

## SPECIALTY GUIDELINE MANAGEMENT

### CHOLBAM (cholic 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 Indications

Cholbam is indicated for:

1. Treatment of bile acid synthesis disorders due to single enzyme defects (SEDs)
2. Adjunctive treatment of peroxisomal disorders (PDs) including Zellweger spectrum disorders in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption

*Limitation of use: The safety and effectiveness of Cholbam on extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders have not been established.*

All other indications are considered experimental/investigational and not medically necessary.

##### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- A. Initial requests:
  1. Mass spectrometry, enzyme assay, biochemical testing results, or genetic testing results confirming diagnosis; and
  2. Lab test results documenting baseline liver function (i.e., transaminases, bilirubin, presence of cholestasis).
- B. Continuation of therapy requests: lab results documenting an improvement in liver function (i.e., reduced transaminases, reduced bilirubin, no evidence of cholestasis on liver biopsy).

##### III. CRITERIA FOR INITIAL APPROVAL

##### A. Bile acid synthesis disorders due to single enzyme defects (SEDs)

Authorization of 6 months may be granted for treatment of bile acid synthesis disorders due to single enzyme defects when both of the following criteria are met:

1. The diagnosis is confirmed by mass spectrometry or other biochemical testing, genetic testing, or enzyme assay.
2. The member has liver dysfunction (i.e., elevated transaminases, bilirubin, presence of cholestasis) at baseline.

##### B. Peroxisomal disorders (PDs) including Zellweger spectrum disorders

Authorization of 6 months may be granted for adjunctive treatment of peroxisomal disorders when the diagnosis is confirmed by mass spectrometry or other biochemical testing or genetic testing, and the member exhibits manifestations of liver disease.

<b>Effective Date: 04/01/2022</b>
Reviewed: 01/2022, 01/2023
Scope: Medicaid

#### IV. CONTINUATION OF THERAPY

##### A. Bile acid synthesis disorders due to single enzyme defects (SEDs)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for bile acid synthesis disorders due to single enzyme defects who have achieved and maintained improvement in liver function (i.e., reduced transaminases, reduced bilirubin, no evidence of cholestasis on liver biopsy).

##### B. Peroxisomal disorders (PDs) including Zellweger spectrum disorders

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for adjunctive treatment of peroxisomal disorders with Cholibam who have achieved and maintained improvement in liver function (i.e. reduced transaminases, reduced bilirubin, no evidence of cholestasis on liver biopsy).

#### IV. REFERENCES

1. Cholibam [package insert]. San Diego, CA: Retrophin, Inc.; November 2021.
2. Gonzales E, Gerhardt MF, Fabre M et al. Oral cholic acid for hereditary defects of primary bile acid synthesis: a safe and effective long-term therapy. *Gastroenterology*. 2009;137:1310-1320.
3. Heubi J, Setchell KDR, Bove KE. Inborn errors of bile acid metabolism. *Seminars Liver Dis*. 2007;27:282-294.
4. Poll-The BT, Gartner J. Clinical diagnosis, biochemical findings and MRI spectrum of peroxisomal disorders. *Biochim Biophys Acta*. 2012;1822:1421-1429.