Medical Policy
Voretigene (Luxturna)

Document Number: 056

	Commercial and Qualified Health Plans	MassHealth
Authorization required	X	
No Prior Authorization		X

Overview

Voretigene is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy.

Criteria

1. Criteria for Initial Approval
   - Member has confirmed genetic diagnosis of biallelic *RPE65* pathogenic variant associated retinal dystrophy
   - Member has confirmed evidence of viable retinal cells as determined by treating physician (identified by optical coherence tomography imaging and/or ophthalmoscopy)
     a) An area of retina within the posterior pole of >100 µm thickness shown on optical coherence tomography, OR
     b) ≥3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole, OR
     c) Any remaining visual field within 30° of fixation as measured by III4e/V4e isopter equivalent, OR
     d) Measurable full-field light sensitivity threshold (FST).
   - Member is ≥ 12 months and ≤ 64 years old
   - Member has not previously received the *RPE65* gene therapy in the intended eye

2. Concomitant Therapy
   - Systemic oral corticosteroids equivalent to prednisone at 1 mg/kg/day (maximum of 40 mg/day) for a total of 7 days (starting 3 days before administration of voretigene to each eye) and followed by a tapering dose during the next 10 days

3. Dosing and Administration
   - Subretinal administration of voretigene to each eye on separate days within a close interval, but no fewer than 6 days apart
   - 1.5 x 1011 vector genomes (vg), administered for each eye by subretinal injection in a total volume of 0.3 mL.

4. Duration of Therapy
   - Single administration in each eye

5. Monitoring
    - Safety monitoring at postoperative day 1, week 1, and month 1-2
    - Full-field light sensitivity threshold testing scores at baseline, 30-90 days, and at 30 months when available

6. Contraindications/Exclusions
    - Patient has had prior intraocular surgery (within 3 months)
    - Patient has an immunodeficiency (congenital or acquired)
    - Patient is female and pregnant or breastfeeding
    - Patient is using prescription retinoid compounds (or precursors) that may potentially interact with the activity of the *RPE65* enzyme (discontinued use for 3 months may become eligible)
    - Patient has preexisting eye condition(s) or complicating systemic diseases that would lead to eventual irreversible vision loss

CPT/HCPC Codes

Authorized Code	Code Description
J3398	Injection, voretigene neparvovec-rzyl, 1 billion vector genomes

Effective

December 2018: Effective date.

References

Spark Therapeutics. Voretigene neparovec-rzyl (Luxturna™) package insert. Philadelphia, PA. 2017.

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Maguire AM, Simonelli, F. et al. Safety and efficacy of gene transfer for Leber's congenital amaurosis *N Engl J Med* 2008;358(21):2240. Epub 2008 Apr 27.

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Russell 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;390:849-60.

Bennett J, Wellman J, Marshall KA, et al. Safety and durability of effect of contralateral-eye administration of AAV2 gene therapy in patients with childhood-onset blindness caused by RPE65 mutations: a follow-on phase 1 trial. *The Lancet* 2016;388(10045):661–72.

FDA Advisory Committee Briefing Document: Spark Therapeutics, Inc. Luxturna™ (voretigene neparvovec). 2017; https://www.fda.gov/downloads/advisorycommittees/committeesmeetingmaterials/bloodvaccinesandotherbiologics/cellulartissueandgenetherapiesadvisorycommittee/ucm579300.pdf. Accessed December 5, 2017