

welcome

Welcome to the 74th Newsletter from TREAT-NMD.

This week's newsletter includes...

- the launch of the Family Friendly Guide for the Standards of Care for DMD
- details of the new FOR-DMD trials
- the second round of applications received for TACT to review
- a call for experts to deliver TREAT-NMD training courses

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

at a glance...

[25-26 Mar 2010 Asian Oceanian Myology Center Annual Meeting - Seoul, South Korea](#)

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

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

[23-24 Apr 2010 2nd Joint Meeting Belgian-Dutch Neuromuscular Study Club and German Reference Center for Neuromuscular Diseases of the DGNN - Valls, Netherlands](#)

[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](#)

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



Diagnosis and Management of DMD – a guide for families

The MDA, PPMD, TREAT NMD and the UPPMD have been working closely to produce a comprehensive "guide for families" based on the Lancet neurology article published early this year. Academic publications are not always written in a way that are easy to understand without a medical background and so the guide has been demedicalised to make it accessible to all, whilst still closely following the full academic article.



Individuals with Duchenne and their families will hopefully find the guide useful background information to use in collaboration with their care providers, and the full academic document, to discuss their own specific care needs. The family guide has been produced in several different formats and will be appearing in many different languages in the near future. In some countries, printed brochures will be made available by the relevant patient organisations.

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Introducing FOR-DMD (a trial of different corticosteroid regimens in Duchenne muscular dystrophy) and a call for patient partners

For many years a major obstacle to the standardisation of care in Duchenne Muscular Dystrophy (DMD) has been indecision about what corticosteroid regime is best to use and how to best prevent side effects. This is a difficult area to decide as the balance as to what is best for any individual patient needs to take into account not only how much benefit the drugs are giving, but also what the side effects are. No randomised controlled trial has yet been done to compare different regimes head-to-head, and unfortunately the long term results from cohorts of patients in different clinics are often uncontrolled and therefore cannot give definitive data on which regime is "best".



It is over 20 years since steroids were shown to benefit DMD, (they prolong the ability to walk as well as increase respiratory function, resulting in much less need for spinal surgery, and also stabilise cardiac function) but there remain clinics around the world that do not use steroids, and amongst those who do there is a bewildering range of different regimes in use. While the recently published care guidelines for DMD and the Cochrane review do suggest that daily steroid regimes have the greatest weight of evidence behind their use, the decision on whether to use daily prednisone or deflazacort, or if an alternative regime might in the long run be better, remains an open question.

FOR-DMD (Finding the Optimum Regimen of Corticosteroids for DMD) is a trial of different steroid regimes that has now been funded by the NIH. It will test daily prednisone and deflazacort regimes against one of the more commonly used alternative regimes (10 days on and 10 days off prednisone). The trial is led by Dr. Robert Griggs from the University of Rochester in the USA and Prof Kate Bushby from the Newcastle University in the UK, linking the [Muscle Study Group](#) and TREAT-NMD network in this joint initiative. Last weekend the operational steering group of the trial met in Freiburg for the first time since the funding has been secured to discuss how the trial will proceed.

It is anticipated that the first patient will be recruited around March 2011. 300 children aged between 4 and 7 years, who have not previously been on steroids, will be recruited and followed for at least 3 years. There will be a standardised, systematic process to minimise and control side effects, and the drugs will be judged not only on their ability to improve muscle function but also how acceptable the side effects are. It is planned that the trial will be carried out in around 11-13 different countries - many

[12-15 Jun 2010 European Human Genetics Conference - Gothenburg, Sweden](#)

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

[19-23 Jun 2010 20th Meeting of the European Neurological Society - Berlin, Germany](#)

[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](#)

[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](#).

sites have already volunteered to take part and the TREAT-NMD patient and care and trial site registries are participating in the feasibility and set up process.

Patient advocacy groups have been an integral part of the process for the planning of the FOR-DMD steroid trial, including pushing for the study in the first place, acting as members of the planning committees, and assisting in the preparation of the grant. The first two planning meetings for FOR-DMD were kindly supported by the ENMC as part of their clinical trials unit programme.

As the grant is due to become operational very soon, we would like to broaden the participation of patients in ensuring the success of this trial so that we can plan for the timeliest translation as possible of the results into clinical practice.

We would like to invite patients and advocacy groups to become part of the FOR-DMD patient partners. We welcome any suggestions or advice but in particular, we are keen to get input on: protocol and patient information material; advice on areas of management, especially relating to weight control and behaviour management; and how we can work together on ways to maximise recruitment and retention through a co-ordinated publicity campaign. We will also be seeking to recruit a patient representative for the Data and Safety Monitoring Board. If you are interested in discussing these roles further, or for any additional information please contact [Karen Rafferty](#).

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TACT applications confirmed for the second round

TACT is pleased to announce that as of March 15th, the closing date for the second TACT review meeting, we received three applications. The TACT secretariat carried out a preliminary assessment to ascertain whether or not they were at an appropriate stage to move forward to a full TACT review. All three applications will be reviewed at the next TACT meeting scheduled for 5th -6th June 2010. The applications are:



1. Laminin-111 for Duchenne Muscular Dystrophy
2. N-Acetyl cysteine for a Congenital Muscle Disorder
3. P-188 for Duchenne Muscular Dystrophy

As with the first review, within 6 weeks following the meeting, TACT will generate a detailed report to the applicant and within 8 weeks a general 'non-confidential' report summary, developed in collaboration with the applicant, will be available via TREAT-NMD website, to inform the community.

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Call for expert trainers

As part of the training and education activity within TREAT-NMD, ENMC are looking for experienced clinicians, scientists and patient representatives who are able to deliver training on topics ranging from diagnostics to physiotherapy and care standards, to get in touch.



One of the training and education aims in the TREAT-NMD project is to offer training to various audiences in Central and Eastern European countries. Two training courses were organised in 2009, the first on standards of care was held in Budapest and the second focussing on clinical trials readiness was in Belgrade. Participants of these courses which included clinicians, geneticists, parents and patient organisations provided very positive feedback about the courses.

This year training courses are planned for St. Petersburg in May and Istanbul in July. In addition to this, we are currently discussing the possibilities of hosting a training course in several additional cities, including Milan.

ENMC are developing a panel of expert trainers capable of delivering training lectures on the specialist topics below and would welcome contacts from anyone who would be interested in sharing their expertise.

- Diagnosis
- Outcome measures
- SMA basic care
- DMD basic care
- Registries
- Use of muscle biopsies
- Analyses of muscle biopsies
- Cardiac management
- Pulmonary management

- Rehabilitation
- Patient perspective

If you are interested in delivering training lectures and cooperating with the ENMC in disseminating knowledge please contact [Katelijne Senden](#) (senden@enmc.org) to discuss further details.

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