



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.

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Summary

Luxturna (voretigene neparvovec-rzyl) was the first in vivo (within living cells) gene therapy approved by the FDA in December 2017 for children (12 months of age and older) and adults with biallelic retinal pigment epithelium-specific 65kDa (RPE65) gene-mutation associated retinal dystrophy, a rare inherited vision disorder. 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 (voretigene neparvovec-rzyl) 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. Injections of Luxturna (voretigene neparvovec-rzyl) should be performed on each eye, on separate days (but no fewer than 6 days apart).

Definitions

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

"Documentation" refers to written information, including but not limited to:

- Up-to-date chart notes, relevant test results, and/or relevant imaging reports to support diagnoses; or
- Prescription claims records, and/or prescription receipts to support prior trials of formulary alternatives.

"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.

“[s]” indicates state mandates may apply.

Clinical Indications

Medical Necessity Criteria for Clinical Review

General Medical Necessity Criteria

Biallelic RPE65 Mutation-Associated Retinal Dystrophy

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 12 months 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 (1) 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 Luxturna (voretigene neparvovec-rzyl) or other RPE65 gene therapy in the same eye; *AND*
6. Luxturna (voretigene neparvovec-rzyl) is dosed at 1.5×10^{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, the requested product will be authorized for a one-time administration at the recommended dosing per lifetime, with an approval duration of 6-months.^[s]

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 / Investigational, or unproven^[5]

Luxturna (voretigene neparvovec-rzyl) for any other use is not considered experimental, investigational, or unproven. Non-covered uses include, but are not limited to, the following:

- Repeat administration. Luxturna (voretigene neparvovec-rzyl) has only been approved for a one-time administration.
- Use in specific populations such as:
 - pregnant or breastfeeding women. There are no high-quality studies to support the safety and efficacy of Luxturna (voretigene neparvovec-rzyl) in pregnant or breastfeeding women.
 - patients younger than 12 months or age 65 years and older. There are no high-quality studies to support the safety and efficacy of Luxturna (voretigene neparvovec-rzyl) in those less than 12 months of age and those 65 years of age and older.

Applicable Billing Codes

Table 1	
CPT/HCPCS codes considered medically necessary if criteria are met:	
Code	Description
67028	Intravitreal injection of a pharmacologic agent (separate procedure)
67036	Vitrectomy, mechanical, pars plana approach;
67299	Unlisted procedure, posterior segment
J3398	Injection, voretigene neparvovec-rzyl, 1 billion vector genomes

Table 2	
ICD-10 diagnosis codes considered medically necessary with Table 1 (CPT/HCPCS) codes if criteria are met:	
Code	Description
H35.50	Unspecified hereditary retinal dystrophy
H35.52	Pigmentary retinal dystrophy

Table 2	
ICD-10 diagnosis codes considered medically necessary with Table 1 (CPT/HCPCS) codes if criteria are met:	
<i>Code</i>	<i>Description</i>
H35.54	Dystrophies primarily involving the retinal pigment epithelium

References

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Clinical Guideline Revision / History Information

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