Corporate Medical Policy: Voretigene Neparvovec-rzyl (Luxturna®) **"Notification"** **POLICY EFFECTIVE APRIL 1, 2026**

Restricted Product(s):

- voretigene neparvovec-rzyl (Luxturna®) subretinal injection for administration by a healthcare professional

FDA Approved Use:

- For the treatment of patients with confirmed biallelic *RPE65* mutation-associated retinal dystrophy. Patients must have viable retinal cells as determined by the treating physician(s).

Criteria for Medical Necessity:

The restricted product(s) may be considered medically necessary when the following criteria are met:

1. The patient is at least 12 months of age, and less than 65 years of age; **AND**
2. The patient has a diagnosis of biallelic *RPE65* mutation-associated retinal dystrophy **[medical record documentation required]**; **AND**
3. The diagnosis is confirmed by genetic testing demonstrating the presence of biallelic *RPE65* mutations **[medical record documentation required]**; **AND**
4. The patient has confirmed presence of viable retinal cells as determined by the treating physician(s), as assessed by optical coherence tomography imaging and/or ophthalmoscopy, defined by one of the following **[medical record documentation required]**:
    a. An area of retina within the posterior pole of > 100 µm thickness shown on optical coherence tomography **[medical record documentation required]**; **OR**
    b. Three or more disc areas of retina without atrophy or pigmentary degeneration within the posterior pole **[medical record documentation required]**; **OR**
    c. Remaining visual field within 30° of fixation as measured by III4e isopter or equivalent **[medical record documentation required]**; **AND**
5. The patient has not had prior intraocular surgery within the past 6 months **[medical record documentation required]**; **AND**
6. The patient does not have any pre-existing eye conditions or complicating systemic diseases that could alter ocular function and/or prohibit the planned procedure (e.g., malignancies requiring radiotherapy of the orbit; leukemia with central nervous system/optic nerve involvement) **[medical record documentation required]**; **AND**
7. The patient does not have any manifestations of advanced retinopathy (e.g., macular edema, proliferative changes) due to diabetes or sickle cell disease **[medical record documentation required]**; **AND**
8. The patient does not have acquired or congenital immunodeficiency **[medical record documentation required]**; **AND**

9. The requested dose is within FDA labeled dosing for the requested indication **[medical record documentation required]**.

Duration of Approval: 30 days (one injection per eye per lifetime)

Please note, for certain identified gene and cellular therapies such as voretigene neparvovec-rzyl (Luxturna®), when coverage is available and the individual meets medically necessary criteria, distribution from a specialty pharmacy provider due to cost (distribution channel restriction) may be required in order for coverage to be provided. **Please contact Blue Cross NC to coordinate this therapy.

FDA Label Reference				
Medication	**Indication**	**Dosing**	**HCPCS**	**Maximum Units***
voretigene neparvovec-rzyl (Luxturna®) subretinal injection	Confirmed biallelic *RPE65* mutation-associated retinal dystrophy in patients ≥ 12 months and < 65 years of age	1.5×10^{11} vector genomes (vg) per eye, administered by subretinal injection in a total volume of 0.3 mL; administration to each eye on separate days, no fewer than 6 days apart	J3398	300 (150 per eye)

***Maximum units allowed for duration of approval**

Other revenue codes that may be applicable to this policy: 0891, 0892

References: all information referenced is from FDA package insert unless otherwise noted below.

Policy Implementation/Update Information: Criteria and treatment protocols are reviewed annually by the Blue Cross NC P&T Committee, regardless of change. This policy is reviewed in Q4 annually.

April 2026: Coding change: Added the following applicable revenue codes associated with policy HCPCS code(s): 0891 (Special Processed Drugs – FDA Approved Cell Therapy) and 0892 (Special Processed Drugs – FDA Approved Gene Therapy). **Policy notification given 2/1/2026 for effective date 4/1/2026**.
December 2025: Criteria update: Minor formatting updates with no change to policy intent.
June 2021: Criteria change: Updated age requirement to ≥ 12 months per FDA labeling; removed requirement for no pregnancy or breastfeeding; medical policy formatting change. **Policy notification given 4/16/2021 for effective date 6/16/2021**.
*Further historical criteria changes and updates available upon request from Medical Policy and/or Corporate Pharmacy.