09 April 09
TREAT-NMD newsletter no. 52
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welcome

Welcome to the 52nd TREAT-NMD newsletter coming to you one day earlier because of the Easter break. This newsletter features the exciting launch of the TREAT-NMD Conference 2009 website with details of the conference programme and speakers. We hope you'll take time to visit it before you enjoy what is a long holiday weekend for many.

Two meeting reports are included this week. Firstly, there is the Canadian Neuromuscular Specialists meeting at Bloorview Kids Rehab in Toronto, Canada. Specialists from across the country discussed both further content and the logistics of Canadian DMD patient registry. Secondly the 7th annual EuroBioBank Network meeting was held on 2-3 April in Paris. Details of this meeting is also included.

Our annual review by the European Commission recently took place and its incusion completes our newsletter for this week. We hope you have a great weekend from everyone here at TREAT-NMD.

Best wishes,
Katie, Volker, Hanns, Steve, Emma, Rachel, Sam and Michael: the Newcastle TREAT-NMD team.

at a glance...

12-15 May 2009 The Nottingham Systematic Review Course 2009

21-23 May 2009 International conference in Ukraine: Recent standards in diagnosis, treatment and medical care for some rare neuromuscular diseases

01-03 Jun 2009 Update in Neuromuscular Disorders course in London

04-06 Jun 2009 TREAT-NMD workshop: clinical trial design in neuromuscular diseases

09-11 Jul 2009 "Therapeutic Targets in CMD", Emory University, Atlanta, Georgia

TREAT-NMD / NIH Conference 2009 - Website Launch

Registration for the conference has just opened and now is a great time to take advantage of the early registration prices available.

The aim of the conference is to share progress in the area of translational medicine in inherited neuromuscular diseases and set the future collaborative agenda.

This will be a major event in the international neuromuscular calendar for 2009 with a strong focus on translational research and "trial readiness". Up to 500 delegates are expected to attend.

Much of the preparatory work for the conference will take place via international focus sessions made up of specialists representing the various areas who will then give presentations representing the "state of the art" during the conference.

The conference will also feature poster sessions focusing on the science behind the therapeutics and we encourage any interested participants to submit abstracts.

We look forward to welcoming you to Brussels in November!

www.treat-nmdconference2009.eu

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EuroBioBank Network/ TREAT-NMD Annual Meeting report 2009

The joint 7th annual EuroBioBank Network/ WP04.1 TREAT-NMD meeting was held on 2-3 April 2009, at the Eurordis headquarters in Paris. The meeting was well-attended by 18 participants, including representatives from most participating member biobanks, of patient organisations such as Eurordis and AFM.

Joining us for the first time were Dr. Ségolène Aymé from Orphanet – Inserm SC11, Paris, Dr. Pascale Guichenet from DR1, Inserm/ Institut de Myologie, Paris, and Dr. Francesca Pampinella, from Fondazione Telethon, Italy.

EuroBioBank joined the TREAT-NMD network of excellence as partner 11 and leader of WP04.1 “Develop and Manage Supranational Biobanks”. EuroBioBank (www.eurobiobank.org) is a European network of DNA, Cell and Tissue Banks established in 2001 and coordinated by the European Organisation for Rare Diseases (Eurordis). The network is currently composed of 13 biobanks from 7 EU countries, most of them focused on neuromuscular research. The EuroBioBank Network was created to increase availability, exchange and use of human biomaterials for research on rare and neuromuscular diseases.

The National Laboratory for the Genetics of Israeli Populations (NLGIP), a biobank from Israel represented by Director Dr David Gurwitz, received full member status to EuroBioBank at the meeting. This biobank will contribute its collection of healthy controls from an ethnically diverse Israeli and Arab population, in relation with pharmacogenetics and rare disorders.

Dr. Ségolène Aymé, Director of Orphanet, gave us her perspective on the future of biobanking for rare diseases in Europe. She introduced the new Orpha nomenclature that is better suited than currently

09-12 Sep 2009 IDMC-7 International Myotonic Dystrophy Consortium

17-19 Nov 2009 TREAT-NMD / NIH International Conference

used codes such as OMIM and ICD to map rare diseases. EuroBioBank partners will explore whether the new nomenclature can be used for the catalogue, the central web-based search tool of EuroBioBank. Dr. Francesca Pampinella, Research Program Manager at FTELE, gave an interesting overview of the Italian network of biobanks.

During this meeting, we took stock of quality control, a process that was established last year based on customer surveys sent to scientists together with the requested samples: a first analysis of the customer surveys showed that the scientists using EuroBioBank materials and services are generally satisfied with the samples received and with the technical advice given. This is also reflected by excellent research that has recently been published acknowledging EuroBioBank support. Three working groups were established at the meeting that will continue to work on quality control, catalogue specifications and website development over the coming months.

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Canadian Neuromuscular Disease Registry Meeting

Canadian Neuromuscular Specialists met at Bloorview Kids Rehab in Toronto, Ontario, for a two-day-meeting on April 2-3, 2009. The meeting was organized by Craig Campbell from London, Ontario, and Doug Biggar from Toronto and brought together clinicians from neuromuscular clinics across Canada, as well as representatives from patient organizations, the Marigold Foundation and TREAT-NMD.

In a joint effort, it is planned to develop a Canadian Neuromuscular Disease Registry to ensure participation of Canadian patients in clinical trials. The registry will initially cover DM1 and DMD, but subsequently also include other neuromuscular diseases. Funding for this project has been granted by the Marigold Foundation and the DMD patient organization Jesse's Journey.

Marigold is a Calgary-based private foundation with a strong interest in supporting research in Myotonic Dystrophy and other rare diseases and was represented by its Executive Director, Don MacKenzie and Cheryl Swaby, research coordinator for the registry. Marigold has been in close contact with the team of the Genemu project of the University of Laval who own a natural history database on DM and were represented by IT specialist Dominic Leclerc and lawyer Emmanuelle Levesque.

John Davidson, father of Jesse Davidson who suffers from Duchenne Muscular Dystrophy, is founder the organization Jesse's Journey and very active in raising funds for research on a cure for DMD. Jesse's Journey has granted funding to Craig Campbell to set up a DMD patient registry for Canada. Talks by Jean Mah from Calgary who presented data of a survey among DMD families, Peter Ray from Toronto on DMD genetics and Sarah Baumeister representing TREAT-NMD provided a basis for a lively discussion on the registry effort. Participants expressed great interest in TREAT-NMD activities and the TREAT-NMD global registry effort and have agreed to include all mandatory and highly encouraged items suggested for TREAT-NMD national DMD registries.

A working group of representatives coming from neuromuscular clinics in Toronto, Vancouver, Calgary and London was formed to discuss further contents and logistics for the Canadian DMD patient registry. A first set of data for the congenital and pediatric DM1 part of the DM1 registry was established at a session sponsored by the William Singeris Centre for Myotonic Dystrophy of London, Ontario, and will be further discussed in the working group.

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TREAT-NMD Annual Review

As an EU-funded Network of Excellence, under the FP6 programme, TREAT-NMD is reviewed annually by the European Commission to ascertain that we reach our agreed milestones in the acceleration of cutting edge treatments for neuromuscular diseases. One of the major procedures for this annual process is the review of the Network by an external reviewer appointed by the Commission.

The reviewer stated that the annual report was impressive and that TREAT-NMD had exceeded expectations in the first two years of its inception and has already made a major impact internationally as well as in Europe. The reviewer continued by concluding that the TREAT-NMD Network was establishing the European community as the leaders on the world stage in the diagnosis and management of a range of neuromuscular disorders - which is precisely the aim of the FP6 funding.

TREAT-NMD has proactively engaged with international partners, collaborators and supporters to combine their respective expertise, for example, in establishing consensus on standards of care for patients and standard operating procedures for animal model assessment in preclinical research. We have worked hard to ensure the entire neuromuscular community is involved in these and other activities, and through this newsletter and our web site we have endeavoured to bring this information to a wide audience.

Finally, the coordinators would like to acknowledge and congratulate the TREAT-NMD partners and all our collaborators and supporters in helping TREAT-NMD to achieve this excellent report from our external reviewer and for ensuring our position in the neuromuscular community. We look forward to

continuing and expanding these close links with you all.

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