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Visionary stem cell technique offers new potential to treat blindness

Wednesday 22 September, 2010

Scientists funded by the Medical Research Council (MRC) are piloting a stem cell treatment to replace diseased parts of the retina, which could lead to a future treatment for retinal diseases that affect 3,000 children in the UK.

The researchers from UCL Institute of Child Health and UCL Institute of Ophthalmology successfully implanted cells from healthy mice in mice with an inherited form of childhood blindness called Leber Congenital Amaurosis (LCA).

The implanted cells express a gene called Crx which is needed to create healthy cone and rod photoreceptors. The cells were able to integrate with the retina and become new cone photoreceptors. This is the first time this has been achieved. Further studies are needed to determine whether it is possible to restore sight using this method.

Dr Jane Sowden from the UCL Institute of Child Health, who led the study, said:

"We have shown for the first time that it is possible to transplant cone photoreceptors into the mature retina. The newly-developed cells looked as good as new. This is an important step forward as cone photoreceptors are essential for reading vision and for colour vision. Loss of this type of cell has the biggest impact on sight."

"It may be possible to translate this success into treatments for humans. Recent research has shown that embryonic stem cells of self-renewal could provide an equivalent source of human cells that express the Crx 'photoreceptor-creating' gene and could be grown in the lab before being transplanted in the retina."

Professor Robin Ali from UCL Institute of Ophthalmology, who co-led the study, said:

"We are now working to assess how much vision can be improved by cone photoreceptor cell transplantation and how effectively they can replace degenerating cells at different stages of disease. There is still a lot of basic research that needs to be carried out, but this is a very promising area that we hope will translate into a future treatment for patients who cannot see because their photoreceptors have degenerated."

Investigating replacement and regeneration techniques that can repair human tissue is a fundamental area of research for the Medical Research Council. Studies like this one, which was supported with around £2 million in funding, aim to develop therapies to treat damaged tissue and overcome disease.

One in 3,000 people are affected by incurable genetically inherited retinal disease. LCA is the most severe form that causes blindness from birth or during the first few months of life and affects around 200 children worldwide. These diseases cause the light sensitive photoreceptor cells in the retina to die, leading to loss of sight.

'Cone and rod photoreceptor transplantation in models of the childhood retinopathy Leber congenital amaurosis using flow-sorted Crx-positive donor cells' is published in *Human Molecular Genetics* today. The work was funded by the MRC, the Macula Vision Research Foundation and Fight for Sight.

Ends

For more information please contact the MRC press office on: 020 7637 6011 or email: pressoffice@headoffice.mrc.ac.uk

To speak to Dr Sowden or Dr Ali, please call the Institute of Child Health press office 020 7239 3126/3119 or email: cox@sgh.nhs.uk

Notes to Editors:

1. The paper, 'Cone and rod photoreceptor transplantation in models of the childhood retinopathy Leber congenital amaurosis using flow-sorted Crx-positive donor cells', doi: 10.1093/hmg/ddq378, is available in *Human Molecular Genetics* at:

<http://hmg.oxfordjournals.org/content/current>

2. For almost 100 years the Medical Research Council has improved the health of people in the UK and around the world by supporting the highest quality science. The MRC invests in world-class scientists who have produced 29 Nobel Prize winners and sustains a flourishing environment for internationally recognised research. The MRC focuses on maximum impact and provides the financial muscle and scientific expertise for medical breakthroughs, including one of the first antibiotics penicillin, the structure of DNA and the lethal link between smoking and cancer. MRC funded scientists tackle research into the major health challenges of the 21st century. www.mrc.ac.uk

3. Founded in 1826, UCL was the first English university established after Oxford and Cambridge, the first to admit students regardless of race, class, religion or gender, and the first to provide systematic teaching of law, architecture and medicine. UCL is the fourth-ranked university in the 2009 THES-QS World University Rankings. UCL alumni include Marie Stopes, Jonathan Dimbleby, Lord Woolf, Alexander Bell, and members of the band Coldplay. UCL currently has over 20,000 undergraduate and 8,000 postgraduate students. Its annual income is over £600 million. www.ucl.ac.uk

4. UCL Institute of Child Health is, with Great Ormond Street Hospital, one of the largest centres for research into childhood illness in the UK.

5. Since 1965, the charity has funded research at leading universities and hospitals throughout the UK. Our major achievements in this area include:

- > saving the sight of thousands of premature babies through a better understanding and controlling levels of oxygen delivery;
- > restoring sight by establishing the UK Corneal Transplant Service