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The TREAT-NMD Care and Trial Site Registry

A Powerful Tool for Clinical Research on Neurodegenerative and Neuromuscular Diseases

Introduction

One of the major organizational hurdles to overcome before initiating a clinical trial is identifying those trial sites capable of recruiting enough patients and offering a specific standard of care and experience in clinical trials. The concept of establishing a Care and Trial Site Registry providing information on personnel, facilities and patient population was born to help pharmaceutical industry and clinical investigators select trial sites, and to help to identify potential partners for upcoming research.

Background

Established in 2007 in the scope of the TREAT-NMD project (EU-funded Network of Excellence, FP6) to identify possible trial sites for rare neuromuscular diseases, the Care and Trial Site Registry (CTSR) collects information on personnel and the experience of the study team, facilities and equipment, as well as patient numbers per disease and age group.

Within the CARE-NMD project (2010-2013, funded by DG Sanco) the CTSR was extended with Duchenne-specific care questions and used to evaluate current clinical practice in different European countries.

In September 2013 the CTSR expanded to cover the field of rare neurodegenerative diseases as a branch of NeurOmics (FP7, 2012-2017) and now encompasses 32 rare diseases.

Content

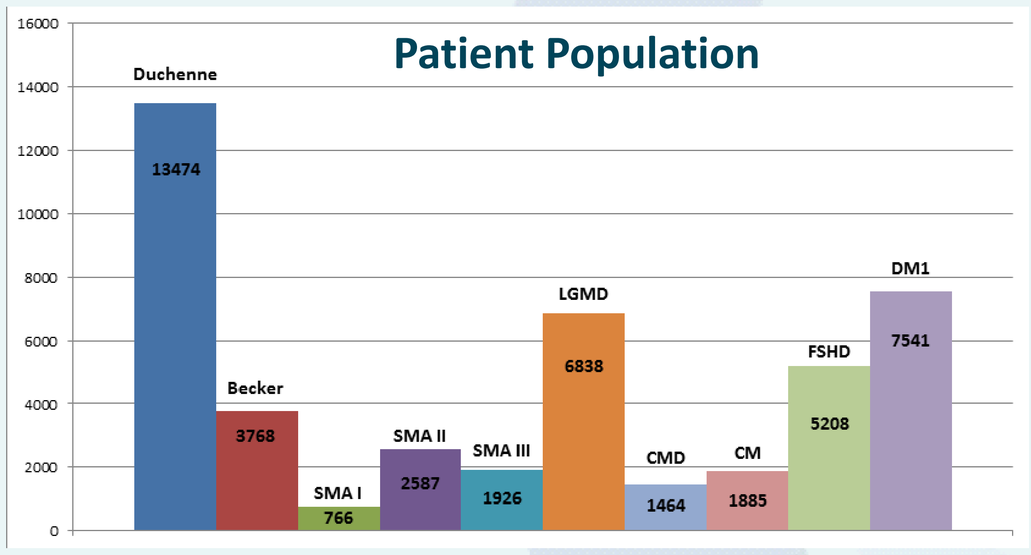
The CTSR is an online self-registration database hosted by the University Medical Center Freiburg, Germany. Data is entered into online forms organised by topic categories such as

- Patient Cohort
- Diagnostic Tools
- Personnel and Experience
- Equipment
- Care Settings
- Research and Education

A new facility in the CTSR is the **PhenoSearch** – it offers the possibility to search for colleagues who are treating patients with a similar phenotype.

Some statistics

As of January 2014 the CTSR contained 310 centres in 45 countries with an overall count of 50,400 reported patients. Since the introduction of the neurodegenerative diseases in September 2013, over 40 NDD centres, caring for 3000 patients, have registered.



The three countries with the largest number of registered centers are Germany (67), the UK (46), and the United States (42), totaling 48% of sites. 55% have a clinical trials unit available.

Various diagnostic possibilities may be queried from the CTSR database: for example, for Duchenne muscular dystrophy, Multiplex Ligation-dependent Probe Amplification (MLPA) is the minimum capability recommended in international consensus care standards. This is available and funded in 69% of reporting sites, with the more advanced ability to detect point mutations available and funded at 62% of these sites.

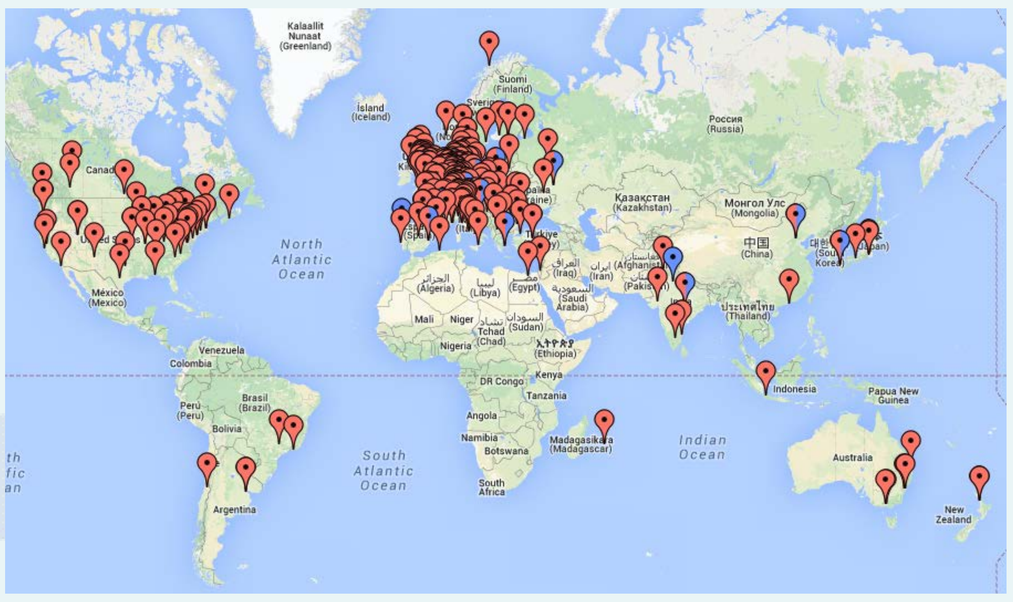
For more information please see the paper on the CTSR that was recently published in the Orphanet Journal of Rare Diseases (Rodger et al., 2013).

Usage

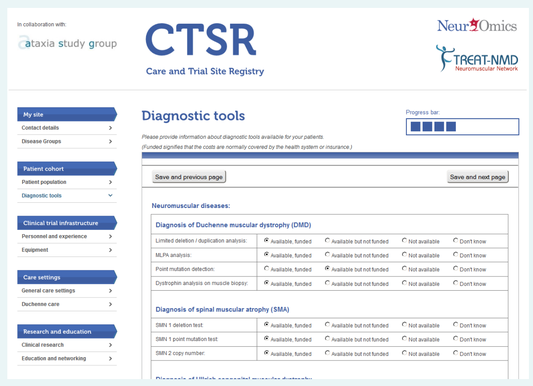
Fourteen official requests for site identification, often in combination with TREAT-NMD patient registry information, were received between 2009 and 2013. 10 trial feasibility enquiries were received from major industrial firms such as PTC, Lilly, Santhera, Acceleron, Trophos, Prosensa or Ultragenyx.

Four enquiries were received from academia. For example, for the FOR-DMD steroid trial the CTSR was used to collect relevant feasibility information from all participating sites.

310 registered sites worldwide



In 2012 the CTSR helped the Muscular Dystrophy Campaign in United Kingdom to conduct an audit of UK sites to gather accurate baseline data on current neuromuscular care.



The CTSR is openly accessible for registration on the world wide web:

https://ctsr.uniklinik-freiburg.de

Conclusion

Registries of care and trial sites have significant utility for research into rare conditions such as neuromuscular diseases, demonstrated by the interest of industry and other researchers in the CTSR. This approach may be applicable to other fields needing to identify centres of expertise with the potential to carry out clinical research and engage in clinical trials.

Such registries may also help build networks of centres of expertise which fit the criteria of EUCERD for European Reference Networks (ERNs).

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NeurOmics

TREAT-NMD Neuromuscular Network