

welcome

Welcome to our 68th newsletter and our last newsletter of 2009!

This week's newsletter includes a reminder about the long-awaited DMD care guidelines which are now available online. The full January edition of the *Lancet Neurology* journal will be available from Monday and will also include a podcast featuring Kate Bushby talking about the document's importance.

This edition also includes information about a call for abstracts from the 2010 European Conference on Rare Diseases, the deadline for which is 31st December 2009, plus news of updates to the TREAT-NMD Regulatory Affairs Database and details of the second issue of the UK Muscular Dystrophy Campaign's annual magazine *Target Research*.

This is our last newsletter of 2009, which has been a very memorable year for TREAT-NMD. We are all looking forward to 2010 and would like to take this opportunity to thank you all for your support and wish you all the very best for the coming year!

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

at a glance...

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

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

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

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



International consensus guidelines set out best-practice care for Duchenne muscular dystrophy

A major international consensus document setting out best practice in care for Duchenne muscular dystrophy (DMD) is now available as an e-publication from the *Lancet Neurology* journal ahead of its printed release in January 2010. The product of an extensive review process by 84 international experts representing 20 disciplines across DMD diagnosis and care, this document is a unique guide for individuals, care providers and families to comprehensive healthcare management for individuals with DMD.

The drafting of these guidelines is the result of a three-year-long project guided by the US Centers for Disease Control (CDC) using a rigorous review process that required expert panels to consider more than 70 thousand different care scenarios. The preparation of the guidelines was supported by advocacy groups worldwide and by TREAT-NMD (www.treat-nmd.eu), an international network formed to advance diagnosis, care and treatment for people with neuromuscular diseases. In a close collaboration between TREAT-NMD, patient advocacy groups and healthcare professionals, the full academic publication is also being transformed into a comprehensive "family guide", which will be made available at the start of 2010.

There is still no cure for DMD, but it is recognised that receiving the best care can dramatically improve the quality of life and life expectancy of individuals with the condition, enabling them to lead fulfilling, independent lives into adulthood. The importance of care recommendations such as these therefore cannot be underestimated. The international guidelines, which cover the diagnostics, cardiovascular, neuromuscular, gastroenterology and nutrition, orthopaedic and surgical, psychosocial, rehabilitation and respiratory fields, can be used by doctors, patients and families worldwide as a guide to the treatment that individuals with Duchenne should receive at each stage of the disease. They are also a valuable tool for lobbying at a national level to enable incorporation of these recommendations into national health systems.

"What is really significant about these guidelines is the weight of international expert opinion behind them," explains Kate Bushby, managing editor of the *Lancet Neurology* article, coordinator of the TREAT-NMD network and herself a doctor specialising in DMD and related neuromuscular conditions. "Guidelines containing really quite similar recommendations have been produced before, but always by a much smaller group of authors or an individual patient advocacy group, which has meant they have been easier to ignore. This document represents real international consensus including both the medical and the patient advocacy perspectives and can be used across the world as a powerful tool to recognize those centres where best practice is already in place and to identify gaps in care."

Most of the recommendations in the document are not for especially expensive or hard-to-obtain treatments, or indeed for care that is not already available in many of the best centres worldwide. What is stressed is the importance of a multidisciplinary approach – the necessity for patients to see specialists in all the fields that are involved in DMD, and for those specialists to talk to one another to ensure a coordinated approach to the care of each individual. With this authoritative document behind them, TREAT-NMD will work with patient advocacy groups, healthcare professionals and health authorities across the world to establish the best ways of implementing these recommendations and ensuring that all individuals with Duchenne have access to best-practice care.

Document details

Bushby K et al, Diagnosis and management of Duchenne muscular dystrophy part 1 and Diagnosis and management of Duchenne muscular dystrophy part 2, *The Lancet Neurology*, In press, 2009

Link to the *Lancet Neurology* article (parts 1 and 2 combined) hosted on the TREAT-NMD website:

<http://www.treat-nmd.eu/diagnosis-and-management-of-dmd>

Links to the *Lancet Neurology* article via Science Direct:

Part 1: [http://dx.doi.org/10.1016/S1474-4422\(09\)70271-6](http://dx.doi.org/10.1016/S1474-4422(09)70271-6)

Part 2: [http://dx.doi.org/10.1016/S1474-4422\(09\)70272-8](http://dx.doi.org/10.1016/S1474-4422(09)70272-8)

Note: A comprehensive family guide will be made available in January 2010. Anyone interested in receiving further details or translating the guide into their own native language is invited to contact TREAT-NMD at info@treat-nmd.eu.

About TREAT-NMD

TREAT-NMD is an international network that was formed to facilitate collaborative research in neuromuscular disease and create the infrastructure to ensure that the most promising new therapies reach patients as quickly as possible. The network brings together the key players in the neuromuscular field and is developing the resources that industry, clinicians and scientists need to bring novel therapeutic approaches through preclinical development and into the clinic, as well as helping to establish best-practice care for neuromuscular patients worldwide.

For more information, visit www.treat-nmd.eu

About the document creation process

The document was created using a rigorous review process (known as the RAND/UCLA Appropriateness Method or RAM) that required 84 international experts to rate interventions and assessments used in the management of DMD for "appropriateness" or "necessity" at different stages of the condition. The US Centers for Disease Control and Prevention (CDC) supported its development and it was funded under the US MD-CARE Act. The managing editor was Professor Kate Bushby of Newcastle University in the UK.

About DMD

Duchenne muscular dystrophy (DMD) is one of the most common genetic disorders affecting children and young adults. It is a severe muscle wasting condition affecting 1 in 3,500 newborn boys worldwide, with onset in early childhood and the ensuing progressive muscle weakness and wasting leading to affected individuals becoming wheelchair bound by their early teens. Without treatment, the condition leads to death by the early twenties. DMD is caused by mutations in the DMD gene that lead to a failure to produce a functional muscle protein called dystrophin. Although several possible treatments are currently in clinical trial, there is currently no cure, but care interventions ranging from psychosocial, cardiac and nutritional care and steroid treatment to respiratory and orthopaedic interventions can have a dramatic effect on quality of life and life expectancy.

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Improvements to the regulatory affairs database

Recent months have seen more improvements to the TREAT-NMD Regulatory Affairs Database, which is a valuable source of advice to all those involved in the planning of mono- or multi-centre clinical trials. The "European regulations" and "International regulations" sections have been extended and a new section on "Austria" has been added, bringing the total number of countries for which information is available to 12.



The database contains detailed regulatory information relating to clinical trials, is open for public use, and can be accessed online. More than 1,500 users have visited the database for information about different national laws and legal procedures in the field of clinical trials.

To ensure the information provided is as accurate and current as possible a new update procedure has been developed. This means all web links are now checked weekly and country specific information is updated at least twice a year.

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5th European conference on rare diseases - call for abstracts

The European Conference on Rare Diseases is the unique platform / forum across all rare diseases, across all European countries, bringing together all stakeholders (academics, health care professionals, industry, policy makers, and patients' representatives).



The conference covers research, development of new treatments, health care, social care, information, public health and support at European, national and regional levels.

This is a biennial event, providing the state-of-the-art of the rare disease environment, monitoring and benchmarking initiative.

A call for poster abstracts has been made and the deadline is 31st December 2009. Further abstract submission details can be found on the conference website or [here](#).

Programme and registration information can be found on the website www.rare-diseases.eu

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Magazine about neuromuscular research published for families

The UK Muscular Dystrophy Campaign has published the second issue of Target Research - the annual magazine dedicated to neuromuscular research for patients, families and supporters. This issue includes features about genetic testing, exercise for muscle disease, drug discovery and clinical trials.



Other articles examine research news and issues from the UK and around the world, provide details of the research that the charity funds and more. You can download an [electronic copy](#) of the magazine or contact research@muscular-dystrophy.org for a hard copy.

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