Clinical Policy: Cholic Acid (Cholbam)

Reference Number: PA.CP.PHAR.390
Effective Date: 10.17.18
Last Review Date: 10.17.18

Revision Log

Description
Cholic acid (Cholbam®) is a bile acid.

FDA Approved Indication(s)
Cholbam is indicated for:

- Treatment of bile acid synthesis disorders due to single enzyme defects (SEDs)
- 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(s) 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.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with PA Health & Wellness® that Cholbam is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Bile Acid Synthesis Disorders or Peroxisomal Disorders (must meet all)**:**

1. Diagnosis of one of the following (a or b):
   a. Bile acid synthesis disorders due to SEDs;
   b. PDs, including Zellweger spectrum disorders;
2. Prescribed by or in consultation with a hepatologist or gastroenterologist;
3. Dose does not exceed 17 mg/kg/day.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53.

II. Continued Therapy

A. All Indications in Section I (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy;

3. If request is for a dose increase, new dose does not exceed 17 mg/kg/day.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.LTSS.PHAR.01) applies.
   Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53.

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy – PA.CP.PMN.53.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
PDs: peroxisomal disorders
SEDs: single enzyme defects

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings
None reported

Appendix D: General Information

- Treatment should be initiated and monitored by a hepatologist or pediatric gastroenterologist.
- Discontinue Cholbam if liver function does not improve within 3 months of starting treatment or complete biliary obstruction develops.
- Discontinue treatment with Cholbam at any time if there are persistent clinical or laboratory indicators of worsening liver function or cholestasis.
- 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.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Bile acid synthesis disorders due to SED, PD including Zellweger spectrum disorders	10 to 15 mg/kg/day administered PO in one or two divided doses	17 mg/kg

Indication	Dosing Regimen	Maximum Dose
	For concomitant familial hypertriglyceridemia: 11 to 17 mg//kg/day PO in one or two divided doses	

VI. Product Availability

Capsules: 50 mg, 250 mg

VII. References

1. Cholbam Prescribing Information. San Diego, CA: Retrophin, Inc.; March 2015. Available at: www.cholbam.com. Accessed August 14, 2018.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy Created	10/18	