



**TREAT-NMD Partner Newsletter No. 20
and Club of Interest Newsletter No. 14**

20th July 2007

Welcome to the fourteenth newsletter for the TREAT-NMD Club of Interest. This week's edition is somewhat quieter than last week's, but nonetheless features an interesting report from DRCI (section 3), some EMEA news (section 4) and two calls for proposals that could be very helpful for anyone interested in carrying out research with international collaborators (section 5).

We hope you enjoy the newsletter and look forward to hearing your comments – please write to info@treat-nmd.eu with anything you'd like to say. Feel free to forward this newsletter to anybody you think might find it of interest, or invite them to sign up to receive the newsletter directly 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 the back-issues. If you have received this 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.

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. The coordination team in Newcastle will be happy to put you in touch with the person most relevant to your particular interest.

3. TREAT-NMD news and reports

DRCI and TREAT-NMD

International links involving countries outside Europe are extremely important to the TREAT-NMD ethos of harmonization, so we are very glad to be working with DRCI, a new international collaboration of voluntary organizations in the DMD field. DRCI have kindly provided this summary of their aims and objectives.



DUCHENNE
RESEARCH
COLLABORATIVE
INTERNATIONAL

About the Duchenne Research Collaborative International (DRCI):

The Duchenne Research Collaborative International (DRCI) is an international collaboration among four of the world's leading voluntary health organizations who are committed to work together to examine, design, and drive the coordinated execution of projects to accelerate translational research in Duchenne muscular dystrophy. Created in 2006, the DRCI is jointly led by the Association Française contre les Myopathies (AFM), Muscular Dystrophy Association (MDA), Parent Project Muscular Dystrophy (PPMD), and United Parent Projects Muscular Dystrophy (UPPMD).

AFM - Created in 1958 by a group of patients and their families, and recognized as being of public utility in 1976, AFM (French Muscular Dystrophy Association) has a single objective: to defeat neuromuscular diseases which are devastating muscle-wasting diseases. It has set itself two missions: curing neuromuscular diseases and reducing the disabilities they cause. For more information, visit: www.afm-france.org.

MDA - The Muscular Dystrophy Association, founded in 1950, is a voluntary health agency - a dedicated partnership between scientists and concerned citizens aimed at conquering neuromuscular diseases that affect more than a million Americans. For more information, visit: www.mda.org.



PPMD - Parent Project Muscular Dystrophy's mission is to improve the treatment, quality of life and long-term outlook for all individuals affected by Duchenne muscular dystrophy (DMD) through research, advocacy, education and compassion. For more information, visit: www.parentprojectmd.org.

UPPMD - UPPMD (United Parent Projects Muscular Dystrophy) is an international nonprofit organization set up by Duchenne Parent organizations around the globe. UPPMD is specifically focused on Duchenne and Becker muscular dystrophy, the most common and most severe of the muscular dystrophies. UPPMD was developed to share aims and goals by working efficiently and collaboratively with the DMD community to accelerate the development of promising treatments and a cure. UPPMD is managed and led by parents under close cooperation with clinicians and researchers. For more information, visit: www.uppmd.org.

Since its official launch in January 2007, the Duchenne Research Collaborative International (DRCI) has undertaken several initiatives consistent with its mission and purpose. They include:

Establishing a Research Information Clearinghouse

DRCI is working to establish and develop an international Clearinghouse to coordinate the sharing of information on funding for Duchenne-related scientific and clinical research.. The DRCI Clearinghouse will:

- Provide the Duchenne research and patient community with one central international repository to track research grants and the location and availability of key research resources.
- Pool DMD knowledge and resources in an attempt to accelerate development of promising treatments and a cure for the most common childhood form of the disease.

Advancing the Global Patient Registry Effort

DRCI is continuing to advance the global registry effort by coordinating efforts on the U.S. side. More specifically, DRCI:

- Held a meeting at the CDC on Feb. 20, 2007 to bring together leaders at the CDC to better coordinate efforts in key offices.
- Held another meeting in Washington, DC on April 30, 2007 to convene stakeholders to discuss next steps to launch a registry in the U.S.
- Continues to work on next steps to lead registry effort to have a DMD focus and increase coordination with TREAT-NMD and other global efforts.

Developing a Clinical Trial Network tool

DRCI is developing a Clinical Trial Network database tool that allows DRCI members to identify information on current and past clinical trials in the various DMD clinical networks. More specifically, the DRCI Clinical Trial Network database will:

- Serve as a platform to identify DMD support systems critical to the efficient conduct of clinical research.
- Allow for accelerated translational research conducted by the global DMD research community by drawing from shared databases and validated research resources, thus avoiding unnecessary and costly duplication of effort.

Developing Ethical Principles for DMD Scientific and Clinical Research

DRCI continues to work on developing ethical principles for investment in DMD scientific and clinical research. The DRCI Ethics Committee is:

- Working with the TREAT-NMD ethics committee to develop ethical principles and recommended guidelines for industry and nonprofit organizations that invest time and resources to help patients with Duchenne.
- Developing an ethical platform for investments in translational research in rare disease, using Duchenne as a model.



4. Other news



EMEA documents on orphan drugs

Orphan drugs and rare diseases at a glance

This EMEA publication provides definitions of orphan medicinal products and lists the incentives manufacturers have to invest in orphan drug production. It also provides information on the definition of a rare disease and provides useful links.

<http://www.emea.europa.eu/pdfs/human/comp/29007207en.pdf>

Practical information for sponsors during the early phase of an orphan drug application

This is an informal set of recommendations designed for orphan drug applicants to help the application process go more smoothly. It is a short list of practical issues and information for sponsors planning to submit an application for designation of a medicine as an orphan medicinal product that does not replace the legal requirements as set in the EU Directives, nor the guidelines available on the EMEA website. Instead it addresses issues that typically arise during the early phases of the application process, including the pre-submission meetings that are strongly encouraged and can take place via teleconference to save travel expenses; the format of the final application at the time of submission; and various items of general advice.

<http://www.emea.europa.eu/pdfs/human/comp/11459307en.pdf>



Useful information for FP7 applicants

Simpler payment scheme adopted for non-EU FP7 participants

The European Commission has implemented a lump-sum payment option for non-EU participating countries involved in applications to the Seventh Framework Programme. It is hoped that the flat-rate lump-sum amounts, which can be incorporated in grant agreements, will simplify the grant-agreement process as well as project administration and will ultimately facilitate the participation of International Cooperation Partner Countries. For more information see <http://tinyurl.com/2heb68>

COST boosts cooperation with Australia and New Zealand

Cooperation between European scientists and colleagues on the other side of the world is set to get a lot easier, thanks to two new schemes between COST (European Cooperation in Science and Technology) and Australia and New Zealand.

Each scheme will provide for up to 20 travel grants worth EUR 2,500 to support the travel and subsistence costs of European scientists travelling to Australia or New Zealand, and scientists from those countries coming to Europe. See <http://tinyurl.com/37e6j9>

5. Calls for proposals

ESF Research Networking Programmes - 2007 Call for Proposals

Deadline: **30 October 2007**; 16.00hrs CET

An ESF Research Networking Programme is a networking activity bringing together nationally funded research activities for four to five years, to address a major scientific issue or a science-driven topic of research infrastructure, at the European level with the aim of advancing the frontiers of science.

Key objectives include:

- creating interdisciplinary fora;
- sharing knowledge and expertise;
- developing new techniques;
- training young scientists.



A successful Programme proposal must show high scientific quality and also demonstrate added value by being carried out at a European level rather than by individual research groups at the national level.

Proposals with a 'global' dimension which intend to interface a European scientific network supported by a new ESF à la carte Programme with network(s) of scientists in other, non-ESF, countries (e.g. Australia, Canada, China, Japan, Russia, USA and others) supported by their own agencies are also encouraged.

A Programme can include the following activities:

- Science meetings (workshops, conferences or schools) organised either by Programme participants or following an open call for proposals;
- Grants for short and exchange visits awarded following an open call for applications;
- Publication of information brochures and leaflets, DVDs and CD Roms, scientific books and meeting proceedings etc; creation of websites;
- Creation of scientific databases at the European level.

Further info: <http://www.esf.org/activities/research-networking-programmes/2007-call-for-proposals.html>

European Cooperation in the field of Scientific and Technical Research (COST)

COST open call for Proposals to support Scientific and Technical Collaboration in Europe

COST invites researchers throughout Europe to submit proposals for research networks and use this unique opportunity to exchange knowledge and to embark on new European perspectives.

COST's main objective is to stimulate new, innovative and interdisciplinary scientific networks in Europe. COST activities (Actions) are carried out by research teams to strengthen the foundations for building scientific excellence in Europe. This continuous call is thematically open.

COST invites proposals for new COST Actions contributing to the scientific, economic, cultural or societal development of Europe. Proposals playing a precursor role for other European programmes and/or initiated by early-stage researchers are especially welcome.

COST finances networking of nationally funded activities in supporting meetings, conferences, short term scientific exchanges and outreach activities. COST supports the networking of specific research themes (COST does NOT fund research projects themselves). Currently more than 200 Actions are supported. Every year approximately 50 new Actions will be approved.

Proposals should include researchers from a minimum of five COST member states. Financial support in the range of 100.000 € p.a. for normally 4 years can be expected.

The next collection date for Preliminary Proposals is **30 September 2007**.

Further info: <http://www.cost.esf.org/index.php?id=721>

6. Job opportunities

The TREAT-NMD website now features a job opportunities page. Please visit <http://www.treat-nmd.eu/jobs.htm> to view all current job opportunities.

If you would like to advertise a relevant job opportunity on our site and in our newsletter, please write to rachel.thompson@treat-nmd.eu



7. Partner-specific items

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.

Discussion lists currently active include:

- Registries and biobanks
- Standardised assessment of animal models
- Outcome measures for clinical trials (especially patient's perspective)
- Standards of diagnosis and care in DMD and SMA
- Coming soon- Gender issues in the network

If you would like to be involved in any of these discussions or if you know someone who would like to contribute, please let the TREAT-NMD office know (by writing to 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.

Deliverable reports

Many partners have deliverable reports due within the next few weeks. Could we please encourage you to observe the due course of action for the production of your report as outlined in the e-mail you will have received from ACIES and as detailed below;

1. The work package leader responsible sends the completed deliverable, using the template attached to the e-mail, to the activity leader
2. The activity leader has 2 weeks for reviewing and iterating with the partner responsible.
3. The activity leader then sends the deliverable to the Project Coordinator (+ copy to eu-new@acies.fr), who will also check it before sending it to the European Commission.

8. 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

