Pharmacy Medical Necessity Guidelines: Juxtapid™ (lomitapide)

Effective: November 10, 2020

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:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- 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)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

RXUM: 617.673.0988

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 other lipid-lowering treatments, including LDL apheresis where available, to reduce low-density lipoprotein cholesterol (LDL-C), total cholesterol (TC), apolipoprotein B (apo B), and non-high-density lipoprotein cholesterol (non-HDL-C) in patients with homozygous familial hypercholesterolemia (HoFH).

Limitations of Use

- The safety and effectiveness of Juxtapid (lomitapide) have not been established in patients with hypercholesterolemia who do not have HoFH, including those with heterozygous familial hypercholesterolemia.
- The effect of Juxtapid (lomitapide) on cardiovascular morbidity and mortality has not been determined.

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

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

1. Documentation is received confirming a clinical or laboratory diagnosis of homozygous familial hypercholesterolemia (HoFH)

AND

2. Medical records document that prior to the initiation of therapy, the member has been and will continue to follow a low-fat diet supplying less than 20% of energy from fat

AND

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

AND

4. Member is 18 years of age or older

AND

5. The Member has demonstrated an inadequate response to an appropriate trial with or a contraindication to Repatha (evolocumab)

LIMITATIONS

1. Initial approval will be limited to 6 months. A new prior authorization may be submitted at that time for continuation of therapy. Subsequent authorization requests may be given in 12-month intervals based on the submission of medical records documenting tolerance and effectiveness of therapy.
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

REFERENCES

1. Bell DA, Hooper AJ, Watts GF, et al. Mipomersen and other therapies for the treatment of severe familial hypercholesterolemia. *Vasc Health Risk Manag*. 2012;8:651-9.
2. Carroll MD, Kit BK, Lacher DA, et al. Total and high-density lipoprotein cholesterol in adults: National Health and Nutrition Examination Survey, 2011–2012. NCHS Data Brief, No. 132. Hyattsville, MD, National Center for Health Statistics; 2013.
3. Centers for Disease Control and Prevention. Vital signs: prevalence, treatment, and control of high levels of low-density lipoprotein cholesterol. United States, 1999–2002 and 2005–2008. *MMWR*. 2011;60(4):109-14.
4. Choudhry NK, Fischer MA, Avorn J, et al. The implications of therapeutic complexity on adherence to cardiovascular medications. *Arch Intern Med*. 2011;171(9):814-22.
5. Cuchel M, Meagher EA, du Toit Theron H, et al. Efficacy and safety of a microsomal triglyceride transfer protein inhibitor in patients with homozygous familial hypercholesterolemia: a single-arm, open-label, phase 3 study. *Lancet*. 2013 Jan 5;381(9860):40-6.
6. Garg A, Simha V. Update on dyslipidemia. *J Clin Endocrinol Metab*. 2007;92(5):1581-9.
7. Goldberg AC, Hopkins PN, Toth PP, et al. Familial hypercholesterolemia: screening, diagnosis and management of pediatric and adult patients: clinical guidance from the National Lipid Association Expert Panel on Familial Hypercholesterolemia. *J Clin Lipidol*. 2011; 5(3):133-40.
8. Gryn SE, Hegele RA. Pharmacogenomics, lipid disorders, and treatment options. *Clin Pharmacol Ther*. 2014;96(1):36-47.
9. Harada-Shiba M, Arai H, Oikawa S, et al. Guidelines for the management of familial hypercholesterolemia. *J Atheroscler Thromb*. 2012;19(12):1043-60.
10. Hopkins PN, Toth PP, Ballantyne CM, et al. Familial hypercholesterolemias: prevalence, genetics, diagnosis and screening recommendations from the National Lipid Association Expert Panel on Familial Hypercholesterolemia. *J Clin Lipidol*. 2011;5(Suppl 3):S9-17.
11. Ito MK, McGowan MP, Moriarty PM. Management of familial hypercholesterolemias in adult patients: recommendations from the National Lipid Association Expert Panel on Familial Hypercholesterolemia. *J Clin Lipidol*. 2011; 5(Suppl 3):S38-45.
12. Juxtapid (lomitapide) [prescribing information]. Cambridge, MA: Aegerion Pharmaceuticals, Inc.; 2020 September.
13. Juxtapid REMS Program. URL: juxtapidremsprogram.com. Available from Internet. Accessed 2017 Feb 22.

14. Nemati MH, Astaneh B. Optimal management of familial hypercholesterolemia: treatment and management strategies. *Vasc Health Risk Manag*. 2010;6:1079-88.
15. PCSK9. National Library of Medicine website. ghr.nlm.nih.gov/gene/PCSK9. Reviewed March 2007. Accessed 2017 Feb 22.
16. Repatha (evolocumab) [prescribing information]. Thousand Oaks, CA: Amgen Inc.; 2017 December.
17. Robinson JG. Management of familial hypercholesterolemia; a review of the recommendations from the National Lipid Association Expert Panel on Familial Hypercholesterolemia. *J Manag Care Pharm.* 2013;10(2);139-49.
18. Sabatine MS, Giugliano RP, Wiviott SD, et al. Efficacy and safety of evolocumab in reducing lipids and cardiovascular events. *N Engl J Med.* 2015;372:1500-9.
19. The Physician's Guide to Homozygous Familial Hypercholesterolemia (HoFH). Danbury, CT: National Organization for Rare Disorders; 2014. nordphysicianguides.org/homozygous-familial-hypercholesterolemia. Accessed 2017 Feb 22.

APPROVAL HISTORY

May 14, 2013: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

- July 9, 2013: Added documented confirmation of a clinical or laboratory diagnosis of HoFH
- July 8, 2014: Updated criteria #3: Member is concurrently taking other lipid-lowering medications or provider indicates clinical inappropriateness to other lipid-lowering medications
- July 14, 2015: Updated the quantity limitation to 28 capsules per 28 days due to recent launch of new higher strengths.
- January 1, 2016: Administrative change to rebranded template.
- March 8, 2016: Effective July 1, 2016 - added Repatha (evolocumab) prerequisite requirement.
- March 14, 2017: No changes
- April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
- March 13, 2018: No changes
- January 8, 2019: Effective 4/1/19, removed requirement for specific laboratory values to confirm diagnosis of HoFH and updated limitations section with quantity limitation to 30 capsules per 30 days for all available strengths.
- November 10, 2020: No changes

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.