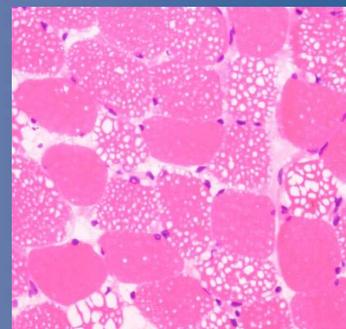




TREAT-NMD

Neuromuscular Network

7th March 2008 · Newsletter No. 28



Welcome to the latest newsletter. This edition features information about our new website section on DMD standards of care (see page 2), plus research positions at Newcastle University and IGBMC Strasbourg (page 4).

Specialists with an interest in clinical trials for neuromuscular diseases may be interested in the upcoming TREAT-NMD workshop on clinical trial development – see page 5.

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

Best wishes,

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

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Become a Member of TREAT-NMD

If you are interested in becoming a member of the TREAT-NMD Network please visit our web site to download our membership charter. An application form is also available for download. The web link to our Members' section is: http://www.treat-nmd.eu/news/item/?members_charter

We look forward to welcoming new members!

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.

DMD Standards of Care Interim Report – now available on TREAT-NMD website

The process of drawing up a comprehensive set of recommendations for standards of care in DMD on the basis of true international consensus is ongoing, under the auspices of the CDC Care Considerations project, and is likely to be complete in autumn 2008. In the intervening period, TREAT-NMD has produced a set of brief interim recommendations, which are now available for download on the TREAT-NMD website at

<http://www.treat-nmd.eu/soc/eng/dmd/>. The aim of these recommendations is to achieve the rapid dissemination of existing knowledge in this area while awaiting the full CDC document. This is a useful summary of expert opinion in this important area.



The screenshot shows the TREAT-NMD website interface. At the top, there is a logo for TREAT-NMD (Neuromuscular Network) and the tagline 'Accelerating treatments for neuromuscular diseases'. The main heading is 'Standards of care for Duchenne muscular dystrophy'. Below this, there is an 'Introduction' section explaining the process of drawing up a comprehensive set of recommendations for standards of care in DMD. A 'Download' button is visible on the right side of the page.

3rd European Conference for Rare Diseases – Conference Report

164 participants from 15 countries attended the 3rd Eastern European Conference on Rare Diseases and Orphan Drugs in Plovdiv, Bulgaria from the 1st – 2nd March.



3rd Eastern European Conference on Rare Diseases and Orphan Drugs
 "RARE DISEASES – PREVENTION, DIAGNOSIS, TREATMENT"
 1-2 March 2008 - Plovdiv - Bulgaria
 Under the auspices of the Bulgarian Ministry of Health

The Bulgarian Minister of Health Professor Rainov opened the conference and the Director of University Hospital in Plovdiv, Professor Batashki welcomed the delegates. Mr Tomovo for the National alliance of people with rare diseases in Bulgaria spoke on behalf of patients with rare diseases.

Invited speakers included representatives from the European Commission, the National Institute of Health, Orphanet, National Centre for Rare Disease Italy and Information Centre for Rare Disease Bulgaria. Several presentations focused on rare neuromuscular diseases, with Dr. Catherine Berens, from DG RESEARCH, providing an overview of the FP6 and FP7 Framework programmes. TREAT-NMD gave a brief overview of the network and an update on the network tools. Members of TREAT-NMD from the University Hospital Alexandrovska in Sofia, Bulgaria, gave an update on the diagnosis and care of neuromuscular disorders in Bulgaria highlighting their collaboration with TREAT-NMD, specifically related to developing patient registries for DMD and SMA and hosting the 38th Annual General meeting of EAMDA.

Representatives from industry were also present and medical students were given the opportunity to present their research results in oral and poster presentations.

The conference programme is available on the conference website: <http://conf2008.raredis.org/prgfirst.php>

Presentations will be available in due course on a conference CD which will be available from the TREAT-NMD Coordination Office (emma.heslop@ncl.ac.uk).

Meeting: **Synthesising qualitative research evidence on patients' views and experiences: methodological issues and utility**



Host: The Society for Social Medicine, in association with The Cochrane Qualitative Methods Review Group & The Cochrane Consumers and Communication Review Group, and The Centre for Health Sciences, Barts and The London School of Medicine and Dentistry



Date: 21 April 2008

Location: Queen Mary University of London, Mile End Campus, UK

Details: A one day meeting focused on the role, methods, challenges and limitations of synthesising experiences of health, illness and care

Contact: <http://www.ihse.qmul.ac.uk/chs/socsocmed/>

Workshop: **Peer review skills training**

Date: 23 April 2008

Location: Oxford, UK

Host: UK Cochrane Centre

Details: A one-day course for individuals who want to learn about peer review and are interested in systematic reviews.

Contact: For further information please see the Cochrane Collaboration

Website: <http://www.cochrane.org/news/workshops.htm>

E-mail: mcdowell@cochrane.co.uk



Course: **Introduction to Statistics for Clinical Trials**

Date: 6-7 October 2008



Course: **Introduction to the Design & Conduct of Clinical Trials**

Date: 8-10 October 2008

Host: The York Trials Unit, University of York

Details: The aim of our courses is to equip participants with the basic skills and knowledge for the design, conduct and analysis of randomised controlled trials. The courses should be of interest to nurses, doctors and allied health professionals who are interested in rigorous evaluation of healthcare interventions.

Contact: Course Administrator Sue Collins 01904321727

E-mail: sc27@york.ac.uk

MRC PhD Studentship at Newcastle University Translational Research in Muscle Disease



Reference Code: IHG33

Closing Date: 14th March 2008

Supervisor (s): Professor Kate Bushby, Professor Volker Straub, Professor Hanns Lochmueller, Professor Doug Turnbull, Professor Patrick Chinnery

Institute: Institute of Human Genetics

Duration: 4 years from September 2008

Provider: MRC

Person specification

Applicants should hold an upper-second-class or first-class degree in any area of biological science.

Value of the award and funding criteria

Due to funding criteria, this award is available to UK and other EU nationals only. The award covers fees and an annual stipend. Non-UK EU nationals must be resident in the UK for three years prior to the start of the studentship otherwise they will be eligible for a fees only award.

How to apply

To apply for the studentship please complete the University's online postgraduate application form (see <http://www.ncl.ac.uk/postgraduate/apply/>), quoting the reference number IHG33, attaching a CV and covering letter.

Further information

Please contact Professor Kate Bushby (kate.bushby@ncl.ac.uk) for further details of the studentship.

Post-doctoral positions on cellular mechanisms of neuromuscular disorders at IGBMC Strasbourg

Post-doctoral positions available in the department of Neurobiology and Genetics at IGBMC-Strasbourg to study "The molecular and cellular mechanisms underlying several neuromuscular disorders"

Our group has identified the myotubularin family of phosphoinositides phosphatases mutated in several neuromuscular disorders including centronuclear/myotubular myopathies and Charcot-Marie-Tooth peripheral neuropathies. The postdoctoral candidates will contribute to characterize the cellular roles of myotubularins, amphiphysin and dynamin on phosphoinositides and membrane trafficking processes and their dysregulation in diseases, using molecular and cellular biology and animal models (*C. elegans* and mouse).

Candidates must be highly motivated and have a PhD in molecular or cellular biology. Candidates should have a strong background in cell biology. Prior experience on membrane trafficking would be an advantage but is not required. French speaking is not a requirement.

The IGBMC is one of the leading European centres of biomedical research located in Strasbourg's science park, the Parc d'Innovation, Illkirch. We have excellent funding and core facilities, which include on-site transgenic services and in-house sequencing, antibody production, and confocal/electron microscopies together with bioinformatics, structural biology and microarray platforms. Scientists from more than 40 countries work at the IGBMC.

Strasbourg is an international and cosmopolitan city, with very nice countryside.

Two years salaries available.

Please send your application, curriculum vitae and 2-3 referees addresses by email to :

Dr. Jocelyn Laporte,

I.G.B.M.C., 1, rue Laurent Fries - B.P. 10142, 67404 ILLKIRCH - FRANCE

Phone : 33 (0)388653412

E-mail : jocelyn@igbmc.u-strasbg.fr <http://igbmc.fr/Laporte>
http://igbmc.fr/actualite/recrut/Post-docs/index_uk.html



EuroNews Report for the first Rare Disease Day on 29th February 2008

In the UK, Volker Straub, Katie Bushby and the team at Newcastle University were filmed and interviewed by EuroNews for a report on TREAT-NMD and the Newcastle Muscle Centre. This report was first broadcast on the 29th February and is now available on the EuroNews website (see the link below). It will also be made available for download on the TREAT-NMD website next week.

<http://www.euronews.net/index.php?page=futuris&lng=1>

<http://www.euronews.net/>

Workshops and meetings

CTCC Workshop

Clinical Trials in Neuromuscular Diseases

19th-21st June 2008

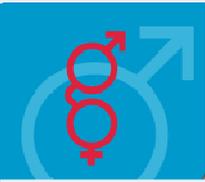
In this workshop on clinical trial development in the field of neuromuscular diseases, participants will learn how to develop a trial protocol from scratch. Lectures on study design, biostatistics, ICH-GCP and European regulatory requirements will provide basic information. Ideally, participants will apply with a concrete idea of a planned study. During hands-on sessions there will be enough time to develop a protocol synopsis during the workshop. Members of the TREAT-NMD CTCC and other experts will assist participants in all aspects of the protocol development. The workshop will be held on 19th-21st, June 2008 in Freiburg, Germany.



Programme http://www.treat-nmd.eu/assets/documents/Workshop-schedule_o80303.pdf

Flyer and registration Form: http://www.treat-nmd.eu/assets/documents/Flyer_o80229.pdf

For further information or to express an interest please contact: annette.pohl@uniklinik-freiburg.de



Genetic Interest Group Family route Map Launch

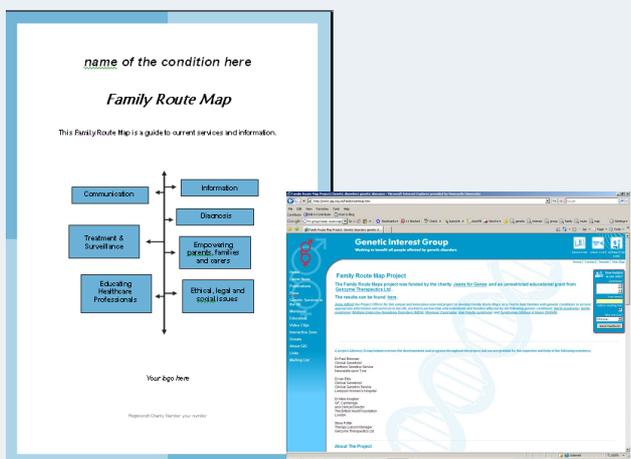
Wednesday 5th March saw the official launch of the Genetic Interest Group Family Route Map. A representative of TREAT-NMD attended the event held at the Wellcome Trust Conference Centre in London, UK. Other delegates included patients, patient support groups, clinicians and industry.

The launch gave the project workers a chance to share the main results from the research and to start to develop a dissemination plan for the 6 Family route maps developed as part of the initial project for the rare genetic diseases ([Multiple Endocrine Neoplasia Disorders](#), [Barth Syndrome](#), [Gorlin Syndrome](#), [Myotonic Dystrophy](#), [Nail Patella Syndrome](#) and [Syndromes without a name](#)).

GIG have secured funding, from the Department of Health, for a follow up project to ensure that the Route Maps are disseminated and to encourage participation of additional groups concerned with rare genetic disorders. TREAT-NMD is interested in working with GIG to develop this tool.

For more information or to download a copy of the Family Route Map Template please visit:

<http://www.gig.org.uk/familyroutemap.htm>



Marie Curie Actions Human Resources and Mobility Activity

MARIE CURIE Individual Fellowships

Intra-European Fellowships (IEF), International Incoming Fellowships (IIF) and International Outgoing Fellowships (IOF)

The next call for Marie Curie Individual Fellowships is expected to come out on 19 March, with an expected deadline of 19 August.

MARIE CURIE Initial Training Networks (ITN)

The next call for Marie Curie Initial Training Networks (ITN) is expected to come out on 4 April with an expected deadline of 2 September.



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