4 September 2009
TREAT-NMD newsletter no. 61
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welcome

Welcome to our 61st Newsletter. We hope you enjoyed the summer break!

Following our joint press release with Prosensa earlier this week we describe in this newsletter how our registries and other resources can help companies with their planned clinical trials. We will also present these capabilities at the WMS Congress in Geneva next week.

TREAT-NMD is also partnering with other clinical networks and this has resulted in an agreement with CINRG to work together to further accelerate translational research into potential therapeutic options.

We'd like to remind you that the deadline for early registration to our International Conference is the 15th September. Please visit the conference website to take advantage of the early registration fee.

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

at a glance...

09-12 Sept 2009 IDMC-7 International Myotonic Dystrophy Consortium

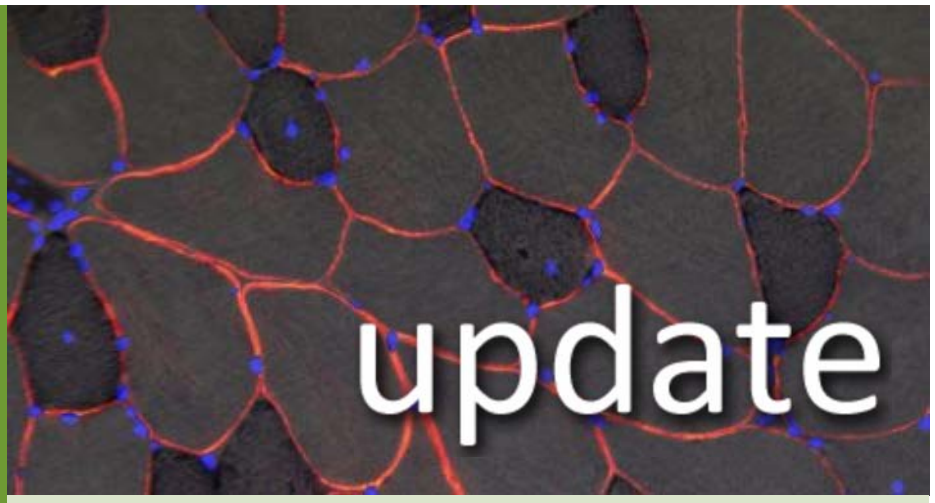
09-12 Sept 2009 14th International Congress of the World Muscle Society, Geneva, Switzerland

21-23 Sept 2009 Muscle Study Group Annual Meeting, Buffalo, New York

25-26 Sept 2009 SMA 'at the Eve of the Cure' conference, Warsaw, Poland

05-06 Oct 2009 6th UK SMA Research Conference, Edinburgh, UK

23-24 Oct 2009 Action Duchenne International Conference Holiday Inn Bloomsbury London



TREAT-NMD industry partnerships

One of the key aims of TREAT-NMD is to help the delivery of clinical trials to move forward more smoothly. Partnership with the pharmaceutical companies involved in the development of drugs aimed at neuromuscular diseases is therefore very important for the network.

The joint press release with Prosensa earlier this week highlighted the utility of our Global Patient Registry and Care and Trial Site Registry (CTSR). This work with Prosensa is just one example of TREAT-NMD's collaboration with industry as we assist them with the planning and development of their neuromuscular disease programmes. In addition to the registries, TREAT-NMD, as a Network of Excellence, is able to offer advisory and regulatory support, as well as a full clinical trial support service similar to that offered by Contract Research Organisations (CROs). This support is available to all companies planning neuromuscular trials with no exclusivity, to ensure that the entire field can benefit from the tools and expertise available within the network. Next week, at the annual World Muscle Society Congress in Geneva, network manager Stephen Lynn will be presenting TREAT-NMD's services and the advantages of partnering with us during a special Industry Forum on 10th September from 7-9pm. More information on the TREAT-NMD trial support infrastructure can be accessed through the new industry pages on our website (http://www.treat-nmd.eu/industry/).

The DMD registries now hold more than 9,000 individual patient entries with standardized items and patient consent, facilitating and accelerating clinical research and trials while giving patients improved access to relevant information on standards of diagnosis and care. These registries have been set up in collaboration with clinicians and patient organizations across the world and contain the key information needed to establish whether a particular patient might be eligible for a trial. The final decisions about where to run a trial and which patients to recruit will still be taken by the companies themselves, but TREAT-NMD's comprehensive and unbiased information provision speeds up this process, as well as allowing patients fairer access to clinical research.

The involvement of TREAT-NMD in the planning for the Prosensa trial has been to help identify potential clinical trial sites and those patients who might fulfil Prosensa's inclusion criteria for the study. At the moment the clinical trial is still in the planning phase and patient recruitment has not been initiated. Once recruitment begins, prospective patients will be contacted directly either by the national registry in which they have registered or by their clinicians. Further screening will be necessary to ensure they fulfil all the inclusion criteria for the study before they are recruited into the trial.

We are delighted that our registries have been used for the planning of the Prosensa trial and this is also great news for patients who can now begin to see the benefit of registering with their national registry. TREAT-NMD is also working with a number of other industry partners and we look forward to informing the community about new ways our services are helping bring benefit to patients sooner than would otherwise be possible.

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TREAT-NMD and CINRG sign collaborative agreement

TREAT-NMD and the Cooperative International Neuromuscular Research Group (CINRG) have signed a Memorandum of Understanding (MoU) that defines how we can enhance and support the existing efforts of each organization by identifying areas for collaboration. This recognises the focus of both

17-19 Nov 2009 TREAT-NMD / NIH International Conference

organisations on accelerating translational research into therapeutic options for the treatment and care of patients affected with neuromuscular disorders.

"Harmonization of therapeutic efforts for NMD must be done on a world-wide basis if rapid progress is to be made. This memorandum affirms the commitment of two of the largest clinical and research networks to work closely together towards the same shared goal: improve patients' lives.", quoted Eric Hoffman, Scientific Director of both CINRG and the NIH Wellstone Muscular Dystrophy Centre.

Katie Bushby, TREAT-NMD co-ordinator added ”collaboration between CINRG and TREAT-NMD is very important to ensure that the neuromuscular community can speak with one voice on important issues relating to trials. Over the past few years, we have collaborated on specific projects, including meetings on outcome measures and the submission of a proposal for a natural history study in dysferlinopathy to the Jain Foundation. This MoU will allow the groups to work together more effectively”.

CINRG is a clinical trial organization with 23 actively recruiting sites, and an 8 year history of conducting high quality clinical trials and natural history studies in muscular dystrophy (www.cinrgresearch.org). It is a non-profit research group that has predominantly focused on Duchenne muscular dystrophy research. It is the vision of CINRG to be preeminent in the advancement of knowledge and development of effective treatments for people with Duchenne muscular dystrophy and related disorders both nationally and internationally.

Further information regarding CINRG can be found in their latest newsletter available for download via this link here.

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TREAT-NMD Conference 2009: Deadline for early registration approaching!

You can still take advantage of the early registration fee for the upcoming conference in Brussels. The deadline for early registration is 15th September. If you need to register please visit the conference web site here.

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Launch of International Registry for CMD

Congenital muscular dystrophy (CMD) represents several rare forms of Muscular Dystrophy that cause muscle weakness at birth.
On August 14, 2009, Cure CMD launched the first and only CMD International Registry (CMDIR) to collect the contact information, disease information and health survey responses of patients with all forms of CMD. Families who participate in the registry will receive assistance from genetic counselors to answer questions. The Department of Human Genetics at Emory University School of Medicine will provide registry coordination, data review, and information and referral services to registrants. The goal of the CMDIR will be to increase physician awareness, facilitate diagnosis and connect affected individuals with research trials and future therapies. Given the rarity of congenital muscular dystrophy, it is vital that this initiative takes place on a global level. The CMDIR will connect with global gene-specific registry initiatives established by internationally focused organizations including TREAT-NMD.
"Having a patient registry for all people with a diagnosis of CMD is a huge step forward in trial readiness for these rare disorders. It will help to raise awareness and promote confidence in the field, and will allow patients and their families to feel connected to the research community. At TREAT-NMD we are delighted to be working with Cure CMD on this project," says Kate Bushby, one of the coordinators of TREAT-NMD.
"Until now, researchers have been reluctant to pursue congenital muscular dystrophy trials since there's been no systematic way to identify on a national or international level those children with the disease," says Dr. Anne Rutkowski, co-founder of Cure CMD and parent of a daughter with the disease. "Building a patient registry will enable us to create a critical mass to participate in clinical trials."

For further information please visit www.cmdir.org or www.curecmd.org.

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