

Meet Mallie, an unstoppable little girl with SLOS

Learn how management with CHOLBAM® (cholic acid) helped her get closer to key milestones.*

Before SLOS Diagnosis

- Born with cleft palate and several health concerns
- G-tube inserted at 11 days old
- Misdiagnosed with Pierre Robin sequence, leading to a 2-year diagnostic journey



After SLOS-Focused Treatment

- At almost 2 years old, SLOS diagnosis confirmed
- Started treatment with CHOLBAM and cholesterol supplementation



Plasma Cholesterol Levels

Spring 2023 = 87 mg/dL

Fall 2024 = 117 mg/dL

Weight and Height

- Weight = 15 pounds
- Height = 28 inches

- Weight = 23 pounds
- Height = 35 inches

Parent Observations

- Frequent hospitalizations, in some cases due to infections
- Not walking or talking
- Not interested in eating
- Played alone and off to the side at daycare
- Needed occupational, physical, and speech therapy

- Mallie hasn't been visiting the doctor or hospital as frequently for infections
- Increased physical activity
- Shows more interest in food and eats pureed pouches (G-tube is still main means of nutrition)
- Increased engagement with friends at daycare
- Continues her occupational, physical, and speech therapy

SLOS-Smith-Lemli-Opitz Syndrome.

*Patient story reported by caregiver. Individual results may vary. Images used with kind permission of the family.

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.

Please see Important Safety Information on next page and [full Prescribing Information](#) for additional Important Safety Information.



IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS — Exacerbation of liver impairment

- Monitor liver function and discontinue CHOLBAM® (cholic acid) 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.



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Visit [CHOLBAMHCP.com](https://www.cholbamhcp.com) to learn whether CHOLBAM may be right for your patients with SLOS