

Pharmacy Policy Bulletin

Title: Cholic acid (Cholbam®)

Policy #: Rx.01.171

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

▸ Intent:

The intent of this policy is to communicate the medical necessity criteria for cholic acid (Cholbam®) as provided under the member's prescription drug benefit.

▸ Description:

Bile acid synthesis disorders (BASD) are extremely rare, genetic, metabolic conditions that exhibit manifestations of liver disease, steatorrhea, and complications from decreased fat soluble vitamin absorption. Individuals with BASD lack the enzymes needed to synthesize cholic acid. If untreated, these individuals fail to grow and can develop life-threatening liver injury.

Cholic acid is a primary bile acid synthesized from cholesterol in the liver. In bile acid synthesis disorders due to single enzyme deficiencies (SEDs) in the biosynthetic pathway, and in peroxisomal disorders (PDs) including Zellweger spectrum disorders, deficiency of primary bile acids leads to unregulated accumulation of intermediate bile acids and cholestasis. Bile acids facilitate fat digestion and absorption by forming mixed micelles, and facilitate absorption of fat-soluble vitamins in the intestine.

Endogenous bile acids, including cholic acid, enhance bile flow and provide the physiologic feedback inhibition of bile acid synthesis. The mechanism of action of cholic acid has not been fully established; however, it is known that cholic acid and its conjugates are endogenous ligands of the nuclear receptor, farnesoid X receptor (FXR). FXR regulates enzymes and transporters that are involved in bile acid synthesis and in the enterohepatic circulation to maintain bile acid homeostasis under normal physiologic conditions.

Cholic acid (Cholbam®) is indicated for:

- A. The treatment of BASD due to SEDs

- B. Adjunctive treatment of PDs including Zellweger spectrum disorders, in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption.

Treatment with cholic acid (Cholbam®) is approved for children aged 3 weeks and older, and adults.

▸ **Policy:**

INITIAL CRITERIA

Cholic acid (Cholbam®) is approved when ALL of the following are met:

- A. One of the following:
- B. Prescribed by a hepatologist or gastroenterologist; and
 - 1. Treatment of bile acid synthesis disorder due to single enzyme defect; or
 - 2. Adjunctive treatment of peroxisomal disorder including Zellweger spectrum disorder in patients who exhibit manifestations of liver disease, steatorrhea, or complications from decreased fat soluble vitamin absorption; and
- C. No documentation of extrahepatic manifestations of bile acid synthesis disorders due to single enzyme defects or peroxisomal disorders including Zellweger spectrum disorder

CONTINUATION CRITERIA

Cholic acid (Cholbam®) is re-approved when there is documentation of an FDA approved indication and improved liver function tests from the start of treatment

Approval duration:

Initial criteria-3 months;

Continuation criteria-Indefinite

▸ **Black Box Warning:**

N/A

▸ **Guidelines:**

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

▸ **References:**

Cholbam® (Cholic acid) [package insert]. Baltimore MD. Asklepion Pharmaceuticals, LLC. March 2015. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2015/205750s000lbl.pdf.

. Accessed July 23, 2018.

Cholic acid. Micromedex 2.0. Truven Health Analytics, Inc. Greenwood Village, CO. Available from: <http://www.micromedexsolutions.com>. Accessed July 23, 2018.

Applicable Drugs:

i Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
Cholbam®	Cholic acid

Cross References:

N/A

Policy Version Number:	4.00
P&T Approval Date:	July 12, 2018
Policy Effective Date:	October 01, 2018
Next Required Review Date:	July 12, 2019

The Policy Bulletins on this web site were developed to assist the Company in administering the provisions of the respective benefit programs, and do not constitute a contract. If you have coverage through the Company, please refer to your specific benefit program for the terms, conditions, limitations and exclusions of your coverage. Company does not provide health care services, medical advice or treatment, or guarantee the outcome or results of any medical services/treatments. The facility and professional providers are responsible for providing medical advice and treatment. Facility and professional providers are independent contractors and are not employees or agents of the Company. If you have a specific medical condition, please consult with your doctor. The Company reserves the right at any time to change or update its Policy Bulletins.