Protocol for Juxtapid® (lomitapide)
Approved April 2021

Background:
JUXTAPID 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).

The safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH.

Criteria for approval:

1. Patient has a diagnosis of HoFH confirmed by one of the following:
    a. Genetic confirmation of two mutant alleles at the LDLR, Apo-B, PCSK9, or ARH adaptor
    b. protein gene locus; OR
    c. Skin fibroblast LDL receptor activity < 20% normal OR
    d. Pre-treatment LDL-C > 500 mg/d; OR
    e. Treated LDL-C greater than 300 mg/dL with one of the following:
        i. Cutaneous or tendon xanthoma before age of 10 years; OR
        ii. History of heterozygous familial hypercholesterolemia (HeFH) in both parents
2. Patient will not receive concurrent treatment with a PCSK-9 inhibitor for the same indication
3. Patient will be on concurrent treatment with another lipid lowering therapy (for example, statin, fibrate, nicotinic acid, ezetimibe, LDL-apheresis) unless contraindicated or non-tolerance
4. Medication is prescribed by or in consultation with, a cardiologist, lipid specialist or endocrinologist
5. Patient does not have any contraindication(s) to therapy, such as:
    a. Pregnancy
    b. Concomitant administration with moderate or strong CYP3A4 inhibitors
    c. Moderate or severe hepatic impairment (based on Child-Pugh category B or C)
    d. Active liver disease, including unexplained persistent elevations of serum transaminases
6. Medication is prescribed in accordance with Food and Drug Administration (FDA) established
7. indication and dosing regimens or in accordance with medically appropriate off-label indication
8. and dosing according to American Hospital Formulary Service, Micromedex, Clinical
9. Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer reviewed evidence
10. Weight must be received for drugs that have weight-based dosing.

Initial Approval: 3 months

Continuation of Therapy:

1. Clinical documentation (for example, chart notes, lab values) confirming reduction of LDL-C, total cholesterol (TC), and/or non-high-density lipoprotein-cholesterol (non-HDL-C) while on therapy will be required for continuation of therapy
2. Monitoring of liver enzymes (for example, ALT, AST).
3. Patient continues to receive other lipid-lowering therapy (for example, statin, ezetimibe, LDL apheresis).
4. Patient will not receive concurrent treatment with a PCSK-9 inhibitor for the same indication
5. Medication is prescribed by or in consultation with, a cardiologist, lipid specialist or endocrinologist
6. For dose increase requests, weight must be received for drugs that have weight-based dosing.
7. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Renewal Approval: 6 months

Note: Juxtapid (lomitapide) has a black box warning for risk of hepatotoxicity. Juxtapid can cause elevations in transaminases. Juxtapid also increases hepatic fat (hepatic steatosis) with or without concomitant increases in transaminases. Because of the risk of hepatotoxicity, Juxtapid is only available through a restricted program, Juxtapid REMS.

References:

1. Juxtapid [Packet Insert]. Amryt Pharmaceuticals DAC. Dublin, Ireland US; December 2020
2. Clinical Pharmacology (online database). Tampa FL: Gold Standard Inc.: 2019. Updated periodically
1. Panno MD et al. Lomitapide: a novel drug for homozygous familial hypercholesterolemia. Clin. Lipodol. (2014) 9(1), 19-32
2. Common Drug Review. Lomitapide (Juxtapid). Canadian Agency for Drugs and Technologies in Health; 2015 Jul. Accessed online at: https://www.ncbi.nlm.nih.gov/books/NBK362563/ February 12, 2021
3. American Heart Association Scientific Statement: The Agenda for Familial Hypercholesterolemia. Circulation 2015; 132: 2167-2192
4. Jellinger PS, Handelsman Y, Rosenblit PD, et al. American Association of Clinical Endocrinologists and American College of Endocrinology Guidelines for Management of Dyslipidemia and Prevention of Cardiovascular disease.
5. AACE 2017 Guidelines. Endocrine Practice Vol 23 (Suppl 2). April 2017.