



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

August 5, 2021

- NDA for maralixibat for cholestatic pruritus in Alagille syndrome under priority review; PDUFA date is September 29, 2021
- Commercial preparations complete in anticipation of U.S. launch of maralixibat in ALGS
- Conference call to provide business update today, August 5 at 1:30 p.m. PT/4:30 p.m. ET

FOSTER CITY, Calif.--(BUSINESS WIRE)--Aug. 5, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), today reported financial results for the quarter ended June 30, 2021 and provided a business update.

"We are pleased with the progress we have made in the last quarter as we prepare for the potential FDA approval of maralixibat and advance our late-stage clinical development pipeline," said Chris Peetz, president and chief executive officer at Mirum. "Alagille syndrome is a terrible disease, and we know patients and physicians are waiting for treatment options. We are launch ready in the United States and have amplified our reach to patients globally with our expanded access program and recent partnerships to accelerate potential approvals in a number of markets."

Recent Key Operational Highlights

- [Entered](#) into an exclusive licensing agreement with GC Pharma to develop and commercialize maralixibat for rare liver diseases in South Korea.
- [Presented](#) maralixibat transplant-free survival data for progressive familial intrahepatic cholestasis type 2 (PFIC2) and long-term safety analyses for Alagille syndrome (ALGS) at the 6th World Congress of Pediatric Gastroenterology, Hepatology and Nutrition (WCPGHAN) Annual Meeting 2021.
- [Appointed](#) Carol L. Brosgart, M.D. as an independent director to the Board of Directors.
- [Presented](#) an integrated safety analysis of maralixibat in patients with ALGS and unveiled a multi-national survey of patient reported outcomes from pregnant women with intrahepatic cholestasis of pregnancy (ICP) at the EASL International Liver Congress 2021.

Financial Results

- Licensing revenue for the quarter ended June 30, 2021 was \$11.0 million, which was associated with our license and collaboration agreement with CANbridge Pharmaceuticals, Inc., compared to none for the second quarter of 2020.
- Total operating expenses for the quarter ended June 30, 2021 were \$48.4 million, compared to \$23.6 million for the second quarter of 2020.
 - Research and development expenses for the quarter ended June 30, 2021 were \$35.0 million, compared to \$18.6 million for the comparable prior-year period. The increase was primarily driven by an upfront payment and funding associated with the Vivet gene therapy programs and costs associated with the initiation of volixibat clinical studies.
 - General and administrative expenses for the second quarter of 2021 were \$13.4 million, compared to \$5.0 million for the comparable prior-year period. The increase was primarily due to increased commercialization and headcount costs.
- For the quarter ended June 30, 2021, Mirum reported a net loss of \$43.9 million, or \$1.45 per share, compared with a net loss of \$23.3 million, or \$0.93 per share for the same period in 2020.
- As of June 30, 2021, Mirum had cash, cash equivalents, and short-term investments of \$238.8 million.

Upcoming Anticipated Milestones

- Commercial and Regulatory
 - The FDA has issued a PDUFA date of September 29, 2021, and, if approved, Mirum will launch maralixibat in the U.S. representing a commercial opportunity estimated at more than \$500 million.
 - Marketing authorization application (MAA) for PFIC2 decision expected in early 2022.
 - Mirum plans to submit an MAA for ALGS in Europe based on results from the ICONIC study and six-year event-free survival analyses.
- Pipeline and New Data
 - Maralixibat
 - Topline data from the Phase 3 MARCH PFIC study is expected in the second quarter of 2022.
 - Enrollment in the Phase 2b EMBARK study of maralixibat in biliary atresia is ongoing; data expected in 2023.

- o Volixibat

- Enrollment ongoing in the potentially registrational Phase 2b OHANA study for ICP; interim analysis expected in 2022.
- Enrollment ongoing in the Phase 2b VISTAS study for primary sclerosing cholangitis (PSC); interim analysis expected in 2022.
- A Phase 2b study in primary biliary cholangitis (PBC) is planned for the second half of 2021.

Business Update Conference Call

Mirum will host a conference call today, August 5, 2021 at 1:30 p.m. PT/4:30 p.m. ET, to provide a review of the second quarter activities and to discuss business updates. Join the call using the following details:

Conference Call Details:

U.S. toll-free: 844.200.6205

International: 646.904.5544

Passcode: 097025

You may also access the call via webcast by visiting the [investors](#) section on Mirum's corporate website. A replay of this webcast will be available for 30 days.

About Maralixibat

Maralixibat is a novel, minimally absorbed, orally administered investigational drug being evaluated in several rare cholestatic liver diseases. Maralixibat inhibits the apical sodium dependent bile acid transporter (ASBT), resulting in more bile acids being excreted in the feces, leading to lower levels of bile acids systemically, thereby potentially reducing bile acid mediated liver damage and related effects and complications. More than 1,600 individuals have received maralixibat, including more than 120 children who have received maralixibat as an investigational treatment for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). In the [ICONIC Phase 2b ALGS clinical trial](#), patients taking maralixibat had significant reductions in bile acids and pruritus compared to placebo, as well as reduction in xanthomas and accelerated growth long-term. In a [Phase 2 PFIC study](#), a genetically defined subset of BSEP deficient (PFIC2), patients responded to maralixibat with an increase in transplant-free survival. The U.S. Food and Drug Administration has granted maralixibat Breakthrough Therapy designation for the treatment of pruritus associated with ALGS in patients one year of age and older and for PFIC2. Maralixibat was generally well-tolerated throughout the studies. The most frequent treatment-related adverse events were diarrhea and abdominal pain. Maralixibat has been studied extensively and its safety database represents the largest database for an ASBT inhibitor.

Until maralixibat is approved and available for prescribing, the medication is available to patients with ALGS through Mirum's expanded access program. For more information, please visit [ALGSEAP.com](#). For further information about maralixibat's ongoing studies in pediatric liver disease, please visit the study websites: [Phase 3 MARCH study](#) for PFIC and [Phase 2b EMBARK study](#) for biliary atresia.

About Volixibat

Volixibat is an oral, minimally absorbed agent designed to selectively inhibit the apical sodium dependent bile acid transporter (ASBT). Volixibat may offer a novel approach in the treatment of adult cholestatic diseases by blocking the recycling of bile acids, through inhibition of ASBT, 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 ASBT inhibition, in addition to decreases in LDL cholesterol and increases in 7 α C4 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](#)) and intrahepatic cholestasis of pregnancy ([OHANA study](#)). Mirum plans to initiate a Phase 2b study for primary biliary cholangitis later this year.

About Mirum Pharmaceuticals

Mirum Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of a late-stage pipeline of novel therapies for debilitating liver diseases. Mirum's lead product candidate, maralixibat, is an investigational oral drug in development for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. Mirum has submitted an NDA for maralixibat in the treatment of cholestatic pruritus in patients with ALGS. The NDA has been accepted for priority review by the FDA with a PDUFA action date of September 29, 2021. Additionally, Mirum's marketing authorization application for the treatment of pediatric patients with PFIC2 has been accepted for review (validated) by the European Medicines Agency. Mirum is also developing volixibat, also an oral ASBT-inhibitor, in primary sclerosing cholangitis, intrahepatic cholestasis of pregnancy, and primary biliary cholangitis. For more information, visit [MirumPharma.com](#).

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

Follow Mirum on [Twitter](#), [Facebook](#), [LinkedIn](#) and [Instagram](#).

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 potential regulatory approval of maralixibat as well as the benefits and expected market opportunity for maralixibat. 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,” “could,” “would,” “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 Statement of Operations Data

(in thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2021	2020	2021	2020
License revenue	\$ 11,000	\$ -	\$ 11,000	\$ -
Operating expenses:				
Research and development	35,048	18,555	73,182	35,895
General and administrative	13,353	5,042	22,832	9,734
Total operating expenses (1)	48,401	23,597	96,014	45,629
Loss from operations	(37,401)	(23,597)	(85,014)	(45,629)
Interest income	80	405	229	1,154
Interest expense	(4,776)	-	(8,157)	
Change in fair value of derivative liability	(1,272)	-	(938)	
Other expense, net	(514)	(56)	(530)	(79)
Net loss before provision for income taxes	(43,883)	(23,248)	(94,410)	(44,554)
Provision for income taxes	11	3	16	7
Net loss	\$ (43,894)	\$ (23,251)	\$ (94,426)	\$ (44,561)

Net loss per share, basic and diluted	\$ (1.45)	\$ (0.93)	\$ (3.13)	\$ (1.79)
Weighted-average shares of common stock outstanding, basic and diluted	30,274,749		25,056,123		30,190,352		24,880,387	

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

Research and development	\$ 2,015	\$ 1,260	\$ 4,758	\$ 2,301
General and administrative	2,808	1,714	5,350	3,246
Total stock-based compensation	\$ 4,823	\$ 2,974	\$ 10,108	\$ 5,547

Mirum Pharmaceuticals, Inc.

Selected Condensed Consolidated Balance Sheet Data

(in thousands)

	June 30,	December 31,
	2021	2020
	(Unaudited)	
Cash, cash equivalents and short-term investments	\$ 238,841	\$ 231,820
Working capital	199,338	217,888
Total assets	247,668	240,864
Accumulated deficit	(267,597)	(173,171)
Total stockholders' equity	95,737	172,095

View source version on [businesswire.com](https://www.businesswire.com/news/home/20210805005953/en/): <https://www.businesswire.com/news/home/20210805005953/en/>

Investor Contacts:

Ian Clements, Ph.D.

ir@mirumpharma.com

Sam Martin

Argot Partners

ir@mirumpharma.com

Media Contact:
Erin Murphy
media@mirumpharma.com

Source: Mirum Pharmaceuticals, Inc.