

## welcome

Welcome to the 65th Newsletter from the TREAT-NMD team.

This newsletter includes a reminder to all DMD preclinical researchers to consider completing our questionnaire on the mdx and GRMD models, a report on the TREAT-NMD session at this year's Action Duchenne conference, details of the vacancy for ENMC Research Director, and an update on the publication of the workshop report from the TREAT-NMD/Marigold workshop on patient registries and trial readiness in myotonic dystrophy.

Our International Conference is nearly upon us but registrations are still being taken and accommodation can also be booked, subject to availability, from the [conference website](#). We are still receiving questions for the various conference sessions. If you have a question that you wish our panel of experts to consider, you still have time to submit it via the [website](#).

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

The main image on today's newsletter shows Kayla, who has the congenital form of DM1, enjoying a day out at Fairy Tale Town.

## ..... at a glance...

[14-18 Nov 2009 Mechanisms Involved in Muscle Atrophy and Hypertrophy, Carmona, Spain.](#)

[17-19 Nov 2009 TREAT-NMD / NIH International Conference](#)

[27 Nov 2009 King's College London Neuromuscular Symposium](#)

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



### Wanted: DMD preclinical researchers' feedback

Anyone involved in preclinical research using animal models for Duchenne muscular dystrophy is invited to complete a short research study to enquire on and improve the services that TREAT-NMD offers researchers in the field of DMD. The survey is in the form of a short online questionnaire that will take about 5 minutes to complete. Your responses are important to us and will help evaluate the success of the TREAT-NMD initiatives in harmonizing and accelerating preclinical treatment development for DMD. The results of the survey will help improve the tools that TREAT-NMD offers to scientists.



Please feel free to forward the link to anyone you think may be interested in participating. Thank you in advance for your collaboration.

[Complete our Questionnaire](#)

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### Myotonic dystrophy patient registry workshop report available online

A report on the TREAT-NMD/Marigold Foundation workshop entitled Patient Registries and Trial Readiness in Myotonic Dystrophy has now been accepted for publication in *Neuromuscular Disorders* and is available online. The workshop, which took place in Naarden, the Netherlands, in June of this year, laid the foundations for an international DM1 registry due to be launched in 2010, as part of the broadening of the TREAT-NMD patient registries initiative to new disease groups.



The report is available [here](#) and further information is available on the TREAT-NMD website [here](#).

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### UK patient conference provides update on DMD research and care

The UK patient advocacy group Action Duchenne hosted their 7th Annual Conference from 23-24 October 2009 at the Holiday Inn, Bloomsbury, London. The conference included a dedicated TREAT-NMD session at which participants could find out about many of the TREAT-NMD activities of direct relevance to Duchenne research and care.



Over 300 delegates, including patients and their families, researchers, clinicians and industry representatives attended the 2-day conference which focused on the latest developments in the pursuit to find treatments for Duchenne.

TREAT-NMD kicked off the presentations with a session on Friday morning aimed at addressing the barriers to translational research and clinical trials. Prof. Kate Bushby (TREAT-NMD Coordinator)

opened the TREAT-NMD session with a discussion and update on the international standards of care (SOC), which are due to be published in *Lancet Neurology* early next year. Katie highlighted the fact that TREAT-NMD in collaboration with the international patient organisations will be a developing family-friendly version of the document and Karen Rafferty, our new TREAT-Duchenne UK coordinator, will be helping to implement the standards of care in the UK over the coming year.

Prof. Dominic Wells, (TACT Core Committee member) then presented an excellent overview of the newly established TREAT-NMD Advisory Committee for Therapeutics (TACT). Nic noted that the aim of TACT is to provide transparent and consistent guidance and advice to the neuromuscular community on the prioritisation and readiness of potential new therapies for neuromuscular diseases. The ultimate goal of TACT is to assist the movement of promising candidates into clinical trial by helping their developers take the necessary steps.

Dr. Pauline McCormack (Research Associate, TREAT-NMD, Policy, Ethics and Life Sciences) gave a presentation entitled 'influencing trial design', which illustrated the need for patients and family members to get involved in the clinical trials process, in particular by becoming advocates. She discussed a new European funded initiative – PatientPartner, which is "based on the belief that involving patient organizations as equal partners at all stages of clinical trials contributes to research that is better adjusted to the real needs of patients" (*from PatientPartner website*).

The last presentation in the TREAT-NMD session was by Dr. Brigitta von Rekowski (TREAT-NMD UK Patient Registry Curator), who discussed the 'purpose, update and utility' of the global patient registries. Brigitta highlighted the importance for all patients to sign up with their own national registries (in the UK with the Action Duchenne registry) and detailed the ways in which industry are proactively making use of the registries when planning their upcoming multi-centre clinical trials.

In addition to the TREAT-NMD sessions, speakers from three different teams working on exon skipping technology presented at the conference, as did the companies involved in exon skipping clinical trials in Duchenne, AVI BioPharma and Prosensa B.V.

The conference was very informative for all and continues to grow from strength to strength.

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## Applications invited for ENMC Research Director

The European Neuro Muscular Centre (ENMC) is a platform of international neuromuscular patient organisations, whose main scope is to facilitate communication among scientists, clinicians and persons affected by neuromuscular diseases, through the organisation of international workshops. The ENMC is a partner in collaborative activities, such as the EU funded Network of Excellence Treat-NMD (Translational Research in Europe for the Assessment and Treatment of Neuromuscular Disease).



To guide and develop ENMC's scientific activities, ENMC is seeking candidates for the position of ENMC Research Director

### Responsibilities:

The Research Director is expected to:

- Chair the Research Committee and guide the assessment of workshop applications.
- Support the Executive Committee in the definition and implementation of the ENMC's scientific policy, and pro-actively support the scientific activities administered by the ENMC office.
- Represent ENMC and support its positioning in the neuromuscular community, also favoring its involvement in new opportunities.
- Advise and support the ENMC Executive Committee Chair and Research Manager to develop the strategic plan for the TREAT-NMD activities led by ENMC dealing with scientific training and education.

### Qualifications:

- Medical doctor, preferably a neurologist, with experience in neuromuscular diseases with a distinguished and sustained research record evidenced by publications in leading internationally refereed journals.
- Excellent organisational and interpersonal skills, strategic vision and leadership.
- Internationally oriented, Europe based, good command of spoken and written English.

### Applications:

Qualified candidates interested in this position should submit their curriculum vitae together with a letter of motivation to [enmc@enmc.org](mailto:enmc@enmc.org) by December 15, 2009. For more information on the ENMC and its activities, please visit our website [www.enmc.org](http://www.enmc.org). For enquiries, please contact [Annette Boersen](#), ENMC Research Manager or [Anna Ambrosini](#), ENMC Executive Committee Chair.

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