

welcome

Welcome to the latest TREAT-NMD newsletter.

This edition includes:

- an update on the TREAT-NMD public consultation
- a meeting report from the biochemical outcome measures working group
- information on AFM's 2012 call for proposals
- a reminder to submit abstracts for the American Academy of Neurology 2011 meeting

We would like to thank those who have contributed to this edition. This newsletter relies on input from our readers. If you have anything you wish to be included in the next newsletter please contact us at info@treat-nmd.eu.

at a glance...

12-16 Oct 2010 World Muscle Society International Congress - Kumamoto, Japan

21-22 Oct 2010 FSH Society FSHD International Research Consortium Meeting - Watertown, MA, USA

11-12 Nov 2010 International Workshop for Glycosylation Defects in Muscular Dystrophies - Charlotte, North Carolina USA

12-13 Nov 2010 Action Duchenne 8th Annual Conference - London, UK

16 Nov 2010 Europlan Conference - Manchester, UK

19-20 Nov 2010 Steps Forward in Pompe Disease - London, UK

9-11 Dec 2010 The 3rd Latin American Summer School of Myology EVELAM - Córdoba, Argentina

15 Dec 2010 Systemic delivery of AAV for neuro-muscular gene therapy - Evry, France



Thanks for your responses to the TREAT-NMD public consultation!

We were delighted by the response to our public consultation, which closed last Friday. In total we received 430 submissions from all over the world. Responses were received from all stakeholder groups, from affected individuals and families to leading specialists and industry, and we would like to thank everyone who participated in what was quite a lengthy and in-depth questionnaire!



The aim of the consultation process was to solicit feedback from the neuromuscular community on the impact of TREAT-NMD's activities to date and to invite comments on the future activities, governance and funding mechanisms available to the network. TREAT-NMD is currently funded by the European Commission, and the feedback received in the consultation will enable the network to define its future priorities as it moves towards a new structure from 2012.

We have now begun to analyse all the submissions and we aim to publish a full consultation report by early November to allow all our stakeholders to see the advice we have received from the community. As well as numerous helpful suggestions and identification of future priorities, we were particularly pleased to note that the feedback on the practical utility of the TREAT-NMD infrastructure was overwhelmingly positive, with 90% of those who responded to the question on defining a roadmap for their disease area agreeing that TREAT-NMD tools, services and infrastructure would be useful in supporting their plans.

I would like to take this opportunity to thank all those who completed the consultation questionnaire. Your feedback will help shape the future of TREAT-NMD.

Dr Stephen Lynn - TREAT-NMD Network Manager

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Biochemical Outcome Measures in DMD: Meeting summary

On September 21st 2010, 30 academic and industrial experts from Europe and US met at The Institute of Child Health, University College, London, UK as part of the Biochemical Outcome Measures initiative in Duchenne Muscular Dystrophy (DMD). The aim of this meeting was to evaluate the accuracy of different methodologies for quantifying dystrophin in skeletal muscle biopsies, to generate a detailed Standard Operating Procedure for distribution to all clinical centres/industrial partners involved in DMD clinical trials. The meeting was organized by TREAT-NMD and the Children's National Medical Center (CNMC) and supported by CureDuchenne, The Foundation to Eradicate Duchenne, Ryan's Quest, Charley's Fund and Duchenne Ireland. Representatives from the funding parent organizations were invited to attend this meeting.



The Biochemical Outcome Measures initiative arose from a regulators' directive at the September 2009 EMA-TREAT-NMD meeting. Here, it was highlighted that since multiple drugs are advancing along the regulatory pathway, drug safety and efficacy evaluation need to be improved by developing appropriate biochemical measures based on sensitivity, reliability and variance. The first biochemical outcome measure to be drawn-up is the development of a sensitive and reliable methodology for quantifying the biochemical marker dystrophin in muscle biopsies. This will serve as a surrogate biochemical marker for use in DMD clinical trials aimed at increasing endogenous dystrophin, or exogenous dystrophin delivery. The intention is to work in close collaboration with the regulatory

[21-22 Feb 2011 7th Annual Update Symposium on Clinical Neurology and Neurophysiology - Tel Aviv, Israel](#)

[24-26 Mar 2011 International Congress of the European Society of Magnetic Resonance in Neuropediatrics - Amsterdam, Netherlands](#)

[29-30 Mar 2011 The Fourth UK Neuromuscular Translational Research Conference - London, UK](#)

[9-16 April 2011 63rd American Academy of Neurology Annual Meeting - Honolulu, Hawaii](#)

[9-13 May 2011 Myology Congress - Lille, France](#)

[18-22 Oct 2011 WMS Conference 2011 - Algarve, Portugal](#)

[8-11 Nov 2011 TREAT-NMD International Conference - Geneva, Switzerland](#)

Please note: This is only a selection of upcoming meetings. To see all our listed meetings [click here](#).

authorities, namely the FDA and the EMEA. This is in order for the test to be developed and qualified for its use as a technique, and to allow the precise quantification of this surrogate marker. The outcome of this initiative will be published in a peer-reviewed journal for use by others in future clinical trials.

Five months prior to this meeting, seven participating labs across Europe and US who are international experts in the field of Duchenne muscular dystrophy, received frozen muscle samples from the same blinded biopsies - three control and seven different Becker muscular Dystrophy- from the CNMC. Each lab analyzed the biopsies for dystrophin content using 1-2 methods of their choice, performing each analysis a minimum of three times/biopsy. The results were passed to an independent biostatistics consultant to provide impartial data analysis. Each participating lab then presented their findings at this meeting for general discussion. Also invited to this meeting were industrial representatives from PTC Therapeutics, AMTBioPharma, AVI Biopharma, GSK and Genzyme, some of whom presented their dystrophin quantification methods.

At the meeting a wide-range of immunoblotting and immunofluorescence quantification techniques were presented. A particular immunoblotting technique was identified as providing the most accurate dystrophin quantification method and this will now be validated by independent labs before a SOP is distributed. All participants at the meeting agreed that the collective data presented at this meeting was suitable for publication in a peer-reviewed journal, since there are no prior publications which compare these techniques for their use in dystrophin quantification.

Co-Chairs

Eric Hoffman, PhD
Francesco Muntoni, MD, PhD

Presenters

Karen Anthony, PhD
John Babiak, PhD
Kristl Claeys, MD, PhD
Judith van Deutekom, PhD
Heather Gordish-Dressman, PhD
Alessandra Ferlini, PhD, MD
Kevin Flanigan, MD
Diane Frank, PhD
Steve Moore, MD, PhD
Bart Nijmeijer, MSc
Thomas Voit, MD, PhD

Other Participants

Abby Bronson, MBA
Jenny Morgan, PhD
Silva Torelli, PhD
Juliet A. Ellis, PhD
Volker Straub, PhD, MD
Emma Heslop, MSc
Annemieke Aartsma-Rus, PhD
Maaïke van Putten, PhD
Gaetano Vattermi, PhD
Patrizia Sabatelli, PhD
Anneke Janson, PhD
Jessica Sipkens, PhD
Laura Taylor, PhD
Lucy Feng, PhD
Bernard Benichou, PhD
Allen Reha, PhD
Debra Miller

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AFM 2011 Call for Proposals

The second round of proposals for AFM financing closes **4th March 2011** with applicants being notified in mid July 2011.

AFM wish to support research that will...

- Lead to a better fundamental understanding of the neuromuscular system
- Encourage the development of therapies for neuromuscular diseases and rare genetic diseases
- Improve care and quality of life of patients with neuromuscular diseases

This call for proposals is open to both French and foreign groups and the types of financial packages available include a one year trampoline grant for innovative investigators who are early in their science career, one year research projects, PhD fellowships and postdoctoral fellowships.

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American Academy of Neurology abstract deadline 25th October

Neuromuscular researchers are encouraged to submit abstracts to the 63rd American Academy of Neurology Annual Meeting to be held April 9 – 16, 2011 in Honolulu, Hawaii.

The deadline for abstract submission is October 25, 2010. Please click [here](#) to submit an abstract. If you are not currently a member of AAN, you may join [here](#).



The SMA Foundation notes that one of the Integrated Neuroscience Sessions for 2011 will be Gene Therapy and Treatment of Motor Neuron Disease, which might be of interest to some of the SMA investigators. For other details on the meeting you can go to the official AAN Meeting website: [63rd AAN Annual Meeting](#).

The Foundation also comments that number of presentations at AAN related to Spinal Muscular Atrophy increased in 2010 by 30% from 2009. It's exciting to see the progress being made toward increasing the visibility of the SMA community and the important research that is taking place.

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