

Talking to Your Doctor About CHOLBAM® (cholic acid)*

A Guide for Caregivers and People With Smith-Lemli-Opitz Syndrome (SLOS)

If you or your loved one has been diagnosed with Smith-Lemli-Opitz Syndrome (SLOS) and is considering CHOLBAM, then this guide is for you.

This guide contains useful information about SLOS and CHOLBAM, and whether CHOLBAM may be the right treatment option. This guide may also help you think of additional questions to ask your doctor during scheduled visits.

*No evaluable patients with SLOS were represented in pivotal clinical trials.



CHOLBAM is a prescription medication. It is a bile acid used for the 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.

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

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



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

Smith-Lemli-Opitz Syndrome is a rare genetic condition caused by a change (called a mutation) in the 7-dehydrocholesterol reductase (*DHCR7*) gene involved in cholesterol production, which disrupts the body's ability to make cholesterol. Cholesterol is essential for proper growth and development.

What happens if the body cannot make cholesterol?

- Low levels of cholesterol may lead to poor nutrition and poor growth.
- Because cholesterol is also needed for making bile acids in the liver, it is likely that low cholesterol levels in SLOS impair bile acids from being made.
- Bile acids help digest dietary cholesterol from the intestine. Low bile acid levels likely prevent dietary cholesterol from being absorbed properly and contribute to the cholesterol deficiency seen in SLOS.
- Some patients may experience elevated or abnormal liver function levels, such as aspartate aminotransferase (AST) and alanine aminotransferase (ALT), which are important measurements of liver health.

The addition of CHOLBAM® (cholic acid), a bile acid replacement therapy, may be able to help address these key issues.

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

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.

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.





How can CHOLBAM[®] (cholic acid) help?

CHOLBAM is a bile acid replacement therapy for cholic acid. CHOLBAM increases cholic acid, which is one of the primary bile acids that patients with SLOS may lack.

- Bile acids are made from cholesterol, so a patient with SLOS with very low cholesterol levels cannot make a sufficient amount of bile acids. Therefore, these patients may have a bile acid deficiency.
- Bile acids are essential for the absorption of fats and fat-soluble vitamins and nutrients in the intestines. Without sufficient bile acids present, patients cannot properly absorb cholesterol and other nutrients.

CHOLBAM has been FDA approved and available since 2015.

IMPORTANT SAFETY INFORMATION (continued)

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.

How to know if CHOLBAM may be right for you*



Do you or your loved one have low cholesterol levels?

- CHOLBAM may help achieve increased cholesterol levels.[†]



Concerned about growing on schedule?

- CHOLBAM may help improve growth.^{*†}



Are liver enzymes high?

- CHOLBAM may help lower liver enzymes.

CHOLBAM is used for the treatment of bile acid synthesis disorders (BASDs), which are caused by defects in the enzyme that makes bile acids.

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

As with any medication, there is a possibility of side effects. In pivotal clinical studies, the most common side effect in about 2% of patients taking CHOLBAM was diarrhea.* All other side effects were less than or equal to 1% of patients.

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

[†]CHOLBAM was studied in a pilot study in which 11 of 12 patients with SLOS were given CHOLBAM 10 mg/kg/day and cholesterol supplementation for 2 months. Long-term studies are needed to determine durability of effect and possible clinical benefits.

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

To learn more about the pilot study for CHOLBAM, visit CHOLBAM.com/SLOS

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

What questions should I ask my doctor?



Take this guide with you and consider asking the following questions during your next visit:

About CHOLBAM® (cholic acid)

- Can CHOLBAM be an option for me or my loved one?
- How does CHOLBAM work?
- How long could it take for CHOLBAM to work?
- How can I know if CHOLBAM is working?

Safety

- What are the possible side effects of CHOLBAM?
 - How can they be managed?
- Can CHOLBAM be taken with other medications or medical conditions?
- Do I take CHOLBAM with cholesterol supplementation?
- Where can I find data from clinical studies?

Monitoring

- What signs/symptoms should I be watching for in my loved one?
- How might the symptoms change over time?
- Are lab tests needed to monitor progress while taking CHOLBAM?
 - If yes, which ones?
- Are my cholesterol levels low or in the normal range?
- Growth and development can be affected in patients with SLOS.
 - Where am I/my loved one on the SLOS growth chart?

Support

- How do I get CHOLBAM?
- Where can I get more information or support?

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

My questions:



IMPORTANT SAFETY INFORMATION (continued)

- **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 [full Prescribing Information](#) for additional Important Safety Information.

Getting started is easy



- CHOLBAM® (cholic acid) capsules may help people*
 - Increase plasma cholesterol†
 - Grow on schedule*‡
 - Lower liver enzymes
- CHOLBAM has been FDA approved and available since 2015
- Mirum Access Plus works closely with your doctor and insurance plan to help facilitate coverage for CHOLBAM.
 - Eligible patients may pay as little as \$0 out of pocket per fill for CHOLBAM§

Learn more at CHOLBAM.com/mirum-access-plus-map/.

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†CHOLBAM was studied in a pilot study. Long-term studies are needed to determine durability of effect and possible clinical benefits.

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

§Subject to program terms and conditions.

Visit CHOLBAM.com to learn more.

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