

MRC Centre for Neuromuscular Diseases in Children and Adults

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Title of project or programme

MRC Centre for Neuromuscular Diseases in Children and Adults

Principal Investigators of project/programme grant

Title	Forname	Surname	Institution	Country
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Address of institution of lead PI

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Country

United Kingdom

Source of funding information

Medical Research Council

Total sum awarded (Euro)

2870886.18

Start date of award

01-02-2008

Total duration of award in months

60

The project/programme is most relevant to

- Motor neurone diseases

Keywords

Research abstract in English

In the UK there is a large gap between basic science discoveries and patient benefit in neuromuscular diseases. In order to reduce this gap, we will establish the first truly multidisciplinary

UK translational research centre for children and adults with these disabling diseases. The centre will be based in the Institute of Neurology in collaboration with the Institute of Child Health, and will take full advantage of the largest UK neuromuscular patient populations at the co-located partner NHS Trusts: Great Ormond Street NHS Trust and the National Hospital for Neurology and Neurosurgery, UCLH, NHS Trust. The mission of the centre will be to translate basic science findings into clinical trials and new treatments for children and adults with disabling neuromuscular diseases.

The main programmes of research will build on existing funded [#10million] themes currently active across UCL. We will develop new cross cutting collaborations and will recruit new world class senior academic personnel to UCL achieving critical mass. All programmes of research will impact upon and benefit from the following core areas that will be newly developed in the centre and which are a current hurdle to effective translational research in the UK;

- 1.education and training to produce a new generation of neuromuscular scientists
- 2.developing a range of specific clinical assessment tools to facilitate future clinical trials in the UK.
- 3.Establishing new cutting edge MRI of nerve and muscle disease in animals and humans.
- 4.Establishing a unique biobank of human neuromuscular patients tissues and cells.
- 5.Establishing a network and resource for elucidating the pathogenesis of neuromuscular conditions in mutant mice.

The research programmes cover major diseases of muscle and nerve and include molecular mechanisms in muscular dystrophy, mitochondrial DNA neuromuscular disease, ion channel neuromuscular disease, muscle stem cells, genetic neuropathies, spinal muscular atrophy, generation of neuromuscular disease mutant mice, MRI of nerve and muscles in animals and humans and trials & outcomes in neuromuscular diseases. This combined approach will address unresolved aspects related to the pathophysiology of common neuromuscular disorders and improve the chances of translation into patient benefit.

Lay Summary