Mirum Pharmaceuticals Reports Second Quarter Financial Results and Provides Corporate Update

August 28, 2019

FOSTER CITY, Calif.--(BUSINESS WIRE)--Aug. 28, 2019-- Mirum Pharmaceuticals, Inc. (NASDAQ: MIRM), a biopharmaceutical company focused on the development and commercialization of a late-stage pipeline of novel therapies for debilitating liver diseases, today reported financial results for the quarter ended June 30, 2019 and provided an update on its clinical programs.

“With the advancement of maralixibat into Phase 3, the expansion of the team and the successful completion of our initial public offering, 2019 has been a notable year for Mirum,” said Chris Peetz, president and chief executive officer of Mirum. “We are excited to have strengthened our capital position as we continue to build Mirum into a leading innovator in rare liver disease. We look forward to further advancing maralixibat and achieving our goal of offering children with cholestasis a new treatment option that targets bile acid overload, a primary driver of liver damage and pruritus in these progressive diseases.”

Key Operational Highlights

- **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 progressive familial intrahepatic cholestasis (PFIC).
- **Completed initial public offering.** 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 $67.3 million, net of underwriting discounts, commissions and offering expenses.
- **Presented New Data.** In April 2019, Mirum presented Phase 2 clinical data at The International Liver Congress™ 2019, demonstrating the potential of maralixibat to durably improve clinical outcomes for children with PFIC Type 2 and Alagille syndrome (ALGS).
- **Progressed Alagille Syndrome Regulatory Pathway.** In May 2019, Mirum held an end-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA) to discuss the adequacy of its Phase 2b data to support a New Drug Application (NDA) submission for maralixibat for pruritus associated with ALGS.

Second Quarter 2019 Financial Results

- For the quarter ended June 30, 2019, Mirum reported a net loss of $13.6 million.
- Total operating expenses for the quarter ended June 30, 2019 were $14.0 million.
    - Research and development expenses for the second quarter of 2019 were $11.6 million.
    - General and administrative expenses during the quarter ended June 30, 2019 were $2.4 million.

As of June 30, 2019, Mirum had cash, cash equivalents and investments of $100.3 million, exclusive of the net proceeds of approximately $67.3 million from the IPO.

Upcoming Milestones and Expectations

- 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.
- Present key data sets at the American Association for the Study of Liver Diseases meeting in November 2019.
- 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 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 amount)

	Three months Ended June 30, 2019 (unaudited)	Six months Ended June 30, 2019 (unaudited)
Operating expenses		
Research and development	$ 11,589	$ 16,452
General and administrative	2,445	3,766
Total operating expenses	14,034	20,218
Loss from operations	(14,034)	(20,218)
Other income		
Interest income	468	700
Other income, net	9	4
Net loss	$ (13,557)	$ (19,514)
Net loss per share, basic and diluted	$ (5.31)	$ (7.70)
Weighted-average common shares outstanding, basic and diluted	2,551,822	2,534,877

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

	June 30, 2019 (unaudited)	December 31, 2018

Cash, cash equivalents and investments	$ 100,300	$ 51,963
Working capital	73,750	49,526
Total assets	104,266	51,975
Series A redeemable convertible preferred stock	119,826	59,849
Redeemable common stock	6,990	6,990
Total stockholders' deficit	(34,940)	(17,313)

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Source: Mirum Pharmaceuticals, Inc.

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