

welcome

Welcome to our 54th TREAT-NMD Newsletter.

As part of the planning and preparations for our International Conference in November we are inviting you to submit questions online via the conference web site. These questions can be directed to any of the planned sessions and will form part of the panel discussions during the conference.

The newsletter also includes an informative report on exon skipping from Dr Günter Scheuerbrandt in which he explains the genetics behind DMD and the clinical trials that have taken place.

We also look at the satellite teaching course on diagnosing NMD's, which is part of the 14th International Congress of the World Muscle Society.

Finally, we would like to draw your attention to a job opportunity that has arisen here in the TREAT-NMD Coordination Office, which is funded by Action Duchenne specifically to disseminate the network's activities through to the UK Duchenne community.

As always, we hope you enjoy the newsletter and look forward to hearing your comments - write to info@treat-nmd.eu with anything you'd like to say. Feel free to forward this message to anybody you think might find it of interest, or invite them to sign up to receive the newsletter by visiting our website. Back-issues of this newsletter can be found on our website at <http://www.treat-nmd.eu/patients/news/ezine-archive/>

Best wishes,

Katie, Volker, Hanns, Steve, Emma, Rachel, Michael and Sam: the Newcastle TREAT-NMD team

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at a glance...

12-15 May 2009 The Nottingham



Conference 2009: Call for Questions

The format of the TREAT-NMD / NIH conference in November will differ from regular meetings with each session made up of a panel of experts who will discuss specific topics and questions submitted prior to the conference. Their findings will then be presented during the session itself and this will then be followed by a panel discussion.

This is a great opportunity to pose any questions that you might have to one of the panels. To ensure that the most relevant and common topics / issues / questions are included the conference, the website now includes an area where questions where can be submitted to the relevant sessions' presenters for their consideration. This will allow the panel to identify those questions of greater concern to the delegates.

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Dr Günter Scheuerbrandt's Report on Exon Skipping

Dr Günter Scheuerbrandt PhD uses his extensive knowledge of Duchenne muscular dystrophy to report on conference presentations and interviews he has conducted with the experts in language that is accessible to all. His most recent report on exon skipping in Duchenne boys continues this trend.

This report, the first in a series of specialized texts, focuses on Exon skipping the most advanced genetic technique for an effective therapy of Duchenne muscular dystrophy. Written for the non-scientific community, Günter explains the genetics behind the disease, the role of dystrophin and the different types of potential exon skipping drugs. Further through the report clinical trials are documented and Günter also talks to Dr. Kate Bushby about the ongoing trials and what their possible outcomes may tell us about this disease.

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7th WMS Satellite Teaching Course: 8-9th September 2009

CHUV Centre Hospitalier Universitaires Vaudois, Lausanne, Switzerland.

As part of the 14th International Congress of the World Muscle Society, a satellite teaching course will be held on the 8th and 9th September 2009. Aimed at medical specialists and those in training this course hopes to improve the diagnostic competence of professionals dealing with patients who have Neuromuscular Diseases.



Although in isolation each Neuromuscular Disease can be thought of as rare, and when taken as a whole the number of patients is considerable. Diagnosis of NMD's takes skill and time, testing can be extensive and vary greatly in nature, ranging from clinical examination to muscle and nerve biopsies, and indeed talking to patients about their experiences.

[Systematic Review Course 2009](#)

[21-23 May 2009 International conference in Ukraine: Recent standards in diagnosis, treatment and medical care for some rare neuromuscular diseases](#)

[01-03 Jun 2009 Update in Neuromuscular Disorders course in London](#)

[04-06 Jun 2009 TREAT-NMD workshop: clinical trial design in neuromuscular diseases](#)

[09-11 Jul 2009 "Therapeutic Targets in CMD", Emory University, Atlanta, Georgia](#)

[09-12 Sep 2009 IDMC-7 International Myotonic Dystrophy Consortium](#)

[17-19 Nov 2009 TREAT-NMD / NIH International Conference](#)

The course in Lausanne provides a context to develop these skills through discussion of patients and review of biopsies. Feedback from the first five courses (held from 2003 onwards) has been overwhelmingly positive.

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TREAT-NMD DMD UK Coordinator

An opportunity has arisen to join the TREAT-NMD team. The post will be based in the TREAT-NMD Coordination Office at the Institute of Human Genetics, Newcastle upon Tyne and is funded in the first instance for 12 months by Action Duchenne.



The role of this post will be to manage, develop and coordinate the dissemination and communication of TREAT-NMD activities to the UK Duchenne Muscular Dystrophy community, reporting directly to the TREAT-NMD Project Manager, as well as close links with the TREAT-NMD Project Coordinators and Action Duchenne.

It will be your responsibility to ensure that the objectives of the Network are communicated across the UK to the wider public in a clear, concise and informative manner and so increase the public profile of the Network and its partners. You will be responsible for the facilitation of the involvement of UK clinical trial centres in upcoming DMD clinical trials by assisting the Principal investigators, identifying funding opportunities and carrying out regulatory document filing and reporting.

The UK coordinator will also be responsible for enhancing the excellence available in UK trials sites / centres by disseminating the TREAT-NMD standards of care and helping centres implement them, organising training within the framework of TREAT-NMD, where necessary. This is a diverse and challenging position for a person with excellent organisational and communication skills and an understanding of issues relating to translational medicine and clinical trials.

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