Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines:
Medical Benefit Drugs

Luxturna® (voretigene neparvovec-rzyl)

Effective: May 1, 2026

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☒ Tufts Health Plan Commercial products; Fax 617-673-0988

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth Accountable Care Partnership Plans; Fax 617-673-0939
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☐ Tufts Health One Care—A dual-eligible product; Fax 617-673-0956

Senior Products

☐ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☐ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☐ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Inherited retinal dystrophies are a group of eye disorders caused by a gene mutation. The RPE65 gene processes a type of vitamin A needed to keep light-sensing photoreceptor cells, the rods and cones of the retina, in operating order. An RPE65 gene mutation can lead to partial or total loss of RPE65 protein function, which can result in severe visual impairment beginning very early in life.

Gene therapy for treatment of RFE65 mutation-associated retinal disorders uses subretinal injection of viral vectors to insert therapeutic genes into viable retinal cells of a person with a known RPE65 mutation. Introduction of a normal allele can correct the mutated gene in the existing viable retinal cells and return these cells to normal functioning. Adeno-associated virus (AAV) is the safest and most effective viral vector. Voretigene neparvovec (Luxturna) is an adeno-associated virus 2 (AAV2) vector containing human RPE65 complementary DNA (cDNA) and to date, is the only FDA approved gene therapy for treatment of RPE65 inherited retinal disorders.

Food and Drug Administration (FDA) Approved Indications:

- LUXTURNA (voretigene neparvovec-rzyl) is an adeno-associated virus vector-based gene therapy indicated 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).

Clinical Guideline Coverage Criteria

An authorization of 90 days for a one-time administration per eye may be granted for LUXTURNA used in the treatment of biallelic RPE65 mutation-associated retinal dystrophy when **ALL** of the following are met:

1. Genetic testing confirms biallelic RPE65 mutation-associated retinal dystrophy.

AND

2. Prescriber is a specialist (e.g., ophthalmologist) or consultation notes from a specialist are provided

AND

3. The RPE65 gene mutations classifications are based on the current American College of Medical Genetics and Genomics (ACMG) standards and guidelines for the interpretation of sequence variants.

AND

4. Pathogenic and/or likely pathogenic classification of the RPE65 mutations has been affirmed within the last 12 months.

AND

5. The Member is at least 12 months of age but less than 65 years of age.

AND

6. The Member has documentation of sufficient viable retinal cells in each eye to be treated as determined by Optical Coherence Tomography (OCT) and/or ophthalmoscopy with any one of the following:
   a. An area of retina within the posterior pole of greater than 100 µm thickness shown on optical coherence tomography (OCT)
   b. Greater than or equal to three-disc areas of retina without atrophy or pigmentary degeneration within the posterior pole
   c. Remaining visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent

AND

7. The Member has not received a previous treatment course of prior gene therapy for biallelic RPE65 mutation-associated retinal dystrophy.

Limitations

The Plan will not cover:

- Treatment of infants under 12 months of age
- Treatment of Members aged 65 and older as safety and efficacy has not been established
- Repeat administration of Luxturna to same eye as safety and effectiveness of repeat administration has not been established
- All other indications are considered experimental/investigational and are not a covered benefit.

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J3398	Injection, voretigene neparvovec-rzyl, 1 billion vector genomes

References:

1. FDA Prescribing Information, sparktx.com/LUXTURNA_US_Prescribing_Information.pdf. 2017. Accessed April 14, 2025. Package Insert - LUXTURNA (fda.gov)
2. Russell S, Bennett J, 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. Erratum in: Lancet. 2017 Aug 26;390(10097):848. doi: 10.1016/S0140-6736(17)32235-3. PMID: 28712537; PMCID: PMC5726391.
3. Kang C, Scott LJ. Voretigene Neparvovec: A Review in RPE65 Mutation-Associated Inherited Retinal Dystrophy. Mol Diag Ther. 2020 Aug;24(4):487-495. doi: 10.1007/s40291-020-00475-6. PMID: 32535767.
4. Maguire AM, Russell S, et. al. Durability of Voretigene Neparvovec for Biallelic RPE65-Mediated Inherited Retinal Disease: Phase 3 Results at 3 and 4 Years. Ophthalmology. 2021 Oct;128(10):1460-1468. doi: 10.1016/j.ophtha.2021.03.031. Epub 2021 Mar 30. PMID: 33798654.
5. Deng C, Zhao PY, et. al. Real-world outcomes of voretigene neparvovec treatment in pediatric patients with RPE65-associated Leber congenital amaurosis. Graefes Arch Clin Exp Ophthalmol. 2022 May;260(5):1543-1550. doi: 10.1007/s00417-021-05508-2. Epub 2022 Jan 10. PMID: 35001204; PMCID: PMC9010358.
6. Retinitis pigmentosa: Treatment. UpToDate.com/login [via subscription only]. Published February 5, 2025. Accessed January 30, 2026.

Approval And Revision History

September 21, 2022: Reviewed by the Medical Policy Approval Committee (MPAC)

Subsequent endorsement date(s) and changes made:

- Originally approved at September 21, 2022 MPAC effective January 1, 2023
- Administrative update: April 2023 added Medical Benefit Drugs to title, updated MATogether and RITogether fax numbers to 617-673-0939
- April 19, 2023: Reviewed by MPAC, renewed without changes
- December 2023: Rebranded Unify to One Care effective January 1, 2024
- March 20, 2024: Reviewed by MPAC, renewed without changes, effective May 1, 2024
- July 22, 2024: Reviewed by MPAC, criteria clarified to require that the prescriber is an ophthalmologist, and clarified no prior gene therapies statement effective September 1, 2024
- March 19, 2025: Reviewed by MPAC, renewed without changes, effective April 11, 2025
- April 16, 2025: Reviewed by MPAC, RITogether added as an applicable product effective June 1, 2025.
- March 18, 2026: Reviewed by MPAC, renewed without changes, references updated, effective May 1, 2026.

Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. For Tufts Health Together (Medicaid), coverage may be available beyond these guidelines for pediatric members under age 21 under the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefits of the plan in accordance with 130 CMR 450.140 and 130 CMR 447.000, and with prior authorization.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.