TREAT-NMD Myotonic Dystrophy Global Registry Network: Providing Data in Congenital Myotonic Dystrophy to Support FDA Regulatory Decision

Campbell C, Porter B, Walker H, Bennett N, Ashley E-J, Esparis B, Allison D, Guglieri M, Ambrosini A, Stevenson T, Cumming K, Marini-Bettolo C, Rodrigues M, Hodgkinson V, Korngut L, Forbes R, Ryan M, Snape M, Evans S, Horrigan J, Peric S, Roxburgh R

On behalf of TREAT-NMD Myotonic Dystrophy Subgroup & TREAT-NMD Global Registry Network. [2]AMO Pharma Ltd

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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 aims

- 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.

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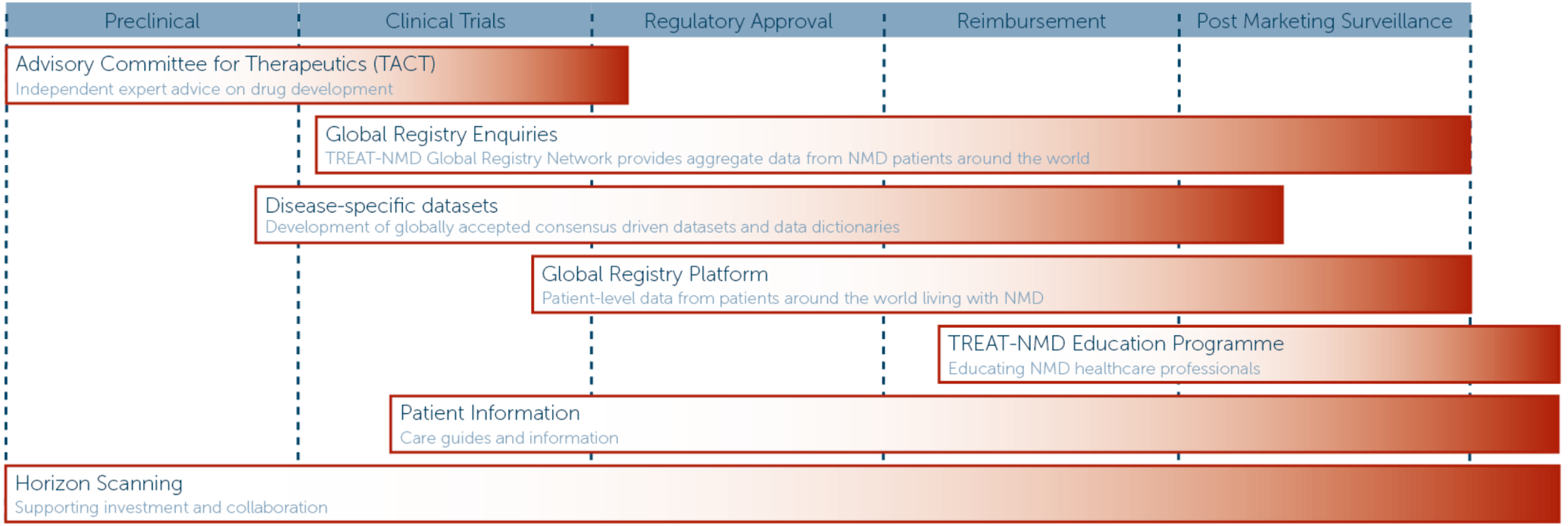
De-risking and accelerating drug development

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



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Background

The TREAT-NMD Myotonic Dystrophy Global Registry Network

The TREAT-NMD Global Registry Network is governed by the TREAT-NMD Data Oversight Committee

The DM Global Registry Network consists of24 registries

- Collecting data on approximately 8,250 patients (6,288 with a genetic diagnosis)

Each registry collects

- A TREAT-NMD disease-specific dataset
- Other data items decided at a registry level

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Method

In 2019, AMO Pharma contacted TREAT-NMD to request data to support an application to the FDA for a Rare Paediatric Disease (RPD) Designation

Little data was available on the prevalence of congenital myotonic dystrophy. The request asked for:

- Details on the prevalence of congenital myotonic dystrophy (cDM1) in patients aged 18 or younger
- Data from registries in 5 countries (USA, Canada, UK, New Zealand & Australia)
- Data on patients where the congenital basis of the disorder was based on self-report from caregivers and patients and verified by clinicians.

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Results

In 2019, AMO Pharma contacted TREAT-NMD to request data to support an application to the FDA for a Rare Paediatric Disease (RPD) Designation

The registries reported 270 patients with congenital myotonic dystrophy (cDM1)

- 148 (54.8%) of patients were aged 18 years or younger
- Percentage of patients ranged from 42.1% in Australia to 71.4% in New Zealand
- AMO Pharma used the data to successfully support their application for a RPD Designation from the FDA for tideglusib

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Conclusions

In 2019, AMO Pharma contacted TREAT-NMD to request data to support an application to the FDA for a Rare Paediatric Disease (RPD) Designation

An RPD designation provides a priority review voucher

Registries can be used in all stages of drug development, including providing data to support regulatory applications where data are scarce

cDM1 is an important subset of DM1 with its own research needs and opportunities

DM1 registries need to collect specific data relevant to these patients to support such activities.

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