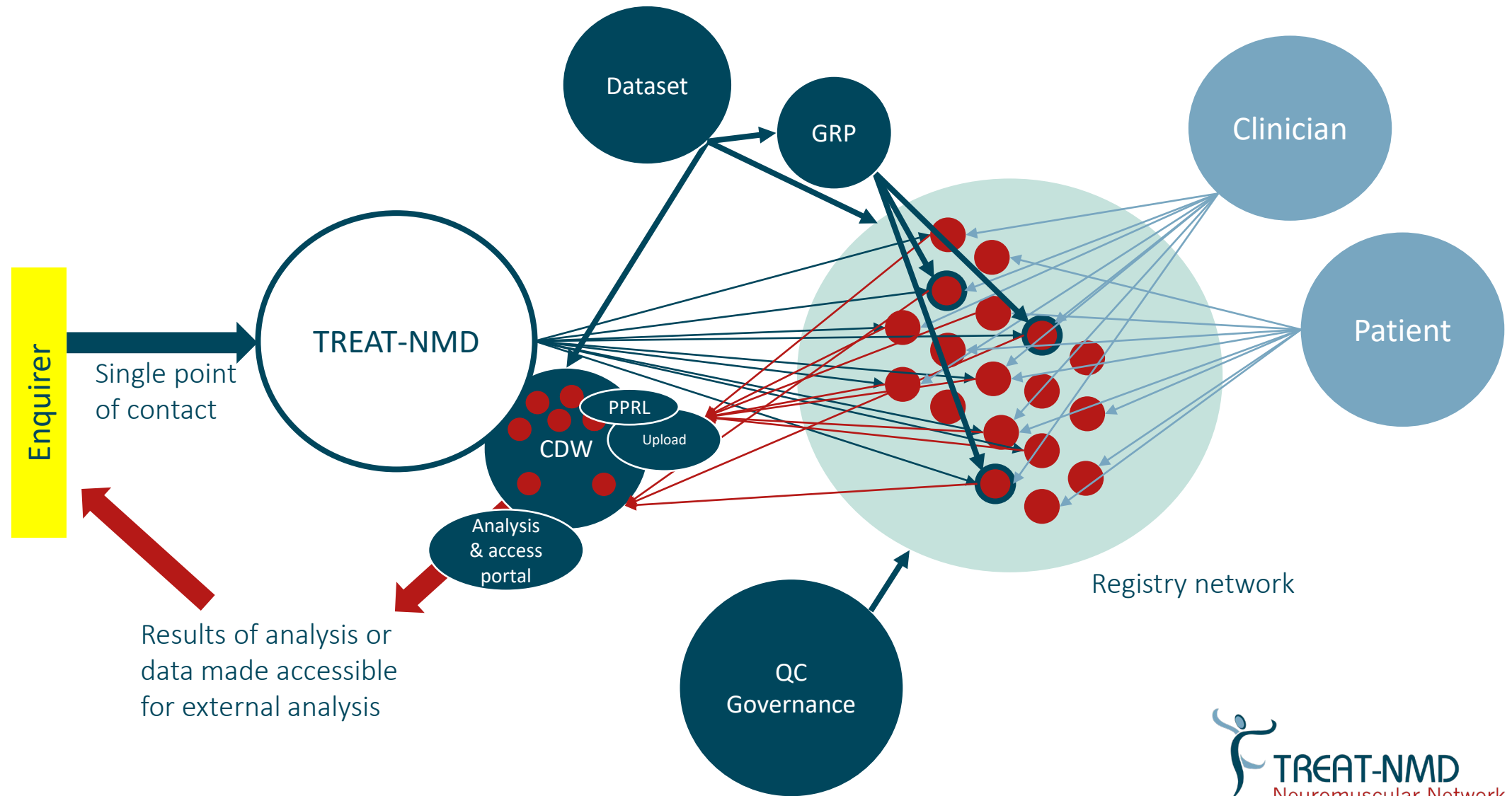
9,276

patients

7,735

genetic diagnosis

TREAT-NMD and EMA Qualification



TREAT-NMD and EMA Qualification

Seeking advice on...	In...	To provide data for...
TREAT-NMD Global Registry Platform	SMA	Post marketing efficacy studies
TREAT-NMD Central Data Warehouse		Natural history provision
TREAT-NMD Dataset		Clinical trial control arm data
Data held in CDW		Validation of outcome measures
The federated network model		

In 2022, TREAT-NMD completed the first step of the Qualification process: Scientific Advice

TREAT-NMD

Seeking advice

TREAT-NMD

TREAT-NMD

TREAT-NMD

Data held in

The federated

In 2022, TREAT-NMD



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

8 December 2022
Doc ref: EMADOC-1700519818-958775
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).

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tific Advice

TREAT-NMD
Neuromuscular Network

Thank you