Mirum Pharmaceuticals Reports First Quarter 2021 Financial Results and Provides Business Update

May 6, 2021

- NDA for maralixibat in Alagille syndrome (ALGS) granted priority review by U.S. FDA; PDUFA date is September 29, 2021
- Received additional $65.0 million in funding from Oberland Capital based on NDA acceptance
- Commercial team hired in anticipation of U.S. launch of maralixibat in ALGS
- Entered into two licensing agreements with Vivet Therapeutics and CANbridge Pharmaceuticals
- Conference call to provide business update today, May 6 at 1:30 p.m. PT/4:30 p.m. ET

FOSTER CITY, Calif.--(BUSINESS WIRE)--May 6, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), today reported financial results for the quarter ended March 31, 2021 and provided a business update.

“We have made significant progress toward launching maralixibat for Alagille syndrome. We have a Prescription Drug User Fee Act date of September 29, 2021, we have hired our sales, payer and field-based medical teams, and designed Mirum Access Plus, our patient services program,” said Chris Peetz, president and chief executive officer at Mirum. “We believe our strong balance sheet supports all of our current and planned clinical programs in multiple cholestatic indications and the addition of our gene therapy collaboration with Vivet.”

Recent Key Operational Highlights

- Received acceptance for filing and priority review of new drug application (NDA) for maralixibat in Alagille syndrome (ALGS) by the U.S. Food and Drug Administration (FDA).
- Announced exclusive worldwide licensing deal with Vivet Therapeutics for two gene therapy programs.
- Entered into exclusive licensing agreement for up to $120.0 million with CANbridge Pharmaceuticals for the development and commercialization of maralixibat in Greater China.
- Completed staffing of U.S. commercial organization through hiring of key positions including marketing, market access, and sales.
- Received $65.0 million additional funding from Oberland Capital in April 2021, related to NDA acceptance.

Financial Results

- Total operating expenses for the quarter ended March 31, 2021 were $47.6 million, compared to $22.0 million for the first quarter of 2020.
    - Research and development expenses for the first quarter ended March 31, 2021 were $38.1 million, compared to $17.3 million for the comparable prior-year period. The increase was primarily driven by milestone payments to Takeda and increased headcount costs.
    - General and administrative (G&A) expenses for the first quarter of 2021 were $9.5 million, compared to $4.7 million for the comparable prior-year period. G&A investment increased in the first quarter of 2021 versus the first quarter of 2020, primarily due to increased headcount costs and investments in launch preparation, including disease state awareness campaigns.
- For the quarter ended March 31, 2021, Mirum reported a net loss of $50.5 million, or $1.68 per share, compared with a net loss of $21.3 million, or $0.86 per share for the same period in 2020.
- As of March 31, 2021, Mirum had cash, cash equivalents, and short-term investments of $213.1 million.

Upcoming Anticipated Milestones

- Commercial
    - The FDA has issued a PDUFA date of September 29, 2021 and, if approved, Mirum plans to launch maralixibat for the treatment of cholestatic pruritus in patients with ALGS one year of age and older in the United States.
    - Mirum is planning to launch maralixibat for the treatment of PFIC2 in Europe in early 2022, if approved by the European Medicines Agency.
        - Mirum plans to file for ALGS as an additional indication in Europe following the PFIC2 approval, if received, based on results from the ICONIC study and six-year event-free survival analyses.
- Pipeline and New Data:
    - Maralixibat:
        - Topline data from the Phase 3 MARCH PFIC study expected in early 2022.
        - New data from maralixibat studies to be presented at upcoming medical congresses.
        - Continuing enrollment in EMBARK Phase 2b study of maralixibat in biliary atresia.

- Volixibat:
  - Continuing enrollment in Phase 2b OHANA study for intrahepatic cholestasis of pregnancy (ICP) and Phase 2b VISTAS study for primary sclerosing cholangitis (PSC), two potentially registrational studies. Interim analyses expected in 2022.
  - Phase 2b study in primary biliary cholangitis (PBC) planned for the second half of 2021.
- Vivet: VTX-803 abstracts accepted for presentation at the American Society of Gene and Cell Therapy Annual Meeting in May 2021.

Business Update Conference Call

Mirum will host a conference call today, May 6, 2021 at 1:30 p.m. PT/4:30 p.m. ET, to provide a review of the first quarter activities and to discuss business updates. Join the call using the following details:

Conference Call Details:

U.S. toll-free: 877-261-8990

International: 847-619-6441

Passcode: 50157000

You may also access the call via webcast by visiting the Events & Presentations section on Mirum's website. A replay of this webcast will be available for 30 days.

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 (ASBT), 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, as well as reduction in xanthomas and accelerated growth long-term. In a Phase 2 PFIC study, a genetically defined subset of BSEP deficient (PFIC2), patients responded to maralixibat with an increase in transplant-free survival. The U.S. Food and Drug Administration has granted maralixibat Breakthrough Therapy designation for the treatment of 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 treatment-related adverse events were diarrhea and abdominal pain. Until maralixibat is approved and available for prescribing, the medication is available to patients with ALGS through Mirum's expanded access program. For more information, please visit ALGSEAP.com. For more information about the Phase 3 study for maralixibat in pediatric patients with PFIC, visit PFICtrial.com.

About Volixibat

Volixibat is an oral, minimally absorbed agent designed to selectively inhibit the apical sodium dependent bile acid transporter (ASBT). Volixibat may offer a novel approach in the treatment of adult cholestatic diseases by blocking the recycling of bile acids, through inhibition of ASBT, thereby reducing bile acids systemically and in the liver. Phase 1 and Phase 2 studies of volixibat demonstrated on-target fecal bile acid excretion, a pharmacodynamic marker of ASBT inhibition, in addition to decreases in LDL cholesterol and increases in 7αC4 which are markers of bile acid synthesis. Volixibat has been evaluated in more than 400 individuals across multiple clinical trials. The most common adverse events reported were mild to moderate gastrointestinal events observed in the volixibat groups.

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. Mirum's lead product candidate, maralixibat, is an investigational oral drug in development for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. Mirum has submitted an NDA for maralixibat in the treatment of cholestatic pruritus in patients with ALGS. The NDA has been accepted for priority review by the FDA with a PDUFA action date of September 29, 2021. Additionally, Mirum's marketing authorization application for the treatment of pediatric patients with PFIC2 has been accepted for review (validated) by the European Medicines Agency. Mirum is also developing volixibat, also an oral ASBT-inhibitor, in primary sclerosing cholangitis, intrahepatic cholestasis of pregnancy, and primary biliary cholangitis. For more information, visit MirumPharma.com.

To augment its pipeline in cholestatic liver disease, Mirum has acquired the exclusive option to develop and commercialize two gene therapy programs, VTX-803 and VTX-802 for PFIC3 and PFIC2, respectively, from Vivet Therapeutics, following preclinical evaluation and IND-enabling studies.

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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, the regulatory approval path for maralixibat and volixibat, the strength of Mirum's balance sheet and the adequacy of cash, cash equivalents and short-term investments on hand, 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", "anticipates," "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 March 31, 2021	Three Months Ended March 31, 2020
Operating expenses:		
Research and development	$ 38,134	$ 17,340
General and administrative	9,479	4,692
Total operating expenses (1)	47,613	22,032
Loss from operations	(47,613)	(22,032)
Interest income	149	749
Interest expense	(3,381)	-
Change in fair value of derivative liability	334	-
Other expense, net	(16)	(23)
Net loss before provision for income taxes	(50,527)	(21,306)
Provision for income taxes	5	4
Net loss	$ (50,532)	$ (21,310)
Net loss per share, basic and diluted	$ (1.68)	$ (0.86)
Weighted-average shares of common stock outstanding, basic and diluted	30,105,017	24,704,651

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

Research and development	$ 2,743	$ 1,041
General and administrative	2,542	1,532

Total stock-based compensation	$ 5,285	$ 2,573

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

	March 31, 2021	December 31, 2020
	(Unaudited)	
Cash, cash equivalents and short-term investments	$ 213,113	$ 231,820
Working capital	182,733	217,888
Total assets	222,241	240,864
Accumulated deficit	(223,703)	(173,171)
Total stockholders' equity	133,883	172,095

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