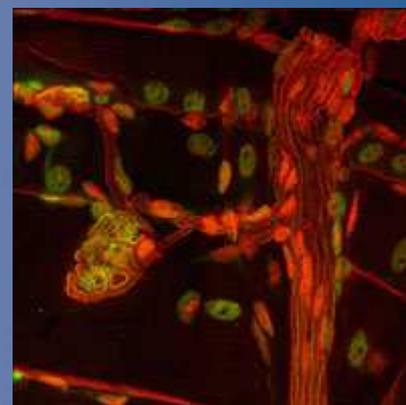




TREAT-NMD

Neuromuscular Network

30th November 2007 · Newsletter No. 22



Welcome to the latest newsletter. This edition features the preliminary results of the TREAT-NMD clinical trials coordination centre's feasibility questionnaire (page 2), plus some Marie Curie fellowship funding calls for 2008 (page 5).

Please forward any items that you would like to be included in future editions of the newsletter to info@treat-nmd.eu.

Best wishes,

Katie, Volker, Stephen, Emma, Arron and Rachel – the TREAT-NMD coordination team

Newsletter contents

About this newsletter	1
Working with us.....	1
TREAT-NMD news and reports.....	2
Other news.....	4
Upcoming meetings	4
Funding calls.....	5
Job and training opportunities	6

About this newsletter

This is a fortnightly newsletter sent to all members of TREAT-NMD's "Club of Interest" worldwide. Earlier editions of the newsletter can be found online at www.treat-nmd.eu/news/newsletter/index.htm. If you would like to subscribe directly, please visit our website at www.treat-nmd.eu/ where you will find a subscription form at the bottom of the homepage. You can also use the same form if you no longer wish to receive this newsletter – just select the unsubscribe button.

Working with us

TREAT-NMD aims to be an inclusive rather than an exclusive network, and you do not have to be based in Europe or be a partner to be involved. International collaboration with experts from all over the world is already taking place, and new links are being developed.

If you are involved in any of TREAT-NMD's areas of interest and have something you'd like to say or a suggestion of where we could work together, we encourage you to get in touch by writing to us at info@treat-nmd.eu. The coordination team in Newcastle will be happy to put you in touch with the person most relevant to your particular interest.

TREAT-NMD Clinical Trial Coordination Centre (CTCC) Feasibility Questionnaire Results

In September TREAT-NMD contacted 260 Clinicians and PIs across Europe, as identified by TREAT-NMD partners, and invited them to register their investigational site with the CTCC by completing the online Feasibility Questionnaire.

The aim of the questionnaire is to facilitate and accelerate clinical trial development by establishing a database to register potential investigational sites. The questionnaire is designed to collect information on the potential eligibility of centres to host a future clinical trial, rather than collecting feasibility information specific to a particular trial.

We had a promising initial response to the questionnaire, and preliminary results as of the end of October show that 68 sites covering 16 different countries have already registered. Regarding the identified patient population to date, we had a particularly good response from centres that have access to DMD patients, with 760 individuals aged 2-11 years being identified.

Using the results of the questionnaire we will:

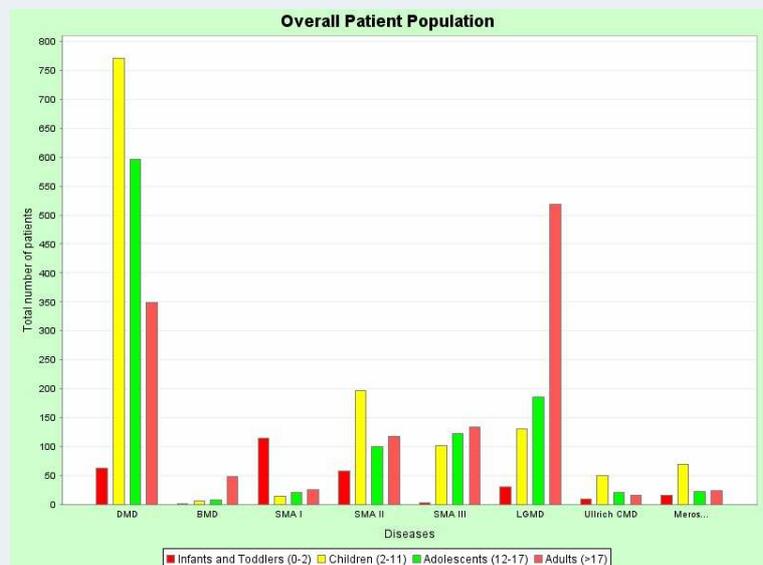
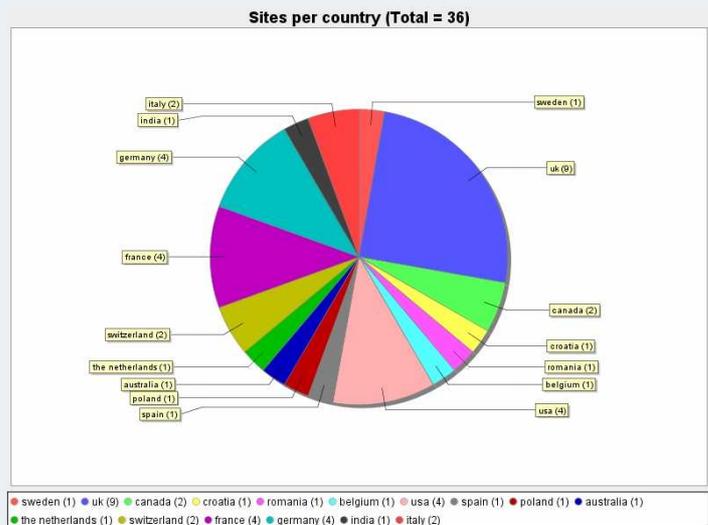
- Identify Clinical Trial Centres across Europe capable of performing Phase I, II and III Clinical Trials in DMD, BMD, SMA, LGMD and CMD
- Continue to engage and liaise with Industry
- Facilitate training and education workshops to promote harmonisation of trials across Europe
- Upload further details on the TREAT-NMD website.

Registration is currently ongoing and is open to everyone, so if your site is not currently registered with the CTCC and you would like to maximise your potential of being involved in future clinical trials then please click on the link below to complete the feasibility questionnaire:

<http://skl14e.ukl.uni-freiburg.de/eu.treatnmd.fq.web/register.jsf>

For more information please contact:

Emma Heslop emma.heslop@treat-nmd.eu or Angela Stanescu angela.stanescu@uniklinik-freiburg.de



MD-NET on its way to securing further funding

The German Muscular Dystrophy Network (MD-NET) was established in 2003 and since then has been funded by the German Ministry of Education and Research (BMBF) within a program for "networks of rare disorders". The MD-NET established a number of highly successful service structures such as a muscle cell biobank, specialized diagnostic services and a clinical trial coordination centre (<http://www.md-net.org>). Moreover, increased collaboration among scientists and clinicians resulted in more than 100 research publications. MD-NET is regarded as a model for successful networking in the rare disease field and is the partner within TREAT-NMD responsible for 2 important integrating activities, biobanks/patient registries and the clinical trial coordination centre. However, it was quite clear right from the start that networking the neuromuscular field in Germany with the aim of improving therapies and services for patients with muscle disease will not be fully accomplished within the initial funding period of 5 years. To our delight, the German Ministry of Education and Research has now allowed the MD-NET to apply for another 3-year period of funding. We are confident that with 5 years of successful networking experience and Drs Maggie Walter (Munich), Janbernd Kirschner (Freiburg) and Matthias Vorgerd (Bochum) as designated coordinators, the MD-NET is in a very good position to succeed in this bid. Further information on the MD-NET application can be obtained from uta.stucka@med.uni-muenchen.de.



Dubowitz Neuromuscular Centre on the move

The Dubowitz Neuromuscular Centre, originally established in 1975 at the Hammersmith Hospital in London and renamed in 1996 after the retirement of Professor Victor Dubowitz, will be moving to the Institute of Child Health / Great Ormond Street Hospital for Children, also in London, UK.

Professor Francesco Muntoni, the current director, explained that this is a planned move to consolidate the delivery of the increasingly complex clinical care to children with neuromuscular diseases, requiring multidisciplinary team input, and allow these children to benefit from future novel experimental therapies. The contiguity with the adult neuromuscular services based at the Institute of Neurology, Queen Square will also ensure appropriate transition of care into adult services.



From a research perspective the move, which will be an integral part of the recently established MRC Neuromuscular Translational Research Centre at Institute of Neurology and Institute of Child Health, of which Professor Muntoni is one of the principal investigators, together with colleagues from Newcastle, will facilitate the creation of one of the largest critical mass of neuromuscular investigators in Europe, focused on facilitating translational research in neuromuscular disorders across the entire age spectrum. Professor Muntoni is currently also the Principal Investigator of a Department of Health and a MRC grant focused on the safety and efficacy of locally and systemically administered antisense oligonucleotides in Duchenne muscular Dystrophy. He was also designated as the theme leader for "novel approaches to treating childhood diseases" in the recently established National Institute for Health Research Biomedical Research centre based at the Institute of Child Health / Great Ormond Street Hospital, with a designated paediatric clinical trial unit.

The move will involve the entire Muscle Centre, including the Nationally Commissioned Centre for Congenital Muscular Dystrophies and Congenital Myopathies, with its team of clinical consultants, specialist registrars and clinical research fellows, specialist muscle nurse and family care advisor, physiotherapists, speech and language therapist and dietician; the pathology team with the Consultant pathologist, clinical scientists and technicians; and the research team comprising some 20 scientists ranging from senior scientists with tenured positions, to postdoctoral fellows and PhD students, who will be accommodated in recently refurbished laboratories on the first floor of the Institute of Child Health.

There will be new job opportunities at the Dubowitz Neuromuscular Centre based at the Institute of Child Health. For further information please contact Francesco Muntoni (f.muntoni@ic.ac.uk).

Networks of Excellence seek permanence

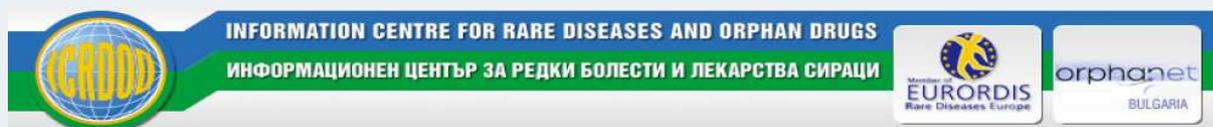
CORDIS, the European Commission's Research and Development Information Service, recently reported that a group of some 60 Networks of Excellence (NoEs), representing over 13 000 researchers, has issued an opinion paper calling on the European Commission to implement actions that would provide them with long-term sustainability and ultimately help them become permanent legal structures. Most NoEs were first created under the EU's Sixth Framework Programme in order to strengthen scientific and technological excellence in specific areas by integrating research capacities. Over 170 NoEs have been created to date. However, reduced funding for NoEs (only 17 NoEs were funded in the first call of FP7 versus 101 in FP6, and continued support for existing NoEs has not been announced) and difficulties in establishing permanent legal structures has prompted the drafting of the opinion paper. Four specific actions have been identified that would ensure more security for NoEs. These include an EC reconfirmation of its commitment to NoEs as tools that provide structure to European research and reduce fragmentation; the possibility of additional funding via a competitive process; a comprehensive review of the impact of NoEs as well as an analysis of long-term sustainability, perhaps leading to the creation of best practice guidelines; and the development of a "network of networks" that would result in new interdisciplinary areas of research. Amongst NoEs supporting the campaign for sustainability that are involved in rare disease research are EuroGentest, TREAT-NMD and Clinigene.

Commission Communication for rare diseases out for public consultation

The European Commission has drafted a Commission Communication on rare diseases. The document is out for public consultation which means that all European citizens and organisations can give their comments and views, which will be taken into account to make a final version of this document. A commission communication is not a legally binding document but this one makes clear that the European Commission considers rare diseases as an important issue in health care. It also gives the future directions for research and policy development in this field, and can therefore be of importance for TREAT-NMD as well.

If you want to read the document and / or submit your personnel comments on behalf of your organisation institute (by 31 January 2008 at the latest), by email to sanco-rarediseases-consultation@ec.europa.eu, you can find more details at the following link: http://ec.europa.eu/health-eu/news/raredis_cons/raredis_cons_en.htm

Upcoming meetings



Third Eastern European Conference on Rare Diseases and Orphan Drugs

"RARE DISEASES – PREVENTION, DIAGNOSIS, TREATMENT"

1-2 March 2008 - Plovdiv, Bulgaria

This event is a logical continuation of the very successful 1st and 2nd Eastern European Conferences on Rare Diseases and Orphan Drugs, organized in 2005 and 2006 (www.conf2005.raredis.org, www.conf2006.raredis.org). Participants from Bulgaria and Eastern Europe will present their achievements in the field of prevention, diagnosis and treatment of rare diseases with oral presentations and posters. The scientific committee of the conference is made up of opinion leaders with great experience in the field. During the conference, a specialized workshop with speakers from leading USA, European and Bulgarian organisations and institutions will be carried out, who will discuss the best practices in the field of rare diseases and orphan drugs access.

FP7: PEOPLE – MARIE CURIE**Indicative Calls for 2008****ACTIVITY 1: INITIAL TRAINING OF RESEARCHERS****1.1. Marie Curie Initial Training Networks**

Call identifier: FP7-PEOPLE-ITN-2008

Date of publication: 4 April 2008

Deadline: 2 September 2008 at 17.00 Brussels local time

Indicative budget: EUR 185 million

Funding scheme: Support for training and career development of researchers

ACTIVITY 2: LIFE-LONG TRAINING AND CAREER DEVELOPMENT**2.1 Marie Curie Intra-European Fellowships for Career Development**

Call identifier: FP7-PEOPLE-IEF-2008

Date of publication: 19 March 2008

Deadline: 19 August 2008 at 17.00 Brussels local time

Indicative budget: EUR 75 million

Funding scheme: Support for training and career development of researchers

2.2 Marie Curie European Re-Integration Grants

Call identifier: FP7-PEOPLE-ERG-2008

Date of publication: 30 November 2007

Deadline: Continuous submission with "cut-off" dates for evaluation of proposals received until then on 3 April 2008 and 8 October 2008, at 17.00 Brussels local time

Indicative budget: EUR 7 million

Funding scheme: Support for training and career development of researchers

2.3 Marie Curie Co-Funding of Regional, National and International Programmes

Call identifier: FP7-PEOPLE-2007-2-3-COFUND

Date of publication: 16 November 2007

Deadline: 13 March 2008 at 17.00 Brussels local time

Indicative budget: EUR 65 million

Funding scheme: Support for training and career development of researchers

Call identifier: FP7-PEOPLE-COFUND-2008

Date of publication: 19 November 2008

Deadline: 19 February 2009 at 17.00 Brussels local time

Funding scheme: Support for training and career development of researchers

ACTIVITY 3: INDUSTRY-ACADEMIA PARTNERSHIPS AND PATHWAYS**3.1 Marie Curie Industry-Academia Partnerships and Pathways**

Call identifier: FP7-PEOPLE-IAPP-2008

Date of publication: 30 November 2007

Deadline: 25 March 2008 at 17.00 Brussels local time

Indicative budget: EUR 45 million

Funding scheme: Support for training and career development of researchers

ACTIVITY 4: INTERNATIONAL DIMENSION**4.1 Marie Curie International Outgoing Fellowships for Career Development**

Call identifier: FP7-PEOPLE-IOF-2008

Date of publication: 19 March 2008

Deadline: 19 August 2008 at 17.00 Brussels local time

Indicative budget: EUR 25 million

Funding scheme: Support for training and career development of researchers

4.2 Marie Curie International Incoming Fellowships

Call identifier: FP7-PEOPLE-IIF-2008

Date of publication: 19 March 2008

Deadline: 19 August 2008 at 17.00 Brussels local time

Indicative budget: EUR 25 million

Funding scheme: Support for training and career development of researchers

4.3 Marie Curie International Reintegration Grants

Call identifier: FP7-PEOPLE-IRG-2008

Date of publication: 30 November 2007

Deadline: Continuous submission with "cut-off" dates for evaluation of proposals received until then on 3 April 2008 and 8 October 2008, at 17.00 Brussels local time

Indicative budget: EUR 17 million

Funding scheme: Support for training and career development of researchers

4.4 Marie Curie International Research Staff Exchange Scheme

Call identifier: FP7-PEOPLE-IRSES-2008

Date of publication: 30 November 2007

Deadline: 28 March 2008 at 17.00 Brussels local time

Indicative budget: EUR 25 million

Funding scheme: Support for training and career development of researchers

ACTIVITY 5: SPECIFIC ACTIONS**5.1 Marie Curie Researchers' Night**

Call identifier: FP7-PEOPLE-NIGHT-2008

Date of publication: 30 November 2007

Deadline: 5 March 2008 at 17.00 Brussels local time

Indicative budget: EUR 3 million

Funding scheme: Coordination and support actions (support)

Job and training opportunities

Postdoc / Senior Scientist – Helsinki, Finland

The Research Group for Nemaline myopathy and Other Neuromuscular Disorders, Helsinki, Finland offers a position for a Senior Scientist / Postdoc.

The group works in close collaboration with the ENMC International Consortium on Nemaline Myopathy whose research activities are co-ordinated by the Research Group Leader and Professor Nigel G. Laing in Perth, Australia. We are addressing questions related to the molecular biology, pathogenesis and clinical correlates of muscle disorders caused by mutations in genes for sarcomeric proteins. Our main focus is on nemaline myopathy and related disorders. For more information, see: http://www.helsinki.fi/haartman/lgo/english/research_groups/nemaline_myopathy_other_neuromuscular_disorders.htm

We are looking for an enthusiastic scientist experienced in cellular biology, molecular biology and protein methods. Knowledge of genetics, preferably in the field of human genetics, is also welcome, and, of course, familiarity with neuromuscular disorders would be an asset. The position offers autonomy in organising the laboratory activities of the research group, especially new projects within the above-mentioned fields.

The post is, in the first instance, 12-18 months, with the possibility of extension, and a negotiable salary, depending on qualifications.

The Research Group for Nemaline Myopathy and Other Neuromuscular Disorders is affiliated with the Folkhälsan Institute of Genetics and the Department of Medical Genetics, University of Helsinki, at Biomedicum Helsinki, Finland, which offer access to high quality research facilities and a multi-professional scientific environment. Finnish, Swedish and English are spoken within the group – you will do fine communicating with us in English. Helsinki, being the capital of Finland is rich in cultural activities, and Finnish nature offers adventures in the vast areas of the lake district, the archipelago with thousands of islands, and mountainous Lapland in the North. Some websites with more information about Finland are:

<http://virtual.finland.fi>

http://www.infopankki.fi/en-GB/welcome_to_finland/

http://www.incomesdata.co.uk/statistics/comp_price_level.htm

Applications should be addressed by 17 December 2007 to the Research Group Leader, Carina Wallgren-Pettersson, M.D., Ph.D.: carina.wallgren@helsinki.fi

Scientist in neuromuscular diseases

based in Liestal, Switzerland.

Santhera is a specialty pharmaceutical company focused on the discovery, development and marketing of small-molecule pharmaceutical products for the treatment of severe neuromuscular diseases in orphan indications. We currently have three compounds in five clinical developments programs. The most advanced program, SNT-MC17 for Friedreich's Ataxia, is under review for market approval in the European Union, Switzerland and Canada. Our shares are traded on the SWX Swiss Exchange (symbol: SANN).

To supplement our pharmacology group we are looking for a creative, top biochemist/cell biologist/in vivo pharmacologist. Previous expertise in neuromuscular diseases would be an advantage.

Job and training opportunities

In this new position you will

- Within the team, set up and fine tune models and then evaluate and compare the effects of compounds.
- identify innovative treatment strategies and identify pharmacological targets
- work independently in a team based small company environment

As an ideal candidate, you should have

- an education as a biochemist/cell biologist /in vivo pharmacologist
- expertise in histology, cell culture, ex vivo preparations, microscopy and biochemical and molecular biological methods (WB, ELISA, DNA cloning, RT-PCR, etc.) is expected
- worked with standard computer applications and ideally have experience in digital image analyses too
- a deep interest in the patho-physiology of the diseases and in pharmacotherapy
- fluency in English

PhD scientist in neuromuscular diseases

based in Liestal, Switzerland.

For our growing pharmacology group we are looking for a creative, top biochemist/cell biologist. In the course of your previous research in the field of neuromuscular development or disease you collected a broad expertise in histology, biochemistry and cell and molecular biology

In this new position you will

- set up models to evaluate effects of compounds
- find innovative treatment strategies and identify pharmacological targets
- work independently in a team based small company environment

As an ideal candidate, you should have

- a background as biochemist / cell biologist
- expertise in neuromuscular diseases
- a deep interest in the patho-physiology of the diseases and in therapeutic strategies
- In vivo experience and have been working with transgenic animals would be an advantage
- have worked as an independent researcher with a couple of first class publications
- fluency in English

<http://www.santhera.com/index.php?mid=33&vid=&lang=en>

Send us your news and views!

We strongly encourage all partners and supporters to send their own news and updates and we will be happy to include them in future editions of the newsletter. Please send your contributions to emma.heslop@treat-nmd.eu