Mirum Pharmaceuticals:

Interim Analysis Results of the VISTAS (PSC) Phase 2b and VANTAGE (PBC) Phase 2b Studies



June 2024

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 business strategy, objectives and opportunities, including the future opportunities for LIVMARLI, CHOLBAM, CHENODAL, and VOLIXIBAT . 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, progress of, and our plans and expectations for commercializing LIVMARLI, CHOLBAM and CHENODAL in the United States and rest of world; the costs of our business strategy, commercialization plans and development programs, and 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 CHENODAL and any of our product candidates, including VOLIXIBAT; our expected growth; 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, including our VISTAS and VANTAGE Phase 2b 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.

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mirum

Established leader in the development and commercialization of life changing rare disease medicines

3	*Standard of Care Medicines*	*4*	*Approved Indications*	*3*	*Late Stage Indications*

Strong Execution Positions Mirum as a High-Growth, Global Biotech

2018

mirum

Founded November 2018

2021

Livmarli® (maralixibat) oral solution

Approved Sept, 2021[1]
(Alagille Syndrome)

2023

Livmarli® (maralixibat) oral solution

Cholbam® (cholic acid) capsules

CHENODAL® (Chenodiol Tablets 250 mg)[2]

3 Commercial Medicines
Global Commercial Footprint

2024+

$310-320M

2024 Net Product Sales Guidance

Positive Interims for Volixibat
LIVMARLI Approved for PFIC[3]
3 Late-Stage Indications
Financially Sustaining Scale

Proven Track Record of Execution and Efficiency

[1] Received U.S. FDA approval for cholestatic pruritus in patients with Alagille syndrome (ALGS) three months of age and older. European Commission has granted marketing authorization for LIVMARLI® (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) two months of age and older

[2] Chenodal® is currently commercially available under medical necessity for CTX as determined by the FDA

[3] Received U.S. FDA approval for cholestatic pruritus in patients with Progressive Familial Intrahepatic Cholestasis (PFIC) five years of age and older. European Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion of LIVMARLI for the treatment of PFIC in patients three months and older. LIVMARLI received favorable opinion from The European Committee for Orphan Medicinal Products (COMP) recommending maintenance of orphan drug designation for LIVMARLI in PFIC. EMA approval anticipated Q3 '24

Commercial Portfolio with Pipeline of Growth Opportunities

3 APPROVED RARE DISEASE PRODUCTS, 3 ADDITIONAL INDICATIONS IN DEVELOPMENT IN HIGH-NEED ORPHAN INDICATIONS

	Indication	Patients (US+EU)	Preclinical	Phase 1	Phase 2b/Phase 3	Approved
Livmarli® (maralixibat) oral solution	Alagille Syndrome[1]	4,000 – 5,500	FDA and EMA Approved			
	Progressive Familial Intrahepatic Cholestasis[2]	1,000+	FDA Approved EMA Positive CHMP & Favorable COMP opinions – anticipated approval Q3 2024			
CHENODAL® (Chenodiol Tablets 250 mg)	Cerebrotendinous Xanthomatosis (CTX)[3]	1,000+ (US)	RESTORE Phase 3 Positive, NDA filing planned H1 2024			*Approved for radiolucent gallstones*
Cholbam® (cholic acid) capsules	Bile acid synthesis disorders and PBD-ZSD[4]	200-300 (US)	FDA Approved			
Volixibat	Primary Sclerosing Cholangitis	~54,000	VISTAS Positive Interim Analysis, expect enrollment completion 2H 2025			
	Primary Biliary Cholangitis	~230,000	VANTAGE Positive Interim Analysis, expect enrollment completion 2026			

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[4] PBD-ZSD = Peroxisome biogenesis disorder-Zellweger Spectrum Disorder

Volixibat

Interim Analysis Results of the VISTAS (PSC) Phase 2b and VANTAGE (PBC) Phase 2b Studies

IBAT Inhibition In Cholestatic Liver Disease

Targeting IBAT Removes Circulating Bile Acids Addressing Toxic Bile Acid Accumulation

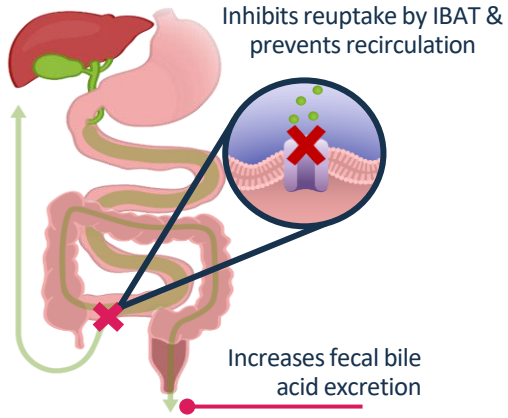
Cholestatic Liver Disease

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

- **Severe Pruritus**
- **Cellular damage**
- **Poor outcomes**

Targeting IBAT Lowers Bile Acids

Mechanism directly addressing bile acid accumulation



IBAT Inhibition Clinical Benefits[1-5]

Transplant-free survival
Quality of Life
Growth

Pruritus
Bilirubin (PFIC)
Xanthomas (ALGS)

IBAT: Ileal bile acid transporter

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.

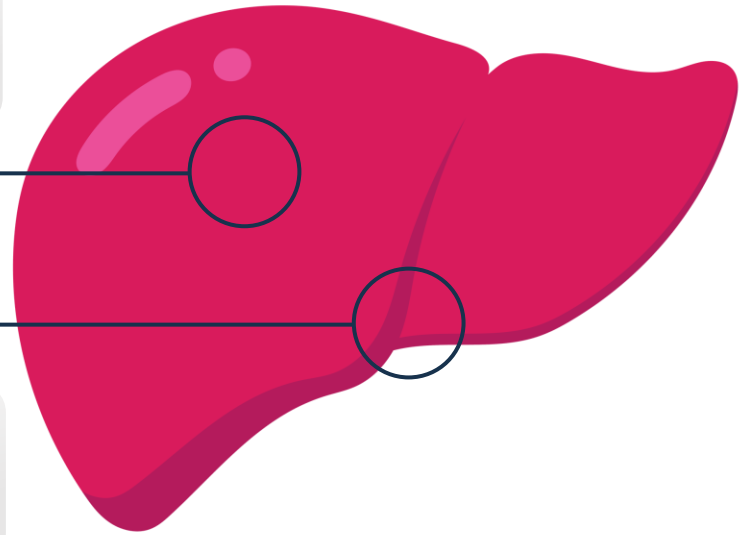
PSC & PBC: Immuno-inflammatory Rare Liver Disease

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% patients with pruritus



PBC: inflammatory driven cholestasis
230,000 patients US/EU
60% patients with 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

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

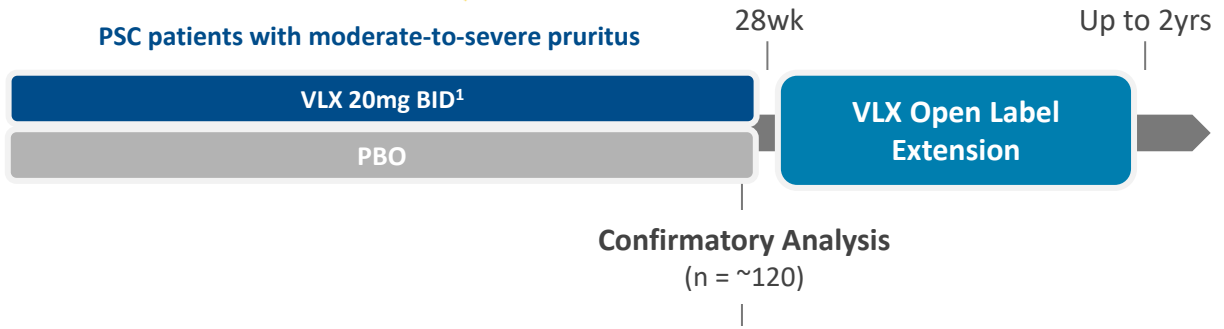
Positive Interim Analysis

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

Enrollment Completion Expected H2 2025

[1] Participants are randomized 1:1 between Volixibat 20mg BID and Placebo

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

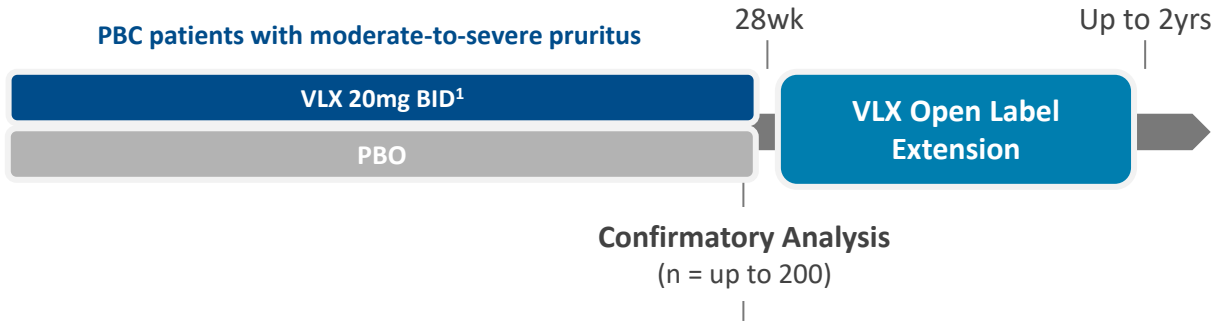
Positive Interim Analysis

Rapid and statistically significant improvement in pruritus

Reductions in sBA and improvements in fatigue

20 mg BID dose selected for confirmatory portion

VANTAGE



Primary Endpoint

Change in pruritus from baseline to 28wk

Enrollment Completion Expected 2026

[1] Participants are randomized 1:1 between Volixibat 20mg BID and Placebo

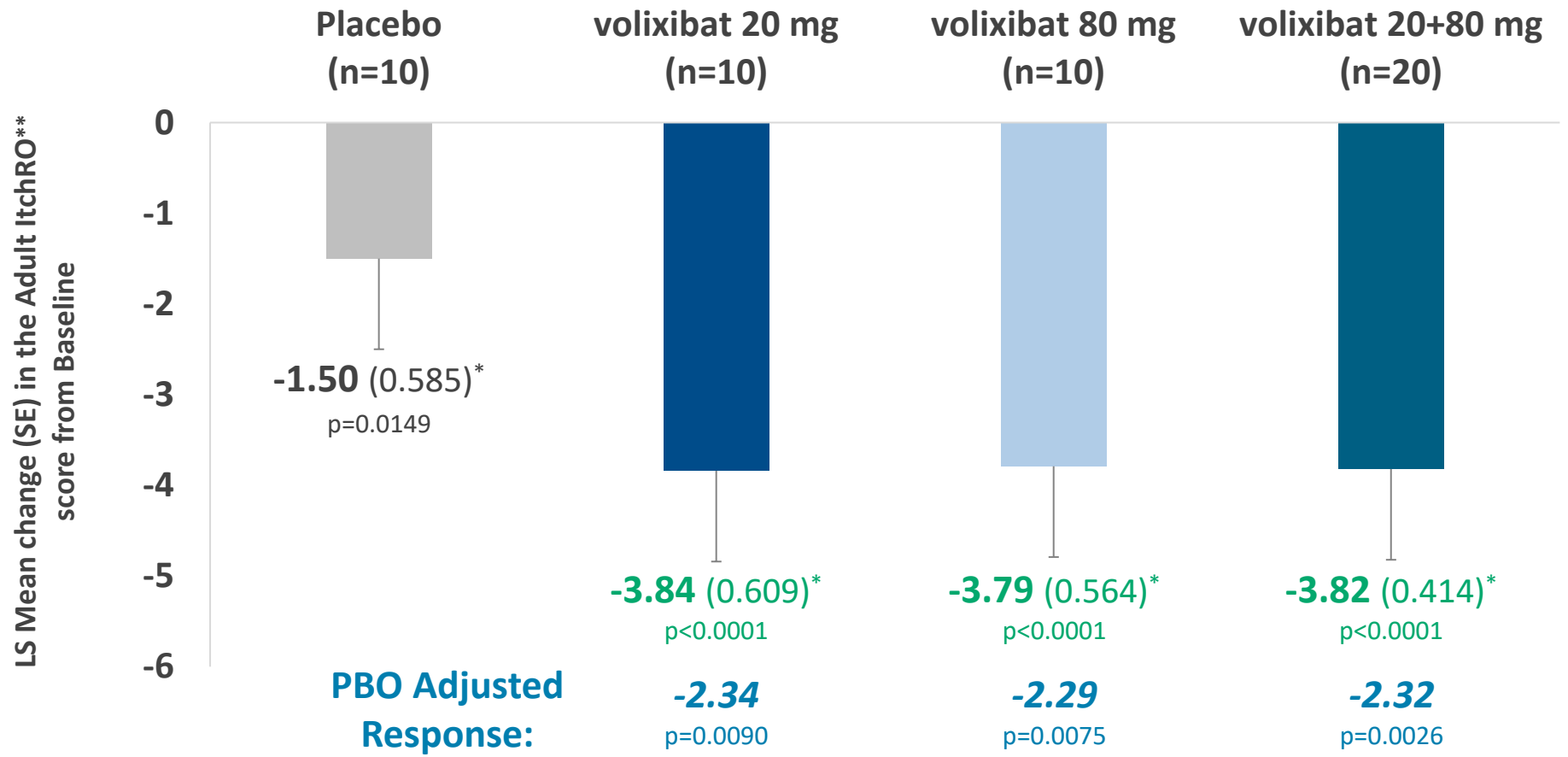
VANTAGE Interim Analysis: Baseline Characteristics Well Balanced

Characteristic	Volixibat BID 20mg (n=10)	Volixibat BID 80 mg (n=10)	Placebo (n=10)
Age (years), mean (SD)	53.9 (15.83)	52.3 (5.91)	62.1 (10.26)
Female, n (%)	8 (80)	9 (90)	9 (90)
Adult ItchRO Score, mean (SD)	6.8 (1.58)	6.3 (1.75)	6.5 (1.37)
sBA in umol/L, mean (SD)	53.3 (52.78)	43.7 (73.22)	34.2 (56.91)
ALP (U/L), mean (SD)	237.5 (134.42)	231.8 (106.88)	175.3 (115.80)

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



* LS Means (SE), LSMean, SE and p-values are from MMRM model
**Adult ItchRO is a 0-10 numerical rating scale

VANTAGE Interim Analysis: Other Observations

- Significant reduction in pruritus as early as Week 1
- Significant improvements in fatigue at Week 16
- 75% 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, all mild to moderate and most transient
    - One patient discontinued due to diarrhea
  - 4 patients experienced SAEs, including one in the placebo arm

Mirum is a Leading High-Growth, Global Rare Disease Biotech

Growing commercial business

$179M 2023 net product sales[1]
$310-320M expected 2024 net product sales

Strong financial performance

Financially self-sustaining, $303M Cash Balance[2]

Attractive pipeline with near-term milestones

2024 Value Drivers

Continued strong revenue growth

LIVMARLI: Continued US/Intl demand growth

Bile Acid portfolio: Growth in line with historical trends

Regulatory and pipeline milestones

- ✓ PFIC US FDA Approval[3]
- ✓ PFIC Positive CHMP & Favorable COMP Opinions
- CTX NDA Filing H1 '24
- ✓ VISTAS (PSC) & VANTAGE (PBC) Interim Analyses
- PFIC EC Approval Endorsement Q3 '24

[1] Mirum Pharmaceuticals Inc. FY 2023 10-K
[2] Cash and cash equivalents as of March 31, 2024
[3] Received U.S. FDA approval for cholestatic pruritus in patients with Progressive Familial Intrahepatic Cholestasis (PFIC) five years of age and older



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