

Drug Policy

Policy:	Cholbam (cholic acid)	Annual Review Date: 05/18/2023 Last Revised Date: 05/18/2023
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OVERVIEW

Cholbam is indicated for the treatment of bile acid synthesis disorders due to single enzyme defects (SEDs). It is also indicated for 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. The effects of Cholbam on extrahepatic manifestations of SEDs, such as neurological symptoms, have not been established.

POLICY STATEMENT

This policy involves the use of Cholbam. Prior authorization is recommended for pharmacy benefit coverage of Cholbam. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Cholbam as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Cholbam be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Cholbam is recommended in those who meet the following criteria:

1. **Bile Acid Synthesis Disorders Due to Single Enzyme Defects (SEDs), Initial Therapy**

Criteria. *Patient must meet the following criteria (A, B, C and D):*

- A.** Diagnosis of bile acid synthesis disorder due to single enzyme defects (SEDs) [documentation required]; AND
- B.** The patient has a diagnosis of SED based on an abnormal urinary bile acid as confirmed by Fast Atom Bombardment ionization – Mass Spectrometry (FAB-MS) analysis; OR based on molecular genetic testing consistent with the diagnosis. AND
- C.** The patient has documentation of liver function testing in the past 6 months (e.g., aspartate aminotransferase [AST], alanine aminotransferase [ALT], gamma glutamyltransferase [GGT], alkaline phosphatase [ALP], bilirubin, and INR levels); AND
- D.** Cholbam is prescribed by or in consultation with a hepatologist, metabolic specialist, or a gastroenterologist.

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2. Bile Acid Synthesis Disorders Due to Single Enzyme Defects (SEDs), for Patients Currently Receiving Cholbam

Criteria. *Patient must meet the following criteria (A, B, and C):*

- A. The patient has responded to initial Cholbam therapy with an improvement in liver function tests (e.g., aspartate aminotransferase [AST], alanine aminotransferase [ALT], gamma glutamyltransferase [GGT], alkaline phosphatase [ALP], bilirubin, and INR levels); AND
- B. The patient does not have complete biliary obstruction; AND
- C. Cholbam is prescribed by or in consultation with a hepatologist, metabolic specialist, or a gastroenterologist.

3. Peroxisomal Disorders (PDs), Including Zellweger Spectrum Disorders, Initial Therapy

Criteria. *Patient must meet the following criteria (A, B, C, D, and E):*

- A. Diagnosis of peroxisomal disorder, including Zellweger Spectrum Disorder [documentation required]; AND
- B. The patient has peroxisomal disorders with an abnormal urinary bile acid analysis by Fast Atom Bombardment ionization – Mass Spectrometry (FAB-MS); OR based on molecular genetic testing consistent with the diagnosis. AND
- C. The patient has liver disease, steatorrhea, or complications from decreased fat soluble vitamin absorption (e.g., rickets); AND
- D. The patient has documentation of liver function testing in the past 6 months (e.g., aspartate aminotransferase [AST], alanine aminotransferase [ALT], gamma glutamyltransferase [GGT], alkaline phosphatase [ALP], bilirubin, and INR levels); AND
- E. Cholbam is prescribed by or in consultation with a hepatologist, metabolic specialist, or a gastroenterologist.

4. Peroxisomal Disorders, Including Zellweger Spectrum Disorders, for Patients Currently Receiving Cholbam

Criteria. *Patient must meet the following criteria (A, B, and C):*

- A. The patient has responded to initial Cholbam therapy as per the prescribing physician (e.g., improvements in liver enzymes, improvement in steatorrhea); AND
- B. Patient does not have complete biliary obstruction; AND
- C. Cholbam is prescribed by or in consultation with a hepatologist, metabolic specialist, or a gastroenterologist.

Initial Approval/ Extended Approval.

- A) *Initial Approval:* 90 days
- B) *Extended Approval:* 365 days

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Cholbam has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

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1. **Combination Therapy with Cholbam.** There are no efficacy data available to support use of combination therapy with Cholbam and Chenodal.
2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Cholbam™ capsules [prescribing information]. San Diego, CA: Retrophin Inc.; March 2015.
2. Cholbam™ (cholic acid capsules). Bile-acid synthesis disorders. Available at: <http://www.cholbam.com/bile-acid-deficiency-disorder-b-a-d-d/single-enzyme-defects/>. Accessed on April 8, 2015.
3. Heubi JE, Setchell KDR, Bove KE. Inborn errors of bile acid metabolism. *Semin Liver Dis.* 2007;27:282-294.
4. Genetics Home Reference. Zellweger Spectrum. Reviewed June 2015. Available at: <http://ghr.nlm.nih.gov/condition/zellweger-spectrum>. Accessed on July 15, 2016.
5. Cholic Acid. In: DRUGDEX (online database). Truven Health Analytics; Greenwood Village, CO. Last updated 22 June 2018. Accessed on 14 May 2019.