Reference number(s)
2119-A

Specialty Guideline Management Cholbam

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Cholbam	cholic acid

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[1]

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.

Limitations 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.

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Documentation

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

- Initial requests:
  - Mass spectrometry, enzyme assay, biochemical testing results, or genetic testing results confirming diagnosis; and
  - Lab test results documenting baseline liver function (i.e., transaminases, bilirubin, presence of cholestasis).
- 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).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of enzyme or metabolic disorders.

Coverage Criteria

Bile Acid Synthesis Disorders Due to Single Enzyme Defects (SEDs)[1-3]

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:

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

Peroxisomal Disorders (PDs) Including Zellweger Spectrum Disorders[1,4]

Authorization of 6 months may be granted for adjunctive treatment of peroxisomal disorders when both of the following criteria are met:

- The diagnosis is confirmed by mass spectrometry or other biochemical testing, or by genetic testing.
- The member exhibits manifestations of liver disease (i.e., elevated transaminases, elevated bilirubin, presence of cholestasis).

Continuation of Therapy

Authorization of 12 months may be granted to members with an indication listed in the coverage criteria section who are currently receiving the requested medication through a paid pharmacy or medical benefit, and who have achieved and maintained improvement in liver function from baseline (i.e., reduced transaminases, reduced bilirubin, no evidence of cholestasis on liver biopsy).

References

1. Cholbam [package insert]. San Diego, CA: Manchester Pharmaceuticals, Inc. a wholly owned subsidiary of Travere Therapeutics, 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;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.