# Mirum Pharmaceuticals Announces Partnership With EVERSANA to Support Launch and Commercialization of Maralixibat for Alagille Syndrome in the United States

October 15, 2020

*Agreement outlines commercialization and distribution services to support patients diagnosed with Alagille syndrome*

FOSTER CITY, Calif.--(BUSINESS WIRE)--Oct. 15, 2020-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today announced it has partnered with EVERSANA™, a leading provider of commercial services to the life science industry, to lead the U.S. market access, distribution and patient services for maralixibat.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20201015005342/en/

Maralixibat is a novel, minimally absorbed, orally administered apical sodium dependent bile acid transporter inhibitor being evaluated for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS). Mirum initiated its rolling New Drug Application (NDA) to the U.S. Food and Drug Administration in August 2020, expects to complete the NDA submission in the first quarter of 2021, and is preparing for launch in the second half of 2021.

Mirum will utilize EVERSANA's commercialization platform to provide integrated nationwide distribution, specialty pharmacy, patient services and Hub support for maralixibat, if approved.

"We are working diligently to prepare for the potential launch of maralixibat in the United States and one of the most important components is ensuring comprehensive support for patients and their families," said Chris Peetz, president and chief executive officer at Mirum. "We are pleased to partner with EVERSANA and are confident in their ability to provide end-to-end services to ensure maralixibat will reach patients and their families efficiently and effectively once the medication is available for prescribing. In parallel, we are preparing to file a marketing authorization application for maralixibat in PFIC2 this year, and these programs will help to refine our strategies as we finalize launch plans in Europe."

"Rare diseases like ALGS demand more than traditional patient support or distribution models. From day one, we knew that our partnership would focus entirely on individual patient's needs at each step of their therapeutic and treatment journey. The platform is designed so that at launch, EVERSANA's infrastructure and experts will be ready with the right services, and each action will be continually optimized through real-time analytics," said Jim Lang, chief executive officer of EVERSANA. "Mirum's patients will have access to comprehensive support delivered by Mirum and EVERSANA, two companies equally committed to the rare disease space."

**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 1,700 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 FDA 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, abdominal pain, and vomiting. Until maralixibat is approved by the FDA 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 Alagille Syndrome**

ALGS is a rare genetic disorder in which bile ducts are abnormally narrow, malformed and reduced in number, which leads to bile accumulation in the liver and ultimately progressive liver disease. The estimated incidence of ALGS is one in every 30,000 people. In patients with ALGS, multiple organ systems may be affected by the mutation, including the liver, heart, kidneys and central nervous system. The accumulation of bile acids prevents the liver from working properly to eliminate waste from the bloodstream and, according to recent reports, 60% to 75% of patients with ALGS have a liver transplant before reaching adulthood. Signs and symptoms arising from liver damage in ALGS may include jaundice (yellowing of the skin), xanthomas (disfiguring cholesterol deposits under the skin), and pruritus (itch). The pruritus experienced by patients with ALGS is among the most severe in any chronic liver disease and is present in most affected children by the third year of life.

**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 for the treatment of patients with cholestatic pruritus associated with ALGS and expects to complete the submission in the first quarter of 2021. Additionally, the company plans to submit a marketing authorization application to the European Medicines Agency for maralixibat in the treatment of patients with PFIC2 in the fourth quarter 2020.

The company 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 regulatory approval path for maralixibat, and the potential launch of maralixibat, if approved, the market access, distribution and patient services of maralixibat to be provided by Eversana, and Mirum's Expanded Access Program 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", "anticipates," "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.*

**About EVERSANA™**

EVERSANA™ is the leading provider of global services to the life sciences industry. The company's integrated solutions are rooted in the patient experience and span all stages of the product lifecycle to deliver long-term, sustainable value for patients, prescribers, channel partners and payers. The company serves more than 500 organizations, including innovative start-ups and established pharmaceutical companies, to advance life science solutions for a healthier world. To learn more about EVERSANA, visit eversana.com or connect through LinkedIn and Twitter.

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