Advances in Cell Therapy for Huntington’s Disease

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The current lack of treatment for Huntington’s disease has led to efforts by the NIHR Cambridge Biomedical Research Centre to translate cell therapy advances into disease modifying treatments. A safety study involving the transplantation of human fetal striatal tissue into the striatum of five patients with Huntington’s disease has demonstrated that the procedure is safe and feasible.

Stereotaxic tissue placement following pre-operative magnetic resonance imaging was performed in Cambridge, UK by investigators from the NIHR Cambridge Biomedical Research Centre (NIHR Cambridge Dementia BRC) as part of a broader UK network. Cambridge-based protocols for the screening of human fetal tissue for potential pathogens have also been developed, with clinical trials planned in Parkinson’s disease. Cambridge surgeons have now grafted five patients and the long term follow up of which has been published in the Journal of Neurology, Neurosurgery & Psychiatry.

Vital Research

Huntington’s disease affects an estimated 6,500 to 8,000 people in the UK. This is an incurable inherited disease that causes the progressive loss of nerve cells in the brain. One of the major sites where this occurs is in a structure called the striatum which sits deep within the brain. Research during the 1980’s and 1990’s showed that it was possible to transplant fetal striatal tissue into the brain of animal models of Huntington’s disease, where it could survive and make connections with the host brain. The ability of this fetal tissue to survive and replace that lost in the experimental model was paralleled by improvements in some of the behaviours in these animals. In 2000, given this and the fact that there are no drugs or therapies that can stop or arrest the disease process in these patients, the NIHR Cambridge Dementia BRC team undertook a similar study in human patients with Huntington’s disease.

Interim Findings

The study involved patients with well established Huntington’s disease, where human fetal striatal tissue was transplanted into the striatum on one side, and then the other. The procedure was found to be safe, with no major complications from the surgical implantation of the tissue. The NIHR Cambridge Dementia BRC team found no major placebo effect from the operation and also saw no major improvements in the patients following the transplant. This suggests that this treatment does not work in Huntington’s disease, at least using this protocol.

Patient Impact

The study is important as it has shown that it is possible to transplant cells into the brains of patients with Huntington’s disease and that there is no major placebo effect from doing so. The treatment has shown no long term side-effect but then again no benefits, which may relate to the protocol used and/or the fact that Huntington’s disease affects many other parts of the brain, not just the striatum.

Professor Roger Barker, Theme Lead for Dementia and Neurodegenerative Disorders at NIHR Cambridge Biomedical Research Centre commented: “This study has enabled the NIHR to develop new ways of thinking about trials of novel cell therapies for diseases of the brain. It will help decide how the NIHR sets about formulating future stem cell-based trials for chronic neurodegenerative diseases of the brain. While this study concluded that using this specific protocol resulted in no major improvements, we have advanced our understanding of potential treatment methods for Huntington’s.”

NIHR Dementia Translational Research Collaboration

Dementia research is characterised by considerable methodological and disciplinary diversity. Addressing the main research questions requires drawing on a wide range of scientific areas and global specialist expertise.

To meet the challenge, the UK Government established the NIHR Dementia Translational Research Collaboration to pull discoveries from basic science into real benefits for patients. The collaboration comprises four new NIHR Dementia Biomedical Research Units as well as six NIHR Biomedical Research Centres with dementia-related research themes. These Units and Centres are world leaders in translational research, based within top university-NHS partnerships. The collaboration is a new and important part of the NIHR clinical research infrastructure and offers a unique opportunity to maximise the significant NIHR investment in dementia translational research.

As with all areas of unmet patient need, industry plays a crucial role in the development of new interventions to tackle dementia. Better collaboration between academia, the NHS and industry, with greater mutual transparency is vital. The opportunity for such collaboration has never been greater given global biopharma is opening up its development programmes to collaboration to access innovative new approaches to research and development.

The Dementia and Neurodegenerative Diseases Research Network (DeNDRoN) is part of the NIHR which supports the development, set up and delivery of dementia clinical research in the NHS. DeNDRoN is the link in the chain that connects researchers with patients and their carers throughout the research process.

For further information, contact NOCRI:

• NIHR Office for Clinical Research Infrastructure (NOCRI), A305 Richmond House, 79 Whitehall, London SW1A 2NS
• NOCRI Microsite: www.nocri.nihr.ac.uk
• NOCRI E-Mail: nocri@nihr.ac.uk
• Twitter: @NIHR_NOCRI