

**TREAT-NMD Partner Newsletter No. 18
and Club of Interest Newsletter No. 12**

6th July 2007

Welcome to the twelfth newsletter for the TREAT-NMD Club of Interest. This week's edition features a report on Duchenne Standards of care by Liz Levy (CDC) and Thomas Sejersen (TREAT-NMD, Activity 10).

It's been a busy week for partners as we attended the first TREAT-NMD Governing Board Meeting in Naarden, The Netherlands at the start of the week. The meeting was a great success and a welcome opportunity for partners to update one another about their progress and discuss the future directions for the network. A full report of the meeting will appear soon.

We hope you enjoy the newsletter and look forward to hearing your comments – write to info@treat-nmd.eu with anything you'd like to say. Feel free to forward this message to anybody you think might find it of interest, or invite them to sign up to receive the newsletter by visiting our website.

Best wishes,

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

Newsletter contents

- 1. About this newsletter**
- 2. Working with us**
- 3. TREAT-NMD news and reports**
- 4. Upcoming conferences, meetings and workshops**
- 5. Pick of the press releases**
- 6. Partner-specific items**
- 7. Send us your news and views!**
- 8. Job opportunities**



1. About this newsletter

This is a weekly newsletter sent to all members of TREAT-NMD's "Club of Interest" worldwide. We are receiving new subscriptions all the time, so if you've missed the earlier editions of the newsletter and would like to catch up, please visit our newsletter archive online at <http://www.treat-nmd.eu/news/newsletter/> where you will find all back-issues. If you have received this letter from a friend or colleague and would like to subscribe directly, please visit our website at <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.

2. 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

3. TREAT-NMD news and reports

Governing Board meeting – a sneak preview

The first TREAT-NMD Governing Board meeting took place in Naarden at the beginning of this week. Discussions on a wide range of network activities took place, many future plans were made, and everyone present found it a valuable opportunity to share information on the progress of their activities during the first six months. A full report of the meeting will appear in a later edition of the newsletter, but in the meantime we thought we'd show you a picture...



Duchenne Standards of Care

Report by Liz Levy of CDC and Thomas Sejersen, leader of the TREAT-NMD Standards of Care activity

Duchenne Muscular Dystrophy Care Considerations Project

One of TREAT-NMD's key activities is to produce consensus documents on standards of care for patients with neuromuscular diseases. In order for these to be as valuable and comprehensive as possible, we are working closely with external groups (ICC for SMA and CDC for DMD) to create documents that are of worldwide relevance. The following report on progress towards this goal for DMD has been drawn up by Liz Levy of CDC and supplemented by TREAT-NMD's Thomas Sejersen.

The U.S. Centers for Disease Control and Prevention (CDC) is facilitating a multidisciplinary effort, in cooperation with TREAT-NMD, PPMD, MDA and other partner organizations, to develop a set of comprehensive care considerations to guide the disease management for boys with Duchenne muscular dystrophy (DMD). The goal of developing the comprehensive care considerations is to enhance the wellbeing of people who have DMD by promoting the best medical, functional, and psychosocial outcomes. These care considerations are intended for providers, patients, and families.

This project is utilizing the RAND Appropriateness Method which combines the best available scientific evidence with the collective judgment of experts to yield a statement regarding the appropriateness of specific assessments/interventions. Eight expert panels in the following topic areas are involved in this effort: 1) Respiratory, 2) Rehabilitation Management, 3) Orthopedics/Surgical, 4) Psychosocial, 5) Gastrointestinal/Nutrition, 6) Neuromuscular, 7) Cardiovascular, and 8) Diagnostics. The expert clinicians and researchers on these panels are rating the assessments and interventions for appropriateness and those that are deemed appropriate will then be rated for necessity. TREAT-NMD is represented by European participants on each of the panels – a great example of working together internationally to avoid duplicative efforts. For TREAT-NMD this collaboration is instrumental in achieving two important deliverables for Activity 10: – to develop standards of diagnosis and standards of care for DMD.

The eight groups have recently met in-person to discuss the rating materials and the results of the first round of ratings for appropriateness. The Appropriateness and Necessity studies will be completed throughout the summer and early fall, and publication in peer-reviewed journals is expected in 2008.

The resulting documents from this project, and summaries of these, will be the basis for the TREAT-NMD standards of diagnosis and care. Of course it is necessary not merely to produce these documents but also to obtain consensus on the recommendations and ensure their implementation. In order for this to be possible we need to identify appropriate routes of dissemination and implementation. We would therefore be grateful for suggestions from you all on which people/organizations we should involve in this important task. If you are in a position to be able to advise us on this matter, or if you are interested in how you can use the TREAT-NMD standards in your own country, please contact Thomas Sejersen at thomas.sejersen@ki.se.

A week of discussion on progress in translational research in muscular dystrophies

TREAT-NMD was represented at two meetings on progress in translational research in muscular dystrophies in the past ten days. The Association Monaguesque contre les Myopathies Round Table was held on June 23rd 2007 in Monaco focussing on the progress from early phase 1 trials in DMD to the development of therapies into clinical use and the NIH hosted a meeting in Washington. These excellent meetings preceded the TREAT-NMD/ DRCI meeting hosted in Naarden, the Netherlands on June 30th - July 1st. Reports on these three meetings will appear in the following weeks.



TREAT-NMD Partner Publications

The following manuscript was accepted for publication on 25th June 2007 from the Journal Human Gene Therapy.

Comparative analysis of antisense oligonucleotide sequence for targeted skipping of exon 51 during dystrophin pre-mRNA splicing in human muscle.

Arechavala-Gomez V, Graham IR, Adams AM, Popplewell LJ, Aartsma-Rus A, Kinali M, Morgan JE, van Deutekom JC, Wilton SD, Dickson G, Muntoni F.

4. Upcoming conferences, meetings and workshops



CliniGene High-scientific level symposium on Molecular Imaging

Open/public afternoon satellite session

Rotterdam, The Netherlands

27 October 2007

This initiative is organised by Clinigene in collaboration with both DiMI & EMIL EU-networks of excellence, coordinated by Andreas Jacobs & Bertrand Tavitian. If TREAT-NMD Partners would like to suggest topics for presentations at this meeting, based on their own Work Packages, please contact Odile Cohen-Haguenauer at odile.cohen@lbpa.ens-cachan.fr



XVth Annual Congress of the European Society of Gene and Cell Therapy

Rotterdam, The Netherlands

27-30 October 2007

Further information: <http://www.congrex.se/esgt/annualmeeting.html>

XVIth Annual Congress of the European Society of Gene and Cell Therapy

Bruges, Belgium

13-16 November 2008

Further information: <http://www.congrex.se/esgt/annualmeeting.html>

5. Pick of the press releases



Santhera Licenses Omigapil for Treatment of Rare Neuromuscular Diseases

Santhera to Develop Omigapil as Potential Treatment for Congenital Muscular Dystrophy

Liestal, Switzerland, July 2, 2007 - Santhera Pharmaceuticals (SWX: SANN), a Swiss specialty pharmaceutical company with a focus on neuromuscular diseases, announced today a licensing agreement with Novartis covering the compound omigapil for the treatment of Congenital Muscular Dystrophy (CMD). Under this agreement Santhera will develop omigapil as a potential treatment for this severe, genetically determined neuromuscular condition which frequently affects infants or young children with life-threatening progressive muscle weakness. Santhera expects to commence a Phase II trial in CMD patients by the end of 2008. Santhera also has the option to expand the development of omigapil (internal project number SNT-317) into other neuromuscular indications while Novartis retains a buy-back option confirming Novartis' continuing interest in the compound and its potential as identified by Santhera.



To read more please visit;

<http://www.santhera.com/index.php?docid=212&vid=&lang=en&newsdate=200707&newsid=1136797&newslang=en>

6. Partner-specific items

Discussion forums / lists

Would you like us to set up a discussion forum for you on the TREAT-NMD website?

If so, please e-mail rachel.thompson@treat-nmd.eu.

Calls for proposals / funding opportunities

Please forward to us at the Coordination Office any calls for proposals and funding opportunities you receive within your institution. We will then advertise these in the newsletter and on the website.

7. 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

8. Job opportunities

INSERM are seeking a fully bilingual Lab Manager (writing and speaking both English and French) for employment in their Research laboratory (Human Molecular Genetics, INSERM unit 827, University Hospital of Montpellier, South of France). Interested applicants should contact:

1. Jean-Michel DENIS

Responsable des Ressources Humaines

Administration Déléguée Régionale

INSERM Languedoc Roussillon

denis@montp.inserm.fr

<http://www.montp.inserm.fr>

2. Pr. Mireille CLAUSTRES, MD, PhD

Service de génétique moléculaire

CHU et INSERM U827

IURC, Institut Universitaire de Recherche Clinique,

Mireille.Claustres@montp.inserm.fr

Candidates will be required to complete a "Dossier de candidature" (<http://www.inserm.fr/fr/rh/>) for submission before July 18th 2007. Successful candidates will be required to present their application in September / October 2007.

