Seeing the light

A new gene therapy has helped restore vision in people who had lost their sight to an inherited retinal disease, scientists have said.

Patients in the study had a condition called Leber congenital amaurosis (LCA), which begins in infancy and progresses slowly, eventually causing complete blindness.

This new, first-of-its-kind gene therapy is currently under review by the U.S. Food and Drug Administration (FDA) for potential approval this year.

There are currently no treatments available for inherited retinal diseases.

Researchers from University of Iowa, U.S., showed that 27 of 29 treated patients or 93% experienced meaningful improvements in their vision, enough to navigate a maze in low to moderate light.

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

More applications

Approval could open the door for other gene therapies that could eventually treat the more than at least 225 genetic mutations known to cause blindness. It could be applied to retinitis pigmentosa, another inherited retinal disease caused by a defective gene.

In the future, gene therapy could possibly provide key proteins needed to restore vision in more common diseases such as age-related macular degeneration.

What it entails

LCA is rare, affecting about one in 80,000 individuals. It can be caused by one or more of 19 different genes.

The tested gene therapy, voretigene neparvovec (Luxturna) from Spark Therapeutics, is currently under FDA review. An injection therapy, it uses a genetically-modified version of a harmless virus that’s been modified to carry a healthy version of the gene to patients’ retinas.

Doctors inject billions of modified viruses into both of a patient’s eyes. 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. It is unclear how long the treatment will last, but so far, most patients have maintained their vision for two years.

More than 200 patients with LCA have participated in gene therapy trials since 2007.

However, no gene therapy has got this close to FDA approval for retinal disease or any other eye disease.

In October, an advisory committee to the FDA unanimously endorsed the treatment.

The agency is expected to make its decision by January next year.

Lifestyle-related risk factors are being cited, compounded by an inadequate number of treatment centres in the region

Without policies to stop the worrying spread of antimicrobial resistance, the mortality rate could be disturbing

END