21 May 2010
TREAT-NMD newsletter no. 78
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welcome

Welcome to the latest TREAT-NMD newsletter.

This week's edition includes...

- a report on the newly launched CARE-NMD project

- an article about the DMD workshop that was held at EMA

- the launch of the BIO-NMD project website

- information about fellowship opportunities at the MRC Centre in London

- a report and literature from the EMA 10 years of orphan drugs conference

- a focus on TREAT-NMD member Progena

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...

12-15 Jun 2010 European Human Genetics Conference - Gothenburg, Sweden

13-15 May 2010 5th European Conference on Rare Diseases - Krakow, Poland

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

CARE-NMD project launched to work towards implementation of DMD care standards across Europe

CARE-NMD project for Duchenne muscular dystrophy funded under the EU's Second Programme of Community Action in the Field of Health with one million Euros.

The CARE-NMD project is led by the University Medical Center Freiburg and will help to improve treatment for Duchenne muscular dystrophy patients throughout Europe.

Duchenne muscular dystrophy or DMD is the most common of the muscular dystrophies, affecting approximately 1 in every 3,500 newborn boys. As Nick Catlin, father of a son with DMD explains: "DMD is a heartbreaking disease. Children begin to see their muscles waste away and families struggle to cope with the diagnosis and day-to-day management of this condition. Young men with DMD die early, in their late teens, or are left wheelchair-bound and unable to move unaided"

Although centres for the care of patients with neuromuscular disorders do exist in most European countries, many patients still do not receive treatment according to current guidelines and recommendations. This is particularly the case in Eastern Europe, where lack of information and lack of access to diagnostic and care expertise creates particular inequalities resulting in reduced life quality and life expectancy for DMD patients.

CARE-NMD (Dissemination and Implementation of the Standards of Care for Duchenne Muscular Dystrophy in Europe) was selected for funding by the Executive Agency for Health and Consumers (EAHC) out of 257 applications. The project spans Europe, with partner institutions and patient advocacy groups in Bulgaria, Denmark, Germany, United Kingdom, Poland, Hungary and Czech Republic (funded partners), as well as Croatia, France, Ireland, Macedonia, Netherlands, Belarus, Romania, Russia, Serbia, Slovakia, Sweden and Ukraine (collaborating partners).

Under the leadership of Dr. Janbernd Kirschner (Department of Neuropediatrics and Muscle Disorders at Freiburg University Medical Center, director Prof. Rudolf Korinthenberg), existing treatment standards and availability in these countries will be evaluated and improved through specific training sessions and other measures. "With a secured diagnosis and adequate treatment, children with DMD and their families can be helped to a far higher life expectancy and improved quality of life." explained Professor Kate Bushby of Newcastle University, United Kingdom. "Better availability of specialist care will also benefit patients with other forms of neuromuscular disease across Europe."

For more detailed information about the project see www.care-nmd.eu

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TREAT-NMD/EMA workshop report on the development of antisense oligonucleotide therapies for DMD published

The scientific journal Neuromuscular Disorders has published a paper reporting the proceedings of a workshop which discussed the issues surrounding the development of antisense oligonucleotide (AO) therapies for DMD. The meeting was held at the European Medicines Agency (EMA), London, U.K. on September 25th 2009 and brought together scientists, clinicians, patient representatives, industry and regulators from EMA and FDA. There were 40 active participants and nearly 100 delegates in attendance.

Muscular Dystrophy Annual Conference - Denver, Colorado, USA

24-27 Jun 2010 Families of SMA Annual Conference - Santa Clara, California, USA

15-16 Jul 2010 MDR2010

19-23 Jul 2010 12th International Congress on Neuromuscular Diseases - Naples, Italy

30 Jul - 1 Aug The Biennial FSHD International Patient and Researcher Network Meeting

9-10 Sept 2010 MD2010 - 4th conference for the Muscular Dystrophy Association of Western Australia - Perth, Western Australia

18 Sept 2010 Muscular Dystrophy Campaign Conference - Birmingham, UK

4-5 Oct 2010 7th UK SMA Researchers' Conference - Llanwyddyn, Wales, UK

12-16 Oct 2010 World Muscle Society International Congress - Kumamoto, Japan

12-13 Nov 2010 Action Duchenne 8th Annual Conference - London, UK

Please note: This is only a selection of upcoming meetings. To see all our listed meetings click here.

The meeting opened with a parent of a DMD boy presenting both the parent's and the teenage patient's perspective of living with DMD and illustrated their concern that the current clinical trial regulations require revising if AO therapy is to become a reality for DMD patients. The meeting then discussed the following themes: clinical trial regulations for AO use, clinical and biochemical outcome measures, toxicology and safety, ethics, inclusion of patients and parents in trial design, and natural history collection. The meeting was hailed a tremendous success in initiating a swift and sensible way forward.

The full paper can be downloaded from here.

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BIO-NMD website launch

BIO-NMD is a three year FP7 research project concentrating on the search for biomarkers in people with Duchenne and Becker muscular dystrophies and Collagen VI myopathies (including Ullrich congential muscular dystrophy and Bethlem myopathy).

Biomarkers are substances in the body that offer a way to measure normal or abnormal processes in the body. This means that processes associated with particular diseases can be measured and disease progression monitored. It also means that the effect of drugs or other therapies on disease progression can be evaluated.

The newly created website provides additional details of the project and details of participant institutions, people involved and their roles within the project along with various overseeing ethical boards.

In particular the BIO-NMD website now has a 'patient-friendly' section written in collaboration with the Patient Advisory Committee members from the Muscular Dystrophy Campaign, United Parents Projects and Telethon. The aim of the patient section is to explain what BIO-NMD is all about in lay-terms, to involve as many interested parties as possible, to provide contact details for the project and to ensure a mechanism for people to ask questions or provide feedback using an online form.

Additionally, project updates and events of interest to the wider community will be featured on the website. For example, the site currently features details of an industry session to be held during the next project meeting in London (July 8-9th, 2010).

For further details please visit the BIO-NMD website or contact BIO-NMD communications officer, Cathy Turner.

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Applications invited for hereditary neuropathy fellowships in London

The MRC Centre for Neuromuscular Diseases is the largest clinical and research centre in the UK for patients with diseases such as peripheral neuropathy. The peripheral nerve group in this centre, led by Dr. Mary Reilly, together with four US centres has been awarded a grant from the NIH to form a Rare Diseases Clinical Research Consortium to carry out research into hereditary neuropathies. We are pleased to announce as part of this consortium the establishment of 1 and 2 year neuromuscular fellowships specialising in hereditary neuropathies in our MRC Centre in the UK. These are exciting fellowships which will be available from July 2010 throughout a five year cycle. Applicants will be expected to be in the final year or have recently completed their SPR neurology training. It is desirable but not essential that the trainees have previously obtained an MD or PhD. For applicants, a track record of interest in hereditary neuropathies and previous publications will be looked upon favourably. An essential requirement will be the long term career aim to pursue research in hereditary neuropathies.

The two year fellowship will offer a broad training in all aspects of clinical research in inherited neuropathies as well as an opportunity to conduct a more detailed research project of the trainee's choice. The trainees in the 2 year programme will be based in the MRC Centre in the Institute of Neurology, Queen Square, London but will have the opportunity to train in other centres including Great Ormond Street Hospital (UK), the University of Pennsylvania School of Medicine, the Childrens Hospital, Michigan; the Children's Hospital of Philadelphia; Miami Institute of Human Genetics and University of Rochester School of Medicine (US). All UK trainees will be encouraged to spend 3 months in the US.

As this programme aims to attract the highest calibre applicants there is flexibility in the training programmes with can be individualised to the successful applicants needs including substituting a 1 year fellowship for the 2 year programme.

Interested applicants should email Dr. Mary ReillyDr. Mary Reilly for further details. Applicants should send a CV and covering letter by email to personnel@ion.ucl.ac.ukpersonnel@ion.ucl.ac.uk.

The closing date for applications is **Friday 4th June 2010**.

The posts are available from 1st July 2010 but can be started anytime up to the 1st October depending on the candidate. Starting salary on the CL8 scale in the range of £39,300 - £41,152 pa

(plus £2,795 London allowance) pa inclusive, superannuable.

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Two day conference marks ten years of orphan drug regulation in the EU

On 3 and 4 May 2010 the European Medicines Agency held a two day conference to mark the 10th anniversary of the Orphan Regulation in the European Union. The Agency brought together representatives from the European Parliament, the European Commission, International and European regulatory agencies, members of the Committee for Orphan Medicinal Products (COMP), patient groups, health professionals, and pharmaceutical industry to review the impact of ten years of orphan medicines legislation and to look ahead at future opportunities and challenges.

Professor Volker Straub, one of the coordinators of TREAT-NMD, participated in this meeting in which he chaired a workshop on the patient view of how the regulation has been implemented and what is still needed. This included discussions on the unmet needs for therapy development in the rare disease field which called for greater patient involvement and further development and support for patient registries.

The consensus between regulators, patient advocacy groups and academics was that patient registries should not be owned by industry and that there shouldn't be therapy-specific registries. Patient registries will in the future become more and more important for long term surveillance of patients on drugs and industry should ideally agree to work with registries not owned by them, even if that would mean that several companies make use of one and the same registry. The TREAT-NMD model was used as a positive example of this.

The meetings TREAT-NMD had organised with EMA were advocated by several organisations, such as Eurordis and the COMP, and it was pointed out that this form of interaction between the regulators and the stakeholders is extremely productive.

The press release from EMA about the meeting can be found here, and the presentations given during the meeting can be found here.

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Focus on Progena - Duchenne parent organisation in Switzerland and member of TREAT-NMD

Progena was founded in 2006 by the parents of a Duchenne boy. When they had to face this terrible fate, they didn't find much help at the existing infrastructures, whether they were public or private. This is how the idea came up to create a foundation to raise funds for the research against the rare genetic diseases of the Child and to bring help and support to the families of the affected children.

Progena also tries to collaborate with the various associations in Switzerland, in order to concentrate the efforts and aim into a common direction. This is not an easy task, as Switzerland is divided into 4 different language regions, with as many different ways of approach.

The aim of Progena is to gather the interests of all the affected people and to create a professional and efficient structure in terms of fund raising and support to the families. Since 2008, Progena has been working in a close relationship with the Swiss Telethon, which raises more than 2 million Swiss Francs each year, some weeks before Christmas. However, Progena wishes to extend the fund raising to medium sized and big corporations in order to obtain a sustainable funding over the whole year and not only at one specific period.

The local commitments of Progena

Parents of children who suffer from rare genetic diseases undergo huge changes in their everyday life. Parents need help with difficulties such as integration at school, logopedics, ergotherapy, physiotherapy, auxiliary means, nutrition, and adapting the household. To find answers, help and especially understanding concerning all these issues, Progena started meeting-evenings in 2008 for parents who have children who suffer from myopathy. These evenings serve as a forum where parents can meet and discuss with other parents. Occasionally, speakers are invited to share views with the parents (scientists, doctors, social workers...). Since then, 3 to 4 meetings are organised each year and they are very successful. Progena also runs a website where the latest information is published and updated regularly: www.progena.ch

And the International commitments

Due to the character and rare incidence of these diseases, it is absolutely essential to join forces and act across borders to find a common solution to fight and eventually treat these diseases. Progena

represents Switzerland amongst larger associations and organizations in Europe. Progena is hence a member of UPPMD – United Parents Projects Muscular Dystrophy – which brings together parents with children suffering from DMD. Progena is also member of TREAT-NMD, a network project created and financed by the European Commission in 2007. Its main objective is to establish therapeutic treatments and to accelerate the availability of medication for patients suffering from neuromuscular diseases. Progena will also help TREAT-NMD in translating brochures in French.

Projects

Clinical trials for treating rare genetic diseases are currently being set up both in Europe and the US. Through national registers, each country needs to ensure the participation of patients who could potentially benefit. In 2008, a national register for patients suffering from SMA and DMD/DMB was established in Switzerland under the supervision of Doctor Pierre-Yves Jeannet, Neurological Pediatrician at CHUV in Lausanne, and Doctor Andrea Klein, Neurological Pediatrician at the Children's Hospital in Zurich. From the outset and since, Progena has contributed financially to the Swiss Patient Register project, ensuring that Swiss patients participate in these crucial clinical trials in Europe and elsewhere. Progena not only finances this type of project but also raises awareness in order to access public funds. New projects are coming along in 2010 and are currently under evaluation.

Comity:
Robert Palm, President
r.palm@progena.ch
079 211 24 18
Huguette Palm, Secretary
h.palm@progena.ch
079 632 46 46

Address:
Rue du Temple 7
Case postale 45
1180 Rolle, Switzerland
contact@progena.ch
www.progena.ch

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