

welcome

Welcome to the latest TREAT-NMD newsletter.

This edition includes:

- a call to support the French Telethon
- an update on the Registry of Outcome Measures
- a call for workshop applications from ENMC
- information about the upcoming myology congress in Lille, France

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...

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](#)

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](#)



TELETHON UNDER THREAT! Sign a petition online to support this crucial event

Since 1987 the annual [French Telethon](#) - initiated by the French muscular dystrophy association AFM - has been an exceptional example of solidarity and generosity that has given hope and support to individuals with rare diseases not only in France but across the world. It has recently been reported that French Television may not allow this event to continue. Don't let this year's Telethon, which begins this evening, be the last!



Collecting almost 100 million Euros per year, the Telethon makes it possible to fund research programmes that would not otherwise exist. It provides support for clinical trials that will give tomorrow's therapies, and for information services and socially innovative support services. The work of AFM-TELETHON is crucial for patients with rare diseases.

The Telethon has a direct impact on the life, well-being and hope of millions of families in Europe. An end to it would have dramatic consequences in France and repercussions throughout Europe and beyond. It would be a hard blow for those who already suffer physically from injustice at birth and for those who fight beside them to improve their daily lives.

Wherever you are in the world, you can support their cause.

[Researchers and clinicians across the world are invited to sign a petition here.](#)

[Eurordis, the multinational federation of 447 rare disease organisations in 44 countries, has a petition here.](#)

Please add your voice to support the continuation of this event... for the benefit of the 30 million rare disease patients in Europe and the many more worldwide.

An Update on the Registry of Outcome Measures (ROM)

ROM was officially launched in October 2008. Since then numerous experts from around the world have contributed information to the Registry, identifying OMs that might be of benefit to the NMD community, entering summary details describing the OMs and providing updates on recent publications which are added to ROM as reference links. Thanks to this contribution we now have over 110 published records in the registry and more are on the way.



In 2009, we introduced a staged approach to publication, which enabled us to alert the community to the availability of OMs by way of a Stage 1 reference record (e.g. OM Name + Key Reference or Official Website). We have found this to be beneficial to expanding the registry and improving access to information. If you notice an OM is missing from the Registry and can supply this information for it, we'll do our best to add it ASAP. We recently did this for the DM1-Activ and are hoping to have more detailed information published for this OM shortly.

Some of the records that were originally added using this approach in 2009 have been updated and

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](#).

now contain a wealth of detailed information for the benefit of the community. A great example of this is the 6MWT for SMA, as mentioned in an earlier edition of this newsletter, which is now advanced to a Stage 3 record, with comprehensive summary information and a number of publication links. These records continue to grow.

In the latter half of 2010 we added a number of Stage 2 records, including the Brief COPE, COPE, Hospital Anxiety and Depression Scale and Life Orientation Test. A suite of detailed manual muscle tests for IBM have also been published in the last couple of weeks with more in draft form for QMT. These were generated by a survey undertaken by Michael Rose and we are hoping surveys underway and planned for coming months will generate similar results and additions for myotonic dystrophy and other NMDs.

We will endeavour to keep the "Recent Additions" (<http://www.researchrom.com/ra>) section of the ROM up to date to keep you apprised of OMs as they are published.

Many many thanks to all the experts who have contributed to the expansion of the Registry since its launch in 2008!

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ENMC now accepting applications for workshops in late 2011

In order to establish a more efficient, standardised and transparent process for assessing workshop applications, ENMC have implemented a new procedure resulting in two grant review rounds per year.

The deadline for sending in applications for ENMC workshops to be conducted in the second half of 2011 is March 1, 2011. The forms to be completed for a workshop application can be downloaded from the website www.enmc.org

Four ENMC workshops are being planned for the first half of 2011. Workshops include RYR1-related myopathies and Pain and fatigue in neuromuscular disorders: prevalence and management. Further workshop details can be found on [this page](#) of the ENMC website.

If you have any questions regarding this, please do not hesitate to contact the ENMC office enmc@enmc.org

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Register now for the Myology 2011 congress

Registration is now open for the Myology Congress 2011, which will take place from May 9 to 13 in Lille, France, at the Lille Grand Palais.

During the five days of the congress, close to 1,000 participants from across the world will receive an update on the scientific and medical advances made in muscle science and its related disorders. Tuesday and Wednesday will be devoted to fundamental science while Thursday and Friday will focus on clinical research. It will be an opportunity to witness the enthusiasm of the new discipline of myology, as exemplified by the numerous therapeutic avenues being explored and by the emerging clinical trials in humans.

Poster submission deadline: 24 January 2011

Deadline for registration: 11 April 2011

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