

Your guide to long-term therapy with

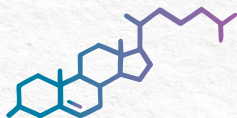


Cholic acid is crucial

If you have a life-long genetic disorder, called **Smith-Lemli-Opitz Syndrome (SLOS)**, your body does not make enough cholesterol. Without it, the body cannot make a bile acid named cholic acid. Cholic acid is needed to digest and absorb cholesterol and other fats, as well as vitamins and nutrients from the food you eat.

CHOLBAM is a prescription medicine that may help increase cholic acid in people with SLOS and other rare bile acid synthesis disorders.

CHOLBAM may help*:



**Increase
cholesterol levels**



**Lower liver enzymes to help
support better liver function**



**Promote growth
on schedule**

- **By improving cholesterol levels**, CHOLBAM may help people improve their weight and height. This may help address growth delays in people with SLOS
- **CHOLBAM may help lower liver enzymes**, such as alanine aminotransferase (ALT) and aspartate aminotransferase (AST)

No evaluable patients with SLOS were in the pivotal studies.

*In a 2-month pilot study, 9 of 12 patients showed an increasing trend in weight gain from baseline but the trend did not reach statistical significance. Improvements in height have also been reported.

INDICATION

What is CHOLBAM® capsules?

CHOLBAM is a prescription medicine. It is a bile acid used for

- Treatment of bile acid synthesis disorders (malfunction in the production of bile acid in the liver) due to a lack of a single enzyme responsible for bile acid production.
- Treatment (in addition to other treatments) of peroxisomal disorders (a loss of function in important parts of the cell), including Zellweger spectrum disorders, in patients who show signs or symptoms of liver disease, pale stools due to too much fat, or problems resulting from not absorbing certain vitamins (A, D, E, K).

The safety and effectiveness of CHOLBAM on symptoms outside of the liver have not been studied.

IMPORTANT SAFETY INFORMATION

CHOLBAM may not be right for you. Before taking CHOLBAM, tell your doctor about:

- All health conditions you may have and any medications you may be taking.
- If you are pregnant or plan to become pregnant.
- If you are breastfeeding or plan to breastfeed.

Please see Important Safety Information throughout and full Prescribing Information.

Getting started

During the first 30 days, focus on building a daily dosing routine



Take CHOLBAM at the same time each day



Set a daily alarm as a reminder



Try using a day-of-the-week pill box to ensure doses are not missed



Mark the prescription refill date on your calendar

Taking CHOLBAM

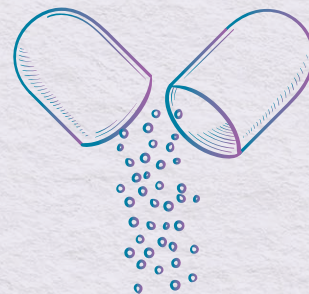
- Take daily with food
- Take by mouth (orally) in one or 2 divided doses, as your doctor instructs you
- The doctor may consider starting with twice daily dosing as you get used to higher doses of CHOLBAM
- Take CHOLBAM at least 1 hour before or 4 to 6 hours after taking a bile acid binding resin or an aluminum-based antacid
- Do not crush or chew tablets
- Do not adjust the dose without first consulting with your or your loved one's doctor
- Dosing is weight based
 - As weight increases, your dosing will change
 - Keep track of weight changes and follow up with your doctor to ensure you are receiving the correct dose

For people who are unable to swallow capsules whole, open and mix the capsule contents with:

- A small amount of infant formula or expressed breast milk
- A small amount of a favorite syrup (chocolate/maple)
- A spoonful of soft foods/thick drinks like:
 - Yogurt
 - Mashed potatoes
 - Apple sauce
 - Coffee creamer

Before and/or after administering, drink a small amount of your favorite juice to further mask the taste.

DO NOT sprinkle on top of food; thoroughly mix capsule contents with a small amount of food to mask the taste.



For additional instructions on administration, visit cholbam.com/slos/starting-cholbam-slos and read full Prescribing Information.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of CHOLBAM?

- **Serious Side Effects:** You will need to undergo laboratory testing periodically while on treatment to assess liver function. CHOLBAM may worsen liver function. Tell your doctor right away if you experience symptoms associated with worsening of liver function (e.g., skin or the whites of eyes turn yellow, urine turns dark or brown [tea colored], pain on the right side of stomach, bleeding or bruising occurs more easily than normal, or increased lethargy). Your doctor will decide if you need to discontinue treatment with CHOLBAM.

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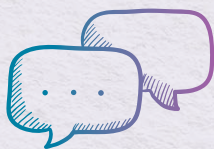


What to know about side effects

**CHOLBAM has a
well-established safety profile.**

Side effects reported in patients taking CHOLBAM:

- The most common side effect experienced in clinical trials was diarrhea (about 2% of patients)
- Other common side effects occurred in 1% of patients, including reflux esophagitis, malaise, jaundice, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy



**Do not stop CHOLBAM without talking to your doctor.
If you experience persistent side effects, call your doctor.**



***“[Mallie] experienced diarrhea
for like a week”***

—Taylor, Mallie's mother Individual results may vary.

**See Mallie's experience with CHOLBAM.
Visit cholbam.com/slos/videos-slos**

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of CHOLBAM?

- **Most Common Side Effects:** Diarrhea, acid reflux that damages the lining of the esophagus, discomfort and fatigue, yellowing of skin or eyes, skin lesion, nausea, abdominal pain, formation of growths in the intestinal tract, urinary tract infection, nerve damage causing change in sensation in extremities.

These are not all the possible side effects of CHOLBAM. Tell your doctor if you have any side effect that bothers you or that does not go away.

**Please see Important Safety Information throughout and full
Prescribing Information.**



TRACKING YOUR PROGRESS

CHOLBAM is part of your long-term care plan and may take time to show improvements. Take CHOLBAM every day as prescribed to ensure your body has what it needs to function properly.

Regular follow-up care is an essential part of treatment. Your doctor will schedule periodic visits to make sure CHOLBAM is working by monitoring:



Growth through tracking height and weight



Nutritional status, including cholesterol, vitamin, and nutrient levels



Liver function through blood tests



GET STARTED WITH MIRUM ACCESS PLUS

A comprehensive support program designed to help people get and stay on CHOLBAM. Receive ongoing education, insurance coverage support, help coordinating medication refills, and more.

For resources, administration tips, and more, visit CHOLBAM.COM/SLOS

IMPORTANT SAFETY INFORMATION (cont'd)

These are not all the possible side effects of CHOLBAM. Tell your doctor if you have any side effect that bothers you or that does not go away.

You may report side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Mirum Pharmaceuticals at 1-855-MRM-4YOU.

Please see Important Safety Information throughout and full Prescribing Information.

