Mirum Pharmaceuticals Reports Third Quarter 2021 Financial Results and Provides Business Update

November 15, 2021

- U.S. commercial launch underway following U.S. FDA approval of LIVMARLI™ (maralixibat) oral solution

- Six-year analysis showing significant improvement in event-free survival with LIVMARLI compared to natural history cohort (p<0.0001) presented as late-breaking, Best of The Liver Meeting presentation at the American Association for the Study of Liver Diseases (AASLD) The Liver Meeting® 2021

- *The Lancet* published data from the pivotal LIVMARLI ICONIC study demonstrating significant and durable responses in pruritus and other clinically meaningful improvements in patients with ALGS

- Conference call to provide business updates and discuss data presented at AASLD today, November 15 at 1:30 p.m. PT/4:30 p.m. ET

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 15, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), today reported financial results for the quarter ended September 30, 2021, and provided a business update.

“The FDA approval and launch of LIVMARLI capped a transformational third quarter for Mirum and the Alagille syndrome patient community. The launch of LIVMARLI, the first and only approved medication for the treatment of cholestatic pruritus in patients one year of age and older with Alagille syndrome, is off to a strong start,” said Chris Peetz, president and chief executive officer at Mirum. “We believe the launch of LIVMARLI, coupled with a strong balance sheet and an advancing late-stage clinical pipeline, positions Mirum for sustained growth as we lead the way in rare liver disease worldwide.”

Recent Key Operational Highlights

- Received U.S. Food and Drug Administration (FDA) approval for and launched LIVMARLI for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) one year of age and older.
- Presented LIVMARLI six-year event-free survival late breaker data at AASLD, showing significant improvement in event-free and transplant-free survival (p<0.0001).
- Presented clinical prognostic markers that are predictive of event-free and transplant-free survival in patients treated with LIVMARLI, furthering the understanding of clinical management of ALGS.
- Published four-year safety and efficacy data from the ICONIC pivotal study in *The Lancet*, highlighting that LIVMARLI provides durable and clinically meaningful improvements across multiple disease parameters in patients with ALGS.
- Submitted a Marketing Authorization Application (MAA) for LIVMARLI for the treatment of cholestatic liver disease in patients with ALGS to the European Medicines Agency (EMA).
- Entered into an exclusive licensing agreement to develop and commercialize LIVMARLI for rare pediatric liver diseases in Japan.
- Pamela Vig, Ph.D., chief scientific officer at Mirum, promoted to Head of R&D.
- Appointed William C. Fairey, a global commercial and corporate strategy leader in rare disease, as an independent director to the Board of Directors.
- Initiated screening in VANTAGE Phase 2b study of volixibat in primary biliary cholangitis.

Financial Results

- Licensing revenue for the quarter ended September 30, 2021 was $5.0 million, which was associated with Mirum’s license and collaboration agreement with GC Pharma, compared to none for the third quarter of 2020.
- Total operating expenses for the quarter ended September 30, 2021 were $47.8 million, compared to $21.7 million for the third quarter of 2020.
    - Research and development expenses for the third quarter ended September 30, 2021 were $30.5 million, compared to $16.0 million for the comparable prior-year period. The increase was primarily due to Vivet Collaboration Agreement program development funding, increases related to volixibat clinical trial expenses for primary sclerosing cholangitis (PSC), primary biliary cholangitis (PBC) and intrahepatic cholestasis of pregnancy (ICP) and related manufacturing activities supporting clinical supply, increases in personnel and other compensation-related expenses and increases for outside consulting services, regulatory fees and other general development expenses.
    - General and administrative (G&A) expenses for the third quarter of 2021 were $17.4 million, compared to $5.7 million for the comparable prior-year period. G&A investment increase in the third quarter of 2021 versus the third quarter of 2020 was primarily due to increased personnel and operational costs associated with the launch of LIVMARLI, as well as expenses related to general legal and public relation activities.

- For the quarter ended September 30, 2021, Mirum reported a net loss of $47.1 million, or $1.55 per share, compared with a net loss of $21.5 million, or $0.86 per share for the same period in 2020.
- As of September 30, 2021, Mirum had cash, cash equivalents, and short-term investments of $205.0 million.

Upcoming Anticipated Milestones

- Pipeline
  - LIVMARLI (maralixibat)
    - Phase 3 MARCH-PFIC topline data expected in the second quarter of 2022.
    - Potential EMA approval of LIVMARLI for cholestatic liver disease in patients with ALGS in second half of 2022.
    - Phase 2b EMBARK study for biliary atresia enrolling; topline data expected in 2023.
  - Volixibat:
    - Interim analyses expected in 2022 for the Phase 2b OHANA study for ICP and Phase 2b VISTAS study for PSC, two potentially registrational studies.

Business Update Conference Call

Mirum will host a conference call today, November 15, 2021 at 1:30 p.m. PT/4:30 p.m. ET, to provide business updates and discuss data presented today at AASLD. Join the call using the following details:

Conference Call Details:

U.S. toll-free: 844-200-6205

International: 646-904-5544

Passcode: 588077

You may also access the call via webcast by visiting the Events & Presentations section on Mirum's website. A replay of this webcast will be available for 30 days.

About LIVMARLI™ (maralixibat) oral solution

LIVMARLI™ (maralixibat) oral solution is an orally administered, once-daily, ileal bile acid transporter (IBAT) inhibitor approved by the U.S. Food and Drug Administration for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older and is the only FDA-approved medication to treat cholestatic pruritus associated with Alagille syndrome. For more information, please visit LIVMARLI.com.

LIVMARLI is currently being evaluated in late-stage clinical studies in other rare cholestatic liver diseases including progressive familial intrahepatic cholestasis and biliary atresia. LIVMARLI has received Breakthrough Therapy designation for ALGS and PFIC type 2 and orphan designation for ALGS, PFIC and biliary atresia. To learn more about ongoing clinical trials with LIVMARLI, please visit Mirum's clinical trials section on the company's website.

IMPORTANT SAFETY INFORMATION

LIVMARLI can cause serious side effects, including:

Changes in liver tests. Changes in certain liver tests are common in patients with Alagille syndrome and can worsen during treatment with LIVMARLI. These changes may be a sign of liver injury and can be serious. Your healthcare provider should do blood tests before starting and during treatment to check your liver function. Tell your healthcare provider right away if you get any signs or symptoms of liver problems, including nausea or vomiting, skin or the white part of the eye turns yellow, dark or brown urine, pain on the right side of the stomach (abdomen) or loss of appetite.

Stomach and intestinal (gastrointestinal) problems. LIVMARLI can cause stomach and intestinal problems, including diarrhea, stomach pain, and vomiting during treatment. Tell your healthcare provider right away if you have any of these symptoms more often or more severely than normal for you.

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. FSV deficiency is common in patients with Alagille syndrome but may worsen during treatment. Your healthcare provider should do blood tests before starting and during treatment.

Other common side effects reported during treatment were bone fractures and gastrointestinal bleeding.

Prescribing information

About Volixibat

Volixibat is an oral, minimally absorbed agent designed to selectively inhibit the ileal bile acid transporter (IBAT). Volixibat may offer a novel approach in the treatment of adult cholestatic diseases by blocking the recycling of bile acids, through inhibition of IBAT, thereby reducing bile acids systemically and in the liver. Phase 1 and Phase 2 studies of volixibat demonstrated on-target fecal bile acid excretion, a pharmacodynamic marker of IBAT inhibition, in addition to decreases in LDL cholesterol and increases in 7αC4 which are markers of bile acid synthesis. Volixibat has been evaluated in

more than 400 individuals across multiple clinical trials. The most common adverse events reported were mild to moderate gastrointestinal events observed in the volixibat groups.

Volixibat is currently being evaluated in Phase 2b studies for primary sclerosing cholangitis (VISTAS study), intrahepatic cholestasis of pregnancy (OHANA study) and primary biliary cholangitis (VANTAGE study).

About Mirum Pharmaceuticals, Inc.

Mirum Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to transforming the treatment of rare liver diseases. Mirum's approved medication is LIVMARLI™ (maralixibat) oral solution which is approved in the U.S. for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older.

Mirum's late-stage pipeline includes two investigational treatments for debilitating liver diseases affecting children and adults. Maralixibat (LIVMARLI), an oral ileal bile acid transporter (IBAT) inhibitor, is currently being evaluated in clinical trials for pediatric liver diseases and includes the MARCH Phase 3 study for progressive familial intrahepatic cholestasis (PFIC) and the EMBARK Phase 2b study for patients with biliary atresia. In addition, Mirum has an expanded access program open in Canada, Australia, the UK and several countries in Europe for eligible patients with Alagille syndrome.

Mirum has submitted a Marketing Authorization Application to the European Medicines Agency for maralixibat for the treatment of cholestatic liver disease in patients with Alagille syndrome.

Mirum's second investigational treatment, volixibat, also an oral IBAT inhibitor, is being evaluated in three registrational studies including the OHANA Phase 2b study for pregnant women with intrahepatic cholestasis of pregnancy, the VISTAS Phase 2b study for adults with primary sclerosing cholangitis, and the VANTAGE Phase 2b study for primary biliary cholangitis.

To augment its pipeline in cholestatic liver disease, Mirum has acquired the exclusive option to develop and commercialize gene therapy programs VTX-803 and VTX-802 for PFIC3 and PFIC2, respectively, from Vivet Therapeutics SAS, following preclinical evaluation and investigational new drug-enabling studies.

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Forward-Looking Statements

This press release includes forward-looking statements pertaining to the Company's planned participation at a scientific conference, which may include discussion of the Company's revenue, business, and operations, including the discovery, development and commercialization of our product candidates and technologies, and the therapeutic potential thereof, the continuation of our clinical trials, and the success of our collaborations with partners and any potential future collaborations. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. Applicable risks and uncertainties include those relating to our preclinical research and clinical programs and other risks identified under the heading "Risk Factors" included in our most recent Form 10-Q and Form 10-K filings and in other future filings with the SEC. The forward-looking statements contained in this press release reflect Mirum's current views with respect to future events, and Mirum does not undertake and specifically disclaims any obligation to update any forward-looking statements.

Mirum Pharmaceuticals, Inc.
Condensed Consolidated Statement of Operations Data
(in thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
License revenue	$ 5,000	$ -	$ 16,000	$ -
Operating expenses:				
Research and development	30,471	15,984	103,653	51,879
General and administrative	17,353	5,732	40,185	15,466
Total operating expenses (1)	47,824	21,716	143,838	67,345
Loss from operations	(42,824)	(21,716)	(127,838)	(67,345)

Other income (expense):				
Interest income	72	237	301	1,391
Interest expense	(5,667)	-	(13,824)	-
Change in fair value of derivative liability	1,355	-	417	-
Other expense, net	(35)	(30)	(565)	(109)
Net loss before provision for income taxes	(47,099)	(21,509)	(141,509)	(66,063)
Provision for (benefit from) income taxes	9	(3)	25	4
Net loss	$ (47,108)	$ (21,506)	$ (141,534)	$ (66,067)
Net loss per share, basic and diluted	$ (1.55)	$ (0.86)	$ (4.68)	$ (2.65)
Weighted-average shares of common stock outstanding, basic and diluted	30,367,727	25,132,916	30,250,127	24,965,178

(1) Amounts include stock-based compensation expense as follows:

Research and development	$ 3,035	$ 1,361	$ 7,792	$ 3,662
General and administrative	4,380	2,067	9,731	5,313
Total stock-based compensation	$ 7,415	$ 3,428	$ 17,523	$ 8,975

Mirum Pharmaceuticals, Inc.
Selected Condensed Consolidated Balance Sheet Data
(in thousands)

	September 30, 2021	**December 31, 2020**
	(Unaudited)	
Cash, cash equivalents and short-term investments	$ 205,031	$ 231,820
Working capital	146,418	217,888
Total assets	235,167	240,864
Accumulated deficit	(314,705)	(173,171)
Total stockholders' equity	56,396	172,095

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