



TREAT-NMD

Neuromuscular Network



4th April 2008 · Newsletter No. 30

Welcome to the latest newsletter. This edition features information about some new updates to the TREAT-NMD website, a meeting report from the physiotherapists' training meeting held in Newcastle last week, and further updates on the Registry of Outcome Measures and the 2009 TREAT-NMD conference.

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

IMPORTANT MESSAGE TO OUR READERS!

We'd like to remind readers that receiving this newsletter does not automatically make you a member of TREAT-NMD. If you are interested in developing closer links with us, we encourage you to read our Members' Charter and complete the membership application form. These documents can be found on our web site at the address below.

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!

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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 / NIH International Conference, November 2009

In the last newsletter we reported on the international conference that TREAT-NMD will jointly organise with the NIH. It is with great pleasure that we can confirm that the conference will be held during the week beginning **16th November 2009**. We ask that you reserve this date in your diary and we hope that you can join us for this key event, which will bring together leading specialists from all over the world to share progress in the area of translational medicine in inherited neuromuscular diseases and set the future collaborative agenda.



The Programme Committee for the 2009 conference will plan and organise the conference agenda and the chair of this committee is Peter Streng of the ENMC. Please contact Peter for further information and sponsorship opportunities (streng@enmc.org).

Registry of Outcome Measures—update

The TREAT-NMD ROM is an on-line database that will benefit the wider NMD research community. It will be a ready reference that provides important base information about existing outcome measures and clear directions to key points of contact and comprehensive sources of information.

Below you will find an updated list of registrations in relation to ROM database population, if you would like to contribute to this register please contact Joanne Auld directly at Joanne.Auld@iop.kcl.ac.uk

| Name of Outcome Measure | Information Supplied By |
|---|--|
| ACTIVLIM: a measure of activity limitations | Peter Van den Bergh |
| Ankle dynamometer | Catherine Champseix, Institut de Myologie |
| Arm Activity Measure (ArMA) | Stephen Ashford, Northwick Park Hospital |
| Balance Quality Tester: an intelligent scale for the assessment of static and dynamic balance | Catherine Champseix, Institut de Myologie |
| Elaps: High Spatial Resolution Surface EMG | Catherine Champseix, Institut de Myologie |
| GMFM | Freiburg Group |
| Goal attainment scale | Freiburg Group |
| Hammersmith Functional Motor Scale for Spinal Muscular Atrophy (HFMS) | Dr Anna Mayhew, ICH, UCL |
| Handheld myometry with the Citec myometer | Freiburg Group |
| KINDL quality of life questionnaire | Freiburg Group |
| Locometrix: Gait analysis of neuromuscular patients using accelerometry | Catherine Champseix, Institut de Myologie |
| MMT for Duchene Muscular Dystrophy; Brooke 1981 (MMT DMD Brooke 1981) | Dr Michael Rose, King's College Hospital |
| MMT for Facioscapulohumeral Dystrophy; Personius 1994 (MMT FSH Personius 1994) | Dr Michael Rose, King's College Hospital |
| MMT for Inclusion Body Myositis; Muscle Study Group 2001 (MMT IBM MSG 2001) | Dr Michael Rose, King's College Hospital |
| Motor Function Measure (MFM) | Carole Berard |
| MRC | Maggie Walter and Sarah Baumeister |
| MyoGrip : Precision grip | Catherine Champseix, Institut de Myologie |
| MyoStret: Home made QMT system | Catherine Champseix, Institut de Myologie |
| MyoTone test: a method for the assessment of myotonia | Catherine Champseix, Institut de Myologie |
| Neuromuscular excitability assessment | Catherine Champseix, Institut de Myologie |
| North Star Ambulatory Assessment (NSAA) | Dr Michelle Eagle, University of Newcastle |
| Personal acceleration and energy recorder | Catherine Champseix, Institut de Myologie |
| Quantitative force measurement by Hand-held dynamometry (Citec) | Maggie Walter and Sarah Baumeister |
| Quantitative force measurement by torque measurement (QSM, M3diagnos) | Maggie Walter and Sarah Baumeister |
| Strength measurements with Biodex | Catherine Champseix, Institut de Myologie |
| TaToo: Tremor Assessment Tool | Catherine Champseix, Institut de Myologie |
| Wrist dynamometer: | Catherine Champseix, Institut de Myologie |



Additional information on the ROM is available via the TREAT-NMD website and update information will be available soon: <http://www.treat-nmd.eu/rom/>

Website news

TREAT-NMD first year update

TREAT-NMD has now been running for just over a year and we have updated the TREAT-NMD website with an overview of our achievements in key areas during 2007. From the enormous progress that has been made in our global patient registries initiative to the standard operating procedures being established for a number of animal models, the international consensus being reached in standards of patient care, and the steps the network is taking towards trial readiness across Europe, the network has made significant progress towards its goal of improving research collaboration and advancing patient care across Europe. Read more on the website:

<http://www.treat-nmd.eu/yearone/>



Working with pharmaceutical companies to accelerate treatments

A close relationship between patients, the academic world and the world of industry is crucial in order to ensure that promising new ideas at the research stage become useful functioning therapies. TREAT-NMD is developing both the research tools and the advisory service that pharmaceutical companies need to move forward quickly in this specialised field, and we have updated our website to provide a clearer overview of the services we offer.

See <http://www.treat-nmd.eu/industry/> for details.

Other news

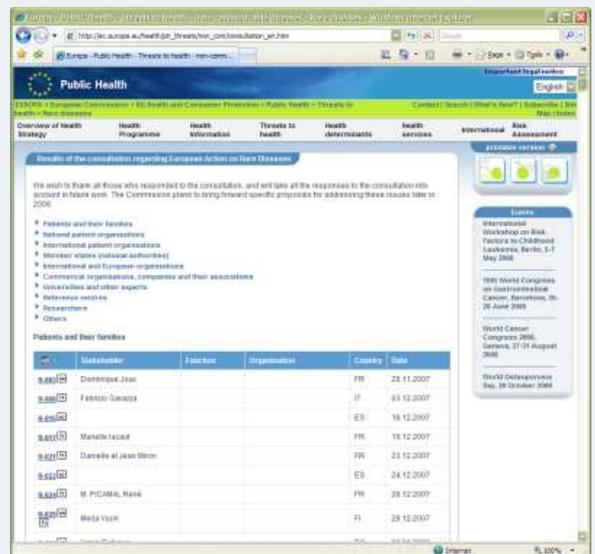
Over 600 Responses to the Public Consultation on European Action in Rare Diseases!

The public consultation regarding European action in the field of rare diseases launched by the European Commission (DG SANCO) received over 600 responses (including of course one from TREAT-NMD), which is a record response to a public consultation on health. The responses have now been posted online and these can be accessed at the address at the end of this article.

An update to the Communication will be presented in May 2008 at the Eurordis Annual Membership Meeting in Copenhagen, Denmark. A final version of the Communication, based on the input received from the public, will be presented to the Council of Ministers and to the European Parliament in June, and voted on in late November 2008.

The Communication on Rare Diseases is great news for the rare disease community as it will shape the future strategy of Community action in the field of rare diseases for healthcare, research and therapy development.

For further information see http://ec.europa.eu/health/ph_threats/non_com/consultation_en.htm



TREAT-NMD Outcome Measures Meeting

28th March 2008, 12.30- 3pm. Newcastle, UK.

Purpose of the Meeting

This meeting was convened due to the unique opportunity provided by the PTC clinical evaluator meeting held in Newcastle between 26th and 28th March 2008,



supported by TREAT-NMD. Physiotherapists from several countries including UK, France, Germany, Spain, Italy, Sweden, Australia, USA, Belgium, Israel, and Canada were attending a meeting to be trained in the clinical evaluation of participants in the PTC 124 2b trial. The aim of the outcome measures meeting was to provide a forum for discussion about outcome measures in NMD. In particular how could we as physiotherapists contribute to the global neuromuscular community and move forward to develop a collective and harmonised approach to clinical evaluation.

The first hour of the meeting consisted of presentations given by Julaine Florence PT, DPT, Director Clinical Studies, Associate Professor, Dept. Neurology, Washington University, Elaine Scott MSc MCSP MDC research physiotherapist, TREAT-NMD implementation officer, Erik Henriksen RA UC Davis Physical medicine, Dr Anna Mayhew PhD MCSP Physiotherapy Co-ordinator Smartnet UK and Dr Michelle Eagle PhD MSc MCSP, Physiotherapist Newcastle Muscle Centre. Julaine explored the role of the clinical evaluator, Elaine described the role and purpose of TREAT-NMD particularly relating to work packages Wp9.1 and 9.2, Erik described a complex natural history study in DMD, Anna discussed her work developing a modular assessment toolkit for SMA and Michelle introduced the discussion by outlining what the community has available and what is required to move forward so that we have an internationally unified approach to outcome measures.

Outcomes

The discussion was wide ranging and thought provoking. There was a tremendous enthusiasm to work together and to use the TREAT-NMD network to facilitate collaboration and to develop a concrete plan to move forward. The recommendations from the participants included using the TREAT-NMD outcome measures web pages to hold *standardised operational definitions* of an agreed set of clinical end points or clinical milestones such as loss of ambulation or loss of ability to feed oneself. In addition it was suggested to collate a library of clinical evaluations, complimenting the ROM work package which would include manuals of operations, suggested potential applications of the assessment, video training material, clinical record sheets and clinical evaluator training opportunities. The material would be collectively available via the dedicated TREAT-NMD web pages with the overall purpose being to facilitate the development of a core set of data collection points using standardised techniques with *standardised operational procedures* which need not be the same across sites but which could be utilised as appropriate. This in turn would facilitate further collaboration between countries and sites for example in the development of natural history studies.

There is much more work to be done but the meeting was considered a great success and a very useful starting point.

Attendees:

Meeting report by
Michelle Eagle

| | | | |
|------------------|-------------------|--------------------|------------------|
| Michelle Eagle | Lisa Berglund | Allan Glazman | Ed Gappmaier |
| Julaine Florence | Anna Mayhew | Elena Mazzone | Erik Henricson |
| Emma Heslop | Maria Gonzalez | Cheryl Scholtes | Valerie Decostre |
| Katy de Valle | Francesca Magri | Shree Pandya | Gwen Oliver |
| Meredith James | Suzanne James | Anna Marie Bonetti | Elaine Scott |
| Kristy Rose | Michelle McCallum | Annamaria Kofler | Stephanie Wicks |
| Pernilla Kipping | Marion Main | Adeline Carlesi | Birgit Steffen |



PTC124 Clinical Investigators and Principal Investigators European Meeting

Newcastle, UK 26th 30th March 2008

Last week an important meeting in the development of treatments for Duchenne Muscular Dystrophy was held in Newcastle as PTC Therapeutics hosted their PTC124 European Clinical Investigator and Principal Investigator meeting. 17 European sites are involved in this study, several of which are TREAT-NMD partner sites. Information on patient recruitment at these sites will be available soon via the patient pages of the TREAT-NMD website.

To see the full list of trial sites and for additional information please visit the PTC website: http://www.ptcbio.com/6.1.1_ptc124_trials.aspx

Patients, Families & Advocacy Groups may contact Diane Goetz (Director, Patient and Professional Advocacy) at PatientInfo@ptcbio.com for further information.

| Country | Physician Name | Institution |
|---------|--------------------|---|
| Belgium | Goemans, Nathalie | University Hospital KU , Leuven |
| France | Voit, Thomas | Groupe Hospitalier La Pitie-Salpetriere, Institut de Myologie, Paris. |
| Germany | Kirschner, Jan | University Hospital, Freiburg. |
| Italy | Bertini, Enrico | Unità di Medicina Molecolare, Dipartimento dei Laboratori, Ospedale Pediatrico Bambino Gesù , Rome. |
| Italy | Mercuri, Eugenio | Dipartimento di Scienze Pediatriche Medico-chirurgiche e Neuroscienze dello Sviluppo, U.O. Complessa di Neuropsichiatria Infantile, Policlinico Agostino Gemelli, Rome. |
| Sweden | Sejersen, Thomas | Neuropediatric unit, Astrid Lindgren Pediatric Hospital, Barnsjukhus Kaolinkska Sjukhuset, Stockholm. |
| UK | Bushby, Kate | University of Newcastle, Institute of Human Genetics, Newcastle upon Tyne. |
| UK | Muntoni, Francesco | Imperial College London, Department of Paediatrics, Hammersmith Hospital, London |

Physiotherapy Department
Paediatric Neuromuscular
Physiotherapist Advanced
Band 7.



0.5 WTE 18.75 hours per week

Salary £28,313 - £37,326 pro rata

Children's Services, John Radcliffe Hospital,
 Oxford, UK.

We are seeking a highly motivated advanced paediatric physiotherapist with a special interest in Paediatric Neurology at the Oxford Children's Hospital, John Radcliffe Hospital in Oxford. This new post is designed to support the expanding paediatric neurological service especially in the field of Mitochondrial diseases. There will be an emphasis on outpatient work and joint specialist clinics, which may, at times include older patients.

There will be a strong emphasis on multiprofessional working.

An effective team player, you must have proven experience of Children's Acute Neurology (at a senior level) Evidence of postgraduate training in neurology is essential. In conjunction with the Principal Physiotherapist you will assist in appraising, developing and evaluating the service and significantly contribute to the delivery of the service. This will include hospital on call, twilight and weekend working.

We are committed to CPD and will support your development in this role.

For further information regarding this vacancy please contact : Erica Woodward Principal Physiotherapist as soon as possible, on 01865 221532 or 01865 -741166 bleep 1759.

All current vacancies are advertised on the webpage: <http://www.treat-nmd.eu/jobs.htm>

CTCC Workshop

Clinical Trials in Neuromuscular Diseases

19th-21st June 2008

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

Myotonic Dystrophy Support Group Annual Conference

The Myotonic Dystrophy Support Group Annual Conference will be held at the Holiday Inn, Telford on 10th May. Sessions include genetics, congenital myotonic dystrophy, looking after yourself, making music with therapeutic drumming, who will look after my children when I am not able to, and help for the heart. Come along for good conversation with other families. For further information see www.mdsguk.org



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