

welcome

Welcome to the 64th Newsletter from TREAT-NMD.

This week we feature the kick-off meeting of TREAT-NMD's Advisory Committee for Therapeutics (TACT), an exciting new venture that will provide unbiased advice to the neuromuscular community on the readiness of drugs and therapeutic targets as likely candidates for therapies for neuromuscular diseases. The importance of the global registry initiative is also highlighted: more than 30 countries worldwide are now involved, and the registries are being used not only by industry for trial planning but also to provide important feedback to patients and give them a link to the research community.

TREAT-NMD offers many services to industry, and the biotech company Santhera is partnering with TREAT-NMD over physiotherapy training for its pivotal Phase III study with Catena®/Sovrima® for DMD. Academic training is also available, and in cooperation with the University of Belgrade TREAT-NMD organised a training course in Belgrade in September with 34 trainees attending from 5 CEE countries.

Full programme details for our International Conference are now online. There is still time to book a place at the conference and also to submit a question for our panels of experts to consider.

As always, if you have a news article that you would like us to include in our fortnightly newsletter, please contact the coordination office - which this week welcomes a new member. Karen Rafferty has joined us to help implement TREAT-NMD tools and services in the UK in a joint project with the UK patient organisation Action Duchenne.

Best wishes from Katie, Volker, Hanns, Steve, Emma, Rachel, Samantha, Karen and Michael, the Newcastle TREAT-NMD team.



TACT kick-off meeting develops strategy for unbiased drug candidate appraisal

The TREAT-NMD secretariat welcomed the members of the core committee of TACT (the TREAT-NMD advisory committee for therapeutics) to Newcastle for the weekend of 3-4 October for the committee's kick off meeting. The formation of TACT has been one of the key achievements of the past year and the secretariat is very grateful to the many people who contributed to the development of its terms of reference and who nominated people to serve on the committee. A very exciting group has been assembled with wide ranging expertise in many areas, and from this group of 43 experts a core group of 10 will assist the chair, Cristina Csimmá, in strategic decisions.



The committee was formed in response to the need to have an impartial means to evaluate the potential of therapeutic targets suggested to play a role in NMD, especially the candidates coming out of studies in animal models. Investigators, potential sponsors and potential funders of such studies were asked to identify drugs that might fall into this category, and the resulting list of 47 proposed therapeutic compounds and targets illustrated the scale of the issue facing the community: the need for a means to appraise the real likelihood of drugs with potentially positive results in animal models coming to trial and ultimately to registration. This pathway requires truly multidisciplinary input, reflected in the make-up of the committee.

The core committee all agreed that key to the success of this venture will be a commitment to high quality advice, so that the people presenting a possible candidate for appraisal by TACT will be assured of getting impartial and excellent information allowing them to plan a route forward to registration or alternatively to allow them to see the risks in such a strategy. Academics often don't have easy access to information on pharmacology and toxicology requirements, for example, and the regulatory issues are also frequently underestimated. Following the meeting, a clear guide for applicants is being developed which will take them through the application process and educate them on the stages of drug development. The application form will be available on the TREAT-NMD website in November and TACT will be encouraging interested groups to submit applications thereafter for appraisal at the first drug review meeting scheduled for 6-8 February 2010.

TACT is an ambitious group with an aim to be useful and helpful to many different groups. For the researcher with interesting preclinical results on a compound - for example a drug registered for other indications - specific advice on the pathway to registration and access to the tools needed for protocol development and the regulatory environment might be most valuable. For a clinician, access to toxicology and pharmacology know-how might be key. TREAT-NMD hopes that industry will also use TACT as a way to understand the target community more as well as to act as a scientific advisory panel on issues relating to preclinical studies. TACT will also work with funders to show how a TACT review can add value to a proposal, while being careful to avoid duplication of effort with other ostensibly similar programmes.

Next year's TACT meetings will begin the process of evaluation of drug targets and TACT will feed back regularly on progress to the community. Please do contact [Emma Heslop](#) for further information.

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TREAT-NMD / NIH International Conference: full programme now online

at a glance...

[23-24 Oct 2009 Action Duchenne International Conference Holiday Inn Bloomsbury London](#)

[14-18 Nov 2009 Mechanisms Involved in Muscle Atrophy and Hypertrophy, Carmona, Spain.](#)

[17-19 Nov 2009 TREAT-NMD / NIH International Conference](#)

[27 Nov 2009 King's College London Neuromuscular Symposium](#)

[26-27 Feb 2010 Towards a Brighter Future - Sydney, Australia.](#)

[10-17 April 2010 American Academy of Neurology Annual Meeting - Toronto, Canada.](#)

The programme for the TREAT-NMD /NIH international conference has been finalised and we encourage all our readers to take a look to find out full details of this key event. The full programme can be downloaded [here](#) and can also be readily found on the front page of the [conference website](#).

The conference is an interactive mixture of plenary lectures plus panel discussions, and each session will also be guided by questions that have been submitted to the panels via the website. You still have time to submit your questions [here](#).

Registration for the conference is still open and you can begin the registration process [here](#).

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Third curator training meeting shows TREAT-NMD patient registries are going from strength to strength

More than 90 participants from 23 countries attended the third annual patient registry curator training meeting, which took place from September 7–9 in Montpellier, France. Over 30 countries worldwide are now part of the TREAT-NMD global registries initiative for Duchenne muscular dystrophy (DMD) and spinal muscular atrophy (SMA) and have national registries either already established and enrolling patients or in the setup phase. Participants at the meeting received training in correct annotation of the genetic diagnosis and discussed the registry dataset. The Global Database Oversight Committee, the body responsible for the ethical governance of the registry, also met to discuss important questions relating to the way patients are recruited for trials through the registries.



DMD and SMA registries: coverage going global

The TREAT-NMD patient registries were set up primarily with future trials and therapies in mind. The global DMD and SMA registries are recognised as the leading resource for trial planning and recruitment in these diseases at an international level and are already being used by pharmaceutical companies for this purpose. They function on the basis of multiple national systems feeding into a single international database. The national registries all collect the same streamlined, internationally harmonised core dataset that includes key information facilitating trial planning and enabling patient recruitment. There is a strong ethos of patient involvement in the project, with many of the registries run by patient organizations and all registries committed to providing regular feedback to patients about new information relevant to their condition, as well as potential trials or new treatment. "We are thrilled to see how many countries are now part of this global initiative," commented Hanns Lochmüller, leader of the registries initiative for TREAT-NMD. "It's a real testament to the hard work of all our colleagues worldwide, both patients and specialists, and we are immensely grateful to all of them for their support. Everyone present at this training meeting had a real sense of the power of this initiative and could feel part of a global network." "The primary purpose of the registries is to help patients get recruited into trials for new treatments, and patients themselves want this as much as the companies who are now coming to us for data," added global registry coordinator Christophe Bérout. "But equally important is to be able to feed back to patients worldwide about all the important developments relating to their condition, and to help them feel more closely linked to the research community. This meeting showed that both these aims are becoming a reality."

A full list of all the existing national registries for DMD and SMA can be found at www.treat-nmd.eu/nationalregistries.

Registries for other conditions: broadening the initiative

For the first time, representatives of registries for other neuromuscular diseases were also present, and participants heard about the plans for an international registry for patients with myotonic dystrophy type 1 (DM1), plus the recent launch of a congenital muscular dystrophy registry, the upcoming launch of a registry for patients with FKRP mutations (LGMD2I and MDC1C) and others (see below). All these registries share the same aims of locating patients in preparation for future trials, and providing feedback and a sense of community to the patients affected by these conditions.

Registries overview

DMD and SMA

TREAT-NMD's flagship registries, as described above.

More information: www.treat-nmd.eu/nationalregistries

Myotonic dystrophy type 1 (DM1)

An international DM1 registry using the DMD and SMA model (national registries feeding into a global database) is due for launch in summer 2010. Existing national registries are currently implementing these items into their systems already recruiting patients while has been defined and national registries are, with the global registry.

More information: www.treat-nmd.eu/DM1registry

FKRP mutations

This gene-specific registry collects data from patients affected by FKRP mutations (most often manifesting as LGMD2I but also MDC1C, Walker-Warburg syndrome and Muscle-Eye-Brain disease). As FKRP mutations are less common than diseases like DMD and SMA, this is a single international registry for all patients worldwide, and involves a combination of patient self-report and specialist clinician report.

More information: www.FKRP-registry.org

Congenital muscular dystrophy international registry

TREAT-NMD has worked with the patient advocacy group Cure CMD to support the setup of an international registry for patients with any form of congenital muscular dystrophy.

More information: www.cmdir.org

Myotubular/centronuclear myopathy

A European patient registry for myotubular myopathy is currently being established by the UK patient organisation the Myotubular Trust with guidance from TREAT-NMD. The system is also linked with US plans in the same area so as to ensure an internationally harmonised effort.

More information: http://www.myotubulartrust.com/research_patientregistry.htm

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TREAT-NMD to assist with physiotherapy training for Santhera's pivotal trial

TREAT-NMD announces that it has agreed to collaborate in providing professional physiotherapist training for Santhera Pharmaceutical's pivotal Phase III study with Catena®/Sovrima® (INN: idebenone) for the treatment of Duchenne Muscular Dystrophy (DMD). The 12-month DELOS study will enrol up to 240 patients in approximately 25 centres in Europe and North America. At least one physiotherapist for each study centre is expected to be trained with support of TREAT-NMD.



Based on Santhera's study protocol, which was developed with input from experts in Europe and the US, TREAT-NMD has developed a training manual and program for the physiotherapists involved in the study. The training is designed and led by Michelle Eagle, PhD, of Newcastle University. Together with Julaine Florence of Washington University, Michelle is developing a TREAT-NMD training course for physiotherapists that will also be utilised in other industry-sponsored studies. Both Michelle and Julaine are experienced clinical evaluators and research physiotherapists who have extensive experience of protocol development and physiotherapy training in DMD.

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TREAT-NMD training course takes place in Belgrade

In cooperation with the Clinic for Neurology and Psychiatry for Children and Youth of the Medical Faculty of the University of Belgrade, Serbia, TREAT-NMD organized a training course for professionals entitled "Hot topics in muscular dystrophies and SMA – preparing for clinical trials". Taking advantage of the hosting in Belgrade of the 39th AGM of EAMDA, the European Alliance of Neuromuscular Disorders Associations, the meeting took place on September 17th, 2009 in the Clinic for Neurology and Psychiatry for Children and Youth.



TREAT-NMD's aim in organizing training in central and eastern Europe is to offer professionals in those countries the opportunity to take part in specialist courses locally, without high travel costs. Local and international experts combine to provide a focused training course on topics of particular interest to local professionals.

This formula again proved its effectiveness, since the audience, adult and paediatric neurologists and geneticists, consisted of 34 trainees originating from 5 different CEE countries (Croatia, Romania, Serbia, Slovenia and Turkey).

The attendees actively participated in the meeting, highlighting their interest in greater interaction within their countries and desire to share both diagnostic tools and experience in patient management.

The training consisted of lectures followed by discussion. The morning session focused on clinical management and innovative studies on muscular dystrophies and SMA ("Standards of diagnosis and care for DMD and SMA"; "Innovative research and clinical trials on DMD and SMA") and on the TREAT-NMD tools to be implemented in order to facilitate clinical trials ("National and Global Registries", "Clinical centres eligibility for participation in international studies"). The afternoon session saw presentations on "Hereditary IBM and myofibrillar myopathies", and on the local experience ("Clinical research activity – The Belgrade experience"). Finally, a round-table discussion involving all the speakers focused on the networking activities within various countries, highlighting how networking problems were addressed. The Scandinavian, German, Italian and Croatian experiences were presented and discussed.

Proceedings of the course were distributed to all participants, together with information on various TREAT-NMD activities.

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American Academy of Neurology - Scholarship Awards 2010

The American Academy of Neurology is offering another



International Scholarship Award for 2010. The deadline for applications is 30th November 2009, as part of the award requirements applications must also submit a scientific abstract for the 2010 meeting, this has a deadline of 2nd November 2009.



Selected research abstracts will be presented in exciting new and interactive scientific programs and showcased in high profile scientific sessions and popular poster sessions.

The 62nd Annual Meeting will be held in Toronto, April 10 to April 17, 2010, and will highlight the latest in neurological research through key lectures, more than 2,000 poster and platform sessions, and 160 educational programs.

The AAN Annual Meeting brings together more than 10,000 neuroscience professionals for one of the world's largest neurology gatherings. It has long been a leading showcase for the latest developments in scientific research, and a place to honor peers at the forefront of this work.

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