Medical Necessity Guidelines:
Gene Therapy: Treatment of Inherited Retinal Disorders

Effective: March 18, 2020

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

Applies to:
COMMERCIAL Products
☒Tufts Health Plan Commercial products; Fax: 617.972.9409
☒Tufts Health Freedom Plan products; Fax: 617.972.9409
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

TUFTS HEALTH PUBLIC PLANS Products
☒Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax:888.415.9055
☒Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans; Fax: 888.415.9055
☒Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax: 857.304.6404
☐Tufts Health Unify* – OneCare Plan (a dual-eligible product); Fax: 857.304.6304
*The MNG applies to Tufts Health Unify members unless a less restrictive LCD or NCD exists

SENIOR Products
- Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product) – Refer to the Tufts Health Plan SCO Prior Authorization List
- Tufts Medicare Preferred HMO, (a Medicare Advantage product) – Refer to the Tufts Medicare Preferred HMO Prior Authorization and Inpatient Notification List

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 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.

CLINICAL COVERAGE CRITERIA

Tufts Health Plan will cover gene therapy, LUXTURNA™ (voretigene neparvovec), for the treatment of an inherited retinal disorder when ALL the following are met:

- Confirmed biallelic *RPE65* mutation-associated retinal dystrophy.
- Sufficient viable retinal cells, estimated by Optical Coherence Tomography (OCT) as an area of retina within the posterior pole of greater than 100 micron thickness[1].
- Treatment is provided at a designated Ocular Gene Therapy Treatment Center (https://mysparkgeneration.com/hcp-support.html#TreatmentCenters)
- Patient has not received *RPE65* gene therapy previously in eye for which requested treatment is intended.

[1] FDA Advisory Committee Briefing Document

Limitations

Tufts Health Plan will not cover:

- Treatment for infants under 12 months of age

References

1. U.S. Food and Drug Administration (FDA) News Release; FDA approves novel gene therapy to treat patients with a rare form of inherited vision loss. December 19, 2017
2. Seema, Garg. Retinitis pigmentosa: Treatment. UpToDate®. Last accessed December 20, 2017.
3. LUXTURNA (voretigene neparvovec-rzyl) [package insert]. Philadelphia, PA: Spark Therapeutics, Inc. December 2017.
4. FDA Advisory Committee Briefing Document. Sparks Therapeutics Inc. Luxturna (voretigene neparvovec). October 12, 2017.
5. Sengillo JD, Justus S, Tsai Y-T, Cabral T, Tsang SH. 2016. Gene and cell-based therapies for inherited retinal disorders: An update. Am J Med Genet Part C Semin Med Genet 172C:349–366.
6. FDA Prescribing Information, https://sparktx.com/LUXTURNA_US_Prescribing_Information.pdf accessed on March 23, 2020

Approval History

January 10, 2018: Reviewed by the Integrated Medical Policy Advisory Committee for an effective date of January 10, 2018.

Subsequent endorsement date(s) and changes made:

- October, 2018: Template and disclaimer upated
- March 20, 2019: Reviewed by IMPAC, renewed without changes
- March 18, 2020: Reviewed by IMPAC, renewed without changes
- March 24, 2020: Unify fax number updated

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.

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.

Provider Services