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**DATE:** November 21, 2019 N.L.: 10-1119  
Index: Benefits

**TO:** All County California Children's Services and Genetically Handicapped Persons Program Administrators, Medical Consultants, and Integrated Systems of Care Division Staff

**SUBJECT:** Voretigene Neparvovec-rzyl (Luxturna) – Authorization Criteria

## I. PURPOSE

The purpose of this Numbered Letter (N.L.) is to establish California Children's Services (CCS) Program policy regarding the authorization of voretigene neparvovec-rzyl (Luxturna), a gene therapy for treatment in adults and children with a confirmed biallelic retinal pigment epithelium 65 (RPE65) mutation-associated retinal dystrophy. Patients prescribed voretigene neparvovec<sup>1</sup> treatment must have viable retinal cells remaining. This must be determined by the prescribing ophthalmologist, prior to treatment.

## II. BACKGROUND

Biallelic RPE65 mutation-associated retinal dystrophy is an inherited form of progressive vision loss that may lead to complete blindness. It affects approximately 1,000 to 2,000 patients in the United States.<sup>2</sup> The RPE65 gene encodes for the RPE65 enzyme protein, an essential component of the retinal pigment epithelium, which allows the retina to respond to light. Mutations to the RPE65 gene leads to reduction or absence of this enzyme protein, leading to impaired and eventual vision loss.

The Food and Drug Administration (FDA) approved voretigene neparvovec on December 19, 2017. Voretigene neparvovec is FDA approved for patients age 12 months and older.

Voretigene neparvovec introduces a functional copy of the RPE65 gene into the retinal pigment epithelial cell, but does not repair the gene mutation. The process involves the following:

1. Vitrectomy (removal of the vitreous gel of the eye)

2. Subretinal injection of voretigene neparvovec into, or near, the macula by a retinal specialist. If bilateral treatment is needed, the second eye is normally treated at least 6 days after the first eye is treated, typically around 7 to 14 days apart. Treatment may be performed as an outpatient surgery.
3. Post-treatment clinic visit day one and seven after each treatment, and additional follow up visits as determined by the treating retinal specialist.

### III. POLICY

- A. Effective the date of this letter, voretigene neparvovec is a CCS Program benefit when the following criteria are met:
  1. Client meets program residential and financial eligibility criteria.
  2. Client has been diagnosed with biallelic RPE65 mutation-associated retinal dystrophy, with viable retinal cells, by a CCS-paneled treating retinal specialist.
  3. Client is under the care and monitoring of a CCS-paneled retinal specialist who has completed training in ocular gene therapy.
  4. Request is consistent with the FDA approved indication and dosage. At the time of this letter, recommended dose in each eye, administer  $1.5 \times 10^{11}$  vector genomes (vg) in a total volume of 0.3ml.
  5. Client is 12 months or older.
  6. Provider requests J3398: voretigene neparvovec, 1 billion vector genomes.
  7. Request is submitted with one of the following appropriate ICD-10-CM codes:
    - a. H35.50, Unspecified retinal dystrophy.
    - b. H35.52, Pigmentary retinal dystrophy.
    - c. H35.54, Dystrophies primarily involving the retinal pigment epithelium.
- B. If the criteria described above are not met, but the requester has clinical documentation and/or scientific evidence that may be relevant to the request, the provider may submit this additional documentation to the Integrated Systems of Care Division (ISCD) Medical Director or designee for consideration during the eligibility determination.

#### IV. POLICY IMPLEMENTATION

- A. Voretigene neparvovec is not covered by a Service Code Grouping authorization. Therefore, providers should submit a separate Service Authorization Request (SAR) for voretigene neparvovec.
- B. For non-Whole Child Model (WCM) independent counties, requests for initial and continuing voretigene neparvovec treatment will be reviewed and authorized by county CCS Programs.
- C. For dependent counties, requests for initial and continuing voretigene neparvovec treatment will be reviewed and authorized by the ISCD Special Populations Unit at [CCS\\_Operations@dhcs.ca.gov](mailto:CCS_Operations@dhcs.ca.gov), or to secure RightFax number, (916) 440-5768.
- D. For WCM counties, requests for initial and continuing voretigene neparvovec treatment will be reviewed and authorized by the Managed Care Plan (MCP) and requests for authorization should be directed to the appropriate county-specific MCP authorization unit.
- E. Requesting CCS Program providers must submit:
  - 1. A SAR along with:
    - a. A copy of the prescription or physician order from the CCS-paneled treating retinal specialist.
    - b. Medical and progress notes from the CCS-paneled treating retinal specialist documenting:
      - (1) Diagnosis of biallelic RPE65 mutation-associated retinal dystrophy with molecular pathology confirming biallelic RPE65 mutation.
      - (2) Status of retinal disease.
      - (3) The client has viable retinal cells based on clinical assessment by a CCS-paneled retinal specialist through objective measurements such as:
        - (a) Optical Coherence Tomography  $\geq 100\mu\text{m}$  retinal thickness in the posterior pole.
        - (b) Three disc areas or more of retina without atrophy or pigmentary degeneration by ophthalmoscopy/retinal photography.

- (c) 30 degrees or more remaining visual field on fixation by III43 isopter or equivalent.
  - (d) By findings from clinical exam of physician certified in administration of voretigene neparvovec.
2. Documentation establishing the CCS-paneled retinal specialist has completed training on ocular gene therapy, and that the location at which the CCS-paneled retinal specialist will be performing the procedure is listed as an Ocular Gene Therapy Treatment Center on the manufacturer's website.<sup>3</sup>
3. All claims for voretigene neparvovec treatment shall include:
- a. A completed copy of the UB-04 claim form.
  - b. A copy of manufacturer's payment invoice for voretigene neparvovec.
  - c. Healthcare Common Procedure Coding System Code J3398 = Injection, voretigene neparvovec, 1 billion vector genomes (vg) based on service date.

If you have any questions regarding this Numbered Letter, please contact the ISCD Medical Director or designee, via email at [ISCD-MedicalPolicy@dhcs.ca.gov](mailto:ISCD-MedicalPolicy@dhcs.ca.gov).

Sincerely,

**ORIGINAL SIGNED BY**

Roy Schutzengel  
Medical Director  
Integrated Systems of Care Division

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<sup>1</sup> FDA guidelines require biologic and biosimilar products contain a four-letter suffix following the active component of the product. This four-letter FDA assigned suffix has no clinical significance and is to differentiate between competing biologic and biosimilar products. The active component name, without the four-digit suffix, will designate intent to address the active drug, not a specific brand or product.

<sup>2</sup> FDA Announcement

<https://www.fda.gov/news-events/press-announcements/fda-approves-novel-gene-therapy-treat-patients-rare-form-inherited-vision-loss>

<sup>3</sup> Manufacturer's Website

<https://mysparkgeneration.com/hcp-support.html#TreatmentCenters>