

Embarc Benefit Protection Policy

POLICY: Ophthalmology – Gene Therapy – Luxturna Embarc Benefit Protection Policy

- Luxturna® (voretigene neparvovec-rzyl subretinal injection – Spark Therapeutics)

DATE: 02/26/2025

OVERVIEW

Luxturna, an adeno-associated virus vector-based gene therapy, is indicated for the treatment of confirmed **biallelic human retinal pigment epithelial 65 kDa protein (RPE65) mutation-associated retinal dystrophy**.¹ Patients must have viable retinal cells as determined by the treating physician(s).

Luxturna is made up of a live, non-replicating adeno-associated virus serotype 2 which has been genetically modified to express the human RPE65 gene.¹ Luxturna is designed to deliver a normal copy of the gene encoding RPE65 to cells of the retina in patients with reduced or absent levels of biologically active RPE65. Treatment with Luxturna is not recommended for patients younger than 12 months of age, because the retinal cells are still undergoing cell proliferation, and Luxturna would potentially be diluted or lost during cell proliferation. The safety and effectiveness of Luxturna have not been established in geriatric patients. Clinical studies of Luxturna for this indication did not include patients ≥ 65 years of age.

Disease Overview

Inherited retinal dystrophies are a broad group of genetic retinal disorders that are associated with progressive visual dysfunction.² RPE65 mutation-associated retinal dystrophy is associated with numerous discrete gene mutations and affects 1,000 to 2,000 patients in the US. Mutations in the RPE65 gene lead to reduced or absent levels of RPE65 isomerohydrolase activity.¹ The absence of RPE65 leads to the accumulation of toxic precursors, damage to RPE-producing cells, and over time, damage to photoreceptors, progressing to near total blindness in most patients.

Dosing Information

The recommended dose of Luxturna for each eye is 1.5×10^{11} vector genomes (vg) administered once per eye by subretinal injection.¹ After completing a vitrectomy (removal of the vitreous gel that fills the eye cavity) and under direct visualization, a small amount of Luxturna is injected slowly until an initial subretinal bleb is observed; the remaining volume is then injected slowly until the total 0.3 mL is delivered. Luxturna should be injected into each eye on separate days within a close interval, but no fewer than 6 days apart.

POLICY STATEMENT

This policy involves the use of Luxturna. Prior Authorization is recommended for medical benefit coverage of Luxturna. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Dosing** for the diagnosis provided. All approvals are for one injection per eye.

Documentation: **Documentation is required** for all of the criteria elements below. Documentation may include, but is not limited to chart notes, laboratory tests, claims records, and/or other information.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Luxturna is recommended in those who meet the following criteria:

FDA-Approved Indication

1. Biallelic Human Retinal Pigment Epithelial 65 kDa Protein (RPE65) Mutation-Associated Retinal Dystrophy. Approve for one-time treatment course (i.e., a total of two injections, one injection in each eye) if the patient meets ALL of the following (A, B, C, D, and E):

- A) Patient has a genetically-confirmed diagnosis of biallelic RPE65 mutation-associated retinal dystrophy; AND
- B) Patient is ≥ 12 months of age and < 65 years of age; AND
- C) Luxturna is administered by a retinal specialist; AND
- D) Patient must have viable retinal cells as determined by the treating physician; AND
- E) Patient is not receiving re-treatment of eye(s) previously treated with Luxturna.

Dosing. Approve the following dosing regimen (A and B):

- A) One 1.5×10^{11} vector genomes (vg) injection administered by subretinal injection into each eye; AND
- B) The doses for the first eye and the second eye are separated by at least 6 days (i.e., injection of the second eye occurs 6 or more days after injection of the first eye).

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Luxturna is not recommended in the following situations:

- 1. **Re-treatment of previously treated eye(s).** Luxturna is for one time use in each eye. Repeat dosing in previously treated eye(s) is not approvable.
- 2. Coverage is not recommended for circumstances *not* listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

- 1. Luxturna® subretinal injection [prescribing information]. Philadelphia, PA: Spark Therapeutics; May 2022.
- 2. FDA news release. FDA approves novel gene therapy to treat patients with a rare form of inherited vision loss. Published on: December 19, 2017. Page last updated: March 16, 2018. Available at: <https://www.fda.gov/news-events/press-announcements/fda-approves-novel-gene-therapy-treat-patients-rare-form-inherited-vision-loss>. Accessed on February 21, 2025.

HISTORY

Type of Revision	Summary of Changes	Date
Annual Revision	Policy Name Change: The designation “Gene Therapy” was added to the policy title: Ophthalmology – Gene Therapy – Luxturna Embarc Policy. No criteria changes.	02/22/2023
Annual Revision	No criteria changes.	02/28/2024
Annual Revision	No criteria changes.	02/26/2025