Retrophin®

Cholbam® (cholic acid) capsules

Patient Enrollment Form for CHOLBAM® Total Care Hub

Phone: 844-CHOLBAM (844-246-5226) — Fax 877-473-3171

PATIENT INFORMATION

Patient First Name			MI
Last Name		Gender	☐ M ☐ F
Date of Birth	SS#		
Address			
City	State	ZIP	
Home Phone	Mobile Phone		
Preferred Method of Contact ☐ Home Phone ☐ Mobile Phone ☐ E-mail			
E-mail			

FOR PATIENTS UNDER 18:

Parent/Guardian First Name			MI
Last Name			
Address			
City	State	ZIP	
Home Phone	Mobile Phone		
E-mail			

PRIMARY INSURANCE Please attach a copy of both sides of the patient's insurance card(s)

Insurance Carrier	
Customer Service Phone	
Subscriber Name	
Relationship to Patient	
Employer Name	
Subscriber Date of Birth	
Subscriber ID Number	
Policy/Employer/Group Number	

PHARMACY BENEFITS–PRESCRIPTION DRUG CARD

Insurance Carrier	
Customer Service Phone	
Subscriber Name	Bin#
Subscriber Date of Birth	
Subscriber ID Number	
Policy/Employer/Group Number	

DIAGNOSIS/MEDICAL INFORMATION (This is for insurance purposes only, not to suggest approved uses for indication)

Diagnosis ☐ Bile Acid Synthesis Disorders (B.A.S.D.) ICD-10-CM Code ICD-10-CM Code/Diagnosis

Due to Single Enzyme Defect (check box):

- ☐ 3β-hydroxy-Δ5-C27-steroid oxidoreductase (also known as 3β-hydroxy Δ5-C27-steroid dehydrogenase/isomerase or 3β-HSD or HSD3β7) deficiency
- ☐ Δ4-3-oxosteroid 5β-reductase (Δ4-3-oxo-R or AKR1D1) deficiency
- ☐ Sterol 27-hydroxylase (CYP27A1) deficiency (presenting as cerebrotendinous xanthomatosis, CTX)
- ☐ 2- (or α-) methylacyl-CoA racemase (AMACR) deficiency
- ☐ Cholesterol 7 α-hydroxylase (CYP7A1) deficiency
- ☐ Smith-Lemli-Opitz
- ☐ Unknown

Due to Peroxisomal Disorder (check box):

- ☐ Peroxisomal Biogenesis Disorder: Zellweger's
- ☐ Peroxisomal Biogenesis Disorder: Neonatal adrenoleukodystrophy
- ☐ Peroxisomal Biogenesis Disorder: Type Unknown
- ☐ Peroxisomal Biogenesis Disorder: Refsum's
- ☐ Peroxisomal Biogenesis Disorder: Generalized peroxisomal disorder

Sex ☐ Male ☐ Female

Height _____ inches

Weight _____ kgs

Age:

Years _____

Months _____

CHOLBAM (cholic acid) ORAL CAPSULES PRESCRIPTION/ORDER

CHOLBAM Total daily dose = _____ **mg (Where the dose calculated is not a multiple of 50, the nearest dose below the maximum of 15 mg/kg/day should be selected, provided that is sufficient to suppress urinary bile acids. If not the next higher dose should be selected.)**

Sig:_____

Dispense 30 day supply **Number of refills** _____

Lab Test	Results	Date (mm/dd/yyyy)
Bilirubin total		
Bilirubin, direct (conjugated)		
Bilirubin, indirect (unconjugated)		
ALT/AST/GGT Liver function tests		
FAB-MS or similar technology		

PHYSICIAN CERTIFICATION

By signing below, I certify that (a) the above therapy is medically necessary and that I will supervise the patient's treatment accordingly; (b) I have received the necessary authorizations, including those required by state law and the Health Insurance Portability and Accountability Act of 1996 (HIPAA), to release the above information and other health and medical information of the patient to Retrophin, Inc., and the company or companies that help Retrophin administer the CHOLBAM Total Care Hub services; (c) I am prescribing the drug listed for the patient listed in this application based upon my independent medical judgment. By my signature below, I agree to receive certain reimbursement support services. I authorize Retrophin and Dohmen Life Science Services ("DLSS"), acting on behalf of Retrophin, to use the information contained in the prescription above, my name, and the name, address, and telephone number of my medical practice, and other applicable information, in order to provide me, my practice, and the patient listed in this application with the aforementioned reimbursement support services. I understand that participation in the CHOLBAM Total Care Hub services described does not constitute a guarantee on the part of Retrophin or parties acting on its behalf that (1) the drug I have prescribed will be reimbursed by the patient's or any insurance program, or (2) the patient will be eligible for any patient assistance program. I appoint Retrophin and its agents to convey this prescription—electronically or otherwise—to the dispensing pharmacy.

Prescriber's Signature **Date**

Prescriber NPI# **Prescriber State License #**

Prescriber's full, usual, and actual signature is required – no stamps. This form cannot be processed without the prescriber's signature.

Prescriber's First Name		MI	Last Name	
Address	City		State	ZIP
Phone	Fax		E-mail	
Office Contact Name		Phone		

Please Note: If you are faxing a prescription, it must be faxed from prescriber's facility to fax number (877) 473-3171.

Please return this form to the CHOLBAM (cholic acid) Total Care Hub by faxing it to (877) 473-3171.

PATIENT AUTHORIZATION AND RELEASE TO COLLECT, USE, AND DISCLOSE PROTECTED HEALTH INFORMATION

Before signing, the patient and/or patient's authorized representative should review and understand the terms of this Authorization and Release ("Authorization") before signing. If an authorized representative signs for the patient, please indicate the relationship to the patient.

I understand that the collection, use, and disclosure of the patient's health information are protected under law. Information contained in this Enrollment Form, such as the patient's name, address, insurance, prescription, and medical information, is "protected health information" ("PHI")." By signing this Authorization, the patient agrees to the collection, use, and disclosure of the patient's PHI as described below.

I understand that I may decline to sign this Authorization, and that doing so will not affect the patient's ability to receive CHOLBAM® (cholic acid) or obtain insurance or insurance benefits.

I understand that once PHI about the patient is released, based on this Authorization, privacy laws may not prevent (Retrophin and company or companies who administer the CHOLBAM (cholic acid) Total Care Hub Support Services ("Services") from further disclosing the patient's PHI. However, I understand that such entities have agreed to use or disclose PHI they receive only for the purposes described in this Authorization or as required by law. I understand that this Authorization will remain in effect for five (5) years. I also understand that I may revoke (withdraw) this Authorization at any time by sending a signed, written statement to the CHOLBAM Total Care Hub and faxing it to (877) 473-3167.

Revoking this Authorization will prohibit PHI disclosures after the date the written revocation is received by the CHOLBAM Total Care Hub, except to the extent that action has been taken already in on this Authorization. After I revoke this Authorization, the patient's PHI may be disclosed among Retrophin and the company or companies that help Retrophin administer the Services in order to maintain records of the patient's participation, but it will not be otherwise disclosed or used.

By signing below, I authorize Retrophin and the company or companies that help Retrophin administer the Services, to do the following:

1. Request and receive information from the patient's treating physician, healthcare professional, health insurer, or pharmacist necessary to investigate and resolve the patient's insurance coverage, coding, or reimbursement inquiry or to provide the reimbursement support service that were requested. Information may include the patient's medical diagnosis, condition, and treatment (including prescription information), the patient's health insurance, name, address and telephone number;
2. Collect, use, and disclose to each other any patient information including PHI provided to Retrophin for the purpose of investigating and resolving the patient's insurance coverage, coding, or reimbursement inquiry or to administer the Services, including entering and maintaining the patient's PHI in a database;
3. Contact me to discuss and receive educational and therapy or treatment support services designed for people taking CHOLBAM, including nutritional support and counseling;
4. Communicate with my healthcare providers and health plans about my benefit and coverage status and product administration (e.g., prescription, dosing, refills);
5. Disclose information to the patient's treating physician, healthcare professional, or pharmacist that I have provided to Retrophin as necessary to resolve patient insurance coverage, coding, or reimbursement inquiry. By signing below, I also authorize the insurer, treating physician, healthcare professional, and pharmacist to release PHI about the patient's prescribed medications and medical condition requested by Retrophin and the company or companies that help Retrophin administer the Services to administer the Services;
6. Contact the patient's insurer, other potential funding sources, social workers, patient advocacy organizations, or patient assistance programs (e.g., the CHOLBAM Total Care Hub) on the patient's behalf to determine if the patient may be eligible for health insurance coverage or other funds, and disclose to them PHI about the patient's prescribed medications and medical condition that has been provided by the patient or patient's authorized representative or physician, healthcare provider, or pharmacist; and
7. Disclose any PHI obtained from the sources listed above to third parties, if required by law, and to conduct surveys to evaluate the effectiveness of the CHOLBAM Total Care Hub program.

Retrophin and Services administer agree to protect the patient's PHI by using and disclosing the patient's PHI only for the reasons listed above or as required by law

Patient's Signature		**Date**	
Print Patient's Name			
Legally Authorized Representative's Signature (if needed)			
Print Legally Authorized Representative's Name			
Relationship to Patient ☐ **Spouse** ☐ **Legal Guardian** ☐ **Representative per Power of Attorney**			
Representative's Address			
Phone		**Mobile Phone**	

Fax this form, along with both sides of the patient's Medical and Prescription Drug Benefit cards to CHOLBAM Total Care Hub at (877) 473-3171.
Retain a copy of this form in the patient's records.

Please see Important Safety Information and full Prescribing Information at www.cholbam.com.

CHOLBAM® (CHOLIC ACID) INDICATION & SAFETY INFORMATION

INDICATIONS AND USAGE

CHOLBAM is a bile acid indicated for:

- Treatment of bile acid synthesis disorders due to single enzyme defects (SEDs). (1.1)
- Adjunctive treatment of peroxisomal disorders (PDs) including Zellweger spectrum disorders in patients who exhibit manifestations of liver disease, steatorrhea or complications from decreased fat soluble vitamin absorption

Limitation of use:

The safety and effectiveness of CHOLBAM on extrahepatic manifestations of bile acid synthesis disorders due to SEDs or PDs including Zellweger spectrum disorders have not been established.

WARNINGS AND PRECAUTIONS - Exacerbation of Liver Impairment

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

- Monitor AST, ALT, GGT, alkaline phosphatase, bilirubin and INR every month for the first 3 months, every 3 months for the next 9 months, every 6 months during the next three years and annually thereafter. Administer the lowest dose that effectively maintains liver function.
- Discontinue CHOLBAM if liver function does not improve within 3 months of starting treatment, if complete biliary obstruction develops, or if there are persistent clinical or laboratory indicators of worsening liver function or cholestasis; continue to monitor liver function and consider restarting a lower dose when parameters return to baseline.

ADVERSE REACTIONS

Most common adverse reactions (≥1%) are diarrhea, reflux esophagitis, malaise, jaundice, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy.

DRUG INTERACTIONS

- Bile Salt Efflux Pump (BSEP) Inhibitors (e.g., cyclosporine): Avoid concomitant use; if concomitant use is necessary, monitor serum transaminases and bilirubin
- Bile Acid Resins and Aluminum-Based Antacids: 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 or aluminum-based antacids.

PREGNANCY

No studies in pregnant women or animal reproduction studies have been conducted with CHOLBAM. Women who become pregnant during CHOLBAM treatment are encouraged to enroll in the COCOA Pregnancy Surveillance Registry.

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. There are no animal lactation data and no data from case reports available in the published literature.

OVERDOSAGE

In the event of overdose (elevated GGT and ALT) the patient should be monitored and treated symptomatically. Elevated serum gamma glutamyltransferase (GGT) and serum alanine aminotransferase (ALT) may indicate CHOLBAM overdose. 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 (cholic acid) 50mg and 250mg capsules

To report SUSPECTED ADVERSE REACTIONS, contact Retrophin, Inc. at 1-877-659-5518 or FDA at 1-800-FDA-1088 or **www.fda.gov/medwatch**.