

## welcome

Welcome to the 77th Newsletter from TREAT-NMD.

This week's newsletter includes...

- a reminder about the CTCC trial design workshop in late June
- an invitation for young researchers to the European Research Conference in Paediatric Neurology
- Acceleron's announcement of their Phase 2 trial and information on how TREAT-NMD is assisting them
- Details of an international neuromuscular conference in Brazil

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](mailto:info@treat-nmd.eu)

## at a glance...

10 May 2010 The George Karpati Symposium on Neuromuscular Disease: Innovation and Application, Montreal, Canada

12-15 Jun 2010 European Human Genetics Conference - Gothenburg, Sweden

13-15 May 2010 5th European Conference on Rare Diseases - Krakow, Poland

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

19-23 Jul 2010 12th International



### Last few places still available for TREAT-NMD trial design and GCP workshop

Scheduled for 24th to 26th June at the Clinical Trial Coordination Centre in Freiburg, Germany, this workshop will focus on the fundamentals of trial design, developing study protocols and fostering contacts to facilitate multinational cooperation. GCP training is also an optional part of the course.



The workshop is aimed at clinicians who specialise in neuromuscular disorders and other rare diseases and who are interested in clinical trial work. Specialist knowledge of the challenges of running robust, adequately powered trials in small patient cohorts is becoming increasingly important in the neuromuscular field as more therapies approach readiness for trial.

Those from Eastern Europe, including accession countries, Western Balkans and Turkey may qualify for a TREAT-NMD scholarship. Further information about the TREAT-NMD scholarships can be found [here](#).

Click "more" for further details of the course, including the full programme and details of how to register.

One of the most common reasons for failed trials is poor protocol design. As neuromuscular disorders are very rare, clinical trials have to be multi-centre or even multinational to include enough patients. As a result, the study design for these trials is usually complex. Also, academic trials have come to face a changed regulatory environment following the implementation of the EU Clinical Trials Directive 2001/20/EC.

Therefore, the aim of this workshop is to improve the efficiency of clinical trials in neuromuscular disorders and other rare diseases by

- introducing future investigators to the fundamentals of effective clinical trial design by means of lectures
- developing study protocol drafts in small group sessions in conformance with ICH-GCP
- fostering contacts between investigators to facilitate later multinational cooperation through informal discussion sessions

#### Target Audience

This workshop is aimed at physicians specialising in neuromuscular disorders and other rare diseases who are interested in clinical trial work. One focus will be on study design in small numbers. Participants are asked to bring a draft study plan to provide the basis for writing a synopsis during the workshop.

#### About the TREAT-NMD CTCC

The Clinical Trials Coordination Centre was established in May 2007 as one activity of TREAT-NMD, a pan-European 'network of excellence' aimed at improving treatments and finding cures for patients with neuromuscular disorders. It was built up in collaboration with one of Germany's leading Departments of Neuropaediatrics and Muscle Disorders and the Clinical Trials Center (ZKS) of the University Medical Center in Freiburg, Germany. Both have longstanding experience in the field of neuromuscular disorders and in the conducting of clinical trials.

Contact for further information:

TREAT-NMD CTCC

[Congress on Neuromuscular Diseases - Naples, Italy](#)

[26 Aug 2010 - 04 Sep 2010  
Advanced symposium and EMBO practical course - Kuopio, Finland](#)

[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](#)

[1-2 October 2010 European Research Conference in Paediatric Neurology - Leuven, Belgium](#)

[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](#)

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

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## Young researchers invited to present their projects at EPNS-sponsored research conference

1-2 October 2010 - Leuven, Belgium

Young researchers with an interest in muscle disease are invited to take part in this year's European Research Conference in Paediatric Neurology in Leuven, Belgium. The Research Conference is supported by the European Paediatric Neurology Society (EPNS) and welcomes researchers from across Europe.



Principal investigators active in the field are encouraged to disseminate this invitation to their research groups.

The aims of the conference are:

- to enable young researchers to meet their colleagues in the field, form links, develop collaborations and discuss their projects with experienced researchers
- to foster the building of research networks.

Most of the meeting is therefore reserved for exchange in working groups, each of which has a half-day session for case presentations and open discussion, followed by a short presentation to the conference as a whole.

The working group in neuromuscular diseases is coordinated by [Markus Schülke](#), [Thomas Sejersen](#), [Volker Straub](#) and [Nathalie Goemans](#).

The neuromuscular group is keen to encourage applications from researchers in any area of muscle disease wishing to present their research to their peers. Presentations should focus on ongoing projects, including challenges faced and plans for future development, e.g. initiating international collaborations. Applications are welcome from researchers in a range of related disciplines, including genetics and muscle imaging, and from those focusing on adult conditions as well as paediatric.

Interested parties are invited to submit a proposed presentation title and a brief (~50-word) topic summary by email to [Markus Schülke](#).

European Research Conference in Paediatric Neurology  
Neuromuscular working group session  
1-2 October 2010, Leuven, Belgium

The deadline for applications is 31 July 2010.

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## Accelaron Pharma announces start of phase 2 DMD trial in Canada

Accelaron Pharma has announced the initiation of a phase 2 clinical trial of ACE-031, a novel therapy that promotes muscle growth, in Duchenne muscular dystrophy (DMD). An earlier phase 1 trial of the compound found that it promotes muscle mass and volume in healthy volunteers. The current trial is taking place in several sites in Canada and is a randomized, double-blind, placebo-controlled, dose-ranging study to evaluate the safety, tolerability and pharmacokinetics of ACE-031 in patients with DMD who are also on corticosteroids.



To assist with the planning of future trials of ACE-031 in additional countries, Accelaron asked TREAT-NMD for data from the global DMD patient registry and care and trial sites registry. The request was for anonymized, aggregate data (patient numbers) for patients in particular countries worldwide with genetically confirmed Duchenne muscular dystrophy who fulfil certain inclusion criteria relating to their age, ambulation status and other clinical aspects of their condition. As well as patient numbers, potential clinical trial sites with experience in DMD treatment are being identified through the Care and Trial Sites Registry. This enquiry is currently ongoing and its results will help Accelaron plan further trials of ACE-031.

To view Accelaron's press release on the current phase 2 trial [click the 'more' link below](#).

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## **International Conference in Brazil - Neuromuscular Diseases: Care and Clinical Trials.**

**Sao Paulo, Brazil, 17–19 June 2010**

You still have one week to

[register](#)

at the early discounted rate for this conference.

Leading international neuromuscular specialists will join Brazilian speakers to create a stimulating program focusing on care and trials in neuromuscular disorders.

Aimed at healthcare professionals with an interest in neuromuscular disorders and patients and families, this exciting conference consists of two days of lectures from a strong panel of international and Brazilian speakers and will be followed by a dedicated family day with research updates for patients and information on physiotherapy and care.

Conference venue:  
Sirio Libanes Hospital

Rua Dona Adma Jafet, 91  
São Paulo - SP,  
Brazil.

Further conference details and conference registration can be found [here](#).

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