

SPECIALTY GUIDELINE MANAGEMENT

LUXTURNA (voretigene neparvovec-rzyl)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

Luxturna is indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. Patients must have viable retinal cells as determined by the treating physician(s).

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Testing or analysis confirming a genetic diagnosis of pathogenic/likely pathogenic biallelic RPE65 gene mutations.

III. CRITERIA FOR INITIAL APPROVAL

Biallelic RPE65 mutation-associated retinal dystrophy

Authorization of 90 days for a one-time administration per eye may be granted for treatment of biallelic RPE65 mutation-associated retinal dystrophy when all of the following criteria are met:

- A. The member has bi-allelic pathogenic and/or likely pathogenic RPE65 mutations via genetic testing (single gene test or multi gene panel test if medically necessary).
- B. The RPE65 gene mutations classifications are based on the current American College of Medical Genetics and Genomics (ACMG) standards and guidelines for the interpretation of sequence variants.
- C. Pathogenic and/or likely pathogenic classification of the RPE65 mutations has been affirmed within the last 12 months.
- D. The member is at least 12 months of age but less than 65 years of age.
- E. The member has viable retinal cells in each eye to be treated as determined by optical coherence tomography (OCT) and/or ophthalmoscopy; and must have any of the following:
 1. An area of retina within the posterior pole of greater than 100 μm thickness shown on OCT
 2. Greater than or equal to 3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole
 3. Remaining visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent
- F. The member has not received a previous treatment course of Luxturna.

IV. REFERENCES

1. Luxturna [package insert]. Philadelphia, PA: Spark Therapeutics, Inc.; December 2017.

Reference number(s)
2458-A

2. 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.
3. Richards S, Aziz N, Bale S, et al; ACMG Laboratory Quality Assurance Committee. Standards and guidelines for the interpretation of sequence variants: A joint consensus recommendation of the American College of Medical Genetics and Genomics and the Association for Molecular Pathology. *Genet Med*. 2015;17(5):405-24.