TREAT-NMD Neuromuscular Network

Patient Registries and Biobanks: Who has access and who has ownership of the data?

Emma Heslop

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TREAT-NMD: an infrastructure for the neuromuscular field

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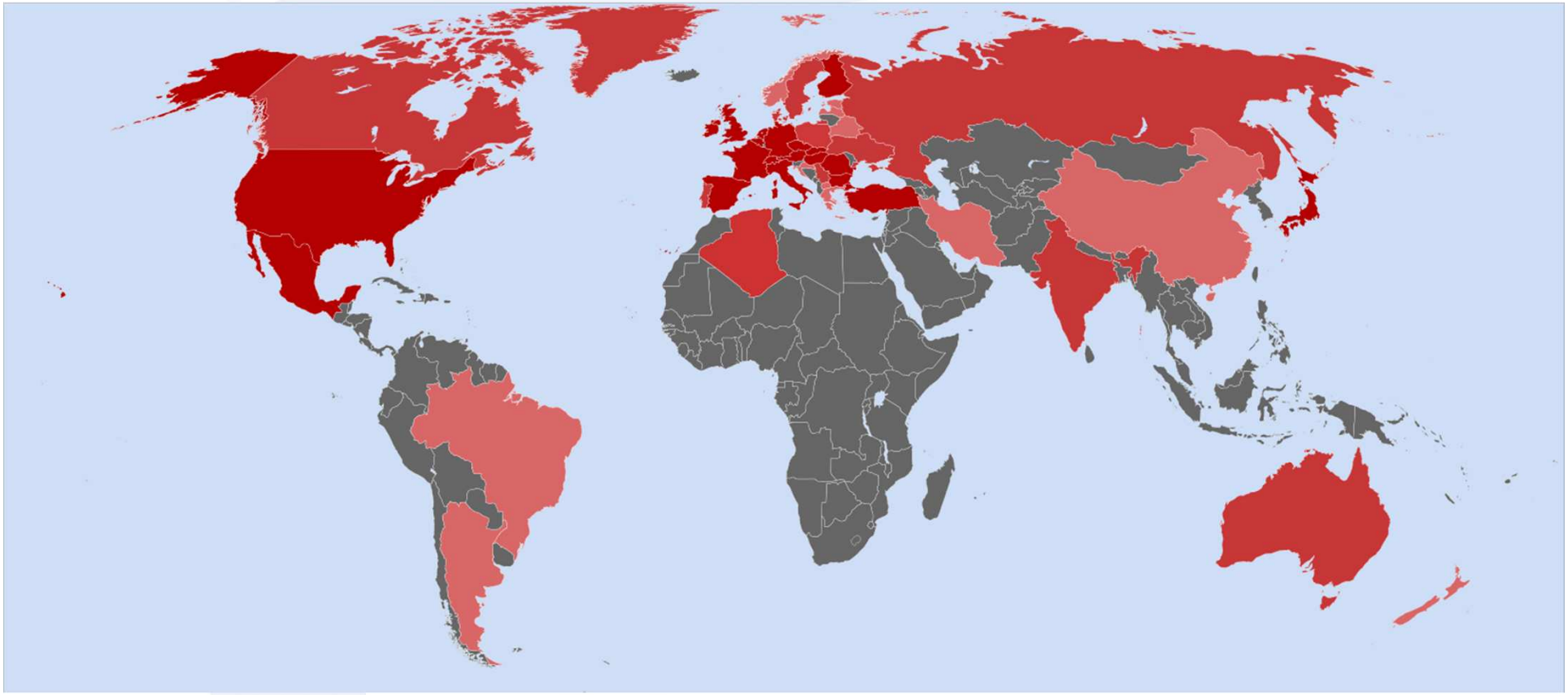
Patient registries:DMD



10,000+ patients

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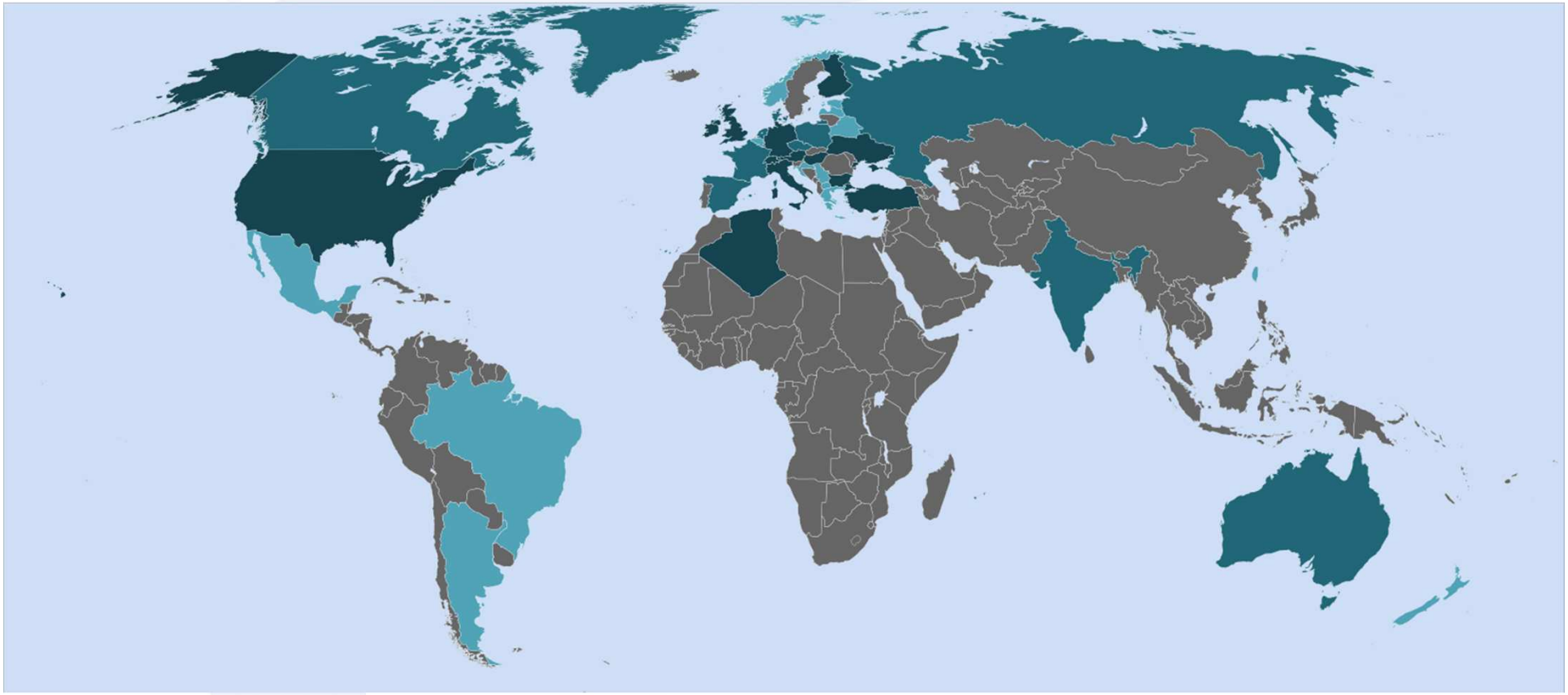
Patient registries: SMA



2,000+ patients

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Patient registries

- >40 countries involved in global registries for DMD and SMA involving >12,000 patients worldwide

- Current activities
  - Working with patient organisations to support registry development in other diseases (toolkit)
  - Working with industry to provide feasibility data and patient recruitment
  - FOR-DMD, CARE-NMD, BIO-NMD
  - Long term follow up for post registration studies

PVWG meeting
Milan, 15-16 Sept 2010


Hanns Lochmüller


Christophe Béroud

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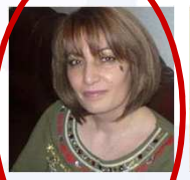
Vedrana Milic Rasic


Ludo van der Pol


Isabela Tudorache


Hugh Dawkins


Svetlana Artemieva


Alexander Baranov


Ole Gredal


Lawrence Korngut


Pat Furlong


Anna Ambrosini


Sylvie Tuffery-Giraud


Peter Van den Bergh


Violeta Mihaylova


Petr Vondráček


Jaana Lähdetie


Inge Schwersenz


Veronika Karcagi


Filippo Buccella


Harumasa Nakamura


Anna Kaminska


M. Rosário dos Santos


Eduardo Tizzano


Ian Murphy


Thomas Sejersen


Jan Verschuuren


Serap İnal


A. Ayşe Karaduman


Pascale Saugier-Veber


Vitaliy Matyushenko


Jacqueline Jackson


Vanessa Rangel Miller


Kevin Flanigan


Marie-Christine Ouillade


Ria Broekgaarden


Fabrizia Bignami


Simon Woods


Lauren Hache

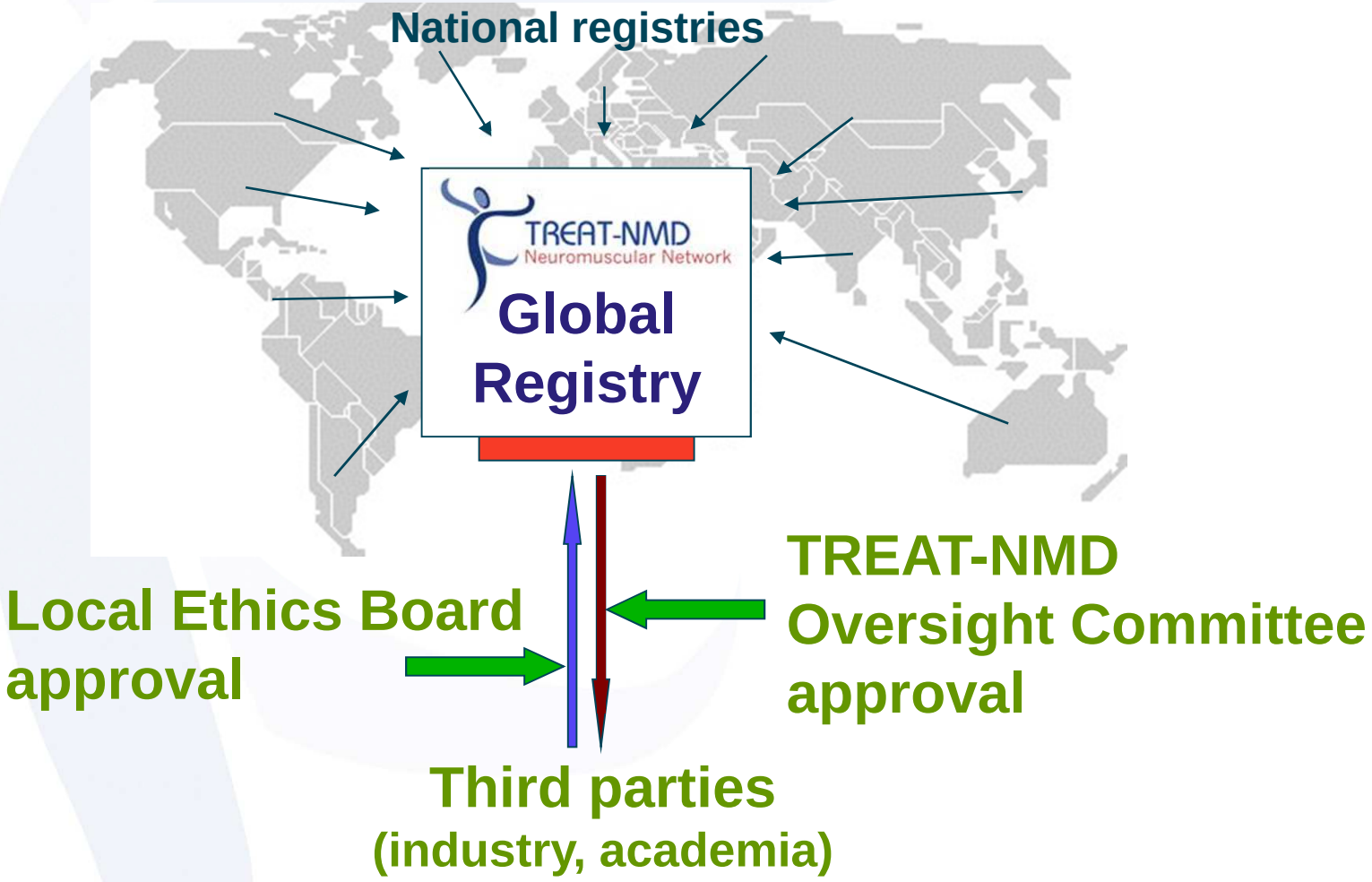

Pierre-Yves Jeannet

Janbernd Kirschner

Nick Catlin

Current TGDOC members

Third-party access to the global patient registries



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Registries used by industry – feasibility studies

- 6 feasibility enquiries from Feb 2009 to June 2010 (5 on DMD, 1 on SMA; 5 industry, 1 academic)
- All enquiries accepted by TGDOC in less than 14 days (>90% participation, all positive)
- Total revenue from enquiries: ca. 50,000.- €
- All enquiries completed in time according to agreements (<3 weeks to 8 weeks)
- Revenue ear-marked for further education and training (curator and OC meeting)

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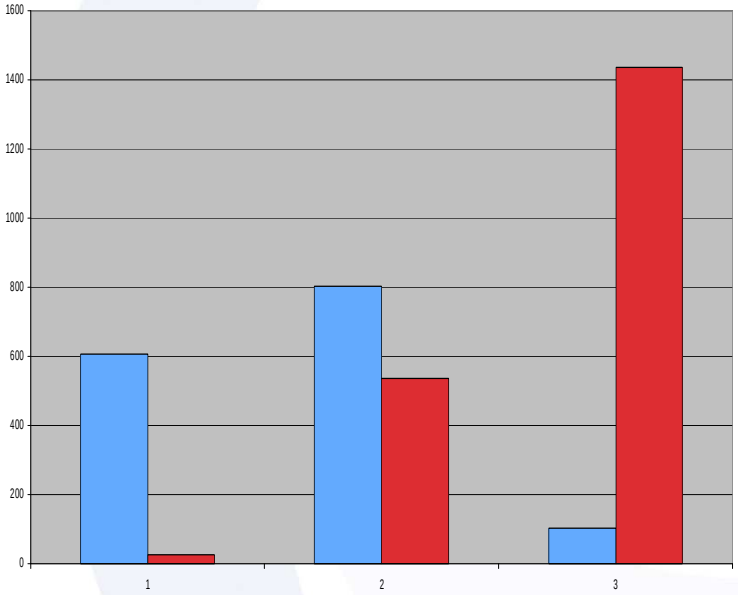
Example:

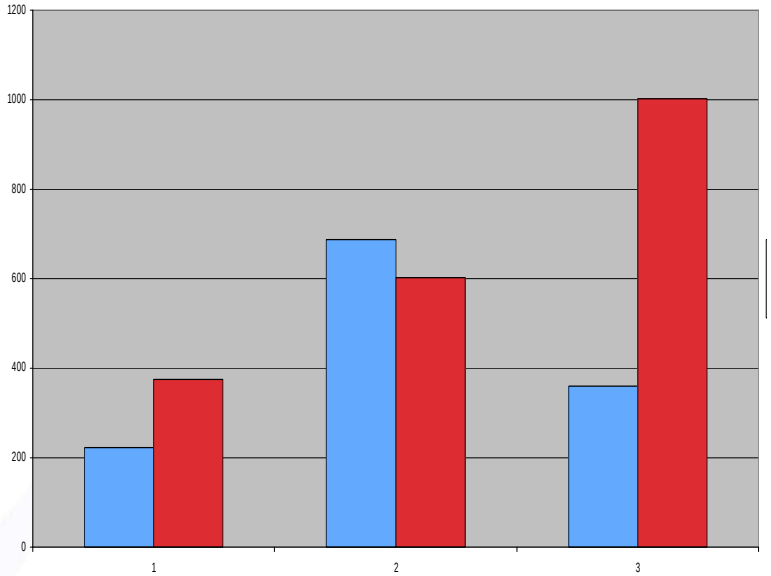
Acceleron enquiry: DMD patients in Europe (June 2010)

Ambulation



age 4-7 age 8-14 aged 15+

Steroid use



age 4-7 age 8-14 aged 15+

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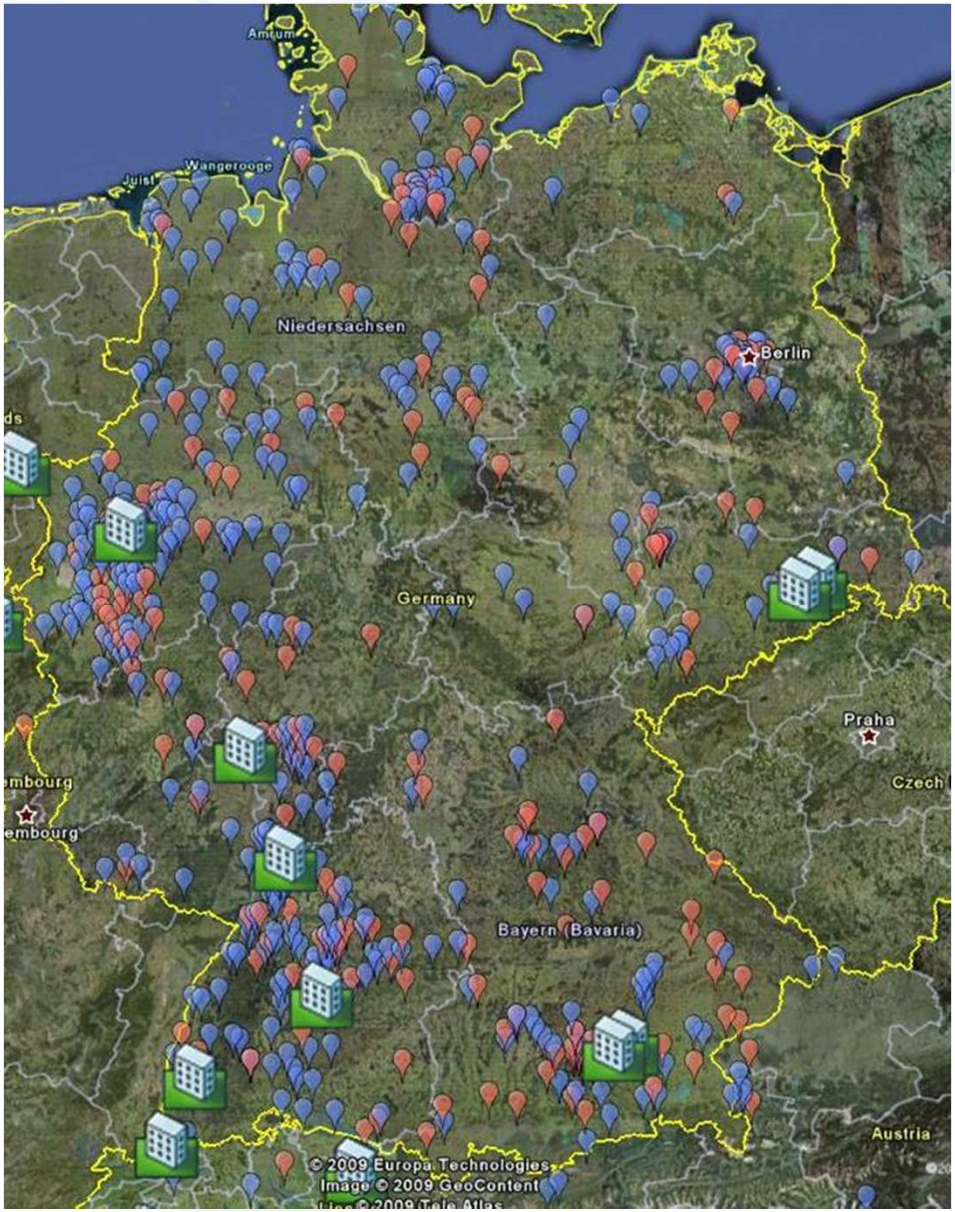

Houses:
German and Austrian trial sites in CTSR

Blue pins:
DMD & BMD patients in German patient registry

Red pins:
SMA patients in German patient registry

Feb 2009

Biobanks

- Initiated by EURODIS and AFM in 2001, 16 founding partners in 8 countries (Belgium, France, Germany, Hungary, Italy, Malta, Slovenia and Spain)
- Coordinated by a patient organisation: EURORDIS

- Financed by the European Commission (FP5 2003-2006)
- Partner of TREAT-NMD (FP6 2007 – present)

- Since 2001 provided quality DNA, Cell and Tissue samples
- EuroBioBank network: 11 Biobanks

- Catalogue of samples, freely accessible to scientists world wide
- A total of 170,000 samples available (DNA / Tissues/ Cells)

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Develop and manage supranational biobanks

- The Eurobiobank network tackles two of the main problems facing European research on rare diseases:

  1. lack of human biological material
  2. quality of samples

- EuroBioBank website (www.eurobiobank.org)

EuroBioBank

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Biobank Ethics

- Respect of **anonymity** in the sample collection
- Respect of the patient's autonomy by using the **Informed Consent** Form, for collection and use of the biological material for research
- **Access to sample**: ad-hoc board approval of the projects for which the biological samples are requested, no distribution of samples for cloning projects (Respect of the Oviedo Convention on Human Rights and Biomedicine and Additional Protocols, 4th April 1994; and International declaration on human genetic information, UNESCO general conference, 32nd session, Paris, October 8th 2003)
- **Confidentiality** of the data associated with the samples
- Information to the patients on the use of collections and the outcomes of the **research projects**.

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Questions / Discussion

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Questions / Discussion

- ***Q1: How should questions of data ownership in clinical research be resolved? To what extent do patients and their families own the data they contribute to researchers, sponsors, and regulatory authorities? What model of data ownership would patients and their organisations see developed?***

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Questions / Discussion

- ***Q2: How can patients and their organisations contribute to the development of best practices for clinical trial registries?***

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Questions / Discussion

- ***Q3: TREAT-NMD has begun the formation of a European patient registry for rare, inherited neuromuscular disorders. How can individual patient organisations within Europe help to ensure this compilation of registries continues successfully 5 to 10 years from now?***

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The role of Eurodis in EuroBiobank

- Coordinate the network administratively
- Apply for grants and find corporate sponsors to ensure long term sustainability of the network
- Maintain the EuroBioBank website (www.eurobiobank.org)
- Serve as the main contact point for the network