MEDICAL COVERAGE POLICY	
SERVICE:	**Voretigene Neparvovec-rzyl (Luxturna®)**
Policy Number:	**249**
Effective Date:	**09/01/2025**
Last Review:	**08/11/2025**
Next Review:	**08/11/2026**

Important note: *Unless otherwise indicated, medical policies will apply to all lines of business. Medical necessity as defined by this policy does not ensure the benefit is covered. This medical policy does not replace existing federal or state rules and regulations for the applicable service or supply. In the absence Aof a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan documents. See the member plan specific benefit plan document for a complete description of plan benefits, exclusions, limitations, and conditions of coverage. In the event of a discrepancy, the plan document always supersedes the information in this policy.*

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PRIOR AUTHORIZATION: Required.

POLICY: Please review the plan's EOC (Evidence of Coverage) or Summary Plan Description (SPD) for details.

For Medicare plans, please refer to appropriate Medicare NCD (National Coverage Determination) or LCD (Local Coverage Determination). If there are no applicable NCD or LCD criteria, use the criteria set forth below.

For Medicaid plans, please confirm coverage as outlined in the Texas Medicaid Provider Procedures Manual | TMHP (TMPPM). Texas Mandate HB154 is applicable for Medicaid plans.

BSWHP may find voretigene neparvovec-rzyl (Luxturna®) medically when documentation is submitted showing ALL of the following criteria are met:

1. Member has a diagnosis of a confirmed biallelic RPE65 mutation-associated retinal dystrophy (e.g. Leber's congenital amaurosis [LCA], retinitis pigmentosa [RP] early onset severe retinal dystrophy [EOSRD], etc.); **AND**
2. Member is between 12 months and 65 years of age; **AND**
3. Voretigene neparvovec-rzyl is prescribed and administered by ophthalmologist or retinal surgeon with experience providing sub-retinal injections; **AND**
4. Genetic testing results are supplied confirming biallelic mutations of the RPE65 gene; **AND**
5. Member has sufficient viable retinal cells as determined by the treating physician(s) using one of the following criteria:
   a) An area of retina within the posterior pole of >100 um thickness shown on optical coherence testing; **OR**
   b) Greater than or equal to 3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole; **OR**
   c) Remaining visual field within 30 degrees of fixation as measured by a lll4e isopter or equivalent (both eyes)

   AND
6. Member has had NO intraocular surgery within 6 months in either eye that is indicated for treatment; **AND**

7. Voretigene neparvovec-rzyl will be dosed and administered according to FDA approved labeling; **AND**
8. Provider attests member will use an designated treatment center; **AND**
9. Member has not previously received RPE65 gene therapy in intended eye; **AND**
10. Injection of second eye is at least 6 days from injection of the first eye.

BSWHP considers only ONE treatment per eye per lifetime is medically necessary as repeat administration of voretigene neparvovec is experimental and investigational because the effectiveness of this strategy has not been established.

BSWHP considers voretigene neparvovec for the treatment of all other indications to be experimental and investigational because the effectiveness of this strategy has not been established.

ALL requests for Voretigene Neparvovec-rzyl will be reviewed by both a clinical pharmacist and a medical director.

BACKGROUND:

Inherited retinal diseases (also called inherited retinal dystrophies or IRD) are a group of eye disorders caused by an inherited gene mutation including RPE65 that can result in vision loss or blindness. The RPE65 gene provides instructions for making a protein that is essential for normal vision produced in the retinal pigment epithelium (RPE), a thin layer of cells at the back of the eye. RPE65 gene mutations lead to a partial or total loss of RPE65 protein function. There is no cure for IRD. Patients with untreated RPE65-mediated IRD lose the ability to detect light of any intensity over time.

Voretigene neparvovec-rzyl (Luxturna®) is a gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. It is an adeno-associated virus 2 (AAV2) vector containing human RPE65 complementary DNA (cDNA) which is inserted as a functional gene into a cell. At this time there is insufficient evidence to draw firm conclusions regarding the safety and efficacy of Luxturna for the treatment of RPE65-mediated retinal dystrophies. There is only a single published phase III trial of 20 participants in this trial received treatment with voretigene. The primary outcome measure in this trial is the multi-luminance mobility test (MLMT), a novel functional vision test that has not been used as an efficacy endpoint in any other clinical studies. Although treated participants demonstrated improvements in mobility and light sensitivity compared with untreated participants, there was no statistically significant improvement in visual acuity in the intention-to-treat population.

CODES:

Important note: *Due to the wide range of applicable diagnosis codes and potential changes to codes, an inclusive list may not be presented, but the following codes may apply. Inclusion of a code in this section does not guarantee that it will be reimbursed, and patient must meet the criteria set forth in the policy language.*

CPT Codes:	

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HCPCS Codes:	J3398 - Injection, voretigene neparvovec-rzyl, 1 billion vector genomes
ICD10 codes:	H35.50 Unspecified hereditary retinal dystrophy
ICD10 Not covered:	

POLICY HISTORY:

Status	Date	Action
New	06/26/2018	New policy
Reviewed	02/26/2019	Code update
Reviewed	04/22/2020	Coverage criteria delineated
Updated	04/22/2021	Updated criteria applied by line of business
Reviewed	04/21/2022	Medicare instructions added
Reviewed	04/27/2023	No changes
Updated	09/28/2023	Updated Medicare and Medicaid instructions
Updated	08/12/2024	Applied new format and layout
Updated	08/11/2025	Added requirement of submitted documentation. Added requirement of attestation to use designated treatment center. Changed systemic corticosteroid requirement to dosing per FDA labeling. Rearranged criteria to standardized order. Updated lifetime treatment and experimental and investigational language. Background section simplified.
Update	8/11/2025	Removed, Medicare NCD/LCD Interqual statement for clarity.

REFERENCES:

The following scientific references were utilized in the formulation of this medical policy. BSWHP will continue to review clinical evidence related to this policy and may modify it at a later date based upon the evolution of the published clinical evidence. Should additional scientific studies become available, and they are not included in the list, please forward the reference(s) to BSWHP so the information can be reviewed by the Medical Coverage Policy Committee (MCPC) and the Quality Improvement Committee (QIC) to determine if a modification of the policy is in order.

1. FDA Label at: https://www.fda.gov/downloads/BiologicsBloodVaccines/CellularGeneTherapyProducts/ApprovedProducts/UCM589541.pdf
2. 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 randomised, controlled, open-label, phase 3 trial. Lancet. 2017 Aug 26;390(10097):849-860. doi: 10.1016/S0140-6736(17)31868-8. Epub 2017 Jul 14. PMID: 28712537
3. 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. Lancet. 2016;388(10045):661-672

4. 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 randomised, controlled, open-label, phase 3 trial. Lancet. 2017 Aug 26;390(10097):849-860. doi: 10.1016/S0140-6736(17)31868-8. Epub 2017 Jul 14
5. Maguire A, et.al. Efficacy, Safety, and Durability of Voretigene Neparvovec-rzyl in RPE65 Mutation-Associated Inherited Retinal Dystrophy. Ophthalmology 2019;126:1273-1285

***Note**:*
Health Maintenance Organization (HMO) products are offered through Scott and White Health Plan dba Baylor Scott & White Health Plan, and Scott & White Care Plans dba Baylor Scott & White Care Plan. Insured PPO and EPO products are offered through Baylor Scott & White Insurance Company. Scott and White Health Plan dba Baylor Scott & White Health Plan serves as a third-party administrator for self-funded employer-sponsored plans. Baylor Scott & White Care Plan and Baylor Scott & White Insurance Company are wholly owned subsidiaries of Scott and White Health Plan. These companies are referred to collectively in this document as Baylor Scott & White Health Plan.

RightCare STAR Medicaid plans are offered through Scott and White Health Plan in the Central Managed Care Service Area (MRSA) and STAR and CHIP plans are offered through SHA LLC dba FirstCare Health Plans (FirstCare) in the Lubbock and West MRSAs.