



TREAT-NMD Neuromuscular Network

14th December 2007 · Newsletter No. 23



A Holiday Message from the Coordinators

TREAT-NMD is now nearly one year old, and we wanted to take the opportunity of the last newsletter of 2007 to thank all the partners and our collaborators for all their hard work. There is no doubt that there has been huge progress in many of the areas of interest of the network, and that TREAT-NMD has taken its place in the world of neuromuscular research. The whole idea of a Network of Excellence is that it is intended to change the way that scientists, clinicians, patient organisations and industry work, so that integration and harmonisation can be fostered, thereby allowing a strengthening of the area of research. Engaging with the concept of collaboration is at the heart of our network, and it has been exciting to see that people are so keen to work together. Our newsletter now has a circulation of over 2400, and the excellent response to the TREAT-NMD initiatives on patient registries and the site feasibility questionnaire are just two examples of how people appreciate that this kind of collaboration is necessary to accelerate clinical trials in these rare disorders. The translation of the SMA standards of care into more and more languages is also a very exciting development and the DMD standards in a similar format will be available next year. In 2008 it also looks like TREAT-NMD will actively start to participate in clinical trials, so we will really be able to see the network in action. We also hope that early in 2008 we will be ready to welcome new members to the network via the adoption of a members' charter.



Partners at the moment are actively involved in planning the next eighteen month period of work, which will take the network beyond the half way point of the funding provided by the EU, and these plans will be discussed in detail at the Governing Board meeting in January. Identifying the core strengths of the network and the elements of it which can be sustainable in the long term is going to be a major task as the network moves forward.



We are always keen to receive feedback and suggestions so please do get in touch! In the meantime, have a relaxing and peaceful Christmas and a very Happy New Year.

Katie, Volker and the coordination team

Newsletter contents

TREAT-NMD news and reports.....	2	Working with us.....	5
Other news.....	4	Job and training opportunities	6
About this newsletter	5	Partner-specific items.....	6

Centres with Previous Clinical Trial Experience

Petra Kaufmann at the Neurological Institute, Columbia University, is working with TREAT-NMD on a project aiming to learn about recruitment in current and past paediatric neuromuscular clinical trials, specifically in recruitment rate (number per site per month), variability between trials, change over time (past and more current trials), and other factors (country, geographic location, placebo group). The goal of this project is to summarise trial experience in a brief report so that we can learn for future trial planning and so that we can see how neuromuscular disease compares with other fields, it does not require you to include any trial data or results. Colleagues at Columbia University have reviewed the literature for information and in addition have designed a short one-page form (one form per trial).

TREAT-NMD have contacted over 56 eligible sites with previous Clinical Trial experience, identified using the feedback we received from our CTCC feasibility questionnaire to ask them to complete and return this form. However, if you have not been contacted directly but have previously coordinated a Clinical trial or have been a PI of a multi-centre study and have sufficient information, we would be grateful if could you download and complete the form (1 per trial) from the TREAT-NMD website

http://www.treat-nmd.eu/documents/questionnaire_recruitment_10_07.doc and return it to Petra Kaufmann PK88@columbia.edu

If you would like to register the potentially eligibility of your site to host future clinical trials in Neuromuscular Disease, please complete the TREAT-NMD CTCC feasibility questionnaire online.

<http://skl14e.ukl.uni-freiburg.de/eu.treatnmd.fq.web/register.jsf>

TREAT-NMD represented at the AFM Telethon

In response to Herve Laouenan's invitation to join in with a "European mobilisation" event for the AFM Telethon, around 20 TREAT-NMD partners and other scientists made their way to Metz on Saturday 8th December to join in the broadcast and publicise the efforts towards European collaboration within TREAT-NMD. It was fascinating to see the engagement of the whole city in the Telethon fundraising- from a yellow Telethon train in the station and boats on the river, to line dancers and wheelchair racing in the Place de Chambre (which became the Telethon village for the day). You don't get long on live television to get your point across, and the assembled group had just their three minutes of fame, but long enough to demonstrate the collaborative will for the acceleration of treatments in neuromuscular diseases!

Latest news is that the Telethon is heading for another great year of fundraising, with over 100 million Euros achieved again, which is excellent news for European science.



CTCC Clinical Trial Workshop

The [Clinical Trials Coordination Centre in Freiburg](#) (Germany) was created as part of a cooperation between the Neuropaediatric Department of the University Children's Hospital and the Clinical Trials Centre of Freiburg University Medical Centre. With the expert knowledge and experience from both sites we can offer advice and assist in all aspects of clinical trials in the field of neuromuscular diseases.



For spring next year we plan to offer a workshop / advanced training course in clinical trials. Therefore we have designed a questionnaire to help define the target audience and their needs regarding the topics of the workshop. If you are interested in such a workshop please, download the questionnaire from the TREAT-NMD website (see link

below) and either e-mail the completed questionnaire to angela.stanescu@uniklinik-freiburg.de or fax it to us at: **0049-(0)761-270-7373**.

Thank you in advance for your help!

To download the questionnaire please click on the link

<http://www.treat-nmd.eu/news/documents/CTCCWorkshopquestionnaire.doc>

For further information please contact:

Angela Stanescu, Clinical Trials Coordination Centre, Freiburg (Germany)

angela.stanescu@uniklinik-freiburg.de

PPMD Annual Connect Conference Meeting Report,

Philadelphia, 12 - 14 July 2007.



More than 400 people attended the 2007 annual conference of the American Parent Project Muscular Dystrophy (PPMD) in Philadelphia from 12 – 14 July 2007. Over three days, about 60 presentations on therapeutic research, medical and social management, and legal affairs were presented. Günter Scheuerbrandt has produced a report on the research presentations of this meeting for the Duchenne boys and young men and their families, who wish to know how the work of the researchers and other experts is progressing to find effective therapies for Duchenne dystrophy.

The full report is available to download from the TREAT-NMD website:

http://www.treat-nmd.eu/news/documents/PPMDAnnualConnectConference_Philadelphia.12-14July2007.pdf

Press Release from the EU Open Forum on the Future of Networks of Excellence

Brussels, 20 November 2007

Alarm raised for “Networks of Excellence”

Current European research strategy may put the future of a remarkable instrument – the Network of Excellence (NoEs) – at risk. According to an opinion paper now available online, practical interventions are needed at EU level to avert future scientific and technical losses that Europe can ill-afford. The Open Forum held today in Brussels will highlight the strengths of NoEs and invites scientists and policy makers to discuss the future of Networks of Excellence in the European Research Area.

Current European research strategy may put the future of a remarkable research instrument, the so-called Networks of Excellence (NoEs), at risk. This is causing great concern within the European research community. “Many scientists are currently feeling concerned about the long-term future and sustainability of Networks of Excellence and for good reason”, says an opinion paper signed by coordinators of more than 60 Networks of Excellence, who represent the voices of 15,000 researchers throughout Europe.

They have been surprised to see that only 17 new Networks of Excellence appear to have been funded within the first call of the Seventh Framework Programme for Research (FP7) compared with 101 in the equivalent call during the previous EU research funding programme. They are also concerned because nothing has been announced in terms of support to existing networks.

The leading European research professors and scientists who wrote the paper fear a huge waste of investment. “We do not believe that Europe can afford to let this instrument disappear and consequently lose the achievements that have been made to date”, they say.

The Open Forum organised on 20 November in Brussels aims at highlighting the role of Networks of Excellence in the European research area and how they can become sustainable. A panel discussion on the Opinion paper will be chaired by Professor Jerzy Buzek, Member of the European Parliament with representatives from the scientific community and the European Commission.

Benefits to Europe

Networks of Excellence were conceived to drive scientific and technological research in particular areas of key significance to Europe. For example, research areas covered by the paper’s signatories include efforts to reduce the impact of climate change through the cleaning up and replacement of fossil fuels; control of antibiotic resistance; control and management of the current epidemic of allergies in Europe; chemical contamination of food, and so on.

The genius of the Network of Excellence research structure is two-fold. On the one hand, it allows “networking” to stimulate cross-fertilisation of ideas, knowledge and data while helping to avoid duplication of efforts. On the other, “sharing of excellence” is promoted via partnerships with scientific practitioners, consumer groups, and industry throughout Europe.

Problems

Everyone expected difficulties to arise during the integration of institutions and departments who might otherwise be in competition with each other. However, an even more challenging problem arose that no-one had foreseen. It has proved highly complicated to establish Networks of Excellence as legal entities. It seems from the experience of the majority of Networks that achieving long-term sustainability will take longer and require a transition period beyond the 4-5 years of initial funding.

According to the opinion paper, a review of the successes that have been achieved in integration and sustainability within existing Networks of Excellence is now urgently needed. The findings should be converted into a practical tool that can help others. The authors suggest that any funding extensions would be made on the basis of a competitive process. The networks that can show real achievements and make a convincing case for the potential of their integrated and sustainable structure should be given the necessary support.

The paper also makes the important point that Networks of Excellence are more than halfway to achieving the structures that the European Commission has outlined in its recent vision statement. The recent Green Paper on the European Research Area says that: "European research should be built on effective European-led coordination, ...with an adequate flow of competent researchers with high levels of mobility between institutions, disciplines, sectors and countries; world-class research structures, open to all; ...effective knowledge-sharing, between public and private sectors, and with the public at large".

The expert authors believe that, far from cutting back, the Networks of Excellence should be further exploited. In so doing, the unique European infrastructure of the Networks of Excellence can ignite new interdisciplinary areas of scientific investigation for the benefit of the European Research Area.

TREAT-NMD was represented at this Open Forum meeting and a full report, along with the opinion paper, can be found on the web site at <http://www.treat-nmd.eu/documents/OpenForumReport.pdf>



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 Neuromuscular Network

Job and training opportunities

New post available for a physiotherapist/ trials co-ordinator at the Newcastle Muscle Centre, UK

We have a post available from the beginning of 2008 until March 2011 for a physiotherapist with trials experience to work with the neuromuscular team in supporting clinical trials and clinical research activity, including standardised assessments of patients. Experience in trials and/ or research would be very useful, as would familiarity with the principles of good clinical practice, though training in these can be provided. The postholder would work under the direction of consultant physiotherapist Michelle Eagle, as well as interacting directly with the clinical trials co-ordinator and the TREAT-NMD team.

The post will be advertised via the University of Newcastle website shortly, but in the meantime we would welcome any approaches from people who would like to discuss the post informally. For further details contact the TREAT-NMD office (info@treat-nmd.eu), Michelle Eagle (michelle.eagle@btinternet.com) or Prof Kate Bushby (kate.bushby@ncl.ac.uk)



Partner-specific items

Poster sessions at Governing Board meeting

The Governing Board meeting this January is not merely a formality but a real opportunity for partners to discuss their achievements and make plans for the upcoming period. Posters are great starting points for discussion, and this January's meeting will be centred around poster sessions taking us through the network's main activities. An overview of the posters is provided below; for full details of the agenda, partners can log on to the partner section of the web site.

- A01 Implementing the TNCC (UNEW)
- A01 Financing network durability (UNEW)
- A01 Tools and Methods (FTELE)
- A01 Toward Eastern Europe (ENMC)
- A02 ICT infrastructure (UNEW)
- A03 Research facilities and tools (UNEW)
- A04 Biobanks (EURORDIS)
- A04 Databases (INSERM)
- A05 Establishing the CTCC (MD-NET)
- A06 Network in Action – DMD (IC/FTELE/UNEW)
- A06 Network in Action - SMA (IC/FTELE/UNEW)
- A07 Pre-clinical studies (SNT)
- A08 Therapeutics (AFM/LUMC)
- A09 Efficacy measurements (FTELE/KCLSM)
- A10 Standards of care/diagnosis (KI/KCLSM)
- A11 International Collaboration and Dissemination (UNEW)
- A12 Training and education (ENMC)
- A14 Ethics and communication (UNEW/ENMC)
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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