Mirum Pharmaceuticals Provides Business Update and Highlights Key 2022 Corporate Milestones

January 11, 2022

- Unaudited estimated total LIVMARLI net product revenue of $3.0 million for fourth quarter 2021.

- Preliminary cash, cash equivalents, and short-term investments of $261.5 million as of December 31, 2021.

FOSTER CITY, Calif.--(BUSINESS WIRE)--Jan. 11, 2022-- Mirum Pharmaceuticals, Inc. (NASDAQ: MIRM) today provided a corporate update and highlighted key 2022 milestones in conjunction with its presentation at the 40th Annual J.P. Morgan Virtual Healthcare Conference. The webcast will be live today, Tuesday, January 11, at 1:30 p.m. ET/10:30 a.m. PT via the Investors page on the Company's website.

"We have had a strong first few months in market with LIVMARLI, the first and only FDA-approved medication for the treatment of cholestatic pruritus in patients one year of age and older with Alagille syndrome. With the excitement and early adoption of this new breakthrough therapy, we achieved preliminary fourth quarter 2021 net product revenue of approximately $3.0 million," said Chris Peetz, president and chief executive officer at Mirum. "The launch of LIVMARLI is only the beginning of Mirum's commitment to spearheading innovation for rare diseases worldwide. With a milestone-heavy year for our pipeline, including significant anticipated developments for our LIVMARLI and volixibat programs, we look forward to providing important updates in the coming months and fulfilling our commitment to those living with rare diseases."

Preliminary unaudited fourth quarter net product revenue*:

Based on preliminary unaudited financial information, Mirum expects total net product revenue from the sale of LIVMARLI for the fourth quarter of 2021 to be approximately $3.0 million. As of December 31, 2021 Mirum had preliminary unaudited cash, cash equivalents, and short-term investments of approximately $261.5 million.

Recent achievements and corporate updates:

- Alagille syndrome
  - Successful first quarter of LIVMARLI commercialization with approximately $3.0 million in net product revenue.
- PFIC
  - Recruitment completed for Phase 3 MARCH study in progressive familial intrahepatic cholestasis.
  - Expanded access program launched.
  - Decision not to exercise option for Vivet gene therapy programs resulting in R&D savings in 2022 and 2023.
- Corporate
  - Completed sale of priority review voucher for LIVMARLI for $110 million.

Anticipated milestones for Mirum include:

- LIVMARLI
  - Launch commercial early access programs in international markets for LIVMARLI in the first half of 2022.
  - Topline data from Phase 3 MARCH-PFIC study expected in the second half of 2022.
  - Alagille syndrome launch in Europe in the second half of the year, if approved by the European Medicines Agency.
  - Phase 2b EMBARK study for biliary atresia currently enrolling; topline data anticipated in 2023.
- Volixibat
  - Interim analyses expected for VISTAS for primary sclerosing cholangitis.
  - First interim analysis expected for OHANA for intrahepatic cholestasis of pregnancy.
  - Continued enrollment and country activation for Phase 2b VANTAGE study for primary biliary cholangitis.

About LIVMARLI™ (maralixibat) oral solution

LIVMARLI™ (maralixibat) oral solution is an orally administered, once-daily, ileal bile acid transporter (IBAT) inhibitor approved by the U.S. Food and Drug Administration for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) one year of age and older and is the only FDA-approved medication to treat cholestatic pruritus associated with Alagille syndrome. For more information, please visit LIVMARLI.com.

LIVMARLI is currently being evaluated in late-stage clinical studies in other rare cholestatic liver diseases including progressive familial intrahepatic cholestasis (PFIC) and biliary atresia. LIVMARLI has received Breakthrough Therapy designation for ALGS and PFIC type 2 and orphan designation for ALGS, PFIC and biliary atresia. To learn more about ongoing clinical trials with LIVMARLI, please visit Mirum's clinical trials section on the company's website.

IMPORTANT SAFETY INFORMATION

LIVMARLI can cause serious side effects, including:

Changes in liver tests. Changes in certain liver tests are common in patients with Alagille syndrome and can worsen during treatment with LIVMARLI. These changes may be a sign of liver injury and can be serious. Your healthcare provider should do blood tests before starting and during treatment to check your liver function. Tell your healthcare provider right away if you get any signs or symptoms of liver problems, including nausea or vomiting, skin or the white part of the eye turns yellow, dark or brown urine, pain on the right side of the stomach (abdomen) or loss of appetite.

Stomach and intestinal (gastrointestinal) problems. LIVMARLI can cause stomach and intestinal problems, including diarrhea, stomach pain, and vomiting during treatment. Tell your healthcare provider right away if you have any of these symptoms more often or more severely than normal for you.

A condition called **Fat Soluble Vitamin (FSV) Deficiency** caused by low levels of certain vitamins (vitamin A, D, E, and K) stored in body fat. FSV deficiency is common in patients with Alagille syndrome but may worsen during treatment. Your healthcare provider should do blood tests before starting and during treatment.

Other common side effects reported during treatment were bone fractures and gastrointestinal bleeding.

Prescribing information

*The Company's fourth quarter and full year 2021 financial results are preliminary and are subject to the completion of the Company's 2021 audit. Audited fourth quarter and full year 2021 financial results will be announced in early March.

About Mirum Pharmaceuticals, Inc.

Mirum Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to transforming the treatment of rare diseases. Mirum's approved medication is LIVMARLI™ (maralixibat) oral solution which is approved in theU.S. for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older.

Mirum's late-stage pipeline includes two investigational treatments for debilitating liver diseases affecting children and adults. Maralixibat (LIVMARLI), an oral ileal bile acid transporter (IBAT) inhibitor, is currently being evaluated in clinical trials for pediatric liver diseases and includes the MARCH Phase 3 study for progressive familial intrahepatic cholestasis (PFIC) and the EMBARK Phase 2b study for patients with biliary atresia. In addition, Mirum has an expanded access program open in Canada, Australia, the UK and several countries in Europe for eligible patients with Alagille syndrome.

Mirum has submitted a Marketing Authorization Application to the European Medicines Agency for maralixibat for the treatment of cholestatic liver disease in patients with Alagille syndrome.

Mirum's second investigational treatment, volixibat, also an oral IBAT inhibitor, is being evaluated in three potentially registrational studies including the OHANA Phase 2b study for pregnant women with intrahepatic cholestasis of pregnancy, VISTAS Phase 2b study for adults with primary sclerosing cholangitis, and the VANTAGE Phase 2b study for primary biliary cholangitis.

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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 anticipated launch and timing of the expanded access program for LIVMARLI in international markets, the results, conduct and progress of Mirum's ongoing and planned studies for its product candidates and the regulatory approval path for its product candidates, the strength of Mirum's balance sheet and the adequacy of cash and cash equivalents on hand, and continued commercial 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," "may," "expects," "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.

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