

For patients with SLOS

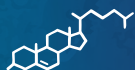
When it comes to cholesterol, fat-soluble vitamins, nutrients, weight, and growth . . .

ABSORB MORE. GAIN MORE.

CHOLBAM is a bile acid indicated for the treatment of bile acid synthesis disorders due to single enzyme defects and adjunctive treatment of peroxisomal disorders, including Zellweger spectrum disorders, in patients who exhibit manifestations of liver disease, steatorrhea, or complications from decreased fat-soluble vitamin absorption.¹

The safety and effectiveness of CHOLBAM on extrahepatic manifestations of bile acid synthesis disorders due to single enzyme defects or peroxisomal disorders, including Zellweger spectrum disorders, have not been established.

In your patients with
Smith-Lemli-Opitz Syndrome (SLOS),
CHOLBAM may help*:



Increase
plasma cholesterol^{2†}



Lower
liver enzymes¹



Improve certain
growth parameters^{1-3‡}



This is 4-year-old Mallie in her clogging class. She has SLOS and has been taking CHOLBAM since 2023. Individual results may vary.

Backed by >18 years of safety data, CHOLBAM has a well-characterized safety profile. There were 12 adverse reactions reported across 9 patients in the clinical trials.¹ In clinical trials, the most common adverse reaction (~2%) reported was diarrhea. All other adverse reactions represented 1% of the patient population.¹

*No evaluable patients with SLOS were in the pivotal studies. Patients had other bile acid synthesis disorders due to single enzyme defects.¹

[†]In 11 of 12 patients with SLOS given CHOLBAM 10 mg/kg/day and cholesterol supplementation for 2 months, in an open-label pilot study. Long-term studies are needed to determine durability of effect and possible clinical benefits.²

[‡]In a 2-month pilot study, 9 of 12 patients showed an increasing trend in weight gain from baseline and did not reach statistical significance.²

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS – Exacerbation of liver impairment

- Monitor liver function and discontinue CHOLBAM in patients who develop worsening of liver function while on treatment.

Please see full Prescribing Information for CHOLBAM.

 **Cholbam**[®]
(cholic acid) capsules

INDICATION

CHOLBAM® (cholic acid) is a bile acid indicated for

- Treatment of bile acid synthesis disorders due to single enzyme defects.
- Adjunctive treatment of peroxisomal disorders, 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 single enzyme defects or peroxisomal disorders, including Zellweger spectrum disorders, have not been established

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS – Exacerbation of liver impairment

- Monitor liver function and discontinue CHOLBAM in patients who develop worsening of liver function while on treatment.
- Concurrent elevations of serum gamma glutamyltransferase (GGT) and alanine aminotransferase (ALT) may indicate CHOLBAM overdose.
- Discontinue treatment with CHOLBAM at any time if there are clinical or laboratory indicators of worsening liver function or cholestasis.

ADVERSE REACTIONS

- The most common adverse reactions ($\geq 1\%$) are diarrhea, reflux esophagitis, malaise, jaundice, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy.

DRUG INTERACTIONS

- Inhibitors of Bile Acid Transporters: Avoid concomitant use of inhibitors of the bile salt efflux pump (BSEP) such as cyclosporine. Concomitant medications that inhibit canalicular membrane bile acid transporters such as the BSEP may exacerbate accumulation of conjugated bile salts in the liver and result in clinical symptoms. If concomitant use is deemed necessary, monitoring of serum transaminases and bilirubin is recommended.

- Bile Acid Binding Resins: Bile acid binding resins such as cholestyramine, colestipol, or colesevelam adsorb and reduce bile acid absorption and may reduce the efficacy of CHOLBAM. Take CHOLBAM at least 1 hour before or 4 to 6 hours (or at as great an interval as possible) after a bile acid binding resin.
- Aluminum-based Antacids: Aluminum-based antacids have been shown to adsorb bile acids *in vitro* and can reduce the bioavailability of CHOLBAM. Take CHOLBAM at least 1 hour before or 4 to 6 hours (or at as great an interval as possible) after an aluminum-based antacid.

PREGNANCY

No studies in pregnant women or animal reproduction studies have been conducted with CHOLBAM.

LACTATION

Endogenous cholic acid is present in human milk. Clinical lactation studies have not been conducted to assess the presence of CHOLBAM in human milk, the effects of CHOLBAM on the breastfed infant, or the effects of CHOLBAM on milk production.

The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for CHOLBAM and any potential adverse effects on the breastfed infant from CHOLBAM or from the underlying maternal condition.

GERIATRIC USE

It is not known if elderly patients respond differently from younger patients.

HEPATIC IMPAIRMENT

- Discontinue treatment with CHOLBAM if liver function does not improve within 3 months of the start of treatment.
- Discontinue treatment with CHOLBAM at any time if there are clinical or laboratory indicators of worsening liver function or cholestasis. Continue to monitor laboratory parameters of liver function and consider restarting at a lower dose when the parameters return to baseline.

OVERDOSAGE

Concurrent elevations of serum GGT and serum ALT may indicate CHOLBAM overdose. In the event of overdose, the patient should be monitored and treated symptomatically. Continue to monitor laboratory parameters of liver function and consider restarting at a lower dose when the parameters return to baseline.

Please see full Prescribing Information for CHOLBAM.

