24 Sept 2010
TREAT-NMD newsletter no. 86
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welcome

Welcome to the latest TREAT-NMD newsletter.

This edition includes:

- a reminder about the close of our public consultation

- a user survey on Cochrane reviews

- agenda and access to streaming infomation for the upcoming FDA / NIH meeting on AONs

- a workshop on systemic delivery of AAV for neuromuscular gene therapy

- the 3rd EVELAM meeting in Argentina

- guidelines to increase patient involvement in the research process

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...

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

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

Last chance to respond to TREAT-NMD public consultation

TREAT-NMD is currently funded as a 'Network of Excellence' by the European Commission(EC) and its activities for its initial 5 years (Jan 2007-Dec 2011) are clearly defined in a work plan or "Description of Work" approved by the EC. After 2011, the structure of the network will evolve as new funding sources are brought in and new partners have the opportunity to play a more active role in the network.

To make sure the future structure and activities of the network reflect the needs of all its stakeholders we need your input via the consultation questionnaire. We would also be grateful if you could dissemminate this information to any colleagues and interested parties who wish to have a say in the future of TREAT-NMD.

Our online questionnaire can be found **here** with a closing date for responses of **Friday 1st October 2010**.

Further details along with documents to help you complete this questionnaire can be found on our dedicated **consultation section** of the website.

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Cochrane Neuromuscular Disease Group seeks input on future strategy

The **Cochrane Neuromuscular Disease Group**, under the editorship of Professor Richard Hughes and now Dr Michael Lunn, has been part of the TREAT-NMD project since the beginning. Twenty two high quality reviews of interventions for neuromuscular conditions have been prepared in five years and a further ten are in preparation.

As TREAT-NMD moves into a new phase we would welcome your opinions on the past, current and future work of the Cochrane Neuromuscular Disease Group. A 3-question questionnaire can be found by following this link. Please be frank.

About the Cochrane Neuromuscular Disease Group
The Cochrane Collaboration seeks to undertake systematic reviews of evidence for interventions into medical conditions. Systematic reviews include only the highest quality evidence available for a given condition. They are kept up to date and are freely available on the Web to anyone in the UK and most European countries as well as the developing world in a number of formats (for example as 'papers', podcasts, PICO digests and journal clubs) and can be found at **www.thecochranelibrary.com**.

The Cochrane library of reviews summates the available evidence for these diseases. Why should we do intervention reviews 'that state the obvious'? Indeed some reviews are 'empty' as there is no high quality evidence available to summarise; why take the trouble to undertake and publish these?

- Firstly the obvious is not always true. Many of the treatments to which we expose patients, simply don't work and we need to start thinking about alternatives. Occasionally a meta-analysis will

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

16 Nov 2010 Europlan Conference - Manchester, 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

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

Please note: This is only a selection of upcoming meetings. To see all our listed meetings click here.

demonstrate efficacy not evident from the individual trials; without doing the work it is impossible to know.

- Secondly it is evident that many trials have used outcome measures or time-points which are inappropriate, not validated or not responsive. Cochrane reviews can highlight this and help to develop appropriate uniformity.

- Cochrane reviews are available to all. Our audience is huge: international researchers, doctors, funding agencies, healthcare providers, patients and carers all read Cochrane Reviews for different purposes. What may be obvious to you may not be obvious to them without the evidence being digested and summarised appropriately.

- Lastly, when we are considering undertaking a new trial it is unethical to start without first accessing a systematic review of the literature, or performing such a review if it has not been done. A systematic review will identify potential duplication of work already performed, find answers that may already be known and identify outcome strategies that might be or might not be appropriate.

We are always looking for high quality author teams to write reviews of interventions with us and look forward to any contact you wish to make please email Dr Michael Lunn for further details.

We look forward to your input into our future development.

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Agenda and weblinks now available for 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 available for the FDA and NIH co-sponsored meeting 'Antisense Oligonucleotide Therapies in Neuromuscular Diseases', taking place on Monday 27th and Tuesday 28th September, 2010 in Washington DC, USA. We hope that stakeholders unable to attend the meeting in person, due to the limited number of spaces available, will be able to utilise these resources to follow the presentations and discussions.

The collaborative meeting will engage approximately 120 stakeholders from multiple different Neuromuscular Muscular Diseases concerned with AON development (Amyotrophic Lateral Sclerosis, Duchenne Muscular Dystrophy, Myotonic Dystrophy and Spinal Muscular Atrophy) and is sponsored by Children's National Medical Centre, CureDuchenne, Foundation to Eradicated Duchenne, MDA, Parent Project Muscular Dystrophy and TREAT-NMD.

The meeting will be structured into four sessions:

1: Toxicology & Preclinical Findings to Date
2: Biomarkers
3: Clinical Trial Design / Endpoints
4: Patient Registries and Assessing Long-Term Outcomes

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 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.

To download the full agenda please click here.

To connect to the web streaming please click here.

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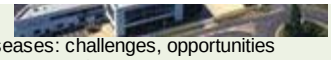
Systemic delivery of AAV for neuromuscular gene therapy - workshop in Evry, France

A workshop on the systemic delivery of AAV for neuromuscular gene therapy will take place in Evry, France on 15th December 2010.

Topics for this workshop will include..



- Intravascular AAV delivery in the context of neuromuscular diseases: challenges, opportunities
- General overview on practical aspects of preclinical studies (POC, TOX)
- Am I ready for a loco-regional muscular gene transfer IND? Items that must be covered by the IND file

Four round table workshops will follow

- Non-regulatory preclinical studies: route and dose rationales, safety pharmacology studies. What should be covered before toxicology studies?
- Regulatory Toxicology and Biodistribution of the product: How to choose the best model(s) and protocol(s) in animals to forecast worst-case tox effects?
- Surgical (isolated limb local perfusion) potential side-effects and local toxicity: what is the best animal setting for a pertinent surgical model?
- Immune response / Immunotoxicology topic. What do we know about the potential immune-related side effects on efficacy and safety? Immune suppression?

The final workshop will be delivered by **Thomas Voit** and is entitled '**The MD's point of view: Is the preclinical file supportive enough in terms of safety, risk-benefit and efficacy. Are the animal models and designs the best for supporting the drug development (phase I and further)?'**

The workshop starts at 9:15am and will end by 5:15pm at Genocentre, Evry, France.

If you would like to participate, you are invited to contact O.-W. Merten from Généthon 00331 69 47 25 90; email: omerten@genethon.fr

Full details of the day can be found by clicking the more link below.

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Third EVELAM summer school to take place in Cordoba 9-11 December 2010

The Third Latin American Summer School of Myology (EVELAM) will be held in Córdoba, Argentina, between 9th-11th December 2010.

EVELAM was conceived with the aim of promoting knowledge and awareness of neuromuscular disorders (NMD) throughout Latin America.

Although many Latin-American countries dedicate large efforts towards solving more common health problems, the medical care of neuromuscular patients is mostly limited to clinical evaluations and physical therapy. Histopathological and molecular genetic studies, as well as clinical research on NMD, are limited to just a few centres, some of which have contributed to advancements in the field.

Communication between these centres and the region as a whole, is poor. In 2008, enthusiastic Latin American scientists and clinicians motivated by the successful French "École d'Été de Myologie" (Institut de Myologie, Paris) organised the first EVELAM in Chile. In 2009, it was held In Uruguay, and its resounding success reinforced the interest of continuing EVELAM in future years.

A new generation of NMD specialists from different countries throughout Latin America are being educated at EVELAM, enabling them to offer expertise and technology across the region. This positive development catalyzed by EVELAM, parallels and contributes to the historical effort and success of the various Latin American Telethons which are associated in a larger regional organization called ORITEL (Organization of Telethons from Latin America).

EVELAM also helps to promote an awareness and understanding that by creating coordinated regional databases of NMD patients this will greatly increase the viability of inclusion in therapeutic trials and multicentre research protocols.

Sponsorship of EVELAM has been provided by both TREAT-NMD and the Association Francaise contre les Myopathies (AFM, France) since 2008.

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New guide to help health professionals involve patients in the research process

The UK National Institute for Health Research's Biomedical Research Centre in London has launched a new guide to help researchers involve patients and families in the research process.

The step-by-step guide has been produced to help researchers involve patients, carers, family members and patient groups and charities effectively at the stages of the research, as follows.

- The development of the grant application
- The design and management of the research
- The undertaking of the research
- The analysis of the research data
- The dissemination of research findings

The guide explains how users can help at each stage of the research process, what issues to consider, and how researchers can involve users. It also includes case study examples from around the country where user involvement is making a real difference during the research process.

Sophie Auckland, the Biomedical Research Centre's User Involvement Manager who produced the guide said: "Medical advances couldn't happen without patients and healthy volunteers being willing to participate in studies. Yet too often these groups are not invited to have a say about what research is prioritised, how a study is designed, or what questions are asked. This can result in important patient issues being overlooked when grant applications or patient recruitment materials are written, studies are designed or the results are publicised.

I hope that this simple guide will demystify the user involvement process for anybody involved in carrying out medical research and encourage researchers to involve patients, carers, family members and patient groups in their research."

Dr David King, Director, NIHR Central Commissioning Facility, said: "The NIHR wants patients and the public to be involved in all stages of research, for example in generating, prioritising and commenting on research ideas, where appropriate. Patient and Public Involvement (PPI) will increase in importance in the work of all NIHR Biomedical Research Centres and Units as it is increasingly recognised that PPI is a win:win for both patients and researchers. This new guide for research staff will greatly enhance PPI across the NIHR, especially in the area of experimental medicine. I welcome the commitment to user involvement at this and other BRCs and BRUs and am looking forward to the further involvement of patients and users in them as a result."

For a copy of Involving users in the research process: A 'how to' guide for researchers, email sophie.auckland@gstt.nhs.uk

This guide complements materials aimed at patients and the public and researchers which have already been produced by the Biomedical Research covering user involvement in research.

Have your say about medical research: How patients and the public can get involved is for patients and members of the public and provides basic information about getting involved in the research process.
There are two other leaflets for research staff:

Involving patients and the public in medical research: An introduction and Recruiting and retaining people for user involvement in research.

For copies of these, email sophie.auckland@gstt.nhs.uk or download them from:

www.biomedicalresearchcentre.org

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