

welcome

Welcome to the latest TREAT-NMD newsletter.

This edition includes:

- an update on the Neuromuscular Stakeholders meeting with FDA and NIH on AON therapies
- a report from the FSMA meeting held in June
- news that the 6 minute walk test can now be accessed via the registry of outcome measures website
- the first BIO-NMD newsletter

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...

[16-19 Sept 2010 EAMDA 40th Annual General Meeting - Milan, Italy](#)

[18 Sept 2010 Muscular Dystrophy Campaign Conference - Birmingham, UK](#)

[20-22 Sept 2010 Muscle Study Group Annual Meeting - Buffalo, NY, USA](#)

[27-28 Sept 2010 Neuromuscular stakeholders to meet with FDA + NIH on AON therapies - Washington, USA](#)

[01-02 Oct 2010 European Research Conference in Paediatric Neurology - Leuven, Belgium](#)

[04-05 Oct 2010 7th UK SMA Researchers' Conference - Llanwyddyn, Wales, UK](#)

[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](#)



Update on the Neuromuscular Stakeholders meeting with FDA and NIH on AON therapies

We are pleased to announce that the agenda and live web streaming links are now available for the FDA and NIH co-sponsored meeting 'Antisense Oligonucleotide Therapies in Neuromuscular Diseases', taking place on the 27th and 28th September, 2010 in Washington DC, USA.



The collaborative meeting will engage over 100 stakeholders from multiple different neuromuscular diseases in which antisense oligonucleotides (AONs) are being explored as a therapeutic strategy (amyotrophic lateral sclerosis, Duchenne muscular dystrophy, myotonic dystrophy and spinal muscular atrophy). It is sponsored by Children's National Medical Centre, CureDuchenne, Foundation to Eradicate Duchenne, MDA, Parent Project Muscular Dystrophy and TREAT-NMD.

The meeting will consist of four sessions:

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

Its goal is to allow stakeholders to explore potential pathways forward for the AONs with the eventual goal of creating a sound scientific foundation for neuromuscular disease clinical development programs. These initial discussions should be seen as first steps to help pave the way for future discussions and collaborations.

The agenda will be available in the near future at [this page](#).

Over 100 stakeholders, consisting of researchers, academics, industry and patient group representatives, will participate in the meeting. 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 dedicated weblink(s).

To connect to the web streaming please click [here](#).

If you have any specific queries regarding this meeting please contact [Emma Heslop](#) (TREAT-NMD), [Abby Bronson](#) (CNMC) or [John Porter](#) (NIH).

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The 6 Minute Walk Test - ROM record for SMA now available

With many thanks to the ICC and members of the Outcome Measures working party, and with particular thanks to Julaine Florence for entering the information into the Registry of Outcome Measures (ROM), we are happy to advise that we recently published a detailed summary record of the 6MWT for application in SMA.

Timed Tasks	
Level of Difficulty/Resistance	Self Determined
Time Allowed	Pre-determined 6 Minutes
Rest: Permitted/Required	Rest is Permitted NB: 2/ participants

The Six-Minute Walk Test (6MWT) is an objective evaluation of functional exercise capacity which measures how far a person can walk in six minutes (ATS 2002). It is a global measure of multiple body systems including cardiopulmonary, vascular and

[12-13 Nov 2010 Action Duchenne
8th Annual Conference - London, UK](#)

[16 Nov 2010 Europlan Conference -
Manchester, UK](#)

[29-30 Mar 2010 The Fourth UK
Neuromuscular Translational
Research Conference - London, UK](#)

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

neuromuscular.

The ROM record contains a detailed summary of the conduct of the test, a snippet of which is provided herewith, and also includes links to several reference papers related to application of the 6MWT in a variety of diseases. We aim to add more information to the record as it comes to hand. For more details of the 6MWT, visit www.researchrom.com

And, if anyone else would like to dedicate some time to entering detailed information into ROM for their favourite OM, or perhaps just wants to alert us to an OM that they would like to know more about, we'd be delighted to hear from you. Just email the Registry Coordinator - Joanne.Auld@iop.kcl.ac.uk

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First BIO-NMD newsletter now available

BIO-NMD is a 3 year EU-funded research project focusing on Duchenne and Becker muscular dystrophies and collagen VI-related myopathies (which includes Ullrich congenital muscular dystrophy and Bethlem myopathy). The project is searching for 'biomarkers' in people with these conditions. Biomarkers are substances in the body that offer a way to measure normal or abnormal processes. Measuring the levels of these biomarkers in clinical trials can show researchers clearly and accurately whether the drug being tested has had an effect or not.



A BIO-NMD patient-focused newsletter has now been published. As it is the first edition, it contains a general overview and explanation of what BIO-NMD is doing as well as who is involved, a diary of events and other news from related projects and organisations.

There is also a topical main article about an important issue for everyone involved in medical research whether as a patient, a clinician or a research scientist – the first issue focuses on the ethical issues in biobanking. The link below will take you to the newsletter where your comments and feedback are most welcomed.

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Highlights from 14th Annual SMA Research Group Meeting now available on FSMA website

The International Spinal Muscular Atrophy Research Group Meeting is the biggest SMA research conference in the world. Families of Spinal Muscular Atrophy organizes the conference, and financially underwrites the meeting by covering hotel, travel, registration for all research presenters, a \$1,000 investment per person. The meeting this year was held in Santa Clara, California, at the end of June. 210 researchers attended from around the world, representing 13 countries, 70 academic / government / non-profit institutions, and 12 biotech and pharmaceutical companies.



This conference, the only open venue for annual communication between international SMA researchers, has tangible benefits for the entire SMA community:

Enables open communication of early, unpublished scientific data among researchers - a key component in accelerating the pace of research. Creates a vital sense of community among SMA researchers that generates a collaborative spirit, resulting in many productive research partnerships. Allows cross-disciplinary dialog among basic researchers, clinicians, and industry representatives that is vital in creating effective therapies. Allows young researchers to interact with leaders in the field, which helps build the future of the SMA research community. Motivates SMA researchers by allowing for direct interaction with families and patients living with SMA.

The conference is organized into several sessions focusing on major unanswered topics in the field. This year there were five distinct sessions with an expert moderator leading the discussion of each.

Additionally, two poster sessions occurred, allowing for presentation of very current and still under investigation research.

The podium sessions included the following topics: Human SMA Studies, Regulation of SMA Expression and Function, New Animal Models of SMA, SMN in Motor Neurons, and Drug Discovery.

Overall, 38 podium talks were given and over 70 posters presented.

For additional information on Research Findings Presented at the 2010 Conference, please visit the [FSMA website](#)

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