



Mirum Pharmaceuticals Reports Fourth Quarter and Full Year 2019 Financial Results and Provides Corporate Update

March 12, 2020

New Drug Application Rolling Submission for Alagille Syndrome Planned to Begin in Third Quarter 2020

Cash, Cash Equivalents and Investments Balance of \$140.0 million before gross proceeds of \$48.0 million from a follow-on public offering in January 2020

On Track for PFIC Phase 3 Top-Line Data and Long-term Outcomes Natural History Comparisons by Year End

FOSTER CITY, Calif.--(BUSINESS WIRE)-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today reported financial and business results for the quarter and year ended December 31, 2019.

"2019 was a year of ground-breaking achievements for Mirum," said Chris Peetz, president and chief executive officer of Mirum. "We established Mirum as a leading company in cholestatic liver diseases and have positioned ourselves to deliver our first NDA submission, planned to begin in the third quarter of this year. Maralixibat has potential to alter the course of disease in pediatric cholestasis and we are excited to be one step closer to delivering medicines to children with debilitating liver diseases. With the additional capital raised in January 2020 we are well positioned to grow our pipeline including executing on the upcoming NDA, maralixibat launch preparation, completing our ongoing studies and launching potentially registrational studies for volixibat."

Key Operational Highlights in Fourth Quarter 2019

- Successful completion of a pre-NDA meeting with the Food and Drug Administration (FDA) for maralixibat with alignment on submission of rolling NDA starting Q3 2020.
- Breakthrough Therapy Designation granted by the FDA for maralixibat for the treatment of pruritus associated with Alagille syndrome in patients 1 year of age and older.
- Rare Pediatric Disease Designation granted by the FDA for Alagille syndrome. If maralixibat's NDA is approved prior to October 1, 2022, the company may be eligible to receive a priority review voucher, which can be redeemed to obtain priority review for any subsequent marketing application and may be sold or transferred.
- Presented data demonstrating the long-term durability of maralixibat in children with cholestatic liver disease. At the Liver Meeting in November 2019, data were presented showing statistically and clinically significant effects on pruritus, xanthomas and growth maintained for up to four years of treatment in Alagille syndrome and durable multi-parameter treatment response and correlation of response with increased bile acid synthesis in progressive familial intrahepatic cholestasis.

Fourth Quarter 2019 Financial Results

- For the quarter ended December 31, 2019 Mirum reported a net loss of \$18.0 million.
- Total operating expenses for the quarter ended December 31, 2019 were \$18.7 million.
 - Research and development expenses for the quarter ended December 31, 2019 were \$14.4 million.
 - General and administrative expenses for the quarter ended December 31, 2019 were \$4.3 million.

As of December 31, 2019 Mirum had cash, cash equivalents and investments of \$140.0 million, before gross proceeds of \$48.0 million from Mirum's follow-on public offering of common stock in January 2020.

Upcoming Milestones and Expectations

- Announce results from Phase 1 study of volixibat in the second quarter of 2020, and subsequently select dose for primary sclerosing cholangitis and intrahepatic cholestasis of pregnancy studies.
- Complete enrollment of the Phase 3 MARCH clinical trial investigating the safety and efficacy of maralixibat in patients with PFIC2 in the second quarter of 2020 and announce topline results by the end of 2020.
- Initiate Alagille syndrome NDA rolling submission in the third quarter of 2020.
- Initiate Alagille syndrome expanded access program in the fourth quarter of 2020.
- Initiate maralixibat study in biliary atresia initiation by year end.
- Initiate potentially registrational study of volixibat in primary sclerosing cholangitis in fourth quarter of 2020.

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 currently enrolling Phase 3 study maralixibat in pediatric patients with PFIC, visit [PFICtrial.com](#). For more information about the North American Expanded Access Program please visit [ALGSEAP.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) 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 clinical trials for maralixibat and volixibat, and the regulatory approval path 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 "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, 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 per share amounts)

	Three Months Ended December 31, 2019 (Unaudited)	Three Months Ended December 31, 2018 (Unaudited)	Year Ended December 31, 2019	Period from May 2, 2018 to December 31, 2018
Operating expenses:				
Research and development	\$ 14,380	\$ 2,328	\$ 42,991	\$ 2,331
In process research and development	-	14,490	-	14,490
General and administrative	4,278	513	11,752	585
Total operating expenses (1)	18,658	17,331	54,743	17,406
Loss from operations	(18,658)	(17,331)	(54,743)	(17,406)
Interest income	747	72	\$ 2,232	72
Other income (expense), net	(20)	(12)	\$ (21)	(14)
Net loss before provision for income taxes	(17,931)	(17,271)	(52,532)	(17,348)
Provision for income taxes	21	-	21	-

Net loss	\$ (17,952) \$ (17,271) \$ (52,553) \$ (17,348)
Net loss per common share, basic and diluted	\$ (0.79) \$ (9.10) \$ (4.58) \$ (19.29)
Weighted-average shares of common stock outstanding, basic and diluted	22,587,752	1,898,515	11,486,367	899,331	

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

Research and development	\$ 820	\$ 8	\$ 2,359	\$ 8
General and administrative	1,247	26	3,711	26
Total stock-based compensation	\$ 2,067	\$ 34	\$ 6,070	\$ 34

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

	December 31,	December 31,
	2019	2018
Cash, cash equivalents and investments	\$ 139,952	\$ 51,963
Working capital	106,287	49,526
Total assets	146,712	51,975
Accumulated deficit	(69,901) (17,348
Total stockholders' equity (deficit)	130,349	(17,313

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Source: Mirum Pharmaceuticals, Inc.