



# LEADING THE WAY IN Rare Disease

Dedicated to supporting you, your patients, and their caregivers with:



A growing portfolio of products focused on treating patients with rare diseases



Select diagnostic testing kits to help detect rare diseases early



A top-tier patient support program to fulfill our promise to the community

MIRUM INVESTIGATIONAL PIPELINE

	Preclinical	Phase 1	Phase 2B/Phase 3	Approved
<b>VOLIXIBAT</b>				
Primary Sclerosing Cholangitis (PSC)			<b>VISTAS</b>	
Primary Biliary Cholangitis (PBC)			<b>VANTAGE</b>	
<b>CHENODAL</b> (chenodiol)				
Cerebrotendinous Xanthomatosis (CTX)			<b>RESTORE*</b>	

\*Chenodal (CDCA) is not indicated for CTX, but has received a medical necessity determination in the United States by the FDA for CTX. Data from the Phase 3 RESTORE clinical trial examining the safety and efficacy of Chenodal for the treatment of CTX were announced on October 2, 2023.

Explore products Mirum has available today.



# COMMERCIALY AVAILABLE PRODUCTS



LIVMARLI is indicated for the treatment of cholestatic pruritus in patients who are 3 months of age and older with Alagille syndrome.

LIVMARLI is indicated for the treatment of cholestatic pruritus in patients who are 5 years of age and older with progressive familial intrahepatic cholestasis (PFIC).

## LIMITATIONS OF USE FOR LIVMARLI

LIVMARLI is not recommended in a subgroup of patients with PFIC type 2 with specific *ABCB11* variants resulting in nonfunctional or complete absence of bile salt export pump protein.

## IMPORTANT SAFETY INFORMATION

### CONTRAINDICATIONS

Contraindicated in patients with prior or active hepatic decompensation events (eg, variceal hemorrhage, ascites, or hepatic encephalopathy).

### WARNINGS AND PRECAUTIONS

**Hepatotoxicity:** In PFIC, drug-induced liver injury occurred in 4 patients, including 1 liver transplant and 1 death. In Alagille syndrome and PFIC, treatment-emergent elevations or worsening of liver tests occurred. Obtain baseline liver tests and monitor during treatment; monitor also for liver-related adverse reactions and signs of hepatic decompensation. Dose reduction or treatment interruption may be considered if abnormalities occur. Permanently discontinue if persistent liver test abnormalities, clinical hepatitis upon rechallenge, or hepatic decompensation occur.

**Gastrointestinal Adverse Reactions:** Diarrhea and abdominal pain were reported as the most common adverse reactions in patients treated with LIVMARLI. Consider reducing the dosage or interrupting treatment if a patient experiences persistent diarrhea or abdominal pain.

**Fat-Soluble Vitamin (FSV) Deficiency:** Obtain baseline levels and monitor during treatment. Supplement vitamins if deficiency is observed. Consider interrupting LIVMARLI if bone fractures or bleeding occur.

**Please see full Prescribing Information for LIVMARLI.**

# COMMERCIALY AVAILABLE PRODUCTS (cont'd)



CHOLBAM<sup>®</sup> (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 FOR CHOLBAM

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.

## Select Important Safety Information

Patients will need to undergo laboratory testing periodically while on treatment to assess liver function. CHOLBAM may worsen liver impairment and patients should immediately report to their health care provider any symptoms associated with liver impairment, like yellowing of the skin or the whites of the eye, dark or tea-colored urine, pain on the right side of stomach, bleeding or bruising more easily than normal, or increased lethargy. Discontinue CHOLBAM if liver function worsens while on treatment.

Please see full [Prescribing Information](#) for CHOLBAM.

MIRUM

## DIAGNOSTIC TEST KITS

Mirum offers a sponsored, no-charge, third-party diagnostic testing program for patients with a family history or suspected diagnosis of rare diseases. For select diagnostic testing, third-party genetic counseling may be available for eligible patients.

We have a selection of different diagnostic kits available. Contact your Mirum representative to learn more or to order a kit.

## EXCEPTIONAL ACCESS AND PATIENT SUPPORT

Mirum Access Plus offers you and your patients the following:



Insurance coverage and access support



Financial support for eligible patients



Personalized patient support



Patient and caregiver support tools



Mirum Access Plus mobile communication tool

Explore more at [MirumAccessPlus.com](https://MirumAccessPlus.com)

