

Protocol for Cholbam[®] (cholic acid)

Approved October 2022

Background: *Bile acid synthesis disorders (BASDs) are a group of rare metabolic disorders characterized by defects in the creation (synthesis) of bile acids.*

Cholbam is a bile acid indicated for the treatment of bile acid synthesis disorders due to single enzyme defects (SEDs) and as an 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.

Criteria for approval:

1. Patient has a documented diagnosis of:
 - a. Bile acid synthesis disorders (BASDs) due to single enzyme defects (SEDs)
 - i. Diagnosis is confirmed by mass spectrometry or other biochemical testing, genetic testing, or enzyme assay
 - ii. Liver dysfunction, with labs showing elevated transaminases, bilirubin, presence of cholestasis at baseline have been submitted
 - OR**
 - b. Peroxisomal disorders, including Zellweger spectrum disorder in patients who exhibit manifestations of liver disease, steatorrhea, or complications from decreased fat-soluble vitamin absorption
 - i. Diagnosis is confirmed by mass spectrometry or other biochemical testing or genetic testing
 - ii. The patient exhibits manifestations of liver disease
2. Medication is prescribed by or in consultation with a hepatologist or gastroenterologist
3. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Initial Approval: 3 months

Continuation of therapy:

1. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Renewal approval: 6 months

References:

1. Cholbam [prescribing information]. Asklepiion Pharmaceuticals LLC. Baltimore, MD 21202. March 2015
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically