

welcome

Welcome to the latest TREAT-NMD newsletter.

This edition includes:

- grant opportunities to attend a TREAT-NMD training course on neuromuscular research and care in Milan in September
- a last chance for projects to be submitted by young investigators for the EPNS researchers' meeting
- final places for the EAMDA meeting
- information about the ciclosporin A trial for DMD conducted by MD-NET

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.

*Thanks to [CureCMD](#) for this week's picture.

at a glance...

[29 Aug - 1 Sept 2010 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](#)

[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](#)



Final places still available on TREAT-NMD training course in Milan

Final places still available on the free TREAT-NMD training course "The multidisciplinary approach to NMDs: towards a harmonized practice" - Milan, Italy, **16 September 2010**.

Medical professionals, students and caregivers interested in receiving specialist training in neuromuscular care are invited to participate in a free 1-day training opportunity on September 16th in Milan, Italy.

The course is held in conjunction with the EAMDA Conference and aims to highlight and discuss the latest guidelines on clinical management and provide information on the more innovative aspects of neuromuscular research.

Its organizers are Professors Thomas Sejersen (Karolinska University Hospital, Stockholm, Sweden) and Massimo Corbo (NEMO NEuroMuscular Omnicentre, Milan, Italy) with the support of the European Neuromuscular Centre (ENMC).

Topics addressed:

- Management of respiratory and cardiological problems in NMDs
- Multidisciplinary approaches
- Rehabilitation
- Customizing orthoses for people with NMDs
- Outcome measures in general practice and for clinical trials
- Quality of life questionnaires
- Training of caregivers on medical support in daily life

The final scientific programme of the course is available at the TREAT-NMD website at www.treat-nmd.eu/about/events/events/921/

In particular, the course will address the implementation of standards of care, highlighting the advantages of operating in multidisciplinary settings. Major topics will be respiratory and pulmonary care, rehabilitation and quality of life. The training lectures will be given by leading professionals and the sessions also offer the opportunity to share the experience and learn about the different approaches in the major multidisciplinary centres in Europe.

The number of participants is limited to 40-50. Candidates should fill in the registration form for the course available at www.treat-nmd.eu/about/events/events/921/ and send it together with a brief CV in English to Mrs. Katelijne Senden at senden@enmc.org by August 30th, 2010. Requests received without CV will not be taken into consideration. Successful applicants will be notified by September 6, 2010.

Location: The TREAT-NMD training course will be hosted at the ATA Hotel Expo Fiera, Via Keplero, Pero (Milan), where a number of bedrooms have also been reserved.

Costs and grants: The course is free of charge. The coffee breaks and the lunch buffet will also be free. Participants are responsible for covering their own travel and accommodation expenses. For further information and hotel reservation, please contact the Italian Muscular Dystrophy Association UILDM, local organiser of the EAMDA Conference (EAMDA@uilmilano.it).

Two TREAT-NMD fellowships are available with a maximum of €400 per grant (Italian participants are not eligible). Participants interested in applying are requested to fill in the Grant Application Form available at the [TREAT-NMD website](#) and submit it via email together with a CV written in English to Katelijne Senden at senden@enmc.org by August 30th, 2010.

We look forward to welcoming you in Milan.

Best regards,

Professor Massimo Corbo

[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](#)

[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](#).

Professor Thomas Sejersen

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Major MD-NET trial shows no benefit of ciclosporin A for DMD but proves the importance and feasibility of randomized controlled trials in rare diseases

A 36-month study into the effects of ciclosporin A both alone and in combination with intermittent prednisone for the treatment of ambulant patients with Duchenne muscular dystrophy (DMD) is published this month in *Lancet Neurology*. Although the trial found no evidence of a beneficial effect of the drug, it is nevertheless an important milestone in DMD trials, proving that with the close collaboration of all stakeholder groups it is possible to perform meaningful randomized controlled clinical trials with sufficient statistical power even in rare diseases like DMD.



Ciclosporin A is one of the essential immunosuppressive drugs used in many immune and non-immune childhood diseases, and two previous open-label trials had reported enhanced muscle strength in boys with Duchenne muscular dystrophy after daily treatment with the drug. More recently, a positive treatment effect of low dose ciclosporin A was also confirmed in mdx mice. Coordinating investigator of the current trial was Rudolf Korinthenberg at the Division of Neuropaediatrics and Muscle Disorders at Freiburg University. The trial was supported financially by the German government and the patient organization aktion benni & co. and in kind by Novartis Pharma (provision of study medication) and the Deutsche Gesellschaft für Muskelkranke (provision of myometers). A total of 146 patients were recruited over 36 months into a double-blind, randomized, placebo-controlled multicentre study at 11 trial sites across Germany and in Austria and Switzerland that are part of the German muscular dystrophy network (MD-NET). The trial concluded that ciclosporin A alone or in combination with intermittent prednisone does not improve muscle strength or functional abilities in ambulant boys with Duchenne muscular dystrophy, but is safe and well tolerated.

"Trials that prove a drug has no benefit are of course extremely disappointing for everyone and often don't get much publicity," explained study coordinator Janbernd Kirschner. "But this is still a very important result that has many implications for future trials in DMD and other rare diseases. We did this trial because two previous trials had reported that ciclosporin had a positive effect on muscle strength, and we hoped to prove that effect. Instead, we proved the drug had no effect. Those previous trials were open-label studies, meaning the doctors and patients knew the drug that was being used, and the patients receiving the drug were not compared against a group receiving a placebo. Our study enrolled a much larger number of boys and neither the doctors nor the patients knew which patients were getting the drug. The fact that it showed no benefit just goes to show how important it is that these randomized, placebo-controlled trials are carried out whenever possible to get a definitive answer about any drug proposed as a treatment for DMD. With a lesser evidence base, we could have been giving our DMD boys a drug that was having no benefit at all and that has some risks associated with it. Equally importantly, we have clearly shown that it is possible to perform meaningful randomized controlled clinical trials with sufficient statistical power even in rare diseases like DMD - which is very promising in terms of the future therapies that are now coming to trial."

This trial therefore sends an important message about the feasibility of trials in rare diseases, and with the increasing interest from pharmaceutical companies in the rare disease field this is a very timely message. But as commercial interest and financial resources can still remain scarce, it is important to note that planning and carrying out such a trial requires close cooperation between researchers, clinicians, patient groups, public funding sources and industry partners. The German muscular dystrophy network MD-NET and the support of patient organizations was instrumental in this trial. Future multinational studies, even those with industry sponsors, will also need to take advantage of networks of trial sites, the support of patient groups, and the assistance of patient registries for recruitment.

The full published paper is available online at [http://www.thelancet.com/journals/lanneur/article/PIIS1474-4422\(10\)70196-4/](http://www.thelancet.com/journals/lanneur/article/PIIS1474-4422(10)70196-4/)

Reference:

Kirschner J et al. Treatment of Duchenne muscular dystrophy with ciclosporin A: a randomised, double-blind, placebo-controlled multicentre trial. *Lancet Neurology* – article in press. Published online August 27, 2010
DOI:10.1016/S1474-4422(10)70196-4

For further information contact:
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[Dr Janbernd Kirschner](#)

Websites:
www.md-net.org
www.benniundco.de
www.dgm.org

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Registration still open for 40th EAMDA meeting

The 40th annual meeting of the European Alliance of



Neuromuscular Disorders Associations, EAMDA, is due to take place in Milan in September. With a stimulating programme from international specialists covering topics ranging from clinical trials to fitness and from quality of life to empowerment, the main aim of the meeting is to promote the sharing of information and knowledge about the advances within the neuromuscular field.



The meeting will be held in **Milan** from **16th - 19th September 2010** and registration is still open.

The full programme is available [here](#).

Please contact EAMDA@uidmmilano.it or visit the meeting's [website](#) for more information.

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Last chance for applications to present neuromuscular projects at EPNS 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

Final applications are still being accepted!

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