19 June 2009
TREAT-NMD newsletter no. 57
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welcome

Welcome to our 57th newsletter!

This week's edition includes an article about our Governing Board Meeting in Paris earlier this week. With a very different format, this fifth meeting was far more interactive. The sunny weather also helped, with the relaxed atmosphere allowing people to connect with one another and share views about the network.

Also included is a reminder of the fast-approaching deadline for the submission of poster abstracts for our November conference. As there will be only one more newsletter before the deadline this is now the best time to submit an abstract via the website to ensure it is considered by our panel. Please also consider submitting a question to one of the expert panels that will be hosting a session in Brussels. This too can be done through our conference website.

A meeting report of the Myotonic Dystrophy workshop that was held in Naarden, Netherlands is also featured. This was a joint venture between TREAT-NMD and the Marigold Foundation of Canada.

The Academy of Sciences in Warsaw, Poland is hosting a two day conference entitled 'SMA at the Eve of the Cure' at the end of September, while the Muscle Study Group will be holding their annual meeting in Buffalo, New York between 21st and 23rd September. This year's focus is 'Fostering Pharma-Academic Partnerships in Neuromuscular Experimental Therapeutics'.

We hope, as always, that you enjoy our newsletters and find them of use.

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

at a glance...

TREAT-NMD Conference - submit your abstract!

We have been really delighted by the number and variety of abstract submissions received so far through the conference website. The November conference in Brussels has scheduled poster sessions throughout its three days and there is still time for anyone considering attending to submit an abstract for a poster presentation.

The plenary sessions in the conference will focus on key issues in translational research, allowing plenty of discussions around the challenges of moving into the clinic with new therapies for NMD. Poster abstracts are invited in several key areas that mirror the focus of the plenary sessions:

* Clinical Trial Design
* Definition of animal models of NMD
* Ethical and social issues around translational research in NMD
* High throughput strategies in NMD
* Outcome measure research and evaluation in NMD
* Preclinical studies in models of NMD
* Patient registries
* Standards of Care and Diagnosis in NMD
* Other

We are strongly encouraging all clinical and research groups engaged in the neuromuscular field to consider submitting an abstract on their work to help us ensure this is a highly interactive conference with cross-fertilization of ideas from all those involved in neuromuscular disease research.

The final deadline for submission of abstracts is Friday 17th July 2009. All abstracts will be reviewed by the programme committee, and applicants will be informed of the outcome of the review by the end of August 2009.

Further details of submission guidelines can be found on the conference website.

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TREAT-NMD / Marigold workshop lays the foundations for international patient registry for myotonic dystrophy

A workshop entitled Patient Registries and Trial Readiness in Myotonic Dystrophy, jointly sponsored by TREAT-NMD and the Marigold Foundation (Canada), was held from 12-14 June 2009 in Naarden, The Netherlands. The twenty-six participants represented eight countries and covered a broad cross-section of the myotonic dystrophy field: scientists, clinicians, patient representatives and industry. The workshop built on the foundations established in two previous ENMC workshops on myotonic dystrophy and the clinical DM working group set up by the Marigold Foundation, and took advantage of the tools developed within the TREAT-NMD network for patient registries and outcome measures.

In recent years the need to collect patient data in a harmonized manner across multiple countries has become increasingly evident. This is particularly true in the rare disease field, where locating patients suitable for a particular trial or therapy poses even more challenges. The first day of the workshop was therefore devoted to establishing consensus on a core dataset for a TREAT-NMD international patient

25-28 June 2009 PPMD Annual Connect Conference in Atlanta, Georgia.

09-11 Jul 2009 "Therapeutic Targets in CMD", Emory University, Atlanta, Georgia

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

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

registry for myotonic dystrophy. Presentations on the existing patient data collections held at various institutions across the world were combined with overviews from industry and patient organizations on their expectations from this initiative. Industry participants stressed the value of an international approach which gives them rapid access to the patients who might be eligible for their trials and allows them to make feasibility assessments with substantially less effort than before. Pivotal trials will always need to be multinational in order to gain marketing approval worldwide, and this again speaks to the need for data to be pooled in a single international resource. Patient representatives were keen that registries should provide two-way communication, that patients should have a say in the way registries are developed and that they should offer ongoing feedback and act as a conduit for reliable information to patients and families.

Presentations from PIs on their existing data collections revealed that substantial amounts of data have already been collected in separate initiatives worldwide, many of which have been in operation for a number of years and have therefore built up valuable longitudinal data about disease severity and progression. Discussions on the core items for the international registry focused on the need for this to be a more streamlined dataset in order to maximize patient enrolment and minimize PI workload. It was concluded that the "mandatory" items to be collected would include patient personal data, genetic mutation, and motor function, while "highly encouraged" items would capture a broader picture of the disease, including repeat count and myotonia, plus cardiac, respiratory, GI and CNS issues, and age at onset of symptoms. In line with the general TREAT-NMD approach, the international registry would comply with the the legal and ethical principles set out in the registry charter, including oversight, patient consent, annual updates and curation of the genetic data.

After reaching consensus on the international registry, the workshop switched its focus to outcome measures in myotonic dystrophy. Participants reported on a number of measures used in existing studies, ranging from myometry to cardiac measures to cognitive evaluations, and there was general agreement that owing to the multi-systemic nature of the disease, the relative lack of data about progression, and the small number of previous trials in myotonic dystrophy, there is a strong need for further work on this area to establish reliable, standardized and validated measures that will be accepted by the regulatory authorities. There was also consensus that more natural history studies are required in order to gain further data on disease progression, and this is an area in which participants felt useful collaborations could be established. The TREAT-NMD Registry of Outcome Measures was presented as a tool for systematizing the use and availability of outcome measures and a resource for all researchers to gain rapid access to information about appropriate measures.

Additional presentations focused on the challenges of outcome measures in the congenital and paediatric population, and the necessity of considering DM2 as separate from DM1 owing to its markedly different presentation. A detailed overview of DM1 genetics revealed that the underlying genetic variability and the challenges of somatic mosaicism and repeat instability are complicating factors that must not be overlooked when moving towards trials. Again, further research is needed in this area, and it is hoped that the data collected in the international patient registry and the collaborations established between researchers can form the basis for future progress. The workshop outcomes will also be presented at the upcoming IDMC-7 conference in Würzburg, Germany, in September this year, and a workshop report will be put forward for publication in Neuromuscular Disorders.

Participants:
Joanne Auld (TREAT-NMD, UK)
Guillaume Bassez (Paris, France)
Christophe Béroud (Montpellier, France)
* Karla Blonsky (Marigold Foundation, Canada)
Craig Campbell (London, Canada)
Giles Campion (Prosensa, The Netherlands)
Valerie Cwik (Muscular Dystrophy Association, USA)
Baziel van Engelen (Nijmegen, The Netherlands)
Bruno Eymard (Paris, France)
Bill Groh (Indiana, USA)
Ralf Krahe (Houston, USA)
* Hanns Lochmüller (Newcastle, UK)
Don MacKenzie (Marigold Foundation, Canada)
Robert MacLeod (Isis Pharmaceuticals, USA)
Giovanni Meola (Milan, Italy)
Bob Mattaliano (Genzyme, USA)
Darren Monckton (Glasgow, UK)
Christopher Pearson (Toronto, Canada)
Virginie Picard (Association Française contre les Myopathies, France)
Mark Rogers (Cardiff, UK)
* Benedikt Schoser (Munich, Germany)
Cheryl Swaby (Marigold Foundation, Canada)
Rachel Thompson (TREAT-NMD, UK)
Charles Thornton (Rochester, USA)
Bjarne Udd (Helsinki, Finland)
Lisa Vittek (Myotonic Dystrophy Foundation, USA)

* co-chairs

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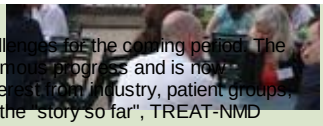
TREAT-NMD Governing Board reflects on network progress after 2.5 years

This June marks the half-way point for the TREAT-NMD network in its EU-funded form, and from June 15-17 Governing Board members met at a venue near Paris to reflect on the



network's achievements so far and identify the priorities and challenges for the coming period. The consensus of all those present was that network has made enormous progress and is now recognised as a global player in the neuromuscular field, with interest from industry, patient groups, clinicians and researchers worldwide. In an opening overview of the "story so far", TREAT-NMD coordinator Volker Straub made reference to this year's Annual Review of the network, performed by an external reviewer appointed by the European Commission, which commented that the network has exceeded expectations and highlighted the impact of the global collaborations established in areas such as standardised assessment of animal models and standards of care for patients with neuromuscular conditions. Volker also demonstrated the global reach of the network and the way it can be used to facilitate new collaborations, such as the successfully funded NMD-chip and BIO-NMD projects and a recent application to the EC's Directorate General for Health and Consumers for a new project called CARE-NMD, which if funded will implement consensus guidelines for DMD care across Europe, including Eastern Europe. The TREAT-NMD patient registries and care and trial sites network were also considered particular successes, with the more than 9000 patients in the global DMD registry and the detailed feasibility information from trial sites worldwide recognised as an enormously valuable resource for trial planning that in the end will ensure that trials and therapies reach patients more quickly. Many of the tools that have been developed for DMD and SMA are now being adopted by other disease groups, and one of the strengths of the achievements so far is the way they are now being mirrored in myotonic dystrophy, the congenital muscular dystrophies, nemaline myopathy and some of the limb girdle muscular dystrophies.

Notwithstanding the positive tone of the opening remarks, the intention of this meeting was to gain an honest appraisal of the network from all its partners and identify areas to focus on in future. It was recognised that ensuring the sustainability of an infrastructure such as TREAT-NMD remains a challenge, and so after the opening overview, the meeting took on a highly interactive form, with partners working in small groups to focus on the opportunities the network should take advantage of and the threats it must avoid. Participants provided a great deal of valuable and honest feedback that the network coordinators can now act on to ensure the network builds on its successes and becomes an enduring infrastructure for the neuromuscular field.

One of the highlights of this meeting was the presence of Cristina Csimma, chair of TACT, the TREAT-NMD Advisory Committee for Therapeutics. This committee will impartially evaluate the therapeutic development potential of drugs with preclinical data suggesting potential activity in a neuromuscular disease, thereby developing a matrix showing how far each drug is on the path towards human trials and helping TREAT-NMD prioritise trials to be run via the network, provide the background for preparing funding applications and investigational drug applications, and provide an unbiased appraisal to be published for the wider neuromuscular community. The committee comprises some of the leading experts in the preclinical, clinical and regulatory fields, and Cristina as chair brings a great deal of industry expertise not only to the committee itself but also to TREAT-NMD as a whole.

The coordinators would like to thank TREAT-NMD partner AFM for arranging such a beautiful meeting venue and all meeting participants for their commitment to the network and their positive and interactive approach to addressing TREAT-NMD's challenges and opportunities for the coming years.

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TREAT-NMD Conference - submit your questions!

Our conference flyer has now been completed and is available to download in electronic PDF format. The flyer provides details of not only the various interactive sessions that will be held throughout the conference but also lists the names of the various chairs and members associated with each session.

Sessions will be guided by submitted questions. This, we feel, will direct the dialogue in a direction that will address the concerns of the majority and so make the sessions as effective and fruitful as possible in the given time.

The involvement of National Institues of Health (NIH) as a co-sponsor of the conference has helped us attract delegates from both sides of the Atlantic.

If you have a question please feel free to submit it using this link to take you to our conference website. The flyer can be downloaded from the bottom of that page.

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SMA - Warsaw Conference

The Polish Academy of Sciences has arranged a two day conference focussing on SMA. Called 'Spinal Muscular Atrophy at the Eve of the Cure' this conference is scheduled for 25th and 26th September at the Academy of Sciences in Warsaw.

The invited speakers come from the USA, Spain, Italy, Germany, Turkey and of course, Poland. Sessions include looking at the history of SMA, Standards of Care for SMA patients, Home ventilation and also Standards of Diagnosis including Prenatal testing.

Irena Hausmanowa-Petrusewicz has had many years experience in organizing SMA conferences in Poland. Details of how to book your place and further conference details can be obtained through this email address.

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Muscle Study Group Annual Meeting in Buffalo, New York.

The Muscle Study Group (MSG) is holding its annual meeting near Buffalo, New York, USA on September 21, 22, and 23rd, 2009:

“Fostering Pharma-Academic Partnerships in Neuromuscular Experimental Therapeutics”.

The conference will bring together translational and clinical scientists from academia and pharma in a forum for discussion of current and novel therapies and draw speakers and participants from experts with an interest in developing clinical trials. The conference has a major focus on trainees and others interested in participating in the experimental therapeutics of neuromuscular disease.

This international conference will be held in an informal, isolated environment designed to expand the expertise and to mentor the career of beginning clinical/translational investigators. It will bring senior investigators, representatives of pharma, NIH program staff, and foundation representatives together with junior/trainee investigators.

Registration is limited to 100 individuals and is close to being full. If you are interested in viewing the agenda or registering please visit the MSG website using this link.

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