

## welcome

Welcome to the 71st TREAT-NMD newsletter. Articles featured this week include:

- A report on the TREAT-NMD Advisory Committee for Therapeutics' first round of applications.
- Details of an international conference on neuromuscular diseases in Sao Paulo
- Guenter Scheuerbrandt's report on the TREAT-NMD conference.
- A call for research proposals from the Dutch Duchenne Parent Project.

We would like to thank those who have contributed to this week's 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](mailto:info@treat-nmd.eu)

We look forward to hearing from you!

## at a glance...

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

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

[25-26 Mar 2010 UK Neuromuscular Translational Research Conference - Oxford, England.](#)

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

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

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

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

[24-27 Jun 2010 Families of SMA](#)

# update

## TACT First Review Meeting

A multidisciplinary group of seventeen members of the international TREAT-NMD Advisory Committee for Therapeutics met in Rome on the 6th-7th February 2010 to conduct the first drug review meeting. Two additional TACT members who were unable to attend in person provided written assessments that contributed to the reviews (Didier Caizergues, Jeff Towbin).



Due to severe snow storms in the US, Jerry Mendell, Kevin Flanigan and Avital Cnaan were unable to attend the meeting in person however they heroically participated via teleconference at appropriate times over the one and a half days of the meeting.

During the meeting TACT reviewed three proposals for clinical trials submitted by academic clinical research applicants from Italy and the US:

1. Losartan - Christopher Spurney, Children's National Medical Centre.
2. Isosorbide plus Ibuprofen - Emilio Clementi and Grazia D'Angelo, H Sacco University Hospital, University of Milano.
3. Flavocoxid - Sonia Messina and Giuseppe Vita, University of Messina.

Each applicant attended the meeting (via teleconference or in person) for approximately one hour to discuss their proposal with the committee, which provided helpful additional context for applicants and reviewers as well as clarified specific areas where the applicants are seeking advice. By March 19th (within 6 weeks of the meeting) the committee will provide to the applicants an assessment report providing recommendations, including specifics to strengthen the proposal and to answer questions raised by the applicants. TACT hopes that this advice will be useful in effectively planning the next steps for the projects.

TACT will also generate a review summary, with input from the applicants, which will be made available via the TREAT-NMD website by 2nd April 2010 as well as some general recommendations for applicants and reviewers based on the experience with the first applications.

The deadline for applications to be reviewed at the next TACT meeting (5th-6th June in Barcelona 2010) is the 15th March 2010 and all potential applicants should contact the TACT secretariat ([TACT\\_Core@treat-nmd.eu](mailto:TACT_Core@treat-nmd.eu)) to discuss their proposal before completing the full application form. The experience of the first meeting suggests that the TACT review process can be very useful for applicants and add real value to their plans. We look forward to their feedback as they move forward with their programs.

**MORE >**

## TREAT-NMD conference report from Guenter Scheuerbrandt

Guenter Scheuerbrandt, known the world over for his accessible and comprehensive reports on current approaches to research and treatment of DMD, has released a report of the TREAT-NMD conference that took place in Brussels last November. Unlike his usual reports, this latest one focusses upon the 'barriers' that must be overcome if new and promising therapies and treatments are to be rapidly translated into the clinic to make them available to patients as quickly as possible.



Systematically covering each session, Guenter summarises not only the speakers' talks but also the

[Annual Conference - Santa Clara, California, USA.](#)

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

[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](#).

participatory elements of the conference which covered a number of neuromuscular diseases. This report is now available to download from the conference section of the website (see the "downloads" link to the right of the page) and we would like to take this opportunity to thank Guenter for the work he has done in creating such a comprehensive overview.

[MORE >](#)

### International conference on care and clinical trials in NMDs

Sao Paulo, Brazil, 17–19 June 2010

#### International Conference on Neuromuscular Diseases: Care and Clinical Trials.



This exciting meeting is aimed at healthcare professionals with an interest in neuromuscular disorders (with a special focus on DMD and SMA) and patients and families. Two days of lectures from a strong panel of international and Brazilian speakers will be followed by a dedicated family day with research updates for patients and information on physiotherapy and care.

Conference venue:  
Sirio Libanes Hospital

Rua Dona Adma Jafet, 91  
São Paulo - SP,  
Brazil.

Further conference details and conference registration can be found [here](#).

[MORE >](#)

### Duchenne Parent Project Netherlands - Call for research proposals

The Dutch Duchenne Parent Project organisation is calling for international researchers to submit research proposals for review.

An international scientific board will consider proposals related to the therapy of Duchenne Muscular Dystrophy that are both innovative and multidisciplinary.



Submissions should be made in English and projects should be in accordance with the aims of DPP NL.

The submission deadline for international researchers is 1st April 2010.

[MORE >](#)

[www.treat-nmd.eu](http://www.treat-nmd.eu)

[Unsubscribe](#) | [view this newsletter online by clicking this link](#)