Luxturna (voretigene neparvovec-rzyl)

Background
Luxturna 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 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

Luxturna may be considered medically necessary for patients 12 months of age and older for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy and when the conditions indicated below are met.

Luxturna may be considered investigational in patients less than 12 months of age and 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
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 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**


### Policy History

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<tr>
<td>January 2018</td>
<td>Addition to PA</td>
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<tr>
<td>March 2018</td>
<td>Annual editorial review</td>
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<td>Addition of viable retinal cells as determined by retinal thickness on spectral domain optical coherence tomography (OCT) [&gt; 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</td>
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### Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 13, 2019 and is effective on October 1, 2019.