voretigene neparvovec-rzyl (Luxturna)

Medical Benefit Drug Policy

Place of Service
Hospital Administration
Outpatient Facility Administration

Drug Details

USP Category: GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT
Mechanism of Action: Adeno-associated virus vector-based gene therapy
HCPCS:
J3398:Injection, voretigene neparvovec-rzyl, 1 billion vector genomes
How Supplied:
Each carton contains one single-dose vial of Luxturna [5 x 10^{12} vector genomes (vg) per mL] and two vials of Diluent

Condition(s) listed in policy *(see coverage criteria for details)*

- RPE65 Mutation-Associated Retinal Dystrophy

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the California Code of Regulations (CCR), Title 22, Section 51303 and 51313 must be met.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

Coverage Criteria
The following condition(s) require Prior Authorization/Preservice.

RPE65 Mutation-Associated Retinal Dystrophy
Meets medical necessity if all the following are met:

1. Prescribed by an ophthalmologist
2. Age is consistent with the FDA-approved indication
3. Patient has genetic laboratory documentation of two pathogenic or likely pathogenic variants in the RPE65 gene or one pathogenic or likely pathogenic variant on both alleles in the RPE65 gene
4. Patient has documentation for sufficient viable retinal cells
5. Patient has not received prior gene therapy for treatment of vision loss

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6. Being administered subretinally to each eye on separate days, at least 6 days apart

Covered Doses:
Up to 1.5 x 1011 vector genomes (0.3 mL) as a subretinal injection per affected eye

Coverage Period:
Once per eye per lifetime

ICD-10:
H35.50, H35.52, H35.54

Additional Information

- 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 to respond to the missing protein and restore visual function.

References

1. AHFS. Available by subscription at http://www.lexi.com
2. DrugDex. Available by subscription at http://www.micromedexsolutions.com/home/dispatch
3. Luxturna (voretigene neparvovec) [prescribing information]. Philadelphia, PA: Spark Therapeutics Inc; May 2022.
4. Mahajan VB, Bennett J, Maguire A et al. RPE65 Mutation Subtype Effect on Baseline Visual Function and Treatment Response in Phase 3 Voretigene Neparvovec Trial. Abstract P0220. Presented at the American Academy of Ophthalmology Annual Meeting, October 27–30, 2018, McCormick Place, Chicago, IL.

Review History

Date of Last Annual Review: 3Q2026
Changes from previous policy version:

- No change to policy following routine annual review

Blue Shield of California Medication Policy to Determine Medical Necessity
Reviewed by P&T Committee