**MVP Health Care Medical Policy**

**Medicare Part B: Luxturna**

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| **Type of Policy:** | **Drug Therapy** |
| **Prior Approval Date:** | **04/01/2025** |
| **Approval Date:** | **04/01/2026** |
| **Effective Date:** | **06/01/2026** |
| **Related Policies:** | **N/A** |

## Drug Requiring Prior Authorization (covered under the medical benefit)

J3398 Luxturna (voretigene neparvovec-rzyl) intraocular suspension

Refer to the MVP website for the Medicare Part D formulary for drugs that may be covered under the Part D benefit.

**Refer to relevant CMS LCDs/NCDs/Policy Articles for most up to date Medicare Part B guidance if available.**

## Overview/Summary of Evidence

Luxturna is an adeno-associated virus vector-based gene therapy indicated as an orphan drug for the treatment of patients with confirmed biallelic *RPE65* mutation-associated retinal dystrophy. Treatment with Luxturna includes one dose per eye per lifetime.

## Indications/Criteria

Luxturna will be considered for coverage when ALL the following are met:

- Prescribed and administered by an ophthalmologist or retinal surgeon with experience providing sub-retinal injections
- Member is at least 12 months of age but not greater than 64 years of age
- Member has a confirmed diagnosis of biallelic *RPE65* mutation-associated retinal dystrophy
- Chart notes document genetic testing to confirm mutation in both copies of the RPE65 gene
- Member must have viable retinal cells, as defined by:

    - an area in the retina within the posterior pole of greater than 100 µm thickness shown on OCT (optical coherence tomography): OR
    - ≥ 3-disc areas of retina without atrophy or pigmentary degeneration within the posterior pole; OR
    - remaining visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent
- Treatment with Luxturna must be done separately in each eye on separate days, with at least six days between surgical procedures
- The Member must not have had treatment with Luxturna previously in the intended eye
- The facility at which Luxturna is administered must be appropriately certified to do so. More information on this can be found here: https://luxturnahcp.com/about-luxturna/treatment-centers/

If approved, **coverage will be provided for a maximum of 1 injection per eye per lifetime.**
Coverage of lost, damaged, or mishandled product will not be covered.
Coverage is contingent on eligibility at the time of administration.

## Exclusions

- Age, dose, frequency of dosing, and/or duration of therapy outside of FDA approved package labeling
- Member is pregnant
- Member has previous administration of gene therapy vector
- Use of retinoid compounds or precursors that could potentially interact with the biochemical activity of the *RPE65* enzyme (individuals must discontinue use of these compounds for 18 months prior to Luxturna administration)
- Prior intraocular surgery within 6 months

## References

1. Luxturna (voretigene neparvovec-ryzel) prescribing information. Philadelphia, PA: Spark Therapeutics, Inc. 2017. Revised 05/2022.
2. A Safety and Efficacy Study in Subjects with Leber Congenital Amaurosis (LCA) Using Adeno-Associated Viral Vector to Deliver the Gene for Human RPE65 to the Retinal Pigment Epithelium (RPE) [AAV2-hRPE65v2-301]. Available online at: https://www.clinicaltrials.gov/ct2/show/NCT00999609?term=voretigene+neparvovec-rzyl&rank=1

3. 3. Russel S, Bennet J, Wellman JA, et al. Efficacy and safety of voretigene neparvovec (AAV2-hRPE65v2) in patients with RPE65-mediated inherited retinal dystrophy: a randomized, controlled, open-label phase 3 trial. Lancet 2017; 390:849-860.
4. Voretigene Neparvovec: A Review in RPE65 Mutation-Associated Inherited Retinal Dystrophy. Molecular Diagnosis & Therapy. 2020. Kang C, Scott LJ.
5. Voretigene Neparvovec for Inherited Retinal Dystrophy Due to RPE65 Mutations: A Scoping Review of Eligibility and Treatment Challenges From Clinical Trials to Real Practice. Eye. 2024. Testa F, Bacci G, Falsini B, et al.