Committed to Advancing Research in Cholestatic Liver Disease

Joanne Quan, MD, Chief Medical Officer

September 12, 2025

Disclaimers

- This presentation contains information regarding investigational products. These products have not been approved by any regulatory body as safe and effective for the treatment of the indications discussed herein
- The information presented herein should not be interpreted or construed in any way as providing medical advice or as a replacement or substitute for medical advice provided by a healthcare provider

Mirum—Our People and Our Commitment

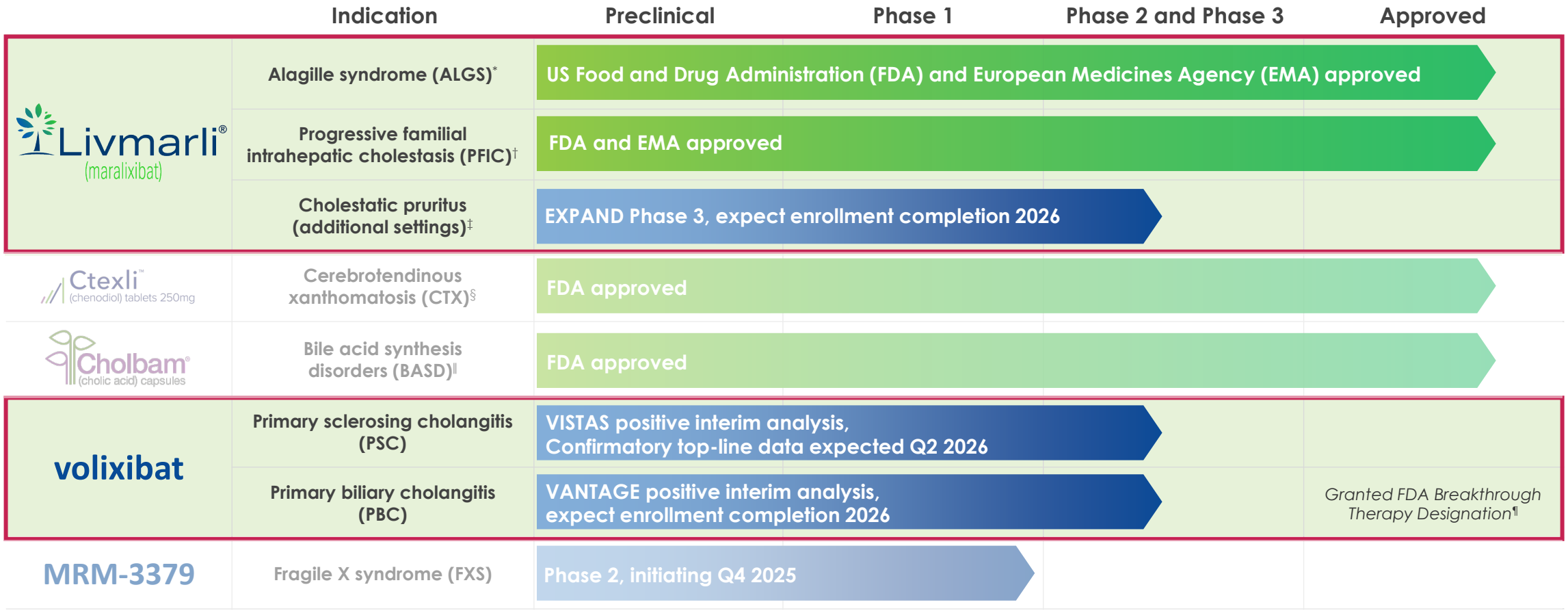
Our people
350+ employees globally—including experts in liver disease, rare disease, and children's medicine

Our purpose
We aim to transform the lives of people with rare diseases with new therapies

Our plan
Highlight the voices of patients, their families, and advocates every step of the way

3 Approved Therapies With More in Development

FOCUSED ON RARE DISEASES WITH HIGH NEED



*Received FDA approval for cholestatic pruritus in patients with ALGS who are 3 months of age and older. European Commission has granted marketing authorization for LIVMARLI® (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with ALGS who are 2 months of age and older.
†Received FDA approval for cholestatic pruritus in patients with PFIC who are 12 months of age and older. European Commission has granted marketing authorization for LIVMARLI® (maralixibat) oral solution for the treatment of PFIC in patients 3 months of age and older.
‡Using liquid oral formulation for the EXPAND study looking at patients with additional settings of ultra rare cholestatic pruritus, excluding intrahepatic cholestasis of pregnancy, ALGS, PBC, PFIC, and PSC.
§Received FDA approval for the treatment of adults with CTX.
‖BASD include peroxisome biogenesis disorder-Zellweger spectrum disorder.
¶Breakthrough Therapy Designation, granted by the FDA, is a process designed to expedite the development and review of drugs for serious or life-threatening conditions, when preliminary clinical evidence suggests the drug may demonstrate a substantial improvement over existing therapies on at least 1 clinically significant endpoint.

Ileal Bile Acid Transporter Inhibition in Cholestatic Liver Disease

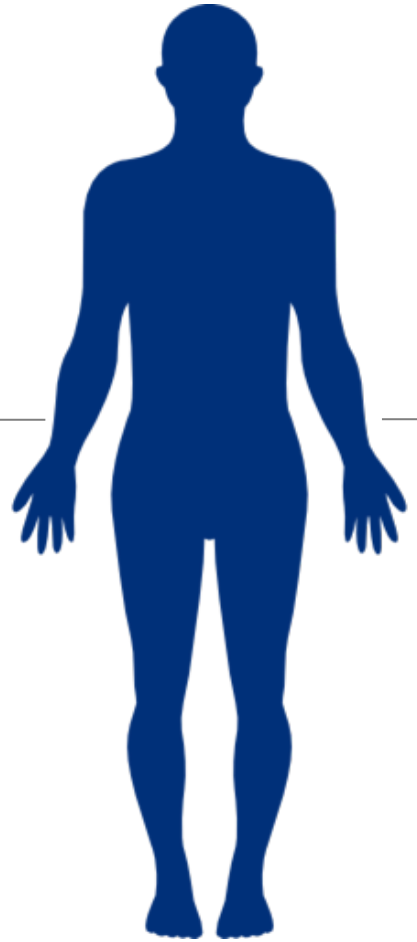
What Symptoms Most Impact Patients With PSC?

AS SHOWN IN THE *VOICE OF THE PATIENT* REPORT*:

ITCH
- Unrelenting, uncontrollable itch; often painful, interrupts sleep and slows down or prevents all routine activities

FATIGUE
- Extreme, different from being tired, sleep does not seem to help

MENTAL AND EMOTIONAL HEALTH ISSUES
- Anxiety, depression, post-traumatic stress disorder (PTSD); emotions can intensify physical symptoms
- Children and young adults may suffer from these issues more intensely



IMPAIRED COGNITIVE FUNCTION
- Brain fog, loss of identity, memory gaps, and an inability to function independently

PAIN
- Chronic, often does not improve with medication, impacts daily life
- Abdominal, liver, joint, and generalized pain

OTHER SYMPTOMS OF CONCERN
- Insomnia, enlarged or swollen veins in the esophagus, loss of appetite/weight loss, nausea/vomiting, and bone loss

*PSC Partners Seeking a Cure. Voice of the Patient: Primary Sclerosing Cholangitis (PSC). Retrieved from the website https://pscpartners.org/about/the-disease/voice-of-the-patient-report-pfdd-forum.html Accessed 3 Sept 2024.

PSC and PBC: Rare Liver Diseases That Lead to Bile Acid Buildup (Cholestasis)

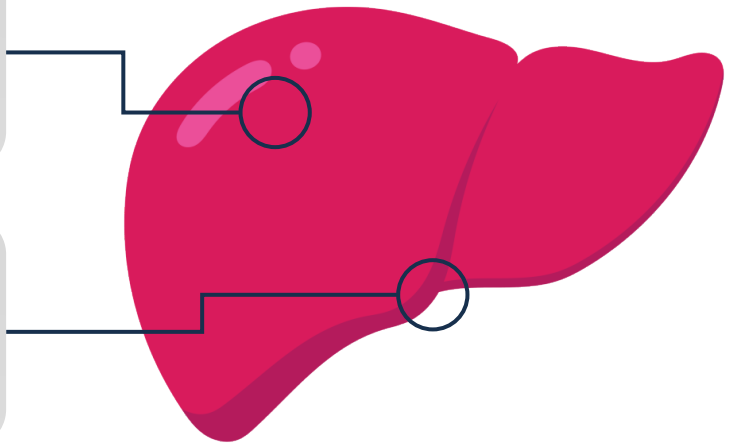
Bile acid buildup is associated with symptoms (itch, fatigue) and progressive liver disease*†

Intrahepatic bile acid buildup

can occur when bile flow is slowed down (cholestasis) by the narrowing of bile ducts†

PSC: Narrowed bile ducts due to scarring (fibrosis)
54,000 patients from the United States and Europe
65% of patients with active itch

PBC: Impaired bile ducts due to inflammation, causing a toxic buildup of bile acids (cholestasis)
230,000 patients from the United States and Europe
60% of patients with active itch



Intrahepatic bile acid buildup leads to*†:

- Liver dysfunction and symptom development
- Liver inflammation and scarring of bile ducts with progressive liver damage
- Cholestatic markers may appear elevated in blood

Blocking the ileal bile acid transporter (IBAT) is expected to reduce the bile acid buildup with improvement of symptoms associated with liver dysfunction (itch and fatigue) in PSC and PBC‡

*Gotthardt DN, Rupp C, Bruhin M, et al. Pruritus is associated with severely impaired quality of life in patients with primary sclerosing cholangitis. *Eur J Gastroenterol Hepatol.* 2014;26(12):1374-1379. doi:10.1097/MEG.0000000000000223
†NIDDK. Definition & Facts for Primary Sclerosing Cholangitis. February 2022. Accessed September 8, 2025. https://www.niddk.nih.gov/health-information/liver-disease/primary-sclerosing-cholangitis/definition-facts.
‡BusinessWire. Volixibat Data from Mirum's VANTAGE PBC Study Showcased at EASL. May 9, 2025. Accessed September 8, 2025. https://www.businesswire.com/news/home/20250509189818/en/Volixibat-Data-from-Mirums-VANTAGE-PBC-Study-Showcased-at-EASL

Blocking IBATs in Cholestatic Liver Disease

Targeting IBATs may stop the harmful buildup of bile acids in cholestasis

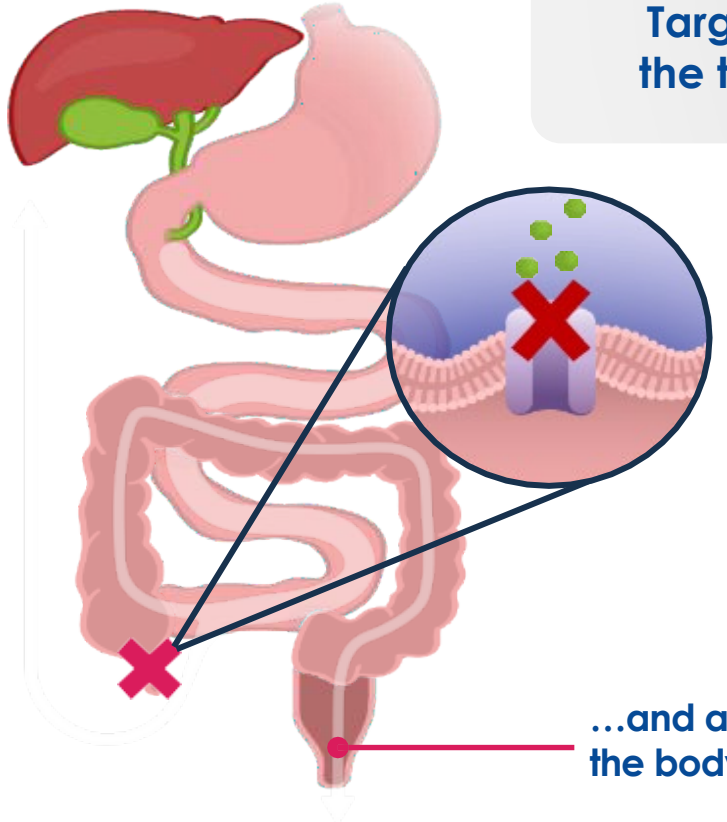
The IBAT

A protein that plays an important role in maintaining normal bile acid levels.

Cholestatic liver disease

Defined by impaired bile flow and toxic **buildup of bile acids**

- **Severe itch**
- **Cellular damage**
- **Poor outcomes**



Targeting IBATs lowers the total bile acid pool

By stopping bile acids from being reabsorbed, they no longer cycle back to the liver...

...and are removed from the body through the stool

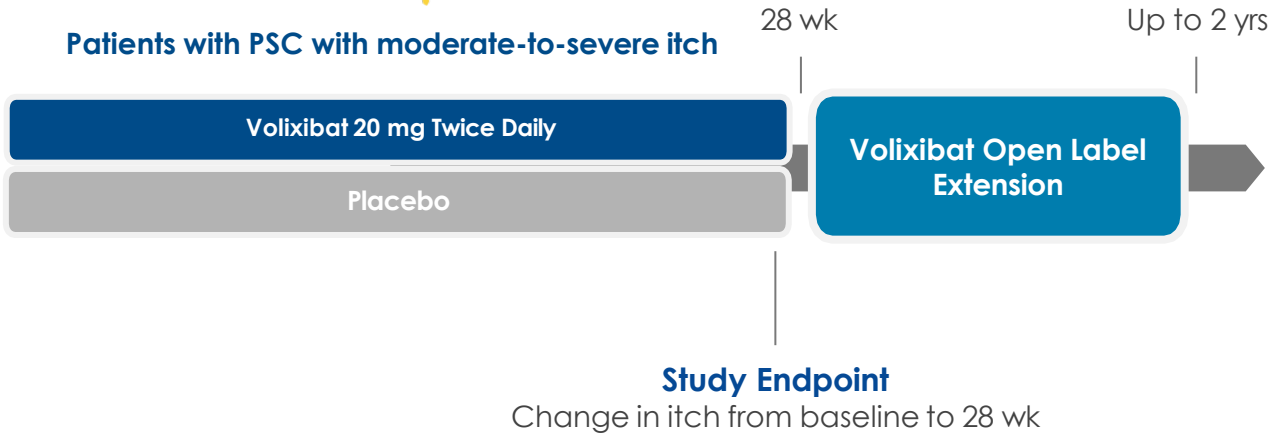
Clinical Studies Update

Study of Volixibat in Patients With PSC With Itch

- Large, global clinical study*
- Early study results show positive signs†

vistas

EFFICACY & SAFETY OF VOLIXIBAT IN THE TREATMENT OF CHOLESTATIC PRURITUS IN PATIENTS WITH PSC



Enrollment Completed!
Preliminary results expected Q2 2026

*Clinicaltrials.gov. A study to evaluate efficacy and safety of an investigational drug named volixibat in patients with itching caused by primary sclerosing cholangitis (PSC) (VISTAS) August 8, 2025. Accessed September 5, 2025. https://clinicaltrials.gov/study/NCT04663308?cond=PSC&intr=Volixibat&rank=1#study-plan

†Mirum's Volixibat Achieves Positive Interim Analyses in VANTAGE PBC and VISTAS PSC Studies. Press release. Mirum Pharmaceuticals, Inc. June 17, 2024. Accessed June 21, 2024 at: https://ir.mirumpharma.com/news-events/News/news-details/2024/Mirums-Volixibat-Achieves-Positive-Interim-Analyses-in-VANTAGE-PBC-and-VISTAS-PSC-Studies/default.aspx.

With deep appreciation to PSC Partners and the entire PSC community,

your collaboration, engagement, and advocacy are vital to advancing our shared mission.

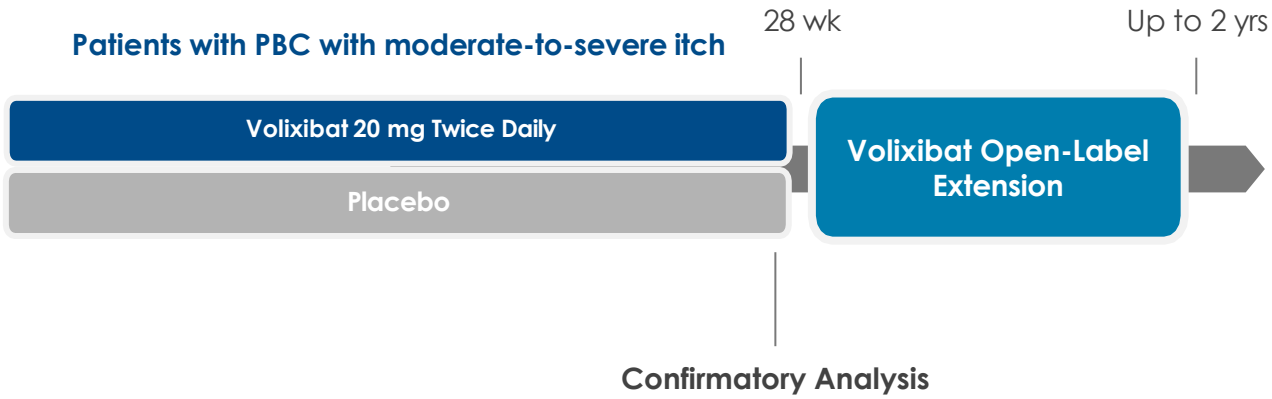
THANK YOU!

vistas
EFFICACY & SAFETY OF VOLIXIBAT IN THE TREATMENT OF CHOLESTATIC PRURITUS IN PATIENTS WITH PSC

Study of Volixibat in Patients With PBC With Itch

- This study is designed to measure the change in itch for patients with moderate to severe itch taking volixibat (20 mg twice daily) vs a placebo over the course of 28 weeks*†
- At the end of 28 weeks, patients are then given the option to take volixibat for up to 2 years (open-label extension)
- Secondary endpoints include: safety, tolerability, bile acid levels, disease markers, and quality of life measures*†

VANTAGE



Participants are randomized 1:1 between volixibat 20 mg and placebo.

Enrollment completion is expected in 2026

*Clinicaltrials.gov. a study to evaluate efficacy and safety of an investigational drug named volixibat in patients with itching caused by primary biliary cholangitis (VANTAGE). September 3, 2025. Accessed September 5, 2025. https://clinicaltrials.gov/study/NCT05050136

†BusinessWire. Volixibat Data from Mirum's VANTAGE PBC Study Showcased at EASL. May 9, 2025. Accessed September 5, 2025. https://www.businesswire.com/news/home/20250509189818/en/Volixibat-Data-from-Mirums-VANTAGE-PBC-Study-Showcased-at-EASL

We Want to Hear From You!

Visit the Mirum Booth

THANK YOU!

Please visit our booth for more information.