Mirum Pharmaceuticals Announces European Medicines Agency Validation of the Marketing Authorization Application for Maralixibat in Patients with PFIC2

November 30, 2020

- Five-year transplant-free survival data from the Phase 2 INDIGO study used as the basis for the MAA submission

- Maralixibat would be the first treatment labeled for patients with PFIC2 in Europe, if approved

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 30, 2020-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today announced that the company's Marketing Authorization Application (MAA) for its investigational medicine, maralixibat, for the treatment of patients with progressive familial intrahepatic cholestasis type 2 (PFIC2), also known as bile salt export pump (BSEP) deficiency, was accepted for review (validated) by the European Medicines Agency (EMA). The validation of the application by the EMA confirms all essential regulatory elements are included in the submission such that the EMA can begin its review.

"PFIC is life-altering for patients and their families as they struggle to manage the round-the-clock care and surgical decisions that many children often need," said Chris Peetz, president and chief executive officer at Mirum. "Validating our MAA is a groundbreaking step towards providing a medicine to address PFIC2. Based on the long-term transplant-free survival improvement in maralixibat responders, we believe that maralixibat could provide a treatment alternative to invasive surgeries for these patients, as well as improve quality of life. We are excited about the opportunity to make maralixibat available to patients with PFIC2 in Europe."

Data from the Phase 2 INDIGO study evaluating maralixibat for pediatric patients with PFIC2 served as the basis of the MAA submission. Mirum recently announced data showing five-year transplant-free survival for patients who achieved serum bile acid control. The data also demonstrated improvements across multiple parameters including pruritus control, improvements of liver enzyme and bilirubin levels, and improvement in growth. These data were presented at the annual meeting of the European Association for the Study of the Liver. The MAA submission also includes data on five-year event-free survival with maralixibat compared to the NAPPED natural history cohort.

To provide further evidence of maralixibat's potential in PFIC2 with higher doses and other PFIC subtypes, Mirum is conducting a Phase 3 study, MARCH, with completion of enrollment expected in the second quarter of 2021.

In addition to the MAA submission for maralixibat in PFIC2, Mirum has also initiated a rolling new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for maralixibat for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS). Mirum expects to complete the submission in the first quarter of 2021, with a planned launch in the second half of the same year. The company also recently launched an Expanded Access Program making maralixibat available to eligible patients with ALGS in the United States, Canada, Australia, and certain countries in Europe.

About Maralixibat

Maralixibat is a novel, minimally absorbed, orally administered investigational drug being evaluated in several 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. The U.S. Food and Drug Administration (FDA) has granted maralixibat Breakthrough Therapy designation for 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 by regulatory authorities 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. 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 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 studies for maralixibat and the regulatory approval path for maralixibat. 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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