Mirum Pharmaceuticals Provides Corporate Update

January 12, 2021

- Completion of maralixibat rolling NDA targeted for Q1 2021

- Expecting first-patient-in for volixibat studies in ICP and PSC, and maralixibat study in biliary atresia in Q1 2021

- Launching volixibat primary biliary cholangitis program in H2 2021

- European commercial leader hired in anticipation of potential approval of maralixibat

FOSTER CITY, Calif.--(BUSINESS WIRE)--Jan. 12, 2021-- 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 provided a corporate update in advance of the company's presentation at the 39 th Annual J.P. Morgan Healthcare Conference.

"I am very excited about what 2021 holds for Mirum. In the first quarter alone, we expect to complete our rolling NDA submission for maralixibat, taking us one step closer to making this medicine available to patients with Alagille syndrome, and expect to initiate three randomized studies of maralixibat and volixibat in new indications," said Chris Peetz, president and chief executive officer at Mirum. "Today we are announcing key progress in maralixibat launch readiness and further expansion of the volixibat program into primary biliary cholangitis with an additional, potentially registrational, randomized study."

2021 Pipeline Highlights

- Expects completion of rolling new drug application (NDA) submission to the U.S. Food and Drug Administration (FDA) for maralixibat for the treatment of cholestatic pruritus associated with Alagille syndrome in the first quarter of 2021.

- Ongoing review of the marketing authorization application to the European Medicines Agency for maralixibat in progressive familial intrahepatic cholestasis, type 2 (PFIC2) in Europe.

- Anticipates randomizing patients into three clinical studies in the first quarter of 2021:

  - Phase 2a/2b study (OHANA) of volixibat for the treatment of intrahepatic cholestasis of pregnancy.
  - Phase 2b study (VISTAS) of volixibat for the treatment of pruritus associated with primary sclerosing cholangitis.
  - Phase 2 study (EMBARK) of maralixibat for the treatment of biliary atresia.

- Planning to initiate a Phase 2b study of volixibat for the treatment of pruritus associated with primary biliary cholangitis in the second half of 2021.

Corporate Highlights - Strategically Financed for Growth

- Cash, cash equivalents and investments of $231.8 million at end of 2020, including:

  - Gross proceeds of $75 million from follow-on public offering of common stock in the fourth quarter.
  - Gross proceeds of $60 million, including a $10 million equity investment, from the funding arrangement with Oberland Capital in the fourth quarter.

- Access to up to an additional $150 million from the Oberland Capital funding arrangement to support maralixibat potential launch and pipeline expansion.

- Maralixibat has rare pediatric disease designation for both the treatment of pruritus associated with Alagille syndrome and the treatment of PFIC2. The designation makes maralixibat eligible for a Rare Pediatric Disease Priority Review voucher upon approval of the therapy by the FDA.

Global Launch Readiness

Today, the company announced the appointment of Alexey Kutahov, MD as its general manager, EMEA, to lead its launch readiness activities for maralixibat in Europe. Dr. Kutahov will be responsible for all European activities related to market development and commercial launch strategies, with direct oversight of marketing, market access and sales. Dr. Kutahov joins Mirum with 20 years leading biopharmaceutical development and commercialization programs, having served most recently as general manager of Europe for Sarepta Therapeutics. Prior to Sarepta, Dr. Kutahov was head of market access innovation at Amgen. Before joining Amgen, he was head of central European market access at Novartis. Dr. Kutahov also held positions of increasing responsibility at Eli Lilly and Sandoz.

In connection with his employment, the Compensation Committee of Mirum's Board of Directors granted Dr. Kutahov an inducement award consisting of a non-qualified stock option to purchase 55,000 shares of common stock under Mirum's 2020 Inducement Plan. The Compensation Committee of Mirum's Board of Directors approved the award as an inducement material to Dr. Kutahov's employment in accordance with Nasdaq Listing Rule 5635(c)(4).

The stock option has an exercise price per share equal to $19.44 per share, Mirum's closing trading price on January 11, 2020, and will vest over four years, with 25% of the underlying shares vesting on the one-year anniversary of the vesting commencement date and the balance of the underlying shares vesting monthly thereafter over 36 months, subject to Dr. Kutahov's continued service relationship with Mirum through the applicable vesting dates. The award is subject to the terms and conditions of Mirum's 2020 Inducement Plan and the terms and conditions of an award agreement covering the grant.

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 initiated a rolling NDA submission for maralixibat in the treatment of cholestatic pruritus in patients with ALGS and expects to complete the submission in the first quarter of 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 and intrahepatic cholestasis of pregnancy. 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 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 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," "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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