BlueCross BlueShield Federal Employee Program

**OCALIVA**
(obeticholic acid)

## RATIONALE FOR INCLUSION IN PA PROGRAM

### 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).

**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.