

welcome

Welcome to our 55th newsletter. This week's newsletter includes an important article regarding the launch of the TREAT-NMD Therapeutics Advisory Committee or T-TAC for short. The many and varied members' profiles are now available to view on the TREAT-NMD website and we are delighted the committee is being chaired by Cristina Csimma who has over twenty years of drug development experience to draw upon. We wish Cristina and her T-TAC team all the very best for the future and we look forward to reporting on their work here in the newsletter.

We have also included a report on the German Muscular Dystrophy Network MD-NET annual meeting which was held in Wildbad Kreuth earlier this month. Focus was placed upon TREAT-NMD's activities, current and future trials and also the future direction of MD-NET itself.

Also in this week's newsletter is an article about the recent exon skipping meeting in Cagliari in early May. Muscle perfusion and splicing was the focus of this meeting and the participants, who signed a confidentiality agreement, presented unpublished data to assist with the future coordination of experiments.

Maryze Schoneveld van der Linde has written and distributed to patient organisations, the first newsletter specifically designed to inform and help those living in Central and Eastern Europe who are affected by NMDs.

The 6th Annual SMA Conference in Edinburgh, Scotland will be held on the 5th and 6th of October, details and links to the conference site have been included on the TREAT-NMD website.

The Cochrane Neuromuscular disease group have a job opportunity in the National Hospital for Neurology and Neurosurgery, the position of Managing Editor is for fourteen months to cover maternity leave.

TREAT-NMD are offering five scholarships to the WMS satellite



TREAT-NMD launches their Therapeutics Advisory Committee: T-TAC

The TREAT-NMD Therapeutics Advisory Committee (T-TAC) consultation process is now completed and we are delighted with the breadth and calibre of international experts we have available as part of the T-TAC. We would therefore like to introduce them to the neuromuscular community via the launch of the TREAT-NMD Therapeutics Advisory Committee webpage on the TREAT-NMD website.



Cristina Csimma, the T-TAC chair, will lead this exciting initiative with the support of the nominated committee members and TREAT-NMD secretariat. The overall aim of the T-TAC is to provide guidance and advice to the neuromuscular community on the prioritisation of non-proprietary drugs as likely candidates for therapies for neuromuscular diseases.

The remit of the committee will be to evaluate the therapeutic development potential of drugs with preclinical data suggesting potential activity in a neuromuscular disease, thereby assisting TREAT-NMD in prioritising clinical trials to be run via the network, provide the background for preparing funding applications and investigational drug applications, and provide an unbiased appraisal to be published for the wider neuromuscular community. We hope that this process would be endorsed by the major funders.

Therapeutic development potential implies not only the interpretation of data from preclinical studies, but also key considerations including formulation, toxicology, the likely regulatory path and commercial potential. Issues pertaining to the practicality of performing rational and economical clinical trials are also critical particularly in today's economic and funding environment. To support this comprehensive assessment we have a range of experts available to assist.

We envisage that the whole T-TAC appraisal process from call for drugs for consideration to specific detailed report available to potential PI, sponsor and funders will take approximately 8 months. T-TAC will not necessarily only consider 1 drug at a time and therefore may have panels of experts reviewing two drugs concurrently, with the same chair for continuity.

T-TAC hopes to have the first drug under review by the end of 2009, so as a next step it will solicit opinion on the drugs to evaluate from amongst the TREAT-NMD network and collaborators.

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TREAT-NMD Conference 2009

We have finalised both sessions and speakers for the conference in Brussels and these details are now available on the conference website. Particular attention has been taken to create what we hope to be a diverse and lively conference.

Keynote speakers include Kenneth Fischbeck (NIH/NINDS) and Gert-Jan van Ommen (Leiden University). Each session has international representation from specialist areas.



We are very keen for this to be an inclusive conference with delegates and speakers interacting throughout. To begin this process we have a page on the conference website which allows delegates to post questions to speakers prior to the conference for their consideration.

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teaching course in Switzerland 8th - 9th September 2009. Details of the course and how to apply are now on our website.

We hope you have a good weekend - best wishes from
Katie, Volker, Hanns, Steve, Emma, Rachel, Sam and Michael: the Newcastle TREAT-NMD team.

at a glance...

[12-15 May 2009 The Nottingham 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](#)

German Muscular Dystrophy Network MD-NET - Annual Meeting

The German Muscular Dystrophy Network MD-NET has been funded by the German Federal Ministry of Education and Research as one of the national networks for rare diseases since 2003. In MD-NET, scientists and clinicians are collaborating in scientific projects on the pathomechanism of muscular dystrophies, as well as in optimizing diagnosis and treatment of patients. Adjacent to the first funding period of three years and an extension period, an additional funding period of three years (1.674.516 € for the period of 2009-2011) has recently been granted. The network coordination office is located at the Friedrich-Baur-Institute, Department of Neurology of the University of Munich. Coordinators are Dr. Maggie Walter from Munich, Prof. Matthias Vorgerd from Bochum and Dr. Janbernd Kirschner from Freiburg. MD-NET is the German partner of TREAT-NMD, actively participating in several workpackages and serving as activity leader of Activities 4 (biobanks and patient registries) and 5 (clinical trials coordination centre).



The annual MD-NET meeting took place on May 7-8, 2009, in Wildbad Kreuth, Germany. On the first meeting day, the current status of MD-NET service structures and research projects of the current funding period were presented by project leaders. Furthermore, Volker Straub, Hanns Lochmüller and Rudolf Korinthenberg gave an update on TREAT-NMD activities and current and future clinical trials for muscular dystrophies with participation of MD-NET trial sites were discussed. The future and sustainability of MD-NET was the central topic on the second day of the meeting. MD-NET members agreed to not limit network activities on muscular dystrophies in the future, but include more hereditary neuromuscular diseases. Excellent talks by Prof. Brunhilde Wirth on modifier genes for SMA, Dr. Ute Hehr on polar body diagnosis and Dr. Anne van Riesen on Arthrogryposis Multiplex Congenita concluded the meeting.

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3rd TREAT-NMD Workpackage 8.2 Exon Skipping Meeting is a Success!

The third workpackage 8.2 meeting on exon skipping was organized by Francesco Muntoni, Emma Heslop and Annemieke Aartsma-Rus and took place in Cagliari (Italy) on May 8. The focus of this meeting was on splicing and muscle perfusion. As usual unpublished data was presented by the participants to compare notes and coordinate future experiments under a confidentiality agreement.



In addition to participants from TREAT-NMD and/or the MDEX consortium (Annemieke Aartsma-Rus (LUMC), Alessandra Ferlini (UniFe), Emma Heslop (UNEW), Hervé Laouenan (AFM), Francesco Muntoni (ICH), Nic Wells (ICL) and Matthew Wood (Oxford)), four invited experts were present to provide additional input on splicing and muscle perfusion (Irene Bozzoni (University of Rome) and Daniel Schümperli (University of Basel, member of the EURASNET network of excellence on alternative splicing) for splicing and Yves Fromes (Institute de Myologie) and Caroline le Guunier (University of Nantes) for perfusion).

Matthew Wood kindly offered to host the next meeting in Oxford (UK) in December 2009.

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Introductory Newsletter for Central and Eastern Europe

TREAT-NMD aims to be an inclusive rather than an exclusive network, and you do not have to be based in Europe or be a partner to be involved. International collaboration with experts from all over the world is already taking place, and new links are constantly being developed.



Maryze Schoneveld van der Linde has written and circulated an introductory newsletter for those living in Central and Eastern Europe (CEE) as part of her new role as European Neuromuscular Centre (ENMC) project manager for TREAT-NMD. The newsletter along with a DVD focussing on ethical issues and a short report from the European Forum for Good Clinical Practice has been sent out to many relevant patient organisations in CEE. The newsletter covers topics such as upcoming conferences, standards of care and registries. Also included are details of contact points, membership details and other publications that might be of interest making this first newsletter a great way to find out what is happening in many aspects of the network in one read.

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6th Annual SMA Research Conference

Jointly funded by the Jennifer Trust for SMA and the SMA Trust the 6th Annual Research Conference will take place in Edinburgh in early October.



Held in the Symposium Hall of the Royal College of Surgeons speakers include...



Dr Margarida Gama Carvalho - University of Lisbon
Professor Eugenio Mercuri - Universita Cattolica del Sacro Cuore

A call for abstracts has been made and the deadline for these is Friday 28th August 2009.

Registration for the conference itself closes on Friday 31st July 2009.

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Job Opportunity - Managing Editor, Cochrane Neuromuscular Disease Group.

National Hospital for Neurology and Neurosurgery. Maternity Cover for up to 14 months.



The Cochrane Neuromuscular Disease Group is part of the Cochrane Collaboration - an international organisation that publishes and maintains systematic reviews of the effects of healthcare interventions on The Cochrane Library.

The group looking to appoint a Managing Editor to cover a period of maternity leave. The postholder will be based at the National Hospital for Neurology and Neurosurgery, MRC Centre for Neuromuscular Disease which houses the editorial base of the Group.

The postholder is required to ensure the efficient and effective operation of the Group's editorial base, including budget management and staff supervision, providing support to systematic review authors on methodology and use of systematic review software and overseeing the editorial processes of the group. The ideal candidate will have managerial, administrative, scientific or publishing experience or equivalent, along with excellent organisational and communication skills. Knowledge of scientific and medical terminology and evidence based health care is desirable. Familiarity with clinical trials, systematic reviews or The Cochrane Collaboration would be welcomed. For a job description, further details and application process visit this [link](#).

For an informal discussion about the post, please contact Kate Jewitt, Managing Editor, Cochrane Neuromuscular Disease Group, 0207 692 2359 or email: cochranenmd@ion.ucl.ac.uk

Closing date for applications: 3 June 2009

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TREAT-NMD fellowships for WMS teaching course

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.



There are five TREAT-NMD scholarships, covering fee and hotel costs up to a maximum of €500,- in total, and is especially meant for young physicians specialising in neuromuscular diseases who are already involved in clinical trial work, coming from the former Eastern part of Europe, including accession countries, Western Balkan and Turkey.

Candidates should send their application for the course to Dr. Pierre-Yves Jeannet at Pierre-yves.Jeannet@chuv.ch and to apply for the TREAT-NMD scholarship to Katelijne Senden at senden@enmc.org. The application for the scholarship needs to be received by Katelijne Senden before June 30, 2009.

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