

**TREAT-NMD Partner Newsletter No. 7  
and Club of Interest Newsletter No. 1**

**20<sup>th</sup> April 2007**

## Welcome to TREAT-NMD!



The TREAT-NMD network has now been officially active for almost four months, and this newsletter is the first to go out not only to our partners but also to our broad base of supporters – our “club of interest” – across the globe. Many of you will already know something of our aims and activities – some of you were even with us at our launch in January (see photo above) – but for those of you who would like to learn more, we’re beginning the newsletter with a brief overview of what we’re about. We’d like to take this opportunity not only to thank you for your interest and support, but also to encourage you to get involved, wherever your particular interest lies. Communication and working together is at the heart of what this network is about, and we’re keen to hear from you. Visit our website at [www.treat-nmd.eu](http://www.treat-nmd.eu) to find out more about us, or write to us at [info@treat-nmd.eu](mailto:info@treat-nmd.eu).

Best wishes,

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

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## 1. About the TREAT-NMD network

TREAT-NMD is an international initiative bringing together some of the world's leading neuromuscular specialists in a pan-European "network of excellence" aimed at improving treatment and finding cures for patients with neuromuscular disorders. Linking 21 partner organisations and over 300 doctors, researchers and other professionals throughout 11 European countries, the EU-funded network will enable experts to work together to share good practice and improve global standards of care.



Recent advances in cutting-edge therapies mean that clinical trials are no longer in the realm of fantasy. But such advances are being delayed because of the fragmentation that currently exists in research and healthcare systems across Europe and indeed the globe. Without validated outcome measures, consensus on standards of care and diagnosis, or uniform patient databases, setting up international trials of the most promising therapies is an uphill struggle. TREAT-NMD addresses all these areas of concern, with working groups of partners devoted to each of the major issues.

- Working with experts from all over the world, TREAT-NMD will develop common standards for diagnosis and care of neuromuscular disorders.
- The lack of national and international patient registries means that researchers and drug companies often have a hard time identifying suitable patient cohorts in their own countries, let alone internationally. Developing harmonised international patient databases and biobanks containing the key genetic data researchers need to target treatments to the appropriate patients is a fundamental goal of the TREAT-NMD project.
- TREAT-NMD will establish a Clinical Trials Coordination Centre to provide specialist advice and support the translation of research into new drugs for clinical practice.
- The network will expand its links with industry to develop new and effective treatments for neuromuscular diseases and will aid the progression of cutting-edge therapies from the lab to the clinic.
- Standardised protocols will be developed to assess the efficacy of novel therapies in the preclinical phase and establish the best animal models for the individual diseases; while in the clinical setting, outcome measures will be evaluated with the aim of standardising on the best approach.
- TREAT-NMD will also draw up standardised procedures for the production of therapeutic agents and for their toxicology, quality and safety assessment.
- Training and education is fundamental to the network's goals, and an online "clearing house" for research placements, visiting professorships and other training opportunities for scientists and clinicians will be created with the aim of "spreading excellence" across the globe.
- Patient organisations are an integral part of the network on a structural level, and further close links are being developed with patient groups worldwide to ensure we know how we can best meet patients' needs and so they can be kept informed of the latest advances in research and treatments.
- Although the TREAT-NMD project's funding was for a European network of excellence, the project already has a worldwide reach, since it makes no sense for each continent to reinvent the wheel. Links with top specialists across the globe are already in existence and will ensure that everyone moves forward together on the key issues.



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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. Much 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. Perhaps you're considering setting up a database for a particular neuromuscular disease in your country – contact us and we can advise you on the legal issues involved, the software you might need and the data you should be collecting and suggest how to make your database compatible with ours so that data can be shared. Perhaps you could offer a training placement or exchange visit for researchers at your institution, or perhaps you'd be interested in opportunities for training yourself. Perhaps you can contribute to our work on standards of care, on outcome measures, on animal models or toxicology. Write to us at [info@treat-nmd.eu](mailto:info@treat-nmd.eu) and we'll put you in touch with the relevant person within the network. We look forward to hearing from you!

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### 3. TREAT-NMD website

The TREAT-NMD website, [www.treat-nmd.eu](http://www.treat-nmd.eu), is developing to become a complete communications platform for the network: an "intranet" for partners and a valuable tool for disseminating information to the wider community. It will be updated on a regular basis over the coming months, so please keep visiting it for news.

This week activity leaders have been asked to provide content for the website relating to their activities and workpackages. All activity leaders should have received a proforma to fill in and return to Rachel Thompson. The website is an important public face for the TREAT-NMD project and as such we want it to provide useful information written in an interesting and straightforward style suitable for "public consumption". We hope you enjoy the challenge and look forward to receiving your contributions.

**TREAT-NMD Website Proforma – deadline 25<sup>th</sup> April 2007**

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### 4. Conference reports

#### **Summary of the WP 8 meeting (March 27, LUMC, Leiden, the Netherlands)**

Participants: Hervé Laouenan (AFM, activity leader), Annemieke Aartsma-Rus (LUMC, WP8.2 leader) and Judith van Deutekom (Prosensa/LUMC)

WP 8 focuses on the production, toxicology, safety and delivery of therapeutics. The different therapeutics consist of antisense-mediated exon skipping (using either 2'O-methyl RNA AONs or morpholinos), gene therapy (using plasmids, AAV, or lentiviral vectors) and cell transplantation (allogenic or autologous *ex vivo* treated).

We acknowledge that these therapies differ to a great extent and consequently there are different requirements and conditions regarding the production (e.g. large scale production is complex for viral vectors, but not for AONs) and delivery (e.g. AAV will home in on muscle, which is not the case for the other therapies). In addition, the different nature of these therapies requires different strategies to determine toxicology and safety. Finally, legislation varies in the different countries, and each country has its own regulatory issues and specific requirements regarding toxicology and safety studies.

In order to be able to set up general guidelines to facilitate development of therapies to the clinic we need to identify which problems are shared between some or all of the approaches. Therefore, we plan a small scale workshop involving one expert on each of the different therapeutic strategies, one toxicology expert and one regulatory issues (EMA) expert. A neutral expert will chair the workshop, guide discussions and supervise the identification of commonalities between the different approaches

Prior to the workshop we will send a questionnaire to the chosen experts.

These experts will then address the specific questions in a short presentation during the workshop, which should allow the identification of common problems.

**Questions:**

- How has proof of concept been ascertained? (cell models, animal models, humans)
- What is known about toxicology? (studies in animals, humans)
- What is known about safety? (studies in animals, humans)
- What is known about large scale production? (feasibility)
- What problems will have to be solved before this therapy is clinically applicable? (briefly, point by point)
- How will the therapy be delivered?
- Is anything known about the expected dose and number of treatments?
- Is an immunological response possible? (against the therapeutic agent, against dystrophin?)

**Questions for toxicology expert:**

- What toxicology tests are advisable/ required for each of the different strategies?

**Question for legislative expert:**

- What tests does EMEA require before each of these therapies can be brought into the clinic?

**General question:**

- Do we need new guidelines for DMD therapy development?

As all participants will be familiar with each of the discussed therapeutics, so there is no need to explain how each therapy works. Also, it is not the aim of this workshop to discuss problems with individual therapies, but to identify those that are common to the different therapies.

A publication will be delivered which summarizes the workshop's aims and conclusions, including a plan to tackle common problems. In addition, for each strategy the toxicology and EMEA requirements will be stated. The statements of experts should provide an excellent tool to facilitate the process of bringing DMD therapeutics to the clinic.

We would like to ask our partners to help us by suggesting experts for the following fields:

- AONs zOMePS
- AONs morpholino
- Viral vectors
- Plasmids
- Cell Therapy
- Toxicology
- Regulation
- Neutral expert

In addition, suggestions or remarks about the proposed questions are welcome – please write to [a.m.aartsma-rus@lumc.nl](mailto:a.m.aartsma-rus@lumc.nl) with your comments

Thanks in advance for your help,  
Annemieke Aartsma-Rus & Hervé Laouenan

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## 5. 'Fact-finding Questionnaire – supplementary questions' – deadline 27<sup>th</sup> April 2007

The fact finding questionnaire was e-mailed to key contacts within each partner organisation on Thursday 12<sup>th</sup> April 2007. In response to requests by some partners, supplementary questions have been e-mailed this week. Please complete both questionnaires and return them to [emma.heslop@newcastle.ac.uk](mailto:emma.heslop@newcastle.ac.uk) before 27<sup>th</sup> April 2007.

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## 6. 3-monthly report

Thank you to all the activity leaders who have submitted their 3 monthly reports to Bénédicte Charrin from ACIES. For those who were unable to submit their reports, Bénédicte has extended the deadline to the end of the month. Please make every effort to submit your report to ([eu-new@acies.fr](mailto:eu-new@acies.fr)) before 30<sup>th</sup> April 2007.

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## 7. Call for proposals

### **Franco-British Research Collaboration: Alliance 2008**

Deadline – 1st June 2007

Details – The aim of the contract is to promote the development of Franco-British scientific co-operation involving universities and public sector research institutes by providing support for new scientific collaborations between two (or more) research teams working on a common project. Grants are made for one year, renewable for a second year.

Further information

For UK-based researchers, full details about the scheme and application form are available from :  
<http://www.britishcouncil.org/france-science-alliance-programme.htm>

For Researchers based in France, full details about the programme are available from  
<http://www.egide.asso.fr/fr/programmes/pai/appels/alliance.html>

For any questions: [alliance@britishcouncil.fr](mailto:alliance@britishcouncil.fr)  
or Sandrine Mahieu on +33 (0)1 49 55 73 32

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## 8. Latest news / research

### **Cooperation is key, say neuromuscular-disease researchers**

**The Lancet Neurology Vol 6 April 2007 p 298-299**

The TREAT-NMD network received a very useful writeup in the current edition of The Lancet Neurology. This two-page article contains interviews with several partners and club of interest members and provides a valuable introduction to the network.

Clinical trials of new compounds to treat rare neuromuscular diseases are being delayed because of Europe's fragmented research and health-care systems. But an EU-funded project called TREAT-NMD aims to provide researchers and clinicians with a road map for integration. James Butcher reports.

<http://www.thelancet.com/journals/laneur/article/PIIS1474442207700674/fulltext>

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### **VASTox gets US patent for Duchenne Muscular Dystrophy drug discovery programme**

LONDON (AFX) - VASTox PLC said the US regulators have granted a patent that provides further protection for its drug discovery programme in Duchenne Muscular Dystrophy (DMD).

The AIM-listed biotechnology company said it is on target to announce by the end of the first half of 2007 a drug candidate that will go into pre-clinical development.

<http://www.hemscott.com/news/latest-news/item.do?newsId=41835128968546>

[http://www.vastox.com/news/press/20070412\\_utrophinpatent.pdf](http://www.vastox.com/news/press/20070412_utrophinpatent.pdf)

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## **SynCo Bio Partners B.V. Signs Pharmaceutical Service Agreement With Prosensa B.V. for RNA Based Therapeutics for the Treatment of Duchenne Muscular Dystrophy**

AMSTERDAM, April 17, 2007 /PRNewswire/ -- SynCo Bio Partners and Prosensa announce today their agreement to collaborate in the formulation and fill of RNA based therapeutics for the treatment of Duchenne Muscular Dystrophy (DMD).

Prosensa, a clinical stage biopharmaceutical company and SynCo Bio Partners, the leading biopharmaceuticals manufacturer are to collaborate on the formulation and fill of Prosensa's lead product, an RNA based therapeutic under development for the treatment of DMD.

Under the terms of the agreement, SynCo will work with Prosensa to formulate and performed the aseptic fill of an RNA based product for use in human clinical trial.

Gerard Platenburg, CEO of Prosensa commented, "We are very pleased to be working with SynCo, a company with a strong track record in providing first class GMP manufacturing services"

Pierre Warffemius, CEO of SynCo commented, "SynCo is pleased to be working with Prosensa on this important clinical program for the life saving treatment of DMD. We aim to establish a long-term relationship with Prosensa to support their expanding portfolio of nucleic acid based therapeutics against neuromuscular disorders, by providing high quality aseptic formulation and filling services"

### **About Prosensa**

Prosensa B.V., a young Dutch Biopharmaceutical Company focused on the discovery, development and commercialization of nucleic acid based therapeutics correcting gene expression diseases with large unmet medical needs, in particular neuromuscular disorders. Prosensa is working closely with the research group of the Leiden University Medical Centre Department of Human Genetics, head professor Dr. G.J.B. van Ommen.

The Company is focused on developing a treatment for DMD (Duchenne Muscular Dystrophy). DMD is a genetic lethal childhood's disease with an incidence of approximately 1 in 3,500 new born boys. Clinical signs of muscle weakness start as early as 2 years of age affecting all muscles. Treatment of patients with DMD to date primarily involves supportive therapy. No curative treatment, re-establishing the function of dystrophin, the protein that is lacking in DMD patients, is yet available. For further information please visit the company's website at [www.prosensa.eu](http://www.prosensa.eu)

### **About SynCo Bio Partners**

Synco Bio Partners B.V., a leading, contract manufacturing organization, is focused on the manufacture, development and aseptic filling of biopharmaceuticals. SynCo has a strong track record in the production and fill of recombinant proteins and vaccines. SynCo offers a fully integrated range of services in manufacturing and supports biopharmaceutical companies from the earliest stage in process development, through clinical trials to market supply. Established in 2000 and privately owned, SynCo collaborates with a number of leading biopharmaceutical companies, in Europe and the USA. Approximately 120 employees are based at the company's headquarters and manufacturing facility in Amsterdam, the Netherlands. Here, SynCo's activities include analytical and process development for new products, manufacture of Active Pharmaceutical Ingredients for licensed products and clinical trials, and aseptic filling and lyophilisation (for clinical trials and market supply). For further information please visit the company's website at [www.syncobio.com](http://www.syncobio.com)

<http://www.pharmalive.com/News/index.cfm?articleid=432776&categoryid=15>

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## **9. Upcoming conferences, meetings and workshops**

### **Developments in Gene Expression Profiling, London, UK**

02 May 2007 - 03 May 2007

Addressing the latest applications of gene expression profiling based technologies within drug discovery and development, including real-time qPCR, RNAi and microarray, whilst examining the key challenges to the market, particularly cost of investment, management and handling of data and sensitivity and specificity of analysis techniques

<http://www.qpcr.net/index.aspx?ID=79836>

### **3rd International Meeting on Genetics of Complex Diseases and Isolated Populations, Torino, Italy**

26 May 2007 - 29 May 2007

This meeting has been designed to explore issues related to genetic studies in isolation and other special populations and to focus on achievements over the last two years.

<http://www.qpcr.net/index.aspx?ID=79594>

### **European Human Genetics Conference 2007, Nice, France**

16 June 2007 - 19 June 2007

<http://www.qpcr.net/index.aspx?ID=76125>

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#### **10. 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@newcastle.ac.uk](mailto:emma.heslop@newcastle.ac.uk). What else would you like us to include in the newsletter? Write to Emma with your feedback.

Please e-mail us with any information you have on upcoming education and training opportunities including workshops, conferences, funding, exchange programmes, clinical placements, visiting professorships and lectureships.

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#### **10. Publicising TREAT-NMD**

As part of efforts to raise the awareness of and publicise TREAT-NMD at conferences, workshops and other meetings we have produced a general double-sided colour flyer introducing TREAT-NMD and detailing the TREAT-NMD partner organisations. The flyer is now available for download from the TREAT-NMD web site at [http://www.treat-nmd.eu/assets/documents/TREAT-NMD\\_Flyer.pdf](http://www.treat-nmd.eu/assets/documents/TREAT-NMD_Flyer.pdf). We have also added an editable version in Microsoft Publisher format (higher resolution, better for those intending to print it), which you can download and have printed yourself at [http://www.treat-nmd.eu/public\\_html/private/docs/TREAT-NMD\\_Flyer.pub](http://www.treat-nmd.eu/public_html/private/docs/TREAT-NMD_Flyer.pub). We have also had a limited quantity of flyers printed, so if you would like some to be posted to you for a specific event, please write to [r.h.thompson@newcastle.ac.uk](mailto:r.h.thompson@newcastle.ac.uk).

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#### **11. TREAT-NMD link from your website**

In an effort to increase the profile of TREAT-NMD we are asking partners to add a link to the TREAT-NMD website from their existing website. Many of the partners have already done this – thank you! For those of you who have not, we would be very grateful if you could arrange for a link to be created. Supporters and members of the Club of Interest are also warmly invited to link to us – please let us know if you do!

To download a web-friendly TREAT-NMD button for your website please click on the following link and copy the appropriate line of code to your website: <http://www.treat-nmd.eu/link.htm>

Alternatively the TREAT-NMD logo is available on the website at <http://www.treat-nmd.eu/private/> for you to create your own hyperlink. Those without a partner login should write to [r.h.thompson@newcastle.ac.uk](mailto:r.h.thompson@newcastle.ac.uk) if they would like a copy of the logo.

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