Mirum Pharmaceuticals Reports Fourth Quarter and Year-End 2021 Financial Results and Provides Business Update

March 9, 2022

- Strong commercial launch underway with LIVMARLI® (maralixibat) oral solution; net product revenue of $3.1 million for the first quarter of launch, total 2021 company revenue of $19.1 million

- Expect at least $8.0 million in LIVMARLI net product sales in first quarter 2022

- Conference call to provide business updates today, March 9 at 1:30 p.m. PT/4:30 p.m. ET

FOSTER CITY, Calif.--(BUSINESS WIRE)--Mar. 9, 2022-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today reported financial results for the fourth quarter and year-end 2021 and provided a business update.

“2021 marked a significant turning point for Mirum and the Alagille syndrome community. With a strong start to our launch of LIVMARLI, the first and only FDA-approved medication for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older, we achieved fourth quarter 2021 net product revenue of $3.1 million,” said Chris Peetz, president and chief executive officer at Mirum. “We expect continued growth in prescriptions and reimbursement as we unlock LIVMARLI’s potential both in the United States and globally. In addition to our growing topline, we look forward to generating data from all five of our late-stage pipeline indications over the coming two years.”

2021 Highlights

Fourth Quarter

- Launched LIVMARLI in the United States, the first and only medicine approved for cholestatic pruritus in patients with Alagille syndrome one year of age and older.
- Sold Rare Pediatric Disease Priority Review Voucher (PRV), granted by the U.S. Food and Drug Administration (FDA) in September 2021 in connection with the approval of LIVMARLI, for $110.0 million.
- Presented late-breaking oral presentation on six-year natural history comparison with LIVMARLI showing event-free survival ($p<0.0001$) and transplant-free survival ($p<0.0001$) in patients with Alagille syndrome. Also presented analysis highlighting prognostic markers of event-free survival for patients with Alagille syndrome.
- Publication in *The Lancet* highlights LIVMARLI ICONIC data demonstrating four-year durable and clinically meaningful improvements across multiple cholestasis parameters, including pruritus.
- Presented data from the ICONIC and INDIGO studies showing the impact of LIVMARLI treatment response on changes in health-related quality of life measures among patients with Alagille syndrome and progressive familial intrahepatic cholestasis with BSEP deficiency (PFIC2).

Full Year

- Received FDA approval for LIVMARLI for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older.
- Submitted marketing authorization application to the European Medicines Agency (EMA) for LIVMARLI for the treatment of cholestatic liver disease in patients with Alagille syndrome.
- Entered into partnership and distribution agreements to accelerate the potential availability of LIVMARLI in key regions.

Additional Key Operational Updates

- Announced today that on January 25, 2022, the U.S. Patent and Trademark Office (USPTO) issued U.S. patent 11,229,647 with coverage through February 12, 2040. This patent is now also listed in the U.S. FDA publication, “Approved Drug Products with Therapeutic Equivalence Evaluations,” commonly known as the “Orange Book,” further protecting LIVMARLI. The patent is supplemented by additional orange book listable patent grants and allowances with coverage to 2031 and 2032, respectively.
- Partner GC Pharma submitted a pharmaceutical approval application to the Ministry of Food and Drug Safety in Korea for LIVMARLI for the treatment of cholestatic pruritus in patients with Alagille syndrome.
- Launched expanded access program for patients with progressive familial intrahepatic cholestasis.

Financial Results

- Total net product revenue from the sale of LIVMARLI for the fourth quarter and year ended December 31, 2021 was $3.1

million, compared to none for the fourth quarter and year ended 2020.

- For the years ended December 31, 2021 and 2020, total licensing revenue was $16.0 million and none, respectively. There was no licensing revenue recorded for the fourth quarter ended December 31, 2021 and 2020.
- Total operating expenses for the fourth quarter ended December 31, 2021 were $48.7 million, compared to $37.0 million for the fourth quarter of 2020. For the years ended December 31, 2021 and 2020, total operating expenses were $192.6 million and $104.3 million, respectively.
    - Research and development expenses for the fourth quarter ended December 31, 2021 were $27.8 million, compared to $29.7 million for the comparable prior-year period. For the years ended December 31, 2021 and 2020, research and development expenses were $131.4 million and $81.6 million, respectively. The increase in full year 2021 was primarily due to activities supporting the volixibat programs, collaboration funding for the Vivet program, development milestone fees, increased personnel costs including stock-based compensation, offset by a decrease in the expense associated with the LIVMARLI programs.
    - Selling, general and administrative (SG&A) expenses for the fourth quarter of 2021 were $19.0 million, compared to $7.2 million for the comparable prior-year period. For the years ended December 31, 2021 and 2020, SG&A expenses were $59.2 million and $22.7 million, respectively. SG&A investment increased in the fourth quarter of 2021 versus the fourth quarter of 2020, primarily due to an increase in expenses associated with commercial launch activities for LIVMARLI, including personnel and stock-based compensation expenses, and increased requirements of operating as a public company.
- For the fourth quarter ended December 31, 2021, Mirum reported net income of $57.5 million, compared with a net loss of $37.2 million for the same period in 2020. For the year ended December 31, 2021, Mirum reported a net loss of $84.0 million, compared to a net loss of $103.3 million for the same period in 2020.
- As of December 31, 2021, Mirum had cash, cash equivalents, restricted cash equivalents, and investments of $261.5 million.

Upcoming Anticipated Milestones

- LIVMARLI (maralixibat)
    - Launch commercial early access programs in international markets for LIVMARLI in the first half of 2022.
    - Topline data from MARCH-PFIC Phase 3 clinical trial expected in the fourth quarter of 2022.
    - Alagille syndrome launch in Europe in the fourth quarter of 2022, if approved by the EMA.
    - EMBARK Phase 2b clinical trial for biliary atresia currently enrolling; topline data anticipated in 2023.
- Volixibat
    - Interim analyses expected for VISTAS Phase 2b clinical trial for primary sclerosing cholangitis in the fourth quarter of 2022.
    - First interim analysis expected for OHANA Phase 2b clinical trial for intrahepatic cholestasis of pregnancy in the fourth quarter of 2022.
    - Enrollment ongoing for VANTAGE Phase 2b clinical trial for primary biliary cholangitis; interim analysis expected in 2023.

Business Update Conference Call

Mirum will host a conference call today, March 9, 2022 at 1:30 p.m. PT/4:30 p.m. ET, to provide business updates. Join the call using the following details:

Conference Call Details:
U.S./Toll-Free: 833-927-1758
International: 646-904-5544
Passcode: 521837

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 (ALGS) 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 (PFIC) 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 Phase 2b clinical trial), intrahepatic cholestasis of pregnancy (OHANA Phase 2b clinical trial), and primary biliary cholangitis (VANTAGE Phase 2b clinical trial).

About Mirum Pharmaceuticals

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 clinical trial for progressive familial intrahepatic cholestasis (PFIC) and the EMBARK Phase 2b clinical trial for patients with biliary atresia. In addition, Mirum has an expanded access program open across multiple countries for eligible patients with ALGS and PFIC.

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, an oral IBAT inhibitor, is being evaluated in three potentially registrational studies including the VISTAS Phase 2b clinical trial for adults with primary sclerosing cholangitis, the OHANA Phase 2b clinical trial for pregnant women with intrahepatic cholestasis of pregnancy, and the VANTAGE Phase 2b clinical trial for adults with primary biliary cholangitis.

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Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding, among other things, the expected continued growth in prescriptions and reimbursement through 2022, the ability for Mirum to reach transformational and value-generating milestones, the increased availability of LIVMARLI in key regions, the anticipated launch and timing of the expanded access program for LIVMARLI in international markets, continued commercial success for LIVMARLI, the results, conduct and progress of Mirum's ongoing and planned clinical trials for its product candidates and the regulatory approval path for its product candidates globally. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "will," "anticipate," "expect," "potential" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Mirum's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with Mirum's business in general, the impact of the COVID-19 pandemic, and the other risks described in Mirum's filings with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. Mirum undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

Mirum Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations Data

(in thousands, except share and per share amounts)

	Three Months Ended	Year Ended

	December 31,		December 31,	
	2021	2020	2021	2020
	(Unaudited)			
Revenue:				
Product sales, net	$ 3,138	$ —	$ 3,138	$ —
License revenue	—	—	16,000	—
Total revenue	3,138	—	19,138	—
Operating expenses:				
Cost of sales	1,903	—	1,903	—
Research and development	27,775	29,726	131,428	81,605
Selling, general and administrative	19,035	7,225	59,220	22,691
Total operating expenses (1)	48,713	36,951	192,551	104,296
Loss from operations	(45,575)	(36,951)	(173,413)	(104,296)
Other income (expense)				
Interest income	65	168	366	1,559
Interest expense	(3,766)	(335)	(17,590)	(335)
Change in fair value of derivative liability	(1,149)	—	(732)	—
Other expense, net	(17)	(83)	(582)	(192)
Gain from sale of priority review voucher, net	108,000	—	108,000	—
Net income (loss) before provision for income taxes	57,558	(37,201)	(83,951)	(103,264)
Provision for income taxes	12	2	37	6
Net income (loss)	$ 57,546	$ (37,203)	$ (83,988)	$ (103,270)
Net income (loss) per share, basic and diluted			$ (2.77)	$ (4.09)
Weighted-average shares of common stock outstanding, basic and diluted			30,321,722	25,251,968
(1) Amounts include stock-based compensation as follows:				
Research and development	$ 2,095	$ 1,467	$ 9,888	$ 5,129

Selling, general and administrative	3,397	2,112	13,128	7,425
Total stock-based compensation	$ 5,492	$ 3,579	$ 23,016	$ 12,554

Mirum Pharmaceuticals, Inc.

Selected Consolidated Balance Sheet Data

(in thousands)

	December 31, 2021	December 31, 2020
Cash, cash equivalents, restricted cash equivalents, and investments	$ 261,524	$ 231,820
Working capital	123,996	217,888
Total assets	294,651	240,864
Accumulated deficit	(257,159)	(173,171)
Total stockholders' equity	120,212	172,095

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