TREAT-NMD
Neuromuscular Network

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Welcome to the latest newsletter. This edition features a number of reports from recent meetings, both within TREAT-NMD and externally.

Today is a sad day for the Coordination Office as we say farewell to Virginia, who has been interning with us for the past 3 months. We want to thank her for all her hard work and wish her all the very best. Have a safe trip back to Brazil!

THANKS VIRGINIA!

Please forward any items that you would like to be included in future editions of the newsletter to info@treat-nmd.eu.

Best wishes,

Katie, Volker, Stephen, Emma, Arron and Rachel – the TREAT-NMD coordination team

Newsletter contents

Virginia and Katie at the farewell party!

About this newsletter

This is a fortnightly newsletter sent to all members of TREAT-NMD's "Club of Interest" worldwide. Earlier editions of the newsletter can be found online at www.treat-nmd.eu/news/newsletter/index.htm. If you would like to subscribe directly, please visit our website at www.treat-nmd.eu/ where you will find a subscription form at the bottom of the homepage. You can also use the same form if you no longer wish to receive this newsletter – just select the unsubscribe button.

Working with us

TREAT-NMD aims to be an inclusive rather than an exclusive network, and you do not have to be based in Europe or be a partner to be involved. International collaboration with experts from all over the world is already taking place, and new links are being developed.

If you are involved in any of TREAT-NMD's areas of interest and have something you'd like to say or a suggestion of where we could work together, we encourage you to get in touch by writing to us at info@treat-nmd.eu. The coordination team in Newcastle will be happy to put you in touch with the person most relevant to your particular interest.

TREAT-NMD Industrial Liaison Council Meeting in Basel

4th October 2007

15 participants from TREAT NMD partner organizations and the club of interest discussed the special interests of the industry in our network at the first Industrial Liaison Council (ILC) meeting that was held on the 4th of October 2007 in Basel. In addition to the industrial parties already involved in the network (Santhera, Vastox/Summit, Prosensa, Trophos) there was a strong interest from companies primarily based in the US, such as AVI Biopharm, PTC and Acceleron. This reflects the increasing interest of small and midsize enterprises (SMEs) in the neuromuscular field. Besides the industrial parties, AFM (representing the Intellectual Property Use and Dissemination Committee, IPUDC), the Clinical Trial Coordination Centre (CTCC) of the University of Freiburg and Michael Rutgers, who is leading on the future sustainability of the network, were participating in the discussions.

Not surprisingly, the main focus of the industrial parties lies on clinical development issues. The expertise within TREAT-NMD regarding the special patient population in DMD and SMA is thought to be of an important help for the conduction of clinical trials. The TREAT-NMD patient registry is highly attractive for all companies active in the field of NMDs. The CTCC who's activities were introduced during the meeting, can offer valuable services, specifically tailored to the need of companies and institutions.

Besides the interaction with the different activity groups in the network the industrial parties also appreciated the interaction with other companies active in the field which offer new possibilities for future collaborative or partnering activities. Also, being part of the TREAT NMD network is thought to booster the credibility of the industrial partners e.g. towards their investors and shareholders. It has become clear that while one single company may have little influence on regulatory processes a group of companies together with patient organizations and clinical experts might have a say in adapting general regulatory processes for diseases with a very small patient population.

In the context of TREAT-NMD sustainability, participants pointed out that TREAT-NMD should extend to other countries (in particular the USA, Japan and India) as well as to other diseases (in addition to DMD and SMA).

The ILC members concluded that it would be useful to install this council for the entire duration of TREAT-NMD. Also, it was agreed that the ILC will approach larger pharmaceutical companies to find out about their interest in the network.

For further information, please contact Stefanie Possekel, Chair of the ILC (Stefanie.Possekel@santhera.com).

Stephanie Possekel
Chair, ILC

Questionnaire on ethical and patient concerns

Next week the European NeuroMuscular Centre (ENMC) and the University of Newcastle will be sending out a questionnaire on ethical and patient concerns with regard to (the development of) new therapies. The questionnaire will be sent to three different target groups: neuromuscular patient organisations, scientists and pharmaceutical/biotech companies active in the neuromuscular field in Europe. The questionnaire consists of two parts. Part A aims to collect data concerning the different organisations in Europe. Part B focuses on ethical and patient concerns in connection with new therapies. The questionnaire will also be made available on the TREAT-NMD website, so if you're interested in filling it in on behalf of your organisation, watch out for the link in the next edition of the newsletter.

PTC Nonsense-mutation mediated DMD survey

The TREAT-NMD Clinical Trials Coordination Centre plans to work with PTC Therapeutics, a biopharmaceutical company, on the conduct of a pivotal trial that is specifically designed for patients with DMD. The trial will involve PTC124, a new chemical entity that has the potential to restore dystrophin expression in those patients who have DMD due to a nonsense mutation. PTC124 has been evaluated in two Phase I studies in healthy volunteers and in a Phase II study in boys with DMD. These studies produced encouraging pharmacodynamic results and showed PTC124 to be well tolerated. Based on these findings, PTC Therapeutics plans to initiate a multi-centre pivotal trial to evaluate the long-term clinical benefit and safety of PTC124 in patients with nonsense-mutation-mediated DMD.

If you would like to complete this survey online to determine your centre's interest in this DMD clinical trial and describe your DMD patient population please use the link below:

http://www.zoomerang.com/survey.zgi?p=WEB226RRKQDGX5

Further information on PTC Therapeutics and PTC124 can be found on the company's website, www.ptcbio.com.

Trophos to work with TREAT-NMD SMA Advisory Committee

The TREAT-NMD Clinical Trials Coordination Centre specialises in helping pharmaceutical companies arrange clinical trials in Europe and is able to set up advisory committees to discuss details of trial design and advise on regulatory issues. At the beginning of this month discussions were held with Trophos SA, a biopharmaceutical company specializing in the discovery and development of drugs for neurological disorders, regarding phase II/III trials for its lead product, TRO19622, in patients with spinal muscular atrophy. Trophos is working with an advisory committee composed of selected specialists from the TREAT-NMD network and SMA patient representatives with the aim of launching phase II/III trials at the end of 2008.

For further details about Trophos please visit their website at http://www.trophos.com/

For more information about TREAT-NMD's facilities for industry, please visit our website at http://www.treat-nmd.eu/activities/clinical_trials.htm or contact us at info@treat-nmd.eu.

Meeting on Skeletal Muscle MR Outcome Measures for Muscular Dystrophy Clinical Trials, September 25th, 2007.

National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) National Institutes of Health, Bethesda, US

This meeting was convened by Dr. Glen Nuckolls, Director of the Muscle Disorders and Therapies Program at the NIAMS/N.I.H. Invited participants were investigators from the muscular dystrophy research and musculoskeletal imaging fields, and included Dr. Richard Finkel (The Children's Hospital of Philadelphia), Professors Stefan Bluml and Vincente Gilsanz (University of Southern California), Professors Krista Vandeborne and Glenn Walter from the University of Florida and Professor Martin Kushmerick (University of Washington Seattle), Dr. Penny Garrood (University of Newcastle), Professor Steven Moore (University of Iowa), Professor Shantanu Sinha (University of California, San Diego), Professor H. Lee Sweeney (University of Pennsylvania), as well as representatives from various institutes and programs within the N.I.H.

The aim of the meeting was to reach a consensus regarding strategies for developing validated skeletal muscle magnetic resonance (MR) outcome measures to accelerate muscular dystrophy clinical trials. Three core goals were identified:

1. To identify a minimum core set of data that can be collected from patients with DMD and other muscle diseases at different research centres using functional outcome measures and currently available MR methods.

2. To outline strategies for the standardisation of data collection and analysis to promote comparisons within and among different studies.

To identify promising MR modalities that could be used to better understand the pathophysiology of various dystrophies and lead to the development of more effective outcome measures for future trials or studies.

Each goal prompted lively and constructive debate, with an emphasis on the practical considerations of using MR techniques in children. Participants also presented their recent research experience and results using the various modalities (such as T1-weighted imaging, proton spectroscopy and diffusion tensor imaging) in order to inform the debate. It was recognised that further work on each goal by subgroups would be needed to further refine research strategies.

A report on the proceedings of the meeting will be posted on the NIAMS website

(http://www.niams.nih.gov/News_and_Events/Meetings_and_Events/default.asp).

Penny Garrood
Clinical Research Fellow,
Newcastle University

SMA Summit on Drug Development
September 28 and 29, 2007 at the Hyatt Bethesda, Maryland

The SMA Summit on Drug development in SMA was held on behalf of the ICC at the Hyatt Regency in Bethesda, Marlyland, USA in September 28 and 29th. The ICC Patient Advisory Group (Families of SMA, Fight SMA, Muscular Dystrophy Association, and SMA Foundation) organized and sponsored the meeting with the help of support from 15 industry and nonprofit supporters. The Steering Committee was composed of Kenneth Fischbeck, Jill Heemskerk, Edward Kaye, and John Kissel (Chairman).
[cont'd]

The main goals of the meeting were the following: 1) to gain a better understanding of the clinical and regulatory requirements for approval of and SMA clinical trial; 2) update on the status of drug development; 3) identify challenges and gaps in current effort for drug development.

The full meeting report by Enrico Bertini can be found on the TREAT-NMD website:
http://www.treat-nmd.eu/assets/documents/REPORT_15.pdf

Annual Neuromuscular Conference and Inaugural Scientific meeting of the new MRC Centre for Neuromuscular Disease

Venue: Institute of Child Health, Central London.

Date: 1-2 February 2008

The conference will focus on translational research in neuromuscular disease and will have a number of world-class scientists speaking, including Professor Mike Shy, Professor Eric Hoffmann, Professor Robert Griggs, Professor Louis Ptacek, Professor Vincent Timmerman, Professor Steve Waxmann, Professor Marinos Dalakas and Professor John Porter.

Deadline for registering: 31 October 2007.

For further information: Julia Ambler [J.Ambler@muscular-dystrophy.org]

www.muscular-dystrophy.org

Activities of international cooperation FP7-INCO-2007-3

The European Commission invites applications under its FP7 Capacities work programme: activities of international cooperation (ERA-NET and ERA-NET Plus).

Activities are called under the following areas:

- 3.1 ERA-NET International Cooperation;
- 3.1.1 preparatory actions for ERA-NET (coordination and support action - supporting);
- 3.1.2 ERA-NET (coordination and support action - coordinating);
- 3.2 ERA-NET Plus International Cooperation (coordination and support action - coordinating).

The objective of the scheme is to step up the cooperation and coordination of research programmes carried out at national or regional level in the member or associated states through the networking of research programmes, towards their mutual opening and the development and implementation of joint activities.

The ERA-NET actions can network four types of activities:

- information exchange amongst member states and community on S&T international cooperation thus promoting an effective and efficient international scientific EU cooperation strategy at EU level;
- definition and preparation of joint activities;
- implementation of commonly agreed objectives and joint activities by facilitating innovative programmatic approaches;
- funding of joint transnational research actions.

For ERA-NET consortia, the minimum number of participants has been set at three independent legal entities which finance or manage publicly funded national or regional programmes. Each of these must be established in a different member state or associated country.

The **eligible partners for ERA-NET actions are only:**

- programme owners, typically national/regional ministries/governments responsible for defining, financing or managing research programmes carried out at national or regional level;
- programme managers (such as research councils or funding agencies) are other national or regional organisations that manage research programmes under the supervision of the programme owners; programme owners (typically national ministries/regional authorities) which do not have a running or fully fledged research programme at the moment of submitting an ERA-NET proposal, but which are planning or have committed to set up such a programme, are also eligible if their participation is well justified and adds value to the overall programme coordination. As such, countries or regions which have less diverse research programmes (in particular new member states and candidate associated countries) will find their involvement in the ERA-NET scheme greatly facilitated.

The ERA-NET Plus actions aims to allow the commission to provide an incentive to the organisation of joint calls between national or regional research programmes by topping-up joint transnational funding with community funding. These projects require programme owners or programme managers from at least five different member or associated states to plan a single joint call. The total planned budget of the joint call shall have a minimum financial volume of 3 million euros. The total budget for both ERA-NET and ERA-NET Plus is 11m euros. FP7-INCO-2007-3. OJ C230 (2007/10/02) p3.

Deadline: 5pm local time Brussels, 12 February 2008

Job and training opportunities

Registry Co-ordinator
Neuromuscular Disease Clinical Trials Outcomes Registry
Department of Clinical Neuroscience

KING'S College LONDON
University of London

Applications are invited for a Registry Co-ordinator with excellent communication skills to administer the Neuromuscular Disease Clinical Trials Outcomes Registry based in the Department of Neurosciences at King's College Hospital. The Neuromuscular disease Outcomes Registry is part of an EU funded Network of Excellence called TREAT NMD.

The successful applicant will be responsible for the NMD Clinical Trials Outcomes Registry and the administration of it, including budget management, maintenance of the Group's website, and facilitating the start-up and maintenance of the register of outcome measures.

The post needs someone who will contribute to the identity of the NMD Clinical Trials Outcomes Registry and ensure it starts well and continues to grow. Experience in health research and clinical trials is highly desirable and some knowledge of medical terminology and information technology would be helpful.

Starting salary in the range £27,162 pa to £34,470 pa (inclusive of £2,323 pa London Allowance), depending on qualifications and experience.

To obtain further particulars, an application form and further information about the Institute, please see our website at http://www.iop.kcl.ac.uk/vacancies or alternatively email vacancies@iop.kcl.ac.uk. Completed application forms should be emailed to this address or posted to the address given in the further particulars. **Please quote reference number 07/A56 in all correspondence. Closing date for applications 26th October 2007.**

Only candidates shortlisted for interview will be contacted.

Equality of opportunity is College policy

Current job and training opportunities are advertised on the TREAT-NMD website.

www.treat-nmd.eu/jobs.htm

www.treat-nmd.eu/activities/training_educ.htm

Partner-specific items

Project Ethics Council meeting minutes

The minutes from the first TREAT-NMD PEC meeting held in Naarden, The Netherlands on the 3rd-4th July 2007 are now available to view on the partner section of the website.

http://www.treat-nmd.eu/private/

Any non-partner interested in the work of the PEC should contact Rachel Thompson for further information (rachel.thompson@treat-nmd.eu)

Send us your news and views!

We strongly encourage all partners and supporters to send their own news and updates and we will be happy to include them in future editions of the newsletter. Please send your contributions to emma.heslop@treat-nmd.eu