Mirum Pharmaceuticals to Present Maralixibat Data and Host Symposium at the 6th World Congress of Pediatric Gastroenterology, Hepatology and Nutrition (WCPGHAN) Annual Meeting 2021

May 26, 2021

- Integrated safety analysis on gastrointestinal tolerability of maralixibat in patients with Alagille syndrome presented in poster session.

- Five-year analysis demonstrating improvements in transplant-free survival for patients with PFIC2 featured in oral presentation; abstract nominated for the Alex Mowat Prize for best oral presentation in hepatology.

- Mirum to host satellite symposium on June 3, 2021.

FOSTER CITY, Calif.--(BUSINESS WIRE)--May 26, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today announced that data from maralixibat studies in patients with Alagille syndrome and progressive familial intrahepatic cholestasis type 2 will be presented in oral and poster sessions during the 6th Annual World Congress of Pediatric Gastroenterology, Hepatology and Nutrition (WCPGHAN) taking place virtually June 2-5, 2021.

Data featured during the congress include:

Oral Presentation
Abstract number: 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.*
Presenter: Professor Richard Thompson, M.D., Ph.D., Professor of Molecular Hepatology at King's College London and principal investigator for the INDIGO study
Session: Highest Scoring Abstracts
Date and time: Thursday, June 3, 2021 – 3:55-4:05 p.m. CEST
This abstract has been nominated for the prestigious Annual Alex Mowat Prize for best oral presentation in hepatology. Results to be announced during the congress closing ceremony on June 5, 2021 at 1:30 p.m. CEST.

Poster Presentation
Abstract number: H-ePwP-030: *Gastrointestinal tolerability of maralixibat in patients with Alagille syndrome: An integrated analysis of short- and long-term treatment.*
Presenter: Rakesh K. Raman, M.D., Mirum Pharmaceuticals, Inc.
Session: ePoster Session Hepatology
Date and time: Saturday, June 5, 2021 – 10:40-11:30 a.m. CEST

Satellite Symposium
Pediatric Cholestasis: Itching for an alternative to liver transplantation.
Date and time: Thursday, June 3 at 5:30 p.m. CEST
Featured Speakers: Dr. Binita M. Kamath (The Hospital for Sick Children (SickKids) Toronto, Ontario, Canada), Dr. Emmanuel Gonzales (Hépatologie Pédiatrique, Hôpital Bicêtre, AP-HP. Université Paris-Saclay, Le Kremlin-Bicêtre, France), Dr. Richard Thompson (King's College, London, UK)

Mirum will be sponsoring a virtual symposium featuring leading experts who will discuss the current treatment landscape for pediatric cholestasis, including the high unmet medical need and the potential for pharmacologic treatments as an alternative to surgical options. This symposium will highlight diagnostic challenges related to Alagille syndrome and progressive familial intrahepatic cholestasis as well as the burden of disease on children and their caregivers. Data supporting the potential rationale for apical sodium-dependent bile acid transporter inhibition as an alternative to surgical treatment options will also be explored.

WCPGHAN registered attendees can view the live symposium via the WCPGHAN website within the calendar of events in the Scientific Programme At-A-Glance section.

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, 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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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 benefits, development and commercialization of 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 "will," "could," "would," "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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