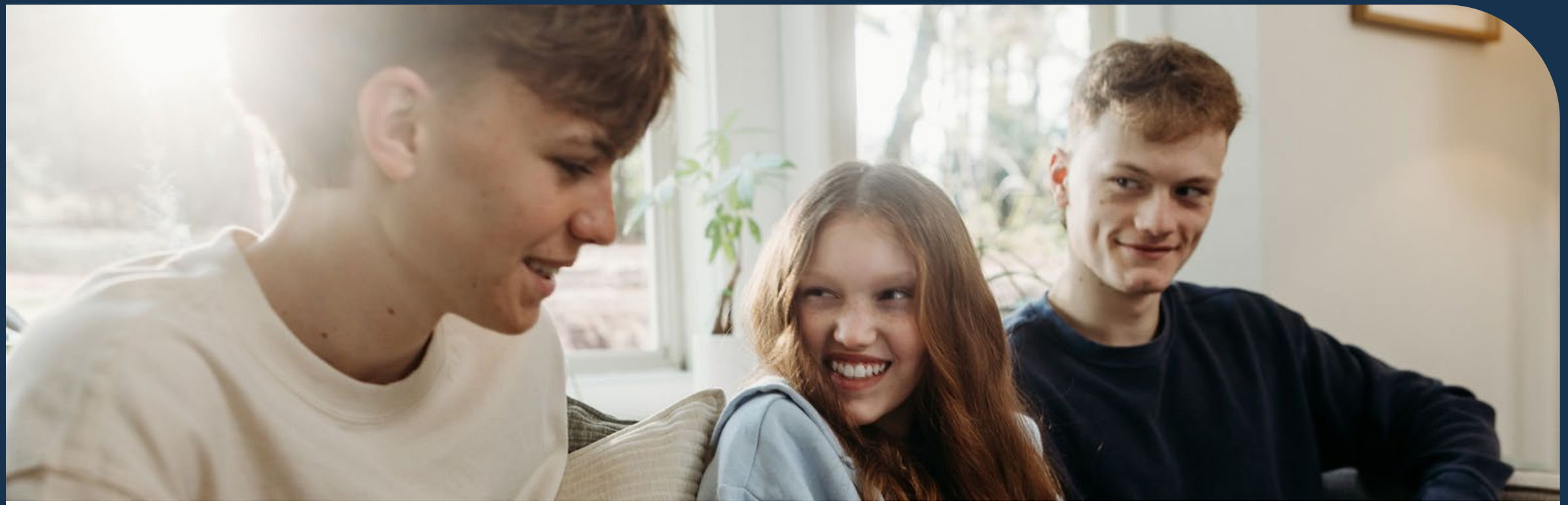


MIRUM PHARMACEUTICALS

DELIVERING HIGH IMPACT MEDICINES FOR RARE DISEASE

November 2026

Forward-Looking Statements

This presentation contains “forward-looking” statements that are based on our management’s beliefs and assumptions and on information currently available to management. Forward-looking statements include all statements other than statements of historical fact contained in this presentation, including, but not limited to, information concerning: our business strategy, objectives and opportunities, including the future opportunities for, and benefits of, our approved medicines and our product candidates; our expectations regarding the timing and achievement of clinical and regulatory milestones for our approved medicines and product candidates; our expectations regarding our future financial performance, including our 2026 net product sales guidance and being cash flow positive in 2027; our estimates of potential revenue for our approved medicines; our estimated total addressable markets for our approved medicines and product candidates; our assumptions regarding the prevalence of the diseases our approved medicines and product candidates are designed to target; the expected benefits of acquisitions and in-licenses; our expectations regarding our intellectual property coverage; and our expectations regarding the performance of third-party collaborators, licensors and distribution partners in the development, regulatory submission and commercialization of our approved medicines and product candidates. Forward-looking statements are subject to known and unknown risks, uncertainties, assumptions and other factors that may cause our actual results, performance or achievements to differ materially and adversely from those anticipated or implied by our forward-looking statements, including, but not limited to: the results, enrollment, conduct and progress of our ongoing and planned studies for our product candidates, including in-licensed product candidates and the product candidates we have acquired; our plans and expectations for commercializing our approved medicines in the United States and rest of world; the costs of our business strategy, commercialization plans and development programs; the financial impact or revenues from any commercialization we undertake; estimates of the number of patients impacted by the diseases or related diseases that we seek to treat and who are appropriate for treatment with our approved medicines; the potential clinical benefits and safety profile of our approved medicines and our product candidates; our expected growth, including the integration of acquired companies and assets; our ability to obtain necessary regulatory approvals for our product candidates within our anticipated timeframes or at all, or predictions of the outcome of any regulatory consideration and, if and when approved, market acceptance of our products; our dependence on third-party clinical research organizations, manufacturers, suppliers and distributors; the design, implementation, timelines and outcomes of our clinical trials; the impact of competitive products and therapies; our ability to obtain necessary additional capital on acceptable terms or at all; our ability to attract and retain key employees; our ability to manage the growth and complexity of our organization; our ability to maintain, protect, enforce and enhance our intellectual property; and our ability to continue to stay in compliance with applicable laws and regulations. You should refer to the section entitled “Risk Factors” set forth in our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and other filings we make with the Securities and Exchange Commission (SEC) from time to time (available at http://www.sec.gov) for a discussion of other important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. You should not rely upon forward-looking statements as predictions of future events. We undertake no obligation to update any forward-looking statements after the date of this presentation except as may be required by law.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth, disease prevalence, peak revenue potential and other data about our industry and product portfolio. These data involve a number of assumptions and limitations, and Mirum makes no representation as to the accuracy of such estimates. Projections, assumptions and estimates of the future performance of the markets in which we operate, and of the future commercial potential of our approved medicines and product candidates, are necessarily subject to a high degree of uncertainty and risk. The trademarks included herein are the property of the owners thereof and are used for reference purposes only.

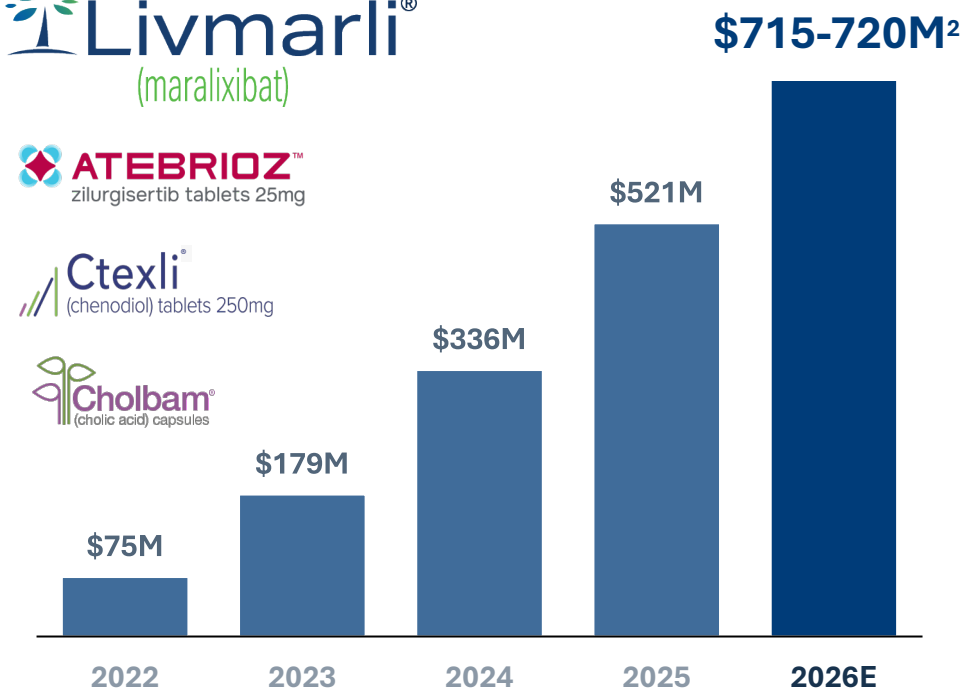
This presentation discusses product candidates that are under clinical study and which have not yet been approved for marketing by the U.S. Food and Drug Administration or other relevant regulatory authorities. No representation is made as to the safety or effectiveness of these product candidates for the use for which such product candidates are being studied.

Livmarli® (maralixibat)

ATEBRIOZ™ zilurgisertib tablets 25mg

Ctexli® (chenodiol) tablets 250mg

Cholbam® (cholic acid) capsules



Annual Net Product Sales[1]

5

Approved Commercial Indications

5

Upcoming Potential Approvals & Label Expansions

$4B+

Peak Revenue Potential of Mirum Portfolio[3]

High impact medicines for patients with rare liver and genetic diseases

Delivering Significant Value Through a Purpose-Built Rare Disease Model

[1] Annual net product sales 2022-2025. [2] 2026 net product sales guidance. [3] Mirum estimates of peak revenue potential.

Driving Growth Through Focus on Rare Liver, Rare Genetic Diseases

Portfolio with Multi-Billion Dollar Revenue Potential[1]

Rare Liver Disease Business

LIVMARLI	ALGS, PFIC Ultra-Rare Cholestasis	**$1Bn+**
Volixibat	PSC & PBC	**$1Bn+**
Brelovitug	HDV	**$1Bn+**

Rare Genetic Disease Business

CHOLBAM & CTEXLI	BASD, CTX	**$200M+**
ATEBRIOZ	FOP	**$200M+**
MRM-3379	FXS	**$1Bn+**

Global Commercial Reach



Worldwide Commercial Rights

LIVMARLI | Volixibat | Brelovitug | ATEBRIOZ | MRM-3379

[1] Mirum estimates of peak revenue potential.

Commercial Portfolio with Pipeline of Growth Opportunities

4 APPROVED RARE DISEASE MEDICINES, 5 ADDITIONAL INDICATIONS IN DEVELOPMENT IN HIGH-NEED ORPHAN INDICATIONS

		Indication	Preclinical	Phase 1	Phase 2 and Phase 3	Approved
Rare Liver Diseases	Livmarli® (maralixibat)	Alagille Syndrome (ALGS)[1]	✓ FDA and EMA approved			
		Progressive Familial Intrahepatic Cholestasis (PFIC)[2]	✓ FDA and EMA approved			
		Cholestatic Pruritus (Additional Settings)[3]	EXPAND Phase 3 primary endpoint met, sNDA submission targeted H1 2027			
	volixibat	Primary Sclerosing Cholangitis (PSC)	VISTAS Phase 2b primary endpoint met, NDA submission targeted H1 2027			***Granted FDA Breakthrough Therapy Designation***
		Primary Biliary Cholangitis (PBC)	VANTAGE Phase 2b positive interim analysis, topline data expected Q1 2027			***Granted FDA Breakthrough Therapy Designation***
	brelovitug	Chronic Hepatitis Delta Virus (HDV)	AZURE-1 Phase 3 primary endpoint met; AZURE-4 topline data expected Q4 2026 (US registrational program) AZURE 2 & 3, Phase 3 topline data expected H2 2027 (EU registrational program)			***Granted FDA Breakthrough Therapy and EMA PRIME Designations***
Rare Genetic Diseases	Ctexli® (chenodiol) tablets 250mg	Cerebrotendinous Xanthomatosis (CTX)[4]	✓ FDA approved			
	Cholbam® (cholic acid) capsules	Bile Acid Synthesis Disorders (BASD)[5]	✓ FDA approved			
	ATEBRIOZ™ zilurgisertib tablets 25mg	Fibrodysplasia Ossificans Progressiva (FOP)[6]	✓ FDA approved			
	MRM-3379	Fragile X Syndrome (FXS)	BLOOM Phase 2, topline data expected in 2027			***Granted FDA Fast Track Designation***

[1] Received U.S. FDA approval for cholestatic pruritus in patients with Alagille syndrome 3 months of age and older. European Commission has granted marketing authorization for LIVMARLI® (maralixibat) for the treatment of cholestatic pruritus in patients with Alagille syndrome 2 months of age and older.
[2] Received U.S. FDA approval for cholestatic pruritus in patients with PFIC 12 months of age and older. European Commission has granted marketing authorization for LIVMARLI® (maralixibat) for the treatment of PFIC in patients 3 months of age and older. [3] Using liquid oral formulation for the EXPAND study looking at patients with additional settings of ultra-rare cholestatic pruritus, excluding PSC, PBC, ICP, ALGS and PFIC. [4] Received U.S. FDA approval for the treatment of adults with cerebrotendinous xanthomatosis (CTX). [5] Bile acid synthesis disorders include Peroxisome biogenesis disorder-Zellweger Spectrum Disorder (PBD-ZSD). [6] Received U.S. FDA approval to reduce the volume of total new heterotopic ossification (HO) in adult and pediatric patients aged 12 years and older with fibrodysplasia ossificans progressiva (FOP).

RARE LIVER DISEASES

Significant Expansion Opportunities within Pipeline Indications

LIVMARLI®

Alagille Syndrome (ALGS)
Progressive Familial Intrahepatic Cholestasis (PFIC)

IBAT Inhibition in Pediatric Cholestatic Liver Disease

Targeting IBAT Removes Circulating Bile Acids Addressing Toxic Bile Acid Accumulation

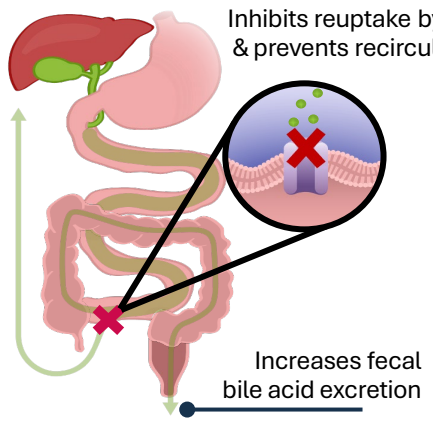
Cholestatic Liver Disease

Defined by impaired bile flow & hepatotoxic build-up of bile acids

- **Severe pruritus**
- **Cellular damage**
- **Poor outcomes**

Targeting IBAT Lowers Bile Acids

Mechanism directly addresses bile acid accumulation



IBAT Inhibition Clinical Benefits[1-5]

Transplant-free survival

Quality of life

Growth

Pruritus

Bilirubin (PFIC)

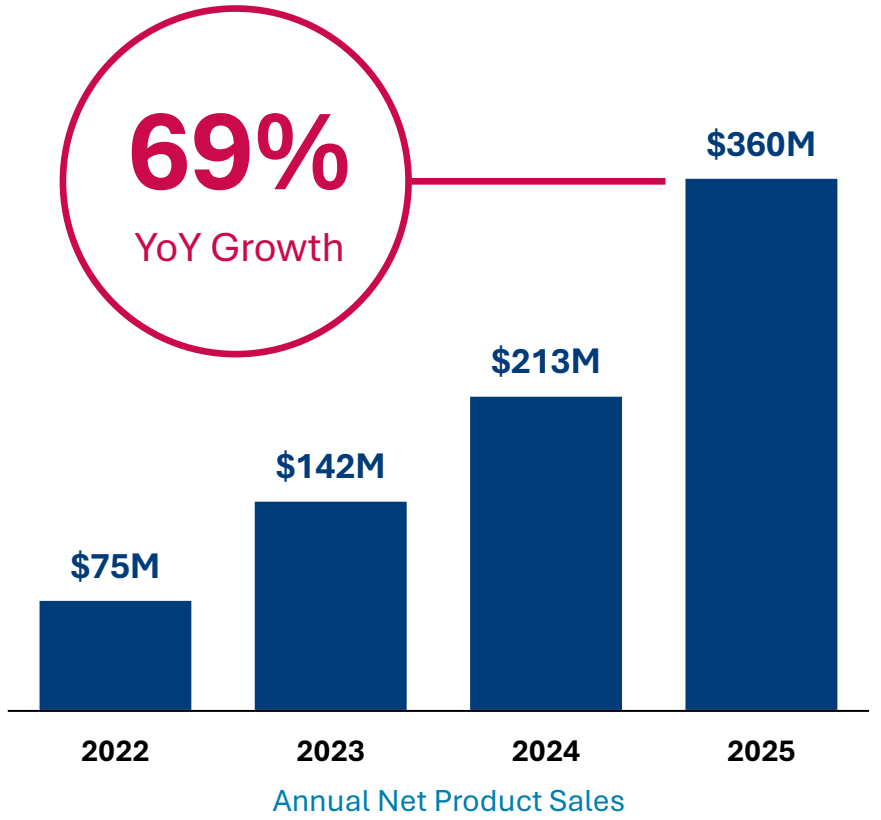
Xanthomas (ALGS)

[1] Gonzales E et al. *Lancet*. 2021;398:1581-1592. [2] Loomes KM et al. *Hepatol Commun*. 2022;6(9):2379-2390. [3] Thompson R. Serum bile acid control in long-term maralixibat-treated patients is associated with native liver survival in children with progressive familial intrahepatic cholestasis due to bile salt export pump deficiency. Presented at: EASL 2020; August 2020. Accessed April 29, 2021. https://linkinghub.elsevier.com/retrieve/pii/S0168827820307571. [4] van Wessel DBE et al. *J Hepatol*. 2021;73(1):84-93. [5] Sokol J, Gonzales E, Kamath BM, et al. Predictors of 6-year event-free survival in patients with Alagille syndrome treated with maralixibat, an IBAT inhibitor. Paper presented at: European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN): Annual Meeting; June 22-25, 2022; Copenhagen, Denmark.

LIVMARLI: A Leading Medicine for Rare Cholestatic Pruritus

Livmarli®
(maralixibat)

Available in Both Oral Solution and Tablet Formulation



Annual Net Product Sales

Expanding Impact in Rare Cholestatic Pruritus



Livmarli®
(maralixibat)

ALGS

Approved
2,000 US/EU addressable

PFIC

Approved
4,000+ US/EU adult addressable
~1,000 US/EU pediatric addressable

EXPAND

Phase 3
Biliary Atresia
2,000 US/EU addressable
sNDA submission targeted H1 '27

Path to Sustained Long-Term Growth

- ✓ Continued growth in new Rx
- ✓ Strong adherence & persistence
- ✓ Weight-based dosing
- ✓ Int'l market expansion
- ✓ IP Protection to 2040+

LIVMARLI (MARALIXIBAT)

Label Expansion Opportunity in Cholestatic Pruritus

LIVMARLI Label Expansion Opportunity in Cholestatic Pruritus

Broad Unmet Need in Multiple Ultra-Rare Cholestatic Conditions

Pruritus in Ultra-Rare Cholestatic Settings

e.g., Biliary Atresia, Secondary Sclerosing Cholangitis, Others

Characteristic Cholestatic Burden



Elevated sBA
Severe pruritus
Stunted growth
Impaired QoL

Estimated Addressable US/EU Biliary Atresia Patients[1]

2,000

LIVMARLI Is Uniquely Positioned to Address the Burden of Cholestatic Pruritus

[1] Biliary Atresia patients with native liver and cholestatic pruritus, Dilwali et al, Abstract #2648 AASLD 2026

EXPAND Phase 3 Study

Key Inclusion Criteria

- Diagnosis of cholestatic liver disease excluding ALGS, PFIC, PSC, PBC, and ICP
- Moderate to severe cholestatic pruritus
- Total serum bile acids >2×ULN



Primary Endpoint
Change in pruritus from baseline to 20wk[2]

All Pediatric Population n=46
Biliary Atresia subgroup, n=25

[1] Maralixibat 285 ug/kg is equivalent to 300 ug/kg maralixibat chloride. BID, twice daily.
[2] ItchRO(Obs) is a Worst Itch 0-4 numerical rating scale. Baseline compared to change in average worst-daily pruritus score from baseline to Weeks 13–20

EXPAND Phase 3 Baseline Demographics – All Pediatric Population

	MRX (N=22)	Placebo (N=24)
Age at enrollment in years (SD)	6.6 (4.4)	6.0 (4.0)
Sex (Female %)	10 (45.5)	11 (45.8)
ItchRO(Obs) Score (SD)	2.65 (0.96)	2.65 (0.70)
sBA, μmol/L (SD)	149.7 (92.9)	155.4 (98.6)
ALT, U/L (SD)	95.4 (59.4)	134.2 (88.2)
Total bilirubin, mg/dL (SD)	1.1 (0.7)	1.2 (1.2)
UDCA usage at baseline (%)	21 (95.5)	23 (95.8)
Disease diagnosis: Biliary Atresia with native liver[1]	11 (50.0)	14 (58.3)

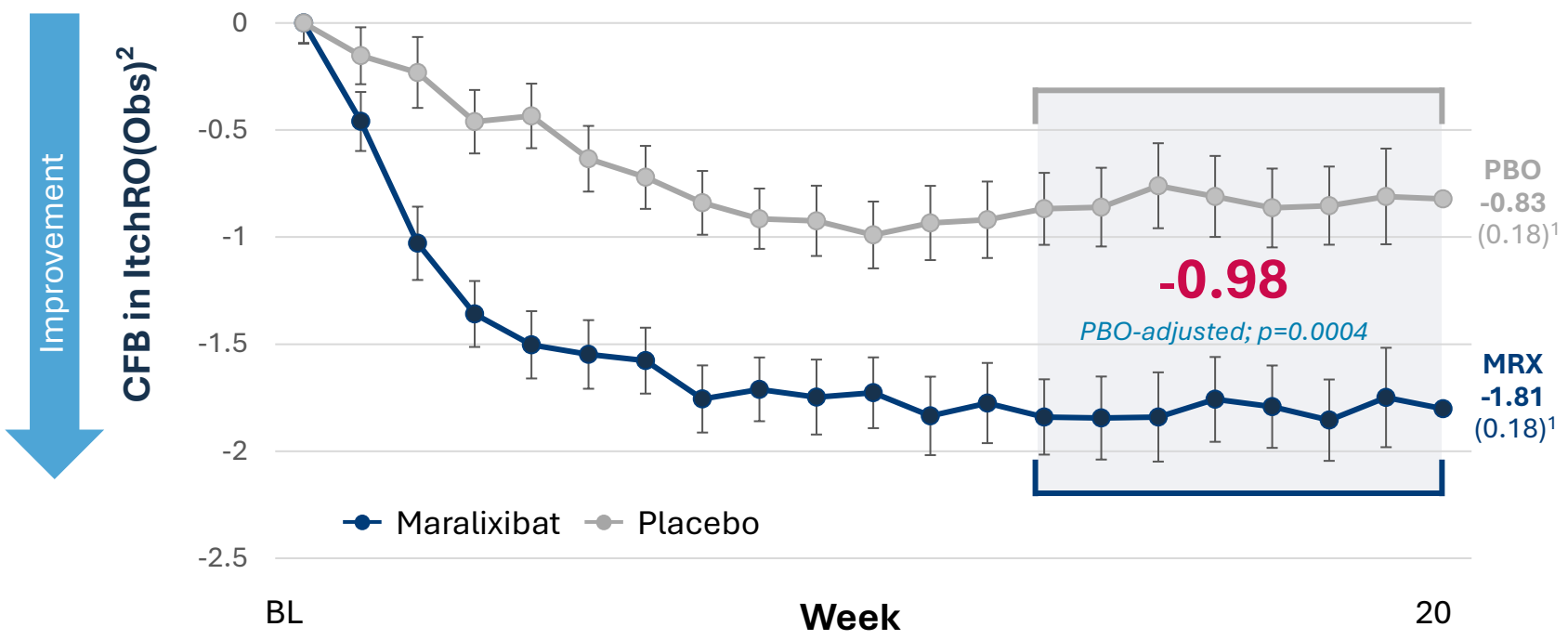
[1]Other disease diagnoses included: ischemic cholangiopathy, ARC syndrome, alpha-1 antitrypsin deficiency, and secondary sclerosing cholangitis

EXPAND Phase 3 Primary Endpoint Met

Rapid, Sustained, Clinically Meaningful Reductions in Pruritus; sNDA Submission Planned 1H 2027

Average Pruritus Score (ItchRO[Obs]) Over 20 Weeks

(All Pediatric Population)



Biliary Atresia Subgroup

-1.15

PBO-adjusted reduction; p=0.0002

Serum Bile Acids

-69%

PBO-adjusted reduction in Overall Population; p<0.0001

All Secondary Endpoints Statistically Significant

[1] LS means (SE). LS mean, SE, and p-values are from MMRM model. Values represent LS mean change from baseline compared to change in average worst-daily pruritus score from baseline to Weeks 13–20. Error bars denote ± standard error (SE).
[2] ItchRO(Obs) is a daily Worst Itch 0-4 numerical rating scale.

EXPAND Phase 3 Safety Summary (Safety Population)

n (%)	MRX (n=25)	Placebo (n=27)
Participants with any treatment-emergent adverse event (TEAE)	24 (96.0)	26 (96.3)
Grade 3 or higher TEAEs	6 (24.0)	6 (22.2)
Related to study drug	1 (4.0)	0
Serious TEAEs	5 (20.0)	5 (18.5)
TEAE that led to death	0	0
TEAE that led to premature discontinuation from study	1 (4.0)	0
TEAEs of interest		
Diarrhea	16 (64.0)	3 (11.1)
Abdominal pain	5 (20.0)	4 (14.8)
ALT increased	2 (8.0)	1 (3.7)

Maralixibat's safety profile was generally consistent with the known effects of IBAT inhibition

Serious adverse events (SAEs) in MRX-treated patients: pneumonia, pyrexia, large intestine infection, acute kidney injury, viral infection with bronchiolitis; none related. SAEs in placebo-treated patients: pneumonia, pancytopenia, sepsis, forearm fracture, cholangitis.

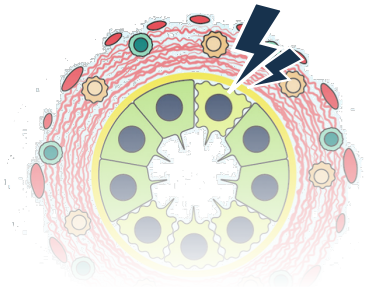
VOLIXIBAT

IBAT Inhibitor for Cholestasis in Adults (PSC, PBC)

PRIMARY SCLEROSING CHOLANGITIS

PSC: A Rare Immuno-Inflammatory Cholestatic Liver Disease

Progressive Biliary Injury Drives Cholestasis and Liver Damage[1]



Progressive biliary strictures

↓

Impaired bile flow, bile acid accumulation

↓

Scarring and destruction of bile ducts

↓

Biliary fibrosis and cirrhosis

Serious and Compounding Disease Consequences[1-4]

Pruritus and Fatigue
Heavy symptom burden

Elevated Laboratory Tests
ALP, Bilirubin, ALT, AST

Cholangitis
Recurrent, unpredictable

Autoimmune Comorbidities
~70% have concomitant IBD

Increased cancer risk
Cholangiocarcinoma, colorectal, gallbladder

No Approved Therapies Significant Need

~30,000

Est. US Prevalence
~54,000 patients US/EU

Elevated Bile Acid Levels Drive Severe Symptom Burden (Pruritus, Fatigue) and Progressive Liver Disease

ALP, alkaline phosphatase; ALT, alanine aminotransferase; AST, aspartate aminotransferase; IBD, inflammatory bowel disease

[1] Trivedi PJ, et al. *Gastroenterol*. 2024. [2] Kuo A, et al. *Clin Gastroenterol Hepatol*. 2019. [3] Tanaka A & Mertens *JC. Inflamm Intest Dis*. 2016. [4] Karlsen TH, et al. *J Hepatol*. 2017.

PSC: Pruritus is Common and Often Moderate to Severe

Pruritus is a Registrational Endpoint

Pruritus Is a Significant Burden

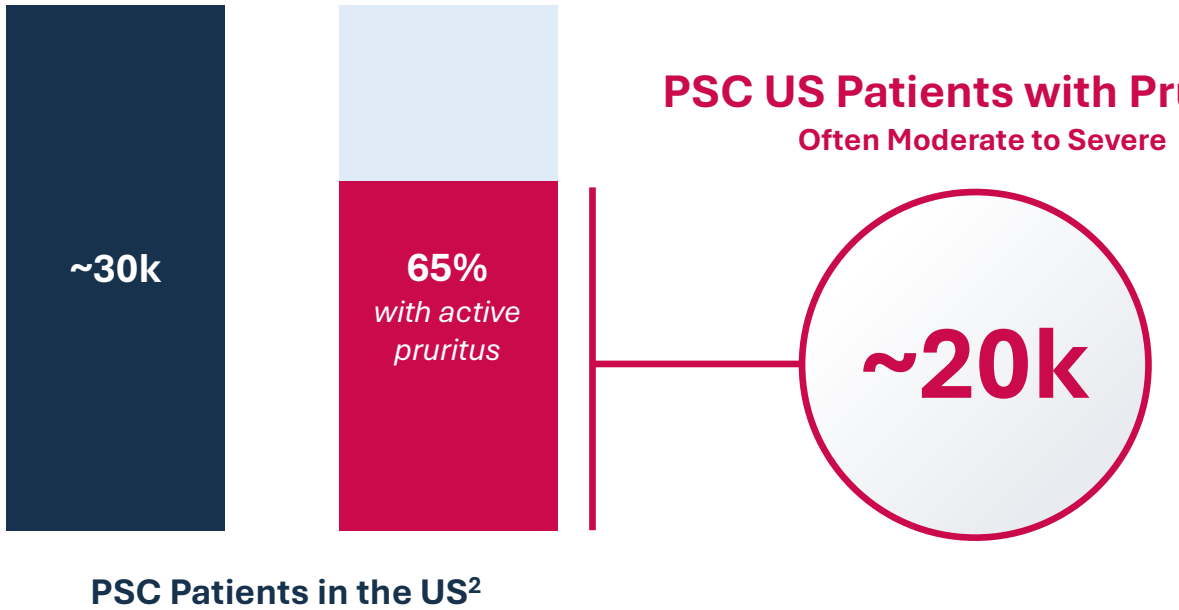
8

Median worst itch score (0-10 NRS) from last itching episode[1]

> ***This debilitating itch is merciless, all consuming, and overwhelming.***
> *- Kristina, patient with PSC[1]*

> ***It is…like your blood is itchy. The bile is in your blood…you can't reach the itch.***
> *- Nicola, patient with PSC[1]*

No Approved Therapies; Significant Opportunity in PSC



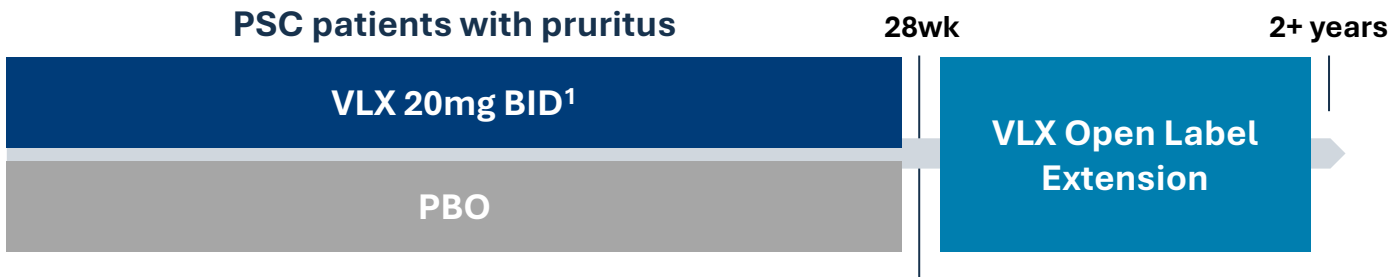
[1] Kowdley KV, et al. Presented at EASL 2022. Survey conducted in 482 patients with PSC; not all patients responded to all questions. [2] Mirum Market Research.

Phase 2b Study of Volixibat in PSC Patients with Cholestatic Pruritus

GRANTED FDA BREAKTHROUGH THERAPY DESIGNATION

vistas

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



Primary Endpoint

Change in pruritus from baseline to 28wk[2]

Primary Cohort, n=111
(moderate to severe pruritus)

Secondary Cohort, n=47
(mild pruritus)

Targeting NDA Submission in H1 2027

[1] Participants are randomized 1:1 between Volixibat 20mg and Placebo. BID, twice daily. 2 Adult ItchRO is a Worst Itch 0-10 numerical rating scale.

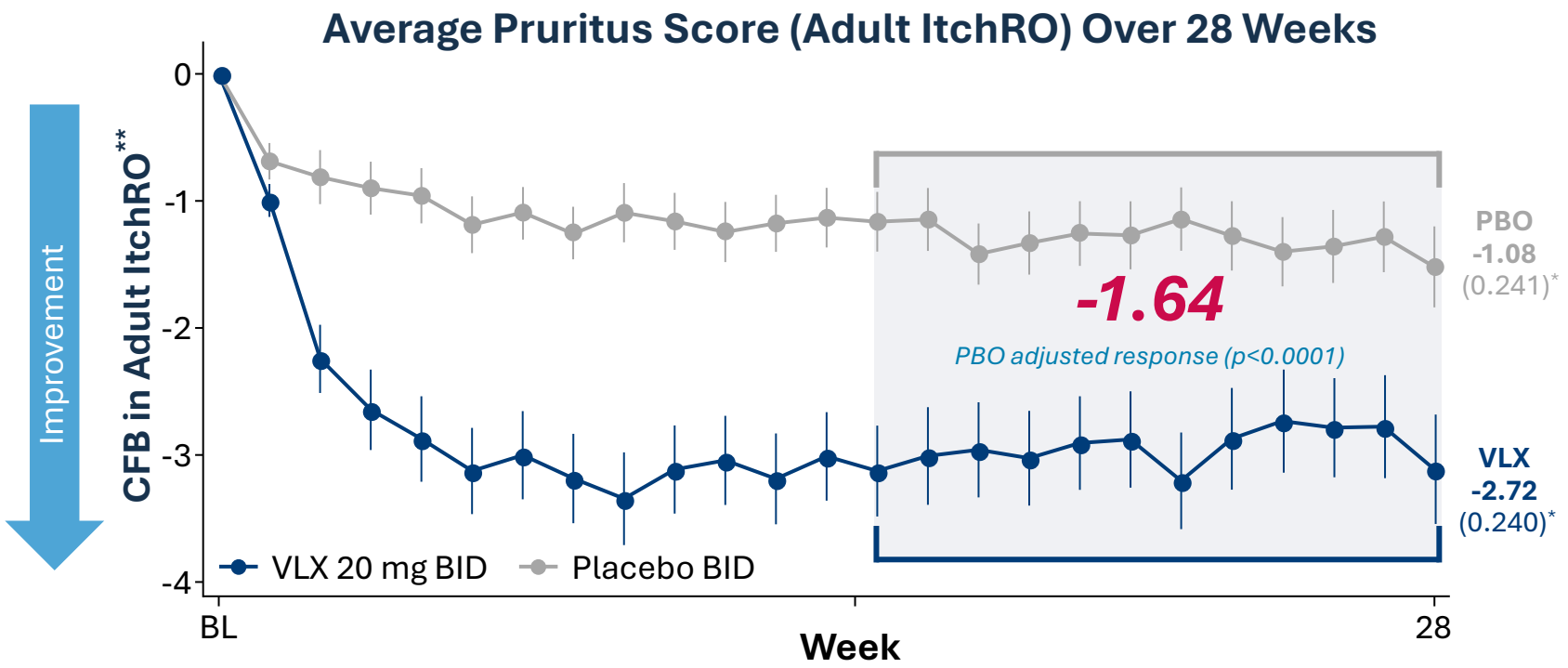
VISTAS Phase 2b Baseline Demographics – Primary Cohort

	VLX 20 mg (N=54)	Placebo (N=57)
Age at enrollment in years (SD)	45.2 (15.7)	44.3 (14.2)
Sex (Female %)	25 (46.3)	35 (61.4)
Adult ItchRO Score (SD)	6.3 (1.6)	6.1 (1.5)
PSC Type (Large Duct %)	49 (90.7)	53 (93.0)
sBA in μmol/L (SD)	109.6 (137.5)	61.4 (83.2)
ALP in U/L (SD)	334 (189.6)	365 (232.2)
ALT in U/L (SD)	73 (46.8)	87 (58.5)
Total Bilirubin mg/dL (SD)	1.2 (0.8)	1.1 (0.8)
IBD (%)	40 (74.1)	42 (73.7)
UDCA usage at baseline (%)	43 (79.6)	43 (75.4)

sBA, serum bile acid; ALP, alkaline phosphatase; ALT, alanine aminotransferase; IBD, inflammatory bowel disease.
Levy, et al., EASL 2026

VISTAS Phase 2b Met Primary Endpoint

Rapid, Sustained, Clinically Meaningful Reductions in Pruritus



56%
≥2 point reduction in pruritus

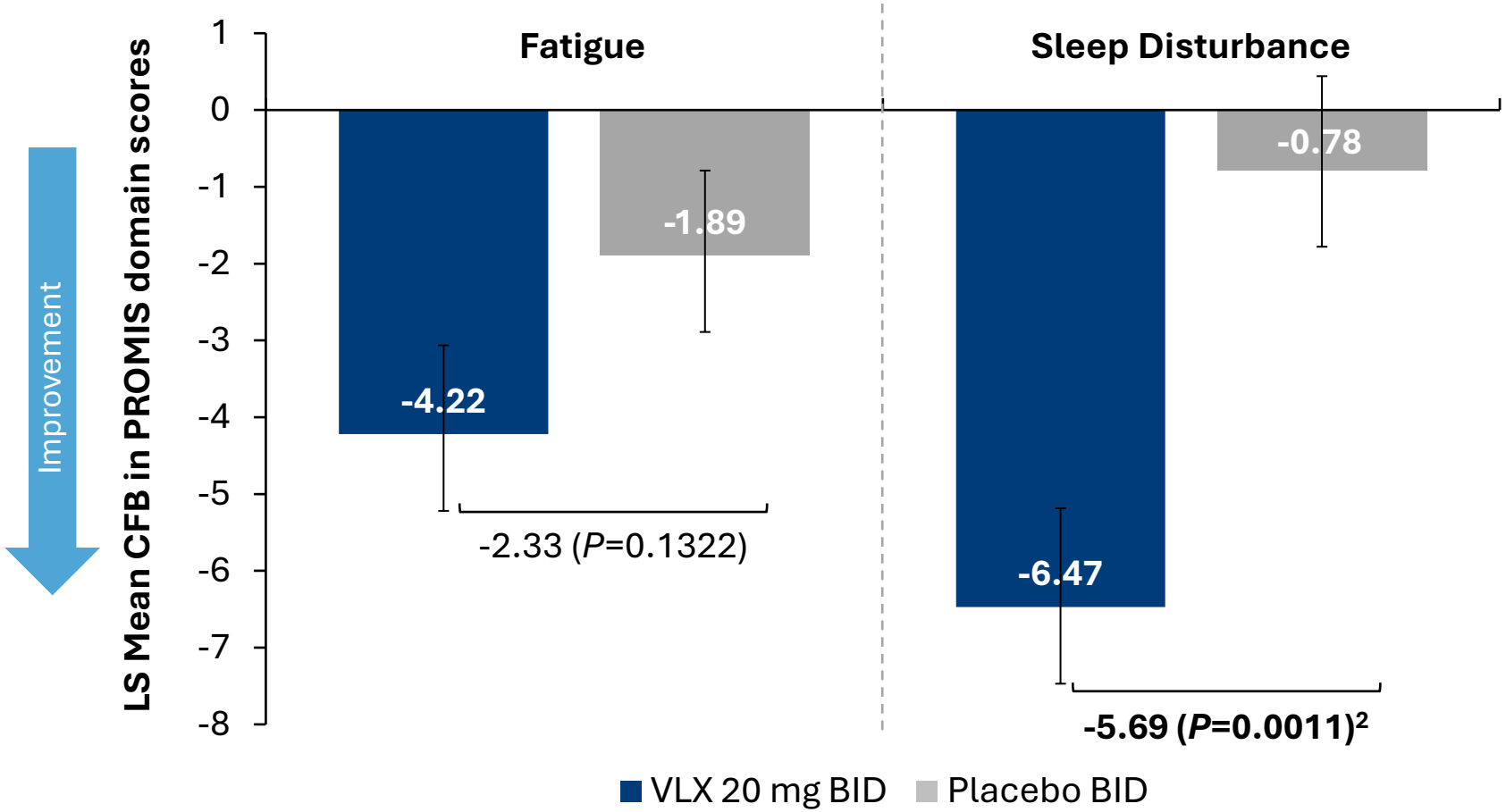
Serum Bile Acids
Statistically significant reduction

Secondary Cohort
Statistically significant reduction in pruritus

* LS Means (SE), LSMean, SE and p-values are from MMRM model. Values represent change from baseline to the average of the last 12 weeks of treatment (weekly averaged worst daily itch score). Error bars denote the 95% CI.
**Adult ItchRO is a 0-10 numerical rating scale.
Levy, et al., EASL 2026

VISTAS: Volixibat Treatment Showed Improvements in Fatigue & Sleep

Change in PROMIS Fatigue and Sleep Scores at Week 28[1]



BID, twice daily; CFB, change from Baseline; HRQoL, health-related quality of life; MMRM, mixed-effects model for repeated measures; PROMIS, Patient-reported Outcomes Measurement Information System; VLX, volixibat.
[1] Primary cohort. Data are LS mean with ±SE. Effect size compared the difference between volixibat and placebo, averaged over the last 12 weeks using MMRM. [2] Nominal p-value due to hierarchy of regression analysis.
Levy, et al., EASL 2026

VISTAS Phase 2b Safety Summary (Primary and Secondary Cohorts)

n (%)	VLX 20 mg (N=77)	Placebo (N=81)
Participants with any treatment emergent adverse event (TEAE)	72 (93.5)	68 (84.0)
Grade 3 or higher TEAEs	10 (13.0)	9 (11.1)
Serious TEAEs	8 (10.4)	5 (6.2)
TEAE that led to death	0	1 (1.2)
TEAE that led to premature discontinuation from study	7 (9.1)	2 (2.5)
Study discontinuation due to diarrhea	3 (3.9)	1 (1.2)

Volixibat's safety profile was generally consistent with the known effects of IBAT inhibition

Serious adverse events (SAEs) in volixibat treated patients included cholangitis, infection (sepsis, liver abscess, viral infection), abdominal pain, cholangiocarcinoma, sclerosing cholangitis, biliary colic, pyrexia, back pain, procedure-related pancreatitis; none related. SAEs in placebo patients included cholangitis, constipation, spontaneous bacterial peritonitis, liver function tests increases, radius fracture.

Elevations in ALT, AST, ALP and bilirubin were observed more frequently in volixibat treated patients than placebo treated patients

Levy, et al., EASL 2026

TEAEs Occurring in ≥5% of Patients (Primary and Secondary Cohorts)

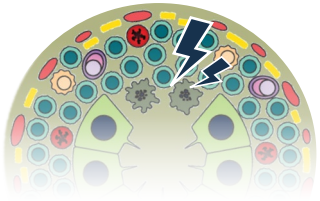
n (%)	VLX 20 mg (N=77)	Placebo (N=81)
Diarrhea	31 (40.3)	7 (8.6)
Abdominal Pain	14 (18.2)	8 (9.9)
Nausea	10 (13.0)	3 (3.7)
Vitamin D deficiency	8 (10.4)	4 (4.9)
Abdominal pain upper	7 (9.1)	6 (7.4)
ALT increased	7 (9.1)	3 (3.7)
Upper respiratory tract infection	6 (7.8)	6 (7.4)
Back pain	5 (6.5)	2 (2.5)
Fatigue	5 (6.5)	4 (4.9)
Blood bilirubin increased	4 (5.2)	3 (3.7)
Jaundice	4 (5.2)	1 (1.2)
Pyrexia	4 (5.2)	1 (1.2)
Influenza	3 (3.9)	5 (6.2)

TEAE, treatment emergent adverse event; ALT, alanine aminotransferase.
Levy, et al., EASL 2026

PRIMARY BILIARY CHOLANGITIS

PBC: A Chronic, Progressive, Immune-Mediated Cholestatic Disease

Destruction of Intrahepatic Bile Ducts Causes Cholestasis and Liver Damage[1]



Autoimmune damage (e.g. AMAs) to intrahepatic bile ducts

↓

Impaired bile flow, bile acid accumulation

↓

Scarring and destruction of bile ducts

↓

Biliary fibrosis and cirrhosis

Slow Progressing, with Serious and Compounding Disease Consequences[1,2]

Pruritus and Fatigue
Heavy symptom burden

Elevated Laboratory Tests
ALP, Bilirubin, ALT, AST

Immune-Mediated Condition Associations
e.g., Sjögren's, thyroid disease

Risk of Liver-Related Complications
Cirrhosis, liver failure, transplant, HCC

Most Prevalent Cholestatic Liver Disease

~85,000

Est. US Prevalence
~230,000 patients US/EU

9:1
female to male
sex ratio[1]

Elevated Bile Acid Levels Drive Severe Symptom Burden (Pruritus, Fatigue) and Progressive Liver Disease

AMA, anti-mitochondrial antibodies; ALP, alkaline phosphatase; ALT, alanine aminotransferase; AST, aspartate aminotransferase; HCC, hepatocellular carcinoma
[1] Park JW, et al. *Biomedicines*. 2022. [2] Trivedi PJ, et al. *Gastroenterol*. 2024.

PBC: Pruritus is Common and Often Moderate to Severe

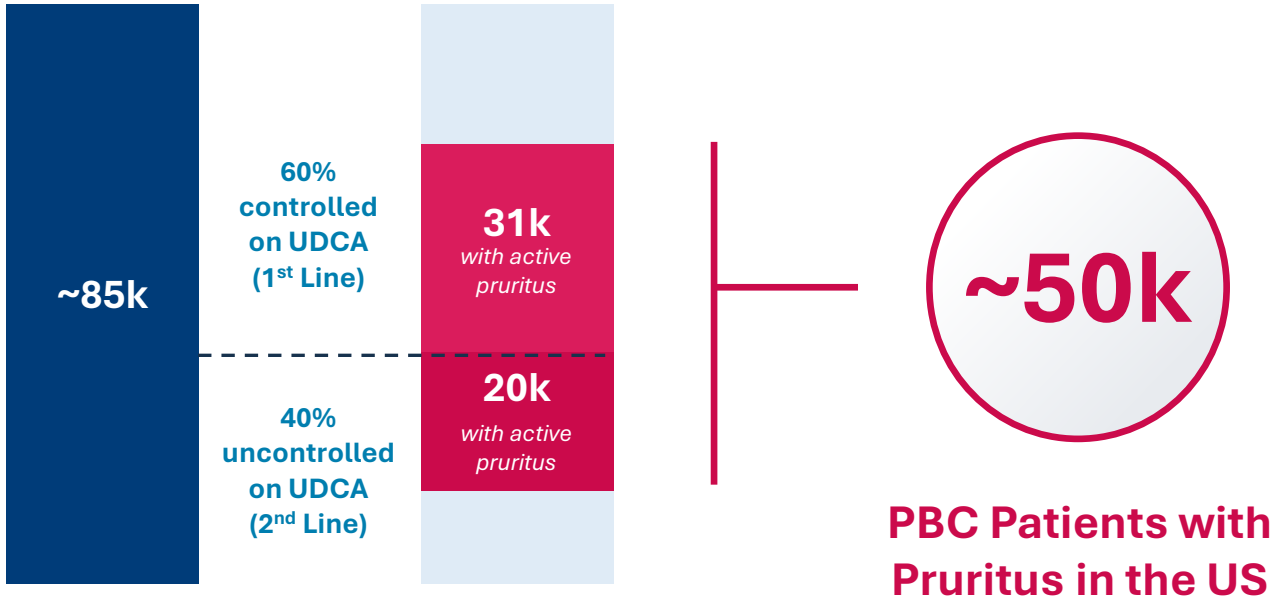
Significant Opportunity Across 1st & 2nd Line Settings

Pruritus

Significantly impacts daily QoL

> ***I found myself itching my arms so much that I had bruises on my arms...***
> *- Rose, patient with PBC[1]*

> ***I began itching all over...It was unlike any itch I ever experienced. I scratched so much, my skin was raw.***
> *- Donna, patient with PBC[1]*



Diagnosed PBC Patients in the US[2]

Similar rate of pruritus in 1st & 2nd line (~60%)

[1] PBCers Organization. PBCers Stories. Retrieved from website https://pbcers.org/stories/. Accessed October 23, 2024. [2] Mirum Market Research.

Phase 2b Study of Volixibat in PBC Patients with Cholestatic Pruritus

Positive Interim Analysis

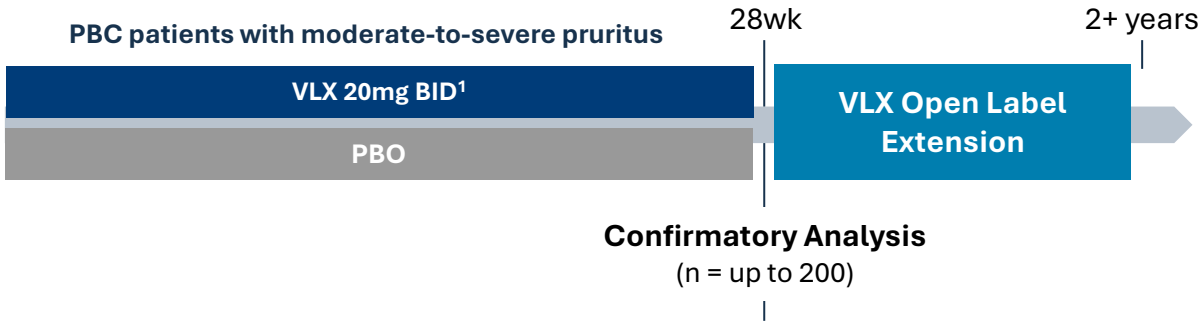
Rapid and statistically significant improvement in pruritus

Reductions in sBA and improvements in fatigue

20 mg BID dose selected

Granted FDA Breakthrough Therapy Designation

VANTAGE



Primary Endpoint

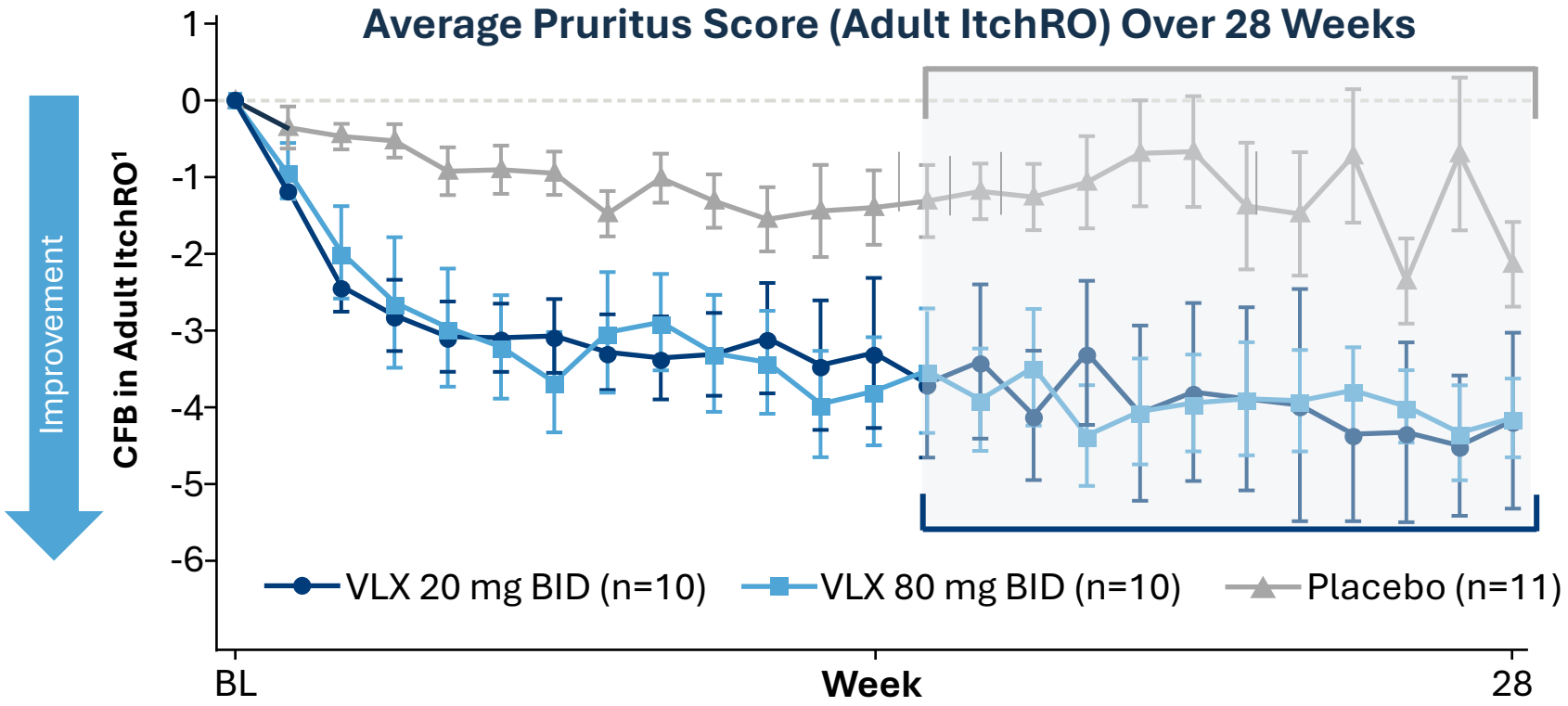
Change in pruritus from baseline to 28wk

Topline Data Expected Q1 2027

[1] Participants are randomized 1:1 between Volixibat 20mg and Placebo. BID, twice daily.

VANTAGE Interim Analysis: Reduction in Pruritus from Baseline

Rapid and Statistically Significant Reductions in Pruritus



PBO Adjusted Response[2]:

VLX 20mg -2.4 (*P*=0.0039)

VLX 80mg -2.6 (*P*=0.0010)

Safety: Diarrhea occurred in 77% of patients (mostly mild) with one discontinuation
3 serious TEAEs reported (one on placebo), not treatment-related

[1]Adult ItchRO is a 0-10 worst itch numerical rating scale where 0 = no itch and 10 = worst possible itch. [2] LS mean (95% CI) change from Baseline to the average of the last 12 weeks of treatment. LS means and P values were calculated using an MMRM model.
Heneghan, et al., EASL 2025

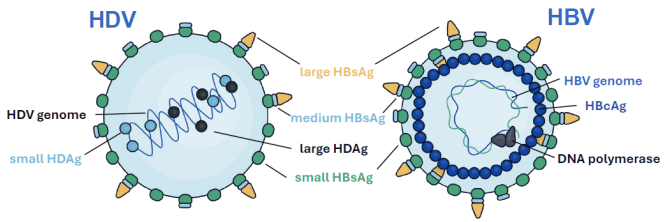
BRELOVITUG

Chronic Hepatitis Delta Virus (HDV)

HDV Is the Most Severe Form of Viral Hepatitis

A Rapidly Progressive Disease with Significant Unmet Need

Hepatitis Delta Virus



Requires Hepatitis B coinfection

Hepatitis B Surface Antigen (HBsAg) necessary for HDV to replicate and spread

>50%

Liver-Related **Death in 10 Years**[1]

5yrs

Avg. Progression to **Cirrhosis** and **Liver Failure**[2]

3x

Risk of **Liver Cancer (HCC)** vs. HBV[3]

~15,000

US pts diagnosed, insured, under care[4]

~40,000 Est. US Prevalence

>230,000 prevalence US/EU, >12M WW

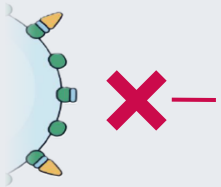
A significant global unmet need

[1] Negro, F. & Lok, A. *S JAMA* 2023. [2] Miao et al, *The Journal of Infectious Diseases* 2019. [3] Sagnelli, C. et al. HBV/HDV Co-Infection: Epidemiological and Clinical Changes, Recent Knowledge and Future Challenges. *Life* 11, 169 (2021). [4] Mirum estimates.

Brelovitug: Preliminary Efficacy and Favorable Safety Profile in HDV

Brelovitug

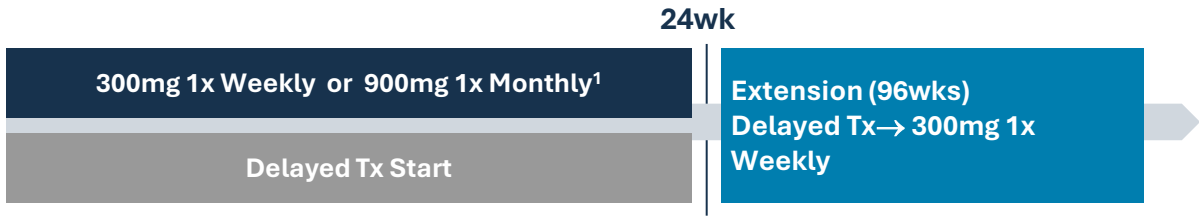
Fully human anti-HBsAg monoclonal antibody
SC injection 1x Weekly or 1x Monthly



Binds to HBsAg
Neutralizes HDV/HBV
Clears virions & subviral particles

100% Virologic Response Demonstrated in P2 Clinical Trial

AZURE-1 Phase 2b/3 Study Design



Primary Endpoint
Week 24 Virologic Response[2] + ALT normalization
P2b n=53; P3 n=153

The Phase 3 portion of the AZURE-1 study met primary endpoint

Granted FDA Breakthrough Therapy & EU PRIME Designations

[1] Participants are randomized 2:2:1. [2] Virologic Response = HDV RNA ≥2 $\log_{10}$ reduction or TND.

AZURE-1 Phase 3 Key Baseline Disease Characteristics

	300 mg QW N=59	900 mg Q4W N=65	Delayed Treatment N=29
Cirrhosis, n (%)[1]	24 (41)	27 (42)	12 (41)
Liver stiffness, kPa, mean (SD)	14.3 (10.78)	13.7 (8.01)	14.6 (11.49)
ALT, U/L, mean (SD)	130.4 (106.88)	121.3 (95.07)	121.0 (84.29)
ALT ≥5x ULN, n (%)	12 (20.3)	11 (16.9)	5 (17.2)
Clinically significant portal hypertension, n (%)[2]	11 (18.6)	13 (20.0)	7 (24.1)
HBsAg, log10 IU/mL, mean (SD)	3.9 (0.58)	3.9 (0.56)	3.9 (0.50)
HDV RNA, log10 IU/mL, mean (SD)	5.6 (1.17)	5.6 (1.07)	5.9 (0.80)

Baseline Disease Characteristics Reflect Inclusion of Broad Patient Population, Including Patients with Advanced Disease

[1] Defined as liver stiffness ≥12.5 kPa.[2] Defined as probable by Baveno VII criteria.

AZURE-1 Phase 3 Week 24 Met Primary Endpoint

	300 mg QW* N=59	900 mg Q4W N=65	Delayed Treatment N=29
Primary Endpoint (Virologic Response + ALT normalization)	33/59 (**56%**) p <0.0001	29/65 (**45%**) p <0.0001	0/29 (**0%**)
Virologic response HDV RNA ≥2 $\log_{10}$ reduction or TND	51/59 (**86%**)	55/65 (**85%**)	0/29 (**0%**)
HDV RNA, LLOQ (<10 IU/ml)	15/59 (**25%**)	17/65 (**26%**)	0/29 (**0%**)
HDV RNA, TND	10/59 (**17%**)	11/65 (**17%**)	0/29 (**0%**)
ALT normalization	37/59 (**63%**)	35/65 (**54%**)	0/29 (**0%**)

**1 participant was HDV RNA TND at Week 20 but was missing Week 24 result due to being out of country. This participant was considered a non-responder at Week 24.*
Full analysis set - participants randomized to brelovitug treatment and received at least one dose after randomization or randomized to delayed treatment (Arm 3).
P-values compare each treatment group against delayed treatment using a stratum-adjusted Miettinen and Nurminen method.

AZURE-1 Phase 3 Safety Through Week 24

Participants who experienced, n (%)	300 mg QW N=59	900 mg Q4W N=65	Delayed Treatment N=29
AEs			
Any	27 (45.8)	40 (61.5)	8 (27.6)
Related to treatment	14 (23.7)	22 (33.8)	0
Grade 3+	0	0	1 (3.4)*
Serious	0	0	1 (3.4)*
AE leading to discontinuation of study drug, n (%)	0	0	0
Injection site reactions, n (%)	6 (10.2)	12 (18.5)	0
Flu-like symptoms, n (%)	3 (5.1)	7 (10.8)	0

** Grade 4 acute myocardial infarction.*

AZURE-1 Phase 2b: Responses Continue to Deepen at Week 48

No New Safety Signals Observed Through 48 Weeks of Treatment

	300 mg QW N=20[a]		900 mg Q4W N=20	
	24 wks	48wks	24 wks	48 wks
Primary Endpoint (Virologic Response + ALT normalization)	45% p = 0.003	55%	35% p = 0.024	55%
Virologic response HDV RNA ≥2 log_{10} reduction or TND	100%	95%[b]	75%[c]	95%[c]
HDV RNA LLOQ, <10 IU/ml	45%	80%	10%	40%
HDV RNA, TND	35%[d]	40%	5%	25%
ALT normalization	45%	55%	40%	55%

[a] *One participant discontinued after first dose and did not provide on-treatment results.*
[b] *One participant considered a virologic responder at Week 24 was lost to follow-up due to relocation and had missing Week 48 data. This participant was considered a non-responder at Week 48.*
[c] *One participant had Week 12 virologic response but was subsequently lost to follow-up. This participant was considered a non-responder at Week 24 and Week 48.*
[d] *One participant had a late Week 24 result (post-Week 24 analysis cutoff) that was TND.*
Full analysis set - participants receiving at least one post baseline efficacy assessment.
P-values compare each treatment group against delayed treatment using a stratum-adjusted Cochran-Mantel-Haenszel (CMH) test.

100% Virologic Response Among Brelovitug-Treated Participants with Available Week 48 HDV RNA

Ongoing Brelovitug Phase 3 Trials Supporting FDA and EMA Submissions

No ALT Limitation for Study Participation

FDA Registration Enabling Studies

Topline Data:

AZURE-1 Primary Endpoint met

AZURE-4 expected Q4 2026



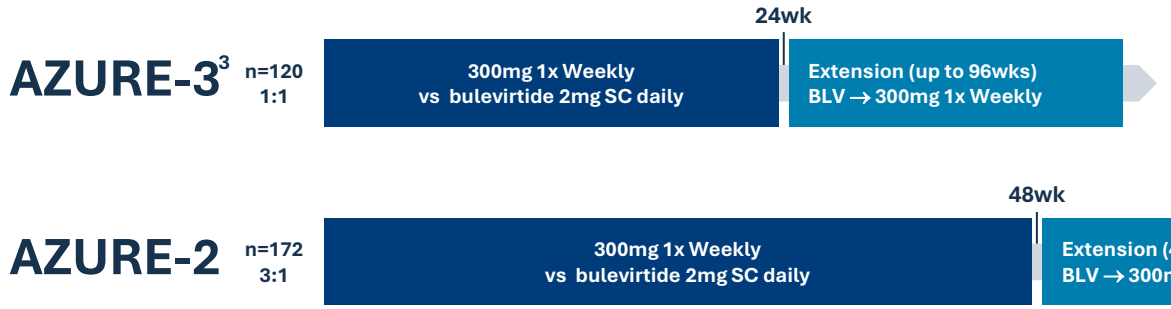
Primary Endpoint

Week 24 Virologic Response[2] + ALT normalization

EMA Registration Enabling Studies

Topline Data:

AZURE-2 and AZURE-3 expected in H2 2027



Primary Endpoint

Week 24 Virologic Response (Proportion TND)

Primary Endpoint

Week 48 TND + ALT normalization

[1] Patients in delayed Tx start arm switch to 300mg 1x Weekly at week 12. [2] Virologic Response = HDV RNA ≥2 log reduction or TND. [3] Enrolling patients on bulevirtide 2mg SC daily who are randomized 1:1 to either 300mg 1x weekly brelovitug or continued bulevirtide.

RARE GENETIC DISEASES

Expanding Presence in Rare Genetic Diseases

BILE ACID PORTFOLIO

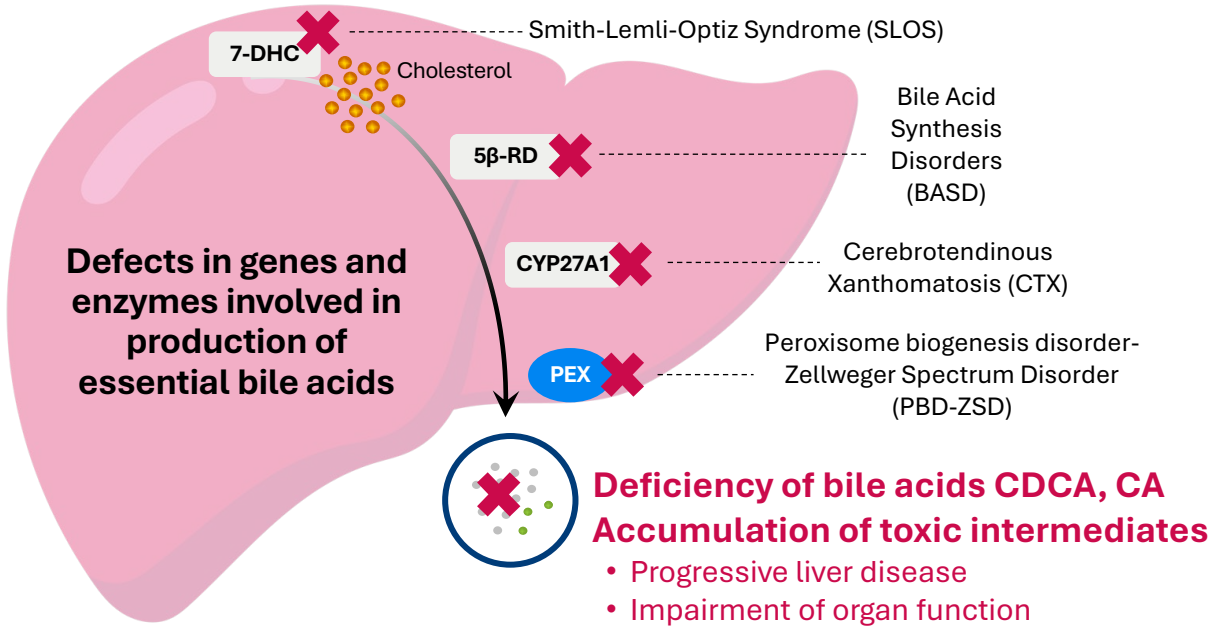
CHOLBAM® & CTEXLI®

Bile Acid Replacement Therapies for Rare Genetic Diseases

Bile Acid Replacement Therapies Provide Bile Acids the Liver Cannot Produce

Impaired Bile Acid Synthesis

Driven by single-enzyme defects and peroxisomal (PEX) disorders



Deficiency of bile acids CDCA, CA
Accumulation of toxic intermediates

- Progressive liver disease
- Impairment of organ function
- Irreparable neurological damage
- Significant morbidity

Bile Acid Replacement Therapies

- ✓ Restoration of bile acid homeostasis
- ✓ Reduction of toxic intermediates
- ✓ Improvement and prevention of adverse clinical manifestations

CDCA: chenodeoxycholic acid, CA: Cholic Acid.

Growing Business with Significant Commercial Synergies

Bile Acid Portfolio Addresses Multiple High Need Settings

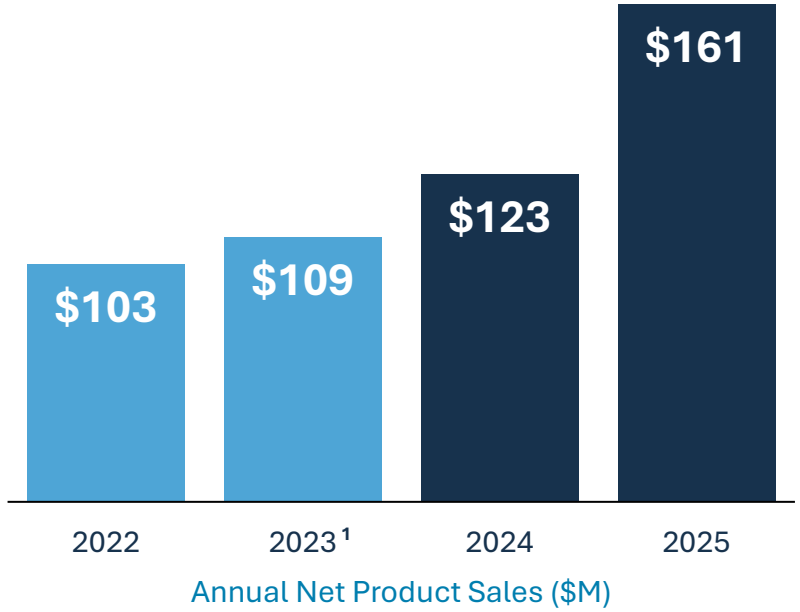
Ctexli® (chenodiol) tablets 250mg

CTX
Approved

Cholbam® (cholic acid) capsules

BASD
PBD-ZSD
Approved

Increased Awareness & Diagnosis Driving Growth



[1] Travere Therapeutics, Inc. and Mirum Pharmaceuticals, Inc. 10-K filings; 2023 net product sales excludes an approximate ~$5M reserve recorded by Travere Therapeutics, Inc. for potential repayment obligations attributed to 2015-2020 net product sales in France.
CTX - cerebrotendinous xanthomatosis; BASD - bile acid synthesis disorders include Peroxisome biogenesis disorder-Zellweger Spectrum Disorder (PBD-ZSD).

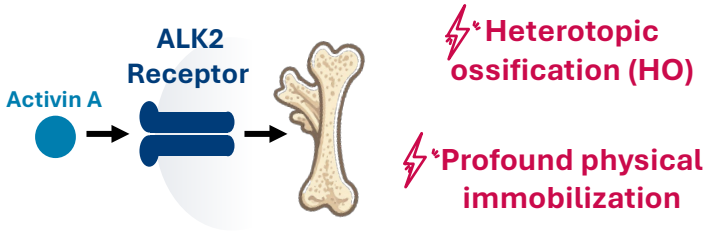
ATEBRIOZ™ (ZILURGISERTIB)

Fibrodysplasia Ossificans Progressiva (FOP)

ALK2 Inhibition in Fibrodysplasia Ossificans Progressiva (FOP)

Targeting ALK2 Receptor Reduces Volume of Heterotopic Ossification in FOP Patients

Fibrodysplasia Ossificans Progressiva



ACVR1 gene mutation results in aberrant overactivation of ALK2 receptor by activin A

Ultra-Rare Genetic Disorder

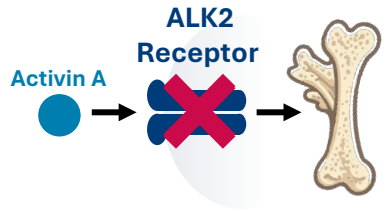
~300

U.S. diagnosed Patients

~900 WW diagnosed prevalence[1]

Care highly concentrated in Centers of Excellence

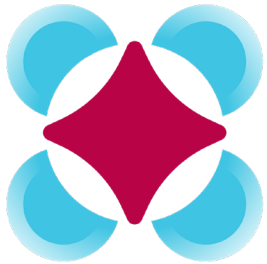
ALK2 Inhibition Benefits Observed[2]



- ✓ Reduction in volume of total HO
- ✓ Reduction in volume of new HOs
- ✓ Reduction in flare activity
- ✓ Improvement in mobility

[1] International FOP Association (IFOPA). What is FOP? 2026. Available from: https://www.ifopa.org/what_is_fop. [2] Pignolo, et al. Poster Presentation, ENDO 2026; Mirum Pharmaceuticals Press Release October 26th 2026

ATEBRIOZ™ (zilurgisertib) Now FDA Available



ATEBRIOZ®

zilurgisertib tablets 25mg

FDA-approved to reduce the volume of total new heterotopic ossification (HO) in adult and pediatric patients aged 12 years and older with fibrodysplasia ossificans progressiva (FOP)

Please see full Prescribing Information

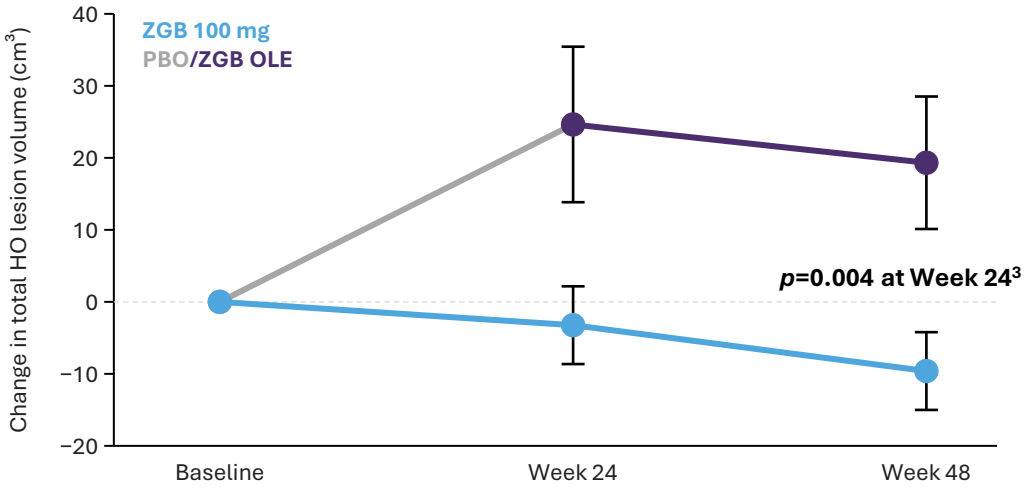
Positive Pivotal Phase 2 Results from PROGRESS Study

Cohort 1: FOP Patients ≥12 y.o.; n=63[1]

Reductions in Total HO Lesion Volume

81% reduction in proportion of patients with new lesions at Week 24

Reduction in total lesion volume



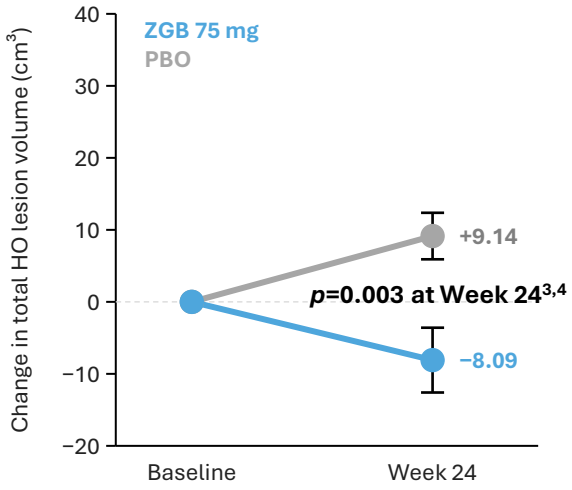
Patients, n			
ZGB 100 mg	32	32	32
PBO/ZGB OLE	31	30	29

Cohort 2: FOP Patients 6 to <12 y.o.; n=25[2]

Reductions in Total HO Lesion Volume

No new HO lesions, improved clinician-assessed joint mobility

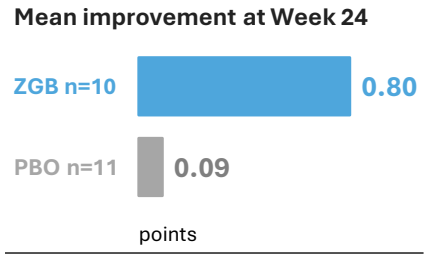
Reduction in total lesion volume



Patients, n		
ZGB 75 mg	12	12
PBO	13	13

Improved joint mobility

Clinician-assessed CAJIS[5] score (0 = no impairment; 30 = maximum impairment)



[1] Pignolo, et al. Poster Presentation, ENDO 2026. [2] Mirum Pharmaceuticals Press Release October 26th 2026. [3] Nominal *P* value. [4] Calculated from Wilcoxon rank sum test. [5] CAJIS: Cumulative Analogue Joint Involvement Scale (CAJIS)

Adverse Reactions Occurring in ≥ 10% of Adult and Pediatric FOP Patients

Patients Aged 12 Years and Older with FOP Receiving ATEBRIOZ in Cohort 1 Through Week 24

Adverse Reaction, n (%)	ATEBRIOZ 100 mg (n=32)	Placebo (n=31)
Headache	7 (22)	5 (16)
Arthralgia	7 (22)	3 (10)
Upper respiratory tract infection	7 (22)	3 (10)
Epistaxis	4 (13)	0
Nausea	4 (13)	1 (3)

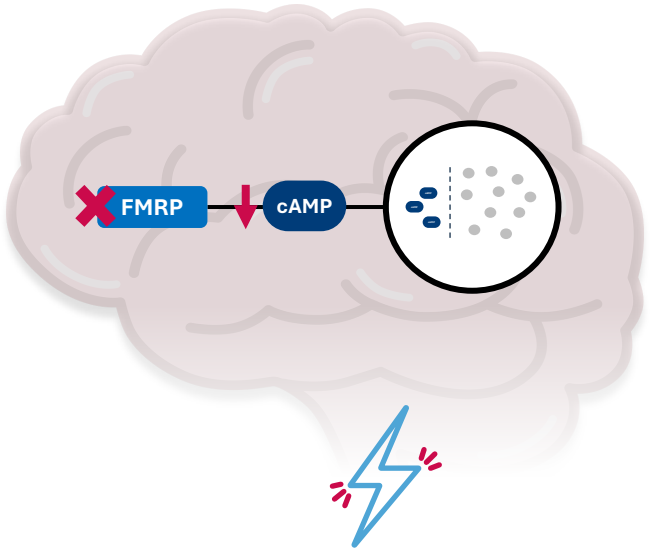
Atebrioz (zilurgisertib): Prescribing Information. Mirum Pharmaceuticals, Inc., 2026. U.S. Food and Drug Administration

MRM-3379

PDE4D Inhibitor for Fragile X Syndrome (FXS)

Fragile X Syndrome (FXS): Rare X-Linked Genetic Disorder

Mutation in the X-linked FMR1 gene Decreases cAMP



Impaired Cognition, Learning and Behavior

- Leading inherited form of intellectual disability and autism spectrum disorder
- Symptoms more pronounced in males
- Diagnosed by genetic testing

~50,000

Males in the US/EU with FXS
~2/3 with full mutation of FMR1 gene[1]

No Approved Therapies for FXS

[1] Hunter et al.– *American Journal of Medical Genetics* 2014.

MRM-3379: Selective PDE4D inhibitor for FXS

PDE4D

Regulates cellular signaling by breaking down cAMP



Highly expressed in brain regions critical for learning, memory, emotional regulation

Inhibition

PDE4D

↑ cAMP levels

FXS

In FXS patients, PDE4D inhibition improves cognition and daily function[1]

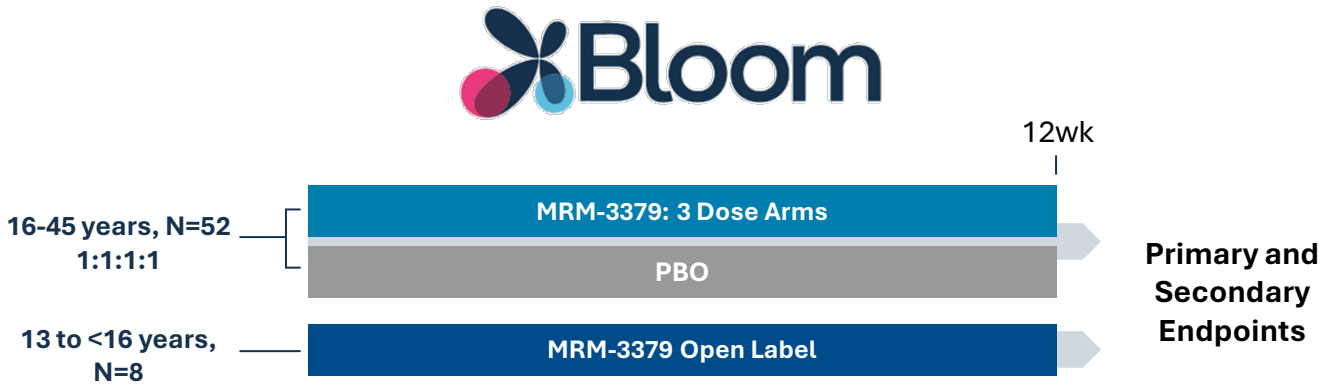
MRM-3379

- Oral, selective PDE4D inhibitor
- 5:1 brain/plasma ratio, potentially increasing therapeutic window
- Efficacy in preclinical models of memory
- Well tolerated in SAD and MAD clinical trials

Granted FDA Fast Track Designation

[1] Kravis et al., *Nature Medicine* 2021.

Phase 2 Study of MRM-3379 in Fragile X Syndrome



Key Inclusion Criteria

- Males, 13-45 years of age
- Diagnosis of FXS with full mutation (≥200 CGG repetitions)

Primary Endpoint

Safety & tolerability

Key Secondary Endpoint

NIH-TCB Crystallized Cognition Composite (CCC)

Composed from the Picture Vocabulary Test (PVT) and Oral Reading Recognition Test (ORRT)

Currently Enrolling; Topline Data Expected 2027

Main cohort randomized in a 1:1:1:1 ratio to one of four 12-week treatment arms of MRM-3379 or PBO
Participants 13 to <16 years of age with FXS will be enrolled in parallel to the main cohort to receive open-label MRM-3379

MIRUM PHARMACEUTICALS

Delivering High Impact Medicines for Rare Disease

Well-Positioned to Execute on Our Planned Strategy

★ 5 potential approval and label expansion opportunities expected

2026 FY Guidance

$715-720M

2026 Net Product Sales Guidance

Cash Flow Positive in 2027

$501M Cash Balance[1]

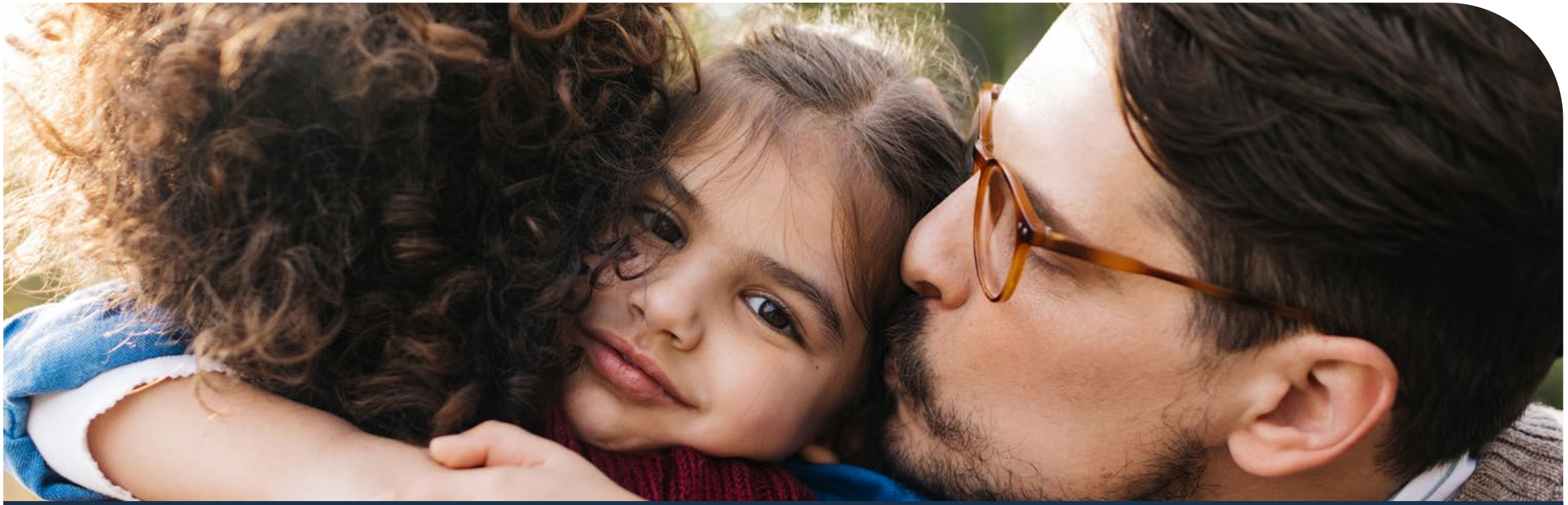
2026

- ✓ **★ VISTAS (PSC) positive topline results**
- ✓ AZURE-1 (HDV) positive interim analysis
- ✓ VANTAGE (PBC) complete enrollment
- ✓ **★ ATEBRIOZ FOP NDA approval**
- ✓ **★ AZURE-1 positive topline results**
- ✓ ATEBRIOZ FOP launch in Q4
- ✓ **★ EXPAND positive topline results**
- ❑ AZURE-4 topline results in Q4

2027

- ❑ **★ VANTAGE (PBC) topline results in Q1**
- ❑ Volixibat PSC NDA submission in H1
- ❑ Brelovitug HDV BLA submission in H1
- ❑ EXPAND sNDA submission in H1
- ❑ ATEBRIOZ sNDA submission in H1
- ❑ AZURE-2 & AZURE-3 topline results in H2
- ❑ Brelovitug HDV approval & launch in Q4
- ❑ BLOOM (FXS) study topline results

[1] Unrestricted cash, cash equivalents and investments as of September 30, 2026.



THANK YOU

RELENTLESSLY RARE

Patient inspired.
Ingenuity driven.

mirumpharma.com

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SUPPLEMENTAL MATERIALS

LIVMARLI Important Safety Information

IMPORTANT SAFETY INFORMATION

LIVMARLI can cause serious side effects, including:

Liver injury: Changes in certain liver tests are common in patients with Alagille syndrome and PFIC but can worsen during treatment. These changes may be a sign of liver injury. In PFIC, this can be serious or may lead to liver transplant or death. Your healthcare provider should do blood tests and physical exams before starting and during treatment to check your liver function.

Stomach and intestinal (gastrointestinal) problems: LIVMARLI can cause stomach and intestinal problems, including diarrhea and stomach pain during treatment.

Fat Soluble Vitamin Deficiency: A condition called Fat Soluble Vitamin (FSV) Deficiency caused by low levels of certain vitamins (vitamin A, D, E, and K) stored in body fat is common in patients with Alagille syndrome and PFIC but may worsen during treatment.

ATEBRIOZ Important Safety Information

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS - Embryo-Fetal Toxicity

- Can cause fetal harm. Verify females of reproductive potential are not pregnant prior to initiating ATEBRIOZ and advise of the potential risk to a fetus and to use effective contraception.
- Advise the patient to discontinue ATEBRIOZ immediately if pregnancy occurs during treatment and to contact their healthcare provider.

Most Common Adverse Reactions: headache, arthralgia, upper respiratory tract infection, epistaxis, and nausea.

Tell your health care provider about all the medications that you take, as ATEBRIOZ may interact with other medicines.

CHOLBAM Important Safety Information

LIMITATIONS OF USE

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.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS – Exacerbation of liver impairment

- Monitor liver function and discontinue CHOLBAM in patients who develop worsening of liver function while on treatment.
- Concurrent elevations of serum gamma glutamyltransferase (GGT) and alanine aminotransferase (ALT) may indicate CHOLBAM overdose.
- Discontinue treatment with CHOLBAM at any time if there are clinical or laboratory indicators of worsening liver function or cholestasis.

ADVERSE REACTIONS

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

CTEXLI Important Safety Information

IMPORTANT SAFETY INFORMATION

CTEXLI can cause side effects, including:

Liver Injury: You will need to undergo laboratory testing before starting and while taking CTEXLI to check your liver function. Changes in certain liver tests may occur during treatment and may be a sign of liver injury. This can be serious. Stop taking CTEXLI immediately and tell your healthcare provider right away if you get any signs or symptoms of liver problems, including, stomach (abdomen) pain, bruising, dark-colored urine, feeling tired (fatigue), bleeding, yellowing of the skin and eyes, nausea, and itching.

Most Common Side Effects: Diarrhea, headache, stomach pain, constipation, high blood pressure, muscular weakness, and upper respiratory tract infection.

Tell your health care provider about all the medications that you take, as CTEXLI may interact with other medicines.

Mirum Quarterly Net Product Sales

Net Product Sales ($M)	Q1 2025	Q2 2025	Q3 2025	Q4 2025	FY 2025	Q1 2026	Q2 2026	Q3 2026
LIVMARLI US	49.5	56.9	64.2	74.0	244.7	83.9	92.1	99.5
LIVMARLI International	23.7*	31.2*	28.1*	32.3	115.3	29.8	36.5*	33.9*
LIVMARLI Total	**73.2**	**88.2**	**92.2**	**106.4**	**360.0**	**113.7**	**128.7**	**133.4**
BAP Total	**38.4**	**39.6**	**40.8**	**42.5**	**161.3**	**46.1**	**47.5**	**52.3**
Total Net Product Sales	**111.6**	**127.8**	**133.0**	**148.9**	**521.3**	**159.9**	**176.2**	**185.7**

**Quarterly sales include recognition of sales to Takeda*

Mirum Quarterly Income (Loss) from Operations

$M	Q1 2025	Q2 2025	Q3 2025	Q4 2025	FY 2025	Q1 2026	Q2 2026	Q3 2026
Total revenue	**111.6**	**127.8**	**133.0**	**148.9**	**521.3**	**159.9**	**176.2**	**185.7**
Less:								
Cost of sales (excluding intangible amortization and other non-cash expenses)	17.1	17.5	19.6	22.5	76.7	22.1	23.7	28.5
General and administrative expenses (excluding SBC)	11.7	13.1	13.2	16.9	54.9	19.2	17.4	18.3
Commercialization and Medical Affairs expenses (excluding SBC)	35.8	39.6	36.6	44.0	156.0	44.2	48.2	52.1
R&D expenses (excluding SBC)	35.8	38.4	37.3	45.5	157.0	73.7	76.3	93.6
Acquired IPR&D	5.0	-	-	-	5.0	726.3	16.4	13.5
Stock-based compensation expense	15.5	18.2	17.8	18.8	70.3	57.1	30.1	32.8
Intangible amortization and other non-cash expenses	5.9	5.9	5.9	5.9	23.6	6.7	6.7	6.7
Income (loss) from operations (GAAP)	**(15.2)**	**(5.0)**	**2.6**	**(4.5)**	**(22.1)**	**(789.5)**	**(42.5)**	**(59.7)**

Source: Mirum SEC filings

Supplemental Material

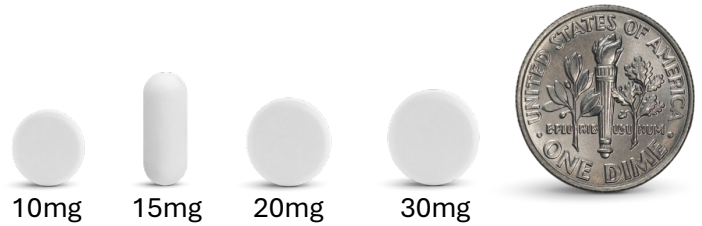
LIVMARLI Available in Both Oral Solution and Tablet Formulation

Flexible Dosing Options for Patients









Size for comparison

One LIVMARLI Tablet Per Dose

Supplemental Material

ALGS: A Debilitating Disease with Severe Cholestasis

Alagille Syndrome

Genetic disease leading to severe cholestasis, unbearable pruritus and multi-system effects

88%

Affected by cholestatic pruritus

6 in 10

Progress to transplant or death by adulthood

Livmarli® (maralixibat)

Lowers Serum Bile Acids	**83%**	of patients experienced ≥20% reduction in sBA levels
Significantly Reduces Pruritus	**84%**	of participants experienced ≥1 point reduction in ItchRO[Obs] in cholestatic pruritus
Pruritus Reduction Leads to Improved Transplant-Free Survival	**93%**	of patients with a >1-point reduction in ItchRO[Obs] remained transplant-free 6 years after starting LIVMARLI*

Kamath BM et al. *J Pediatr Gastroenterol Nutri.* 2018, Kamath BM et al, *Liver Transpl* 2012, Vandriel SM, et al. EASL 2020 (oral presentation), Gonzales E et al. *Lancet.* 2021;398:1581-1592.

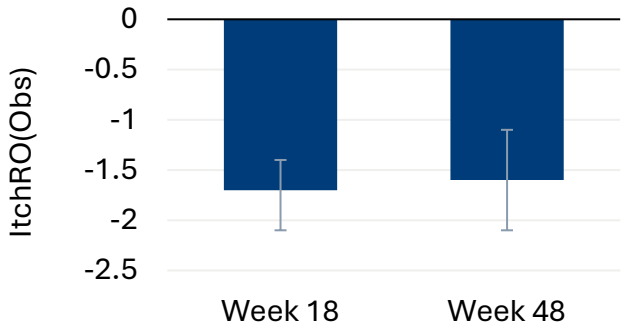
*Post Hoc Analysis, included data from 3 long-term studies (N=76); Transplant-free survival was defined as time to liver transplant or death; Sokol J, Gonzales E, Kamath BM, et al. ESPGHAN: Annual Meeting 2022.

Supplemental Material

ICONIC: ALGS Pivotal Study Shows Significant Long-term Benefit

Clinically Meaningful and Sustained Improvements in Pruritus, sBA, Growth, and QoL from Baseline.[1]

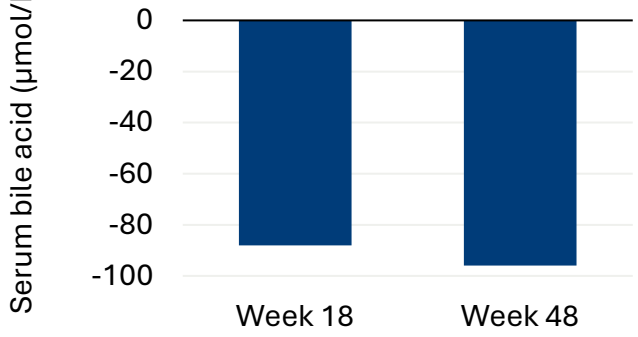
Reduced Pruritus



84%

of participants experienced clinically meaningful improvements (≥1 point reduction in ItchRO[Obs]) in cholestatic pruritus

Reduced sBA



83%

of patients experienced ≥20% reduction in sBA levels



LIVMARLI also improved other key symptoms of ALGS including growth, quality of life, and fatigue.

View data published in *The Lancet*

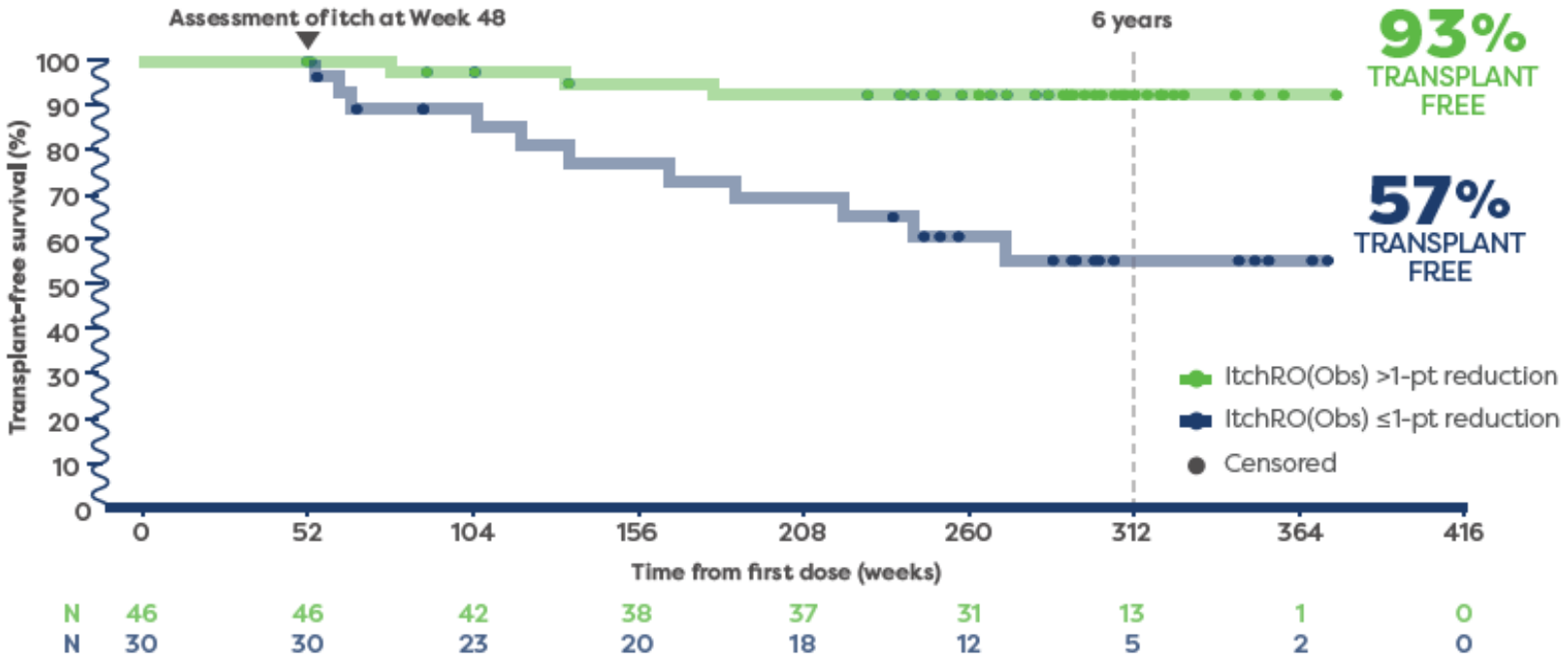
[1] Gonzales E et al. *Lancet.* 2021;398:1581-1592.

Supplemental Material

Significant Improvement in Transplant-Free Survival in Patients with ALGS Treated with LIVMARLI

Post-hoc Analysis of Long-Term Impact: >1-point Reduction in ItchRO[Obs] Was a Predictor of Transplant-Free Survival*

Transplant-Free Survival Over 6 Years of Treatment with LIVMARLI*



of patients remained transplant-free 6 years after starting LIVMARLI

of patients who had ≤1-point reduction in ItchRO(Obs) (n=30) remained transplant-free 6 years after starting LIVMARLI

*Transplant-free survival was defined as time to liver transplant or death; post-hoc analysis included data from 3 long-term studies (N=76)

Sokol J, Gonzales E, Kamath BM, et al. Predictors of 6-year event-free survival in patients with Alagille syndrome treated with maralixibat, an IBAT inhibitor. Paper presented at: European Society for Paediatric Gastroenterology, Hepatology and Nutrition (ESPGHAN): Annual Meeting; June 22-25, 2022; Copenhagen, Denmark.

Supplemental Material

PFIC: Progressive Diseases of Bile-Related Transporters

PFIC

(Progressive Familial Intrahepatic Cholestasis)

Multiple genetic subtypes



Severe pruritus
Stunted growth
Impaired QoL

~80%

Require liver transplant by 18yrs of age

Livmarli® (maralixibat)

Significant Improvements in Pruritus, Serum Bile Acids, and Bilirubin

Improvements Consistent Across Multiple Subtypes

(PFIC1, PFIC2, PFIC3, PFIC4, PFIC6 and unidentified mutational status)*

62%

With Minimal to No Itch

(Proportion of pruritus score assessments ≤1 after 26wks of treatment)**

Karpen et al, *JPGN* 2021; Englert et al, *Transplantation* 2007;84: 1361–1363; Thompson, et al. Oral Presentation, AASLD 2022

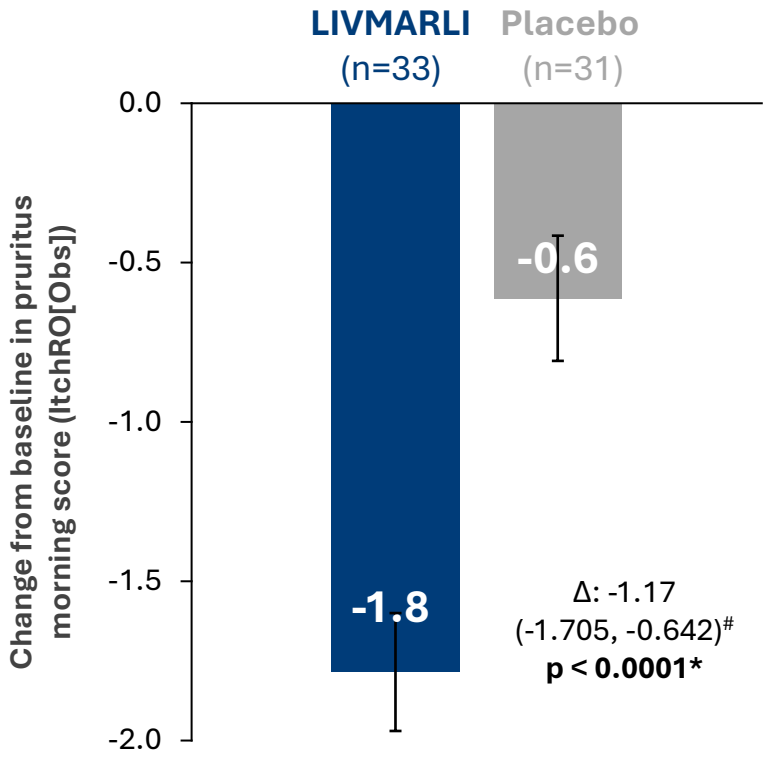
*LIVMARLI is not recommended in a subgroup of PFIC type 2 patients with specific ABCB11 variants resulting in non-functional or complete absence of bile salt export pump (e.g. BSEP-3 variant which accounts for approximately 21% of PFIC type 2 patients)

** Proportion of pruritus score assessments recorded as a 0 or 1 on the 0-4 ItchRO[Obs]

Supplemental Material

MARCH PFIC: Positive Phase 3 Data Across Broad Spectrum of PFIC Types

Significant Pruritus Improvements in All-PFIC Patients



Proportion of pruritus score assessments ≤ 1 point:

62% LIVMARLI vs 28% placebo (p<0.0001)



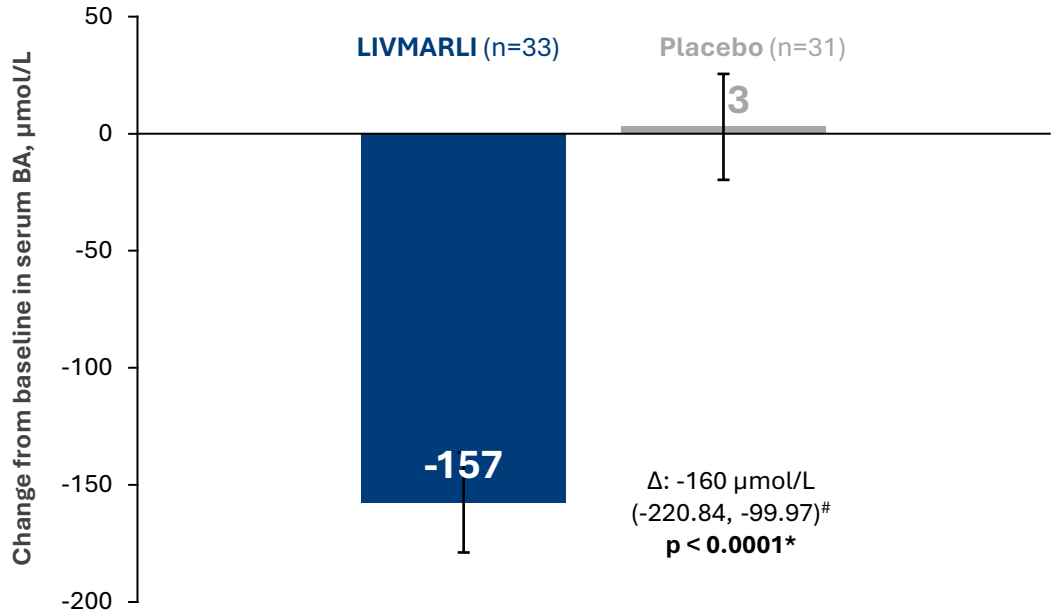
Data are LS Mean with standard error bars. Effect size compared the difference between LIVMARLI and placebo, averaged over the last 3 time periods using a repeated measures mixed effect model.
*LIVMARLI LS Mean = Placebo LS Mean; #LS Mean Delta with 95% CI.
Thompson, et al. Oral Presentation, AASLD 2022

Supplemental Material

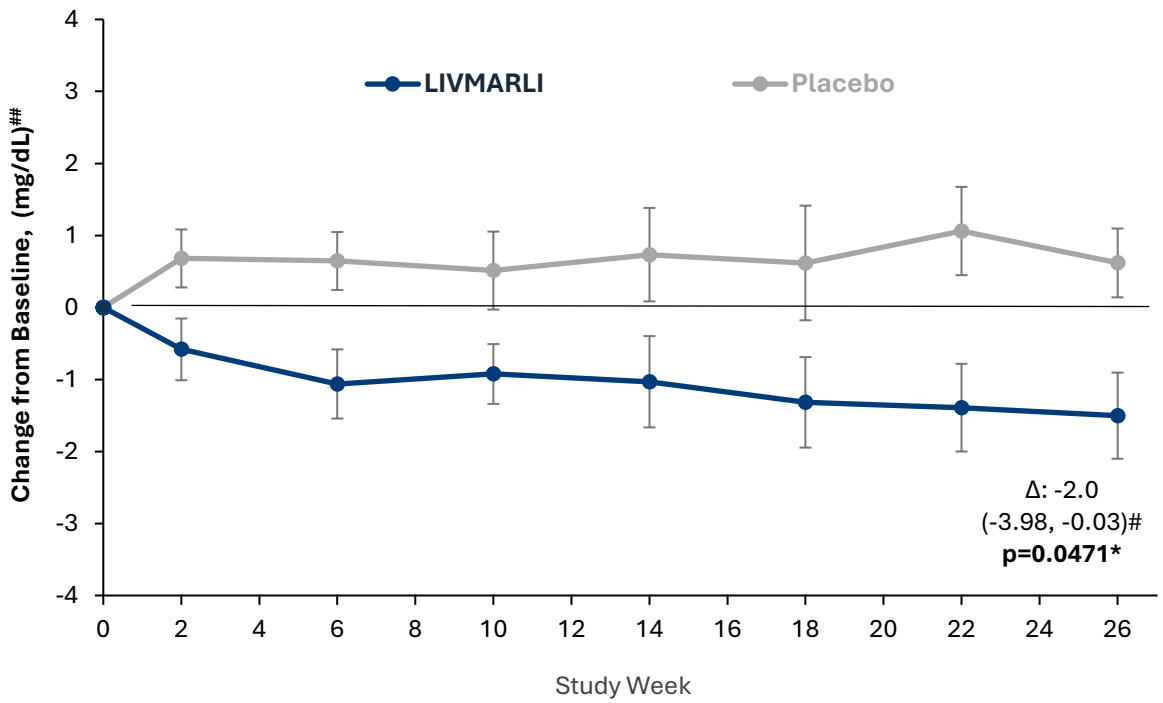
MARCH PFIC: Significant Improvements in Markers of Liver Disease

Significant Improvements in All-PFIC Patients (PFIC1, PFIC2, PFIC3, PFIC4, PFIC6)

Serum Bile Acid



Bilirubin[1]

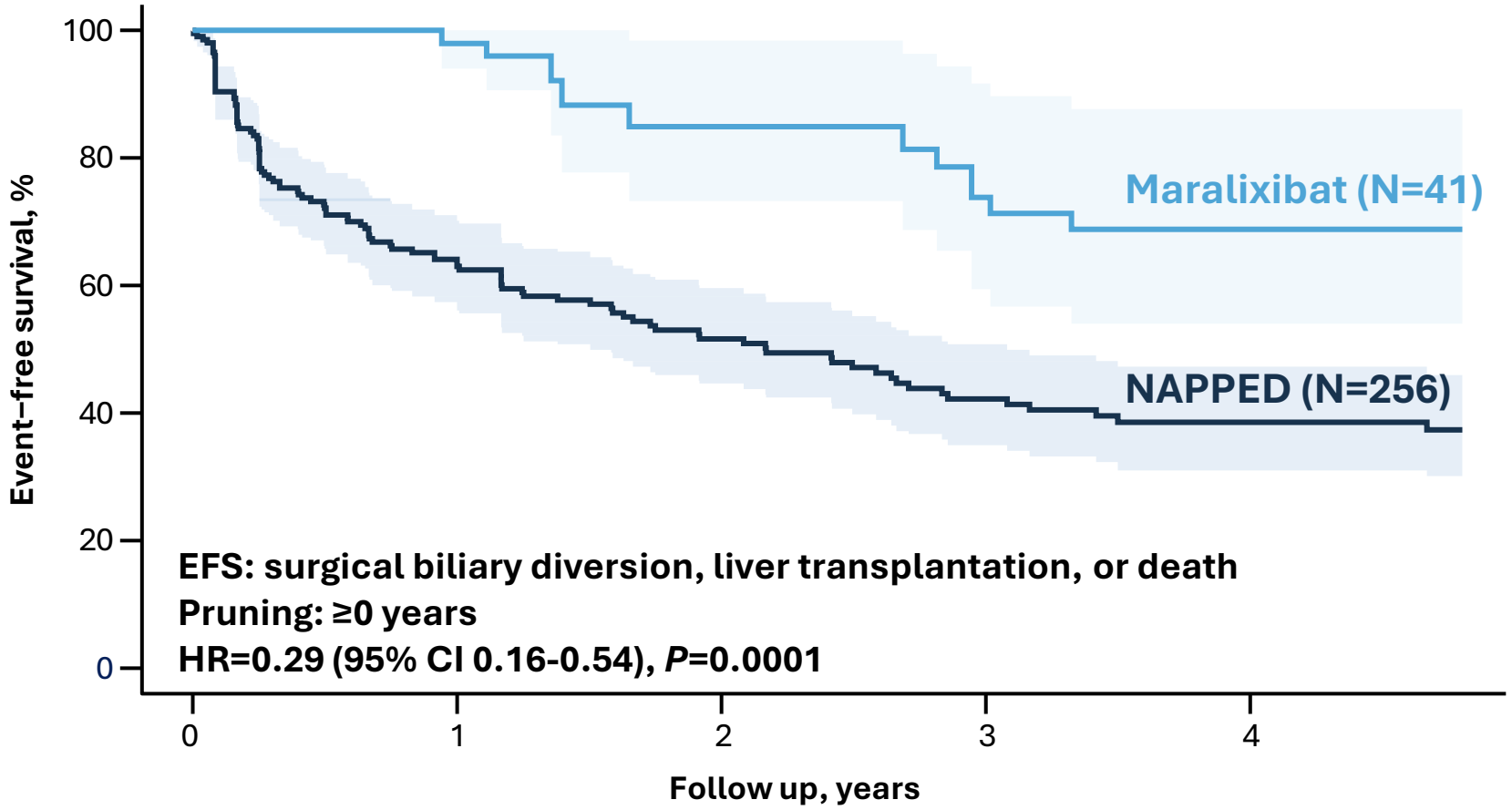


Data are LS Mean with standard error bars. Effect size compared the difference between LIVMARLI and placebo, averaged over the last 3 time periods using a repeated measures mixed effect model.
* LIVMARLI LS Mean = Placebo LS Mean; # LS Mean Delta with 95% CI; ## Data are mean with standard error bars
Thompson, et al. Oral Presentation, AASLD 2022.
[1] Bilirubin was not a prespecified primary or secondary endpoint that was in hierarchical order.

Supplemental Material

PFIC: Treatment with LIVMARLI® (maralixibat) Demonstrates Improved EFS

Event-Free Survival (EFS) of LIVMARLI-Treated PFIC Patients Compared with Real-World Data of Untreated Patients from NAPPED[1]



[1]NAPPED: NAtural Course and Prognosis of PFIC and Effect of Biliary Diversion Database
Hansen, et al. Oral Presentation, EASL 2026.



Supplemental Material

Well-Characterized Safety Profile of LIVMARLI



Safety Data of LIVMARLI includes 5 Years of follow-up from 3 randomized studies in ALGS, and 93-patient randomized MARCH study in PFIC



Most common adverse events were diarrhea and abdominal pain (ALGS: 41.6 and 38.6 events per 100 person-years, respectively; PFIC: 57.4% vs 19.6% pbo, 27.7% vs 15.2% pbo, respectively)



GI adverse reactions were generally mild or moderate severity and self-limiting

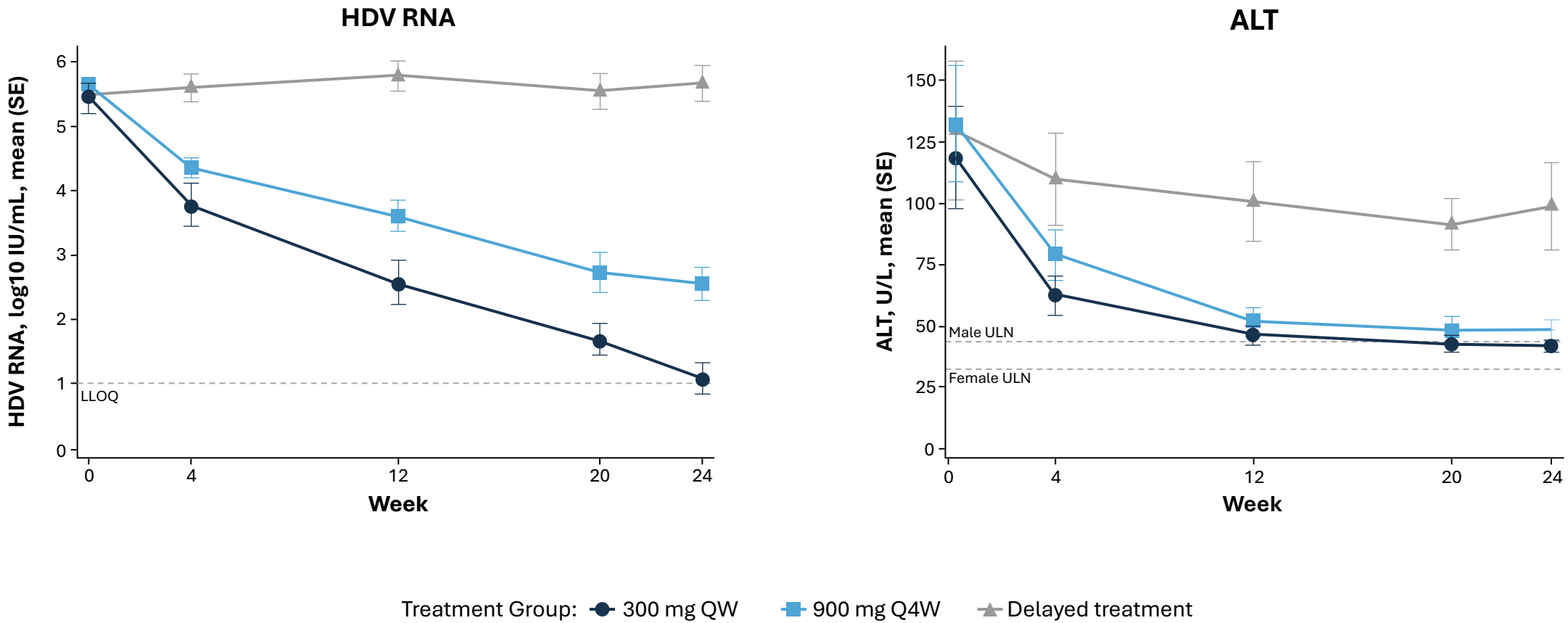


6% of patients experienced dose reductions or interruptions due to diarrhea, abdominal pain (ALGS, PFIC)

LIVMARLI can cause serious side effects, including liver injury. Changes in certain liver tests are common in patients but may worsen during treatment and should therefore be monitored prior to and during treatment. These changes may be a sign of liver injury and, in PFIC, can be serious or may lead to transplant or death

Supplemental Material

AZURE-1 Phase 2b: Rapid and Sustained Reductions in HDV RNA and ALT



Kushner, et al., EASL 2026

INTELLECTUAL PROPERTY OVERVIEW

LIVMARLI IP Coverage in the United States to 2040+

	Indication	
Livmarli® (maralixibat)	ALGS	**Method of Treatment: Dosing (2031, 2040)** Orange Book Listed – Patent No. 11,229,647 / 11,497,745 / 11,918,578 / 11,260,053 **Method of Treatment (2032, 2037)** Orange Book Listed – Patent No. 11,376,251 **Orphan Designation (2030)** **[Pending] Formulation, Manufacturing, Additional Dosing (2042, 2043)**
	PFIC	**Method of Treatment (2032)** Orange Book Listed – Patent No. 10,512,657 / 11,229,661 / 12,350,267 **Orphan Designation (2031)** **[Pending] Formulation, Manufacturing, Additional Dosing (2042)** **[Pending] Method of Treatment: Dosing (2040, 2043)**
	ALGS & PFIC	**Tablet Formulation – Method of Treatment: Dosing and Formulation (2043)**[1]

[1]Approved for grant

IP Coverage for Pipeline Indications in the United States

	Indication	
Volixibat	PBC PSC	**Method of Treatment: Dosing (2043)** Patent No. 12,576,079
		PBC Granted Orphan Designation, 7 years from approval
		PSC Granted Orphan Designation, 7 years from approval
		Method of Treatment: PBC (2032) Patent No. 12,576,079
		Composition of Matter (2027) Patent No. 7,956,085
		[Pending] Additional Dosing (2040, 2042) , Pharmaceutical compositions (2044)
		[Pending] Method of Treatment: PSC (2032)

IP Coverage for Pipeline Indications in the United States

	Indication	
Ctexli® (chenodiol) tablets 250mg	CTX	Orphan Designation (2032)
Brelovitug	HDV	Composition of Matter (2041) Patent No. 11,932,681 Granted Orphan Drug Designation, 7 years from approval
MRM-3379	FXS	Composition of Matter (2039)[1] Patent No. 9,120,770
ATEBRIOZ™ zilurgisertib tablets 25mg	FOP	Composition of Matter (2040)[1] Patent No. 10,710,980 Orphan Drug Designation (2033)

[1] Assumes standard patent term extension.

LIVMARLI IP Coverage in Europe to 2040+

	Indication	
Livmarli® (maralixibat)	ALGS	[Approved for Grant] Method of Treatment: Dosing (2040) Orphan Designation (2034) Method of Treatment (2032, Spain and France 2037) Patent No. 2,771,003 [Pending] Formulation, Manufacturing, Additional Dosing (2042)
	PFIC	Method of Treatment: Dosing (2040) Method of Treatment (2032) Patent No. 2,771,003 Orphan Designation (2036) [Pending] Formulation, Manufacturing, Additional Dosing (2042)

IP Coverage for Pipeline Indications in Europe

	Indication	
Volixibat	**PBC** **PSC**	**Composition of Matter (2027)** Patent No. 2,084,172
		PBC Granted Orphan Designation, 10 years from approval
		PSC Granted Orphan Designation, 10 years from approval
		[Pending] Method of Treatment: Dosing (2040)
		[Pending] Formulation, Manufacturing, Additional Dosing (2042)