

welcome

Welcome to the latest TREAT-NMD newsletter. This week's edition features information on three training grants available from TREAT-NMD, plus a call for research projects concerned with SMA along with the forthcoming Eastern European conference into rare diseases to be held in Bulgaria in June.

As always, we hope you enjoy the newsletter and look forward to hearing your comments - write to 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. Back-issues of this newsletter can be found on our website at <http://www.treat-nmd.eu/patients/news/ezine-archive/>
Best wishes,

Katie, Volker, Hanns, Steve, Emma, Rachel, Sam and Michael: the Newcastle TREAT-NMD team

at a glance...

[12-15 May 2009 The Nottingham Systematic Review Course 2009](#)

[21-23 May 2009 International conference in Ukraine: Recent standards in diagnosis, treatment and medical care for some rare neuromuscular diseases](#)

[01-03 Jun 2009 Update in Neuromuscular Disorders course in London](#)

[04-06 Jun 2009 TREAT-NMD workshop: clinical trial design in neuromuscular diseases](#)

[09-11 Jul 2009 "Therapeutic Targets in CMD", Emory University, Atlanta, Georgia](#)

[09-12 Sep 2009 IDMC-7 International Myotonic Dystrophy Consortium](#)

[17-19 Nov 2009 TREAT-NMD / NIH International Conference](#)



Training Opportunities

TREAT-NMD has three grant opportunities available for various courses, workshops and summer schools. These are:

Neuromuscular Disorders course in London, 01-03 Jun 2009 - further details can be found [here](#).

TREAT-NMD workshop: clinical trial design in neuromuscular diseases to be held in Freiburg Germany, 04 -06 June 2009 - more details about this workshop can be found [here](#).

The 12th Annual Summer School of Myology, 17 - 26 June 2009, Institut de Myologie, Paris, France - details [here](#).



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SMA Europe call for research projects

deadline 15 June 2009

The SMA-EUROPE association has launched a second call for projects aimed at funding international research for finding a cure to SMA. The dead line for submission is **June 15, 2009**. This follows the successful conclusion of the first Call for Projects in 2008.

The call for projects will be given adequate publicity. The screening of the projects will be provided by the same high-level Scientific Advisory Board as in 2008, through a thorough peer-review process.

The Call for Projects will be open to any research project aimed to find a cure or therapy for Spinal Muscular Atrophy (SMA). Priority will, however, be given to projects concentrating on the following aspects/fields:

1. Basic research
2. Gene and/or Cell therapy
3. Clinical trials
4. Biomarkers and/or functional outcome measures
5. Drug development.

There is a desire for this Call for Projects to both foster international collaboration between SMA

researchers and to address potential bottlenecks in the search for cures and therapies for SMA, and preference may be given to applicants who address these concerns.

This call will support innovative, interdisciplinary, multi-project approaches to accelerate cures and therapies for SMA. The initiative provides a framework for interactions that will reduce the time from concept to product. A multi-institutional partnership approach involving academic, non-profit, and/or commercial/industrial institutions - as well as collaboration between investigators from two or more countries - are encouraged.

Further details and application form scan be found [here](#).

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SMA Europe research awards announced

SMA Europe recently announced the award of over €750,000 for research into Spinal Muscular Atrophy (SMA) in response to its recent Call for Research Projects 2008.



TREAT-NMD partners working on outcome measures were amongst those to benefit. The grant on outcome measures in spinal muscular atrophy is a multicentric project presented by Eugenio Mercuri in Rome as part of a large international network including 12 centres from 9 European countries. The aim of this project is to validate a number of outcome measures that have recently been selected by TREAT NMD, in collaboration with ICC, as the most suitable measures for type II and III SMA. The project will also take into account the suggestions and the criticisms raised at the recent meeting with EMEA. This project will help to move TREAT NMD forward, providing opportunities for all the centres to ready themselves for multicentric clinical trials and to collect natural history data.

The five institutions which attracted support are :

- University of Lisbon : Professor Margarida Gama Carvalho (“Characterization of post-transcriptional control mechanisms regulating SMN2 gene expression.”)
- Università Cattolica, Rome : Professor Eugenio Mercuri (“Outcome measures in SMA types II and III”)
- University of Milan : Professor Giacomo Comi (“Development of a stem cell approach for treating Spinal Muscular Atrophy”)
- Institut de Myologie, Paris : Dr Martine Barkats (“Evaluation of AAV-mediated gene therapy in murine and feline SMA models”)
- University of Missouri, USA : post-doctoral fellow Dr Monir Shababi (“A Two-Pronged Approach to Develop a Treatment for Spinal Muscular Atrophy”)

SMA is a rare but devastating disease. Approximately one in every 6,000 children is born with it; it is the largest genetic killer of babies and knows no national or racial boundaries. Muscle strength is gradually reduced until even breathing unassisted is not possible for those worst affected.

Significant research progress has been made in recent years in understanding SMA, and much of this research has commonalities with that of other neuromuscular conditions.

SMA Europe was founded in early 2007, bringing together a group of European patient groups involved with Spinal Muscular Atrophy. All the SMA Europe representatives are affected by SMA or are parents of affected children, and have personal experience of its effects on patients and their families.

Since SMA Europe's inception, there has been a determination to combine resources in Europe in order to fund the best researchers on SMA worldwide. With the help of our Scientific Advisory Board, which includes some of the best international experts on neuromuscular diseases, we have succeeded.

We sincerely hope that this first year bears fruit in the search for effective therapy for all those suffering from Spinal Muscular Atrophy.

Our plan is to make this Call for Projects an annual event. The 2009 Call for Projects is now being launched.

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Fourth Eastern European Conference for Rare Diseases and Orphan Drugs

13-14 June 2009, Plovdiv, Bulgaria

This event is a continuation of previous Conferences on Rare Diseases and Orphan Drugs.



This year, the conference is supported and co-funded by a grant from the European Union, in the framework of the Public Health Programme.



The **general objective** of the conference is to **present rare diseases** as a **common issue** of all policies at **national / EU level** requiring integrated approach by all stakeholders.

Rare diseases are a common issue of all policies at national/EU level and cover variety of aspects: science, medicine, industry, finance, health care policy, social policy. Different groups of people are involved and good coordination and cooperation between those groups are the guarantee for successful practice in the area of rare diseases which is a challenge of the contemporary United Europe.

The 4th Eastern European Conference for Rare Diseases and Orphan Drugs is a continued action in the field of rare diseases aimed to improve knowledge, facilitate the access of the EEC to information on these diseases and add value to the public health knowledge. The conference will become an Eastern European unified stage where all stakeholders involved in the area of rare disease will meet and manifest their good will to integrative approach toward the objective of rare diseases.

For more information about deadlines, abstract submission and registration, please visit the [conference website](#).

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