

welcome

Welcome to the latest TREAT-NMD newsletter.

This week's edition includes:

- A report on an international neuromuscular conference in Brazil
- An overview of EuroBioBank, a major resource for neuromuscular biomaterials
- A report from the TREAT-NMD CTCC clinical trial workshop in Freiburg, Germany
- Details of the upcoming ICND meeting in Naples, Italy

We would like to thank those who have contributed to this week's edition. This newsletter relies on input from our readers. If you have anything you wish to be included in the next newsletter please contact us at info@treat-nmd.eu

at a glance...

[15-16 Jul 2010 MDR2010 - Padova, Italy](#)

[19-23 Jul 2010 12th International Congress on Neuromuscular Diseases - Naples, Italy](#)

[30 Jul - 1 Aug The Biennial FSHD International Patient and Researcher Network Meeting - Las Vegas, Nevada, USA](#)

[29 Aug - 1 Sep European Meeting on Next Generation Sequencing - Leiden, Netherlands](#)

[9-10 Sept 2010 MD2010 - 4th conference for the Muscular Dystrophy Association of Western Australia - Perth, Western Australia](#)

[18 Sept 2010 Muscular Dystrophy Campaign Conference - Birmingham, UK](#)

[20-22 Sept 2010 Muscle Study Group Annual Meeting - Buffalo, NY, USA](#)



Networking neuromuscular research and care in Brazil: conference report

From 17-19 June 2010 a group of international neuromuscular specialists joined Brazilian healthcare professionals and patient groups at the Hospital Sirio-Libanês in Sao Paulo, Brazil, to take part in the International Conference on Neuromuscular Diseases: Care and Clinical Trials. Around 350 people from across the country, including clinicians, physiotherapists, orthotics providers, and patients and families, as well as medical students from the Sao Paulo universities, participated in a two-day scientific programme followed by a dedicated family day with research updates for patients and information on physiotherapy and care.



The conference programme covered the latest updates in research and care in Duchenne muscular dystrophy (DMD) and spinal muscular atrophy (SMA), including the published international care standards for these conditions. Presentations on outcome measures, patient registries, recent clinical trials and the TREAT-NMD Advisory Committee for Therapeutics, while highlighting the current optimism that therapies for neuromuscular diseases are on the horizon, also focused attention on the many challenges that remain before a treatment that has interesting preclinical results in the lab can actually make it to the stage of being a marketed therapy available to patients. In a field where there can sometimes be much hype over unproven therapies, the well-informed realism presented at the conference was greatly valued by the attendees.

Brazilian speakers revealed the current situation in patient care in Brazil, highlighting its positive aspects, such as the availability of expertise and treatment in line with international standards, as well as its challenges, including lack of universal availability of genetic diagnosis and the variability of access to care between regions. Presentations from the international patient advocacy organisations UPPMD and SMA Europe served to emphasise the importance of equal partnerships between clinicians and patient groups and showed what parents and families can achieve by working together, while the family day itself was a highly interactive event that gave patients and families the opportunity to ask the Brazilian and international specialists specific questions about trials, therapies and care. The patient groups are now considering planning a longer dedicated "family conference" in the coming years.

The scientific programme closed with a panel session led by local organiser Professor Arnaldo Godoy that brought together government representatives with clinicians from specialist centres across Brazil and Argentina for a discussion about the creation of a Brazilian or indeed a South American neuromuscular network. The aim of such an initiative would be to improve integration into international initiatives in the neuromuscular field, prepare for participation in future clinical trials, and provide an infrastructure for dissemination of information relating to NMDs and for the implementation of best-practice care. The enthusiasm of the panellists and audience members was evident and concrete proposals have already been put forward, with a follow-up meeting planned in August during the Brazilian Congress of Neurology.

For further information on the conference itself or future plans in Brazil, please contact Professor Arnaldo Godoy.

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[4-5 Oct 2010 7th UK SMA
Researchers' Conference -
Llanwyddyn, Wales, UK](#)

[12-16 Oct 2010 World Muscle
Society International Congress -
Kumamoto, Japan](#)

[12-13 Nov 2010 Action Duchenne
8th Annual Conference - London, UK](#)

[16 Nov 2010 Europlan Conference -
Manchester UK](#)

Please note: This is only a selection of
upcoming meetings. To see all our listed
meetings [click here](#).

Clinical trial design workshop in Freiburg teaches the fundamentals of trial planning in rare NMDs

The TREAT-NMD Clinical Trial Coordination Centre has just hosted its second workshop on "clinical trials in neuromuscular disorders and other rare diseases". Held in Freiburg, Germany from 24-26 June 2010, the workshop welcomed participants from 5 different countries as far afield as Hong Kong, who used this great opportunity to work in a small group and learn in detail about all aspects of clinical trials in neuromuscular disorders and other rare diseases.



The workshop consisted of presentations and practical working sessions in small groups. Speakers included neuromuscular experts from academic institutions, pharmaceutical industry representatives (Santhera and Novartis) and patient representatives (Initiative 'Forschung und Therapie für SMA). Prof. Elbers from the German regulatory authorities BfArM and member of the EMA committee for orphan medicinal products (COMP) presented the agency's view of drug development in rare diseases.

During the course participants learned how to successfully develop and manage a clinical trial according to the guidelines of ICH-GCP. Topics covered included regulatory, organisational and statistical aspects as well as adverse event reporting, outcome measures in neuromuscular disorders, and data management. During hands-on sessions participants worked on their own concrete study ideas and discussed their ideas with the group.

Feedback from participants was very positive and the overall evaluation revealed very positive marks for content (1.3), comprehension (1.1), and learning (1.1) on a scale from 1 to 6. (1=very good; 6=unsatisfactory). They felt that the workshop was well-prepared and that it helped to increase their level of awareness and fostered contacts all over the world.

For further information about trial design training opportunities in Freiburg, please contact [Kathrin Gramsch](#).

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Focus on EuroBioBank - a unique international network of neuromuscular biomaterial collections

EuroBioBank (www.eurobiobank.org) is an international network of DNA, cell and tissue banks for research on rare diseases. Established in 2001 through initial funding under the European Union's Fifth Framework Programme and coordinated by EURORDIS, the network's objective is to increase the availability of and facilitate access to and use of high quality human biomaterials for research on rare diseases, with a special focus on neuromuscular diseases.



In 2007 EuroBioBank became part of TREAT-NMD, where it is responsible for biobanking activities. The EuroBioBank network is currently composed of 15 biobanks from 8 EU countries, with three new members (including one from Canada) being ratified at the most recent annual meeting in April.

Biobanks and biomaterial collections across the world may join EuroBioBank. All samples remain in the possession of the member biobank, with EuroBioBank acting as a clearing house or "virtual" bank with an online catalogue and search engine for locating samples. Researchers from anywhere in the world who locate a sample of interest through the catalogue then liaise directly with the bank holding the sample, with sample exchange being facilitated by conditions set out in the EuroBioBank charter and standardised material transfer agreements.

EuroBioBank Network/ TREAT-NMD Annual Meeting 2010

The joint 8th EuroBioBank (EBB) Network/ WP04.1 TREAT-NMD Annual Meeting was held in Paris on 29-30 April 2010. The meeting was attended by 19 participants, including representatives from most member biobanks and from EURORDIS and AFM.

Within TREAT-NMD, EuroBioBank/EURORDIS is leader of WP04.1 "Develop and Manage Supranational Biobanks". During the April meeting, participants were updated on the recent involvement of EuroBioBank in setting up the BBMRI (European Biobanking & Biomolecular Resources Research Infrastructure) prototype, in which EBB is one of the participating network of biobanks. In addition, Prof. Hanns Lochmüller, Newcastle University and Scientific Coordinator of EuroBioBank, explained how EBB will contribute to the new BIO-NMD project on biomarkers for neuromuscular diseases, by providing neuromuscular samples.

In view of the BBMRI initiative, EuroBioBank, like many other networks of biobanks in Europe, is moving towards improving its biobanking standards. In particular, quality control will be further implemented by sending a feedback questionnaire to former users of EBB samples, from three years ago, while with the goal of improving the EBB online catalogue of samples, biobankers were encouraged to continue testing the new Orphanet nomenclature to code samples (<http://www.orpha.net> > Services for Researchers > Classification of Rare Diseases). Finally, it was reiterated that data associated with the samples and diagnosis are the most important specificities of all biological samples, in particular of those coming from rare disease patients.

Three new candidate biobanks to the EuroBioBank Network were granted full membership status by the EBB General Assembly: the London and Newcastle MRC Centres for Neuromuscular Diseases and the Quebec DM Catalogue.

As innovative treatments for neuromuscular disorders need to be tested in vivo and in vitro before they can be tested in clinical trials, these additional member biobanks will contribute to increasing the choice and availability of rare neuromuscular biomaterials for translational research. Therefore, by expanding its network, EuroBioBank further fulfils its role as a core resource within TREAT-NMD.

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Twelfth International Congress on Neuromuscular Diseases taking place in Naples, Italy

The Twelfth International Congress on Neuromuscular Diseases will take place in Naples, Italy between 17th and 22nd July.

The Congress emphasises both the scientific and clinical aspects of neuromuscular diseases through a diverse programme of lectures, poster sessions, and discussions. Sessions include a series of "meet the professors" events hosted by leading experts in a range of conditions, plus the 6th assembly of WANDA, the World Alliance of Neuromuscular Diseases Associations. For more information, including a full programme, see the Congress website.

TREAT-NMD is helping to sponsor the Congress and coordinating a poster session during the WANDA meeting, which all participants are welcome to attend. The Congress itself is organised by the Second University of Naples and the Veterinary Medical School of Federico II Naples University on behalf of the World Federation of Neurology.

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