Effective Date: 03/01/2021
Reviewed: 12/2020, 06/2021, 05/2022, 05/2023, 06/2024 Scope: Medicaid

JUXTAPID (lomitapide)

POLICY

I. CRITERIA FOR APPROVAL

An authorization of 6 months may be granted when all the following criteria are met:

A. Patient is 18 years or older; **AND**
B. Patient has documented diagnosis of Homozygous Familial Hypercholesterolemia confirmed by at least one of the following:
    a. Documented DNA test for functional mutation(s) in LDL receptor alleles or alleles known to affect LDL receptor functionality; **OR**
    b. Untreated LDL-C > 500 mg/dL or treated LDL-C ≥ 300 mg/dL; **AND**
        i. Cutaneous or tendon xanthoma before age 10 years; **OR**
        ii. Untreated LDL-C levels in both parents consistent with HeFH; **AND**
C. Medication is prescribed by, or in consultation with a cardiologist, lipidologist, or endocrinologist who is enrolled in the Juxtapid REMS program; **AND**
D. Patient has tried and failed at least a 3-month trial of adherent therapy with: ezetimibe used in combination with the highest available (or maximally tolerated*****) dose of atorvastatin OR rosuvastatin, unless contraindicated; **AND**
E. Patient has tried and failed at least a 3 month trial of adherent therapy with: combination therapy consisting of the highest available (or maximally tolerated*****) dose of atorvastatin OR rosuvastatin, ezetimibe, AND a PSCK9 inhibitor indicated for HoFH (e.g., evolocumab unless contraindicated; **AND**
F. Despite pharmacological treatment with a PCSK9 inhibitor, statin, and ezetimibe, the patient's LDL cholesterol ≥ 100 mg/dL (or ≥ 70 mg/dL for patients with clinical atherosclerotic cardiovascular disease [ASCVD]); **AND**
G. Patient has had an inadequate response or contraindication to Evkeeva (evinacumab); **AND**
H. Patient will not be using in combination with Evkeeva (evinacumab); **AND**
I. Patient does not have moderate or severe liver impairment (Child-Pugh B or C) or active liver disease.

*****If the patient is not able to use a maximum dose of atorvastatin or rosuvastatin due to muscle symptoms, documentation of a causal relationship must be established between statin use and muscle symptoms.

- Documentation must demonstrate that the patient experienced pain, tenderness, stiffness, cramping, weakness, and/or fatigue and all of the following:
    - Muscle symptoms resolve after discontinuation of statin; **AND**
    - Muscle symptoms occurred when re-challenged at a lower dose of the same statin; **AND**
    - Muscle symptoms occurred after switching to an alternative statin; **AND**
    - Documentation ruling out non-statin causes of muscle symptoms (e.g., hypothyroidism, reduced renal function, reduced hepatic function, rheumatologic disorders, such as polymyalgia rheumatica, steroid myopathy, vitamin D deficiency, or primary muscle disease); **OR**
- The patient has been diagnosed with rhabdomyolysis associated with statin use

The diagnosis should be supported by acute neuromuscular illness or dark urine **AND** an acute elevation in creatine kinase (usually > 5,000 IU/L or 5 times the upper limit of normal [ULN])

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II. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for all members who are tolerating treatment, meet all initial criteria and have achieved or maintained a LDL-C reduction greater than 20% from the levels immediately prior to initiation of treatment with Juxtapid.

III. QUANTITY LIMIT

- Juxtapid 5mg, 10mg, 20mg & 30mg 28 capsules per 28 days

IV. COVERAGE DURATION

- Initial: 6 months
- Continuation: 6 months

V. REFERENCES

1. Juxtapid [package insert]. Dublin, Ireland: Amryt Pharmaceuticals, Inc.; April 2022
2. Cuchel M, Raal FJ, Hegele RA, et al. Update on European atherosclerosis society consensus statement on homozygous familial hypercholesterolaemia: new treatments and clinical guidance. *Eur Heart J*. 2023;44(25):2277-2291.
3. 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 hypercholesterolaemia: a single-arm, open-label, phase 3 study. *Lancet*. 2013;381(9860):40-46.
4. McGowan MP, Hosseini Dehkordi SH, Moriarty PM, et al. Diagnosis and treatment of heterozygous familial hypercholesterolemia. *J Am Heart Assoc*. 2019; 8(24):e013225.
5. Bays HE, Jones PH, Orringer CE, et al. National Lipid Annual Summary of Clinical Lipidology 2016. *J Clin Lipidol 2016*;10(1 Suppl):S1-S43.
6. Lloyd-Jones DM, Morris PB, Ballantyne CM, et al. 2022 ACC Expert consensus decision pathway on the role of nonstatin therapies for LDL-cholesterol lowering in the management of atherosclerotic cardiovascular disease risk: A report of the American college of cardiologic solution set oversight committee. *J Am Coll Cardiol*. 2022;80(14):1366–1418.