

**The new MRC Centre for translational research in neuromuscular diseases:
A London-Newcastle partnership to form UK-wide translational research networks**

About the Centre

Genetic and acquired neuromuscular diseases represent a major cause of mortality and morbidity in children and adults. In the UK there is a large gap between major science discoveries and patient benefit in these important disorders. This gap is larger in the UK than in other countries such as Germany, France and the USA who have already moved forward with translational research initiatives. The new MRC Centre aims to reduce this gap by establishing a multidisciplinary translational research activity in these disabling diseases.



This is a joint centre between the UCL Institute of Neurology and the UCL Institute of Child Health, London and the University of Newcastle. The Centre will build on long-established UCL-Newcastle research and clinical links. The centre will form reciprocal clinical and research links with other neuromuscular research groups and patient organisations throughout the UK. The Centre will work with the very large adult and paediatric neuromuscular disease patient populations cared for at the co-located hospitals: Great Ormond Street NHS Trust, the National Hospital for Neurology and Neurosurgery - Queen Square, UCLH NHS Foundation Trust and Newcastle Upon Tyne Hospitals NHS Foundation Trust.



Our mission is to translate basic science findings into clinical trials and new treatments for children and adults with disabling neuromuscular diseases. Current world-class science programmes in London and Newcastle attracting in excess of £20m of grant income will underpin the activities of the Centre. The Centre will develop new cross-cutting collaborations and will capitalise on the recruitment of world-class senior academic personnel to UCL and to the University of Newcastle. We have identified five key areas

which we consider to be current obstacles to effective translation of basic science findings into patient benefit. These are: clinical trials support, availability of patient tissues and cells, assessing animal models, applying MRI to humans and animals and developing capacity for the future. Over the next five years the Centre will specifically address each of these obstacles.

- We will facilitate clinical trials in neuromuscular disease in the UK by forming a single clinical trials support activity drawing on and combining the expertise in London and Newcastle. We will take advantage of the geography by forming north and south neuromuscular clinical trials centres. We will work together to facilitate clinical trial design, to develop biostatistical support, to develop clinical trial coordination, and to establish patient registries and clinician networks. We will take advantage of well-established, government funded, collaborative specialist neuromuscular diagnostic services which already exist between London and Newcastle (NCG services). In addition, we aim to develop a range of specific clinical assessment tools and outcome measures.
- A shortage of human cell lines and neuromuscular tissues currently hinders basic science efforts and in vitro testing of potential therapies. We will establish a unique UK biobank of human neuromuscular patient tissues and cells.
- Assessing the validity of animal models of neuromuscular disease and correlating phenotypes with human disease remains an important problem. We will link clinical and basic scientists, thereby establishing a network and resource for elucidating the validity of mouse models.
- We believe that the application of new MRI techniques has the potential to revolutionise the assessment and monitoring of neuromuscular disease in both animal models and patients. We will take advantage of major new MRI facilities in London and Newcastle to establish cutting edge MRI of nerve and muscle disease in animals and humans.
- We recognise the critical importance of training the basic and clinical neuromuscular scientists of the future. The Centre has developed a brand new four-year neuromuscular disease PhD programme. We will deliver exciting translational research environments to attract and train a new generation of basic and clinical neuromuscular scientists to build future capacity in the UK.



By developing these five core areas the Centre will promote translational research and add value to basic science neuromuscular research themes currently active in London, Newcastle and other centres.

Contact details:

Zoë Scott, Centre Senior Administrator
MRC Centre for Neuromuscular Diseases
Box 102, National Hospital for Neurology & Neurosurgery
Queen Square
London WC1N 3BG
0845 155 5000 x723432
z.scott@ion.ucl.ac.uk
www.cnmd.ac.uk