Harvard Pilgrim Health Care – Pharmacy Prior Authorization Guideline

<table>
<thead>
<tr>
<th>Guideline Name</th>
<th>Cholbam (cholic acid)</th>
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1. **Criteria**

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<tr>
<th>Product Name: Cholbam</th>
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<tr>
<td>Approval Length</td>
<td>12 Month(s)</td>
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<tr>
<td>Therapy Stage</td>
<td>Initial Authorization</td>
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<tr>
<td>Guideline Type</td>
<td>Prior Authorization, Non-Formulary</td>
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**Approval Criteria**

1. - Prescribed by (or in consultation with) a hepatologist, gastroenterologist, or specialist in treating bile acid synthesis or peroxisomal disorders

    AND

2. - Documentation of ONE of the following:

   2.1 - Diagnosis of bile acid synthesis disorder due to a single enzyme defect

   OR

2.2 - Diagnosis of a peroxisomal disorder with BOTH of the following:

   • Patient exhibits manifestations of liver disease, steatorrhea or complications from decrease in fat soluble vitamin absorption; AND
   • Cholbam will be used as adjunctive therapy

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**Approval Criteria**

1. - Patient has experienced an improvement in liver function while on therapy, as noted by at least ONE of the following:

   • ALT or AST values reduced to less than 50 U/L, or baseline levels reduced by 80%; OR
   • Total bilirubin values reduced to less than or equal to 1 mg/dL; OR
   • No evidence of cholestasis on liver biopsy
# 2. Background

## Benefit/Coverage/Program Information

### RATIONALE
To promote the appropriate use of Cholbam (cholic acid) for Food and Drug Administration (FDA)-approved indications.

### FDA APPROVED INDICATIONS
Cholbam (cholic acid) is a bile acid 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

Limitation of use: The safety and effectiveness of Cholbam (cholic acid) on extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders have not been established.

### Bile Acid Synthesis Disorder Subtypes include, but may not be limited to:

- 3-beta-hydroxy-delta-5-C27-steroid oxidoreductase (3β-HSD) deficiency [Congenital bile acid synthesis defect type 1]
- Alpha-methylacyl-CoA racemase (AMACR) deficiency [Congenital bile acid synthesis defect type 4]
- Amino acid n-acyltransferase deficiency
- Bile acid CoA ligase deficiency
- Cholesterol 7alpha-hydroxylase (CYP7A1) deficiency
- Delta4-3-oxosteroid 5-beta-reductase (AKR1D1) deficiency [Congenital bile acid synthesis defect type 2]
- Oxysterol 7-alpha-hydroxylase deficiency [Congenital bile acid synthesis defect type 3]
- Sterol 27-hydroxylase deficiency (cerebrotendinous xanthomatosis; CTX)
- Trihydroxycholestanolic acid CoA oxidase deficiency

### Peroxisomal Disorder Subtypes include, but may not be limited to:

- Zellweger syndrome (ZWS)
- Adrenoleukodystrophy (NALD)
- Infantile Refsum disease (IRD)
- Rhizomelic chondrodysplasia punctata type 1 (RCDP1)
- X-linked adrenoleukodystrophy (X-ALD)
- Refsum disease (phytanoyl CoA hydroxylase deficiency)
- Acyl CoA oxidase deficiency (pseudo-NALD)
- D-bifunctional protein deficiency (DBP deficiency)
- Rhizomelic chondrodysplasia punctata type 2 (RCDP2; dihydroxy-acetone phosphate acyltransferase deficiency)
- Alpha-methylacyl-CoA racemase deficiency (AMACR deficiency)
- Peroxisomal sterol carrier protein-X deficiency (SCPx deficiency)
- Acatalasemia (catalase deficiency)
- Hyperoxaluria type 1 (alanine glyoxylate aminotransferase deficiency)

### REFERENCES
- Cholbam (cholic acid) [prescribing information]. San Diego, CA. Manchester
Pharmaceuticals, Inc.; Mar 2015.


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<tr>
<td>Revised:</td>
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<td>- Annual review (effective: 1/1/20)</td>
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<td>- 6/19/20 - Annual review: updated background; no changes to criteria</td>
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<tr>
<td>P&amp;T Approval: 12/7/20</td>
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<td>Effective: 9/1/20</td>
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