

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

ABSORB MORE. GAIN MORE.

This guide is intended to help you learn about Smith-Lemli-Opitz Syndrome (SLOS) and CHOLBAM so you can be the best advocate for yourself or your loved one.

Talk to a health care provider to learn more about CHOLBAM.

CHOLBAM is a cholic acid replacement therapy that may help patients with SLOS increase cholesterol levels, lower liver enzymes, and improve certain growth parameters.*

No evaluable patients with SLOS were included in the pivotal studies.

*CHOLBAM was studied in a pilot study. Long-term studies are needed to determine durability of effect and possible clinical benefits. In a 2-month pilot study, 9 of 12 patients showed an increasing trend in weight gain from baseline but did not reach statistical significance.

INDICATION

What is CHOLBAM® (cholic acid) 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.

Please see additional Important Safety Information for CHOLBAM throughout and accompanying full [Prescribing Information](#).

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

 **Cholbam®**
(cholic acid) capsules

What is Smith-Lemli-Opitz Syndrome (SLOS)?

SLOS is a rare genetic condition that **stops your body from making enough cholesterol** due to a change, or mutation, in the 7-dehydrocholesterol reductase (*DHCR7*) gene.

Healthy cholesterol levels help the body with important functions:



Building blocks
for cells



Helps produce
hormones



Aids brain
health



Used to make
bile acids like
cholic acid



Supports growth
and development

~90% of people with SLOS have lower than normal cholesterol levels.

When the body cannot make or absorb enough cholesterol, it can lead to various health challenges.

Cholesterol makes bile acids, which help digest fats and absorb nutrients

Low cholesterol may lead to low bile acids

How are cholesterol and bile acids linked?

Low cholesterol levels can lead to low bile acid levels, which makes it harder for the body to absorb cholesterol and other important nutrients. **Even if you take cholesterol supplements, your body may not absorb enough cholesterol if it doesn't have enough bile acid.**



Bile acids are made from cholesterol in your liver.
People with Smith-Lemli-Opitz Syndrome (SLOS) who have low cholesterol may also have low levels of bile acids.



Bile acids help with essential functions in the body.

- Aid in the digestion and absorption of nutrients from food, such as cholesterol, fats, and fat-soluble vitamins
- Help to maintain liver function



One important bile acid is cholic acid.

- Cholic acid is key for proper digestion, absorption of nutrients like cholesterol from the food we eat, and the removal of toxins from the body
- People with SLOS may lack healthy levels of cholic acid

Cholic acid replacement therapy may help raise cholesterol levels,
making it easier for the body to absorb more nutrients.

CHOLBAM is cholic acid

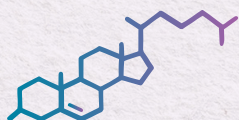
CHOLBAM is a prescription medicine that may help increase cholic acid in people with Smith-Lemli-Opitz Syndrome (SLOS) and other rare bile acid synthesis disorders.



Cholic acid is a substance made by the liver

- Everyone already has it in their bodies to help digest fats and absorb nutrients
- People with SLOS usually have less cholic acid
- Increasing cholic acid levels can help the body digest fats and absorb important nutrients, like cholesterol

CHOLBAM may help patients with SLOS*:



Increase cholesterol[†]

CHOLBAM may help raise the amount of cholesterol in the blood, an essential nutrient that plays vital roles in the body.



Grow 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.



Lower liver enzymes

CHOLBAM may help lower liver enzymes, such as alanine aminotransferase (ALT) and aspartate aminotransferase (AST). Lowering these enzymes may support better liver function.

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

§Side effects from trial were not tracked in an organized way. More than 1% of patients may experience side effects.

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 additional Important Safety Information for CHOLBAM throughout and accompanying full [Prescribing Information](#).



What are the possible side effects of CHOLBAM?

CHOLBAM has a well-established safety profile. It has been FDA approved and available since 2015.

As with any medication, there is a possibility of side effects.

- 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

Patients taking CHOLBAM will need to undergo laboratory testing periodically while on treatment to assess liver function. CHOLBAM may worsen liver function. Call your or your loved one's doctor right away if any of the following occur, as these may be signs of worsening liver function:

- Yellowing of the skin or whites of the eyes
- Dark or brown (tea-colored) urine
- Pain on the right side of the stomach
- Bleeding or bruising that occurs more easily than normal
- Increased tiredness

Always talk to your doctor about any possible side effects you or your loved one may be experiencing.



How do you know if CHOLBAM may be right for you or your loved one?

- ☐ Low cholesterol levels
- ☐ Concerned about growth milestones
- ☐ High liver enzymes

If you selected 1 or more above, CHOLBAM may be a treatment option for you or your loved one. Always talk to your doctor when considering new medications.

How is CHOLBAM taken?

Recommended dose



- 10 mg/kg to 15 mg/kg of body weight
- CHOLBAM comes in 50-mg and 250-mg capsules
- The doctor will prescribe the right amount of capsules based on weight and needs
- The doctor may consider starting with twice daily dosing as you or your loved one gets used to higher doses of CHOLBAM

Take with food



- CHOLBAM is taken by mouth (orally)
- Take CHOLBAM with food
- 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 the capsules. For patients who cannot swallow capsules, see additional steps on next page



Schedule

- CHOLBAM is taken once daily, or in 2 divided doses



Weight and dose adjustments

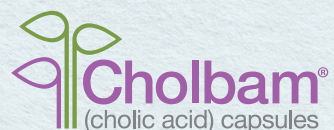
- Do not adjust the dose without first consulting with your or your loved one's doctor. The recommended dosage will change as weight changes. The doctor will adjust as needed, particularly during periods of rapid growth

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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How should capsules be taken if they cannot be swallowed?

CHOLBAM capsules can be opened and the contents mixed with other foods to mask any unpleasant taste. It can be mixed with:



- Infant formula or expressed breast milk (for younger children)
- A spoonful of yogurt or apple sauce (for younger children, older children, and adults)
- Your favorite syrup, like chocolate syrup or maple syrup (for older children and adults)
- Coffee creamer (for older children and adults)
- Soft foods, such as mashed potatoes or apple puree (for older children and adults)

Steps for opening CHOLBAM capsules and mixing contents



- 1 Hold the capsule over the prepared liquid/food, gently twist open, and allow the contents to fall into the liquid/food.
- 2 Mix the entire capsule contents with 1 or 2 tablespoons (15 mL–30 mL) of infant formula, expressed breast milk, syrup, yogurt, coffee creamer, or soft foods, such as mashed potatoes or apple puree.
- 3 Stir for 30 seconds.
- 4 The capsule contents will remain as fine granules in the milk or food and will not dissolve.
- 5 Administer the mixture immediately.

Watch a family prepare and administer CHOLBAM

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.

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 additional Important Safety Information for CHOLBAM throughout and accompanying full [Prescribing Information](#).



Have a conversation about CHOLBAM with your doctor

Take this guide with you and consider asking the following questions about CHOLBAM at your next visit.

How do I know if CHOLBAM is the right treatment for me or my loved one?

How will I know if CHOLBAM is working?

What are the possible side effects of CHOLBAM? How can you help me manage side effects if I experience them?

How do I get CHOLBAM?

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Meet

Mallie

A spirited girl with Smith-Lemli-Opitz Syndrome (SLOS)

Patient profile reported by caregiver.
Individual results may vary.

Life before SLOS treatment

Mallie was born in 2020 with a cleft palate and several health concerns.



Mallie had a G-tube inserted to help her get the nutrition she needed.



She had initially been misdiagnosed, which led to a 2-year diagnostic journey.



At almost 2 years old, Mallie was correctly diagnosed with SLOS by her geneticist. She began cholesterol supplementation and treatment with CHOLBAM in 2023.

Watch Mallie's story

Life after SLOS treatment

Mallie has seen multiple improvements and her parents have observed:

- | | | |
|-------------------------|---|---|
| • Increased weight | • Less frequent doctor and hospital visits for infections | • Shows more interest in food and eats pureed pouches (G-tube is still main means of nutrition) |
| • Increased height | • Increased physical activity | • Continues her occupational, physical, and speech therapy |
| • Increased cholesterol | • More engagement with friends at daycare | |

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How can I get started on CHOLBAM?

Getting started on CHOLBAM is easy with **Mirum Access Plus**



Mirum Access Plus is a comprehensive support program for patients who have been prescribed CHOLBAM and their caregivers. Once enrolled in the program, patients and caregivers are assigned a dedicated team of professionals to help them throughout their treatment.

If you and your doctor decide CHOLBAM is right for you, have your doctor fill out the enrollment form, which can be found at [CHOLBAMhcp.com](https://www.CHOLBAMhcp.com).

Mirum Access Plus includes coordinators, pharmacists, and navigators who can:

- **Help you understand** Smith-Lemli-Opitz Syndrome (SLOS)
- **Provide tips** to help manage treatment
- **Answer questions** about dosing and treatment regimens
- **Eligible patients may pay as little as \$0 out of pocket** per fill for CHOLBAM*
- **Offer support** with questions about insurance
- **Coordinate monthly prescription refills** and send reminders so you or your loved one never run out of CHOLBAM
- **Arrange for delivery** of medication
- **Communicate** with your doctors

If you have questions about Mirum Access Plus,
contact us at: 1-855-MRM-4YOU (1-855-676-4968)
Monday through Friday, 8:00 AM to 8:00 PM ET.

*Subject to program terms and conditions.

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What other resources are available?

There are several websites dedicated to the Smith-Lemli-Opitz Syndrome (SLOS) community



The Smith-Lemli-Opitz Foundation works worldwide to improve the quality of life for people with SLOS. The Smith-Lemli-Opitz Foundation raises awareness of the syndrome and funds research to improve treatments with the hope of someday finding a cure. smithlempiopitz.org

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The National Organization for Rare Disorders (NORD) is an organization dedicated to identifying, treating, and curing rare disorders through education, advocacy, research, and service. rarediseases.org

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Genetic and Rare Diseases Information Center (GARD) is a program of the National Institutes of Health (NIH) that provides free access to reliable, easy-to-understand information about genetic and rare diseases. rarediseases.info.nih.gov

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The Human Phenotype Ontology (HPO) project provides an ontology of medically relevant phenotypes, disease-phenotype annotations, and the algorithms that operate on these. hpo.jax.org

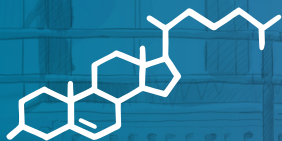
Mirum Pharmaceuticals is not affiliated with the listed resources.

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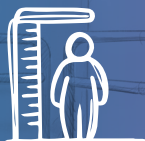
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Increase
cholesterol[†]



Grow on
schedule[‡]



Lower
liver enzymes

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