BlueCross BlueShield Federal Employee Program

CHOLBAM
(cholic acid)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Cholbam is the first FDA approved treatment for pediatric and adult patients with rare bile acid synthesis disorders due to single enzyme defects, and for patients with peroxisomal disorders (including Zellweger spectrum disorders). Patients with these rare, genetic, metabolic conditions exhibit manifestations of liver disease, steatorrhea (presence of fat in the stool) and complications from decreased fat-soluble vitamin absorption. Individuals with these rare disorders lack the enzymes necessary to produce cholic acid, a primary bile acid synthesized by the liver from cholesterol (1).

The mechanism of action of cholic acid has not been fully established. Endogenous bile acids including cholic acid enhance bile flow, provide the physiologic feedback inhibition of bile acid synthesis, and regulate bile acid circulation (1).

Regulatory Status

FDA-approved indications: Cholbam (cholic acid) is a bile acid indicated for: (1)

1. Treatment of bile acid synthesis disorders due to single enzyme defects (SEDs).
2. 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.

Limitations of Use:

The safety and effectiveness of Cholbam on extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders have not been established (1).

Treatment with Cholbam should be initiated and monitored by an experienced hepatologist or pediatric gastroenterologist (1).

The Cholbam label includes a warning for exacerbation of liver impairment. Patients should be monitored every month for the first 3 months for serum aspartate aminotransferase (AST), serum alanine aminotransferase (ALT), serum gamma glutamyltransferase (GGT), alkaline phosphatase (ALP), bilirubin and INR levels then every 3 months for the next 9 months, every 6 months during the subsequent three years and annually thereafter; or more frequently during periods of rapid growth, concomitant disease, and pregnancy. Cholbam should be discontinued in patients who

develop worsening of liver function or cholestasis while on treatment (1).

The safety and effectiveness of Cholbam has been established in pediatric patients 3 weeks of age and older for the treatment of bile acid synthesis disorders due to SEDs, and for adjunctive treatment of patients with PDs including Zellweger spectrum disorders who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption (1).

Summary

Cholbam is an oral bile acid therapy approved for treatment of bile acid synthesis disorders and adjunctive treatment of peroxisomal disorders. Cholbam has a warning for possible exacerbation of liver impairment. Patients should be monitored for worsening liver function or cholestasis. More frequent monitoring may be required during periods of rapid growth, concomitant disease, and pregnancy (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Cholbam while maintaining optimal therapeutic outcomes.

References

1. Cholbam [package insert] San Diego, CA: Travere Therapeutics, Inc.; June 2024.