Reference number(s)
2457-A

STANDARD MEDICARE PART B 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 will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
Testing or analysis confirming a genetic diagnosis of biallelic RPE65 gene mutations.

III. CRITERIA FOR INITIAL APPROVAL

Biallelic RPE65 mutation-associated retinal dystrophy
Authorization of 1 month may be granted for treatment of biallelic RPE65 mutation-associated retinal dystrophy when all of the following criteria are met:
A. The member has not received a previous treatment course of Luxturna.
B. The member has viable retinal cells in both eyes as determined by retinal thickness on spectral domain optical coherence tomography, fundus photography, and clinical examination.
C. The member must have either of the following in both eyes:
    1. Visual acuity of 20/60 or worse.
    2. Visual field less than 20 degrees in any meridian.

IV. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:
1. The prescribing information for Luxturna.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)

d. Lexi-Drugs
e. Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Luxturna are covered.

V. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information. Additional coverage requirements can be found in the published clinical trial for this drug. In the open-label, randomized, controlled phase 3 trial, individuals aged 3 years or older with, in each eye, best corrected visual acuity of 20/60 or worse, or visual field less than 20 degrees in any meridian, or both, with confirmed genetic diagnosis of biallelic RPE65 mutations, sufficient viable retina, and ability to perform standardized multi-luminance mobility testing (MLMT) within the luminance range evaluated, were eligible.

VI. REFERENCES

1. Luxturna [package insert]. Philadelphia, PA: Spark Therapeutics, Inc.; May 2022.
2. Russel S, Bennett 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.