Mirum Pharmaceuticals Showcases Maralixibat Transplant-Free Survival Data for Progressive Familial Intrahepatic Cholestasis and Long-Term Safety Analyses for Alagille Syndrome during WCPGHAN Annual Meeting

June 3, 2021

- Maralixibat-treated patients achieving serum bile acid control have improved five-year native liver survival; nominated for prestigious Alex Mowat Prize for best oral presentation in hepatology.

- Safety data from more than five years of maralixibat treatment across three Phase 2 ALGS studies demonstrate that gastrointestinal events were generally mild to moderate and transient in nature, with no discontinuation of maralixibat treatment.

FOSTER CITY, Calif.--(BUSINESS WIRE)--Jun. 3, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) presented analyses from its clinical studies evaluating maralixibat in patients with Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis type 2 (PFIC2), two rare liver diseases affecting children.

“The breadth of maralixibat data collected for more than five years, coupled with our understanding of the safety and tolerability of long-term use, helps to underscore its importance as a potentially transformative treatment for patients with ALGS and PFIC2,” said Pam Vig, Ph.D., chief scientific officer at Mirum. “These data highlight that maralixibat may be a meaningful alternative to invasive surgeries that are the current standard of care in these devastating diseases.”

Oral Presentation: H-O-012: Serum bile acid control in long-term maralixibat treated patients is associated with native liver survival in children with progressive familial intrahepatic cholestasis due to bile salt export pump deficiency.
Presented June 3, 2021 at 3:55 p.m. CEST
This abstract has been nominated for the Annual Alex Mowat Prize for best oral presentation in hepatology. Results to be announced on June 5, 2021 at 1:30 p.m. CEST
The presentation is available on Mirum’s website in the Publications and Presentations section.

An analysis from the Phase 2 INDIGO open-label study evaluating long-term clinical efficacy and transplant-free survival with maralixibat in patients (n=19) with bile salt export pump (BSEP) deficiency, or progressive familial intrahepatic cholestasis type 2 (PFIC2) was presented during the congress. These data demonstrated that patients with long-term maralixibat treatment who achieved serum bile acid (sBA) response had five-year transplant-free survival. In addition, those patients who achieved sBA response also had significant improvements in pruritus, liver parameters, quality of life and growth. The study showed that maralixibat was generally well-tolerated and the most common adverse events were mild to moderate diarrhea and abdominal pain, which were transient in nature; no gastrointestinal events led to discontinuation of maralixibat.

Poster Presentation: H-ePwP-030: Gastrointestinal tolerability of maralixibat in patients with Alagille syndrome: An integrated analysis of short- and long-term treatment
To be presented on Saturday, June 5, 2021 at 10:40 a.m. CEST
Poster now available via the WCPGHAN ePoster exhibition section of the website and on Mirum’s website in the Publications section.

Data from more than five years of evaluation across three maralixibat Phase 2 clinical trials and their extension studies were analyzed to assess treatment-emergent adverse events of diarrhea and abdominal pain (GI events) in patients with ALGS. In the 86 patients evaluated, the analysis included incidence, severity, seriousness, time to onset, and duration of events, irrespective of relatedness to treatment with maralixibat, as determined by the investigator.

Findings from the integrated safety study concluded:

- Data from the 13-week placebo group demonstrated that rates of diarrhea were similar between maralixibat and placebo, with a slight difference in abdominal pain.
- The majority of GI events occurred within the first four weeks of treatment and lasted less than one week in duration.
- For patients who experienced GI events, the majority of diarrhea and abdominal pain were mild to moderate in severity and transient in nature.
- There were no GI-related discontinuations of maralixibat over the five years of evaluation.

About Mirum Pharmaceuticals, Inc.

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 gene therapy programs VTX-803 and VTX-802 for PFIC3 and PFIC2, respectively, from Vivet Therapeutics SAS, following preclinical evaluation and investigational new drug-enabling studies.

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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.

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 potential of maralixibat to be a meaningful alternative to the current standard of care for ALGS and PFIC2, and the regulatory approval pathway 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 "could," "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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