



MIRUM PHARMACEUTICALS

AZURE-1 Phase 3 Topline Results

AZURE-1 Phase 2b 48-Week Topline Results

September 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 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.



BRELOVITUG

CHRONIC HEPATITIS DELTA VIRUS (HDV)

AZURE-1 Phase 3 Topline Results

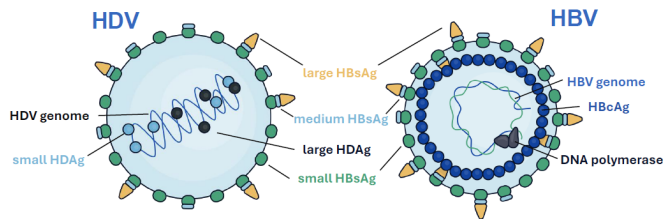
AZURE-1 Phase 2b 48-Week Topline Results



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 Years¹

5yrs

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

3x

Risk of **Liver Cancer (HCC)** vs. HBV³

~15,000

*US pts diagnosed, insured, under care*⁴



~40,000 Est. US Prevalence

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

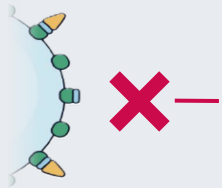
A significant global unmet need

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

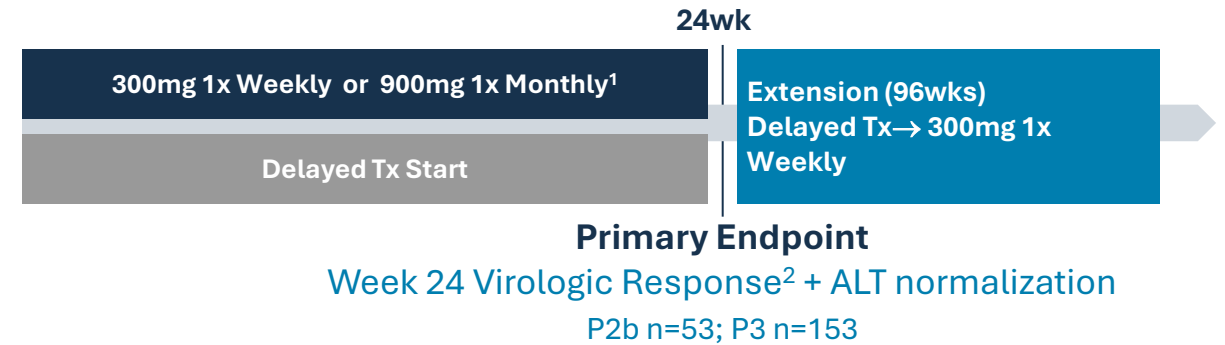


Binds to HBsAg
Neutralizes HDV/HBV
Clears virions & subviral particles

100%

Virologic Response
Demonstrated in P2
Clinical Trial

AZURE-1 Phase 2b/3 Study Design



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 $\geq 2 \log_{10}$ 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 $\geq 2 \log_{10}$ 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 $\geq 2 \log_{10}$ 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:

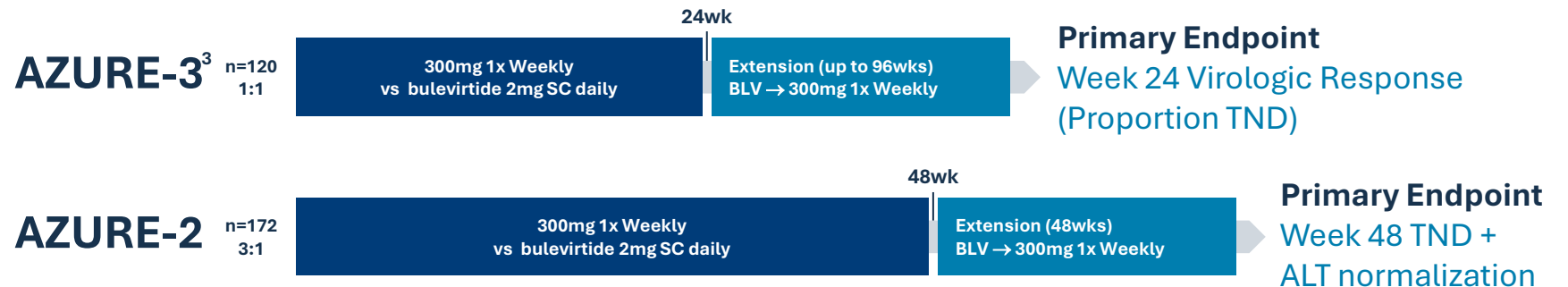
AZURE-1 Primary Endpoint met
AZURE-4 expected Q4 2026



EMA Registration Enabling Studies

Topline Data:

AZURE-2 and AZURE-3
expected in H2 2027



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.

Well-Positioned to Execute on Our Planned Strategy

★ 5 potential approval and label expansion opportunities expected



2026

2027

2026 FY Guidance

\$680-700M

2026 Net Product Sales Guidance

Cash Flow Positive in 2027

\$561M Cash Balance¹

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

- ☐ ★ **VANTAGE (PBC) topline results in Q1**
- ☐ Brelovitug HDV BLA submission in H1
- ☐ Volixibat PSC NDA 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 June 30, 2026.