

welcome

Welcome to the latest newsletter from TREAT-NMD, which includes details of a great opportunity to study and work in neuromuscular translational medicine in Newcastle. A new article addressing the realities and myths surrounding stem cell "therapies" is now available on the TREAT-NMD website, and this week we also highlight the Institut de Myologie's 13th annual summer school in Paris and two interesting opportunities to develop skills in trial design for small patient cohorts.

As always, if you have anything you wish to include in our next newsletter, please contact the coordination office and we will be happy to help.

Best wishes from Katie, Volker, Hanns, Steve, Emma, Rachel, Brigitta, Samantha, Karen and Michael, the Newcastle TREAT-NMD team.

at a glance...

[12-14 Feb 2010 Italian Parent Project Meeting - Rome, Italy](#)

[26-27 Feb 2010 Towards a Brighter Future - Sydney, Australia.](#)

[28 Feb 2010 Rare Disease Day - Worldwide Event.](#)

[25-26 Mar 2010 UK Neuromuscular Translational Research Conference - Oxford, England.](#)

[3-7 May 2010 International Child Neurology Congress 2010 - Cairo, Egypt.](#)

[10 May 2010 The George Karpati Symposium on Neuromuscular Disease: Innovation and Application, Montreal, Canada.](#)

[13-15 May 2010 5th European Conference on Rare Diseases 2010 Krakow, Poland.](#)

[17-19 Jun 2010 International Conference on Neuromuscular Diseases - Sao Paulo, Brazil.](#)

[24-26 Jun 2010 14th Annual](#)



"Stem Cell Tourism" - Hope versus Hype: an online guide

TREAT-NMD is aware that new scientific discoveries and innovations can lead to raised expectations and enthusiasm and this may in turn lead to hype, which can be seen as increased publicity and unfounded claims. To help inform patients and the public, TREAT-NMD has put together a new resource to give both patients and researchers guidance on stem cell research and how this can lead to stem cell tourism. With the ever-increasing media attention that stem cells have received over the years, it is no wonder that people may get confused about what is actually safe and scientifically valid and what is not.



There have been some promising results in animal model studies using stem cells for neuromuscular disorders (NMDs), however there have not yet been any clinical trials in humans. It will probably be a long time yet until the work on stem cells currently ongoing in the lab can be translated into reality for NMDs in the clinic.

In recent years, "stem cell tourism" has become more and more popular with patients who have NMDs. Unregulated clinics all over the world are charging substantial amounts of money for stem cell "therapies", which have not been shown to be effective, and most importantly safe. These clinics advertise their "treatments" on their websites without giving any scientific evidence to back up their claims, with only anecdotal evidence of their limited successes. Therefore, there is great concern from scientists that public confidence in their work could be undermined by these unregulated clinics selling bogus treatments.

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Clinical training opportunity in neuromuscular translational medicine at Newcastle University

A new opportunity has arisen for a doctor wishing to develop a specialisation in neuromuscular medicine. Based in a UK Centre of Excellence at Newcastle University this programme has been developed around the partnership between academic and industrial partners, namely Newcastle University and PTC Therapeutics. Together they offer a unique opportunity for translational medicine training in the UK.



This four year course begins with a taught first year around many of the issues of drug development and trial design which is then followed by a three year PhD project. This is a full-time salaried position on a Clinical Research Associate salary scale (*currently £27,523 - £46,426, although more senior candidates could be appointed to a higher scale*).

Industry and academic leads deliver the courses and the PhD programmes also have industry components, providing a broad education in the field of drug development and translational medicine.

The scheme is competitive, and applicants will need to be eligible for General Medical Council registration in the UK as well as being able to fulfil residency requirements.

The successful applicant will be based in the Newcastle muscle team, and will be able to take part in clinical, research and TREAT-NMD activities within the centre.

It is likely that successful applicants will be on or have just finished a training pathway in Neurology, Paediatric Neurology, Genetics or other specialties relevant to neuromuscular diseases.

[International SMA Research Group Meeting - Santa Clara, California, USA.](#)

[24-27 Jun 2010 Parent Project Muscular Dystrophy Annual Conference - Denver, Colorado, USA.](#)

[24-27 Jun 2010 Families of SMA Annual Conference - Santa Clara, California, USA.](#)

[19-23 Jul 2010 12th International Congress on Neuromuscular Diseases - Naples, Italy.](#)

[12-16 Oct 2010 World Muscle Society International Congress - Kumamoto, Japan.](#)

Please note: This is only a selection of upcoming meetings. To see all our listed meetings click [here](#).

Closing date for applications **26th February 2010**.

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Trial Design Training

With increasing numbers of neuromuscular therapies approaching the stage of readiness for clinical trial, the issue of trial design in small patient cohorts is assuming increasing importance. The Office of Rare Diseases Research at the US National Institutes of Health (NIH), and the Office of Orphan Products Development at the US Food and Drug Administration (FDA), have announced the 2010 edition of their collaboratively developed course, "The Science of Small Clinical Trials." This is a broad survey course (not a high-level statistical seminar) intended to heighten awareness of the methods that exist to design and analyze clinical trials using small numbers of participants. The course comprises seven 2-hour lectures, presented at the Lister Hill Center Auditorium on the NIH campus in Bethesda, MD, from 16 February through 8 March, 2010. Registration for attendance is already full; however, the lectures will also be available by online streaming (both live and on-demand). For more information see <http://small-trials.keenminds.org/>



Those wishing to have a more hands-on experience of trial design in small patient cohorts are reminded that the TREAT-NMD Clinical Trials Coordination Centre's June workshop is now open for registration - see <http://www.treat-nmd.eu/patients/events/events/760/>

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13th Summer School of Myology in Paris

Each summer, the Institute of Myology offers training in myology in the form of a condensed 10-day course organized in Paris. The course is open to all foreign students with special priority given to those posted in the French Overseas Territories (DOM-TOM) and those working in developing countries.



The content of the Summer School is similar to that of the University Diploma delivered jointly by Paris-6 and Aix-Marseilles Universities all year long. Only a certificate of attendance will be given upon completion of the Summer School.

Most aspects of myology are addressed during the course: from basic science to cutting-edge therapies and clinical and genetic approaches to muscle diseases.

The course this year will run between 21-29 June.

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