



Mirum Pharmaceuticals Announces Second Quarter 2020 Financial Results and Provides Clinical Program Updates

August 6, 2020

- *Rolling NDA submission for maralixibat in Alagille syndrome (ALGS) to be initiated in third quarter 2020; planning for potential launch in second half of 2021.*
- *Submission of European marketing authorization application for maralixibat in PFIC2 planned for fourth quarter of 2020.*
- *Expanded Access Program for maralixibat in ALGS planned to open for registration in September 2020.*
- *Orphan Drug Designation received by European Medicines Agency for maralixibat in biliary atresia.*
- *Cash, cash equivalents and investments of \$149.3 million.*

FOSTER CITY, Calif.--(BUSINESS WIRE)--Aug. 6, 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 business updates for the quarter ended June 30, 2020.

"We made significant progress toward making maralixibat available as an alternative to liver transplantation in devastating pediatric cholestatic settings in the United States and Europe," said Chris Peetz, president and chief executive officer at Mirum. "In the United States, we are initiating a rolling NDA submission for maralixibat in ALGS and are planning to start an expanded access program broadening availability to this important medicine for eligible patients. By the end of this year, we intend to submit a marketing authorization application for maralixibat in PFIC2 in Europe, dramatically shifting our timeline for a potential launch in this region."

Second Quarter Highlights

- [Released](#) maralixibat data demonstrating five-year transplant-free survival for pediatric patients with PFIC2. Data will be presented at the upcoming Digital International Liver Congress, August 27-29, 2020.
- Completed pre-submission meeting with European regulators resulting in feedback enabling submission of a marketing authorization application (MAA) for maralixibat in the treatment of patients with PFIC2 in Europe in the fourth quarter of 2020.
- Investigational new drug (IND) application cleared by FDA for the initiation of a Phase 2 study evaluating volixibat in adult patients with pruritus associated with primary sclerosing cholangitis.
- Received orphan drug designation in Europe for maralixibat in the study of patients with biliary atresia; planning for a Phase 2 study is underway.
- As of June 30, 2020, Mirum had cash, cash equivalents and investments of \$149.3 million.

Upcoming Anticipated Program Milestones

- *Maralixibat in ALGS:*
 - Initiation of rolling new drug application (NDA) submission for maralixibat in ALGS on track for third quarter of 2020.
 - Expect to complete NDA submission in the first quarter of 2021.
 - Planning for launch in ALGS in the second half of 2021.
 - Maralixibat ALGS Expanded Access Program planned to open for registration in the U.S. and Canada in September 2020.
- *Maralixibat in PFIC:*
 - Submission of MAA to European regulators for maralixibat in the treatment of patients with PFIC2 on track for fourth quarter of 2020.
 - Enrollment ongoing in Phase 3 MARCH clinical trial in PFIC.
- *Maralixibat in biliary atresia:*
 - Phase 2 study initiation planned.
- *Volixibat:*
 - Planned expansion into adult patients with studies in primary sclerosing cholangitis and intrahepatic cholestasis of pregnancy.

Digital International Liver Congress™ 2020

New findings from maralixibat studies will be presented in a late-breaker and poster sessions at the Digital International Liver Congress 2020, August 27-29, 2020. Featured presentations to include the following abstracts:

Late-breaker Oral Presentation

August 29, 2020 – 1:45-2:00pm CET

Oral Presentation LB008: Serum bile acid control in long-term maralixibat-treated patients is associated with native liver survival in children with progressive familial intrahepatic cholestasis due to bile salt export pump deficiency.

Poster Presentations

August 28, 2020

Poster FRI-131: Psychometric evaluation of the adult itch reported outcome tool, a worst-itch numeric rating scale in adults with cholestatic liver disease.

August 28, 2020

Poster FRI-296: Differential expression of bile acid subspecies with maralixibat treatment in pruritus responders.

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, abdominal pain, and vomiting. For more information about the North American 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, as well as Mirum's Expanded Access Program for maralixibat, 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," "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. 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		Six Months Ended	
June 30,		June 30,	
2020	2019	2020	2019

Operating expenses:

Research and development	\$ 18,555	\$ 11,589	\$ 35,895	\$ 16,452
General and administrative	5,042	2,445	9,734	3,766
Total operating expenses ⁽¹⁾	23,597	14,034	45,629	20,218
Loss from operations	(23,597)	(14,034)	(45,629)	(20,218)
Interest income	405	468	1,154	700
Other income (expense), net	(56)	9	(79)	4
Net loss before provision for income taxes	(23,248)	(13,557)	(44,554)	(19,514)
Provision for income taxes	3	-	7	-
Net loss	\$(23,251)	\$(13,557)	\$(44,561)	\$(19,514)
Net loss per share, basic and diluted	\$(0.93)	\$(5.31)	\$(1.79)	\$(7.70)
Weighted-average shares of common stock outstanding, basic and diluted	25,056,123	2,551,822	24,880,387	2,534,877

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

Research and development	\$ 1,260	\$ 638	\$ 2,301	\$ 709
General and administrative	1,714	1,003	3,246	1,150
Total stock-based compensation	\$ 2,974	\$ 1,641	\$ 5,547	\$ 1,859

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

	June 30,	December 31,
	2020	2019
	(Unaudited)	
Cash, cash equivalents and investments	\$ 149,336	\$ 139,952
Working capital	135,495	106,287
Total assets	155,766	146,712
Accumulated deficit	(114,462)	(69,901)

Total stockholders' equity	136,208	130,349
----------------------------	---------	---------

View source version on [businesswire.com](https://www.businesswire.com/news/home/20200806005852/en/): <https://www.businesswire.com/news/home/20200806005852/en/>

Investor Contact:

Ian Clements, Ph.D.

ir@mirumpharma.com

Media Contact:

Erin Murphy

media@mirumpharma.com

Source: Mirum Pharmaceuticals, Inc.