

welcome

Welcome to the latest TREAT-NMD newsletter.

This week's edition includes...

- an announcement about a neuromuscular stakeholder meeting with FDA / NIH
- a look at 'The Art of TREAT-NMD', an arts-based research project
- news of a podcast service from the Nationwide Childrens' Hospital in Ohio
- information about the venue alteration for the MDR2010 meeting

We would like to thank those who have contributed to this week's 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...

[12-15 Jun 2010 European Human Genetics Conference - Gothenburg, Sweden](#)

[17-19 Jun 2010 International Conference on Neuromuscular Diseases - Sao Paulo, Brazil](#)

[19-23 Jun 2010 20th Meeting of the European Neurological Society - Berlin, Germany](#)

[24-27 Jun 2010 Parent Project Muscular Dystrophy Annual Conference - Denver, Colorado, USA](#)

[24-27 Jun 2010 Families of SMA Annual Conference - Santa Clara, California, USA](#)

[15-16 Jul 2010 MDR2010 - Padova, Italy](#)

[19-23 Jul 2010 12th International Congress on Neuromuscular Diseases - Naples, Italy](#)

[30 Jul - 1 Aug The Biennial FSHD International Patient and Researcher](#)



Neuromuscular stakeholders to meet with FDA and NIH on AON therapies in September

We are pleased to announce the upcoming FDA and NIH co-sponsored meeting titled Antisense Oligonucleotide (AON) Therapies in Neuromuscular Diseases, taking place on the 27th and 28th September, 2010 in Washington DC, USA.



Given the growing number of AONs entering clinical development for multiple different neuromuscular disorders, the FDA and NIH, in collaboration with the research and advocacy communities, are taking a proactive role in developing and promoting regulatory science for the AONs by providing a forum for neuromuscular disease stakeholders to present the 'state-of-the-science' and exchange information on issues relevant to the AONs.

The meeting will focus on four sessions:

- Toxicology and Preclinical Findings to Date
- Biomarkers
- Clinical Trial Outcomes
- Patient Registries and Assessing Long-Term Outcomes

and will be concerned with four neuromuscular disorders:

- Amyotrophic Lateral Sclerosis
- Duchenne Muscular Dystrophy
- Myotonic Dystrophy
- Spinal Muscular Atrophy

The goal of this meeting is to allow stakeholders to explore potential pathways forward for the AONs with the eventual goal of creating a sound scientific anchoring for neuromuscular disease clinical development programs. These initial discussions should be seen as first steps in what is likely to be an iterative process intended to lay the ground work for future collaborations.

Over 120 stakeholders will be invited to participate in the meeting, consisting of researchers, academics, industry and patient group representatives. In order to accommodate all those unable to attend the meeting due to space limitations or geographical distance, the whole meeting will be streamed on the web via a dedicated weblink (to be made available shortly).

Additional details will follow in due course. In the meantime, if you have any specific queries please contact [Abby Bronson](#) or [Emma Heslop](#).

The Steering Committee Chairs

Kate Bushby, Newcastle, TREAT-NMD
Ed Connor, CNMC
Elizabeth McNeil, FDA
John Porter, NIH

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Network Meeting - Las Vegas, Nevada, USA

29 Aug - 1 Sep European Meeting on Next Generation Sequencing - Leiden, Netherlands

9-10 Sept 2010 MD2010 - 4th conference for the Muscular Dystrophy Association of Western Australia - Perth, Western Australia

18 Sept 2010 Muscular Dystrophy Campaign Conference - Birmingham, UK

20-22 Sept 2010 Muscle Study Group Annual Meeting - Buffalo, NY, USA

4-5 Oct 2010 7th UK SMA Researchers' Conference - Llanwyddyn, Wales, UK

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

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

16 Nov 2010 Europlan Conference - Manchester UK

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

Language Lab: rethinking medical communication through art and metaphor

"If your DNA is like a recipe book, a mutation is like a typo that causes you to make the wrong dish."

"Imagine you're a computer; the hardware is fine but your software needs some attention."

TREAT-NMD is collaborating on a research project with artist Marianne Wilde that will be looking at the ways in which doctors, patients and scientists communicate both visually and linguistically when explaining the complexity of genetic diseases. The use of linguistic metaphors is commonplace when interpreting the how, what, why and where of DNA and it is these types of metaphor that will form the basis of the investigation.

As TREAT-NMD coordinator Professor Volker Straub explains, *"In a highly specialized and complex field like that of inherited muscle diseases, specialists tend to simplify complex facts related to genetic diagnosis, disease mechanisms and potential treatment strategies by using metaphors, analogies and models. Patients and families do the same thing when talking about their conditions. Based on our differing backgrounds we visualize and reflect on things in different ways and Marianne's project is exploring these processes by using art as a more general, non-linguistic concept. Particularly in a multinational, multilingual network like TREAT-NMD, this kind of project has the potential to give us new insights into ways of explaining the diseases we deal with every day."*

Communications of this type are often referred to as scientific metaphors and are used generally throughout the scientific community. (Evidently the Milky Way looks like a fried egg.) These terms are often widely debated and are constantly being developed by scientists, writers, artists and indeed the general public.

This three year AHRC funded research project is being carried out by Marianne at Northumbria University and is entitled *Words as things: visual metaphors and scientific explanations in the context of arts and health research*. Over the course of the project the metaphorical language used within the network will be collected, interpreted into visual art forms and an archive created.

By identifying gaps in communication between the network stakeholders of TREAT-NMD and studying how linguistic, visual and artefactual metaphors impact on the construction of technical explanations within this network it is hoped that we can come closer to answering how we can make a 'thing' that we cannot see into something that we can say? Or, conversely, how can we make a 'thing' that we cannot say into something that we can see?

You can follow the project and contribute by adding metaphors at the Language Lab website, which can be found at www.theartoftreat-nmd.eu

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US children's hospital offers podcasts focusing on neuromuscular disorders

Scientists and clinicians at the Nationwide Children's Hospital in Columbus, Ohio have launched a monthly podcast entitled, "This Month in Muscular Dystrophy," featuring internationally known scientists discussing the latest research in muscular dystrophy and other neuromuscular disorders.

The programs are posted each month at www.NationwideChildrens.org/muscular-dystrophy-podcast.

The podcasts are geared toward patients, their families and primary care physicians who take care of patients with neuromuscular diseases.

Hosted by Kevin Flanigan, MD, an attending physician in Neurology at Nationwide Children's Hospital and a principal investigator in the Center for Gene Therapy in The Research Institute at Nationwide Children's, the programs include interviews with authors of recent scientific publications discussing how their work improves understanding of inherited neuromuscular diseases and what their findings might mean for treatment.

The initial programs include:

- Jerry Mendell, MD, director of the Center for Gene Therapy in The Research Institute at Nationwide Children's Hospital and professor of Pediatrics and Pathology at The Ohio State University College of Medicine, discusses gene transfer of follistatin and the implications for muscle disease.
- Brian Kaspar, PhD, an investigator in the Center for Gene Therapy in The Research Institute at Nationwide Children's and assistant professor of Pediatrics at Ohio State, explains exciting new results in animal models of gene transfer in spinal muscular atrophy.
- Kate Bushby, MBChB, MD, professor of Neuromuscular Genetics at Newcastle University (UK), discusses the recent publication of care standards in Duchenne muscular dystrophy (DMD). These standards address the diagnosis and management of DMD, including pharmacologic, orthopedic and psychological management. Dr. Bushby is also the coordinator of TREAT-NMD (Translational Research in Europe – Assessment and Treatment of Neuromuscular Diseases), a European network focusing on bringing advances in neuromuscular disease treatment rapidly and effectively to patients.



Nationwide Children's Hospital Podcasts

"There is a lot of exciting work going on in the field of neuromuscular disease, and for patients and their families, it may be hard to get access to information about new results," Dr. Flanigan said. "Our goal in offering this monthly podcast is to provide a way for people affected by the muscular dystrophies and related disorders to hear directly from top researchers about their latest results. It's my job to converse in understandable terms with these researchers about what is useful or exciting in their work."

Patients and their families are eager to find reliable information, especially about what new therapies are entering trials. These monthly podcasts will provide reliable information directly from leading scientists and physicians in the field to empower patients to take the information they learn into their own clinics to discuss with their doctors. The podcasts also serve to provide reliable information to primary care physicians who often have the most contact with patients who have neuromuscular disorders.

"Through these podcasts, I think we can reinforce the hope shared by all families, and let them know that many pathways that may lead to meaningful treatments are being explored," Dr. Flanigan added.

Dr. Flanigan's primary research interest is in the genetic and molecular characterization of inherited neuromuscular diseases and the development of therapies directed toward these diseases.

Neuromuscular Disorders at Nationwide Children's Hospital is comprised of a multidisciplinary staff that diagnoses and treats disorders affecting peripheral nerves and muscles in children. Scientists in the Center for Gene Therapy in The Research Institute at Nationwide Children's investigate and employ the use of gene and cell-based therapeutics for prevention and treatment of neuromuscular and neurodegenerative diseases, lysosomal storage disorders, ischemia and re-perfusion injury, neonatal hypertension, cancer and infectious diseases.

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MDR2010 to be held in Padova

The MDR2010 conference due to be held 15-16 July in both Venice and Padova will now wholly take place in Padova. The venue consolidation should help those attending the ICNMD (International Congress on Neuromuscular Diseases), which begins 17th July, to make an easier transfer to Naples.



Registration for the MDR2010 meeting begins at 13:00 on 15th July in Padova.

Day one is centred around the progress in treatment of muscular dystrophies, this is then followed on day two by concentrating on the progress in glycogen and lipid myopathies treatment.

Abstracts should be submitted to lab.neuromuscolare@unipd.it by **21 June 2010**

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The adjusted program is now available on the events section of the website, please click [here](#).

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