Reference number(s)
2458-A

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[1]
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.

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.