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Spark Therapeutics' LUXTURNA got FDA approval

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Luxturna is the first gene therapy approved in the U.S. to target a disease caused by mutations in a specific gene

The U.S. Food and Drug Administration approved Luxturna (voretigene neparvovec-rzyl); a new gene therapy, to treat children and adult patients with an inherited form of vision loss that may result in blindness.

Luxturna is the first directly administered gene therapy approved in the U.S. that targets a disease caused by mutations in a specific gene.

It is approved for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy that leads to vision loss and may cause complete blindness in certain patients.

Hereditary retinal dystrophies are a broad group of genetic retinal disorders that are associated with progressive visual dysfunction and are caused by mutations in any one of more than 220 different genes.

Luxturna works by delivering a normal copy of the RPE65 gene directly to retinal cells.

These retinal cells then produce the normal protein that converts light to an electrical signal in the retina to restore patient's vision loss.

Luxturna uses a naturally occurring adeno-associated virus, which has been modified using recombinant DNA techniques, as

a vehicle to deliver the normal human RPE65 gene to the retinal cells to restore vision.

Luxturna should be given only to patients who have viable retinal cells as determined by the treating physician(s).

Treatment with Luxturna must be done separately in each eye on separate days, with at least six days between surgical procedures.

It is administered via subretinal injection by a surgeon experienced in performing intraocular surgery.

The safety and efficacy of Luxturna were established in a clinical development program with a total of 41 patients between the ages of 4 and 44 years.

All participants had confirmed biallelic RPE65 mutations. The primary evidence of efficacy of Luxturna was based on a Phase 3 study with 31 participants by measuring the change from baseline to one year in a subject's ability to navigate an obstacle course at various light levels.

The group of patients that received Luxturna demonstrated significant improvements in their ability to complete the obstacle course at low light levels as compared to the control group.

The FDA granted this application Priority Review and Breakthrough Therapy designations.

Luxturna also received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases.