Reference number(s)
4545-A

JURISDICTION SPECIFIC MEDICARE PART B

LUXTURNA (voretigene neparvovec-rzyl)

POLICY

I. COVERED USES

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

A. Retinitis pigmentosa
B. Leber congenital amaurosis

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:

A. Clinical documentation confirming diagnosis of Leber congenital amaurosis or retinitis pigmentosa including clinical features, funduscopic appearance, and results of testing such as dark-adapted thresholds, Ganzfeld-flash electroretinography (ERG), and when appropriate, perimetry. When performed, documentation of baseline (pre-treatment) white light FST per eye and baseline (pre-treatment) MLMT score should be included.
B. Documentation of positive genetic test result confirming a biallelic pathogenic or likely pathogenic RPE65 mutation (homozygote or compound heterozygote) by a MolDX-approved mutational test. Genetic testing showing the presence of a homozygous pathogenic mutation will be considered to meet this requirement. All results demonstrating 2 different pathogenic or likely pathogenic mutations on the RPE65 gene must be accompanied by segregation analysis results confirming biallelic involvement (trans configuration). A single heterozygous RPE65 mutation cannot be biallelic. If segregation analysis is not possible (e.g., in cases of adoption, absence of surviving biologic family members relevant for testing, refusal of genetic testing by relevant biological family members), documentation that the treating physician has determined that:
    1. The genetic sequencing test results match the member's phenotype with a high degree of specificity based on the scientific literature related to RPE65
    2. Other pathogenic variants associated with the member's phenotype have been ruled out or deemed less likely than biallelic RPE65 mutation- associated disease
C. Documentation that the member is 3 years of age or older
D. Documentation confirming sufficient viable photoreceptors in each eye planned for treatment by both ophthalmoscopy and 1 or more of the following:

1. Optical coherence tomography (OCT) thickness greater than 100 micrometers with presence of neural retina in the posterior pole
2. Great than 3 disc areas of retina free atrophy and/or pigmentary degeneration in the posterior pole
3. Intact visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent

E. Documentation of clinical presentation, duration of symptoms, progression, and any prior interventions including current visual function and supportive care management

F. No history of participation in a gene therapy study or treatment with the requested drug or other similar or dissimilar gene therapy product prior to treatment of the first eye

III. EXCLUSIONS

Coverage will not be provided for members with 2 pathogenic or likely pathogenic mutations involving only 1 copy of the RPE65 gene (cis configuration).

IV. PRESCRIBER SPECIALTIES

The requested drug must be administered by a qualified vitreoretinal surgeon with evidence of completion of the manufacturer's surgical and pharmacy training program for the appropriate storage, handling, and administration of the requested drug.

V. CRITERIA FOR APPROVAL

A. Retinitis pigmentosa

Authorization of 1 month for a single dose of the requested drug (1.5×10^{11} vector genomes) per eligible eye, per lifetime may be granted for the treatment of retinitis pigmentosa when both of the following are met:

1. The member has a confirmed biallelic RPE65 mutation.
2. Treatment of the contralateral eye will occur no sooner than 6 days and no later than 18 days after treatment of the first eye

B. Leber congenital amaurosis

Authorization of 1 month for a single dose of the requested drug (1.5×10^{11} vector genomes) per eligible eye, per lifetime may be granted for the treatment of Leber congenital amaurosis when both of the following are met:

1. The member has a confirmed biallelic RPE65 mutation.
2. Treatment of the contralateral eye will occur no sooner than 6 days and no later than 18 days after treatment of the first eye

VI. REFERENCES

1. VORETIGENE NEPARVOVEC-RZYL LCD (L37863) Version 5. Available at: https://www.cms.gov/medicare-coverage-database/indexes/national-and-local-indexes.aspx. Accessed December 14, 2021.
2. Billing and coding: voretigene neparvovec-rzyl (A56419) Version 6. Available at: https://www.cms.gov/medicare-coverage-database/indexes/national-and-local-indexes.aspx. Accessed December 14, 2021.
3. Luxturna [package insert]. Philadelphia, PA. Spark Therapeutics; December 2017.

Reference number(s)
4545-A