Welcome to the latest newsletter. This edition features two meeting reports, one from the ENMC International Workshop on Patient registries for rare inherited muscular disorders and a second on the Inaugural Scientific Meeting of the new MRC Centre, London.

We are also pleased to inform you about our updated registry pages which contain important information for patients, clinicians and researchers.

Don't forget rare disease day on the 29th February!

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

---

**Newsletter contents**

- About this newsletter..............................1
- Working with us.................................1
- TREAT-NMD news and reports.............2
- Other news........................................4
- Meetings and workshops....................4
- Funding calls......................................5
- Website update....................................5
- Tools.....................................................6
- Partner-specific items..........................6

---

**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](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/newsletter/index.htm](http://www.treat-nmd.eu/newsletter/index.htm). If you would like to subscribe directly, please visit our website at [www.treat-nmd.eu](http://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.
157th ENMC International Workshop on “Patient registries for rare, inherited muscular disorders”

25-27 January 2008, Naarden, the Netherlands

At the end of January, the European Neuromuscular Centre (ENMC), in collaboration with TREAT-NMD partners, hosted a group of experts in Naarden for a discussion on patient registries for rare, inherited muscular disorders. Patients’ registries represent crucial start-ups for new therapeutic strategies for neuromuscular diseases, as well as to answer research questions such as the prevalence of neuromuscular disorders in Europe and support other activities such as assessing standards of care. A major goal of the integrating activity within TREAT-NMD is the introduction of European patient databases for DMD, SMA and other muscular dystrophies, and harmonized TREAT-NMD global registries for DMD and SMA have been already set up, together with a Charter for Treat-NMD patient database/registries. A “toolkit” applicable to registry development in other diseases including a discussion on the selection of mandatory items, ethical and confidentiality issues, as well as the Charter for data sharing is available on the TREAT-NMD website at http://www.treat-nmd.eu/registries/index.htm

The ENMC workshop aimed to create a collaborative action towards the set up, development and harmonization of patient registries for additional inherited neuromuscular disorders, both in Europe and world-wide. This workshop provided an excellent development of the work of TREAT-NMD and ENMC into rare muscle diseases towards clinical trial readiness, complementing the ongoing TREAT-NMD activities for DMD and SMA registries. The workshop was attended by 20 participants from 7 European countries (Finland, France, Germany, The Netherlands, Spain, Switzerland, United Kingdom) and from the U.S.A. Participants included bioinformatics experts, geneticists, neurologists, paediatricians, representatives of patient advocacy organizations, as well as members of the TREAT-NMD and ENMC. The discussion was focused on existing databases and registries, ranging from laboratory and clinical based databases to patient led initiatives, relating to several types of congenital muscular dystrophy and myopathy, limb girdle muscular dystrophies, oculopharyngeal muscular dystrophy, myofibrillar myopathies, Pompe Disease, facioscapulohumeral muscular dystrophy and myotonic dystrophy. Technical and legal issues, objectives and requirements for each registry, development and agreement on mandatory items across genes and diseases, agreement on best practices and further plans for collaboration were discussed as well. Agreement was reached on the importance of European and global harmonized and coordinated patient registries for accelerating progress, in particular in translational research. This will be facilitated by action taken by individual, disease-specific ENMC workshops and consortia, but also through joint action across diseases. This includes a web-based inventory of patient registries to be published, harmonization of registration practices and registry content, increased collaboration with patient organizations and empowerment of patients, and sharing of resources and dissemination through ENMC and TREAT-NMD.

The participants of the workshop wish to invite our partners and additional representatives for rare, inherited muscle disorders to get involved and form a consortium for further work in this area. Additional inputs are welcome! A full report will be soon published in Neuromuscular Disorders and will be complemented by an inventory of the registries available. Further details will be released in the TREAT-NMD website.
Inaugural Scientific Meeting of the new MRC Centre for Translational Research in Neuromuscular diseases.

1 and 2 February 2008, London

Centre Director Professor Michael G Hanna
Centre Deputy Directors Professor KM Bushby and Professor Martin Koltzenburg

There was an exciting boost for therapy development in nerve and muscle disorders in the UK with over 330 delegates attending the inaugural scientific meeting of the first MRC funded centre for translational research in neuromuscular disease. The meeting was jointly hosted between by the MRC Centre and the Muscular Dystrophy Campaign. This new centre aims to bring together clinicians, scientists, patient organizations and patients in order to advance UK translational research in neuromuscular disease. The centre aims to work with all its partners to support the development of a trials culture for patients with neuromuscular diseases.

The MRC Centre is a joint partnership between the Institute of Neurology UCL, the Institute of Child Health, UCL and the University of Newcastle-upon-Tyne. There are close links to its partner NHS organizations, University College London Hospitals NHS Foundation Trust, Great Ormond Street Hospital for Children and Newcastle Upon Tyne Hospitals NHS Foundation Trust. Several TREAT-NMD partners are involved with the MRC centre, with Francesco Muntoni of the Institute of Child Health and Kate Bushby, Volker Straub and Hanns Lochmueller being integrally involved and many TREAT-NMD partners including Kay Davies, Serge Braun, Thomas Voit and representatives from FTELE, Santhera, ENMC and Summit attending the meeting.

Over the two days a programme of international speakers delivered state-of-the art lectures in the basic science foundations of translational research and in relation to the tools required to deliver translational research. Over 60 high quality posters were presented. There was also an important session which explored how the MRC Centre can contribute to developing UK patient/expert clinician networks and to TREAT-NMD. It is these networks that are critical to the delivery of effective translational research and clinical trials, and this funding from the MRC is an important recognition of the importance of this field by the major funding body in the UK.

For further information on the centre please contact:
http://www.centre-london.mrc.ac.uk/

Read MDC Press release:
http://www.muscular-dystrophy.org/news/mdc_hosts_1.html
CTCC Workshop

Clinical Trials in Neuromuscular Diseases

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, GCP-ICH 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 and registration is open to all.


Online-Registration Form: Coming Soon

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

Friday 29th February 2008 is Rare disease day

We would like to encourage partners and members to get involved! For more information on how to do this in your country please visit the rare disease website http://www.rarediseaseday.org/

In the UK, Professor Volker Straub and the team at Newcastle University were filmed and interviewed by EuroNews for a report on TREAT-NMD to be broadcast on the 29th February 2008. This report will be translated into 7 European languages and will be available on the EuroNews website and the TREAT-NMD website next week.

http://www.euronews.net/
FP7 HEALTH – Early draft work programme 2009

2. TRANSLATING RESEARCH FOR HUMAN HEALTH

2.4 TRANSLATIONAL RESEARCH IN OTHER MAJOR DISEASES

2.4.4 Rare diseases

- Rare neurological diseases
- Preclinical development of substances with a clear potential as orphan drugs

The 3rd call under the FP7 HEALTH theme is expected to be published in summer 2008 with a deadline in the autumn. It has been indicated that Translational Research in Rare Diseases will be one of the topics. **We would therefore like to encourage partners and members to consider applying under this topic!**

For further discussion or information please contact the TREAT-NMD coordination team at info@treat-nmd.eu

TREAT-NMD Website Update

**Update on patient registries - new web section online**

Since a number of TREAT-NMD national registries are coming online in the near future, the TREAT-NMD web pages on the patient registries have undergone a major update, and lots more information is now available online. If you are interested in the patient registries initiative, we encourage you to visit the new site section at http://www.treat-nmd.eu/registries/index.htm where you will find more details about the individual national registries that make up the TREAT-NMD global registry.
MFM launched its web site: [http://www.mfm-nmd.org/home.aspx](http://www.mfm-nmd.org/home.aspx)

**Motor Function Measure (MFM)** is a quantitative scale that makes it possible to measure the functional motor abilities of a person affected by a neuromuscular disease.

Whatever the diagnosis and the extent of motor deficiencies, MFM allows us to:

- **specify** the symptomatology and the evolution of neuromuscular diseases
- **objectivize** the repercussion of the therapeutic measures
- **direct** the rehabilitation and adaptation measures
- **facilitate** communication between the various persons in charge of care
- **select** homogeneous groups of patients in view of therapeutic trials

MFM is to date the only validated and published function measurement scale usable in the majority of neuromuscular diseases, among patients from 6 to 60 years old.

For further information see: [http://tinyurl.com/272gzq](http://tinyurl.com/272gzq)

At the MFM site you can find contact information for different countries and proposals for training or involvement in validation studies.

For further information please contact: HLAOUENAN@afm.genethon.fr

---

WMS2008 Abstract submission and Registration reminder:

Please submit **abstracts** for the 13th International WMS congress, in Newcastle before the end of March.

Please **register** for the conference before the end of April.


---

Summer 2008 Governing Board Meeting:

The next GB meeting will be held in Newcastle. We encourage all partners to reply to Stephen.Lynn@ncl.ac.uk regarding their attendance as soon as possible.

**The Coordination Team looks forward to welcoming everyone to Newcastle!!!**

---

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