



Background

One of the major hurdles to overcome before initiating a clinical trial is identifying those sites capable of recruiting enough patients and offering specific standards of care and experience in clinical trials. The concept of creating a Care and Trial Site Registry to provide information on patient population, personnel and facilities was born to help pharmaceutical industry and clinical investigators select trial sites.

The CTSR was established in 2007 as one branch of TREAT-NMD, an EU-funded 'network of excellence' whose goal it is to improve treatments and find cures for patients with **neuromuscular disorders** (www.treat-nmd.eu). It is hosted by the University Medical Centre Freiburg.

The CTSR is a flexible database and has been continuously expanded with questions e.g. on clinical research activities and care settings (as part of the EU-funded CARE-NMD project www.care-nmd.eu)

Funded by the European Union Neuromics project (www.rd-neuromics.eu) the CTSR was expanded in 2013 to include **neurodegenerative centres**.

Currently the sites registered in the CTSR care for more than 41,000 patients. 3,500 of these belong to the neurodegenerative diseases, which were introduced into the CTSR in September 2013.

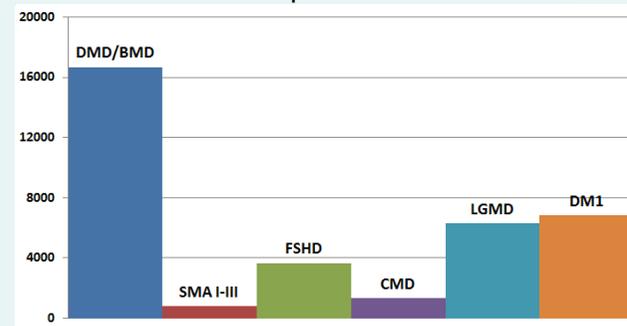
The Care and Trial Site Registry today

As of January 2014, 310 centres across the world have registered. The database has attracted significant interest from industry and academia as a registry of potential sites for multi-centre clinical trials. 14 official requests for site identification, often in combination with TREAT-NMD patient registry information, were received between 2009 and 2013.

The CTSR has also become a valuable resource for an international network of centres treating many types of neuromuscular -- and also now neurodegenerative -- disorders. It can also be used to disseminate therapy recommendations and to evaluate current clinical practice on a national or international basis.

Recently, a phenotype search facility 'Pheno-Search' has been introduced. This allows researchers to put out a call to registered centres for patients fitting a particular clinical profile. It is hoped that this will speed up the identification of new disease causing mutations and better stratification of patients.

Total number of NMD patients seen at CTSR 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): www.ojrd.com/content/8/1/171

Information gathered in the CTSR

General Site Information

- Name of institution, potential principal investigator and contact details

Patient Cohort

- Number of patients specified by age for over 25 diseases
- Available diagnostic tools

Clinical Trial Infrastructure

- Personnel and experience
- GCP training
- Equipment

Care Settings

- Members of interdisciplinary team
- Arrangements for transition from paediatric to adult care
- Pulmonary and cardiologic care
- Disease specific questions (e.g. steroid treatment for Duchenne)

Research and Education

- Participation in clinical trials
- Peer-reviewed publications
- Participation in networks
- Training activities

If you would like to see the complete questionnaire, please go to

<https://zks-internet.ukl.uni-freiburg.de/ctsr/>

and click 'View the questionnaire'

Benefits for registered sites

- Increase publicity of your centre among patients and clinicians
- Potential participation in clinical trials and research projects
- Participation in an international network of neuromuscular and neurodegenerative centres
- Receive information about care recommendations and new developments
- Post a 'phenotype search' to identify particular patients

Benefits for industry and researchers

- Use an existing network for your research project
- Use the CTSR as a single point of access for feasibility information
- Improve recruitment through combination of trial sites and patient registry data

How to register as a neuromuscular or neurodegenerative centre

The CTSR is a password protected database. Registration is free of charge and you can define for what purpose your data is used. Once you have provided your data it is easy to log-in later and update your information as needed.

To sign up, please go to

<https://zks-internet.ukl.uni-freiburg.de/ctsr/>



For technical issues or if you are not sure if your centre has already registered, please contact ctsr@uniklinik-freiburg.de.

TREAT-NMD: www.treat-nmd.eu
Neuromics : www.rd-neuromics.eu

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Care and Trial Site Registry (CTSR)

A worldwide database of neuromuscular and neurodegenerative centres