

Cholbam (cholic acid)

Override(s)	Approval Duration
Prior Authorization	Initial Requests: 3 months
Quantity Limit	Continued Therapy Requests: 12 months

Medications	Quantity Limit
Cholbam (cholic acid) capsules	May be subject to quantity limit

APPROVAL CRITERIA

Initial requests for Cholbam (cholic acid) may be approved when the following criteria are met:

- I. Individual has a diagnosis of bile acid synthesis disorders (BASDs) due to single enzyme defects (SEDs) [including but not limited to 3β -hydroxy- Δ^5 -C₂₇-steroid oxidoreductase (3β -HSD) defects]; **AND**
 - A. Documentation is provided that diagnosis is confirmed by any of the following (NORD 2020):
 1. Fast atom bombardment-mass spectrometry (FABS-MS); **OR**
 2. Electrospray ionization-mass spectrometry (ESI-MS); **OR**
 3. Gas chromatography-mass spectrometry (GC-MS); **OR**
 4. Molecular genetic testing;

OR

- II. Documentation is provide that individual has a diagnosis peroxisomal disorders (PDs) [including but not limited to Zellweger spectrum disorders (ZSD)]; **AND**
- III. Documentation is provided that individual has one of the following present:
 - A. Manifestations of liver disease (for example, jaundice, hepatomegaly); **OR**
 - B. Steatorrhea; **OR**
 - C. Complications from decreased fat-soluble vitamin (such as but not limited to, vitamin D and K) absorption (for example, rickets, hypocalcemia, bleeding);

AND

- IV. Individual is using as adjunctive therapy in conjunction with supportive care (such as but not limited to vitamin supplementation).

Requests for continued use of Cholbam (cholic acid) may be approved if the following criteria are met:

- I. Documentation is provided that individual has had a clinical improvement (symptoms, lab values) in liver function and/or cholestasis; **AND**
- II. Individual has not developed a complete biliary obstruction.

Requests for Cholbam (cholic acid) may not be approved for the following:

- I. Individual is using to treat extrahepatic manifestations (such as but not limited to neurologic symptoms) of SED-associated-BASDs or PDs.

Key References:

1. Bile Acid Synthesis Disorders. Rare Disease Database. National Organization for Rare Disorders (NORD). 2020. Available from: <https://rarediseases.org/rare-diseases/bile-acid-synthesis-disorders/>. Accessed on July 13, 2021.
2. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2021. URL: <http://www.clinicalpharmacology.com>. Updated periodically.
3. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. <http://dailymed.nlm.nih.gov/dailymed/about.cfm>. Accessed: July 13, 2021.
4. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
5. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2021; Updated periodically.
6. Berendse K, Klouwer FCC, Koot BGP, et al. Cholic acid therapy in Zellweger spectrum disorders. *J Inherit Metab Dis*. 2016; 39(6):859-868. Available from: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5065608/pdf/10545_2016_Article_9962.pdf.
7. 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. Available from: [http://www.gastrojournal.org/article/S0016-5085\(09\)01163-9/pdf](http://www.gastrojournal.org/article/S0016-5085(09)01163-9/pdf).
8. Klouwer FCC, Koot BGP, Berendse K, et al. The cholic acid extension study in Zellweger spectrum disorders: results and implications for therapy. *J Inherit Metab Dis*. 2018. Available from: <https://link.springer.com/article/10.1007/s10545-018-0194-z#citeas>.

Federal and state laws or requirements, contract language, and Plan utilization management programs or policies may take precedence over the application of this clinical criteria.

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