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Section:	Prescription Drugs	**Effective Date:**	October 1, 2024
Subsection:	Gastrointestinal Agents	**Original Policy Date:**	June 24, 2016
Subject:	Ocaliva	**Page:**	1 of 6

Last Review Date: September 6, 2024

Ocaliva

Description

Ocaliva (obeticholic acid)

Background
Ocaliva (obeticholic acid) is used for the treatment of primary biliary cholangitis (PBC) which is a disease that causes the small bile ducts in the liver to become inflamed, damaged, and ultimately destroyed. This causes the bile to remain in the liver, which damages the liver cells over time, and results in cirrhosis, or scarring of the liver. As cirrhosis progresses, and the amount of scar tissue in the liver increases, the liver loses its ability to function. Ocaliva increases bile flow from the liver and suppresses bile acid production in the liver, thus reducing the exposure of the liver to toxic levels of bile acids (1).

Regulatory Status
FDA-approved indication: Ocaliva, a farnesoid X receptor (FXR) agonist, is indicated for the treatment of adult patients with primary biliary cholangitis (PBC)

- without cirrhosis or
- with compensated cirrhosis who do not have evidence of portal hypertension,

either in combination with ursodeoxycholic acid (UDCA) with an inadequate response to UDCA or as monotherapy in patients unable to tolerate UDCA (1).

Ocaliva has a boxed warning for hepatic decompensation and failure in primary biliary cholangitis patients with cirrhosis. Ocaliva is contraindicated in PBC patients with decompensated cirrhosis, a prior decompensation event, or with compensated cirrhosis who have evidence of portal hypertension (e.g., ascites, gastroesophageal varices, persistent thrombocytopenia). In addition, Ocaliva has warnings regarding severe pruritus and reduction in HDL-C (1).

Ocaliva may cause liver-related adverse reactions including jaundice, worsening ascites, and primary biliary cholangitis flares. Patients should be monitored during treatment for elevations in liver biochemical tests, for the development of liver-related adverse reactions, and for changes in serum lipid levels. Physicians should weigh the potential risks against the benefits of continuing treatment with Ocaliva in patients who have experienced clinically significant liver-related adverse reactions. Ocaliva is contraindicated in patients with complete biliary obstruction and should not be used in these patients. Ocaliva should be discontinued in patients who develop complete biliary obstruction. For patients who do not respond to Ocaliva after 1 year at the highest recommended dosage that can be tolerated (maximum of 10 mg once daily), and who experience a reduction in HDL-C, weigh the potential risks against the benefits of continuing treatment (1).

The recommended starting dosage of Ocaliva is 5 mg orally once daily in adults who have not achieved an adequate response to an appropriate dosage of UDCA for at least 1 year or are intolerant to UDCA. If adequate reduction in alkaline phosphatase (ALP) and/or total bilirubin has not been achieved after 3 months of Ocaliva 5 mg once daily and the patient is tolerating Ocaliva, the dosage may be increased to 10 mg once daily. The maximum dosage is no more than 10 mg once daily. Initiation of therapy with Ocaliva 10mg once daily is not recommended due to an increased risk of pruritus (1).

The safety and effectiveness of Ocaliva in pediatric patients have not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Ocaliva may be considered **medically necessary** if the conditions indicated below are met.

Ocaliva may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age or older

Diagnosis

Patient must have the following:

1. Primary biliary cholangitis (PBC)

AND submission of medical records (e.g., chart notes, laboratory values) documenting **ONE** of the following:

a. Inadequate response
    i. History of a minimum of a 1 year trial of ursodeoxycholic acid (UDCA)
b. Intolerance
    i. An intolerance which is unable to be resolved with attempts to minimize the adverse effects where appropriate (e.g., dose reduction) with a history of a trial of ursodeoxycholic acid (UDCA)

AND submission of medical records (e.g., chart notes, laboratory values) documenting **ALL** of the following:

a. Ocaliva must be used in combination with UDCA in patients who are tolerant or used as monotherapy in patients who are unable to tolerate UDCA
b. Patient has **ONE** of the following:
    i. **NO** cirrhosis
    ii. Compensated cirrhosis with no evidence of portal hypertension
c. **NO** preliminary biliary obstruction prior to initiation of therapy and agreement to discontinue therapy if complete biliary obstruction develops
d. Physician agrees to frequently monitor patient during treatment for elevations in liver biochemical tests, development of liver-related adverse reactions, and for changes in serum lipid levels
e. Confirmation of diagnosis with elevated serum alkaline phosphatase level **AND ONE** of the following tests:
    i. Positive antimitochondrial antibody test
    ii. Liver biopsy
    iii. Ultrasound scan of liver

All approved requests are subject to review by a clinical specialist for final validation and coverage determination once all required documentation has been received. Current utilization, including samples, does not guarantee approval of coverage.

Prior – Approval *Renewal* Requirements

Age 18 years of age or older

Diagnosis

Patient must have the following:

1. Primary biliary cholangitis (PBC)

AND submission of medical records (e.g., chart notes, laboratory values) documenting **ALL** of the following:

a. Patient monitoring during treatment for elevations in liver biochemical tests, development of liver-related adverse reactions, and for changes in serum lipid levels
b. The physician has weighed the potential risks against the benefits of continuing treatment in patients experiencing clinically significant liver-related adverse reactions
c. Patient has **ONE** of the following:
    i. **NO** cirrhosis
    ii. Compensated cirrhosis with no evidence of portal hypertension
d. **NO** evidence of complete biliary obstruction
e. Confirmation of patient improvement with **ALL** of the following:
    i. Serum alkaline phosphatase (ALP) decrease of at least 15%
    ii. Total bilirubin level of ≤ 1.1 mg/dL for females and ≤ 1.5 mg/dL for males

All approved requests are subject to review by a clinical specialist for final validation and coverage determination once all required documentation has been received. Current utilization, including samples, does not guarantee approval of coverage.

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 90 tablets per 90 days

Duration 6 months

Prior – Approval *Renewal* Limits

Quantity 90 tablets per 90 days

Duration 12 months

Rationale

Summary

Ocaliva (obeticholic acid), a farnesoid X receptor (FXR) 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 monotherapy in adults unable to tolerate UDCA. Patients should be monitored during treatment for elevations in liver biochemical tests, for the development of liver-related adverse reactions, and for changes in serum lipid levels. The safety and effectiveness of Ocaliva in pediatric patients have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Ocaliva while maintaining optimal therapeutic outcomes.

References

1. Ocaliva [package insert]. New York, NY: Intercept Pharmaceuticals, Inc.; May 2022.

Policy History

Date	Action
June 2016	Addition to PA Addition of Managed PA
September 2016	Annual review
March 2017	Change in initiation duration from 3 months to 6 months
June 2017	Annual review
November 2017	Addition of a physician requirement to reduce the dosing to once or twice weekly for patients who progress to moderate or severe liver impairment and discontinue Ocaliva if liver injury is suspected and start patients on 5mg weekly who have moderate to severe liver impairment (Child-Pugh B and C), Revision to PA limits
March 2018	Annual review
March 2019	Annual editorial review and reference update

September 2019	Revised quantity limits to set strengths together and allow 10 mg tablets for initiation
October 2019	Revised requirements to require a baseline serum alkaline phosphatase level. Removed submission of medical records needed for certain requirements and removed trial of 1 year for intolerance to UDCA
December 2019	Annual review
March 2020	Annual review and reference update
June 2021	Revised indication and boxed warning. Removed requirements regarding dosing for patients with moderate to severe hepatic impairment. Added requirement that patient does not have cirrhosis or has compensated cirrhosis with no evidence of portal hypertension
September 2021	Annual review
September 2022	Annual review and reference update
June 2023	Annual review
June 2024	Annual review
September 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 6, 2024 and is effective on October 1, 2024.