



# TREAT-NMD

## Neuromuscular Network

2<sup>nd</sup> November 2007 · Newsletter No. 20



Welcome to the latest newsletter. This edition features a link to the questionnaire on ethical and patient concerns with regard to developing new therapies, and information on the TREAT-NMD Training and Education programmes. We also have a link to the NIH workshop report on translational research in muscular dystrophy held earlier this year. We would also like to encourage feedback from our readers to a proposal to establish a training and education module in standards of care (see page 6).

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

Best wishes,

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

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### 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](http://www.treat-nmd.eu/news/newsletter/index.htm). If you would like to subscribe directly, please visit our website at [www.treat-nmd.eu/](http://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](mailto: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.

### Questionnaire on ethical and patient concerns

As mentioned in the last edition, the European NeuroMuscular Centre (ENMC) and the University of Newcastle have sent out a questionnaire on ethical and patient concerns with regard to (the development of) new therapies. We are very interested in receiving responses from a wide range of participants, so if you were not on our questionnaire mailing list but have an interest in these issues, we would encourage you to download the questionnaire from our website by clicking here:

[http://www.treat-nmd.eu/assets/documents/TREAT-NMD\\_patient\\_questionnaire.doc](http://www.treat-nmd.eu/assets/documents/TREAT-NMD_patient_questionnaire.doc)

Please return completed questionnaires to [enmc@enmc.org](mailto:enmc@enmc.org)



### Santhera pilot study demonstrates beneficial effects of SNT-MC17 (idebenone) in DMD patients

Santhera Pharmaceuticals have recently announced positive, first results from a pivotal study with SNT-MC17 (idebenone) in Duchenne muscular dystrophy as measured by cardiac and respiratory parameters. This exploratory Phase IIa trial was a 12-month double-blind, randomized, placebo-controlled study conducted by Professor Gunnar Buyse at the University of Leuven, Belgium. In total 21 DMD boys between the age of 8 and 16 years were enrolled. There was no difference in the safety and tolerability of SNT-MC17 compared to placebo confirming the excellent safety profile of SNT-MC17 in DMD boys.



After treatment for twelve months with SNT-MC17, DMD patients showed a trend to improve cardiac function. In addition, patients on SNT-MC17 improved on certain respiratory parameters. Most striking and statistically significant was the improvement of DMD patients' lung function measured by peak flow. Patients treated with SNT-MC17 demonstrated improved lung function, while patients on placebo deteriorated over the study period.

Based on the positive results of this pilot study, Santhera, who is also an industrial partner of TREAT-NMD, will continue the clinical development of SNT-MC17 for DMD patients. The capabilities and expertise within the TREAT-NMD Network will help to ensure that clinical development and future trials are accelerated for the benefit of patients.

The full press release can be found on the Santhera web site at <http://www.santhera.com>

### TREAT-NMD Registry Training Course for Curators

Christophe Beroud and his team from the University of Montpellier (INSERM) will host the first TREAT-NMD registry curator training course from 7-9 November. Representatives of national patient registries for DMD and SMA will receive detailed information on the harmonized content of the registries and best practices.

Following the meeting we plan to host the training materials on the TREAT-NMD web site so curators can revisit the information and any new and upcoming national registries can access this valuable resource to help in their implementation and harmonization.

More than 50 participants from 16 countries from Europe and including Australia, Canada, Japan and the US have registered for the training course next week. This world-wide enthusiasm for the course provides evidence for creating a harmonized, global patient database for DMD and SMA—this will become a valuable resource for translational research.

### TREAT-NMD Training and Education Programmes

One of the important aims of the TREAT-NMD Network is to promote integration of different teams and disciplines in the area of neuromuscular disease. Also, the network aims to stimulate an integrated and multidisciplinary approach by physicians and researchers in the development of new diagnosis and treatment options that will lead to more optimal care for patients.

#### Training and exchange programme

TREAT-NMD aims at building a European network in neuromuscular diseases. The network should stimulate and enable cooperation between various partners in different countries and of different disciplines. To reach this goal a training and exchange programme will be set up, which will help researchers and physicians to exchange knowledge, expertise and techniques on site in a multidisciplinary way.

Apart from summer schools, training courses and dedicated workshops, we will set up an exchange programme, for both candidates within and outside the consortium.

Candidates willing to participate in the programme, as well as institutes willing to host, can apply to Pauline Evers at [evers@enmc.org](mailto:evers@enmc.org).

#### Young scientists training visits

Aim: Training and education                      Time span: 2 months maximum

Young scientists are defined as those in the first four years (full-time equivalent) of their research careers. These training visits are reserved for those who wish to work for a short period in a laboratory or a clinic in another country with the goal to:

- learn or develop new techniques
- use equipment or technology not available in their own lab
- gain experience with certain diagnostic tools
- gain experience with certain treatment methodologies

Candidates should submit their proposal, including CV and aim of their stay, to the proposed institution or to Pauline Evers at [evers@enmc.org](mailto:evers@enmc.org).

#### Senior scientists, exchange visits

Aim: exchange and networking                      Time span: 1-2 weeks maximum

Experienced researchers are defined as those who, at the time of recruitment, are, (i) in possession of a doctoral degree, or (ii) have at least four years of full-time equivalent research experience.

During the short stay dialogue can be started between laboratories / industrialists / clinical centres / agencies with complementary expertise in order to accelerate cooperation. Visits can be used to exchange know-how or technical issues or to give training on site to the personnel of the host institution.

Candidates can submit their proposal, including CV and aim of their stay, to the proposed institution or to Pauline Evers at [evers@enmc.org](mailto:evers@enmc.org).

Further information on all the training and education opportunities can be found on the TREAT-NMD web site at [http://www.treat-nmd.eu/activities/training\\_educ.htm](http://www.treat-nmd.eu/activities/training_educ.htm).

Please visit <http://www.treat-nmd.eu/news/item/?other-meetings-of-interest-to-treat-nmd-members> for further information on courses available outside TREAT-NMD.

**NIH Workshop on Translational  
Research in Muscular Dystrophy  
June 25-27, 2007**



**National Institutes of Health**  
The Nation's Medical Research Agency

The workshop report from the recent NIH Workshop on Translational Research in Muscular Dystrophy is now available online. John Porter gave a brief report on this at the recent TREAT-NMD Outcome Measures Workshop and we published a brief abstract in a previous edition of the TREAT-NMD Newsletter. The report highlights that international collaborations, such as that of TREAT-NMD, may be the only way forward to overcome the obstacles in translational medicine to ensure new therapies for muscular dystrophy.

The report is available from the NINDS website and via the link below;

[http://www.ninds.nih.gov/news\\_and\\_events/proceedings/Translational\\_Research\\_in\\_Muscular\\_Dystrophy.htm](http://www.ninds.nih.gov/news_and_events/proceedings/Translational_Research_in_Muscular_Dystrophy.htm)

For further information or if you have any questions about the report, please contact

Dr John D. Porter, Executive Secretary, Muscular Dystrophy Coordinating Committee (MDCC) via e-mail;  
[porterjo@ninds.nih.gov](mailto:porterjo@ninds.nih.gov)

## Meetings

**EMA-EPPIA Workshop on adaptive designs in confirmatory clinical trials**

**14 December 2007, EMA, London**

The potential to conduct clinical trials with an adaptive design has stimulated much excitement in the pharmaceutical industry, for both statisticians and non-statisticians alike. Considerable efforts have been made in academia, industry and regulatory circles in developing appropriate statistical and clinical trial methodology and in defining the boundaries for the use of adaptive designs in confirmatory clinical trials. Some controversial methodological issues remain, in particular those relating to sponsor involvement in interim decision making, assessing homogeneity of results from different trial stages and the regulatory acceptability of Phase II/III 'seamless' trials in the confirmatory setting.

One objective of this meeting is to gain a common understanding of the usefulness and the limitations of confirmatory trials conducted with an adaptive design. It is important that the implementation of 'adaptive' methodology is appropriate, in the early stages of its use so that the reputation of the methodology is not tarnished in regulatory circles. A further objective is to discuss the major areas of controversy which, perhaps appropriately, prohibit the wider use of such designs. The meeting is aimed at both statisticians and non-statisticians from the pharmaceutical industry and from regulatory agencies. A basic understanding of clinical trial methodology will be assumed, but extensive knowledge of statistical methodology will not be necessary to follow the presentations and resulting discussions.

Registration: Participants from Regulatory Authorities, Hospitals, Universities and Learned Societies should register using the registration form at <http://www.emea.europa.eu/meetings/conferences/14deco7.htm>. Participants from the Pharmaceutical Industry should contact Dr Christine-Lise Julou ([christinelisejulou@efpia.org](mailto:christinelisejulou@efpia.org)) at the EFPIA to register.

## The Association Francaise contre les Myopathies (AFM) current Call for Proposals for the second session: deadline is the 29<sup>th</sup> February 2008



This Call for Proposals is open to French and foreign groups.

AFM support includes the following:

- 1) Annual or biennial grants
- 2) Limited contracts for one year, for:
  - Young scientists for thesis work (Master's degree (M2) must already be acquired when the application is submitted) (renewable twice)
  - Post-doctoral fellows (renewable once, with very few exceptions)

AFM does not finance the second year of a post-doctoral fellowship abroad.

For more information please visit the AFM website:

[http://www.afm-france.org/afm-english\\_version/ewb\\_pages/r/research\\_callforproposals-2008.php](http://www.afm-france.org/afm-english_version/ewb_pages/r/research_callforproposals-2008.php)



## TREAT-NMD Neuromuscular Network

working with you to accelerate treatments for neuromuscular diseases

## Job and training opportunities

### Training Course in Standards of Care for SMA

The work on facilitating the implementation of the Standards of Care for SMA is in progress. We are currently working on a number of translations of the précis document that will be published on the web site as soon as they are completed. A Russian translation of the full paper by Wang et al., is already available on the web site. If you want to work on a translation of the Standards of Care we have funding to help with this task. Contact Stephen Lynn ([stephen.lynn@treat-nmd.eu](mailto:stephen.lynn@treat-nmd.eu)) for further information. To view the new Standards of Care section of our web site please visit <http://www.treat-nmd.eu/soc/>.

In addition, we are planning to establish a training/educational module in Standards of Care. This would either be a set of presentations, to be used by yourself, or we can offer a half or full day programme with speakers on the subject. The team of presenters would come to your site. The programme could be integrated for instance into your annual meeting of your national society or Neuromuscular study group, or as a single day symposium.

Before investing time and resources into this programme we would like to know whether there is an interest in organising and running such an initiative. Please express your interest (you do not have to have a defined date or plan at this time) if you feel this is a good idea and would be a benefit to you. Please contact Pauline Evers ([evers@enmc.org](mailto:evers@enmc.org)) to express your interest.

### Training Courses offered outside TREAT-NMD

We have listed many of the training opportunities available to the neuromuscular community on our web site. If you have a training course that you would like us to publicise on our web site then please contact Rachel Thompson ([rachel.thompson@treat-nmd.eu](mailto:rachel.thompson@treat-nmd.eu)).

## 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](mailto:rachel.thompson@treat-nmd.eu))

### Congratulations to Thomas Meier!

Thomas Meier, Chief Scientific Officer of Santhera, has been honoured with the prestigious BioValley Basel Award 2007 "Goldig Läggerli" for his contributions to the greater Basel area, his merits in advancing science in the field of neuromuscular diseases and for his successful founding of MyoContract AG, one of the two predecessor companies that in 2004 combined businesses to form today's Santhera.



### Governing Board Meeting — Summer 2008

We need to find a venue for the Governing Board meeting scheduled for the summer of 2008. If you would like to host this meeting please contact Stephen Lynn at the Coordination Office. Thank you.

## 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](mailto:emma.heslop@treat-nmd.eu)