

welcome

Our international conference is now behind us and we'd like to thank all participants and everyone who worked so hard to make it a success. You'll find a brief overview of what went on in this edition of our newsletter, and more will follow in future editions. The TREAT-NMD Advisory Committee for Therapeutics drew a lot of interest at the conference, and we're pleased to announce that the application form for those wishing to submit an application for review is now available online. Some interesting job opportunities in the neuromuscular field are also highlighted, together with a report of a recent SMA researchers conference in Edinburgh.

We have one more edition before the Christmas break, and as always, if you have anything to be considered for inclusion, please contact the coordination office with your suggestions.

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

at a glance...

27 Nov 2009
King's College London 9th Annual Neuromuscular Symposium

26-27 Feb 2010
Towards a Brighter Future - Sydney 2010

10-17 Apr 2010
American Academy of Neurology - Annual Meeting 2010



TREAT-NMD/NIH conference establishes interaction as the basis for future progress

The TREAT-NMD/NIH International Conference is now successfully behind us, and we'd like to thank everyone who worked so hard to make it such an exciting event. The conference brought together 350 delegates from over 30 countries, and one of the highlights for many was its interactive nature. Extensive panel discussions, questions and contributions from the floor and a large number of questions submitted online all ensured that this was not a traditional didactic lecture series but an open and informed debate addressing the challenges facing the whole of the neuromuscular field as we move into an era where therapeutic development is becoming a reality. The eight sessions and 134 accompanying posters covered the major topics in translational research, and the conference made use of the latest results from ongoing projects to draw conclusions of broader relevance to the field as a whole, encourage cross-talk between disciplines, and highlight the areas in which further collaborative efforts are needed. The audience was an almost equal mix of patients, families, researchers, clinicians and industry representatives, and this too was a unique experience for many, with all stakeholders appreciating the opportunity to hear the perspective of the others and to learn from their expertise.



Over the coming weeks and months a number of publications will be produced with detailed results of the main sessions. An online version of the poster abstract book and a link to photographs of the conference is available via the conference website [HERE](#).

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TREAT-NMD Advisory Committee for Therapeutics (TACT) now accepting applications for review

Application form available to download at www.treat-nmd.eu/TACT



Of the many promising research results presented at conferences, published in journals and hailed as the basis for possible future treatments and cures for neuromuscular disorders, only a very small number ever make it to the clinical trial stage. Evaluating which of them are actually ready for this step is a challenge not only for potential funders and sponsors, but often also for the researchers themselves.

TACT, the TREAT-NMD Advisory Committee for Therapeutics, is an expert body set up by TREAT-NMD to provide free and transparent guidance and advice on the trial-readiness of potential new therapies for neuromuscular diseases and to help the developers of the most promising compounds bring them to trial more quickly. Clinicians and researchers working on new therapies with promising preclinical results can contact TACT for advice on the steps to be taken to move into clinical trials and an unbiased appraisal of the readiness of their therapy for this step. Patient advocacy groups and industry are also invited to come to the committee for guidance.

The strength of TACT is in the calibre of its experts and the rigour and independence of its review process. The committee is chaired by Cristina Csimma who, along with a core group of 8 leading specialists, is guiding the effort and ensuring consistency and continuity of the evaluation process by

being involved in the review of all compounds. The broader committee comprises 35 high calibre professionals with relevant expertise in specific areas (including preclinical, clinical, regulatory, ethical, drug discovery/medicinal chemistry and clinical trial coordination centre representation) who will be brought in for consultation on individual applications.

The need for TACT was identified in discussions between TREAT-NMD and patient organisations, industry and academics who felt that the neuromuscular field lacked a comprehensive, consistent and unbiased way to evaluate novel and already existing compounds put forward as potential therapies. The establishment of TACT is very timely, as the growing number of therapeutic approaches recently claimed to show promise for neuromuscular conditions has led to an urgent need to select the most appropriate targets and compounds for clinical trial and eventual registration, in the context of the limited number of patients eligible for trials and limited funds available. The community needs impartial, expert advice on the status and potential of promising therapeutics and TACT aims to provide this.

An initial (and incomplete) list of over 40 potential drug candidates which have been claimed to show promise for neuromuscular disorders either from theoretical considerations or preclinical studies has been compiled. However, the pathway from a promising idea to drug registration is complex and needs to take into account multiple areas of expertise, and this requires a multidisciplinary approach that includes industry drug development know-how. With perspective and experience over the overall drug development process, including regulatory implications, TACT will be an essential educational tool for the field.

Applicants presenting a possible candidate for appraisal by TACT will be assured of receiving impartial and broadly informed recommendations. The committee does not itself make go/no-go decisions, but its recommendations will allow applicants to plan a realistic route forward to trial and registration or highlight challenges and potential solutions.

TACT will assess both novel approaches, not yet tested in other conditions and requiring a greater level of clinical study-enabling data, and approved drugs with potential for repurposing. In both situations the goal of the advice is to position the potential therapy along a realistic pathway to eventual clinical trial and registration.

TACT review meetings will take place every 6 months, and interested parties are invited to submit applications for the first review meeting now. The application form, designed to facilitate a comprehensive appraisal of each proposal that considers all available information, is available to download at www.treat-nmd.eu/TACT/. Interested groups should contact Emma.Heslop@ncl.ac.uk to indicate to the secretariat their intention to submit an application as soon as possible and **completed applications must be received before 5pm (GMT) on the 15th December 2009** in order to be considered for the first review meeting.

Based on their expertise and a critical review the TACT core group will shortlist the 2 or 3 most promising applications to move forward to full appraisal by the committee, and all applicants will be notified whether or not their application has been selected for full appraisal before the 30th December 2009. The first TACT review meeting will take place in Rome on the 6th-8th February 2010. Evaluation will be provided to applicants within approximately 6 weeks of the review meeting and a non-confidential summary will be published on the TREAT-NMD website within 2 months.

The application process will focus the applicant on a number of important considerations including scientific rationale, the appropriateness and interpretation of preclinical studies performed; safety and toxicology issues; drug distribution and kinetics; feasibility and cost of drug manufacturing and supply; context of project in the clinical development plan and regulatory considerations critical to advancing a compound into the clinic.

TACT is an ambitious committee with an aim to be helpful to many different groups. For the researcher with interesting preclinical results on a compound TACT can offer specific advice. For a clinician, TACT can provide perspective on the non-clinical aspects of drug development. For all groups, TACT can advise on clinical trial design, funding and regulatory applications, access to the other TREAT-NMD resources and regulatory advice. TREAT-NMD hopes that industry will also use TACT as a way to better understand the target NMD community and finally, TACT will work with funders to show how a TACT review can add value to a proposal.

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Clinical and research job opportunities advertised on TREAT-NMD website

Job vacancy: Clinical Research Associate

Salary: £27523 - £30685
Closing Date: 29 November 2009

BIO-NMD is a new EU funded project aimed at defining novel biomarkers in neuromuscular disease.

An opportunity has arisen for a clinical research associate to join an international team of clinicians and scientists to focus on the identification, collection and distribution of biological samples in support of the BIO-NMD project. The successful applicant will work closely with the TREAT-NMD coordination team and an international collective of researchers working on this exciting project.

You will be medically qualified with higher medical training in paediatrics, paediatric neurology,



neurology or genetics and some laboratory based science experience. The post requires a highly motivated self-starter capable of effective team work, yet able to work independently.

The post is tenable for 3 years.

For further information please contact Prof. Hanns Lochmüller, hanns.lochmuller@ncl.ac.uk or apply online at the Newcastle University website.

Research positions in muscle biology: 9 PhD and 4 post-doctoral positions - Positions open at 8 universities/research institutions in 6 European countries from November 2009
The Marie Curie Initial Training Network on Muscle Z-disk Protein Complexes: from atomic structure to physiological function (ITN MUZIC) offers 9 ESR (PhD) and 4 ER (Post-doctoral) positions in cellular and structural biology

Main Research Field
Cellular and Structural Biology

Job Description

The partners of ITN MUZIC are dedicated to producing the next generation of research scholars working in the interdisciplinary field of cellular structural biology. ESR and ER's individual projects will be integrated into one comprehensive research and training network. In order to train Cellular Structural Biologists profile they will be offered an innovative training in a unique combination of scientific methods that span from biophysical characterisation of proteins and their complexes, high resolution (X-ray crystallography) and low resolution structural biology methods (SAXS, EM, cryo-EM tomography, atomic force microscopy) to a variety of cell biology oriented techniques, ranging from FRET and live-cell imaging, cellular and animal models to animal physiology.

The network is strongly based on the exchange and transfers of knowledge between participating institutions and will provide its fellows with network-wide training activities (workshops, summer schools, scientific symposium) and training-in-collaborations (working visits and secondments).

The ITN MUZIC research programme is organised in four work packages (WP):

- Atomic structure of filamin, telethonin and alpha-actinin in the Z-disk - WP1
- Z-disk Ultrastructure and Molecular Architecture of Its Complexes - WP2
- Dynamic changes of the Z-disk interactome - WP3
- Signalling pathways regulating Z-disk development and remodelling - WP4

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UK SMA researchers conference facilitates collaboration in the scientific community

This year marked the 6th gathering of UK SMA researchers - and a few from overseas as well. Fifty-four people attended, and the extent of expertise and fields of interest represented was impressive, ranging from clinicians to scientists specialising in proteomic work, neuromuscular junction vulnerability and alternative splicing events. A conference overview report is available online via the "more" link below.

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