

# Charter for the Australian Neuromuscular Disorders Registry

This charter is a statement of the scope, objectives and participants in the activities of the Australian Neuromuscular Disorders Registry (ANMDR). It provides a delineation of roles and responsibilities, outlines the objectives, defines the governance structures and identifies the members of the National Neuromuscular Disorders Registry Advisory and international partners. The charter serves as a reference of authority for the future activities of the Registry.

This charter has been developed in consultation with members of the ANMDR Advisory Committee and draws from the 'TREAT-NMD Charter for TREAT-NMD patient database/registry'<sup>1</sup>.

## Context for the ANMDR within the changing healthcare landscape

Neuromuscular disorders (NMD) include a range of disorders including Duchenne (DMD) and Becker muscular dystrophy, Myotonic dystrophy, Spinal muscular atrophy and other neuropathies. These disorders are associated with a range of symptoms from muscle weakness or atrophy to pain or numbness and abnormal muscle movement that can become progressively more severe. The disorders may involve difficulty with swallowing, speech or breathing and other organ systems such as the cardiac or urinary systems<sup>2</sup>.

A survey of the literature estimated the overall prevalence of neuromuscular disorders (including rare forms) in both males and females at approximately 1 in 3000<sup>3</sup>. Although this is relatively uncommon the impact of NMD is enormous with the burden of disease being grossly disproportionate to its frequency.

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<sup>1</sup> TREAT-NMD Neuromuscular Network, Charter for the TREAT-NMD patient database/registry. [http://www.treat-nmd.eu/downloads/file/registries\\_toolkit/Charter\\_Global\\_Registry\\_Revised\\_090312\\_FINAL1.pdf](http://www.treat-nmd.eu/downloads/file/registries_toolkit/Charter_Global_Registry_Revised_090312_FINAL1.pdf) Date Accessed: May 9 2012.

<sup>2</sup> Amato AA and Russell JA, 2008. Neuromuscular disorders. McGraw-Hill Medical.

<sup>3</sup> Emery A. Population frequencies of inherited neuromuscular diseases - a world survey. *Neuromuscular Disorders* 1991; 1:1: 19-29.

The burden of disease for all muscular dystrophies, of which DMD is the most common, has been reported to be \$435 million per year for health care, or \$126,000 per affected person<sup>4</sup>. The value of lost well being (disability and premature death) is estimated to cost a further \$1 billion annually<sup>5</sup>. The per case Disability Adjusted Life Years lost for DMD is greater than that of any National Health Priority Area, including cancer and multiple sclerosis<sup>6</sup>.

Advances in scientific knowledge have the potential to greatly benefit patients, their families, and the community. In Australia, despite several centres being involved in research on, and management of, individuals with DMD, there is insufficient coordination to ensure the translation of new knowledge into improved patient outcomes. Many countries, facing the same issues as Australia have established a National Registry for Duchenne Muscular Dystrophy and are planning further registries for Spinal muscular atrophy and other neuromuscular disorders. These countries are now forming a network to create a global registry for each condition to be able to systematically and more quickly address the significant unmet needs for new therapeutic strategies.

This global network of national registries links to one another through TREAT-NMD (Translational Research in Europe for the Assessment and Treatment of Neuromuscular Diseases) Alliance. The TREAT-NMD Alliance is a network which creates a non-identified database of patients worldwide enabling multi-centre international clinical trials to find people appropriate for the therapy or intervention being investigated.

## **1. Purpose of the ANMDR Charter**

a. This charter shall:

- Regulate the interaction and sharing of data between the ANMDR and the TREAT-NMD Alliance global database and between the ANMDR and third parties (such as pharmaceutical or industrial and academic or research institutes interested in using the database for clinical trials or research).

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<sup>4</sup> Report by Access Economics for the Muscular Dystrophy Association Access (2007)

<sup>5</sup> Report by Access Economics for the Muscular Dystrophy Association Access (2007)

<sup>6</sup> Report by Access Economics for the Muscular Dystrophy Association Access (2007)

- Form a key part of any access or data-sharing agreements between ANMDR and the TREAT-NMD Alliance, and between ANMDR and third parties (such as pharmaceutical or industrial and academic institutions interested in using the database for clinical trials or research).
- Be made publicly available on the ANMDR website.

## **2. Definitions**

- a. Patient registries and databases are structured as searchable data collections of individuals with a shared characteristic such as a disease or a gene defect.
- b. National registry refers to a registry that aims to enlist the majority of patients in a given region, country or several countries.
- c. TREAT-NMD (Translational Research in Europe for the Assessment and Treatment of Neuromuscular Diseases) Alliance is a network of excellence established through the European Commission funding under contract 036825. Partners relates to institutions and organizations as defined in the EC contract.
- d. TREAT-NMD Alliance national registries are the national registries that are organized under the TREAT-NMD Alliance and pledge to adhere to the TREAT-NMD Alliance charter.
- e. The TREAT-NMD Alliance global database is a meta-database that compiles data transferred from the national registries.

## **3. Objectives of ANMDR**

- a. Collect personal, clinical and molecular genetic data on people living with neuromuscular disorders.
- b. Provide aggregate data to the TREAT-NMD Alliance of national registries on Australians with neuromuscular disorders.
- c. Improve opportunities for international collaboration by facilitating and accelerating recruitment of Australian patients into new clinical trials.
- d. Improve care of neuromuscular patients through the coordination of diagnosis and therapies by ensuring intervention strategies are available in an equitable and consistent manner across Australia.

#### 4. Activities of ANMDR

The ANMDR stores, processes, and uses the data to:

- contribute to the TREAT-NMD Alliance global database and through this database enable eligible Australian patients to participate in international clinical trials recruited through TREAT-NMD Alliance;
  - provide a national conduit through which best practices and guidelines for the management of Neuromuscular disorders may be shared with clinical providers;
  - provide a national database for Australian researchers investigating Neuromuscular disorders;
- a. The ANMDR collects and processes data according to Australian laws and best practices (e.g. the Commonwealth *Privacy Act 1988* and the National Health and Medical Research Council, Australian Research Council and Australian Vice-Chancellors' Committee *National Statement on Ethical Conduct in Human Research 2007*) and update the data at least every 12 months.
  - b. The ANMDR will retain custodianship of the data and will grant access, re-utilization or permit extraction or grant any right in accordance with this custodianship and in accordance with the specific objectives stated above in (3.).
  - c. ANMDR will share the following information with patients, clinical and genetic service providers:
    - data transferred to TREAT-NMD Alliance;
    - details of trials and research projects recruiting through TREAT-NMD Alliance;
    - information on research projects granted access to the ANMDR;
    - information on other TREAT-NMD Alliance activities;
    - information on results with direct relevance to patients; and
    - any other way to return benefit in accordance with the ethical principal of benefit sharing.
  - d. An access agreement may/will be drawn up between ANMDR and a third party requesting access to the registry on the basis of the requirements of this charter and of the Australian Neuromuscular Disorders Registry Advisory Committee (ANMDR Advisory Committee).

## 5. Relationship of ANMDR with TREAT-NMD Alliance

- a. The ANMDR will provide data to the TREAT-NMD Alliance global database for non-exclusive use. Prior to transfer the data will be coded or aggregated into raw data. Only aggregated or coded data will be transferred to TREAT-NMD Alliance. The transferred data will only include a standardised set of core data as specified by TREAT-NMD Alliance. Mutations will be annotated in accordance with the Human Genome Variation Society (<http://www.hgvs.org/>). The TREAT-NMD Alliance global database will not receive identified data from the ANMDR and will not be able to contact patients directly. Patients determined to be eligible will be contacted by the ANMDR through their nominated clinicians.
- b. A document on the standardised mandatory and desirable data fields to collect and the items to be transferred is attached to the charter (Attachment 1).
- c. ANMDR will refer inquiries by third parties for access for international multi-centre studies to the TREAT-NMD Alliance database. It is mandatory for inquiries for multicentre international clinical trials to go exclusively through TREAT-NMD Alliance, inquiries for access only to ANMDR are excluded from this.
- d. Financial compensation by third parties for services of the TREAT-NMD Alliance global database will be shared between TREAT-NMD Alliance and national registries such as ANMDR, in proportion to the data utilised by the third parties. This compensation will also take into account the effort incurred including the degree of data curation. TREAT-NMD Alliance will negotiate on behalf of the national registries with third parties. A fee structure for such services will be developed by the TREAT-NMD Alliance global database oversight committee (TREAT-NMD Alliance GDOC) and approved by the TREAT-NMD Alliance Governing Board and national registries, and will be reviewed on a regular basis.

Any funds received by ANMDR from TREAT-NMD Alliance from third parties for services will be used for the management and upkeep of the registry.

- e. The TREAT-NMD Alliance website displays information about the activities of the TREAT-NMD Alliance global database and informs patients and the public about contributions made to the TREAT-NMD Alliance global database, including those of the ANMDR.
- f. ANMDR displays the TREAT-NMD Alliance logo on the website and on any documentation. TREAT-NMD Alliance may display the ANMDR name and logo on the TREAT-NMD Alliance website.
- g. ANMDR is a signatory on the TREAT-NMD Alliance Charter and as such must adhere to the TREAT-NMD Alliance Charter, contribute to the TREAT-NMD Alliance global database, and in return has a seat and voting rights on the TREAT-NMD Alliance global database oversight committee.
- h. ANMDR will endeavour to develop registries with interoperability with other known networks of National registries and will strive to use a common data elements

## **6. Relationship of ANMDR with third parties**

- a. Third parties will not be given direct access to patients or identifiable data under any conditions.
- b. The ANMDR will grant access to encrypted data only to third parties under the following conditions:
  - The access is approved by the ANMDR Advisory Committee.
  - Institutional ethics approval is provided/obtained.
  - Specific ethics approval is provided/obtained (e.g. WA Health ethics approval obtained for linkage to Department of Health databases.).
  - The study is not in conflict with ANMDR objectives.
- c. An Access Agreement is authorised by the third party and the National Curator, with copies to be held by the National Curator and circulated to the members of the ANMDR Advisory Committee.
- d. The access agreement will include acknowledgement that data derived from the ANMDR and TREAT-NMD Alliance global database may be used for registering medicinal products through the TGA, FDA and EMEA.

- e. Access by research and academic institutions will be provided free of charge. Any publications must acknowledge support by ANMDR and TREAT-NMD Alliance.
- f. Access by commercial companies will be charged a service fee agreed to by the company and the ANMDR Advisory Committee.
- g. All parties agree with the ethical principle of benefit sharing, which requires that benefits resulting from any scientific research and its applications should be shared especially with the persons and groups that have taken part in the research.
- h. Individuals whom unwittingly or knowingly cause breaches of privacy or personal health information legislation will be subject to the legislation in their jurisdiction

## **7. Australian Neuromuscular Disorders Registry Advisory Committee**

- a. The ANMDR Advisory Committee is the governing body responsible for the ANMDR.
- b. The ANMDR Advisory Committee is composed of representatives from each of the jurisdictions in Australia and New Zealand and includes a range of neurologists, researchers, geneticists, support organisation representatives and parent representatives. Membership of the committee is dynamic and reflects the professional diversity appropriate for informing the registry needs of specific NMDs. The chairperson of the ANMDR Advisory Committee is the National Curator of the ANMDR. There are no industry representatives on the committee.
- c. All members of the ANMDR Advisory Committee must disclose conflicts of interests and update the disclosure statements on an annual basis.
- d. All members of the ANMDR Advisory Committee will be required to sign confidentiality agreements if a third party requests them prior to having access to the inquiry of the third party.
- e. The ANMDR Advisory Committee will meet by teleconference or in person at least once per year, and upon request.
- f. The ANMDR Advisory Committee will report to ANMDR stakeholders annually.
- g. The ANMDR Advisory Committee will review all applications for access to the ANMDR. All inquiries for global/international studies will be referred to the

TREAT-NMD Alliance GDOC. For inquiries specific to the ANMDR the ANMDR Advisory Committee will come to a decision within 30 calendar days upon receipt of the inquiry and will report the decision in writing to the third party, and the TREAT-NMD Alliance GDOC as per the approval process agreed by the GDOC.

If the ANMDR Advisory Committee cannot reach a decision the inquiry will be rejected. In the case of a rejection, the ANMDR Advisory Committee will report the reason for rejection of access and allow reconsideration of the application.

#### Australian National Neuromuscular Disorders Registry Advisory Committee

Dr Hugh Dawkins (Chair, National Curator)	Office of Population Health Genomics, Department of Health
Dr Anita Cairns (QLD)	Royal Children’s Hospital & Mater Children’s Hospital Brisbane
Dr Damian Clark (SA)	Women’s and Children’s Hospital Adelaide
Dr Alastair Corbett (NSW)	Concord Repatriation General Hospital
Mark Davis (WA)	Neurogenetics Unit, Royal Perth Hospital
Desiree Du Sart (VIC)	Murdoch Institute, Royal Children’s Hospital
Dr Michelle Farrar (NSW)	Neurology Department, Sydney Children’s Hospital
John Gummer (WA)	Muscular Dystrophy Foundation
Dr Emma Hammond	Office of Population Health Genomics, Department of Health
Dr Graeme Hammond-Tooke (NZ)	
Dr Val Hyland (QLD)	Molecular Genetic Unit, Royal Brisbane Hospital
Dr Alexa Kidd (NZ)	
Dr Phillipa Lamont (WA)	Neurology, Royal Perth Hospital
Lesley Murphy (WA)	Parent

Dr Kathryn North (NSW)	Institute for Neuromuscular Research, Children's Hospital at Westmead
Deborah Robins (QLD)	Duchenne Foundation
Miriam Rodrigues (NZ)	Muscular Dystrophy Association New Zealand
Dr Peter Rowe (WA)	Neurology, Princess Margaret Hospital for Children
Dr Monique Ryan (VIC)	Neurosciences Department, The Royal Children's Hospital Melbourne
Boris Struk (VIC)	Muscular Dystrophy Association
Dr Peter Taylor (NSW)	South Eastern Area Laboratory Services, Prince of Wales Hospital
Dr Sui Yu (SA)	Department of Genetic Medicine, Women's and Children's Hospital Adelaide

## 8. Validity of the charter

- a. This charter will take effect following its endorsement by the ANMDR Advisory Committee at a scheduled teleconference or meeting.
- b. Endorsement of the charter by the ANMDR Advisory Committee will be noted in the agenda and minutes from the relevant meeting.
- c. The charter will be valid for the duration of the ANMDR. The charter can be subject to change by the ANMDR Advisory Committee at any time without prior notification.

## 9. Ethical and legal principles

- a. The ethical guidelines endorsed by the TREAT-NMD Alliance Network:
  - World Medical Association, Declaration of Helsinki (June 1964, amended in Tokyo in 2004) Council for International Organizations of Medical Sciences, International Ethical Guidelines for Biomedical Research Involving Human Subjects (CIOMS 2002)
  - HUGO (Human genome organization), Statement on benefit sharing (April 2000)
  - UNESCO International Declaration on Human Genetic Data (16 October 2003)

- UNESCO Universal Declaration on Bioethics and Human Rights (19 October 2005)
- b. Some of the binding laws applicable to the activities of the TREAT-NMD Alliance databases:
- Council of Europe, Convention N° 108 for the Protection of Individuals with regard to Automatic Processing of Personal Data
  - Directive 95/46/EC of the European Parliament and of the Council of 24 October 1995 on the protection of individuals with regard to the processing of personal data and on the free movement of such data
  - Directive 96/9/EC of the European Parliament and of the Council of 11 March 1996 on the legal protection of databases
- c. Australian ethical principles applicable to the ANMDR:
- NHMRC National Statement on the Ethical Conduct of Human Research
  - NHMRC Databases Protocol

## **Revision history**

Draft 1.0 by Leanne Youngs, 23 August 2012.

## **Endorsement:**

Australian Neuromuscular Disorders Registry Advisory Committee at teleconference Tuesday July 17<sup>th</sup> 2012.