

## welcome

Welcome to the first TREAT-NMD newsletter of 2010.

This week's edition includes news of an update on the TREAT-NMD website from Dr. Annemieke Aartsma-Rus about current therapeutic approaches for DMD, an announcement of the drugs for review at the upcoming TACT meeting, and information on a new AFM call for proposals.

Details of a postdoctoral opportunity at the Institut de Myologie in Paris are also available, and news is provided of a new neuromuscular centre in Zürich, Switzerland that opened its doors in October 2009, and of the London teaching course "Update in Neuromuscular Disorders".

We would also like to take this opportunity to congratulate Professor Baziel van Engelen on his new appointment as ENMC Research Director.

Finally we would like to wish you all a very happy and fulfilling 2010!

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

## at a glance...

[28-30 Jan 2010 BIO-NMD Kick Off Meeting - Ferrara, Italy.](#)

[12-14 Feb 2010 Italian Parent Project Meeting - Rome, Italy](#)

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

[28 Feb 2010 Rare Disease Day - Worldwide Event.](#)

[18-20 Mar 2010 International Conference on Rare Diseases and Orphan Drugs - Buenos Aires, Argentina.](#)

[25-26 Mar 2010 UK Neuromuscular Translational Research Conference -](#)



### DMD research overview available online

Therapeutic approaches for Duchenne are rapidly evolving. To help patients and families keep abreast of the latest developments, Dr. Annemieke Aartsma-Rus of Leiden University Medical Center has produced an article to provide information about the different therapeutic approaches currently under investigation, to describe the advantages and disadvantages of each approach and to list the hurdles that have to be overcome before these approaches can be applied to patients.

This is a valuable resource for patients and families that complements the existing research reports created by Guenter Scheuerbrandt, and we would like to take this opportunity to thank Annemieke and Guenter for the time and effort they have spent "translating" information on these complex therapies into a more understandable form.



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### TACT announces drugs for first review

TACT, the [TREAT-NMD Advisory Committee for Therapeutics](#), is pleased to announce that as of 15 December, the closing date for the first TACT review round, four applications were received. The TACT core group carried out a preliminary review of the applications to ascertain whether or not they were at an appropriate stage to move forward to a full TACT review.



The core group agreed to progress three of the applications to full review and defer the fourth application for possible review at the second TACT meeting in the summer.

The applications to be considered during the TACT review meeting scheduled for 6-7 February 2010 in Rome are:

1. Losartan
2. Isosorbide and NSAID
3. Flavocoxid.

All three drugs will be considered for indication in Duchenne Muscular Dystrophy.

Following the meeting, and within 6 weeks, TACT will generate a specific detailed report to be provided to the applicant and within 8 weeks a general 'non-confidential' report summary will be available via TREAT-NMD website, to inform the community.

The second call for applications will formally open on 5 February and close on 15 March 2010. We would like to encourage potential applicants to contact

[Emma.Heslop@ncl.ac.uk](mailto:Emma.Heslop@ncl.ac.uk)

[Oxford, England.](#)

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

[3-7 May 2010 International Child Neurology Congress 2010 - Cairo, Egypt.](#)

[5-8 May 2010 The Ottawa Conference on New Directions in Biology and Disease of Skeletal Muscle - Ottawa, Canada.](#)

[10 May 2010 The George Karpati Symposium on Neuromuscular Disease: Innovation and Application, Montreal, Canada.](#)

[13-15 May 2010 5th European Conference on Rare Diseases 2010 Krakow, Poland.](#)

[17-19 Jun 2010 International Conference on Neuromuscular Diseases - Sao Paulo, Brazil.](#)

[24-26 Jun 2010 14th Annual International SMA Research Group Meeting - Santa Clara, California, USA.](#)

[24-27 Jun 2010 Parent Project Muscular Dystrophy Annual Conference - Denver, Colorado, USA.](#)

[24-27 Jun 2010 Families of SMA Annual Conference - Santa Clara, California, USA.](#)

[19-23 Jul 2010 12th International Congress on Neuromuscular Diseases - Naples, Italy.](#)

[23-25 Jul 2010 Jennifer Trust Conference and AGM - Stratford upon Avon, UK.](#)

[12-16 Oct 2010 World Muscle Society International Congress - Kumamoto, Japan.](#)

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

early in the process, before completing the form, to discuss their application and potentially complete a pre-application form. The second review meeting is scheduled for 5-6 June 2010 in Barcelona.

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### AFM call for research proposals and new translational research committee

AFM, the French Muscular Dystrophy Association, finances more than 300 basic and clinical research programs per year through a biannual call for projects. It is currently overseeing 34 clinical trials for rare diseases and more than 25 strategic projects.



AFM welcomes international applications and is especially interested in funding projects in the following areas:

- I. Accelerating translational research with the goal of overcoming obstacles in the discovery of effective therapies on rare diseases. To evaluate these projects, the AFM has put in place a new Translational Research Committee composed of members with expertise to select the most promising projects dedicated to overcoming hurdles in the clinical development for neuromuscular diseases.
- II. Fundamental research projects on the physiopathology of diseases of the neuromuscular system which target the comprehension of the disease mechanisms.
- III. Creation of integrated clinical and translational research sites for neuromuscular diseases.
- IV. Clinical projects which aim to improve patient care

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### New Research Director for ENMC

ENMC is pleased to announce the appointment of Professor Baziel van Engelen (Nijmegen, the Netherlands) as ENMC Research Director. Professor van Engelen is succeeding Professor Kate Bushby who is leaving this post after three years to lead the NIH steroid trial. ENMC would like to thank Katie for her great achievements during this period.



Professor van Engelen was appointed by the ENMC Executive Committee following his experience within ENMC, as an active member of the Research Committee as well as organiser and participant in many ENMC workshops.

Professor van Engelen's previous experience as director of the Dutch Neuromuscular Research Support Centre (ISNO) will benefit ENMC in the coming years as it enters an era of new opportunities.

Professor van Engelen is professor in neuromuscular disorders in Nijmegen, the Netherlands and principal investigator at the Donders Centre for Neuroscience.

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### New Centre for Neuromuscular Diseases opens in Zurich

A new Centre for Neuromuscular Diseases was opened in Zürich, Switzerland in October 2009. It represents the main contact point for patients with neuromuscular diseases in Switzerland and merges the competence and expertise of the University Hospital with the Children's Hospital in Zürich. The Neuromuscular Centre is built on interdisciplinary cooperation to ensure an integral therapeutic approach: patients can profit from the medical know-how of the Institutes of Neurology, Pneumology, Rheumatology, Cardiology, Orthopaedics and Gastroenterology, have easy access to Physiotherapy, Occupational Therapy and Speech Therapy, and have guidance and advice for psychological, social and economic problems.



By closely collaborating with the Institute of Human Movement Sciences of the Swiss Federal Institute of Technology and with the Institute of Experimental Immunology of the University Hospital, and by participating to international and national studies, this new centre contributes to the development of new therapies and treatments.

[More information \(only in German\)](#)

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### Update in Neuromuscular Disorders teaching course - London, UK



Scheduled to take place from 24th - 27th May 2010, this course, now in its third year, is the result of the merging of two popular annual courses with an established international reputation: the "Hammersmith Hospital update" on the latest research aspects, clinical and management advances related to childhood neuromuscular disorders, and the "Institute of Neurology neuromuscular short course", a stimulating update on adult inherited and acquired neuromuscular disorders.



The London course provides updates on a wide range of neuromuscular conditions and attracts an international range of participants. Further programme information will become available shortly along with registration information.

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### Post-doctoral position in Paris

A postdoctoral position is available at the INSERM-UPMC-Paris VI UMRS\_974, Institut de Myologie, Groupe Hospitalier Pitié-Salpêtrière (Paris), from Feb-March 1st 2010.



The candidates must have a Ph.D. and a strong background in cell culture/biology or muscle physiology. Previous experiences in confocal microscopy and molecular biology will be an important asset.

The successful candidate will join a multidisciplinary, highly dynamic group working at the interface between basic and biomedical research and covering all fields of research on muscle disorders (genetics, pathophysiology, therapeutic approaches).

He/she is expected to conduct with high autonomy a research project on the regulation of mechanotransduction in skeletal muscle cells using 3D culture, and to have the potential to develop eventually his/her own lines of research.

The successful applicant will integrate an internationally-known reference center on muscle disorders, with a highly stimulating scientific and medical environment.

Applications, including a curriculum vitae and contact details for at least two referees, should be sent to:

Catherine COIRAULT, MD-PhD  
E-mail: [c.coirault@institut-myologie.org](mailto:c.coirault@institut-myologie.org)  
Phone : 331 42 16 57 55  
<http://www.institut-myologie.org/>

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### Call for meetings for publication on TREAT-NMD website

2010 is already shaping up to be a very busy year with many meetings and conferences of relevance to the neuromuscular field. The [meetings and events](#) section of the website reflects this and we hope it is of use to you when scheduling your events or considering which ones to attend.



If you know of a relevant event that is not listed please [email the TREAT-NMD Coordination office](#) with the details.

Your support will help us keep this area of the website as comprehensive as possible and will benefit all visitors.

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