Commercial/Healthcare Exchange PA Criteria

Effective: March 1, 2008

Prior Authorization: Ocaliva

Products Affected: Ocaliva (obeticholic acid tablets)

Medication Description:

Ocaliva 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.[1] Ocaliva was approved for this indication under accelerated approval based on reduction in alkaline phosphatase (ALP). An improvement in survival or PBC-related symptoms has not been established. The prescribing information notes that continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. Ocaliva is structurally similar to an endogenous bile acid, with the addition of an ethyl group in the 6-alpha position (6α-ethyl-CDCA), which makes it a 100-fold more potent agonist at the Farnesoid X receptor (FXR), a nuclear receptor expressed in the liver and intestine.[1-2] FXR is a key regulator of bile acid, inflammatory, fibrotic, and metabolic pathways.[1] Activation of FXR reduces the intracellular concentrations of bile acids in hepatocytes by suppressing de novo synthesis from cholesterol and by increased transport of bile acids out of the hepatocytes. In general, these mechanisms limit the amount of circulating bile acid, while promoting choleresis, and therefore reduce hepatic exposure to bile acids.

Covered Uses: Primary biliary cholangitis

Exclusion Criteria: Complete biliary obstruction, Patients with alcoholic hepatitis[1,3], Nonalcoholic Fatty Liver Disease (NAFLD), including Nonalcoholic Fatty Liver (NAFL) or Nonalcoholic Steatohepatitis (NASH) [1,4-7].

Required Medical Information: Documentation of diagnosis

Age Restrictions: Patient is 18 years of age or older

Prescriber Restrictions: Ocaliva is prescribed by or in consultation with a gastroenterologist, hepatologist, or liver transplant physician

Coverage Duration: 12 months

Other Criteria:

Approve Ocaliva for the coverage year if the patient meets the following criteria:

1. Patient has a diagnosis of primary biliary cholangitis (PBC) as defined by TWO of the following criteria (a, b, and/or c) according to the prescribing physician[8]:
   - **a)** Alkaline phosphatase (ALP) elevated above the upper limit of normal as defined by normal laboratory reference values; AND/OR
   - **b)** Positive anti-mitochondrial antibodies (AMAs); AND/OR
   - **c)** Histologic evidence of primary biliary cholangitis (PBC) from a liver biopsy; AND
2. Patient meets ONE of the following criteria[1] (a or b):

a) Patient has been receiving ursodiol therapy (e.g., ursodiol generics, Urso 250®,Urso Forte®, Actigall®) for ≥ 1 year and has had an inadequate response according to the prescribing physician; OR

b) According to the prescribing physician the patient is unable to tolerate ursodiol therapy.

References:

1. Ocaliva® tablets [prescribing information]. San Diego, CA: Intercept Pharmaceuticals Inc.; May 2016.
2. Intercept Pharmaceuticals. Obeticholic Acid. Briefing document for the Food and Drug Administration Gastrointestinal Drugs Advisory Committee. Meeting Date: April 7, 2016. Available at: http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/GastrointestinalDrugsAdvisoryCommittee/UCM494110.pdf. Accessed on April 21, 2016.
3. O'Shea RS, Dasarathy S, McCullough AJ, et al. Alcoholic liver disease: practice guideline by the American Association for the Study of Liver Diseases and the American College of Gastroenterology. Hepatology. 2010;51(1):307-328.
4. Neuschwander-Tetri BA, Loomba R, Sanyal AJ, et al. Farnesoid X nuclear receptor ligand obeticholic acid for non-cirrhotic, non-alcoholic steatohepatitis (FLINT): a multicenter, randomized, placebo-controlled trial. Lancet. 2015;385:956-965.
5. Ratziu V, Sanyal AJ, MacConell L, et al. REGENERATE: a phase 3, double-blind, randomized, long-term, placebo-controlled, multicenter study evaluating the safety and efficacy of obeticholic acid in subjects with nonalcoholic steatohepatitis [poster 488]. Presented at: the International Liver Congress 2016, the 51st Annual Meeting of the European Association for the Study of Liver Disease (EASL); Barcelona, Spain; April 13-17, 2016.
6. US National Institutes of Health. In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited 2016 May 17]. Available from: https://clinicaltrials.gov/ct2/results?term=obeticholic acid&Search=Search. Search term: obeticholic acid.
7. Chalasani N, Younassi Z, Lavine JE, et al. The diagnosis and management of non-alcoholic fatty liver disease: practice guideline by the American Association for the Study of Liver Diseases, American College of Gastroenterology, and the American Gastroenterological Association. Hepatology. 2012;55(6):2005-2023.
8. Lindor KD, Gershwin ME, Poupon R, et al. American Association for the Study of Liver Diseases (AASLD) practice guidelines: primary biliary cirrhosis. Hepatology. 2009;50(1):291-308.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	6/30/2016

2	Policy Update	CCI adoption of EH policy Removal from CCI PA to indication Policy	Reviewed covered uses and exclusion criteria to FDA label	4/27/2020