# Mirum Pharmaceuticals Reports Fourth Quarter and Year-End 2020 Results and Provides Business Updates

March 9, 2021

- U.S. launch of maralixibat for Alagille syndrome (ALGS) planned for second half of 2021, if approved by FDA

- Phase 2b programs initiated in Primary Sclerosing Cholangitis, Intrahepatic Cholestasis of Pregnancy and Biliary Atresia

- Financial runway expected to support maralixibat launch in U.S. and Europe and pipeline expansion over the next three years

FOSTER CITY, Calif.--(BUSINESS WIRE)--Mar. 9, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), today announced financial results for the fourth quarter and year-end 2020 and provided business updates.

“In 2020, Mirum achieved several critical milestones, further advancing maralixibat for Alagille syndrome and PFIC2, and continuing to build value in our business by initiating the volixibat programs in adult cholestasis,” said Chris Peetz, president and chief executive officer at Mirum. “We move into 2021 poised to launch maralixibat for Alagille syndrome in the United States, if approved, and with a strong cash runway expected to support three years of growth and expansion into additional orphan liver diseases.”

**Key Operational Highlights and Business Updates**

- Completed rolling NDA submission to FDA for maralixibat for the treatment of pruritus in patients with Alagille syndrome (ALGS) one year of age and older in January 2021.
- Received validation (acceptance) of Mirum’s marketing authorization application (MAA) to the European Medicines Agency (EMA) for maralixibat for the treatment of patients with Progressive Familial Intrahepatic Cholestasis Type 2 (PFIC2).
- Announced new maralixibat data highlighting more than five years of durable improvements in pruritus and quality of life in children with ALGS treated with maralixibat during AASLD.
- Along with existing balance sheet, secured access to approximately $400 million in capital, with cash expected to support operations for the next three years including the planned launches of maralixibat and the advancement of Mirum’s development pipeline.
- Initiated Phase 2b VISTAS study evaluating volixibat in patients with primary sclerosing cholangitis, and the Phase 2b OHANA study evaluating volixibat in patients with intrahepatic cholestasis of pregnancy.
- Initiated EMBARK Phase 2b study evaluating maralixibat in children with biliary atresia.
- Launched “Unbearable ALGS” in January 2021, a disease education program intended to increase awareness of Alagille syndrome with the goal of improving time and rate of diagnosis.

**Financial Results**

- Total operating expenses for the quarter ended December 31, 2020 were $37.0 million, compared to $18.7 million for the fourth quarter of 2019. For the years ended December 31, 2020 and 2019, total operating expenses were $104.3 million and $54.7 million, respectively.
    - Research and development expenses for the fourth quarter were $29.7 million, compared to $14.4 million for the comparable prior-year period. For the years ended December 31, 2020 and 2019, research and development expenses were $81.6 million and $43.0 million, respectively. The increase in full year 2020 was primarily due to increased personnel related expenses, manufacturing activities to support Mirum’s NDA, and higher consulting expenses.
    - General and administrative expenses for the fourth quarter of 2020 were $7.2 million, compared to $4.3 million for the comparable prior-year period. For the years ended December 31, 2020 and 2019, general and administrative expenses were $22.7 million and $11.8 million, respectively. The increase was primarily due to personnel and other compensation-related expenses.
- For the quarter ended December 31, 2020, Mirum reported a net loss of $37.2 million, or $1.43 per share, compared with a net loss of $18.0 million, or $0.79 per share for the same period in 2019. For the year ended December 31, 2020, Mirum reported a net loss of $103.3 million, or $4.09 per share, compared to a net loss of $52.6 million, or $4.58 per share, for the same period in 2019.
- As of December 31, 2020, Mirum had cash, cash equivalents, and investments of $231.8 million.

**Upcoming Anticipated Milestones**

- Commercial
  - Anticipated launch of maralixibat for the treatment of cholestatic pruritus in patients with ALGS one year of age and older in second half of 2021, if approved by the FDA.
  - Launch of maralixibat for the treatment of PFIC2 in Europe planned for early 2022, if approved by the EMA.
- Pipeline
  - Maralixibat: Enrollment completion for Phase 3 MARCH PFIC study expected in the second quarter 2021.
  - Volixibat: Phase 2b study in primary biliary cholangitis planned for the second half of 2021.

**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 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. 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, another oral ASBT-inhibitor, in primary sclerosing cholangitis, intrahepatic cholestasis of pregnancy, and primary biliary cholangitis. For more information, visit MirumPharma.com.

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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 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", "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 per share amounts)**

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | 2020 | 2019 | 2020 | 2019 |
| | (Unaudited) | | | |
| Operating expenses: | | | | |

| | | | | |
|---|---|---|---|---|
| Research and development | $ 29,726 | $ 14,380 | $ 81,605 | $ 42,991 |
| General and administrative | 7,225 | 4,278 | 22,691 | 11,752 |
| Total operating expenses (1) | 36,951 | 18,658 | 104,296 | 54,743 |
| Loss from operations | (36,951) | (18,658) | (104,296) | (54,743) |
| Interest income | 168 | 747 | 1,559 | 2,232 |
| Interest expense | (335) | — | (335) | — |
| Other expense, net | (83) | (20) | (192) | (21) |
| Net loss before provision for income taxes | (37,201) | (17,931) | (103,264) | (52,532) |
| Provision for income taxes | 2 | 21 | 6 | 21 |
| Net Loss | $ (37,203) | $ (17,952) | $ (103,270) | $ (52,553) |
| Net loss per share, basic and diluted | $ (1.43) | $ (0.79) | $ (4.09) | $ (4.58) |
| Weighted-average shares of common stock outstanding, basic and diluted | 26,106,102 | 22,587,752 | 25,251,968 | 11,486,367 |

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

| | | | | |
|---|---|---|---|---|
| Research and development | $ 1,467 | $ 820 | $ 5,129 | $ 2,359 |
| General and administrative | 2,112 | 1,247 | 7,425 | 3,711 |
| Total stock-based compensation | $ 3,579 | $ 2,067 | $ 12,554 | $ 6,070 |

**Mirum Pharmaceuticals, Inc.**

**Selected Consolidated Balance Sheet Data**

**(in thousands)**

| | **December 31, 2020** | **December 31, 2019** |
|---|---|---|
| Cash, cash equivalents and investments | $ 231,820 | $ 139,952 |
| Working capital | 217,888 | 106,287 |
| Total assets | 240,864 | 146,712 |

| | | |
|---|---|---|
| Accumulated deficit | (173,171) | (69,901) |
| Total stockholders' equity | 172,095 | 130,349 |

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