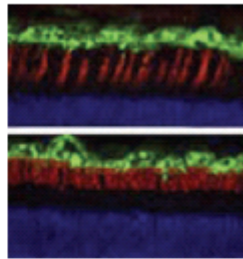


Gene therapy safety trial for childhood blindness under way

A gene therapy for childhood blindness is being tested for safety in a phase I clinical research study conducted by the University of Pennsylvania and the University of Florida with support from the National Eye Institute of the National Institutes of Health.



In November 2007, a young adult with a form of hereditary blindness called Leber congenital amaurosis type 2 (LCA2) received an injection in the retina of one eye, making the volunteer one of the first people in the world to undergo the procedure. LCA2 affects about 2,000 people in the United States and is one of several incurable forms of blindness collectively known as retinitis pigmentosa, which affects about 200,000 Americans. In all, six adults, and then three children between the ages of 8 and 17, will undergo the gene transfer procedure at the University of Florida over the next year or more before safety data are fully evaluated. A multicenter team of clinicians and scientists first established proof of concept for gene transfer for LCA in rodent models of the disease and in a breed of vision-impaired dogs. The trial will evaluate the use of a modified adeno-associated virus to deliver the missing RPE65 gene to the retina.