

welcome

Welcome to the latest TREAT-NMD newsletter.

This edition includes:

- Exciting news from Australia, where the national DMD registry has just gone live and the newly established Australasian Neuromuscular Network has sent out its first newsletter.
- News of the drugs to be reviewed at the next TACT meeting in January
- An update from the recent meeting of the NMD-chip project steering committee
- News of a specialist symposium on SMA in Barcelona
- An update from the TREAT-NMD regulatory affairs database.

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.

at a glance...

[19-20 Nov 2010 Steps Forward in Pompe Disease - London, UK](#)

[9-11 Dec 2010 The 3rd Latin American Summer School of Myology EVELAM - Córdoba, Argentina](#)

[29 Nov 2010 The SMN genes and spinal muscular atrophy: 15 years of progress - Barcelona, Spain](#)

[15 Dec 2010 Systemic delivery of AAV for neuro-muscular gene therapy - Evry, France](#)

[21-22 Feb 2011 7th Annual Update Symposium on Clinical Neurology and Neurophysiology - Tel Aviv, Israel](#)

[24-26 Mar 2011 International Congress of the European Society of Magnetic Resonance in Neuropediatrics - Amsterdam, Netherlands](#)



Australian DMD registry goes live!

This week saw the official launch of the national DMD registry across Australia thanks to an effective lobbying campaign led by the Duchenne Foundation Australia in conjunction with the Muscular Dystrophy Association and the Muscular Dystrophy Foundation. The new DMD registry is endorsed by paediatric neurologists, clinical geneticists, and muscular dystrophy support groups along with patient advocates and conforms to national guidelines.

The registry has been modelled on pre-established registries already operating as part of the TREAT-NMD network which have already proved to be invaluable in coordinating approaches to diagnosis, therapy, research and prevention of disease.

The Australian national DMD registry will also be included in the global network of DMD registries that upload data to the TREAT-NMD global registry for DMD, thus improving opportunities for international collaboration by facilitating and accelerating the recruitment process for Australian DMD patients into new clinical trials.

Already efforts have now turned to the development and launch of Australian registries for other neuromuscular diseases such as spinal muscular atrophy (SMA) and myotonic dystrophy (DM1).

For further details of the Australian DMD registry, including information on how to register, please visit the registry website by clicking the more link below.

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Australasian Neuromuscular Network: working together across Australia and New Zealand

The first edition of the Australasian Neuromuscular Network (ANN) newsletter is now available. The ANN was formed in 2010 with a commitment to establishing a cohesive, integrated neuromuscular network that enables people to work together across Australia and New Zealand for the well-being of patients. Structured to address issues relating to clinical care and research effort, the network will provide a forum to advance and disseminate information, and be a single voice to advocate for patients and guide best practice in diagnosis, care and treatment.



Since its inception, the ANN has had close links with TREAT-NMD and has joined the network as a TREAT-NMD member. The network will provide access to Standards of Care and Data Collection Proformas for patient management and diagnosis; a unified approach to ethical approvals and consent; improved communication to discuss patients; notification of opportunities to participate in registries, research studies and clinical trials; opportunity for Special Interest Groups to develop in specific areas – e.g. diagnosis of neuropathies, adult neuromuscular disorders, allied health network; opportunities to provide integrated training programs for clinicians and researchers and assistance to centres to set up multidisciplinary services.

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TACT applications for review confirmed



[29-30 Mar 2011 The Fourth UK Neuromuscular Translational Research Conference - London, UK](#)

[7-8 April 2011 Chinese conference on translational research in DMD - Guangzhou, China](#)

[9-16 April 2011 63rd American Academy of Neurology Annual Meeting - Honolulu, Hawaii](#)

[9-13 May 2011 Myology Congress - Lille, France](#)

[18-22 Oct 2011 WMS Conference 2011 - Algarve, Portugal](#)

[8-11 Nov 2011 TREAT-NMD International Conference - Geneva, Switzerland](#)

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

TACT is pleased to announce that based on the preliminary review of the applications and inquiries received as of October 15th, the closing date for the third TACT review meeting, the following two applications will be reviewed at the next TACT meeting scheduled for 15th-16th January 2011 in Charlotte, NC, US:



1 - Tetracycline Derivatives as SMN2 Splicing Modifiers for the Treatment of SMA- submitted by Dr. Paul Higgins, Paratek Pharmaceuticals, Boston, MA, US.

2 - Therapy for Muscular Dystrophy by Inhibition of Mechanosensitive Ion Channels- submitted by Dr. Frederick Sachs, SUNY Physiology and Biophysics, Buffalo, NY, US.

As with previous reviews, TACT will generate a detailed report to the applicant within 6 weeks following the meeting. A general 'non-confidential' report summary, developed in collaboration with the applicant, will be available via the TREAT-NMD website within 8 weeks following the meeting, to inform the community.

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NMD-chip project update

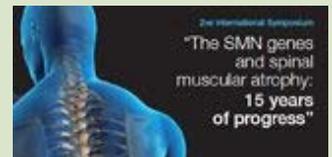
The fourth NMD-chip Steering Committee meeting took place in Wuerzburg, Germany, from 4-6 October 2010. The meeting focus was primarily on validation of the chips and included a validation workshop. The NMD-chip project is developing new sensitive high-throughput DNA microarrays ("gene chips") to diagnose patients affected by inherited neuromuscular disorders. The project addresses two main issues facing neuromuscular genetics: diagnosis of mutations in genes already known to be involved in NMDs and discovery of new genes not previously known to be involved. The scientific strategy is to develop both comparative genomic hybridization (CGH) arrays and sequence capture chips for both known genes and candidate genes. The first generation of CGH arrays for known genes has been validated and the validation of the sequence capture chips is ongoing. Technological advances that have occurred in the field over the past two years have resulted in a review of the sequence capture chip design, which in future iterations may make use of in-solution capture techniques. Once validated, the chips will be available to labs and companies for use in their standard procedures, and plans for exploitation of the technology are ongoing. A detailed project update is now available on the NMD-chip website via the "more" link below.



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Symposium in Barcelona - The SMN genes and spinal muscular atrophy: 15 years of progress

To mark the 15th anniversary of the discovery of the SMA gene, a one-day specialist symposium is taking place in Barcelona at the Hospital de la Santa Creu i Sant Pau on 29 November 2010. Presentations will include overviews of the progress made so far in knowledge of the disease pathogenesis, animal models, and the SMN protein, and perspectives for clinical trials and therapy development. The day will end with a round-table discussion between all invited speakers. For further details, click "more" below.



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TREAT-NMD regulatory affairs database - details of regulations governing trials across Europe

The TREAT-NMD Regulatory Affairs Database is a valuable source of advice for those involved in the planning of mono- or multi-centre clinical trials within different European countries. More than 3000 visitors from all over the world have used this resource since its launch in 2008.

The database is open for public use and can be accessed online - a login/username is not required.

The database has recently been updated with two additional countries and now contains contact details for the relevant national authorities as well as national legislation and documents from 13 European countries.

Additionally, European regulations and other important international documents and guidelines are provided (e.g. from ICH and EMEA).

The database collaborates closely with ECRIN (the European Clinical Research Infrastructures Network), a sustainable, not-for-profit infrastructure supporting multinational clinical research projects in Europe (see www.ecrin.org).

It was the help of ECRIN members that enabled regulatory information from Austria and Denmark to

be incorporated. In 2011 this close collaboration regarding the update of the information will continue.

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