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5.90.033

Section:	Prescription Drugs	**Effective Date:**	April 1, 2025
Subsection:	Topical Products	**Original Policy Date:**	January 19, 2018
Subject:	Luxturna	**Page:**	1 of 4

Last Review Date: March 7, 2025

Luxturna

Description

Luxturna (voretigene neparvovec-rzyl)

Background
Luxturna (voretigene neparvovec-rzyl) is a gene therapy suspension for subretinal injection for the treatment of patients with a particular genetic cause of vision loss that can lead to blindness. More specifically, it is indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. This gene is responsible for making a protein essential for normal vision, however, these patients have a mutations in both copies of the gene, and over time lose their vision due to this mutation (1-2).

Regulatory Status
FDA-approved indication: Luxturna is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. Patients must have viable retinal cells as determined by the treating physician(s) (2).

The most common adverse reactions in the clinical trials were conjunctival hyperemia, cataract, increased intraocular pressure, retinal tear, dellen (thinning of the corneal stroma), macular hole, subretinal deposits, eye inflammation, eye irritation, eye pain, and maculopathy (wrinkling on the surface of the macula). Perform subretinal administration of luxturna to each eye on separate days within a close interval, but no fewer than 6 days apart (2).

Use in infants under 12 months of age is not recommended because of potential dilution or loss of Luxturna after administration to the active retinal cells proliferation occurring in this age group (2).

Safety and effectiveness in pediatric patients 12 months of age and older have been established (2).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Luxturna may be considered **medically necessary** if the conditions indicated below are met.

Luxturna may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 12 months of age or older

Diagnosis

Patient must have the following:

Biallelic RPE65 mutation-associated retinal dystrophy

AND ALL of the following:

1. Confirmation through genetic testing verifying both copies of the RPE65 gene are mutated
2. Viable retinal cells as determined by **ONE** of the following:
    a. Retinal thickness on spectral domain optical coherence tomography (OCT) with > 100 µm within the posterior pole
    b. Clinical exam that shows ≥3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole
3. If both eyes are to be treated, the initial eye's injection and the second eye's injection must be administered at least 6 days apart

Prior – Approval *Renewal* Requirements

None

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 1 injection per eye per lifetime

Prior – Approval *Renewal* Limits

None

Rationale

Summary

Luxturna (voretigene neparvovec-rzyl) is a subretinal gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. The patient must have viable retinal cells as determined by treating physician(s) for the use of this medication. Use in infants under 12 months of age is not recommended because of potential dilution or loss of Luxturna after administration to the active retinal cells proliferation occurring in this age group (2).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Luxturna while maintaining optimal therapeutic outcomes.

References

1. Genetics Home Reference: RPE65 gene, RPE65 retinoid isomerohydrolase. Lister Hill National Center for Biomedical Communications. U.S. National Library of Medicine. National Institutes of Health. Published: January 2, 2018. Website: https://ghr.nlm.nih.gov/gene/RPE65.
2. Luxturna [package insert]. Philadelphia, PA: Spark Therapeutics, Inc.; May 2022.

Policy History

Date	Action

January 2018	Addition to PA
March 2018	Annual editorial review Addition of viable retinal cells as determined by retinal thickness on spectral domain optical coherence tomography (OCT) [> 100 µm within the posterior pole] or by clinical exam (≥3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole) per SME
June 2018	Annual review
September 2019	Annual review
September 2020	Annual review
June 2021	Annual review
June 2022	Annual review
June 2023	Annual review and reference update. Changed policy number to 5.90.033
March 2024	Annual review
March 2025	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.