Mirum Pharmaceuticals to Showcase Analyses From Its Rare Liver Disease Programs at The International Liver Congress 2021

June 16, 2021

FOSTER CITY, Calif.--(BUSINESS WIRE)--Jun. 16, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a leader in rare liver disease, today announced its participation in The International Liver Congress™ taking place virtually June 23-26, 2021. The company will be presenting an integrated safety analysis of maralixibat in 86 patients with Alagille syndrome (ALGS). Mirum will also be unveiling a multi-national survey of patient reported outcomes data from pregnant women with intrahepatic cholestasis of pregnancy (ICP), conducted in collaboration with ICP Support, a leading patient advocacy group focused on ICP, based in the United Kingdom.

Posters to be presented during the congress include:

Abstract PO-1285:
An integrated analysis of long-term clinical safety in maralixibat-treated participants with Alagille syndrome

Abstract PO-2657:
Patient perspectives on pruritus in intrahepatic cholestasis of pregnancy: a multinational survey

All posters will be available on The International Liver Congress website beginning Wednesday, June 23 at 8:00 a.m. CEST (2:00 a.m. ET).

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 further information about maralixibat's ongoing studies in pediatric liver disease, please visit the study websites: Phase 3 MARCH study for PFIC and Phase 2b EMBARK study for biliary atresia.

About Volixibat

Volixibat is an oral, minimally absorbed agent designed to selectively inhibit the apical sodium dependent bile acid transporter (ASBT). Volixibat may offer a novel approach in the treatment of adult cholestatic diseases by blocking the recycling of bile acids, through inhibition of ASBT, thereby reducing bile acids systemically and in the liver. Phase 1 and Phase 2 studies of volixibat demonstrated on-target fecal bile acid excretion, a pharmacodynamic marker of ASBT inhibition, in addition to decreases in LDL cholesterol and increases in 7αC4 which are markers of bile acid synthesis. Volixibat has been evaluated in more than 400 individuals across multiple clinical trials. The most common adverse events reported were mild to moderate gastrointestinal events observed in the volixibat groups.

Volixibat is currently being evaluated in Phase 2b studies for primary sclerosing cholangitis (VISTAS study) and intrahepatic cholestasis of pregnancy (OHANA study). Mirum plans to initiate a Phase 2b study for primary biliary cholangitis later this year.

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 of maralixibat and volixibat. 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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