Mirum Pharmaceuticals

VISTAS Phase 2b Topline Results
AZURE-1 Phase 2b Topline Results

May 4, 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 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; and the expected benefits of acquisitions and in-licenses. 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 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 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 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 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.

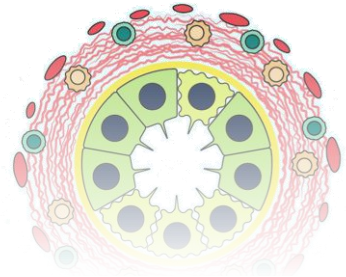
This presentation discusses product candidates that are under clinical study and which have not yet been approved for marketing by the U.S. Food and Drug Administration or other relevant regulatory authorities. No representation is made as to the safety or effectiveness of these product candidates for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

VISTAS Phase 2b
Primary Sclerosing Cholangitis (PSC)

Topline Results

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[1-3]

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. Gasteroenterol. 2024, Kuo A, et al. Clin Gastroenterol Hepatol. 2019; 2 Tanaka A & Mertens JC. Inflamm Intest Dis. 2016; 3. 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

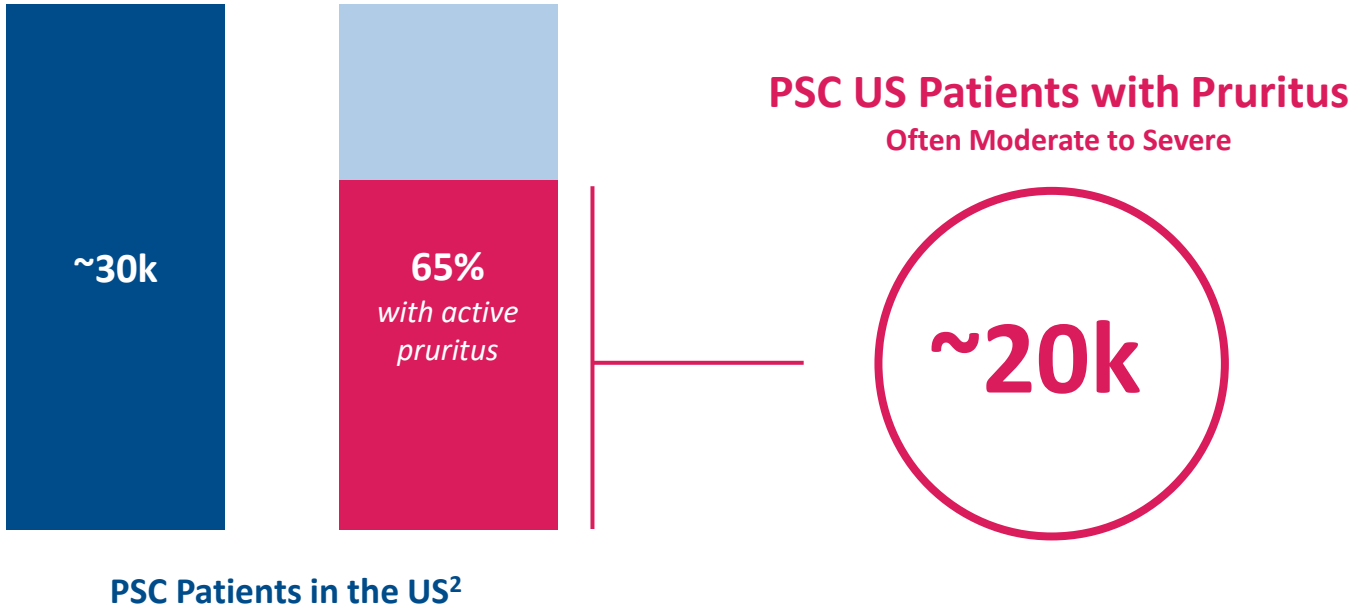
8

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]*

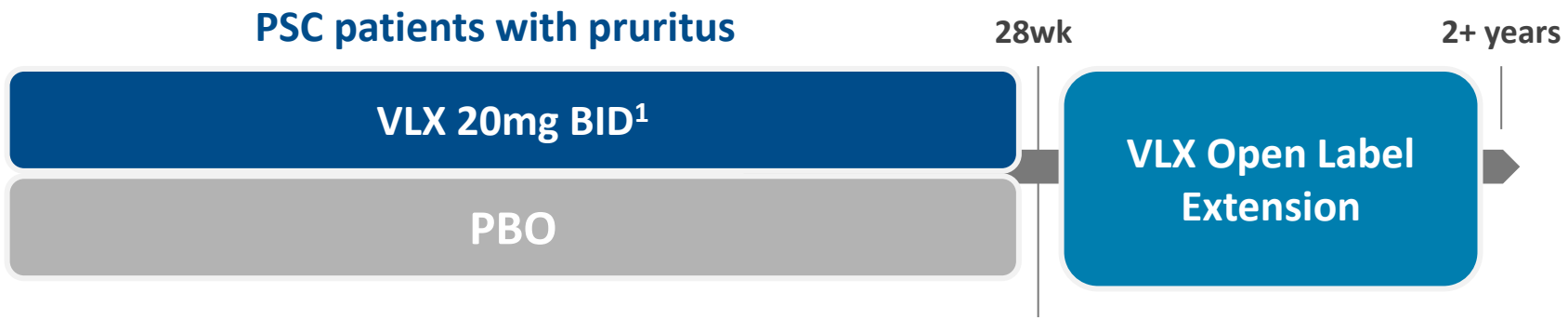
No Approved Therapies; Significant Opportunity in PSC



[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

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)

[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

	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

VISTAS Phase 2b Met Primary Endpoint

RAPID, STATISTICALLY SIGNIFICANT REDUCTIONS IN PRURITUS

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

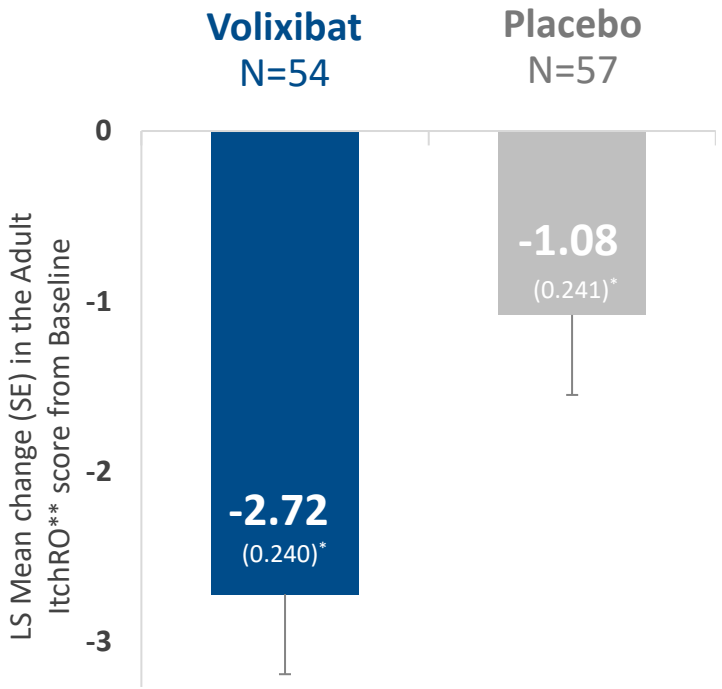
-1.64

PBO Adjusted Response

p<0.0001

Primary Cohort

(moderate to severe pruritus)



55.6%

≥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.

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)

- 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

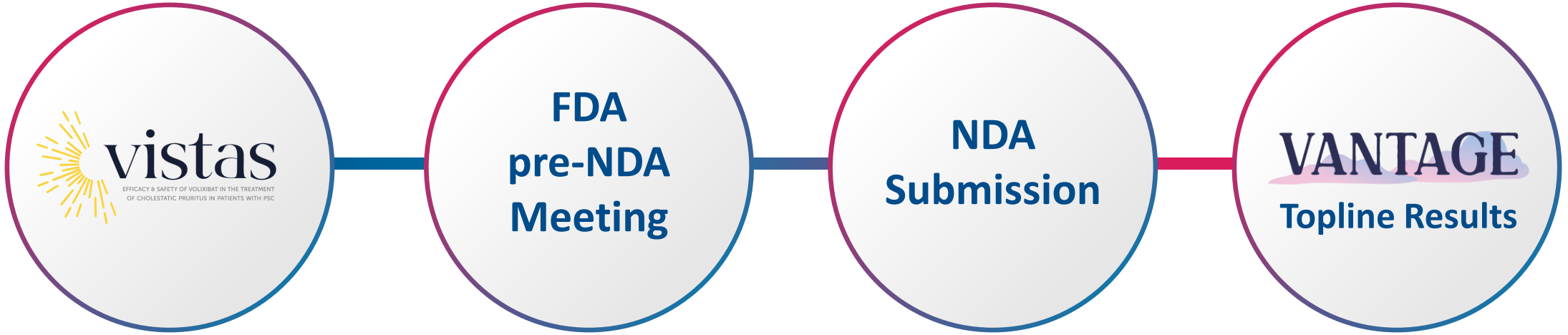
Volixibat's safety profile was generally consistent with the known effects of IBAT inhibition

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

Volixibat Program Next Steps



Oral Late Breaker, EASL International Liver Congress May 2026

Scheduled Summer 2026

Planned H2 2026

Now Expected Q1 2027

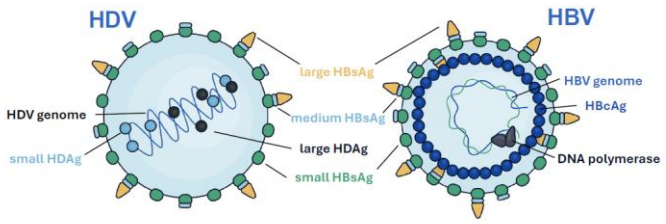
AZURE Phase 2b
Chronic Hepatitis Delta Virus (HDV)

Topline Results

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

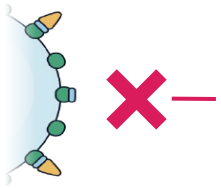
A significant global unmet need

[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 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 (Virologic Response + ALT normalization)	9/20 (**45%**) p = 0.003	7/20 (**35%**) 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

AZURE-1 n~200 2:2:1

AZURE-4 n~110 2:1:1

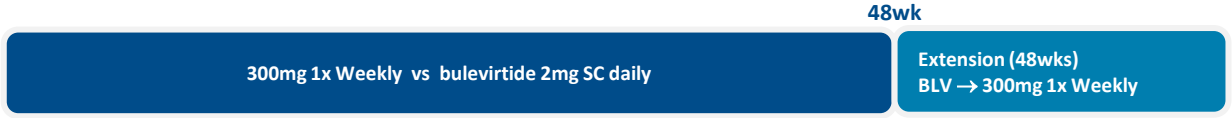
Primary Endpoint
Week 24 Virologic Response[2]
+ ALT normalization

EMA Registration Enabling Studies
Enrolling

AZURE-3[3] n=120 1:1

Primary Endpoint
Week 24 Virologic Response
(Proportion TND)

AZURE-2 n=172 3:1



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

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 12 months

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 Q2
- ❑ 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 Q1**
- ❑ 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