Pharmacy Management Drug Policy

SUBJECT: Ocaliva® (obeticholic acid)
POLICY NUMBER: PHARMACY-124
EFFECTIVE DATE: 01/2025
LAST REVIEW DATE: 03/06/2025

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☐ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☐ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☐ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Primary biliary cholangitis (PBC), formerly known as primary biliary cirrhosis, is a chronic liver disease resulting from progressive destruction of the bile ducts in the liver. Bile produced in the liver travels via these ducts to the small intestine where it aids in digestion and absorption of fats. When the ducts are destroyed, bile builds up in the liver causing inflammation and scarring (fibrosis). Over time, this can lead to cirrhosis and eventually liver failure.

Primary biliary cholangitis mainly affects adult women aged 40-70 years, but an increasing incidence in adult men worldwide has reduced the female-to-male ratio.[1] Compared with women, men with primary biliary cholangitis have more advanced stages of liver disease at diagnosis, an increased incidence of hepatocellular carcinoma, and worse prognosis.[2]

There is no cure for primary biliary cholangitis, however, there are medications that can help slow disease progression and manage symptoms. Initial therapy for all patients with PBC is ursodiol, a naturally occurring bile acid (ursodeoxycholic acid or UCDA) that helps move bile out of the liver and into the small intestine. Liver biochemical testing is performed in three to six months after initiating therapy and a determination of a biochemical response (i.e., normalization of alkaline phosphatase) is made after one year of UCDA treatment.[3] Unfortunately, approximately 35 percent of patients have an inadequate biochemical response after one year of UCDA, while 5 to 10 percent of patients are unable to tolerate UDCA.[4] These patients should be evaluated for subsequent treatment options.

On May 27, 2016, Ocaliva (obeticholic acid) was approved for the treatment of Primary Biliary Cholangitis (PBC) in combination with UDCA in adults with an inadequate response to UDCA, or as a single therapy in adults unable to tolerate UDCA. It was approved under accelerated approval based on a reduction in alkaline phosphatase (ALP). Continued approval of Ocaliva for this indication may be contingent upon verification of clinical benefit in a confirmatory trial.

On September 13, 2024, the FDA's Gastrointestinal Drugs Advisory Committee voted 13 to 1 with no abstentions that the benefits of obeticholic acid on clinical outcomes in patients with PBC could not be verified with available data from the post marketing requirement confirmatory trial 747-302 and the observational study 747-405. The committee also voted 10 to 1, with 3

abstentions, that obeticholic acid did not have a favorable benefit-risk assessment for use as a second-line treatment in the United Stated Prescribing Information population.

On November 12, 2024, the FDA declined full approval of Ocaliva. Ocaliva continues to be available in the U.S. under the accelerated approval status while the FDA continues to review safety data for the drug.

POLICY:

Based upon our criteria and assessment of the peer-reviewed evidence, the use of Ocaliva (obeticholic acid) has not been medically proven to be effective and, therefore, is considered **experimental/investigational** for the treatment of primary biliary cholangitis.

The justification for Ocaliva (obeticholic acid) to be considered investigational is as follows:

1. Based on our assessment of the peer-reviewed literature, there is inconclusive evidence that the drug has a definite positive effect on health outcomes.
2. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug, over time, leads to improvement in health outcomes (e.g., the beneficial effects of the service outweigh any harmful effects).
3. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug is at least as effective in improving health outcomes as established services or technologies.
4. Based upon our assessment of the peer-reviewed medical literature, there is inconclusive evidence that the drug provides improvement in health outcomes in standard conditions of medical practice, outside the clinical investigatory settings.

Refer to Corporate Medical Policy #11.01.03 Experimental or Investigational Services

POLICY GUIDELINES:

1. Prior authorization is contract dependent.
2. This policy does not apply to Medicare Part D and D-SNP. The drugs in this policy may apply to the following formularies: Commercial, Exchange, Child Health Plus, and Essential Plan. If a drug referenced in this policy is non-formulary, please reference the Non-Formulary Medication Exception Review Policy for all Lines of Business (Pharmacy-69).
3. Clinical documentation must be submitted for each request (initial and recertification) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments/treatment history, diagnostic testing, laboratory testing results, genetic testing/biomarker results, and imaging.
    - Continued approval at the time of recertification will require documentation that the drug is providing ongoing benefit to the patient in terms of improvement or stability in disease state or condition.
4. All requests will be reviewed to ensure they are being used for an appropriate indication and may be subject to an off-label review in accordance with our Off-Label Use of FDA Approved Drugs Policy (Pharmacy-32).
5. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract.

CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.
Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

Code Key:
Experimental/Investigational = (E/I),
Not medically necessary/ appropriate = (NMN).

HCPCS:

UPDATES:

Date	Revision
03/06/2025	Revised
01/01/2025	Created and Implemented
11/21/2024	P&T Committee Approval

REFERENCES:

1. Tanaka A, Ma X, Takahashi A, Vierling JM. Primary biliary cholangitis. Lancet. 2024;404:1053-1066
2. Colapietro F, Bertazzoni A, Lleo A. Contemporary epidemiology of primary biliary cholangitis. Clin Liver Dis 2022; 26:555-570
3. Murillo Perez CF, Harms MH, Lindor KD, et al. Goals of Treatment for Improved Survival in Primary Biliary Cholangitis: Treatment Target Should be Bilirubin Within the Normal Range and Normalization of Alkaline Phosphatase. Am J Gastroenterol 2020;115(7):1066-1074
4. Kumagi T, Hansen BE, de Vries RA, et al. Improved prognosis of patients with primary biliary cirrhosis that have a biochemical response to ursodeoxycholic acid. Gastroenterology 2009;136:1281
5. Lindor KD, Bowlus CL, Boyer, J, Levy C, Mays M. Primary Biliary Cholangitis: 2018 Practice Guidance from the American Association for the Study of Liver Diseases. Hepatology 2019; 69(1): 394-419
6. Ocaliva® (obeticholic acid) tablets [prescribing information]. Morristown, NJ: Intercept Pharmaceuticals, Inc.; Revised May 2022. Accessed December 10,2024.