

October 2026

Mirum Pharmaceuticals

EXPAND Phase 3 Topline Results





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.

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

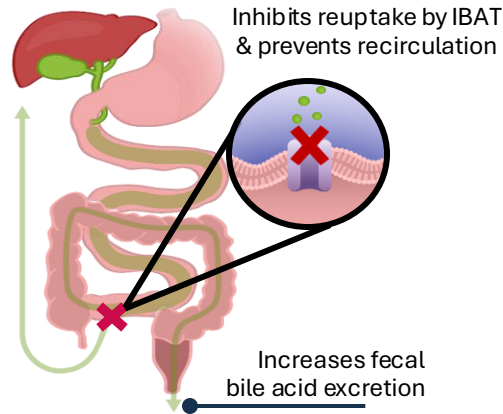
⚡ **Severe pruritus**

⚡ **Cellular damage**

⚡ **Poor outcomes**

Targeting IBAT Lowers Bile Acids

Mechanism directly addresses bile acid accumulation



IBAT Inhibition Clinical Benefits¹⁻⁵

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/S016827820307571>. 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.

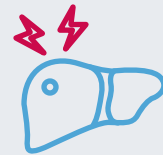
LIVMARLI Label Expansion Opportunity in Cholestatic Pruritus

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 Patients¹

2,000

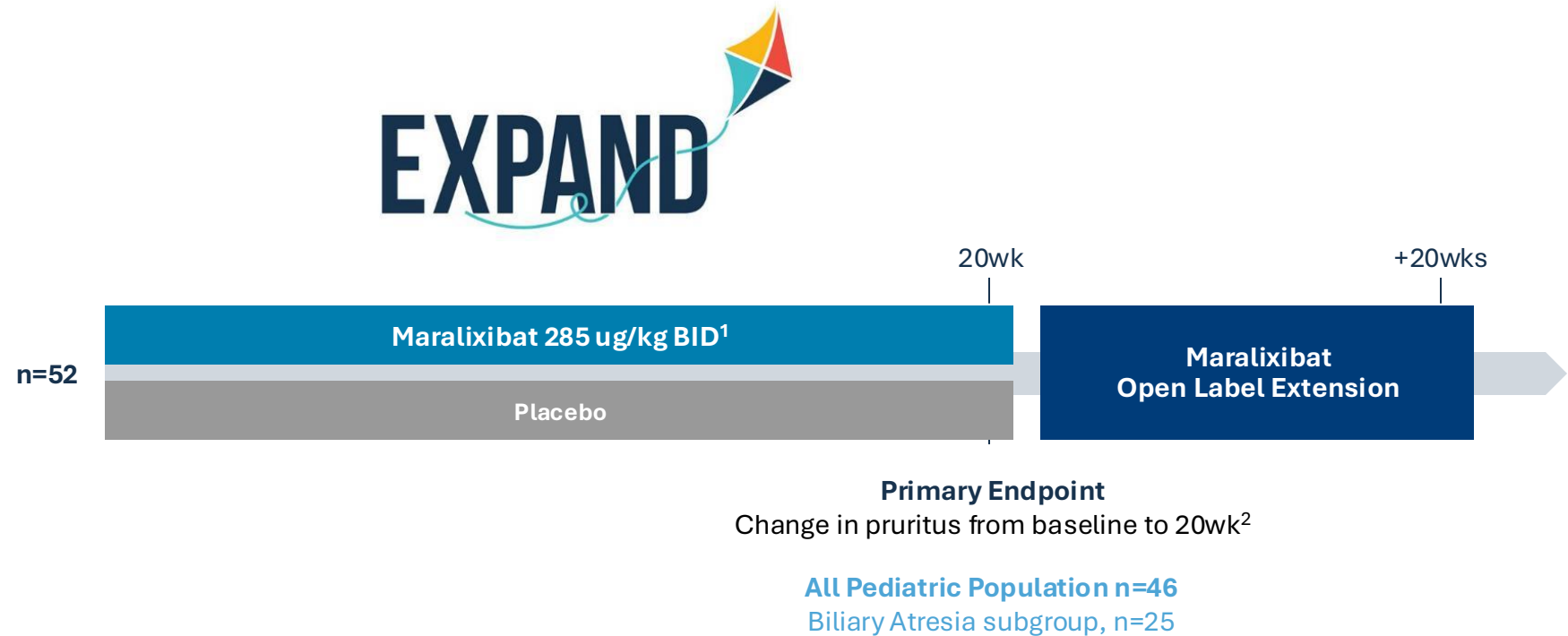
LIVMARLI Is Uniquely Positioned to Address the Burden of Cholestatic Pruritus

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 $>2 \times \text{ULN}$



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

² Itch RO(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

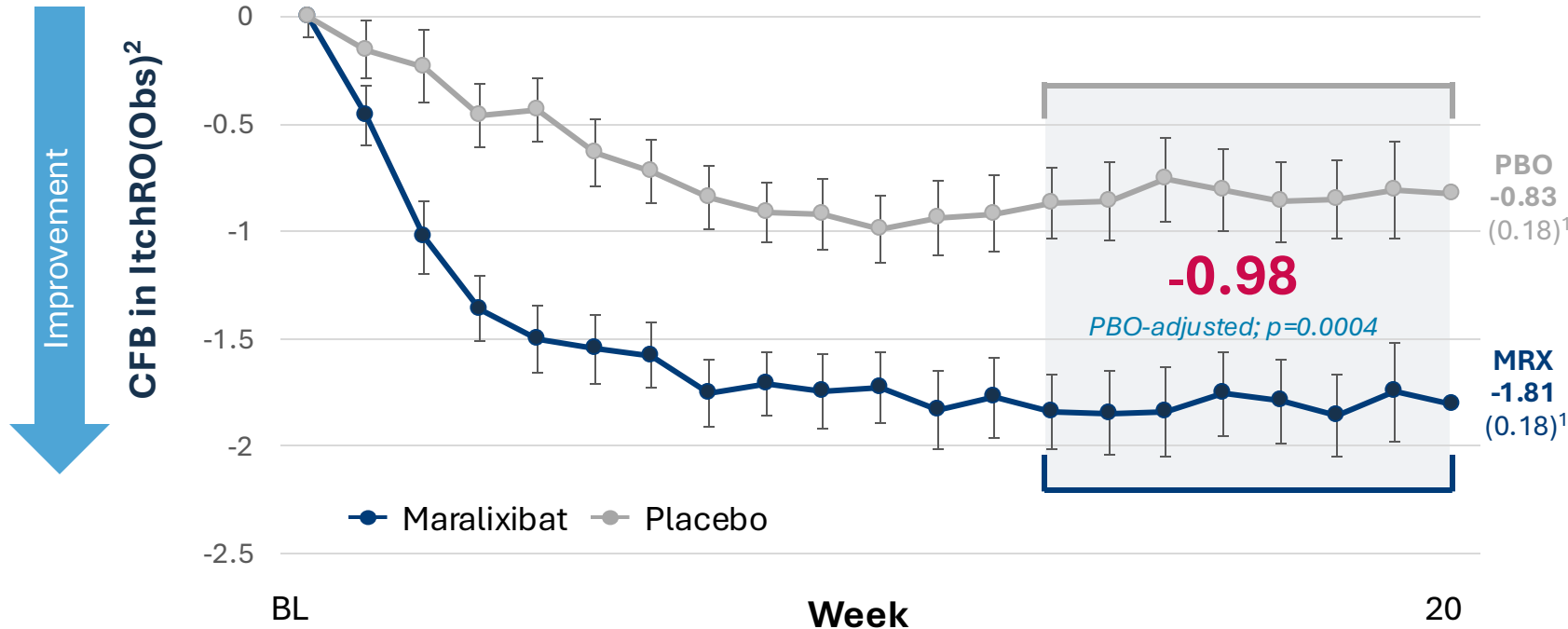
	MRX (N=22)	Placebo (N=24)
Age at enrollment in years (SD)	6.6 (4.4)	6.0 (4.0)
Sex (Female %)	10 (45.5)	11 (45.8)
ItchRO(Obs) Score (SD)	2.65 (0.96)	2.65 (0.70)
sBA, $\mu\text{mol/L}$ (SD)	149.7 (92.9)	155.4 (98.6)
ALT, U/L (SD)	95.4 (59.4)	134.2 (88.2)
Total bilirubin, mg/dL (SD)	1.1 (0.7)	1.2 (1.2)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

¹Other 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.



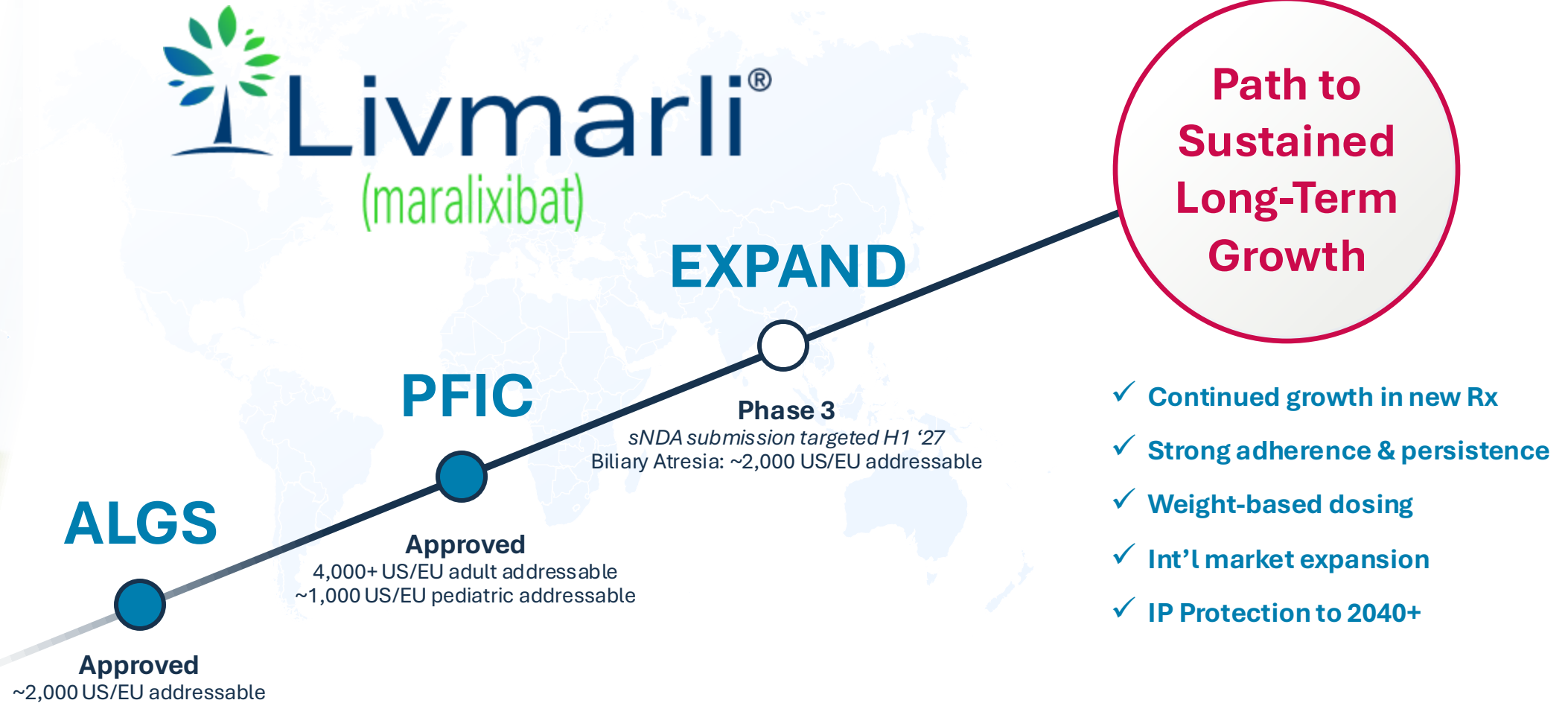
EXPAND Phase 3 Safety Summary (Safety Population)

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.

Expanding Impact in Rare Cholestatic Pruritus





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

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 positive topline results**
- Zilurgisertib FOP launch in Q4

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



THANK YOU

RELENTLESSLY RARE

Patient inspired.
Ingenuity driven.

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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.