

Luxturna™ (voretigene neparvovec-rzyl)

When requesting Luxturna™ (voretigene neparvovec-rzyl), the individual requiring treatment must be diagnosed with an FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

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.

Coverage Guidelines

The individual must meet **all** of the following criteria for approval:

- The diagnosis of retinal dystrophy is confirmed by genetic testing showing mutations in BOTH copies of the *RPE65* gene;
- Has viable retinal cells as determined by the treating physician;
- Is at least 1 year of age and less than 65 years of age;
- Luxturna will be administered by a retinal specialist; AND
- Has not previously received Luxturna therapy.

Approval: One-time treatment course (2 injections total, 1 injection in each eye).

Dosing Recommendation

The recommended dose of Luxturna for each eye is 1.5×10^{11} vector genomes (vg), administered by subretinal injection in a total volume of 0.3 mL.

The subretinal administration of Luxturna should be performed to each eye on separate days within a close interval, but no fewer than 6 days apart.

References

1. Luxturna® subretinal injection [prescribing information]. Philadelphia, PA: Spark Therapeutics; December 2019.
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, 2022.
3. Spark Therapeutics. Luxturna™ (voretigene neparvovec). FDA Advisory Committee Briefing Document. Meeting of the Cellular, Tissue, and Gene Therapies Advisory Committee. Meeting date: October 12, 2017. Available at: <https://www.fda.gov/media/108385/download>. Accessed on February 21, 2022.