



## TREAT-NMD Partner Newsletter No. 12 and Club of Interest Newsletter No. 6

25<sup>th</sup> May 2007

Welcome to the sixth newsletter for the TREAT-NMD Club of Interest. This week's edition features a report from the recent EURORDIS membership meeting and a valuable training opportunity in myoblast cultures.

We hope you enjoy the newsletter and look forward to hearing your comments - write to [info@treat-nmd.eu](mailto:info@treat-nmd.eu) with anything you'd like to say. Feel free to forward this message to anybody you think might find it of interest, or invite them to sign up to receive the newsletter by visiting our website.

Best wishes,

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

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## 1. About this newsletter

This is a weekly newsletter sent to all members of TREAT-NMD's "Club of Interest" worldwide. We are receiving new subscriptions all the time, so if you've missed the earlier editions of the newsletter and would like to catch up, please visit our newsletter archive online at <http://www.treat-nmd.eu/news/newsletter/> where you will find all back-issues. If you have received this letter from a friend or colleague and would like to subscribe directly, please visit our website at <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.

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## 2. 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](mailto:info@treat-nmd.eu)

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## 3. TREAT-NMD news and reports

### Active discussion lists

As you can see from our website, TREAT-NMD is working on generating agreed standards in various measures which we hope will be important tools as we take part in clinical trials. Active discussions are ongoing in a number of areas and we are very keen that the debate is as broad as possible so that these standards will be universally accepted and applied. For this reason, we are asking you to think about colleagues in your institution or elsewhere who may not yet be involved in TREAT-NMD but who could help either to contribute to the development of these standards or in their national or regional application.

Active discussion is ongoing at the moment about registries and biobanks, standardised assessment of animal models, outcome measures for clinical trials (in particular getting the patient's perspective on what are the most important outcomes), and standards of diagnosis and care in DMD and SMA.

Please let the TREAT-NMD office know (by writing to [rachel.thompson@treat-nmd.eu](mailto:rachel.thompson@treat-nmd.eu)) if you want to take part in these areas of debate, or if you know someone else who should be involved in these discussions. TREAT-NMD does not wish to be an exclusive organisation, but rather to be truly broadly representative, so please help us to achieve this!

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### Training opportunity

The EuroBioBank network ([www.eurobiobank.org](http://www.eurobiobank.org)) coordinated by Eurordis offers specialised training in working with neuromuscular human biomaterial. Prof. Dr. Hanns Lochmüller, MTCC, University of Munich, Germany, and Ao4 leader, can help organise training with primary human muscle cells.

Myoblast training courses are available in Munich for TREAT-NMD members during the following weeks:

06.08.2007 - 10.08.2007

15.10.2007 - 19.10.2007



Prof. Lochmüller is also willing to coordinate a limited number of courses "on demand" to accommodate the calendars of the trainees. If any TREAT-NMD partners are interested can they please email Prof. Lochmüller at [hanns.lochmueller@med.uni-muenchen.de](mailto:hanns.lochmueller@med.uni-muenchen.de)

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## EURORDIS conference report

By Jerome Parisse-Brassens

The Eurordis 10 year anniversary membership meeting and the European Workshop on "Gaining access to rare disease research resources" on 4 and 5 May 2007 at the Pasteur Institute in Paris saw 270 participants from 27 countries getting together and discussing the issues closest to patients' hearts. The audience was made up of 75% rare disease patient representatives and 25% researchers, healthcare professionals, industry representatives and public policy makers. The European workshop was part of the *Capacity Building for Patient Organisations in Research Activities* project (CAPOIRA). 'It was a milestone in the empowerment of patient groups on the way to playing a greater role in the European research agenda,' said Fabrizia Bignami, Therapeutic Development Officer at Eurordis, who was in charge of the workshop. The workshop, which lasted a day and a half, saw presentations on rare disease research resources and integration of patient groups in research. Two sub-groups were asked to meet and debate on the needs and wishes of patient groups on two different topics: European tools for rare disease research, and getting involved in research. The results of this Eurordis workshop will be presented at another workshop on rare disease research organised by the European Commission on 14 June 2007. 'It will be an opportunity to further stress the role of patients in EU research,' said Bignami, who will participate in this meeting. The outcomes of the 14 June discussions will in turn be presented at a conference entitled 'Rare Diseases Research: Building on Success', which will take place in Brussels on 13 September 2007, with members of the EU Parliament, representatives of rare disease research funding institutions, national and European health and regulatory authorities, researchers, and patient organisations. Another opportunity for the rare disease community to express its needs in terms of research, and another step towards full participation of rare disease patients in the European research agenda and decision making process – something Eurordis has long fought for, and always will.

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TREAT-NMD notes:

- Eurordis is a TREAT-NMD partner with special responsibility for the network's biobanking activities.
- Professor Volker Straub addressed the conference participants during the Friday session, giving a presentation about the TREAT-NMD network. The interest expressed by members from across the globe was a clear indicator of the value of the network's goals and the importance of ensuring the network is of worldwide relevance.

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## 4. Pick of the journals

### 'Muscle noise' could reveal diseases' progression

A new non-invasive elastography technique that measures "muscle noise" could provide a way of monitoring neuromuscular disease without exposing the patient to radiation. The elastic properties of a muscle can reveal their condition. Muscles become harder, for example, when they contract during exertion. Neuromuscular disease also produces changes in muscle stiffness. So detecting these changes could provide a way to monitor the progress of the disease.

Karim Sabra and colleagues at the Scripps Institute of Oceanography in California, US, measured muscle vibrations by placing 16 sensors, each 1.5 centimetres apart, along the thigh of a healthy male volunteer while attaching increasing loads to his ankle. This allowed them to measure the vibrations produced by the volunteer's *vastus lateralis* thigh muscle under different stresses. The researchers used the sensor array to follow vibrations in muscle fibres across the muscle as it contracts. The velocity of these vibrations depends on



the local muscle stiffness. By measuring velocity variations as the weight increased, the team was able to build up a comprehensive picture of the muscle's elastic properties under different conditions.

The researchers say it should be easy to monitor the progression of muscular diseases after analysing more muscles and building up a database of vibration responses. The key advantage of the new technique is that it is non-invasive. This means that it does not require external sources, such as indentation or ultrasound, to produce the propagating waves. The method could be complementary to X-rays and MRI (magnetic resonance imaging) as it gives quantitative information about the muscle's mechanical properties. However, it could also be used alone, eliminating the need for radiation altogether. Apart from monitoring neuromuscular disease, the technique could also be used to check on sports injuries, such as pulled tendons. The team now plans to look at gathering more detailed information about thigh muscle vibration, which could improve the accuracy of the technique.

Journal Reference: *Applied Physics Letters* (vol 90, p 194101)

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## 5. Calls for proposals

We regularly publish calls for proposals that might be of relevance to TREAT-NMD in this section of the newsletter. The network has ambitious plans in areas such as training and education, and since these are not covered by our initial funding from the EU, we have to seek funds from elsewhere. We strongly encourage anyone outside the network who is interested in developing links with us to look through these calls and see if there is anything of relevance to you. We also encourage partners to consider possibilities for academic exchanges via these proposals.

Attracting additional funding to the network activities is vital if we are to ensure the future sustainability of the network. There are a number of research-based calls for proposals under FP7 that would be suitable for the network, and we would like to ensure that the network has every opportunity to obtain funding through FP7. Therefore, it is important that the Coordination Team at Newcastle ([stephen.lynn@treat-nmd.eu](mailto:stephen.lynn@treat-nmd.eu)) are kept up-to-date on any of your research proposals that are associated with the network activities, as we can offer help and support as well as a united front in which we work together to win European funding either as a single partner or in collaboration with other partners and non-network colleagues.

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## 6. Upcoming conferences, meetings and workshops

### Seventeenth Meeting of the European Neurological Society 2007

Date: 18–20 June 2007

Venue: Rhodes, Greece

[http://www.lswn.it/meetings/2007/seventeenth\\_meeting\\_of\\_the\\_european\\_neurological\\_society](http://www.lswn.it/meetings/2007/seventeenth_meeting_of_the_european_neurological_society)

### European Muscle Conference

Date: 8–12 September 2007

Venue: Stockholm, Sweden

Deadline for abstracts: 30 May 2007

More info: [www.esmr.org](http://www.esmr.org)

**TREAT-NMD meetings will be advertised in both the weekly newsletters and the TREAT-NMD website – please see the “news and events” section for details.**

If there is an event you would like us to publicise in future newsletters and on our website, please send details to [rachel.thompson@treat-nmd.eu](mailto:rachel.thompson@treat-nmd.eu)



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## 7. Partner-specific items

### TREAT-NMD 3 month consolidated report

The 3 month report is now available to download from the private section of the website.

### Discussion forums / lists

Would you like us to set up a discussion forum for you on the TREAT-NMD website?

If so, please e-mail [rachel.thompson@treat-nmd.eu](mailto:rachel.thompson@treat-nmd.eu)

### Calls for proposals / funding opportunities

Please forward to us at the Coordination Office any calls for proposals and funding opportunities you receive within your institution. We will then advertise these in the newsletter and on the website.

### Posters and presentations for Governing Board meeting

Partners have been asked by Stephen Lynn to prepare and present posters and/or presentations for the Governing Board meeting. Templates are available to download from the private section of the website. Please do contact us if you require any assistance.

### TREAT-NMD flyers

We have had a number of glossy flyers printed to distribute within the neuromuscular community and would be happy to send some to any partner wanting to help publicise TREAT-NMD, whether this be at international conferences or internal meetings. If you would like to receive a pack please e-mail Rachel Thompson at [rachel.thompson@treat-nmd.eu](mailto:rachel.thompson@treat-nmd.eu)

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## 8. Send us your news and views!

We 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](mailto:emma.heslop@treat-nmd.eu).

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