



Mirum Pharmaceuticals Reports Third Quarter Financial Results and Provides Corporate Update

November 6, 2019

Breakthrough Therapy Designation for Maralixibat for the Treatment of Pruritus Associated with Alagille Syndrome Granted

Cash, Cash Equivalents and Investments Balance of \$153.6 million

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 6, 2019-- 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 for the quarter ended September 30, 2019, and provided an update on its clinical programs.

"The first nine months of 2019 have seen great progress toward bringing maralixibat to market for Alagille syndrome and progressive familial intrahepatic cholestasis," said Chris Peetz, president and chief executive officer of Mirum. "The granting of breakthrough designation by the Food & Drug Administration and an oral late breaking presentation at the Liver Meeting are testimony of the tremendous potential of maralixibat for Alagille syndrome. The next 12 months are poised to be a transformational year as we prepare for commercialization and complete the MARCH-PFIC Phase 3 study."

Key Operational Highlights

- Breakthrough Therapy Designation granted by the U.S. Food and Drug Administration (FDA) for maralixibat for the treatment of pruritus associated with Alagille syndrome (ALGS) in patients 1 year of age and older.
- Announced [key data presentations](#) at American Association for the Study of Liver Diseases (AASLD), continuing to demonstrate the potential of maralixibat to durably improve clinical outcomes for children with progressive familial intrahepatic cholestasis (PFIC) Type 2 and ALGS.
- Launched Phase 3 MARCH-PFIC clinical trial. In July 2019, Mirum [enrolled the first patient](#) in the Phase 3 [MARCH-PFIC](#) clinical trial of its lead drug candidate, maralixibat, in pediatric patients with PFIC.
- In July 2019, Mirum [completed its initial public offering](#) (IPO) of 5,000,000 shares of common stock at an offering price of \$15.00 per share. Proceeds from the IPO totaled approximately \$67.2 million, net of underwriting discounts, commissions and offering expenses.

Third Quarter 2019 Financial Results

- For the quarter ended September 30, 2019, Mirum reported a net loss of \$15.1 million.
- Total operating expenses for the quarter ended September 30, 2019, were \$15.9 million.
 - Research and development expenses for the third quarter of 2019 were \$12.2 million.
 - General and administrative expenses during the quarter ended September 30, 2019, were \$3.7 million.

As of September 30, 2019, Mirum had cash, cash equivalents and investments of \$153.6 million.

Upcoming Milestones and Expectations

- Present key data sets at the AASLD meeting in November 2019.
- Meet with the FDA in the fourth quarter of 2019 to continue discussions regarding the adequacy of Mirum's Phase 2b data to support an NDA submission for maralixibat for pruritus associated with ALGS. Mirum anticipates providing an update in January 2020.
- Complete enrollment of the Phase 3 MARCH-PFIC clinical trial investigating the safety and efficacy of maralixibat in patients with PFIC2 by mid-2020 and announce topline results by the end 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, which results 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 a Phase 2 PFIC study, a genetically defined subset of BSEP (bile salt export pump) deficient, or PFIC2, patients responded to maralixibat, which led to maralixibat receiving Breakthrough Therapy designation from the U.S. Food and Drug Administration for PFIC2. In a Phase 2b ALGS study, patients taking maralixibat had significant reductions in bile acids and pruritus compared to placebo. Maralixibat was generally well-tolerated throughout the studies. The most frequent adverse events were diarrhea, abdominal pain and vomiting.

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 progressive familial intrahepatic cholestasis (PFIC) and Alagille syndrome (ALGS). 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, 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. 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.

Selected Consolidated Statement of Operations Data (in thousands, except per share amounts)

	Three Months Ended		Nine Months Ended		Period from
	September 30,		September 30,		May 2, 2018 to
	2019	2018	2019	2018	September 30, 2018
Operating expenses:					
Research and development	12,159	3	\$ 28,611		3
General and administrative	3,708	62	\$ 7,474		72
Total operating expenses	15,867	65	36,085		75
Loss from operations	(15,867)	(65)	(36,085)		(75)
Interest income (expense)	785	(2)	\$ 1,485		(2)
Other income (expense), net	(5)	-	\$ (1)		-
Net loss	\$ (15,087)	\$ (67)	\$ (34,601)		\$ (77)
Net loss per common share, basic and diluted	\$ (0.84)	\$ (0.14)	\$ (4.47)		\$ (0.26)
Weighted-average common shares outstanding, basic and diluted	17,996,065	476,905	7,745,241		290,570

Mirum Pharmaceuticals, Inc.

Selected Consolidated Balance Sheet Data
(in thousands)

	September 30, December 31,	
	2019	2018
Cash, cash equivalents and investments \$	153,559	\$ 51,963
Working capital	118,408	49,526
Total assets	158,273	51,975
Accumulated deficit	(51,949)	(17,348)
Total stockholders' equity (deficit)	146,203	(17,313)

View source version on businesswire.com: <https://www.businesswire.com/news/home/20191106005841/en/>

Source: Mirum Pharmaceuticals, Inc.

Media Contact:

Heidi Chokeir, Ph.D.
Canale Communications
619-203-5391
heidi@canalecomm.com

Investor Contact:

Ian Clements, Ph.D.
650-667-4085
ir@mirumpharma.com