

1

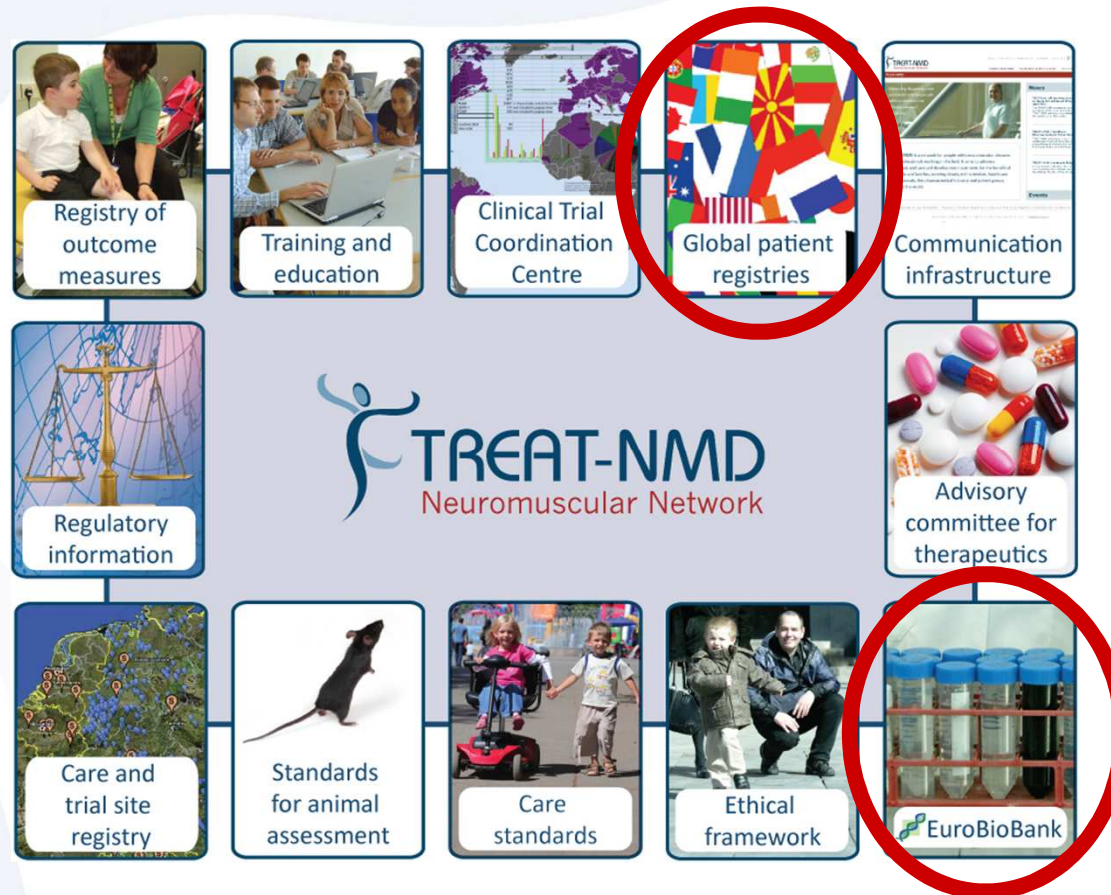
PVWG meeting
Milan, 15-16 Sept 2010



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

Emma Heslop

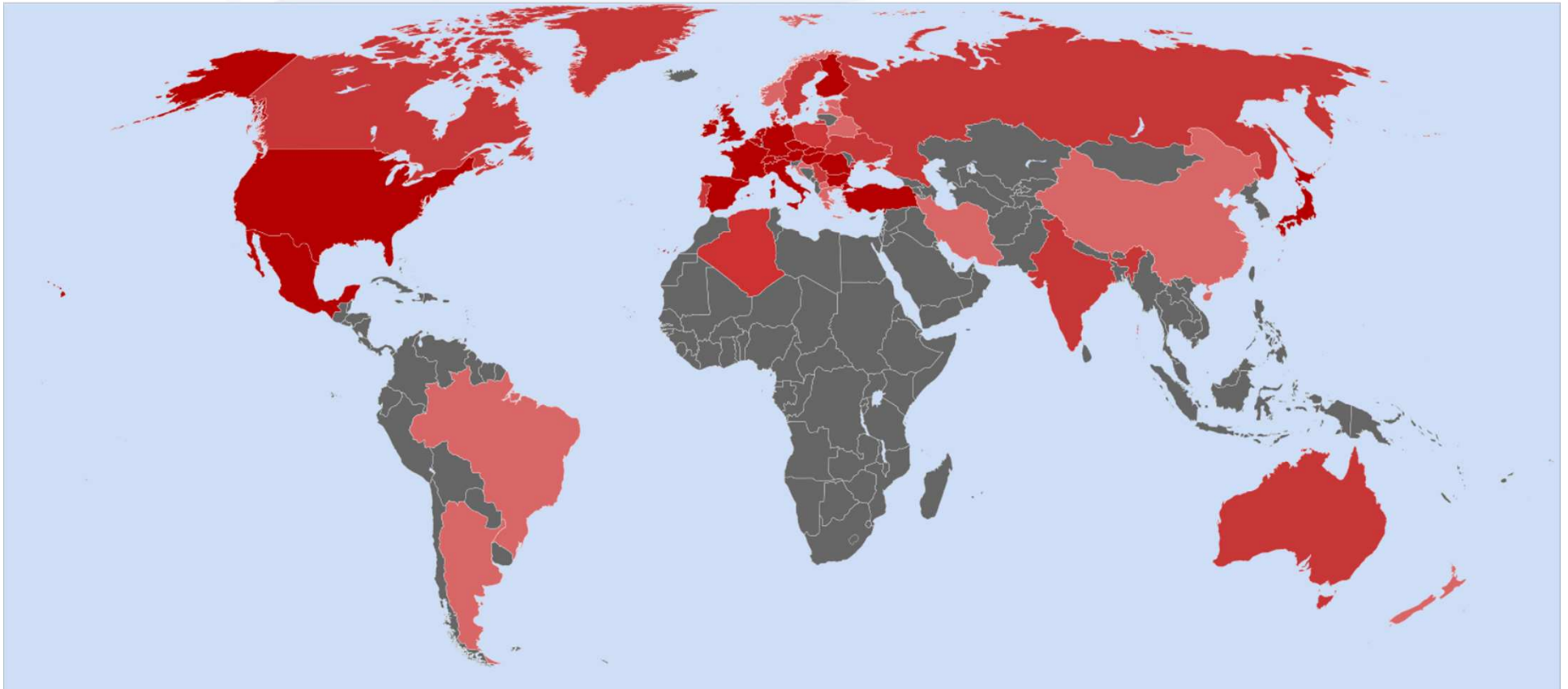
TREAT-NMD: an infrastructure for the neuromuscular field



3 | PVWG meeting
Milan, 15-16 Sept 2010



Patient registries:DMD



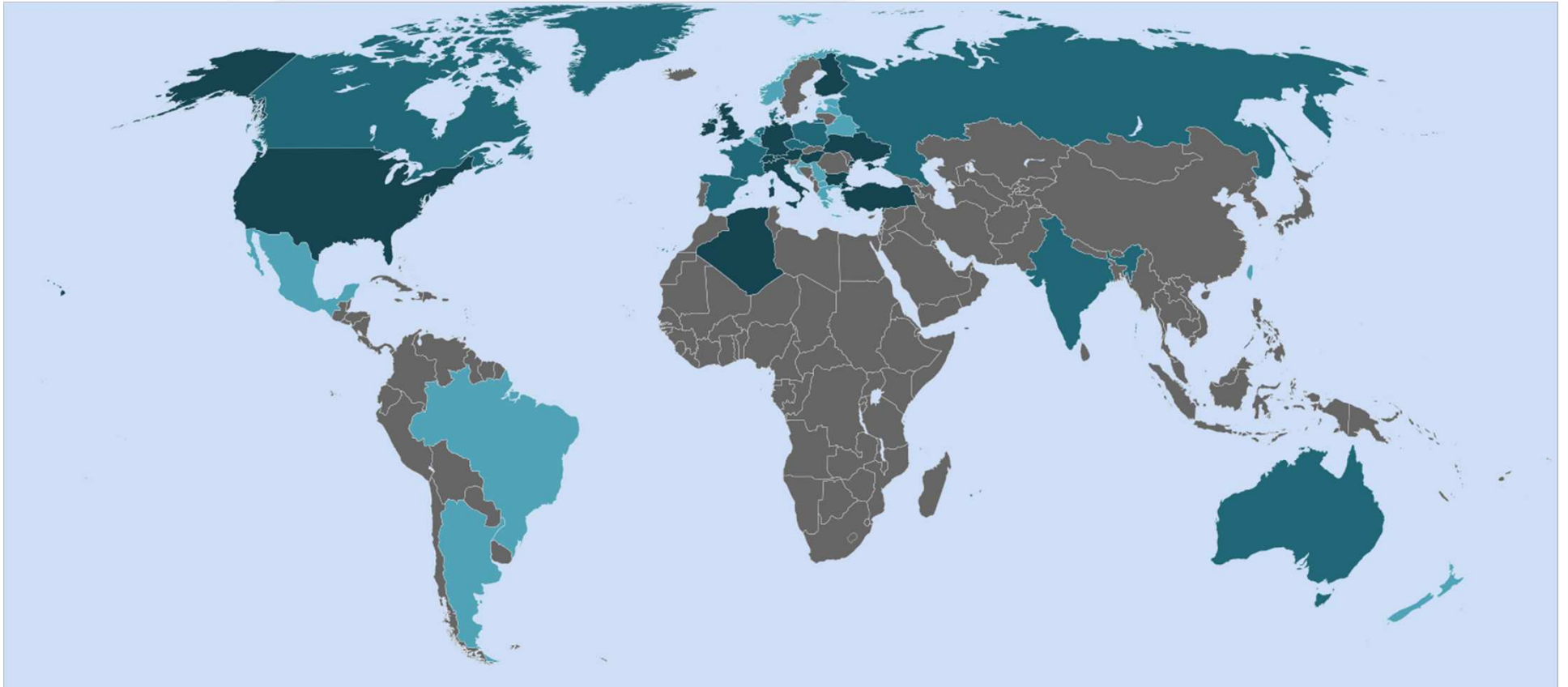
10,000+ patients

4

PVWG meeting
Milan, 15-16 Sept 2010



Patient registries: SMA



2,000+ patients

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

6

PVWG meeting
Milan, 15-16 Sept 2010

Hanns
Lochmüller



Christophe
Bérout



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



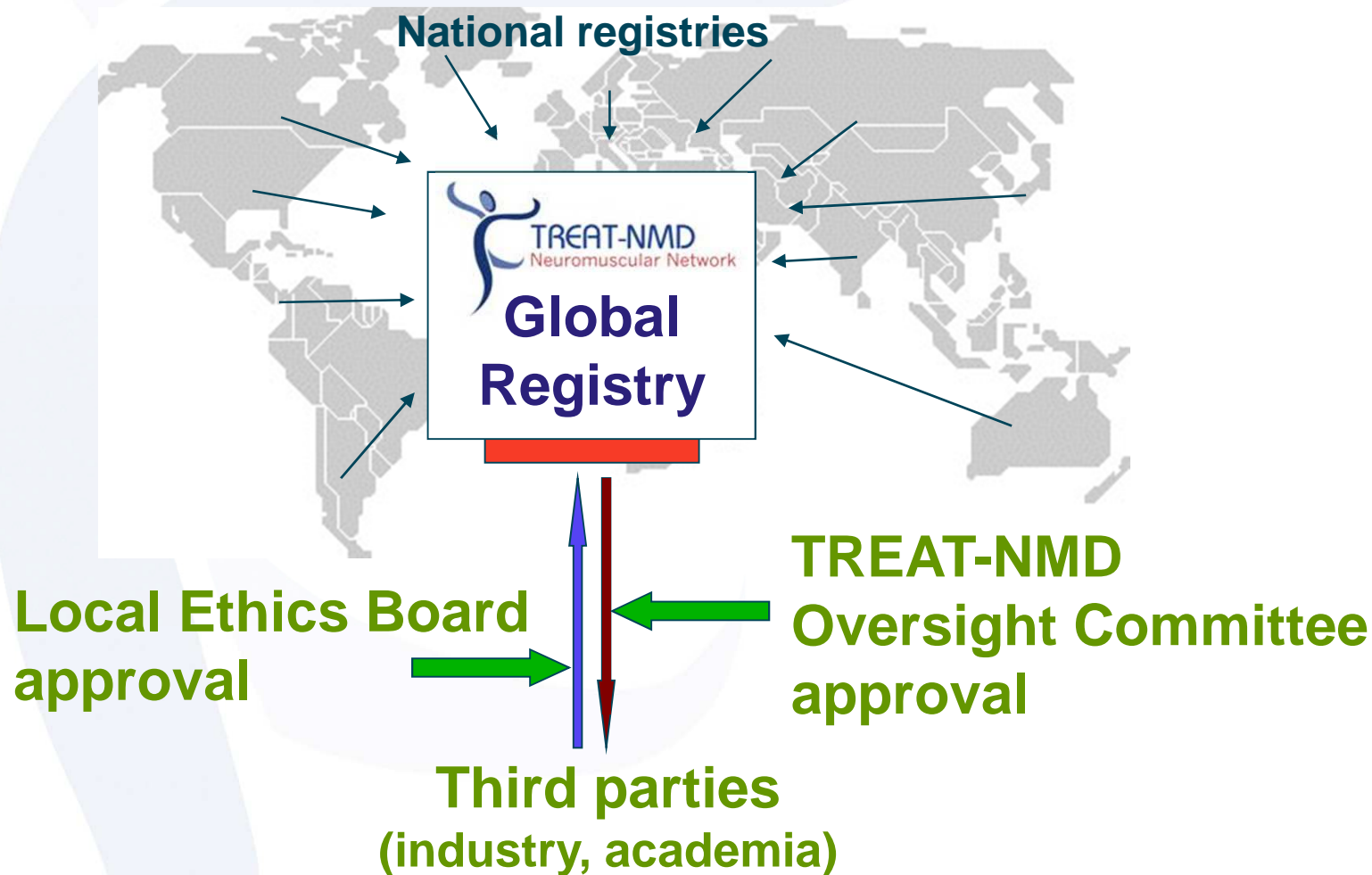
Janbernd
Kirschner



Nick
Catlin

Current TGD OC members

Third-party access to the global patient registries



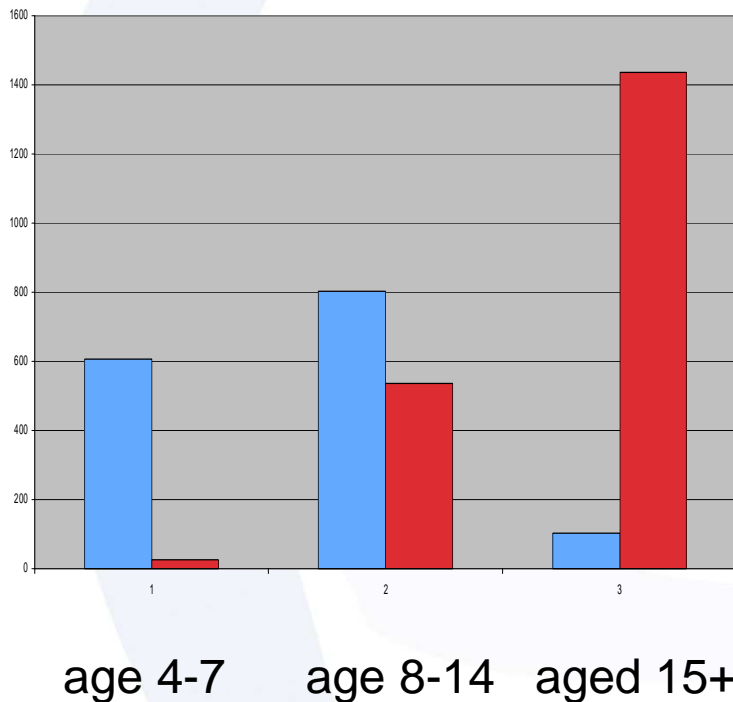
Registries used by industry – feasibility studies

- 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)

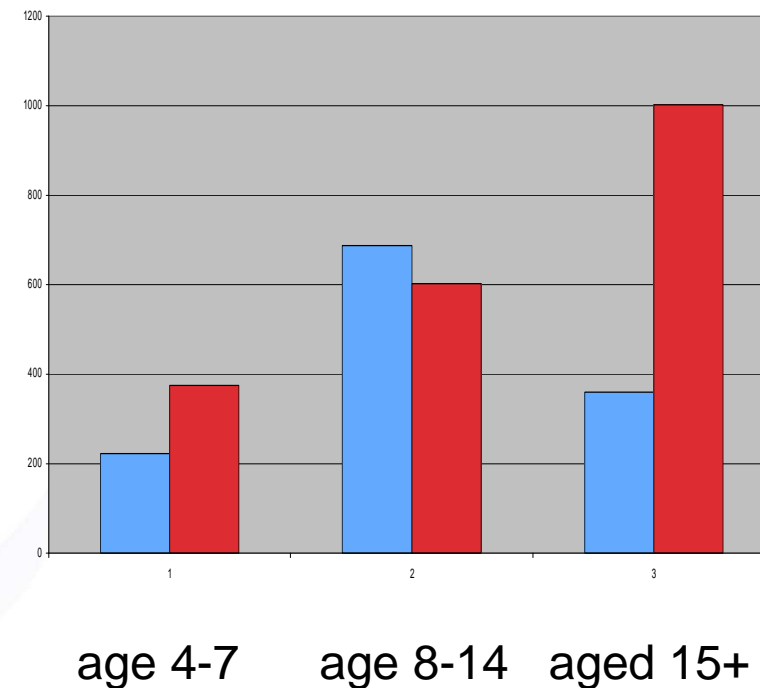
Example:

Acceleron enquiry: DMD patients in Europe (June 2010)

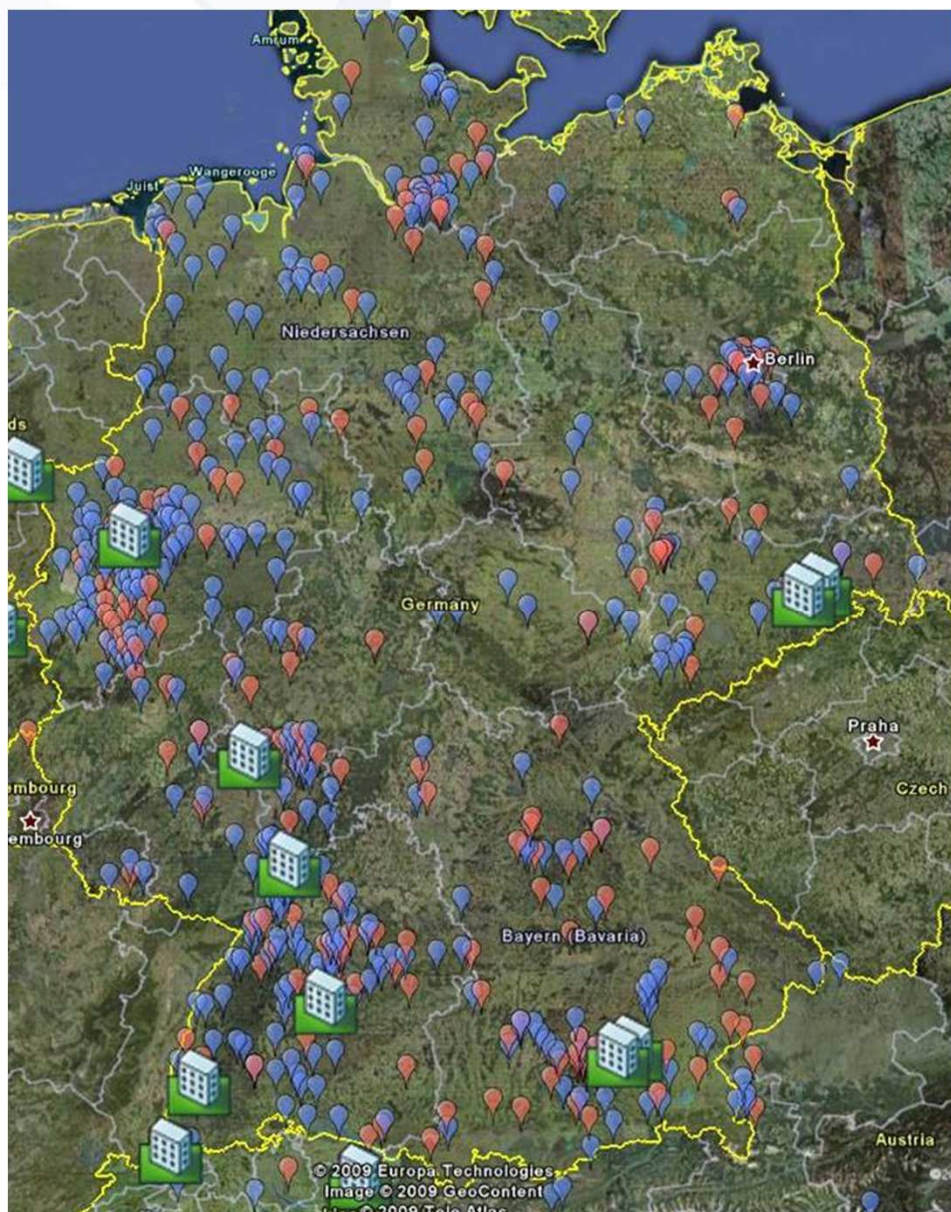
Ambulation



Steroid use



10 | PVWG meeting
Milan, 15-16 Sept 2010



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)



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)



Eurodis - A step forward for EuroBioBank



The screenshot shows the Eurodis website interface. At the top, there is a navigation bar with links: Home, Newsletter, Donate, Online payments, Contact, Get Help, and Bigger Text. Below this is a search bar and a language selector set to English. The main content area features a sidebar with a menu: Who We Are, Our Members, What We Do, Get Involved, Advocacy & EU Policy-Making, and Our Services for Patients. The main text area is titled "A step forward for EuroBioBank" and contains a paragraph about the EuroBioBank network's participation in the TREAT-NMD project. A small image of laboratory glassware is visible on the right side of the text.

EURORDIS
European Organisation for Rare Diseases

Home • Newsletter • Donate • Online payments • Contact • Get Help • Bigger Text

Search English

You are here : English Home Page / What We Do / A step forward for EuroBioBank /

A step forward for EuroBioBank

Who We Are

Our Members

What We Do

Get Involved

Advocacy & EU Policy-Making

Our Services for Patients

The EuroBioBank network joined the European Network of Excellence TREAT-NMD "Rare inherited neuromuscular diseases: from molecular basis to cutting edge therapies"(6th Framework Programme, 4th Call) in January 2007 for 5 years. As administrative coordinator of the EuroBioBank network since its creation, Eurodis is Partner 11 in TREAT-NMD and leader of the work package on biobanks, WP04.1: "Develop and Manage Supranational BioBanks". Most biobanks of the EuroBioBank network store neuromuscular disease samples, hence their involvement in this new neuromuscular project which aims to address the fragmentation currently hindering translational research - from bench to bedside.

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**.

14

PVWG meeting
Milan, 15-16 Sept 2010



Questions / Discussion

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?***

Questions / Discussion

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

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?***

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