Pharmacy Medical Necessity Guidelines:
Cholbam® (cholic acid)

Effective: March 10, 2026

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	Rx	Department to Review	RXUM
These pharmacy medical necessity guidelines apply to the following: ☒ Tufts Health RITogether – A Rhode Island Medicaid Plan		**Fax Numbers:** RXUM: 617.673.0939	

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

Cholbam (cholic acid) is a bile acid indicated for:

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

COVERAGE GUIDELINES

The plan may authorize coverage of **Cholbam (cholic acid)** for Members when **ALL** of the following criteria are met:

Bile acid synthesis disorders

1. Documented diagnosis of bile acid synthesis disorders due to single enzyme defects

Peroxisomal disorders

1. Documented diagnosis of peroxisomal disorders; including Zellweger spectrum disorders in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption

AND

2. Documentation Cholbam (cholic acid) will be used as adjunctive therapy

LIMITATIONS

None

CODES

None

REFERENCES

1. Cholbam (cholic acid) [prescribing information]. San Diego, CA. Manchester Pharmaceuticals, Inc.; March 2023.
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 Oct;137(4):1310-20.
3. Gould SJ, Valle D. The genetics and cell biology of the peroxisome biogenesis disorders. *Trends Genet*. 2000 Aug;16(8):340-5.
4. Moser AB, Rasmussen M, Naidu S, et al. Phenotype of patients with peroxisomal disorders subdivided into sixteen complementation groups. *J Pediatr*. 1995 Jul;127(1):13-22.
5. Poll-The BT, Gootjes J, Duran M, et al. Peroxisome biogenesis disorders with prolonged survival: phenotypic expression in a cohort of 31 patients. *Am J Med Genet A.* 2004 May 1;126A(4):333-8.
6. Sundaram SS, Bove KE, Lovell MA, et al. Mechanisms of disease: inborn errors of bile acid synthesis. *Nat Clin Pract Gastroenterol Hepatol.* 2008 Aug;5(8):456-68.

APPROVAL HISTORY

October 11, 2022: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. June 13, 2023: No changes.
2. March 12, 2024: Effective April 1, 2024, updated the fax number for RxUM.
3. March 11, 2025: No changes
4. March 10, 2026: No changes

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.

Provider Services