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Role of the Care and Trial Sites Registry

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Registry of outcome measures

Training and education

Clinical Trial Coordination Centre

Global patient registries

Communication infrastructure

Regulatory information

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Advisory committee for therapeutics

Care and trial site registry

Standards for animal assessment

Care standards

Ethical framework

EuroBioBank

Infrastructure that developed from the CTCC

Clinical Trials Coordination Centre

- WP 05.1 Design an Implement the CTCC
  - Give support in planning, initiation, conducting, and closing of clinical trials in neuromuscular disorders
  - Training investigators for clinical trials in neuromuscular disorders
  - Establish a network a trial sites for neuromuscular disorders
- WP 05.2 Format all the knowledge on national legal regulations and overcome regulatory hurdles
  - Regulatory affairs database for regulations in different countries
  - Discussions with EMEA/FDA on protocol design and outcome measure in neuromuscular disorders

Support in planning and conducting clinical trials

- Discussions with several companies about their clinical trials
- Assisting Trophos with trial setup for SMA trial
  - Writing protocol and site selection visits
- NIH-funded steroid trial
  - Trial preparation and conduct together with U of Rochester und U of Newcastle

Training investigators for Clinical Trials

- Annual workshops on clinical trials in neuromuscular disorders and other rare diseases
  - Most interest from countries that want to be more involved in clinical trials in neuromuscular disorders
- GCP-Training for Investigators
- Lectures on clinical trial readiness at different workshops (ENMC and others)

Establish a network of trial sites

- Setting up a database with feasibility information from sites interested to participate in clinical trials
- Online database with individual user account and possibility to expand questionnaire and update local information regularly
- Contains information on personnel, patient cohort, facilities, experience with clinical trials etc.

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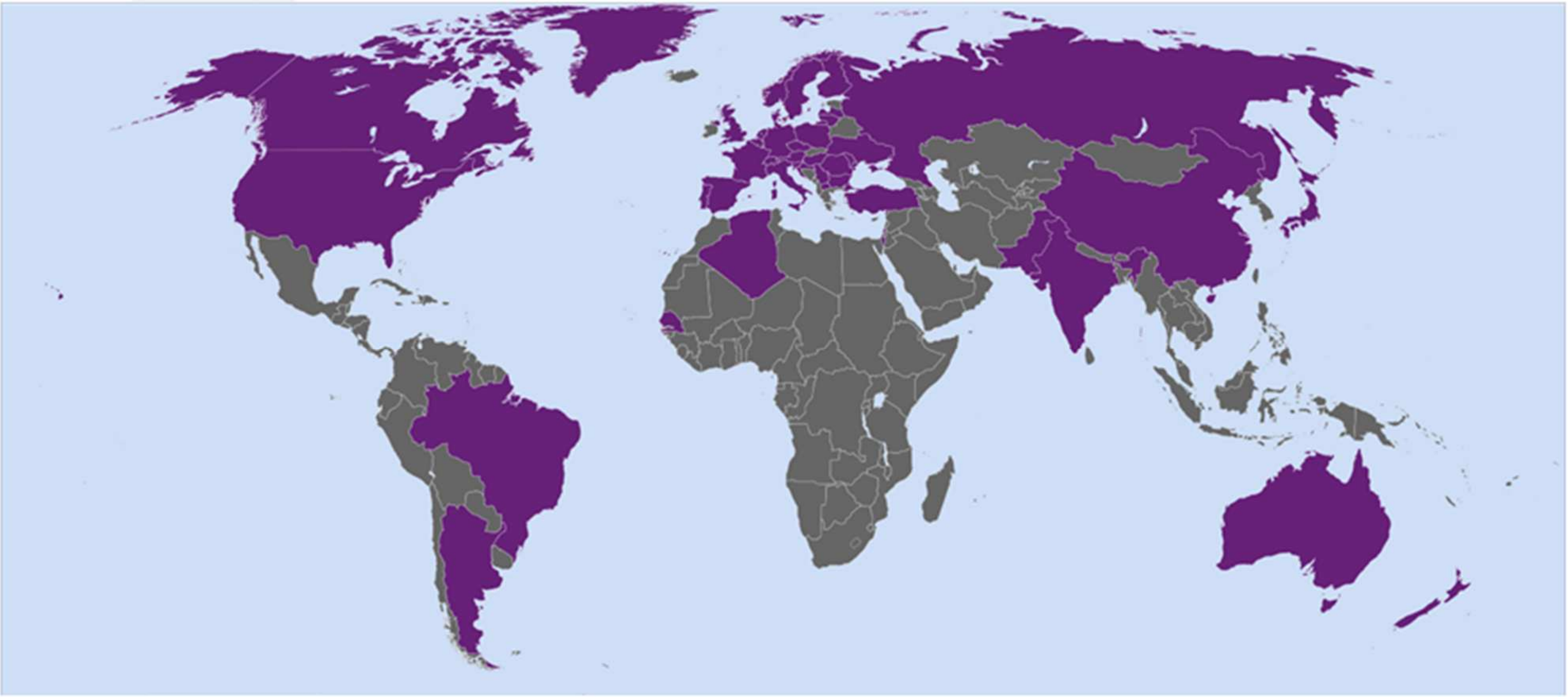
- Extension into a CARE and Trial Site Registry (CTSR)

Content of the CTSR

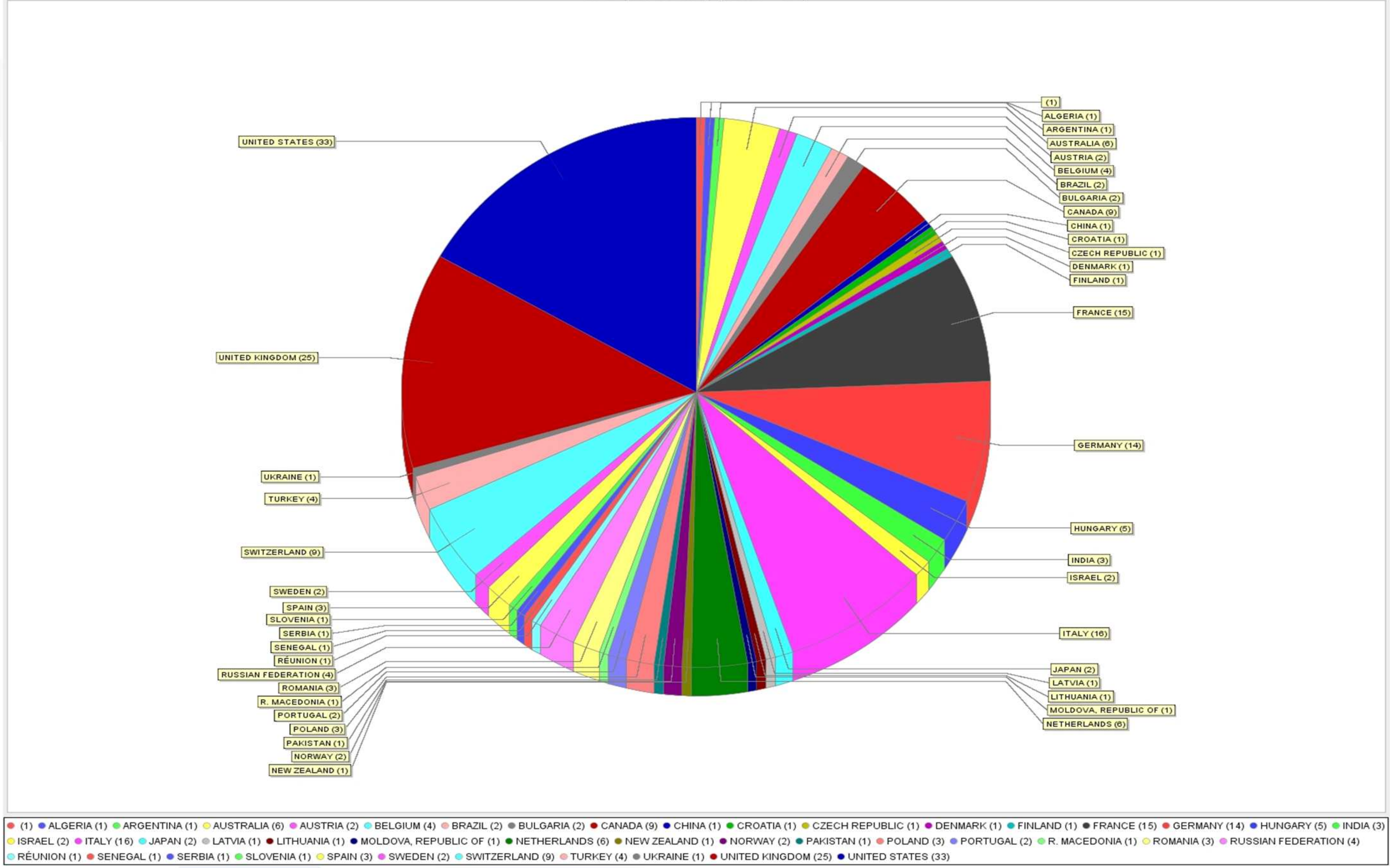
- Contact data for clinical centre
- Patient cohort characteristics
  - Number of patients in each disease and age group (Duchenne, Becker, LGMD, Ullrich, SMA, Myotonic Dys …)
- Diagnostic techniques
  - Genetic testing, muscle biopsy, echo, DEXA, lung function…
- Local infrastructure
  - Personnel: Study nurses, physiotherapists, investigators …
  - Equipment: Intensive care unit, freezer, computer …
- Experience with clinical trials
  - Participation in Phase I, II, III trials, GCP training
  - Participation in existing networks (MDA, NorthStar, MD-NET…)

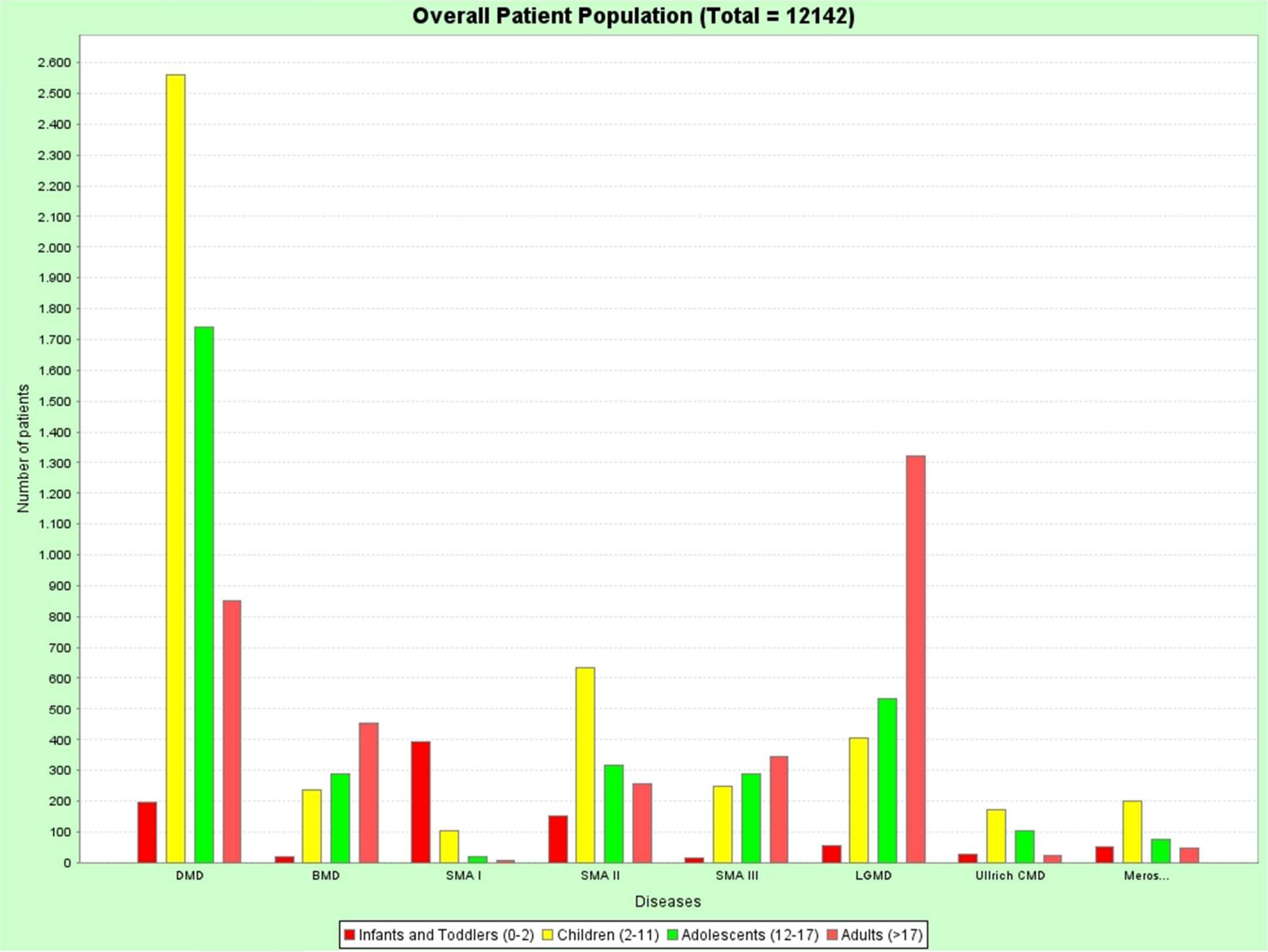
Care and trial sites



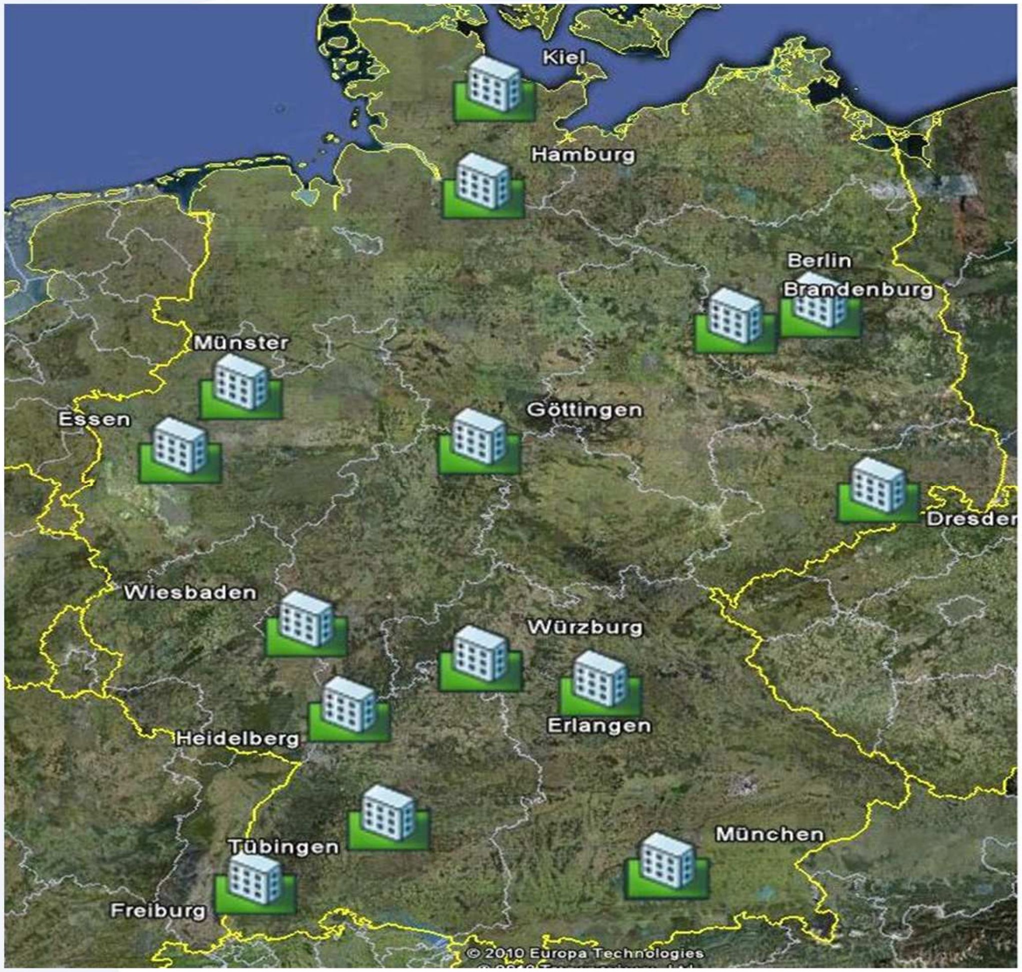
~ 200 sites

Sites per country (Total = 197)




Overall Patient Population (Total = 12142)
Number of patients
2.600
2.500
2.400
2.300
2.200
2.100
2.000
1.900
1.800
1.700
1.600
1.500
1.400
1.300
1.200
1.100
1.000
900
800
700
600
500
400
300
200
100
0
DMD
BMD
SMA I
SMA II
SMA III
LGMD
Ullrich CMD
Meros...
Diseases
Infants and Toddlers (0-2)
Children (2-11)
Adolescents (12-17)
Adults (>17)

Location of CTSR centres



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How the CTSR is used for clinical trials

1. Investigator (industry or academic) interested in trial sites (and patient registries) for planning or conducting a clinical trial
2. Defining the selection criteria for potential trial sites and setting up an agreement, defining financial compensation
3. Add items to the database if necessary, request for update
4. Electronic search in the database to produce a list of centres fulfilling the selection criteria, requesting missing information
5. Ask selected sites for permission to forward information to investigator
6. Provide investigator with a list of potential trial sites (often in combination with information from patient registries)

CTSR has been used for

- Prosensa/GSK exon skipping trials for DMD
- Trophos trial for spinal muscular atrophy
- Acceleron
- FOR-DMD (NIH funded trial on steroid use in DMD)

CTSR beyond clinical trials

- Worldwide Network of clinical centres caring for patients with NMD
- Information of patient cohorts and infrastructure
- Direct was to inform clinicians about care guidelines, clinical trials and other relevant developments
- CTSR might be used for cooperation with national care networks, reference centres
- CTSR is already used for the CARE-NMD project to improve care for patients with DMD

Summary and Perspectives

- Care and Trial Site registry powerful tool for clinical trials and potential basis for a network of care centres
- CARE-NMD project will help to develop the care aspect of the CTSR and expand the use of registries

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Summary and Perspectives

- Care and Trial Site registry powerful tool for clinical trials and potential basis for a network of care centres
- CARE-NMD project will help to develop the care aspect of the CTSR and expand the use of registries
- Advisory group is planned to discuss further development of the CTSR and its integration into existing infrastructure in the area of neuromuscular disorders and rare diseases

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www.treat-nmd.eu/trialsites