Gene therapy success reported in blindness cases
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A gene therapy was used to partially reverse blindness due to an inherited condition, doctors said this week, in clinical trial results hailed as one of the first clear successes for gene therapy.

Researchers used the treatment for a type of inherited blindness and said they found it to cause no side effects, and slight improvements in vision in four out of six patients studied.

The trials involved young patients with a condition called Leber's congenital amaurosis, a rare inherited disease caused by an abnormality in a gene called RPE65. The condition appears at birth or in the first few months of life and causes progressive deterioration and loss of vision.

Gene therapy is a class of experimental treatments aimed at replacing or fixing defective genes, but past gene therapy clinical trials have met with limited success despite great hopes.

The new findings were published yesterday in a pair of studies in the New England Journal of Medicine.

"Showing for the first time that gene therapy can work in patients with eye disease is a very significant milestone," said University College London opthalmologist Robin Ali, a researcher in one of the studies. "This trial establishes proof of principle of gene therapy for inherited retinal disease and paves the way for the development of gene therapy approaches for a broad range of eye disorders."

The researchers in both studies used harmless viruses to deliver corrected versions of a defective gene into the eyes of the patients. "We developed surgical techniques to enable access to the cells beneath the retinas of patients, using a very fine needle to deliver the modified virus," said the university's James Bainbridge, who led the surgical team in the Ali study. "It is tremendously exciting to see that this technique is safe in an extremely fragile tissue and can improve vision in a condition previously considered wholly untreatable."

Other institutions involved in the research included Michigan State University, the University of Pennsylvania, Howard Hughes Medical Institute in Chevy Chase, Md. and the Second University of Studies in Naples, Italy.