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TREAT-NMD newsletter no. 50
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# welcome

Welcome to the latest TREAT-NMD newsletter. This week's edition features information about the publication of the outcome of TREAT-NMD animal models work and highlights the TREAT-NMD Therapeutics Advisory Committee or T-TAC, a new steering committee to evaluate the case for moving non-proprietary drugs towards clinical testing. Other articles include a report from the first Asian DMD conference and the recent TREAT-NMD High Throughput Screening workshop.

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, Sam and Michael: the Newcastle TREAT-NMD team

# at a glance...




## TREAT-NMD Therapeutics Advisory Committee (T-TAC) terms of reference open for discussion

One of the key aims of the network in the next period is to develop a plan for prioritisation of non-proprietary drug targets as likely candidates for therapies for neuromuscular diseases. The first stage in achieving this aim is to develop a steering committee to impartially evaluate the case for moving these drugs forward into clinical trials. Therefore, TREAT-NMD has been busy developing the Terms of Reference for this committee (T-TAC). Our partners, members and close collaborators have been involved in developing these terms of reference and we are now at a stage where we are able to invite interest from the wider neuromuscular field. We are keen to establish the membership of the steering committee (including chair, experts in preclinical, toxicology, regulatory and marketing feasibility, clinical trial co-ordination centre representation, clinical representatives, patient organisation representation and ethical input) and aim to establish a funding plan within the next few months. The committee, once established, will meet for the first time in mid 2009 to define the tasks required to enable the T-TAC to start accepting enquiries.

If you would be interested in viewing a copy of the T-TAC terms of reference and or contribute to the development of the T-TAC we would invite you to apply to receive a copy by e-mailing Samantha.Cook@ncl.ac.uk at the TREAT-NMD coordination office.

If you require any additional information please contact Emma.Heslop@ncl.ac.uk

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## DMD animal models consensus paper published in Neuromuscular Disorders

One of TREAT-NMD's key aims in the research area is to harmonize the tools used in preclinical research and improve the comparability of results obtained in different centres. Part of this work involves evaluating the multitude of animal models, readouts and measurement protocols used in preclinical research with the aim of reaching a consensus within the international community as to which models, readouts and protocols are most appropriate for the efficacy testing of potential new treatments. This will open the possibility of designing and conducting experiments that can be compared between different laboratories, thus allowing proof-of-concept studies.

As the outcome of workshops involving experts from all over the world, a paper has just been published in the Neuromuscular Disorders journal that summarises the recommendations on animal models for Duchenne muscular dystrophy. The authors believe that these measures will increase the value of preclinical data collected for regulatory filings with health authorities, and will accelerate the preclinical stage of drug development.

For further information on TREAT-NMD's animal models work and to download the paper, click here.

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09-12 Sep 2009 IDMC-7 International Myotonic Dystrophy Consortium

17-19 Nov 2009 TREAT-NMD / NIH International Conference

## TREAT-NMD High Throughput Screening (HTS) Workshop


TREAT-NMD recently organised a workshop in Naarden, The Netherlands, to discuss the optimal properties and usage of high through-put screening (HTS) assays in neuromuscular diseases, and how they can be applied in clinical research. The workshop was co-chaired by Kay Davies and Jon Tinsley, who are partners in TREAT-NMD responsible for developing new HTS models, and Gillian Butler-Browne of the Institut de Myologie and John Porter of the NIH/NINDS. Workshop participants from across Europe and North America, including academics, clinicians, and industry, discussed the development of strategies for successful translation of HTS assays from the lab to the clinic. The workshop covered four main areas:

- How to set-up the right assay – different disease mechanism in tissue culture
- Screening assays in model systems
- Issues with chemical modification and optimisation
- Review of successful HTS strategies

Feedback from the workshop has been very positive and the co-chairs are now working on drafting a full meeting report for publication and an abridged version for the TREAT-NMD web site.

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## First Asian DMD Conference a Success!

After a keynote lecture by Professor Jean-Claude Kaplan, (inter)national speakers presented data on Duchenne muscular dystrophy in general (genetics, clinical aspects and learning problems), and the hurdles and challenges for genetic testing and developing therapies for the disease. Lacking therapies, good care is very important, and standards of care in Europe and Northern America were presented on the second day, followed by an overview of the current state of care and genetic testing in India, with a focus on how this can be improved in the future.

There were break-out sessions for physiotherapists, young scientists, genetic counseling as well as an expert meeting on genetic testing and DMD patient registries. In India, 8-10 sites are involved in genetic testing, and 5 of them were represented (Mumbai, Hyderabad, Lucknow, New-Delhi and Chennai). For DMD, PCR analysis is performed as a standard and only a few use MLPA, when necessary complete genotyping is performed abroad. The challenge for DMD patient registries in India is to coordinate diagnose sites and clinicians to establish fruitful network ready to join TREAT-NMD registry in a near future.

Over three hundred parents and patients, 160 physiotherapists and 100 scientists and clinicians participated actively in the meeting, and presentations could -thanks to Filippo Buccella, DPP Italy- be followed live through the UPPMD.org website (and will be available shortly on www.duchenne-community.org).

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## UK Neuromuscular Translational Research Conference


Following the success of the first translational research conference for neuromuscular disease hosted by the MRC Centre and the MDC in London in 2008, a second UK conference is planned for 2009. This two-day conference aims to showcase the best UK neuromuscular science, and highlight its translation into patient benefit. The conference will include invited keynote speakers providing state of the art overviews, as well as platform and poster presentations from submitted abstracts. Professor Dame Kay Davies will deliver the John Walton Lecture.

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## Job opportunity in Munich: MD-NET Manager

**Closing Date: 1st April 2009**


The role of this post will be to manage the German Muscular Dystrophy Network (MD-NET). It will be the responsibility of the project manager to determine how to meet the objectives of the network and to assure the future sustainability of the project. The post holder will be responsible for the co-ordination of the MD-NET, and will also be expected to play an interactive role in communication and management with partners within Germany and in Europe. A

network secretary will support the work of the network manager part-time.

The MD-NET brings together clinicians and researchers from all over Germany who are interested in research on Muscular dystrophies. The network is funded by the German Ministry of Education and Research.

MD-NET management includes project management, budget management, contribution to strategic planning, public relations, national implementation, cooperation with other networks for rare diseases and patient organizations on a national and international level. Essential skills include a university degree in economic or natural sciences, excellent knowledge in English, outstanding soft skills, ability to innovate and solve problems while contributing to the strategic direction of the network, IT literate across a wide range of applications, evidence of project management experience and delivery of projects to deadlines ideally in a life sciences arena, experience of budget setting and monitoring of large and diverse budgets. Experience of a research environment and issues related to funding agencies and research contracts is highly desirable.

Salary will be based upon the German salary scale TV-L (Entgeltgruppe 13). The University welcomes applications from all sections of the community including candidates with a disability.

Please send your application to the following contact address:

Klinikum der Universität München
Friedrich-Baur-Institut
Ziemssenstr. 1
80336 München
Email maggie.walter@lrz.uni-muenchen.de

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