

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 1 year of age or older;
- 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, Inc.; March, 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: December 21, 2017. Available at: <https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm589467.htm>. Accessed on February 8, 2019.
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/downloads/advisorycommittees/committeesmeetingmaterials/bloodvaccinesandotherbiologics/cellulartissueandgenetherapiesadvisorycommittee/ucm579300.pdf>. Accessed on February 8, 2019.
4. Voretigene Neparvovec-rzyl (Luxturna™). In: DRUGDEX® System Micromedex. Greenwood Village, CO: Thompson Reuters (Healthcare) Inc. [Updated; November 06, 2019; Accessed March 24, 2020].