LUXTURNA

First gene therapy to treat a rare form of inherited vision loss

Summary

- On December 19, 2017, the FDA announced the approval of Luxturna (voretigene neparvovec-rzyl), a new gene therapy, to treat children and adult patients with vision loss due to RPE65-mediated inherited retinal disease, a condition that may result in blindness.[1]
- Luxturna is expected to be available for administration in select treatment centers late in the first quarter of 2018.[2]
- Luxturna will be covered under medical benefit plans subject to medical necessity review. The Wholesale Acquisition Cost (WAC) of Luxturna is $425,000 per eye or $850,000 for treating both eyes in an individual.[2] Luxturna is manufactured by Spark Therapeutics.
- According to information from Spark Therapeutics, it is estimated that between 1,000 and 2,000 people in the United States have vision loss due to RPE65-mediated inherited retinal disease.[2]

What is Luxturna and how does it work?

Luxturna is the first directly administered gene therapy approved in the United States that targets a disease caused by mutations in the RPE65 gene (RPE is retinal pigment epithelium). It is used to treat a rare, inherited form of vision loss caused by these mutations. A normal functioning RPE65 gene makes a protein that is essential for normal vision. Individuals with this inherited form of vision loss have defects (i.e., mutations) in both copies of the RPE65 gene which result in an absence or reduced level of the protein needed for normal vision.

To be considered a candidate for Luxturna, a genetic test is required to confirm the presence of two copies of the RPE65 genetic mutation.

Luxturna works by delivering a normal copy of the RPE65 gene directly to retinal cells. These retinal cells are then able to produce the normal protein in the retina to restore an individual's vision.[3] Luxturna uses a naturally occurring virus, which has been modified using recombinant DNA tenchology, as a vehicle to deliver the normal human RPE65 gene to the retinal cells to restore vision.[3]

How is Luxturna covered?

Luxturna is covered under Cigna-administered medical benefit plans.* All requests for Luxturna are subject to medical necessity review. Cigna administers coverage for Luxturna for a single course of therapy (one injection per eye) as medically necessary for the treatment of vision loss due to RPE65-mediated inherited retinal disease caused by a documented mutation of the RPE65 gene (confirmed by genetic testing). Luxturna is not recommended for patients younger than 12 months of age because the retina is still growing, which may affect how Luxturna works.[4]

What is the cost of Luxturna?

The Wholesale Acquisition Cost (WAC) of Luxturna is $425,000 per eye so the cost of treating an individual in both eyes in $850,000. There are additional facility and professional charges associated with the administration procedure, as well as costs for the genetic test to confirm the presence of the RPE65 mutation.[2]

* Health benefit plans vary, but in general to be eligible for coverage a drug must be approved by the Food and Drug Administration (FDA), administered by a licensed health care professional, and medically necessary. Depending on the plan, customers may be required to use an in-network provider to receive coverage. Costs and complete details of coverage are set forth in the applicable plan documents.

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How many people are candidates for Luxturna in the United States?

According to Spark Therapeutics, it is estimated that between 1,000 and 2,000 people in the United States have vision loss due to RPE65-mediated inherited retinal disease. From a prevalence rate perspective, this equates to between 3.5 and 10 individuals per one million lives.[5]

How is Luxturna administered?

Luxturna is injected directly into each eye as a subretinal (i.e., under the retinal layer of the eye) injection. The administration to each eye occurs on separate days at least six days apart. Luxturna should be administered in a surgical suite by a surgeon experienced in performing intraocular surgery.[4]

Luxturna must be administered at a select treatment center in the United States by retinal surgeons, who will receive surgical education provided by Spark Therapeutics on the administration procedure. Luxturna is expected to be available for administration in these treatment centers late in the first quarter of 2018.

Where can people go to get treated with Luxturna?**

Treatments will occur in an outpatient setting. According to Spark Therapeutics, the following treatment centers in the United States will be designated to provide Luxturna.

- Bascom Palmer Eye Institute, University of Miami Health System
- Baylor University Medical Center
- Casey Eye Institute, Oregon Health and Science University
- Children's Hospital Los Angeles
- Children's Hospital of Philadelphia
- Cincinnati Children's Hospital Medical Center
- Massachusetts Eye and Ear
- Scheie Eye Institute, at Presbyterian Medical Center – University of Pennsylvania
- University of Iowa Hospitals and Clinics

This list of facilities designated by Spark Therapeutics is subject to change.[2]

What contracting and reimbursement options has Spark Therapeutics proposed for Luxturna?

In a January 3, 2018 press release, Spark Therapeutics communicated several financial options that they are offering relative to reimbursement of Luxturna.[6] As described in the Spark Therapeutics' **press release**, those options include:

- **Outcome-based contract arrangement with payer** – Will link payment to both short-term efficacy (30 to 90 days post-treatment) and longer-term durability (30 months) using vision improvement metrics.
- **Direct contracting with payer or specialty pharmacy** – The manufacturer is offering to payers an "Innovative Contracting Model" (ICM) that will allow the payer's specialty pharmacy to be the purchaser of Luxturna invoiced directly from Spark, thereby eliminating the risk of a facility markup via a "buy and bill" model. The specialty pharmacy will not actually purchase or distribute Luxturna, but will coordinate product ordering and ultimately the medication billing to Cigna.
- **Payment for Luxturna in installments over several years** – Spark is in discussions with Centers for Medicare & Medicaid Services (CMS) regarding this type of installment or financing option.
- **Patient assistance programs** – Spark will assist patients with coverage under a commercial health plan with options to support their travel and accommodation logistics to and from treatment centers, as well as with other out-of-pocket costs associated with treatment.

Cigna will provide an update on strategies it may undertake to appropriately manage the affordability of Luxturna.

How effective is Luxturna at treating RPE65-mediated inherited retinal disease?

Luxturna was studied in a total of 31 patients between the ages of 4 and 44 years (average age was 15 years) who had confirmed RPE65-mediated inherited retinal disease. The primary outcome of the study was 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 compared with the control group. This improvement in vision has been sustained out to two years after receiving the Luxturna treatment.[4]

** These facilities may not be in all Cigna plan networks and customers should review their plan's provider directory and plan documents to confirm network participation and details of coverage.

What safety issues are associated with Luxturna treatment?

The most common adverse reactions from treatment with Luxturna included eye redness, cataract, increased intraocular pressure and retinal tear.[4]

Is financial assistance for customers available from the manufacturer?

Spark established Spark Therapeutics Generations Patient Services to support eligible patients and their families. The team at Spark Therapeutics Generations Patient Services will assist eligible and enrolled patients and provide options to support travel and logistics to and from treatment centers.

More information will be available for patients and health care providers in the United States at **www.mysparkgeneration.com** or by calling **833.SPARK.PS (833.772.7577)**.

References

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