I. Medication Description

Voretigene neparvovec-rzyl is an adeno-associated virus vector-based gene therapy that delivers a normal copy of the gene encoding human retinal pigment epithelial 65 kDa protein (RPE65) to retinal cells thus augmenting reduced or absent levels of biologically active RPE65. The RPE65 gene mutations lead to reduced or absent levels of RPE65 isomerohydrolase activity, blocking the visual cycle and ultimately impairing vision.

II. Position Statement

Coverage is determined through a prior authorization process with supporting clinical documentation for all requests.

III. Policy

Coverage is provided for Luxturna when all of the following apply:

- Member is at least 12 months of age AND
- Medication is prescribed and administered by ophthalmologist or retinal surgeon with experience providing sub-retinal injections AND
- Medication will be administered in one of the manufacturer-designated Ophthalmic Gene Therapy Treatment Centers AND
- Diagnosis of biallelic RPE65 mutation-associated retinal dystrophy has been confirmed as determined by genetic testing for biallelic mutation of the RPE65 gene (supporting documentation must be provided) AND
- Documentation supporting the following must be provided:
  - Member has sufficient viable retinal cells as determined by all of the following:
    - Optical coherence tomography (OCT) confirming an area of retina within a posterior pole of >100 µm thickness
    - Fundus photography
    - Clinical examination AND
  - The member has ONE of the following in both eyes:
    - Visual acuity of 20/60 or worse OR
    - Visual field less than 20 degrees in any meridian AND
- Patient has not previously received RPE65 gene therapy in the intended eye(s)

IV. Quantity Limitations

One treatment course is allowed per lifetime:

- 1.5 x 10^{11} vector genomes (vg) in a total volume of 0.3 mL for each eye.
V.  **Coverage Duration**

Coverage may be granted for a 3 month period and may not be renewed.

VI.  **Coverage Renewal Criteria**

Coverage may not be renewed.

VII.  **Billing/Coding Information**

- Available as a suspension for subretinal injection, supplied in a 0.5 mL extractable volume in a 2 mL single dose vial; the supplied concentration (5 x 10^{12} vg/mL) requires a 1:10 dilution prior to administration.
- The administration of Luxturna to each eye is to be performed on separate days within a close interval, but no fewer than 6 days
- J3398: 1 billable unit = 1 billion vector genomes
- Pertinent indications:
  - Unspecified hereditary retinal dystrophy: H35.50
  - Pigmentary retinal dystrophy: H35.52
  - Dystrophies primarily involving the retinal pigment epithelium: H35.54

VIII.  **Summary of Policy Changes**

- 3/29/18: new policy
- 8/15/18: updated coverage criteria to require documentation supporting sufficient viable retinal cells and visual acuity/visual field parameters
- 12/13/18: updated billing/coding
- 1/3/19: updated billing/coding

IX.  **References**


*These guidelines are not applicable to benefits covered under Medicare Advantage. Medicare Advantage benefit coverage requests are reviewed in accordance with the guidance set forth in Chapter 15 Section 50 of the Centers for Medicare & Medicaid Services Medicare Benefit Policy Manual.

The Plan fully expects that only appropriate and medically necessary services will be rendered. The Plan reserves the right to conduct pre-payment and post-payment reviews to assess the medical appropriateness of the above-referenced therapies.

The preceding policy applies only to members for whom the above named pharmacy benefit medications are included on their covered formulary. Members with closed formulary benefits are subject to trying all appropriate formulary alternatives before a coverage exception for a non-formulary medication will be considered.

The preceding policy is a guideline to allow for coverage of the pertinent medication/product, and is not meant to serve as a clinical practice guideline.