



Mirum Pharmaceuticals Reports First Quarter 2020 Financial Results and Provides Business Updates

May 7, 2020

- On track to begin rolling submission of NDA for maralixibat in Alagille Syndrome (ALGS) in third quarter 2020
- Cash, cash equivalents and investments balance of \$166.1 million

FOSTER CITY, Calif.--(BUSINESS WIRE)--May 7, 2020-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today reported financial results and provided business updates for the quarter ended March 31, 2020.

"Despite the COVID-19 pandemic, we remain on track to begin the rolling submission of our new drug application to the U.S. Food and Drug Administration in the third quarter of this year with the goal of launching maralixibat for patients with Alagille syndrome in the second half of 2021," said Chris Peetz, president and chief executive officer at Mirum. "Further, we are exploring the potential to accelerate our regulatory filings for maralixibat in PFIC2 and will be discussing the Phase 2 INDIGO study 5-year outcomes versus a natural history cohort with regulators this year."

First Quarter 2020 Highlights

- Bolstered balance sheet with \$48.0 million gross proceeds from a follow on public offering in January 2020.
- As of March 31, 2020, Mirum had cash, cash equivalents and investments of \$166.1 million; projected to fund current operations through the anticipated FDA approval of maralixibat in Alagille syndrome (ALGS) in the second half of 2021.
- Completed Phase 1 dose-ranging and drug-drug interaction study for volixibat to inform dose regimens for planned registrational program.

Upcoming Anticipated Milestones

- Initiate rolling submission of NDA for maralixibat in ALGS in the third quarter of 2020.
- Presentations highlighting data from maralixibat and volixibat studies planned for upcoming medical congresses including European Association for the Study of the Liver and the American Association for the Study of Liver Diseases.
- Initiate ALGS expanded access program in the United States and Canada in the fourth quarter of 2020.
- Discuss with regulators the suitability of maralixibat INDIGO study 5-year outcomes data in PFIC2 patients compared to natural history for potential regulatory submission.
- US launch for maralixibat in ALGS remains on track for 2021.

COVID-19 Pandemic Impact

The COVID-19 pandemic has had a significant economic and social impact across the globe, presenting challenges to maintaining business continuity. In order to reduce the risk of spreading the COVID-19 virus, the company has mandated that all employees work from home. At the same time, Mirum is working diligently to ensure the advancement of all of its clinical development programs in the safest manner possible. However, due to health and safety concerns for patients, their caregivers, and healthcare personnel, there have been, and may continue to be delays in the advancement of some of the company's programs, including the completion of enrollment for the Phase 3 MARCH study in PFIC2 patients. Mirum is unable to predict with confidence the duration of such delays. As such, Mirum is withdrawing previous guidance regarding the timing of the initiation and enrollment of the ongoing and planned maralixibat and volixibat studies. Mirum's planned timeline for submission of its NDA for maralixibat in ALGS has not been impacted. Mirum continues to collect data in the INDIGO study, in which PFIC2 patients have been treated with maralixibat for more than five years.

During this time, ensuring that patients have access to treatment is paramount. Mirum is working closely with the study sites to develop methods in which patients can safely continue on treatment, including adopting new protocols to have study medication shipped to patients' homes and to conduct virtual check-ins, as opposed to attending standard hospital visits, providing a safer alternative for all persons involved in the program.

About Maralixibat

Maralixibat is a novel, minimally-absorbed, orally administered investigational drug being evaluated in several rare cholestatic liver diseases for pediatric populations. 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 1,500 individuals have received maralixibat, including more than 100 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 Alagille syndrome 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, abdominal pain, and vomiting. For more information about the Phase 3 study for maralixibat in pediatric patients with PFIC, visit [PFICtrial.com](#). For more information about the North American Expanded Access Program please visit

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) and progressive familial intrahepatic cholestasis (PFIC). 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 clinical trials for maralixibat and volixibat, the regulatory approval path for maralixibat, the strength of Mirum's balance sheet and the adequacy of cash, cash equivalents and investments on hand, and the impacts of COVID-19, including, but not limited to, delays or difficulties in enrolling patients in its ongoing and planned clinical trials, delays or difficulties in clinical site initiation, including difficulties in recruiting clinical site investigators and clinical site staff, delays in clinical sites receiving the supplies and materials needed to conduct its clinical trials, including interruption in global shipping that may affect the transport of clinical trial materials, and effects of the executive order, the shelter-in-place order and Mirum's work-from-home policies and related effects on productivity. 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," "believes," "anticipates," "expects," "intends," "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. In addition, the COVID-19 pandemic continues to rapidly evolve and actual results are highly uncertain and cannot be predicted with confidence. 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 March 31, 2020	Three Months Ended March 31, 2019
Operating expenses:		
Research and development	\$ 17,340	\$ 4,863
General and administrative	4,692	1,321
Total operating expenses (1)	22,032	6,184
Loss from operations	(22,032)	(6,184)
Interest income	749	232
Other income (expense), net	(23)	(5)
Net loss before provision for income taxes	(21,306)	(5,957)
Provision for income taxes	4	-
Net loss	\$ (21,310)	\$ (5,957)
Net loss per share, basic and diluted	\$ (0.86)	\$ (2.37)

Weighted-average shares of common stock outstanding, basic and diluted	24,704,651	2,517,743
--	------------	-----------

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

Research and development	\$ 1,041	\$ 71
General and administrative	1,532	147
Total stock-based compensation	\$ 2,573	\$ 218

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

	March 31,	December 31,
	2020	2019
	(Unaudited)	
Cash, cash equivalents and investments	\$ 166,061	\$ 139,952
Working capital	148,888	106,287
Total assets	172,803	146,712
Accumulated deficit	(91,211)	(69,901)
Total stockholders' equity	156,118	130,349

View source version on [businesswire.com](https://www.businesswire.com/news/home/20200507005852/en/): <https://www.businesswire.com/news/home/20200507005852/en/>

Media Contact:
Erin Murphy
media@mirumpharma.com

Investor Contact:
Ian Clements, Ph.D.
ir@mirumpharma.com

Source: Mirum Pharmaceuticals, Inc.