Pharmacy Medical Necessity Guidelines:
Juxtapid™ (lomitapide)

Effective: October 1, 2026

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RXUM
These pharmacy medical necessity guidelines apply to the following: ☒ Tufts Health RITogether – A Rhode Island Medicaid Plan		**Fax Numbers:** RXUM: 617.673.0939	

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND ADMINISTRATION-APPROVED INDICATIONS

Juxtapid (lomitapide) is a microsomal triglyceride transfer protein inhibitor indicated as an adjunct to a low-fat diet and exercise and other low-density lipoprotein cholesterol (LDL-C) therapies to reduce LDL-C in adult and pediatric patients aged 2 years and older with homozygous familial hypercholesterolemia (HoFH).

Juxtapid (lomitapide) has a REMS program consisting of elements to assure safe use and an implementation system for the program. The elements to assure safe use are training and certification of healthcare providers who prescribe Juxtapid (lomitapide), Dear Healthcare Provider and Dear Professional Society letters, the requirement that Juxtapid (lomitapide) be dispensed only by specially certified pharmacies, and the requirement that Juxtapid (lomitapide) be prescribed only to patients with HoFH who have had liver function tests measured. In addition, Juxtapid (lomitapide) has a medication guide educating patients about potential hepatotoxicity and teratogenic effects.

Familial hypercholesterolemia results mainly from autosomal dominant genetic defects in the LDL-C receptor, apo B, or proprotein convertase subtilisin kexin type 9 (PCSK9), all of which are involved in the normal processing and trafficking of LDL-C. It is estimated that one in every 500 individuals in the United States has heterozygous familial hypercholesterolemia, while one in one million individuals is affected by HoFH. Patients with HoFH carry two of the same defective genes, while patients with the heterozygous form of the condition carry one defective gene.

For patients with HoFH, plasma LDL-C levels are often five times greater than normal, and in a small sample of patients with HoFH, untreated TC levels were commonly between 700 mg/dL and 800 mg/dL. Other signs and symptoms of HoFH are a deposition of cholesterol (xanthomas) in the skin and tendons, especially the elbows, knees, Achilles tendon, and hands. Patients may also present with cholesterol deposits in the cornea (corneal arcus). Xanthomas may become apparent during childhood in patients with HoFH, and severe coronary artery disease resulting in myocardial infarction or requiring interventions such as coronary artery bypass grafting is often present by the age of 20 years.

COVERAGE GUIDELINES

Initial Coverage:

The plan may authorize coverage of **Juxtapid** (lomitapide) for members when **ALL** of the following criteria are met:

1. Documentation of diagnosis of homozygous familial hypercholesterolemia (HoFH) received by, or in written consultation with (documentation required), a lipid specialist (e.g., cardiologist, endocrinologist, lipid specialist/lipidologist)

AND

2. Confirmed diagnosis of homozygous familial hypercholesterolemia (HoFH) based on both of the following (a and b):
    a. Documented generic mutation at both alleles of at least one of the following genes by an FDA approved generic test: low-density lipoprotein receptor (LDLR), Apolipoprotein B (apo B), or Proprotein convertase subtilisin kexin type 9 (PCSK9)
    b. Recognized diagnostic criteria is met (documentation provided):
        i. Dutch Lipid Clinic criteria

OR

        ii. Simon Broome criteria

AND

3. Member is concurrently taking other lipid-lowering medications or provider indicates clinical inappropriateness to other lipid-lowering medications

AND

4. Member is 2 years of age or older

AND

5. The Member has demonstrated an inadequate response to an appropriate trial with or a contraindication and or intolerance to each of the following classes:
    a. Maximally tolerated statin
    b. PCSK9 Inhibitor

AND

6. Baseline LDL-C is provided

Reauthorization Criteria:

The plan may reauthorize coverage of **Juxtapid** (lomitapide) for members when **ALL** of the following criteria are met:

1. The member continues to meet all initial authorization criteria

AND

2. The member has achieved or maintained an LDL-C reduction while on medication

LIMITATIONS

1. Initial approval will be limited to 6 months. Reauthorization period is for a period of 12 months.
2. The plan does not cover Juxtapid (lomitapide) for patients with hypercholesterolemia who do not have homozygous familial hypercholesterolemia (HoFH).
3. Coverage of Juxtapid (lomitapide) is limited to 30 capsules per 30 days for all strengths.

CODES

None

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APPROVAL HISTORY

September 13, 2022: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. September 12, 2023: No changes.
2. September 10, 2024: Administrative change to fax number.
3. September 9, 2025: No changes
4. September 15, 2026: Effective October 1, 2026, updated overview and coverage criteria to reflect expanded indication in pediatric patients 2 years of age and older with HoFH.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be 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 the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy 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 Pharmacy 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 policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.

Provider Services