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US researchers develop a gene therapy to cure LCA

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The new genetic treatment, called voretigene neparvovec (Luxturna, Spark Therapeutics), involves a genetically modified version of a harmless virus.



Leber congenital amaurosis (LCA) is a rare condition that begins in infancy and progresses slowly, eventually causing complete blindness. It affects about one in 80,000 individuals.

US researchers have developed a gene therapy that may help patients, who have lost their sight to an inherited retinal disease, to see well enough to navigate a maze.

While the treatment does not restore normal vision, it does, however, allow patients to see shapes and light, allowing them to get around without a cane or a guide dog, said the researchers led by Stephen R. Russell, Ophthalmologist at the University of Iowa.

The new genetic treatment, called voretigene neparvovec (Luxturna, Spark Therapeutics), involves a genetically modified version of a harmless virus.

The virus is modified to carry a healthy version of the gene into the retina. Doctors inject billions of modified viruses into both of a patient's eyes.

The findings showed that after the treatment 93 per cent of patients experienced meaningful improvements in their vision, enough that they could navigate a maze in low to moderate light.

These patients also showed improvement in light sensitivity and peripheral vision, which are two visual deficits these patients experience.

However, it is unclear how long the treatment will last, but so far, most patients have maintained their vision for two years, the researcher said while presenting the results at the 121st Annual Meeting of the American Academy of Ophthalmology 2017 in New Orleans.

While, there are currently no treatments available for inherited retinal diseases, the new gene therapy is under review by the US Food and Drug Administration.

The FDA is expected to make its decision no later than January 2018, the researchers said.

An approval could open the door for other gene therapies that could eventually treat more than 225 genetic mutations known to cause blindness, the study noted.