

## Commercial/Healthcare Exchange PA Criteria Effective: January 1, 2019

**Prior Authorization:** Cholbam

**Products Affected:** Cholbam (cholic acid) oral capsules

**Medication Description:**

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.

**Covered Uses:**

1. Bile Acid Synthesis Disorders due to Single Enzyme Defects
2. Peroxisomal Disorders Including Zellweger Spectrum Disorders

**Exclusion Criteria:**

1. Extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders

**Required Medical Information:**

1. Diagnosis of SED confirmed by mass spectrometry or other biochemical testing or genetic testing.
2. Liver function tests

**Age Restrictions:** N/A

**Prescriber Restrictions:** Prescribed by or in consultation with a hepatologist, metabolic specialist, or a gastroenterologist.

**Coverage Duration:**

Initial: 3 months

Renewal: 12 months

**Other Criteria:**

**Bile Acid Synthesis Disorders Due to Single Enzyme Defects (SEDs)**

Initial:

- A. Patient has a diagnosis of SED based on an abnormal urinary bile acid as confirmed by mass spectrometry or other biochemical testing or genetic testing.

Renewal:

- A. Patient has responded to initial Cholbam therapy with an improvement in liver function tests (e.g., aspartate aminotransferase [AST], alanine aminotransferase [ALT], bilirubin levels); AND
- B. Patient does not have complete biliary obstruction

**Bile-Acid Synthesis Disorders Due to Peroxisomal Disorders (PDs), Including Zellweger Spectrum Disorders**

**Initial:**

- A. Patient has peroxisomal disorders with an abnormal urinary bile acid analysis confirmed by mass spectrometry or other biochemical testing or genetic testing.; AND
- B. Patient has liver disease, steatorrhea, or complications from decreased fat-soluble vitamin absorption (e.g., rickets)

**Renewal:**

- A. 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

**References:**

- A. Cholbam™ capsules [prescribing information]. San Diego, CA: Retrophin Inc.; March 2015.

**Policy Revision history**

<b>Rev #</b>	<b>Type of Change</b>	<b>Summary of Change</b>	<b>Sections Affected</b>	<b>Date</b>
1	New Policy	New Policy	All	01/01/2019
2	Annual Review	Added Limitation of Use: Extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders	Exclusion criteria	9/18/2020