Pharmacy Medical Necessity Guidelines: Ocaliva™ (obeticholic acid)

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 DRUG ADMINISTRATION (FDA)-APPROVED INDICATIONS

Ocaliva (obeticholic acid), a farnesoid X receptor agonist, is indicated for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as a monotherapy in adults unable to tolerate UDCA.

This indication is approved under accelerated approval based on a reduction in alkaline phosphatase (ALP). An improvement in survival or disease-related symptoms has not been established. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

PBC is a chronic cholestatic disease with a progressive course that may extend over many decades. The disease is thought to be caused by a combination of genetic predisposition and environmental triggers. Patients with early disease often present with symptoms of fatigue, pruritus, and jaundice. Very few patients present with complications of advanced disease which include portal hypertension, ascites, hepatic encephalopathy or esophageal variceal bleeding. Patients with PBC may also develop bone disease and hyperlipidemia over the course of the disease.

Most patients with PBC will have abnormal liver tests showing elevations in ALP. But the characteristic serologic hallmark of PBC is the antimitochondrial antibody (AMA), a highly disease-specific autoantibody that can found in 90 to 95% of patients. In addition, PBC is associated with specific bile duct pathology. Diagnosis of PBC can be made with confidence in patients with elevations in ALP and presence of AMA. A liver biopsy can be used to further substantiate the diagnosis if required, or to asses staging of the disease.

UDCA was the first drug FDA-approved for the treatment of PBC. Data from randomized trials and long-term observational studies demonstrate treatment with UDCA improves biochemical indices and delays histologic progression. The effects of UDCA on survival have been challenged but recent reports demonstrate favorable effects of treatment on long-term survival. Treatment guidelines recommend patients with PBC at any stage should be treated with UDCA (13-15 mg/kg/day) on a long-term basis. Response to UDCA treatment is based on the amount of ALP reduction that is achieved after one year of treatment. Patients with a suboptimal response to UDCA should be assessed for noncompliance, the development of superimposed liver disease, or coadministration with bile acid sequestrants. UDCA is associated with minimal side effects.

Patients with PBC who had a true suboptimal response to treatment with UDCA had limited alternative treatment options until the FDA-approval of Ocaliva (obeticholic acid). Ocaliva (obeticholic acid) is a farnesoid X receptor agonist indicated as monotherapy or in combination with UDCA for the treatment of PBC. Farnesoid X receptors are expressed in the liver and intestine and are key regulators of bile acid, inflammatory, fibrotic and metabolic pathways. Activation of farnesoid X receptors decreases the

intracellular hepatocyte concentrations of bile acids by suppressing their synthesis and increasing their transport out of hepatocytes. Therefore, as an agonist, Ocaliva (obeticholic acid) ultimately limits the overall size of the circulating bile acid pool while promoting choleresis, thus reducing hepatic exposure to bile acids. Clinical trial data demonstrates that treatment with Ocaliva (obeticholic acid) reduces ALP levels compared to placebo.

COVERAGE GUIDELINES

The plan may authorize coverage of Ocaliva (obeticholic acid) for Members when all of the following criteria are met:

1. Member is at least 18 years of age

AND

2. Documented diagnosis of primary biliary cholangitis confirmed by at least two of the following:
    a. Biochemical evidence of cholestasis with an elevated of alkaline phosphatase level for at least a duration of 6 months
    b. Presence of antimitochondrial antibodies (titer ≥1:80 by immunofluorescence or M2 positivity by enzyme immunoassay) or primary biliary cholangitis-specific antibodies (e.g., anti-GP210, anti-SP100)
    c. Histologic evidence of nonsuppurative destructive cholangitis and destruction of interlobular bile ducts on liver biopsy

AND

3. Documentation the Member has an alkaline phosphatase level ≥1.6 times the upper limit of normal prior to initiation of therapy.

AND

4. Documentation of one of the following:
    a. The Member has had an inadequate response to at least one year of therapy with ursodeoxycholic acid/ursodiol and that Ocaliva (obeticholic acid) will be administered in combination with ursodeoxycholic acid/ursodiol
    b. The Member is intolerant to ursodeoxycholic acid/ursodiol

LIMITATIONS

- Ocaliva (obeticholic acid) will not be authorized for the treatment of nonalcoholic steatohepatitis.
- Initial authorization will be approved for 6 months duration. Subsequent 12 month approvals will be authorized with documentation of at least a 15% reduction in alkaline phosphatase.
- The following quantity limits apply: 30 units per 30 days.

CODES

None

REFERENCES

1. European Association for the Study of the Liver. EASL Clinical Practice Guidelines: management of cholestatic liver diseases. *J Hepatol*. 2009;51:237-67.
2. Hirschfield GM, Mason A, Luketic V, et al. Efficacy of obeticholic acid in patients with primary biliary cirrhosis and inadequate response to ursodeoxycholic acid. Gastroenterology. 2015 Apr;148(4):751-61.
3. Lindor KD, Gershwin E, Poupon R, et al. Primary biliary cirrhosis. *Hepatology*. 2009;50:291-308.
4. Ocaliva (obeticholic acid) [prescribing information]. New York, NY: Intercept Pharmaceuticals, Inc.; February 2020.

APPROVAL HISTORY

July 12, 2016: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
2. July 11, 2017: No changes.
3. May 8, 2018: No changes.
4. March 12, 2019: No changes.
5. 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.