# Mirum Pharmaceuticals:

## Delivering High Impact Medicines for Rare Disease

April 2026

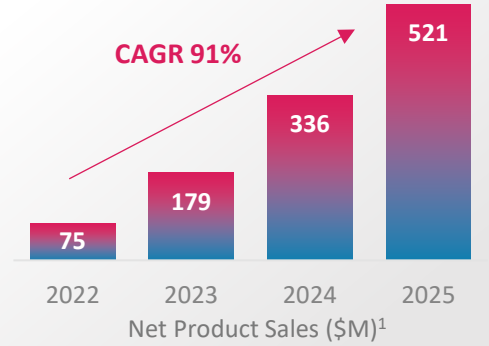
# Forward-Looking Statements

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 information concerning our acquisition of Bluejay Therapeutics ("Bluejay"), our business strategy, objectives and opportunities, including the future opportunities and clinical and regulatory milestones for LIVMARLI, CHOLBAM, CTEXLI or Chenodiol, our product candidates and the product candidates that we recently acquired in connection with the acquisition of Bluejay. 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 acquired in connection with the acquisition of Bluejay, and our plans and expectations for commercializing LIVMARLI, CHOLBAM and CTEXLI 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 commercial products; the potential clinical benefits of LIVMARLI, CHOLBAM and CTEXLI (or chenodiol tablets under other brand names) and any of our product candidates, including volixibat, MRM-3379 and brelovitug; our expected growth, including the integration of Bluejay and its operations; our ability to obtain necessary regulatory approvals for our product candidates 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; our ability to attract and retain key employees; our ability to manage the growth and complexity of our organization; our ability to maintain, protect 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 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. Neither we nor any other person assumes responsibility for the accuracy and completeness of the forward-looking statements. 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 and other data about our industry. 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 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.

*Strong Commercial Performance*




*$630-$650M 2026 Net Product Sales Guidance*

*4 FDA Breakthrough Designations[2]*

**4**

*Potentially Registrational Topline Readouts*

*Expected in Next 18 Months*

vistas AZURE VANTAGE EXPAND

**$4B+**

*Peak Revenue Potential of Mirum Portfolio[3]*

## Delivering Significant Value Through a Purpose-Built Rare Disease Model

[1] Annual net product sales 2022-2025
[2] Includes designations for Mirum's existing products and product candidates
[3] Mirum estimates of peak revenue potential

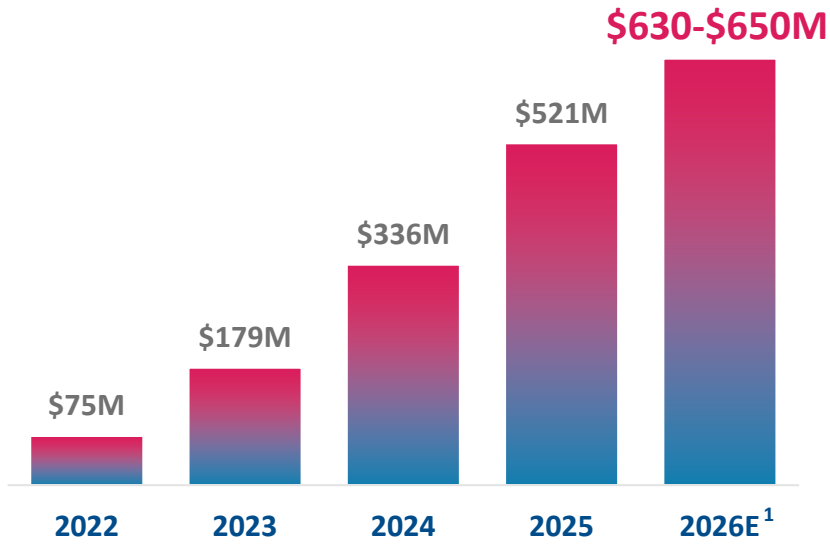
# Strong Execution Positions Mirum as a Global Rare Disease Leader

## Global Commercial Reach

Hepatology & GI

Rare Genetic Neurology & Metabolics


## Annual Net Product Sales




## Portfolio with Multi-Billion Dollar Revenue Potential[2]

| LIVMARLI | Volixibat | MRM-3379 | Brelovitug |
|---|---|---|---|
| ALGS, PFIC & Ultra-Rare Cholestasis | PSC & PBC | FXS | HDV |
| **$1Bn+** | **$1Bn+** | **$1Bn+** | **$750M+** |

[1] 2026 Net Product Sales Guidance
[2] Mirum estimates of peak revenue potential

# Commercial Portfolio with Pipeline of Growth Opportunities

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

| | Indication | Preclinical / Phase 1 / Phase 2 and Phase 3 | Approved |
|---|---|---|---|
| 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, topline data expected Q4 2026 | |
| Ctexli® (chenodiol) tablets 250mg | Cerebrotendinous Xanthomatosis (CTX)[4] | FDA approved | |
| Cholbam® (cholic acid) capsules | Bile Acid Synthesis Disorders (BASD)[5] | FDA approved | |
| volixibat | Primary Sclerosing Cholangitis (PSC) | VISTAS Phase 2b positive interim analysis, confirmatory topline data expected Q2 2026 | |
| | Primary Biliary Cholangitis (PBC) | VANTAGE Phase 2b positive interim analysis, expect enrollment completion H2 2026 | *Granted FDA Breakthrough Therapy Designation* |
| brelovitug | Chronic Hepatitis Delta Virus (HDV) | AZURE 1 & 4, Phase 3 topline data expected H2 2026 (US registrational program)<br>AZURE 2 & 3, Phase 3 topline data expected H1 2028 (EU registrational program) | *Granted FDA Breakthrough Therapy and EMA PRIME Designations* |
| 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)

# LIVMARLI®

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

# IBAT Inhibition In Cholestatic Liver Disease

## Targeting IBAT Removes Circulating Bile Acids Addressing Toxic Bile Acid Accumulation

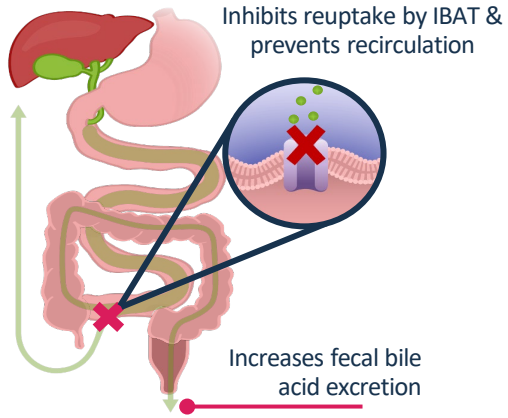
### Cholestatic Liver Disease

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)

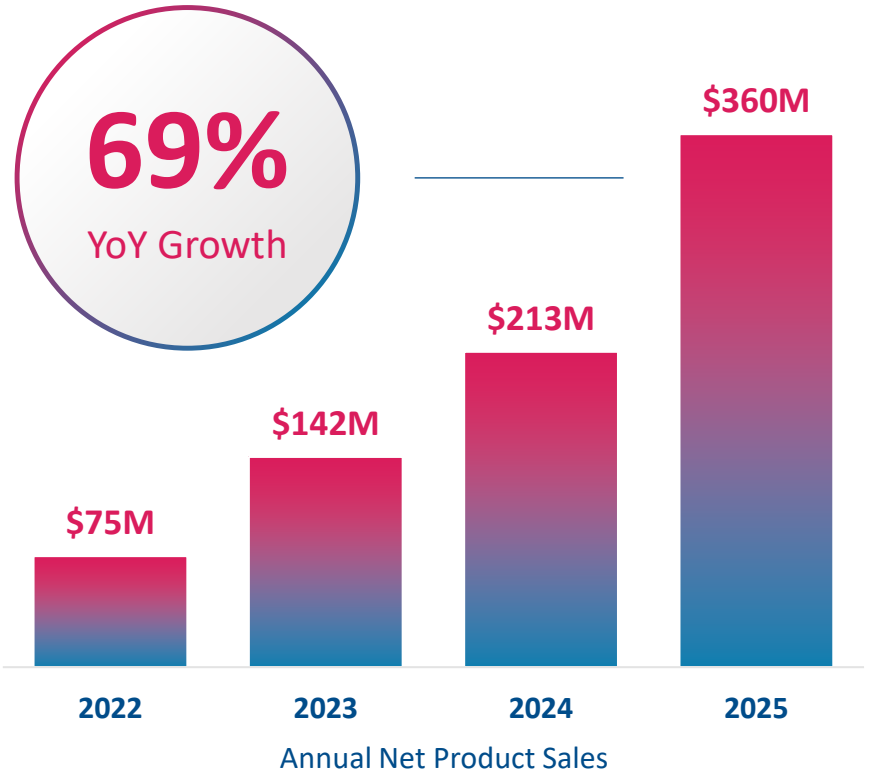
Xanthomas (ALGS)

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 Ultra-Rare Cholestatic Pruritus

Livmarli®
(maralixibat)

*Available in Both Oral Solution and Tablet Formulation*



# Expanding Impact in Ultra-Rare Cholestatic Pruritus

Livmarli®
(maralixibat)

**ALGS**

**Approved**
4,000-5,500 US/EU prevalence
~200/yr annual incidence

**PFIC**

**Approved**
1,000+ US/EU prevalence
70+/yr annual incidence

**EXPAND**

**Phase 3**
*Ultra-Rare Cholestasis*
1,000+ potential US/EU prevalence

**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+

# Bile Acid Portfolio

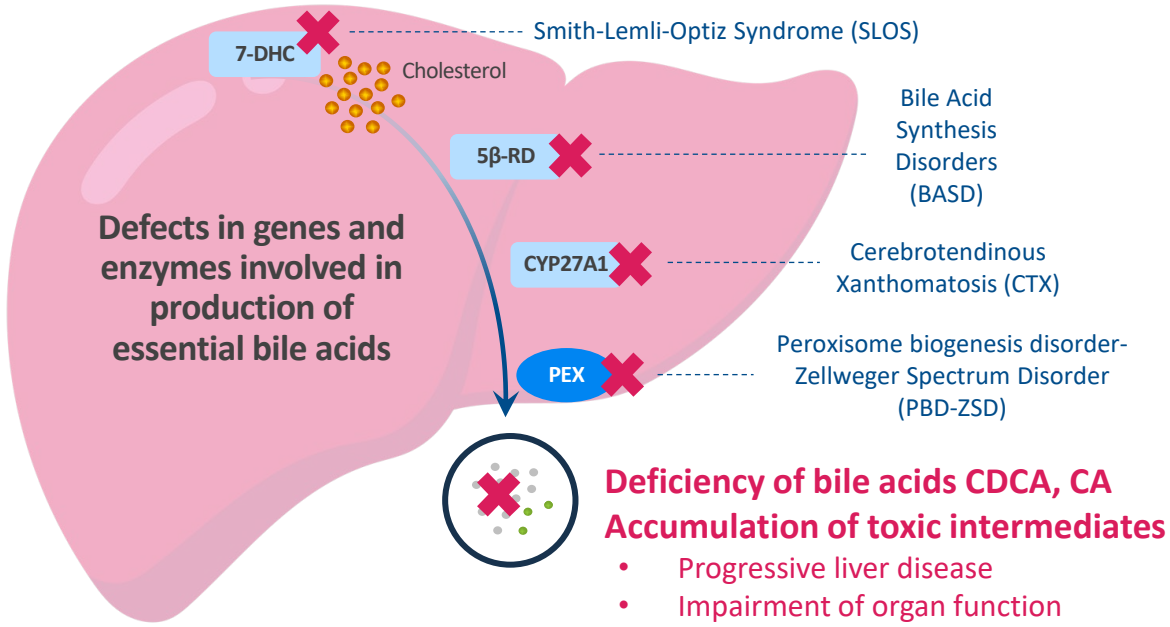
CHOLBAM® & CTEXLI®

# 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

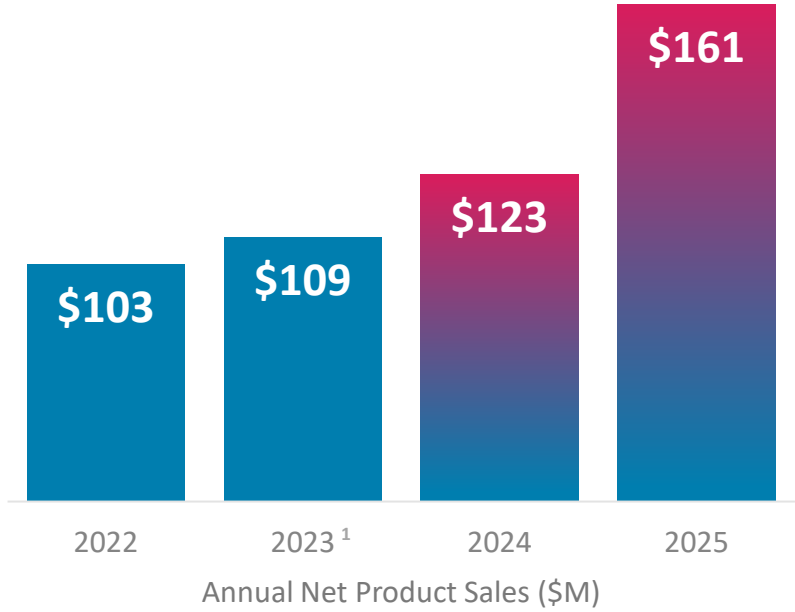
Ctexli® (chenodiol) tablets 250mg

**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)

# Pipeline

Significant Expansion Opportunities
within Pipeline Indications

# LIVMARLI (maralixibat)

# 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 Patients**

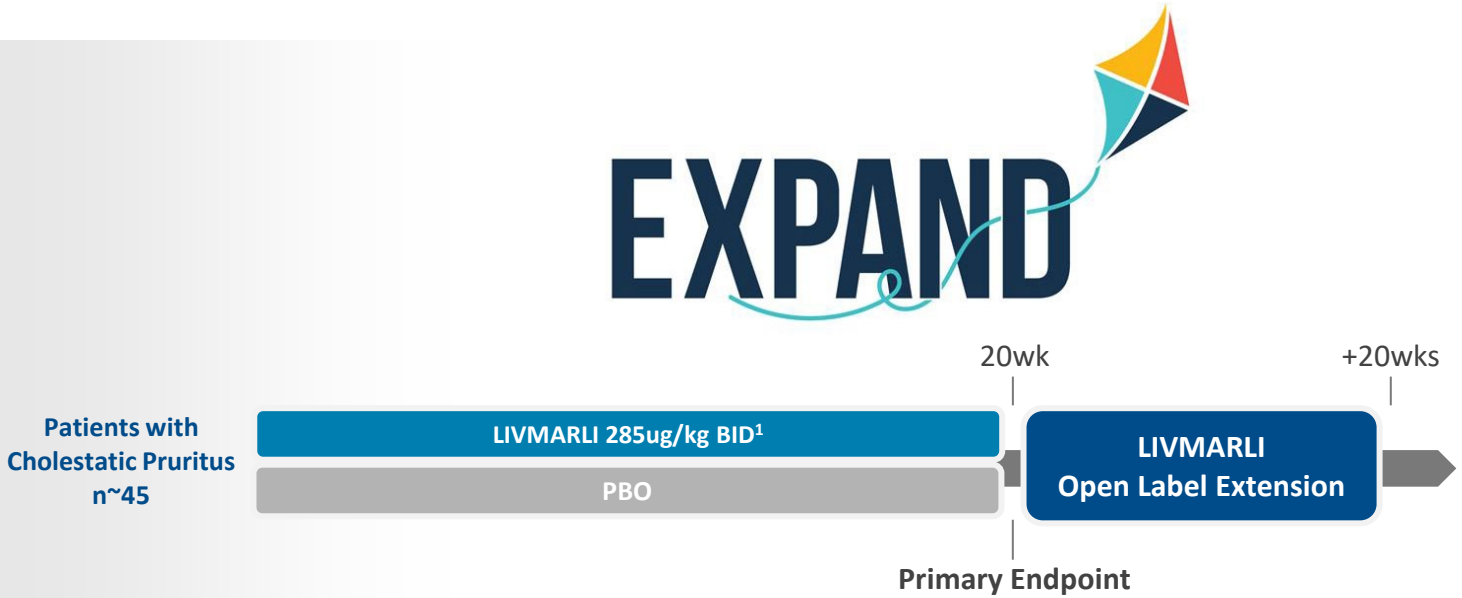
**1,000+**

**with cholestatic pruritus[1]**

**LIVMARLI Is Uniquely Positioned to Address the Burden of Cholestatic Pruritus**

[1] Mirum Market Research

# 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 sBA >2× ULN

## Primary Endpoint

Change in pruritus from Baseline to 20wk

## Secondary Endpoints

Safety & tolerability

Markers of disease and QoL

**Topline data expected Q4 2026**



[1] LIVMARLI 285ug/kg is equivalent to 300 ug/kg maralixibat chloride. BID, twice daily

# Volixibat

IBAT Inhibitor for Cholestasis in Adults

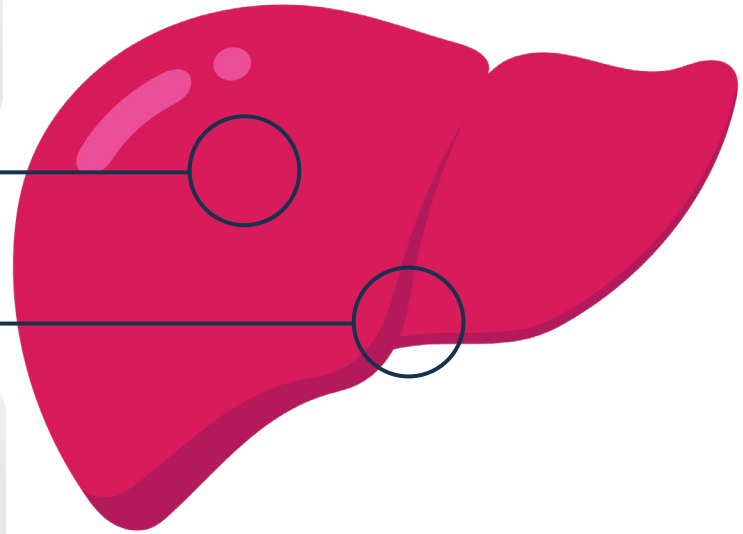
# PSC and PBC: Immuno-inflammatory Rare Liver Disease

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

## Bile Acid Overload

*Obstruction of bile flow via impairment of intrahepatic and extrahepatic bile ducts*

**PSC:** fibrotic strictures of bile ducts
*54,000 patients US/EU*
*65% of patients with active pruritus*



**PBC:** inflammatory driven cholestasis
*230,000 patients US/EU*
*60% of patients with active pruritus*

## Bile Acid Accumulation Associated with:

- Severe symptomatic burden
- Reduced bile acid synthesis
- Inflammation and fibrosis of bile ducts and liver
- Progressive liver damage

**IBAT inhibition Reduces Pruritus and sBA in PSC & PBC**

# Primary Sclerosing Cholangitis

# PSC: Pruritus is Common and Often Moderate to Severe

## Pruritus is a Registrational Endpoint

### Pruritus Is a Significant Burden

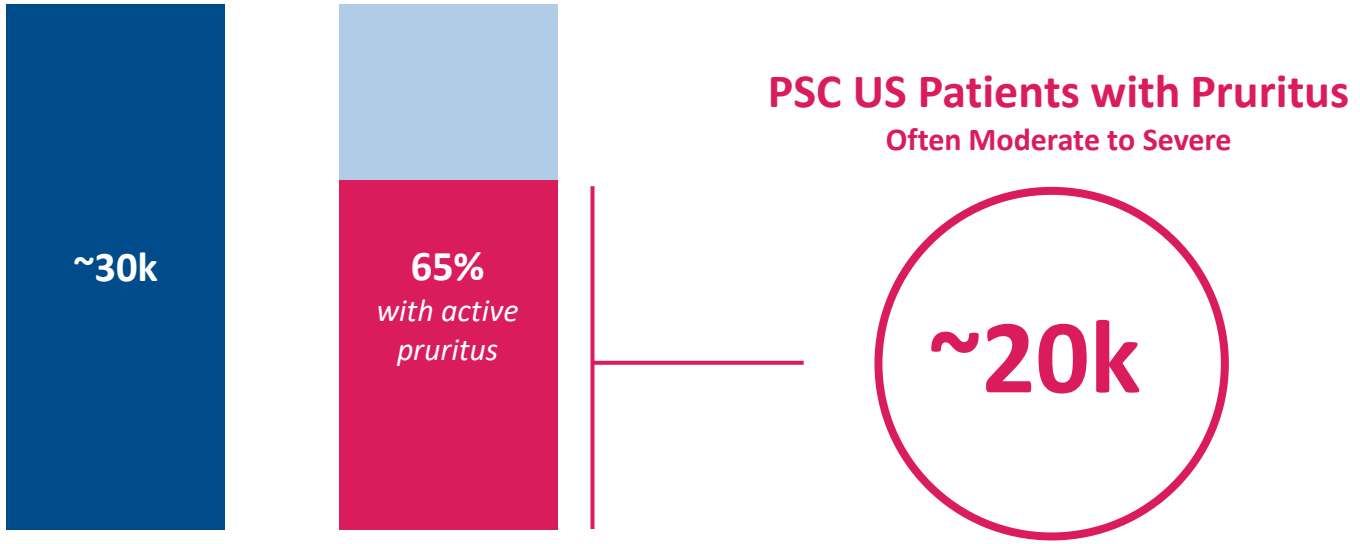
**8**

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

# IBAT Inhibition Reduces Pruritus and Serum Bile Acids in PSC

## CAMEO Study

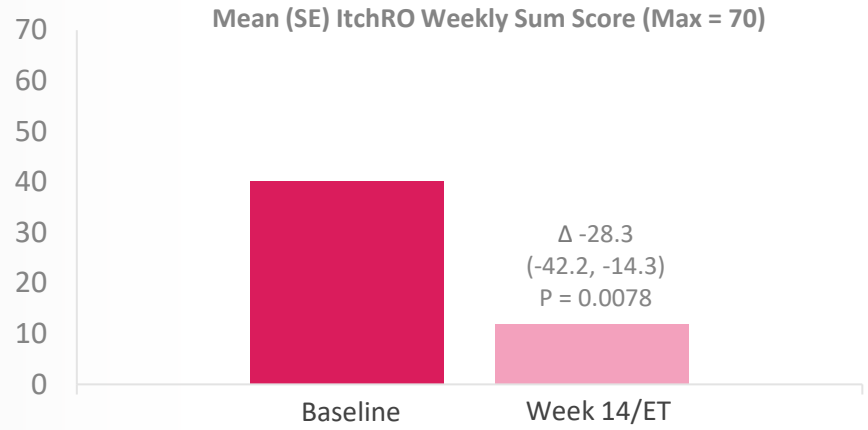
IBAT inhibitor Proof of Concept in PSC

Data from patients with ItchRO >3 at Baseline, n=8

**Significant Reductions in Pruritus and Bile Acids**

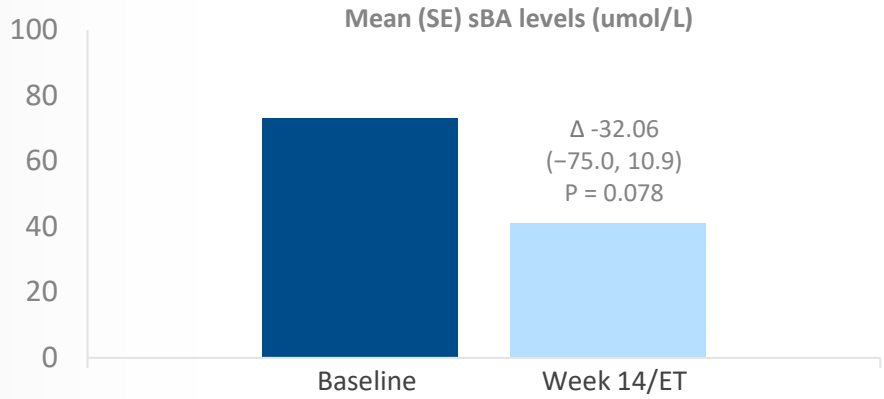
## Pruritus

**-70%**




## Bile Acids

**-40%**




**Volixibat: Highly Active on Bile Acid Pathway; 48-Week Safety Data in Prior Studies**

CAMEO Study: Open label maralixibat 10mg QD for 14wks, N=27
ItchRO: 0-10 Numerical Rating Scale (0=No Itch, 10=Worst Itch Imaginable)
All values are mean (95% CI)

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

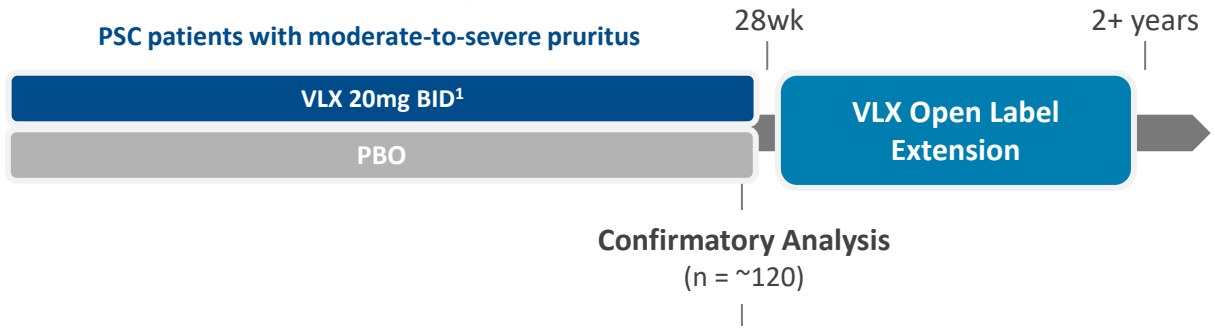
## Positive Interim Analysis

**Exceeded prespecified efficacy and safety thresholds for continuation**

**20 mg BID dose selected**

**VISTAS continues with no changes**

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




**Primary Endpoint**
Change in pruritus from baseline to 28wk

**Confirmatory Topline Data Expected Q2 2026**

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

# Primary Biliary Cholangitis

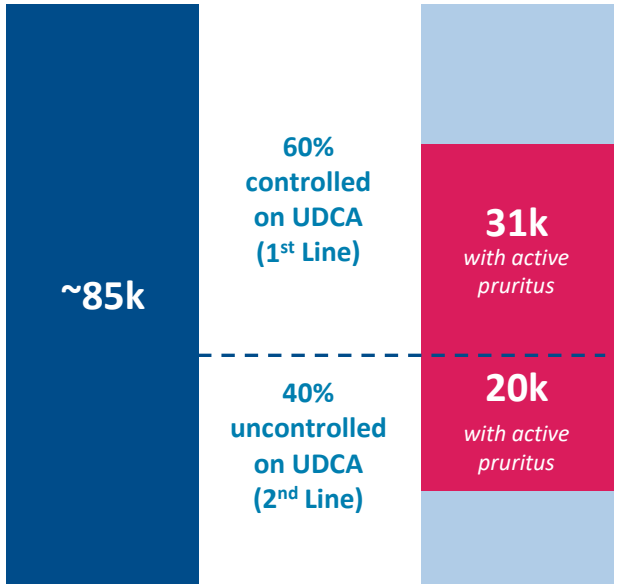
# PBC: Most Prevalent Cholestatic Liver Disease

## Significant Opportunity Across 1st & 2nd Line Settings

### No approved therapies for pruritus

> *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%)

**~50k**

**PBC Patients with Pruritus in the US**

**Often Moderate to Severe**

[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

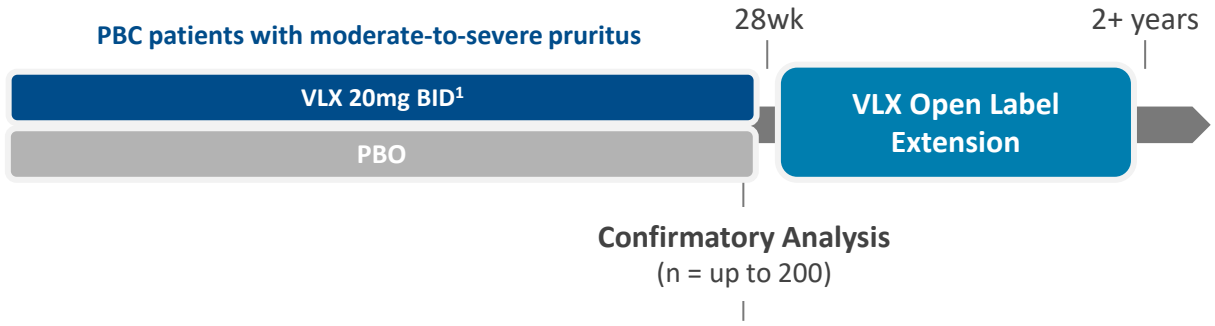
**Rapid and statistically significant improvement in pruritus**

**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

**Enrollment Completion Expected H2 2026**

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

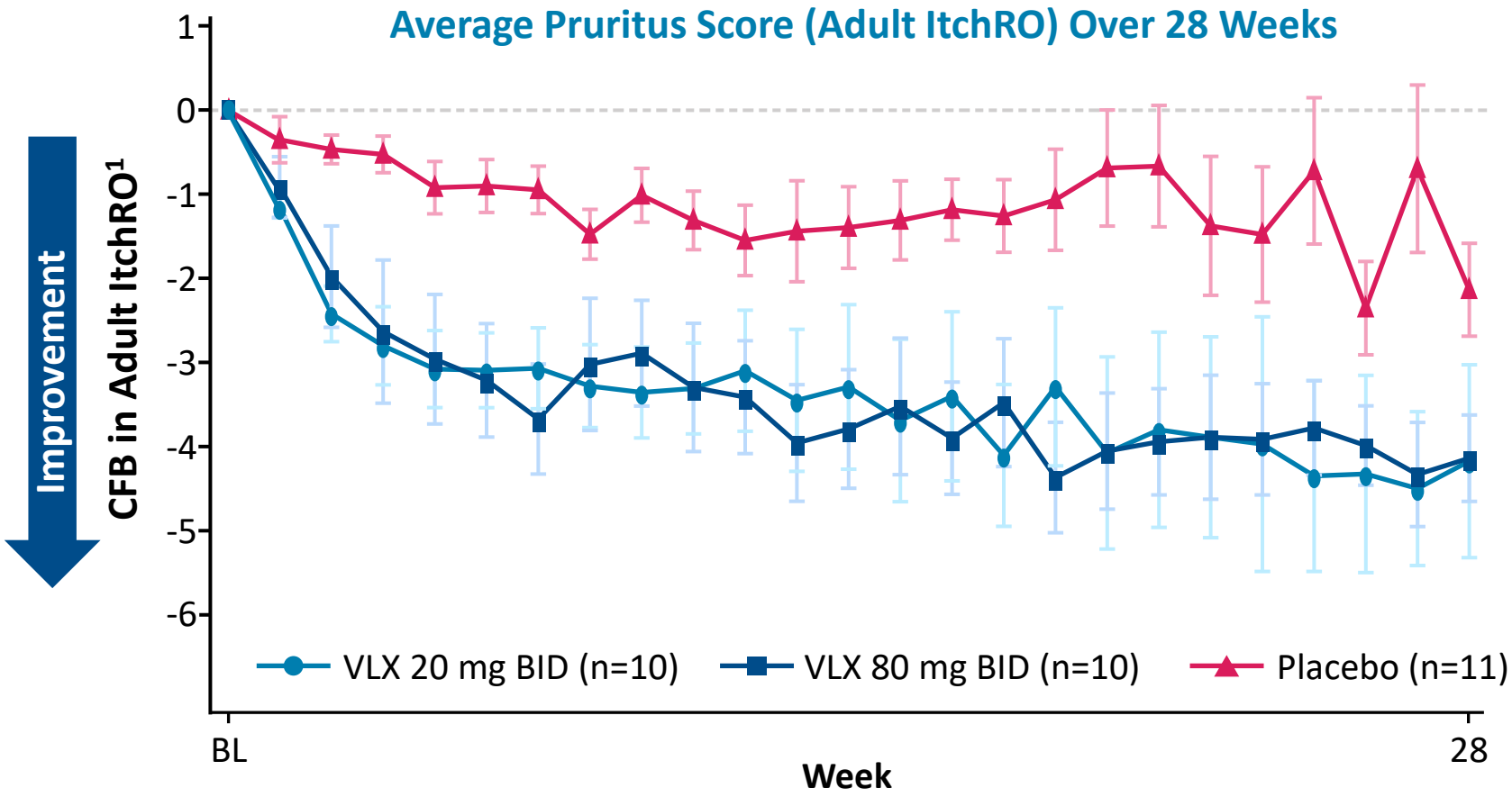
# VANTAGE Interim Analysis: Baseline Characteristics Well Balanced

| Characteristic | Volixibat BID 20mg (n=10) | Volixibat BID 80 mg (n=10) | Placebo (n=11) |
|---|---|---|---|
| Age (years), mean (SD) | 53.9 (15.8) | 52.3 (5.9) | 62.1 (9.7) |
| Female, n (%) | 8 (80) | 9 (90) | 10 (91) |
| Adult ItchRO Score, mean (SD) | 6.8 (1.6) | 6.3 (1.8) | 6.2 (1.5) |
| sBA in umol/L, mean (SD) | 53 (53) | 44 (73) | 31 (52) |
| ALP (U/L), mean (SD) | 238 (134) | 232 (107) | 167 (114) |

Kowdley et al, AASLD 2024
Adult ItchRO is a 0-10 worst-itch numerical rating scale completed once 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)

Heneghan et al, EASL 2025

[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.

# VANTAGE Interim Analysis: Other Observations

- Significant reduction in pruritus as early as Week 1
- Significant improvements in fatigue at Week 16
- 70% of patients on volixibat achieved >50% reduction in sBA
- No new safety signals:
  - No clinically meaningful changes in liver laboratory tests for patients on volixibat
  - 77% of patients on volixibat experienced diarrhea which was mild in severity and led to 1 discontinuation
  - 3 patients experienced serious TEAEs, including one in the placebo arm; none related to study drug

Kowdley et al, AASLD 2024

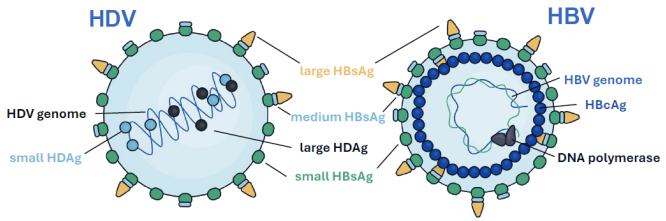
# Brelovitug

Chronic Hepatitis Delta Virus (HDV)

# HDV Is the Most Severe Form of Viral Hepatitis

**No Approved Therapies in the US**

## 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*

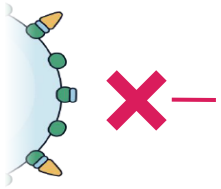
*A significant global unmet need*

[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=~150

***The Phase 2b portion of the AZURE-1 study met primary endpoint***
***Accepted for presentation at EASL 2026***

**Granted FDA Breakthrough & EU PRIME Designations**

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

# AZURE-1 Phase 2b Baseline Demographics

| | 300 mg QW N=21 | 900 mg Q4W N=20 | Delayed Treatment N=12 |
|---|---|---|---|
| **Age, years, mean (SD), range** | 45.8 (±7.0), 37 - 61 | 44.8 (±8.4), 26 - 58 | 49.0 (±9.9), 34 - 65 |
| **Men, n (%)** | 13 (62%) | 15 (75%) | 9 (75%) |
| **White, n (%)** | 19 (90%) | 19 (95%) | 11 (92%) |
| **Cirrhosis, n (%)[1]** | 10 (48%) | 11 (55%) | 7 (58%) |
| **Liver stiffness, kPa, mean (SD), range** | 16.4 (12.7), 4.9 - 57.3 | 16.8 (9.6), 6.0 - 40.9 | 16.6 (8.6), 6.7 - 39.2 |
| **ALT, U/L, mean (SD), range** | 116 (91), 25 - 357 | 132 (106), 47 - 492 | 124 (91), 35 - 273 |
| **HBsAg, log10 IU/mL, mean (SD), range** | 4.0 (0.4), 2.9 - 4.7 | 4.1 (0.5), 2.8 - 4.7 | 3.9 (0.8), 2.0 - 4.9 |
| **HDV RNA, log10 IU/mL, mean (SD), range** | 5.4 (1.0), 2.9 - 6.6 | 5.6 (0.6), 4.6 - 6.9 | 5.5 (0.9), 3.7 - 6.4 |

[1] Defined as liver stiffness ≥12.5 kPa

# AZURE-1 Phase 2b Week 24 Met Primary Endpoint

| | 300 mg QW N=20 | 900 mg Q4W N=20 | Delayed Treatment N=12 |
|---|---|---|---|
| **Virologic response**<br>HDV RNA ≥2 $log_{10}$ reduction or TND | 20/20 (**100%**) | 15/20 (**75%**) | 0/12 (**0%**) |
| **HDV RNA <LLOQ, TND** | 6/20 (**30%**) | 1/20 (**5%**) | 0/12 (**0%**) |
| **ALT normalization** | 9/20 (**45%**) | 8/20 (**40%**) | 1/12 (**8%**) |
| **Primary Endpoint**<br>(Virologic Response + ALT normalization) | 9/20 (**45%**)<br>p = 0.003 | 7/20 (**35%**)<br>p = 0.024 | 0/12 (**0%**) |

*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*

# AZURE Phase 2b Safety

| Participants who experienced, n (%) | 300 mg QW N=21 | 900 mg Q4W N=20 | Delayed Treatment N=12 |
|---|---|---|---|
| **AEs** | | | |
| Any | 11 (52) | 10 (50) | 3 (25) |
| Related to treatment | 7 (33) | 7 (35) | 0 |
| **Grade 3+** | | | |
| Any | 1 (5)[†] | 0 | 0 |
| Related to treatment | 0 | 0 | 0 |
| **Serious** | | | |
| Any | 0 | 1 (5)[#] | 0 |
| Related to treatment | 0 | 0 | 0 |
| **AE Leading to Discontinuation of study drug, n (%)** | 0 | 0 | 0 |
| **Injection site reactions, n (%)** | 3 (14) | 4 (20) | 0 |
| **Flu-like Symptoms, n (%)** | 0 | 1 (5) | 0 |

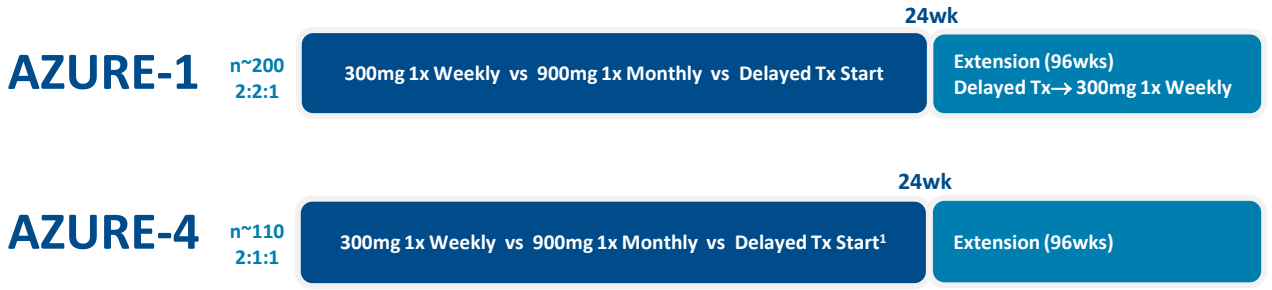
[†] Grade 3 AE of musculoskeletal pain, not related

[#] Hospitalization for liver cirrhosis, class B, in a patient with recent history of ascites and hypoalbuminemia, not related and resolved

# Ongoing Brelovitug Phase 3 Trials Supporting FDA and EMA Submissions

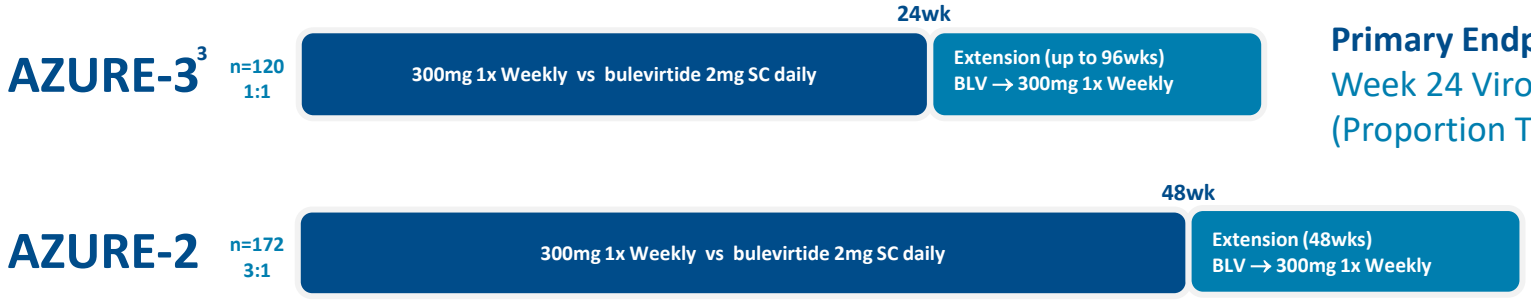
## No ALT Limitation for Study Participation

**FDA Registration Enabling Studies**
*Topline Data*
*Expected H2 2026*




**Primary Endpoint**
Week 24 Virologic Response[2]
+ ALT normalization

**EMA Registration Enabling Studies**
*Enrolling*




**Primary Endpoint**
Week 24 Virologic Response
(Proportion TND)

**Primary Endpoint**
Week 48 TND +
ALT normalization

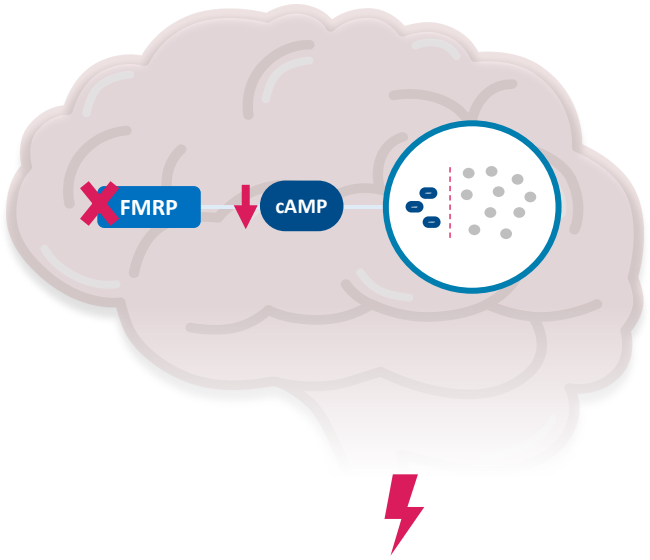
[1] Patients in delayed Tx start arm switch to 300mg 1x Weekly at week 12
[2] Virologic Response = HDV RNA ≥2 log reduction or TND
[3] Enrolling patients on bulevirtide 2mg SC daily who are randomized 1:1 to either 300mg 1x weekly brelovitug or continued bulevirtide

# 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]

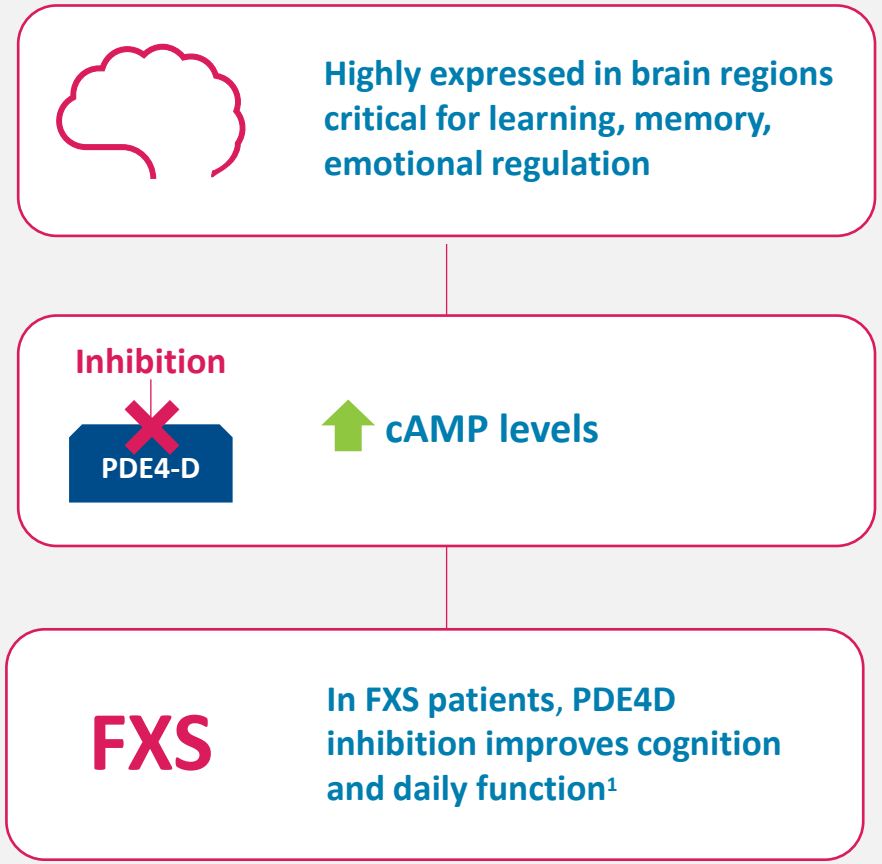
**No Approved Therapies for FXS**

[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*




## 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**

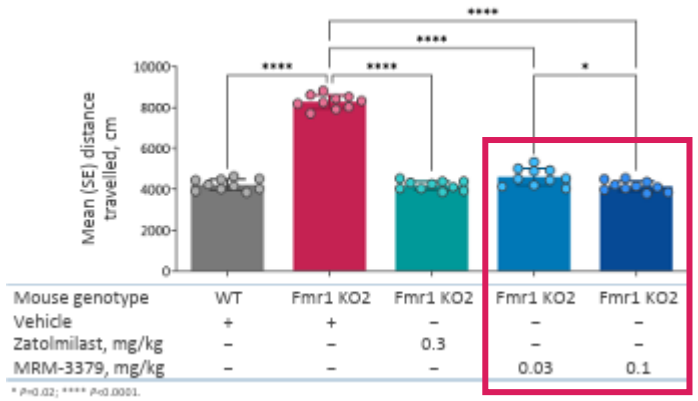
[1] Kravis et al., *Nature Medicine* 2021

# MRM-3379 Was Evaluated in a Mouse Model of FXS

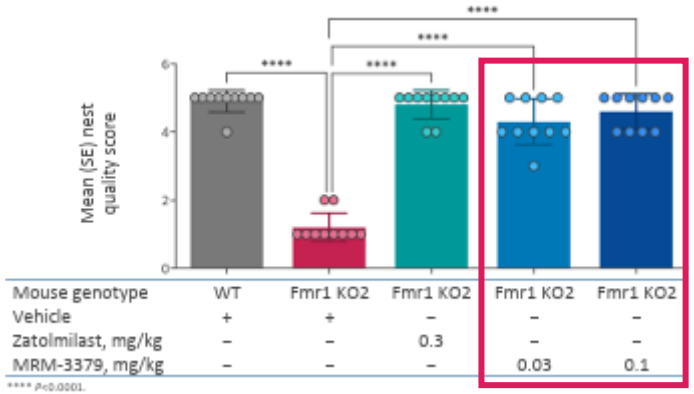
## MRM-3379 improved multiple behavioral domains relevant to the FXS phenotype in a mouse model

### FMR1 KO2 Mouse Model of FXS

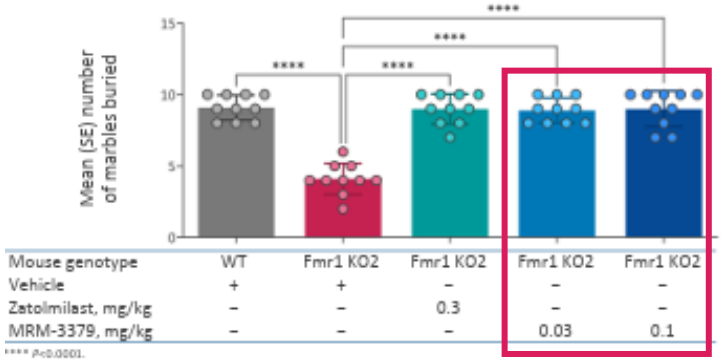
#### Hyperactivity Phenotype Was Reversed




#### Nesting Phenotype Was Reversed

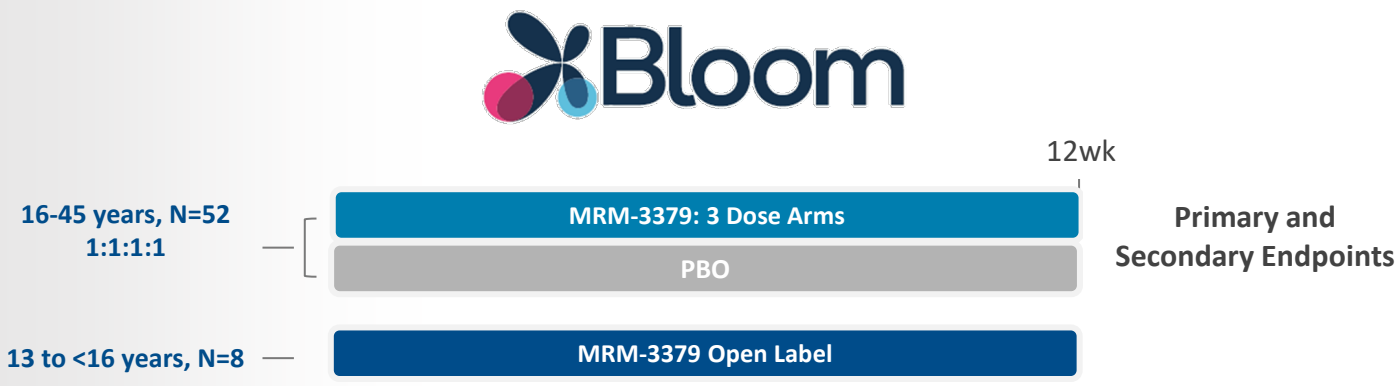



#### Marble Burying Phenotype Was Reversed




**Results support the potential for MRM-3379 as a treatment for FXS**

# 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

**★ 4 potentially registrational topline readouts expected in the next 18 months**

mirum

## 2026 FY Guidance

**$630-650M**

*2026 Net Product Sales Guidance*

***Cash Flow Positive in 2027***

**$391M Cash Balance[1]**

## 2026

- ❑ **★ VISTAS (PSC) topline results in Q2**
- ✓ AZURE-1 (HDV) Interim Analysis in Q2
- ❑ VANTAGE (PBC) complete enrollment in H2
- ❑ Volixibat PSC NDA submission in H2
- ❑ **★ AZURE-1 & 4 (HDV) topline results in H2**
- ❑ **★ EXPAND topline results in Q4**

## 2027

- ❑ **★ VANTAGE (PBC) topline results in H1**
- ❑ Volixibat PSC Approval/Launch in H1
- ❑ Brelovitug HDV BLA Submission H1
- ❑ Brelovitug HDV Approval & Launch H2
- ❑ BLOOM (FXS) study topline results

[1] Unrestricted cash, cash equivalents and investments; Mirum Pharmaceuticals Inc. FY 2025 10-K

# Thank You

# 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.

# 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

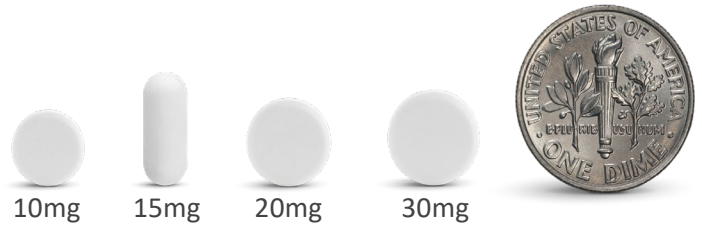
| *Net Product Sales ($M)* | Q1 2024 | Q2 2024 | Q3 2024 | Q4 2024 | FY 2024 | Q1 2025 | Q2 2025 | Q3 2025 | Q4 2025 | FY 2025 |
|---|---|---|---|---|---|---|---|---|---|---|
| LIVMARLI US | 30.8 | 35.5 | 43.5 | 44.7 | 154.5 | 49.5 | 56.9 | 64.2 | 74.0 | 244.7 |
| LIVMARLI International | 12.1 | 11.7 | 15.6 | 19.4 | 58.8 | 23.7* | 31.2* | 28.1* | 32.3 | 115.3 |
| **LIVMARLI Total** | **42.8** | **47.2** | **59.1** | **64.1** | **213.3** | **73.2** | **88.2** | **92.2** | **106.4** | **360.0** |
| **BAP Total** | **26.1** | **30.5** | **31.2** | **35.3** | **123.1** | **38.4** | **39.6** | **40.8** | **42.5** | **161.3** |
| **Total Net Product Sales** | **68.9** | **77.8** | **90.3** | **99.4** | **336.4** | **111.6** | **127.8** | **133.0** | **148.9** | **521.3** |

* *Quarterly sales include recognition of sales to Takeda*

Supplemental Material

# LIVMARLI Available in Both Oral Solution and Tablet Formulation

## Flexible Dosing Options for Patients


*Size for comparison*

**One LIVMARLI Tablet Per Dose**

# 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* |

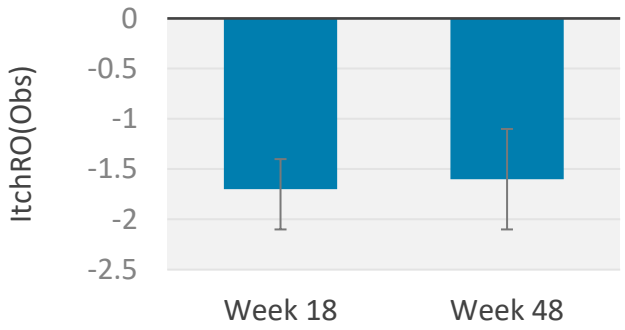
Kamath BM et al. *J Pediatr Gastroenterol Nutri*. 2018, Kamath BM et al, Liver Transpl 2012, Vandriel SM, et al. EASL 2020 (oral presentation), Gonzales E et al. Lancet. 2021;398:1581-1592.
*Post Hoc Analysis, included data from 3 long-term studies (N=76); Transplant-free survival was defined as time 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]

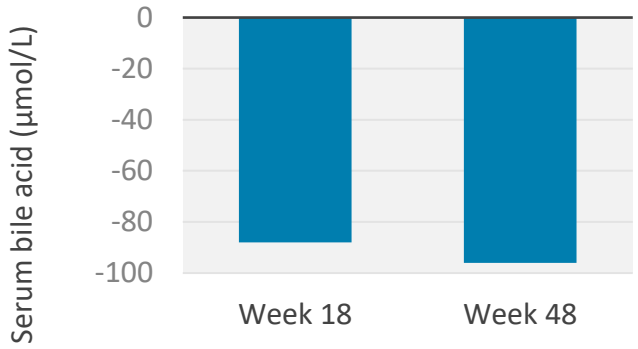
### Reduced Pruritus




**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

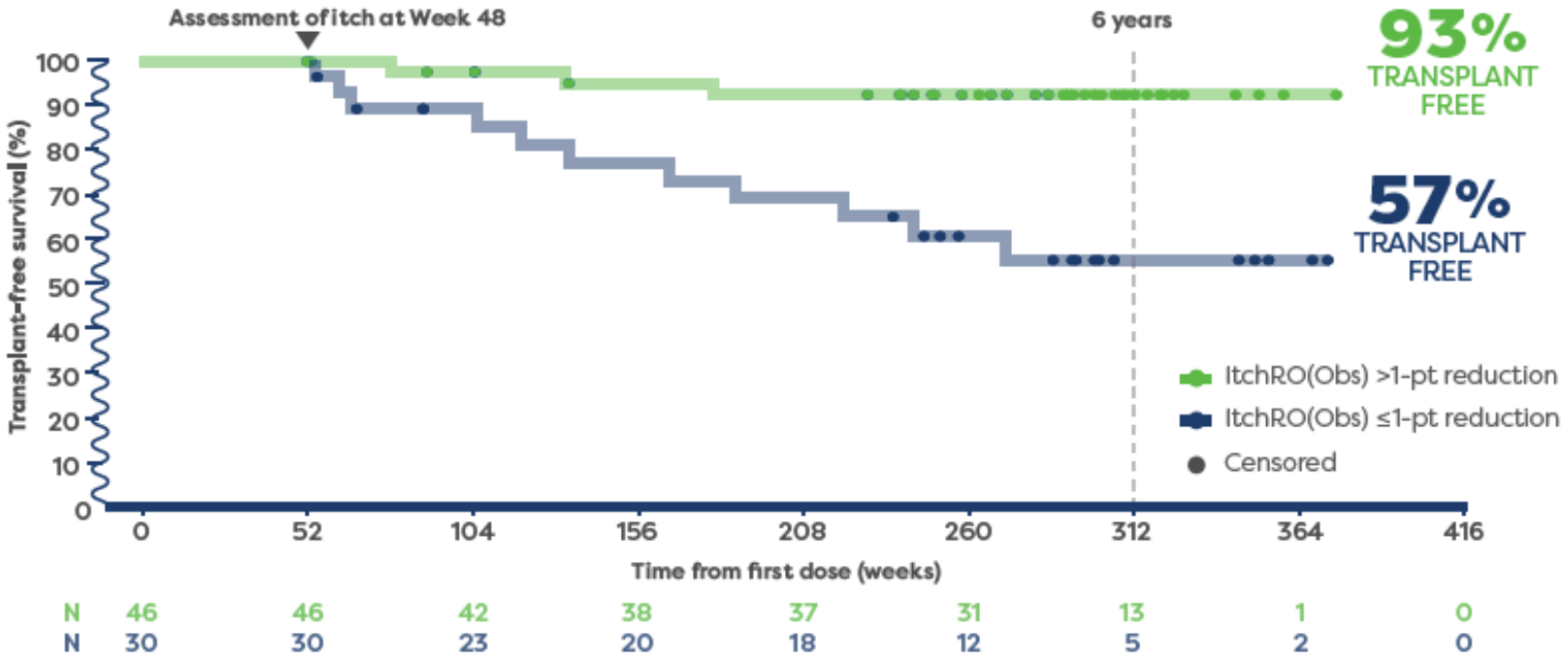

[1] Gonzales E et al. *Lancet*. 2021;398:1581-1592.

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.

# 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)**

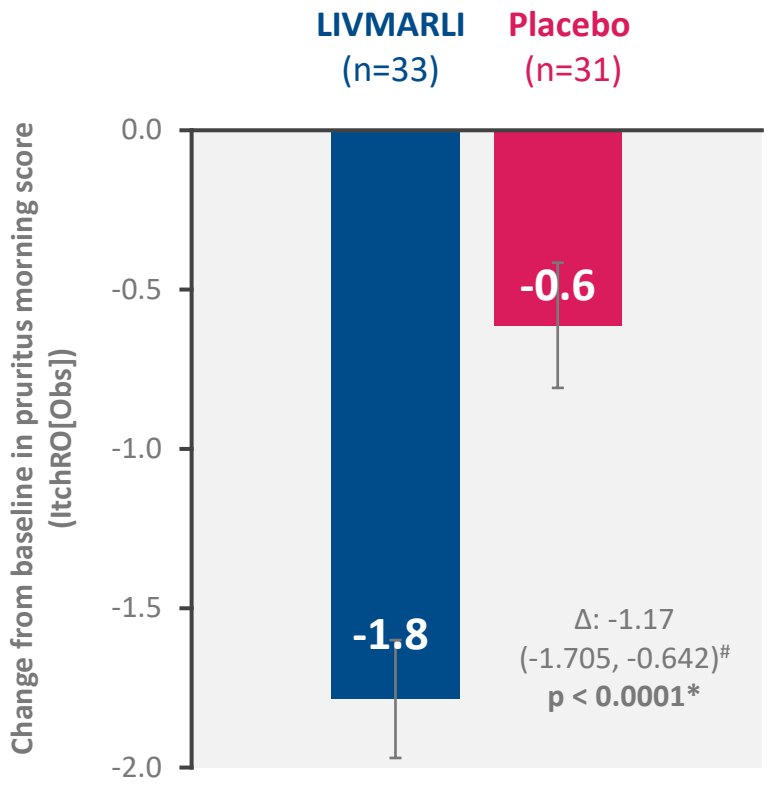
Karpen et al, JPGN 2021; Englert et al, Transplantation 2007;84: 1361–1363; Thompson, et al. Oral Presentation, AASLD 2022

*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]

# 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
($p<0.0001$)

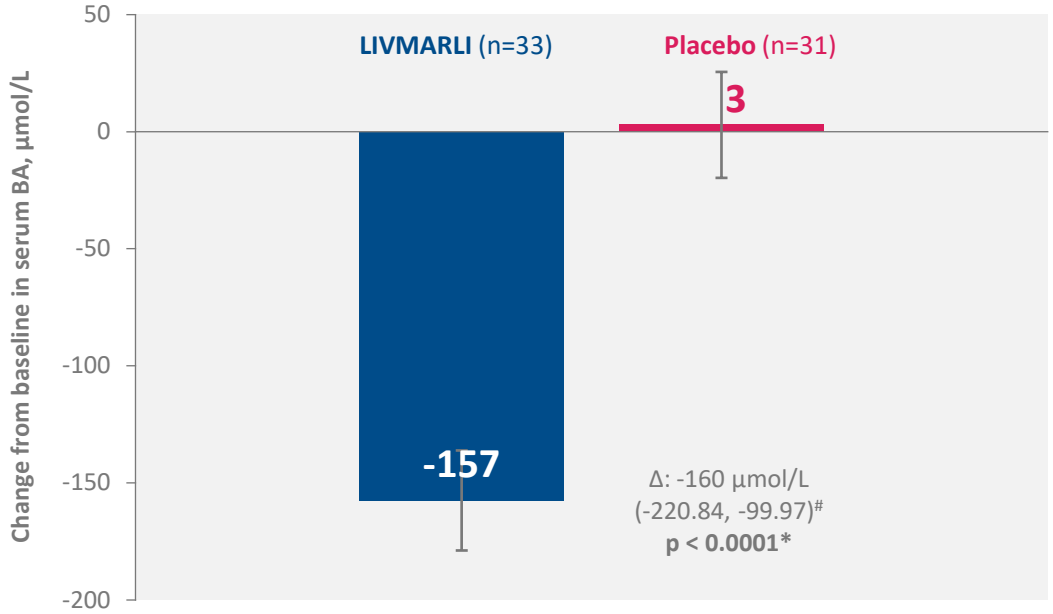

Data are LS Mean with standard error bars. Effect size compared the difference between LIVMARLI and placebo, averaged over the last 3 time periods using a repeated measures mixed effect model.
* LIVMARLI LS Mean = Placebo LS Mean; #LS Mean Delta with 95% CI
Thompson, et al. Oral Presentation, AASLD 2022

Supplemental Material

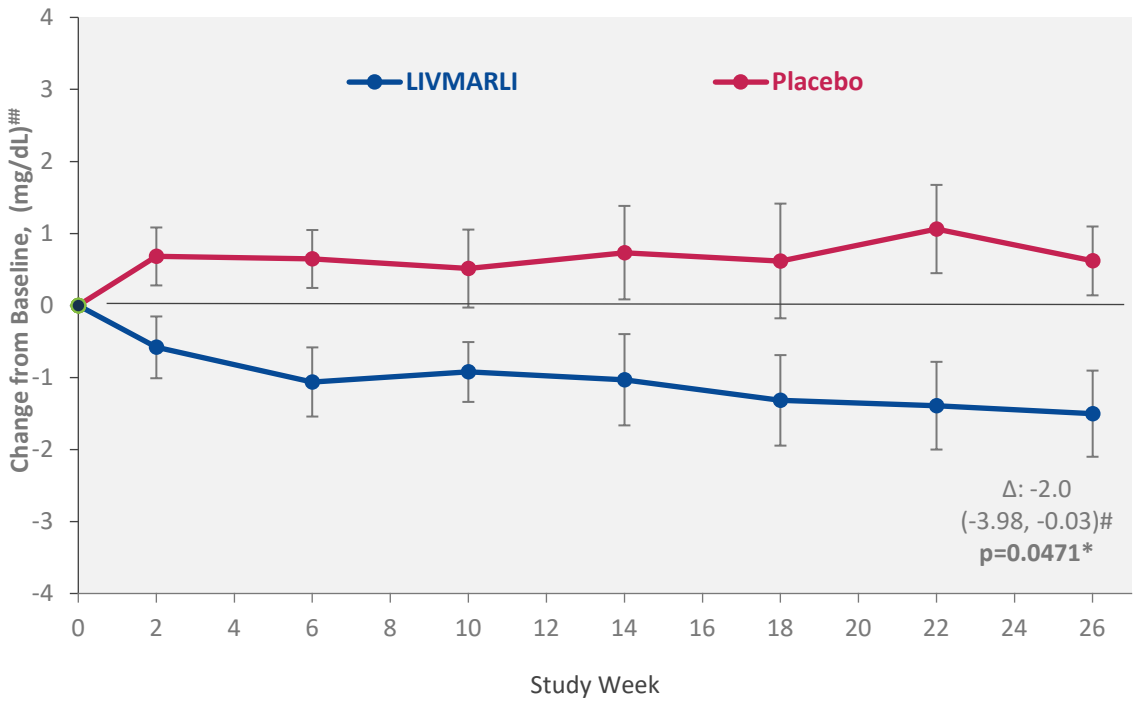
# MARCH PFIC: Significant Improvements in Markers of Liver Disease

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

## Serum Bile Acid




## Bilirubin[1]

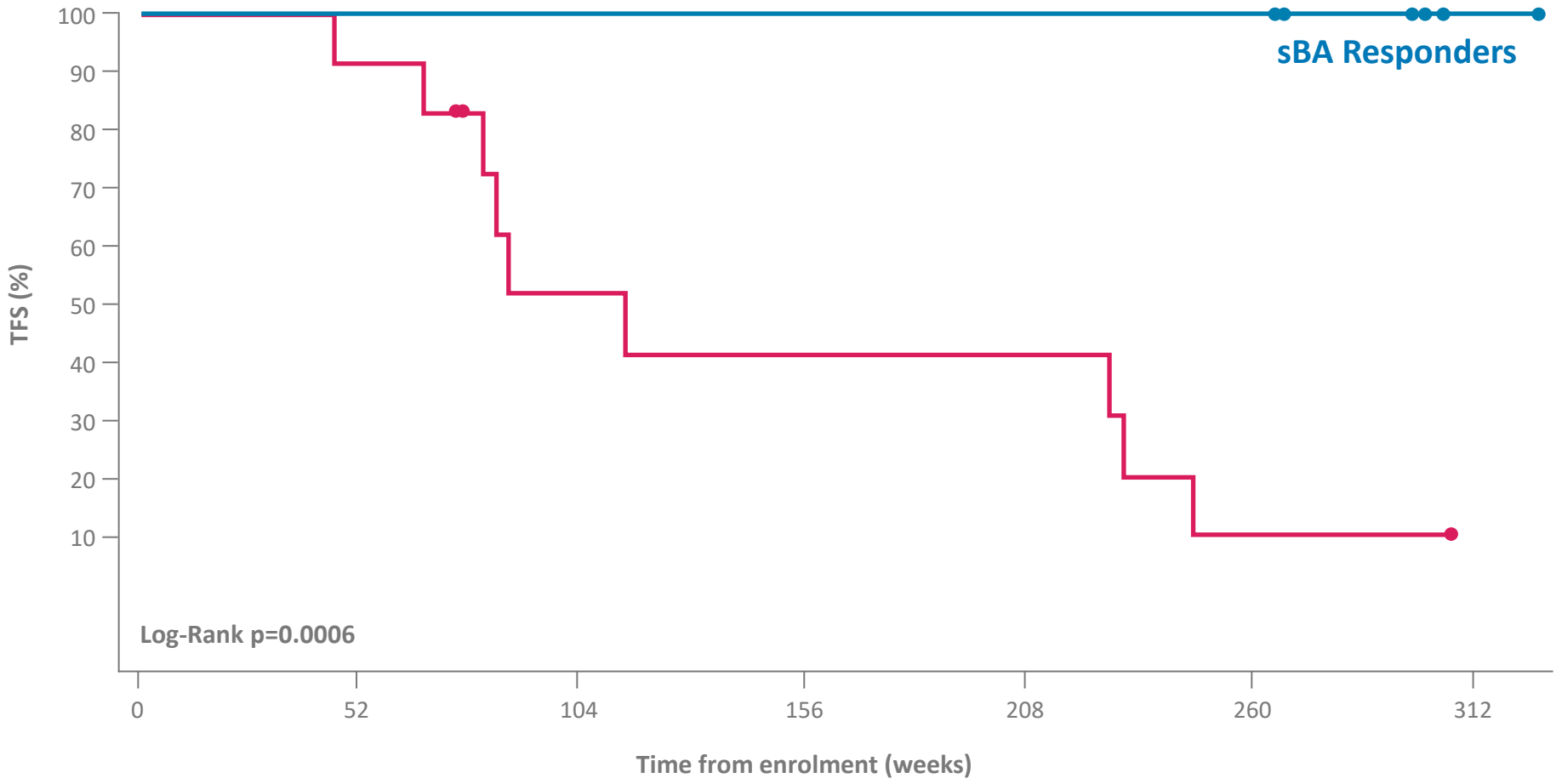



Data are LS Mean with standard error bars. Effect size compared the difference between LIVMARLI and placebo, averaged over the last 3 time periods using a repeated measures mixed effect model.
* LIVMARLI LS Mean = Placebo LS Mean; #LS Mean Delta with 95% CI; ## Data are mean with standard error bars
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: sBA Response Associated with Transplant-Free Survival

## INDIGO Phase 2: 100% 5-yr Transplant Free Survival in sBA Responders*




Loomes K et al. *Hepatol Commun*. 2022;6:2379-2390; n=19 (7 sBA responders, 12 sBA non-responders)

*NAPPED criteria (van Wessel et al, 2021): sBA responders defined as having an average sBA of <102 µmol/L (if baseline sBA ≥102 µmol/L), OR a ≤-75% average percent change from baseline

# 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.

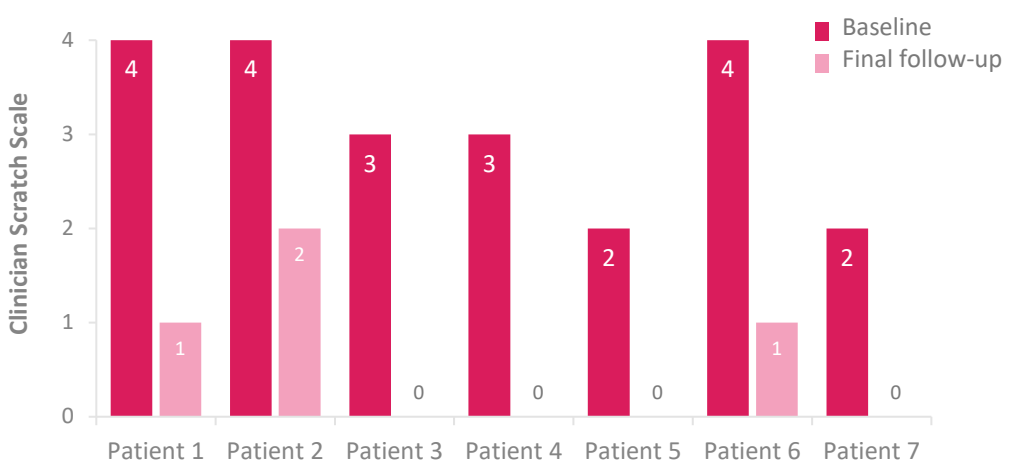
Supplemental Material

# Real World Evidence: IBAT Inhibition Reduces Pruritus and sBA in PSC

## LIVMARLI Compassionate Use in PSC Patients with Pruritus n=7

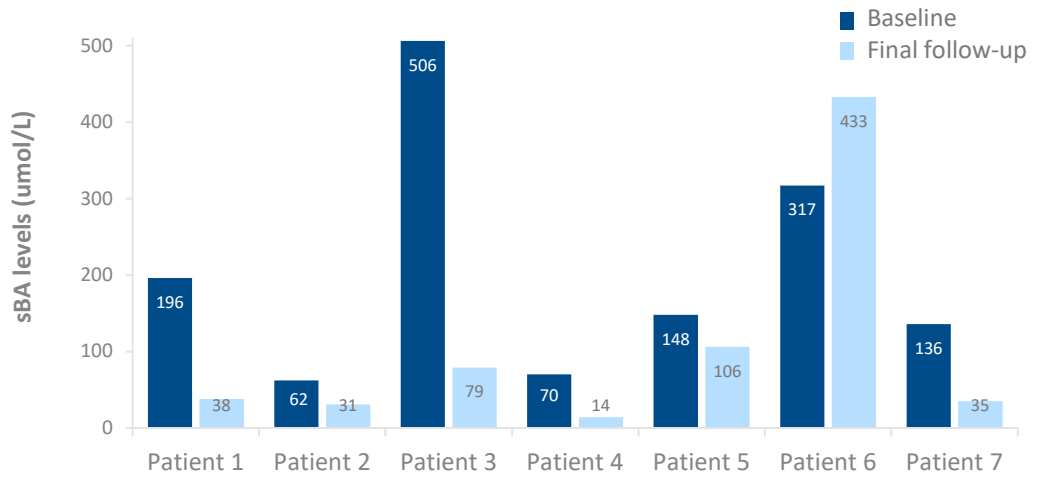
### Pruritus

**6 of 7 subjects with complete or near complete resolution**




### Bile Acids

**Most patients showed reductions in sBA levels**




Hochberg et al, DDW 2025

Clinician Scratch Scale (CSS) is a 5-point pruritus assessment scale for which 0 = none and 4 = cutaneous mutilations, hemorrhage, scarring; A ≥1-point reduction in CSS is considered clinically meaningful.

# Intellectual Property Overview

# LIVMARLI IP Coverage in the United States to 2040+

Livmarli® (maralixibat)

| Indication | |
|---|---|
| ALGS | **Method of Treatment: Dosing (2031, 2040)**<br>Orange Book Listed – Patent No. 11,229,647 / 11,497,745 / 11,918,578 / 11,260,053<br>**Method of Treatment (2032, 2037)**<br>Orange Book Listed – Patent No. 11,376,251<br>**Orphan Designation (2030)**<br>**[Pending] Formulation, Manufacturing, Additional Dosing (2042, 2043)** |
| PFIC | **Method of Treatment (2032)**<br>Orange Book Listed – Patent No. 10,512,657 / 11,229,661 / 12,350,267<br>**Orphan Designation (2031)**<br>**[Pending] Formulation, Manufacturing, Additional Dosing (2042)**<br>**[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**<br>**PSC** | **Composition of Matter (2027)**<br>Patent No. 7,956,085<br>**PBC Granted Orphan Designation, 7 years from approval**<br>**PSC Eligible for Orphan Designation, 7 years from approval**<br>**[Pending] Method of Treatment: Dosing (2032, 2040)**<br>**[Pending] Additional Dosing (2042)** |
| Ctexli® (chenodiol) tablets 250mg | **CTX** | **Orphan Designation (2032)** |
| **MRM-3379** | **FXS** | **Composition of Matter (2039)[1]**<br>Patent No. 9,120,770 |

[1]Assumes standard patent term extension

# LIVMARLI IP Coverage in Europe to 2040+

Livmarli® (maralixibat)

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

# IP Coverage for Pipeline Indications in Europe

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