

Market Applicability														
Market	DC	FL & FHK	FL MMA	FL LTC	GA	KS	KY	MD	NJ	NV	NY	TN	TX	WA
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

*FHK- Florida Healthy Kids

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-C -steroid oxidoreductase (3β -HSD) defects];

OR

- II. Individual has a diagnosis peroxisomal disorders (PDs) [including but not limited to Zellweger spectrum disorders (ZSD)]; **AND**
- III. 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. 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.

PAGE 1 of 2 12/27/2018

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.

CRX-ALL-0330-19

Market Applicability														
Market	DC	FL & FHK	FL MMA	FL LTC	GA	KS	KY	MD	NJ	NV	NY	TN	TX	WA
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

*FHK- Florida Healthy Kids

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.

State Specific Mandates		
State name	Date effective	Mandate details (including specific bill if applicable)
N/A	N/A	N/A

Key References:

1. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2018. URL: <http://www.clinicalpharmacology.com>. Updated periodically.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. <http://dailymed.nlm.nih.gov/dailymed/about.cfm>. Accessed: August 15, 2018.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2018; Updated periodically.
5. 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. Accessed on: August 15, 2018.
6. 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). Accessed on: August 15, 2018.

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.