Oscar Clinical Guideline: Luxturna (voretigene neparvovec-rzyl) (CG060, Ver. 6)

Luxturna (voretigene neparvovec-rzyl)

Disclaimer

Clinical guidelines are developed and adopted to establish evidence-based clinical criteria for utilization management decisions. Clinical guidelines are applicable according to policy and plan type. The Plan may delegate utilization management decisions of certain services to third parties who may develop and adopt their own clinical criteria.

Coverage of services is subject to the terms, conditions, and limitations of a member's policy, as well as applicable state and federal law. Clinical guidelines are also subject to in-force criteria such as the Centers for Medicare & Medicaid Services (CMS) national coverage determination (NCD) or local coverage determination (LCD) for Medicare Advantage plans. Please refer to the member's policy documents (e.g., Certificate/Evidence of Coverage, Schedule of Benefits, Plan Formulary) or contact the Plan to confirm coverage.

Summary

Luxturna was the first in vivo (within living cells) gene therapy approved by the FDA in December 2017 for children and adults with rare inherited vision disorders caused by mutated retinal pigment epithelium-specific 65kDa (RPE65) gene. For individuals who have mutations in both copies of the RPE65 gene (biallelic RPE65 mutation), they cannot make proteins in the eye that convert light, which is needed for normal vision. Luxturna is delivered as a single-dose, subretinal injection in each eye that works by targeting these mutations to create proteins again for light detection. Subretinal injection occurs after complete vitrectomy; therefore members first must receive a pars plana vitrectomy.

Definitions

"Biallelic Mutation" refers to a mutation in both copies of a particular gene which affects function of both copies.

"Gene therapy" is a technique that replaces a mutated gene with a healthy gene, inactivates a mutated gene, or introduces a new gene that helps fight against diseases and disorders. The gene therapy can be thought of as a genetic modification of DNA so as to produce a therapeutic effect. This is accomplished by the replacement of a mutated gene, addition of a missing gene or the modification of an existing one.

"Inherited retinal dystrophy/diseases" are a group of rare genetic eye diseases (inherited gene mutations that can cause vision loss and blindness). There are more than 220 different types of gene mutations that can cause vision loss.

"Mutation" refers to a change In the Initial DNA sequence of a particular gene. This change may prove to be harmful, beneficial or neutral in effect.

"Pars Plana Vitrectomy" is a surgical procedure undertaken by a retina specialist where the vitreous humor gel that fills the eye cavity is removed in the posterior segment to provide better access to the retina.

"Retina" refers to light sensitive tissue which Is located at the back of the eye and transmits Images along the optic nerve to the brain.

"Retinal pigment epithelium 65 (RPE65) gene" provides instructions to make proteins for normal vision in the eyes. When there is a mutation in the RPE65 gene, it causes the loss of ability for the eyes to detect light over time, e.g., Leber congenital amaurosis, Retinitis pigmentosa.

Medical Necessity Criteria for Authorization

The Plan considers **Luxturna (voretigene neparvovec-rzyl)** medically necessary when **ALL** of the following criteria are met:

1. The prescribing physician is an ophthalmologist or retinal surgeon experienced in performing intraocular surgery including subretinal injection; ***AND***
2. The member is at least three (3) years of age **AND** less than 65 years of age at the time of treatment; ***AND***
3. The member has a confirmed diagnosis of inherited biallelic RPE65 mutation-associated retinal dystrophy confirmed by genetic testing documenting biallelic mutations (both copies) of the RPE65 gene; ***AND***

4. There is evidence of viable retinal cells as confirmed by optical coherence tomography (OCT) and/or ophthalmoscopy showing **ONE** of the following:
    a. an optical coherence tomography showing more than a 100-micrometre thickness in an area of retina within the posterior pole; ***or***
    b. presence of three (3) or more disc areas without atrophy or pigmentary degeneration within the posterior pole; ***or***
    c. a remaining visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent; ***AND***
5. The member has not previously been treated with with Luxturna (voretigene neparvovec-rzyl) or other RPE65 gene therapy in the same eye; ***AND***
6. Luxturna (voretigene neparvovec-rzyl) is dosed at 1.5 x 10^{11} vector genomes (vg), administered by subretinal injection in a total volume of 0.3 mL for each eye on separate days within a close interval (i.e., 6-18 days between eyes), but no fewer than 6 days apart.

If the above criteria are met, Luxturna (voretigene neparvovec-rzyl) will be approved for one-time administration at the recommended dosing.

Please note:

1. *Approval is provided for one-time bilateral ocular administration only, in alignment with the product's FDA-approved labeling.*
2. *Retreatment with Luxturna (voretigene neparvovec-rzyl) is considered investigational, as safety and efficacy of repeat administrations have not been clinically established.*

Experimental or Investigational / Not Medically Necessary

Luxturna (voretigene neparvovec-rzyl) for any other indication is not considered medically necessary by the Plan. Experimental or investigational indications include, but are not limited to, the following:

- Repeat administration.
- Use in specific populations such as:
    - pregnant or breastfeeding women .
    - patients younger than 3 years or age 65 years and older.

Applicable Billing Codes (HCPCS/CPT Codes)

CPT/HCPCS Codes considered medically necessary if criteria are met:	
Code	*Description*
67028	Intravitreal injection of a pharmacologic agent (separate procedure)
J3398	Injection, voretigene neparvovec-rzyl, 1 billion vector genomes
ICD-10 codes considered medically necessary if criteria are met:	
Code	*Description*
H35.50	Unspecified hereditary retinal dystrophy
H35.52	Pigmentary retinal dystrophy
H35.54	Dystrophies primarily involving the retinal pigment epithelium

References

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12. Weleber RG, Pennesi ME, Wilson DJ, et al. Results at 2 years after gene therapy for RPE65-deficient Leber congenital amaurosis and severe early-childhood-onset retinal dystrophy. Ophthalmology. 2016;123(7):1606-1620.

Clinical Guideline Revision / History Information

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