



## Mirum Pharmaceuticals Provides Third Quarter 2020 Financial Results and Business Update, and Announces Virtual Investor Day

November 12, 2020

- Initiated rolling NDA submission and launched Expanded Access Program for maralixibat in Alagille syndrome.
- Presented five-year transplant-free survival data for patients with PFIC2 at Digital International Liver Congress (EASL).
- European Marketing Authorization Application submission for maralixibat in PFIC2, planned by year-end 2020.
- Cash, cash equivalents and investments of \$133.7 million.

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 12, 2020-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today announced financial results and a corporate update for the quarter ended September 30, 2020.

"This quarter marked several milestones toward providing better treatment options for Alagille syndrome and PFIC, with the initiation of our rolling NDA submission, the launch of an expanded access program, and presentation of five-year transplant free survival data in PFIC2," said Chris Peetz, president and chief executive officer of Mirum. "Looking forward to next year, we are planning for the U.S. launch of maralixibat in Alagille syndrome and the expansion of our programs, with upcoming study starts in biliary atresia, primary sclerosing cholangitis and intrahepatic cholestasis of pregnancy, all settings with high disease burden and no currently approved therapies."

### Key Operational Highlights

- [Presented](#) five-year transplant-free survival data for patients with PFIC2 at Digital International Liver Congress (EASL).
- [Initiated](#) rolling submission of New Drug Application (NDA) to U.S. Food and Drug Administration (FDA) for maralixibat for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS).
- Launched maralixibat Expanded Access Program (EAP) for the treatment of cholestatic pruritus associated with ALGS in [United States, Canada](#), Australia, and 10 countries in [Europe](#).
- [Announced](#) partnership with EVERSANA™ to support the planned launch and commercialization of maralixibat in ALGS in the United States, if approved.
- Received Orphan Drug Designation from the U.S. FDA for maralixibat in biliary atresia; Phase 2 study initiation planned for the first quarter of 2021.
- Received rare pediatric disease designation for maralixibat for the treatment of PFIC.

### Third Quarter 2020 Financial Results

- Total operating expenses for the quarter ended September 30, 2020 were \$21.7 million, compared to \$15.9 million for the third quarter of 2019.
  - Research and development expenses were \$16.0 million, compared to \$12.2 million for the comparable prior-year period. This increase was primarily due to increased personnel related expenses, manufacturing activities to support Mirum's NDA, and higher consulting expenses.
  - General and administrative expenses were \$5.7 million, compared to \$3.7 million for the comparable prior-year period. The increase was primarily due to personnel and other compensation related expenses.
- For the quarter ended September 30, 2020, Mirum reported a net loss of \$21.5 million, or \$0.86 per share, compared with a net loss of \$15.1 million, or \$0.84 per share for the same period in 2019.
- As of September 30, 2020, Mirum had cash, cash equivalents and investments of \$133.7 million.

### Upcoming Anticipated Milestones

- *Corporate*
  - Data from the maralixibat and volixibat studies, including long-term maralixibat data (up to 220 weeks) for the treatment of patients with ALGS, to be presented at The Liver Meeting Digital Experience™ (AASLD), November 13-17, 2020.
  - Hosting inaugural Investor Day on December 9, 2020. Additional details below.
- *Regulatory*
  - Complete rolling NDA submission to FDA for treatment of cholestatic pruritus in patients with ALGS in the first quarter 2021.

- Marketing Authorization Application submission to European regulators for maralixibat in the treatment of patients with PFIC2 by the end of 2020.
- *Pipeline*
  - *Maralixibat:*
    - Phase 2 study initiation planned for biliary atresia by first quarter 2021.
    - Completion of enrollment for MARCH PFIC study anticipated in second quarter 2021.
  - *Volixibat:*
    - Presenting dose-ranging data at AASLD to inform regimens for potentially pivotal studies in adult cholestasis.
    - Phase 2 study in primary sclerosing cholangitis planned for first quarter 2021.
    - Phase 2 study in intrahepatic cholestasis of pregnancy planned for first quarter 2021.

#### **Investor Day – December 9, 2020**

Mirum will be hosting its inaugural Investor Day to highlight Mirum's pipeline progress and commercial plans to bring potentially transformational new treatments to patients with cholestatic liver diseases. The virtual event will take place on December 9, 2020 at 11:00 a.m. ET. Additional information will be provided closer to the event date.

#### **AASLD – The Liver Meeting Digital Experience™ 2020**

New data from maralixibat and volixibat studies will be presented at The Liver Meeting Digital Experience, the annual meeting of the American Association for the Study of Liver Diseases, taking place November 13-17, 2020. Featured presentations to include the following abstracts:

##### ***Late-breaker Oral Presentation***

#### **L05: Preliminary Analysis of ITCH and IMAGINE II – Outcome of long-term administration of maralixibat in children with Alagille syndrome**

- Presented by Benjamin Shneider, M.D. on November 15, 2020 during the 5:30-7:00 p.m. ET session. View the [abstract](#).

##### ***Poster Presentations***

**Abstract #1221: A Phase 1 dose-ranging study assessing fecal bile acid excretion by volixibat, an apical sodium-dependent bile acid transporter inhibitor, and coadministration with loperamide**

**Abstract #341: Pruritus intensity is associated with cholestasis biomarkers and quality of life measures after maralixibat treatment in children with Alagille syndrome**

**Abstract #1792: Natural variability of pruritus in Alagille syndrome; an analysis from the ICONIC study utilizing the Itch Reported Outcome Observer (ItchRO[Obs]) tool**

All posters will be available at the start of the congress on November 13, 2020 and available throughout the duration of the meeting. Abstracts are available via [Hepatology](#) on the AASLD website.

##### **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, 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. In a [Phase 2 PFIC study](#), a genetically defined subset of BSEP (bile salt export pump) deficient (PFIC2), patients responded to maralixibat. The FDA has granted maralixibat Breakthrough Therapy designation for 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 adverse events were diarrhea and abdominal pain. For more information about the Maralixibat Expanded Access Program please visit [ALGSEAP.com](#). For more information about the Phase 3 study for maralixibat in pediatric patients with PFIC, visit [PFICtrial.com](#).

##### **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. The company's lead product candidate, maralixibat, is an investigational oral drug in development for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. The company is also developing volixibat, also an oral ASBT-inhibitor, in primary sclerosing cholangitis and intrahepatic cholestasis of pregnancy. For more information, visit [MirumPharma.com](#). Follow Mirum on [Twitter](#), [Facebook](#) and [LinkedIn](#).

##### **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 results, conduct, progress and timing of Mirum's ongoing and planned studies for maralixibat and volixibat, the regulatory approval path for maralixibat and volixibat, the strength of Mirum's balance sheet and the adequacy of cash, cash equivalents and investments on hand, the impacts of the COVID-19 pandemic, and commercial readiness activities. 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 "plans," "will," "anticipates," "goal," "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		Nine Months Ended	
	September 30,		September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	\$ 15,984	\$ 12,159	\$ 51,879	\$ 28,611
General and administrative	5,732	3,708	15,466	7,474
Total operating expenses <sup>(1)</sup>	21,716	15,867	67,345	36,085
Loss from operations	(21,716 )	(15,867 )	(67,345 )	(36,085 )
Interest income	237	785	1,391	1,485
Other income (expense), net	(30 )	(5 )	(109 )	(1 )
Net loss before provision for income taxes	(21,509 )	(15,087 )	(66,063 )	(34,601 )
Provision for (benefit from) income taxes	(3 )	-	4	-
Net loss	\$ (21,506 )	\$ (15,087 )	\$ (66,067 )	\$ (34,601 )
Net loss per share, basic and diluted	\$ (0.86 )	\$ (0.84 )	\$ (2.65 )	\$ (4.47 )
Weighted-average shares of common stock outstanding, basic and diluted	25,132,916	17,996,065	24,965,178	7,745,241

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

Research and development	\$ 1,361	\$ 830	\$ 3,662	\$ 1,539
General and administrative	2,067	1,314	5,313	2,464

Total stock-based compensation	\$ 3,428	\$ 2,144	\$ 8,975	\$ 4,003
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**Mirum Pharmaceuticals, Inc.**

**Selected Condensed Consolidated Balance Sheet Data**

(in thousands)

	<b>September 30, December 31,</b>	
	<b>2020</b>	<b>2019</b>
	<b>(Unaudited)</b>	
Cash, cash equivalents and investments	\$ 133,749	\$ 139,952
Working capital	119,359	106,287
Total assets	141,865	146,712
Accumulated deficit	(135,968 )	(69,901 )
Total stockholders' equity	120,255	130,349

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Investor Contact:  
 Ian Clements, Ph.D.  
[ir@mirumpharma.com](mailto:ir@mirumpharma.com)

Media Contact:  
 Erin Murphy  
[media@mirumpharma.com](mailto:media@mirumpharma.com)

Source: Mirum Pharmaceuticals, Inc.