



What's New in Ophthalmology Today?

GENE THERAPY TO RESTORE VISION

"A revolutionary treatment for blindness in children" is undergoing the first clinical trial at the University College London. The study involves adults and children **Leber's Congenital Amaurosis**, a retinal condition which leads to blindness at a young age. The deterioration of the retinal cells are due to an abnormality in the gene RPE65. This genetic defect interrupts the normal function of the retina- the layer of light sensitive tissue that lines the back of the eye. The retina acts like the film in a camera. The researchers insert "healthy copies of the gene in to cell of the retina to help them function normally. Restoring the activity in these cells should restore vision." A virus is used to carry the genes into the retinal cells. "This trial is the first to treat an eye disease using the administration of gene therapy vectors to human retinas". The purpose of the trial is to determine the efficacy and safety in humans.

Extensive animal experiments have shown that this treatment can preserve and restore vision. Dogs with this defect were able to walk through a maze without difficulty after treatment. Professor Robin Ali at Moorfields Eye Hospital in London is in charge of this project. This is the first step in establishing gene therapy as a mode of treatment for many different eye diseases. They have already begun this treatment on young patients with **Leber's** who lost vision as children. The effect of the treatment will be assessed in the coming months. This opens a new frontier in the treatment of visual loss.

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