Reference number(s)
2029-A

SPECIALTY GUIDELINE MANAGEMENT

OCALIVA (obeticholic acid)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
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. 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.

All other indications are considered experimental/investigational and are not a covered benefit.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. For initial requests: Pretreatment serum alkaline phosphatase (ALP) level
B. For continuation of therapy: Current serum alkaline phosphatase (ALP) and/or current total bilirubin level

III. CRITERIA FOR INITIAL APPROVAL

Primary biliary cholangitis (PBC) (previously known as primary biliary cirrhosis)
Authorization of 6 months may be granted for treatment of PBC in members 18 years of age or older when all of the following criteria are met:
A. Diagnosis of PBC is confirmed by at least two of the following three criteria:
    1. Biochemical evidence of cholestasis with elevation of alkaline phosphatase (ALP) level for at least 6 months duration
    2. Presence of antimitochondrial antibodies (AMA) (titer >1:40 by immunofluorescence or immunoenzymatic reactivity) or PBC-specific antinuclear antibodies (ANA) (eg, anti-gp210, anti-sp100)
    3. Histologic evidence of PBC on liver biopsy (eg, non-suppurative inflammation and destruction of interlobular and septal bile ducts)
B. Member has an elevated serum ALP level prior to initiation of therapy with obeticholic acid
C. Member meets at least one of the following requirements:
    1. Inadequate response to at least 12 months of prior therapy with ursodeoxycholic acid (UDCA)/ursodiol and the member will continue concomitant therapy with UDCA/ursodiol, or
    2. Intolerance to UDCA/ursodiol

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for members who have achieved or maintained a clinical benefit from Ocaliva therapy (i.e., at least a 15% reduction in ALP level, ALP level less than 1.67-times ULN, or total bilirubin less than or equal to ULN).

V. REFERENCES

1. Ocaliva [package insert]. New York, NY: Intercept Pharmaceuticals, Inc.; February 2018.
2. Lindor KD, Gershwin E, Poupon R, et al. Primary biliary cirrhosis. *Hepatology.* 2009;50:291-308.
3. European Association for the Study of the Liver. EASL Clinical Practice Guidelines: management of cholestatic liver diseases. *J Hepatol.* 2017;67:145-172.