



TREAT-NMD

Neuromuscular Network

11th January 2008 · Newsletter No. 24



Welcome to the latest newsletter. This edition includes an announcement of a new course in neuromuscular disorders to be hosted at the National Hospital for Neurology and Neurosurgery, Queen Square, London, plus a job advertisement for a post in Newcastle working with the TREAT-NMD team.

TREAT-NMD will also be holding its Governing Board meeting next week (14-16th January) to discuss our progress in 2007 and to look forward to future activities. The TREAT-NMD Network has just entered its second year and we thank you for all your support.

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

From TREAT-NMD, we would like to wish you all a very happy and healthy New Year. We look forward to a successful and prosperous 2008.

Best wishes from 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. 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 Governing Board Meeting, 14-16th January 2008

Next week the TREAT-NMD Governing Board will meet in Milan, Italy, to discuss the network's outputs from the first year and its future activities. Immediately prior to the Governing Board meeting, the Scientific and Technological Advisory Council (STAC) will meet to review the network's achievements and advise on future plans. The Project Ethics Council (PEC) will also meet to discuss ethical issues raised by the network to date.

One of the challenges facing the TREAT-NMD Network is the issue of sustainability, and this will be further discussed to see if we can determine a roadmap to ensure that TREAT-NMD will continue in the long-term. The sustainability of networks such as TREAT-NMD is crucial if the support from the European Commission through the Framework Programmes is not to be wasted and the promise of new therapies and treatments is to be realised. TREAT-NMD will continue to work toward this goal of sustainability to ensure that all patients with NMDs benefit from this valuable infrastructure.

TREAT-NMD partner is made a Dame of the British Empire (DBE)

Professor Kay Davies of Oxford University has been made a Dame of the British Empire (DBE) for services to science in the New Years Honours list announced on 29th December 2007. Kay is the Director of the Medical Research Council Functional Genetics Unit, Co-Director of the Oxford Centre for Gene Function and a founding Fellow of the Academy of Medical Sciences. Kay's group is working on selecting, developing and characterising high throughput screening models for TREAT-NMD, and we would like to congratulate Kay on this latest recognition of her work.

Calls for Proposals

Marie Curie

1 International Staff Exchange Scheme (IRSES)

Deadline: 28th March 2008

http://cordis.europa.eu/fp7/dc/index.cfm?fuseaction=UserSite.PeopleDetailsCallPage&call_id=98

2 Industry-Academia Partnerships & Pathways (IAPPs)

Deadline: 25th March 2008

http://cordis.europa.eu/fp7/dc/index.cfm?fuseaction=UserSite.PeopleDetailsCallPage&call_id=97

3 International Re-integration Grants

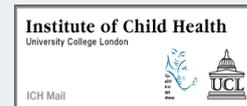
Deadlines: Continuous submission with cut-off dates for evaluation of 3rd April and 8th Oct 2008

http://cordis.europa.eu/fp7/dc/index.cfm?fuseaction=UserSite.PeopleDetailsCallPage&call_id=96

4 European Re-integration Grants

Deadlines: Continuous submission with cut-off dates for evaluation of 3rd April and 8th Oct 2008

http://cordis.europa.eu/fp7/dc/index.cfm?fuseaction=UserSite.PeopleDetailsCallPage&call_id=95



Update in Neuromuscular Disorders

Monday - Friday, 16 - 20 June 2008

at The Wolfson Lecture Theatre, National Hospital for
Neurology and Neurosurgery, Queen Square, London WC1N

This new course is the result of the merging of two popular yearly courses with an established international reputation: the "Hammersmith Hospital update" in the latest research aspects, clinical and management advances, related to childhood neuromuscular disorders, and the "Institute of Neurology Neuromuscular short course", a stimulating update on adult inherited and acquired neuromuscular disorders and marks the inauguration of the new MRC Centre for Neuromuscular Diseases and the move of the Dubowitz Neuromuscular Unit from Imperial College/ Hammersmith Hospital to the Institute of Child Health/ Great Ormond Street.

Course organisers; Francesco Muntoni, Adnan Manzur, Stephanie Robb, Mary Reilly and Mike Hanna

Topics to be covered include:

Duchenne MD – standards of care and latest research update;

Anaesthetic management of muscular dystrophies

The Limb Girdle muscular dystrophies;

Controversies in management of cardiac complications in muscular dystrophies

Assessment of respiratory muscle strength;

The floppy infant

Structural congenital myopathies

Congenital myasthenias; clinical and molecular genetic aspects

Spinal muscular atrophies: clinical aspects; functional scales; standards of care

Transition of care in neuromuscular disorders

Quality of life issues

Charcot Marie Tooth disease,

Chronic inflammatory demyelinating polyradiculoneuropathy

Peripheral nerve vasculitis

Paraproteinaemic neuropathies

Paraneoplastic neuropathies and myopathies

Infectious neuropathies

Muscle channelopathies

Mitochondrial myopathies

Myotonic dystrophy

<http://www.ucl.ac.uk/neuromuscular/mrccentre/>

for full programme when finalised

Ketty Schwartz, president of the Scientific Council of the AFM since 1998, has died

It is with deep sadness that the AFM has learned of the death of Ketty Schwartz on 25 December. She had been a member of the AFM's Scientific Council since its creation in 1981, and its president since 7 May 1998. She was also to have been the honorary president of the next AFM-organised international congress of myology – Myology 2008 – which will take place in Marseille next May.

For Laurence Tiennot-Herment, president of the AFM, and Christian Cottet, director general: "The French scientific community has lost one of its leading researchers, our Scientific Council a great president and our association its most loyal companion and guide. Just as we're setting out on the great turning point towards treatments, her absence will be felt all the more keenly."

TREAT-NMD would like to express their sincere condolences to Ketty's family, friends and colleagues.



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Articles

Leiden University Medical Center and Prosensa B.V. Announce New England Journal of Medicine Publication of First Successful Clinical Study with RNA-based Therapeutic in Duchenne Muscular Dystrophy

Novel RNA-based therapeutic PRO051 to enter Phase I/II clinical trials in DMD

Leiden University Medical Center (LUMC) and Biotech Company Prosensa announced on the 27th December 2007 in the *New England Journal of Medicine* (van Deutekom et al.) positive results from the first ever clinical study with RNA-based therapeutic PRO051 in four patients with Duchenne Muscular Dystrophy (DMD), in which PRO051 has restored dystrophin expression in the treated muscle fibers of all four patients with Duchenne Muscular Dystrophy (DMD) tested.

In this study, DMD patients between 10 and 13 years of age received a single injection of PRO051 (a 2'-O-methyl antisense oligonucleotide) in a small area of a muscle in the lower leg. In a biopsy taken four weeks later, novel dystrophin expression was observed in the vast majority of muscle fibers with protein levels that are expected to be clinically relevant. This pilot study is the first ever with an RNA-based therapeutic agent for DMD and is an important step towards treatment of this devastating disease, for which as yet no therapy is available. Following this first clinical proof-of-mechanism, Prosensa has started the preparations for a phase I/II clinical study to explore the effects and safety of PRO051 after repeated systemic injections.

Full article:

http://www.parentprojectmd.org/site/DocServer/NEJM_van_Deutekom_et_al.pdf?docID=3281

Editorial: *Skipping toward Personalized Molecular Medicine*, by Eric P. Hoffman, Ph.D.

<http://content.nejm.org/cgi/content/short/357/26/2719>

For more information, please contact: Gerard Platenburg: g.platenburg@prosensa.nl



MYOLOGY 2008 - May 26-30, 2008 - Convention Centre - Parc Chanot - Marseille, France

This is the **3rd international Myology congress** organized by the AFM since 2000. Thomas Voit will chair the congress.

As specified by Thomas Voit: "It is necessary for us to exchange, compare and learn from others how to progress, such are the objectives of this congress. And this will all be done in a collaborative frame of mind". This unique gathering of international myology experts is of great importance for the community of 'myologists' because it encourages exchanges necessary to overcome the obstacles for the development of innovative therapeutics and the establishment of clinical trials in man for neuromuscular diseases.

Among the developments for this Myology 2008 edition is the advocacy to approach both themes of the conference each day -fundamental and therapeutic- across transverse subjects or pathology. Another novelty is that the last day of the congress will also be the **1st day of the 5th international congress of rehabilitation in neuromuscular diseases** (May 30 to June 1, 2008).

Please visit the web site of the congress www.myology2008.org to get the programme, submit your posters and register on-line.

Submission of posters before 6th February 2008.



TREAT-NMD Neuromuscular Network

working with you to accelerate treatments for neuromuscular diseases

Job and training opportunities

Database Curator (50%) Institute of Human Genetics, Newcastle University, UK



The post will be based in the TREAT-NMD Coordination Office at the Institute of Human Genetics, Newcastle upon Tyne., UK and is tenable until the end of 2011 .

The role of this post will be to manage and develop the UK patient database, reporting to Professor Hanns Lochmuller and Professor Volker Straub. It will be the post holders responsibility to ensure that patient information is correctly monitored, stored and linked with the TREAT-NMD Global Patient Registry. The post holder will be responsible for the coordination of the UK national registry, and will also be expected to play an interactive role in communication with partners across Europe and the world. The specific work will be to lead on the development of a durable registry for future clinical trials, as well as to work with the project management team to ensure timely reporting. This is a diverse and challenging position for a person with excellent communication skills and an understanding of genetics with a background in academia, clinically-related disciplines or the pharmaceutical industry.

Informal enquiries can be made to Professor Hanns Lochmuller (hanns.lochmuller@ncl.ac.uk)

Closing date: 18th January 2008

Please forward your application, quoting the appropriate job reference number (A1109C), to: Human Resources, Newcastle University, William Leech Building, The Medical School, Framlington Place, Newcastle upon Tyne, NE2 4HH. You can download an application form from the TREAT-

Partner-specific items

TREAT-NMD Governing Board Meeting 14th-16th January, Milan, Italy

We are looking forward to seeing our partner representatives at what promises to be an exciting and productive meeting.



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