Mirum Pharmaceuticals to Present New Data at The Liver Meeting 2019

October 1, 2019

FOSTER CITY, Calif.--(BUSINESS WIRE)--Oct. 1, 2019-- Mirum Pharmaceuticals, Inc. (NASDAQ: MIRM), a biopharmaceutical company focused on the development and commercialization of a late-stage pipeline of novel therapies for debilitating liver diseases, today announced that multiple maralixibat abstracts will be presented at The Liver Meeting® 2019, the Annual Meeting of the American Association for the Study of Liver Disease (AASLD), taking place November 8-12, 2019 in Boston, Massachusetts.

Oral Presentation:

Title: Genotype and dose-dependent response to maralixibat in patients with bile salt export pump deficiency
Date and Time: Sunday November 10, 2019 at 2:45 p.m. ET
Location: Constitution Ballroom, Hynes Convention Center
Abstract Number: 0082

Poster Presentations:

Title: Safety and efficacy of maralixibat in patients with primary sclerosing cholangitis: an open-label proof-of-concept study (Presidential Poster of Distinction)
Date and Time: Duration of the meeting
Location: Hynes Convention Center, Hall B
Abstract Number: 1262

Title: Dose-dependent fecal bile acid excretion with apical sodium-dependent bile acid transporter inhibitors maralixibat and volixibat in a dose-ranging phase 1 study in overweight and obese adults
Date and Time: Duration of the meeting
Location: Hynes Convention Center, Hall B
Abstract Number: 1298

About Maralixibat

Maralixibat is a novel, minimally absorbed, orally administered investigational drug being evaluated in several rare cholestatic liver diseases for pediatric populations. Maralixibat inhibits the apical sodium dependent bile acid transporter which increases bile acid excretion in the feces, lowering levels of bile acids systemically, thereby potentially reducing bile acid-mediated liver damage and related complications. More than 1,500 individuals have received maralixibat, including more than 100 children as an investigational treatment for Alagille syndrome (ALGS) or progressive familial intrahepatic cholestasis (PFIC). In a Phase 2 PFIC study, a genetically defined subset of PFIC2 patients responded to maralixibat, leading to maralixibat receiving Breakthrough Therapy designation from the U.S. Food and Drug Administration for PFIC2. In a Phase 2b ALGS study, patients taking maralixibat had significant reductions in bile acids and pruritus compared to placebo. Maralixibat was generally well-tolerated. The most frequent adverse events were diarrhea, abdominal pain and vomiting.

About Volixibat

Volixibat is a novel, oral, minimally-absorbed agent designed to selectively inhibit ASBT. Volixibat may offer a novel approach in the treatment of adult cholestatic diseases by blocking recycling of bile acids, through inhibition of the apical sodium dependent bile acid transporter (ASBT), thereby reducing bile acids systemically and in the liver. Phase 1 and Phase 2 clinical trials 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 C4 which are markers of bile acid synthesis. Volixibat has been evaluated in more than 300 subjects across multiple clinical trials. The most common adverse events reported were mild to moderate gastrointestinal events observed in the volixibat groups.

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 progressive familial intrahepatic cholestasis (PFIC) and Alagille syndrome (ALGS). For more information, visit MirumPharma.com. Follow Mirum on Twitter, Facebook and LinkedIn.

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, progress and timing of Mirum's clinical trials, 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," "believes," "anticipates," "expects," "intends," "goal," "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, 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. 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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Source: Mirum Pharmaceuticals, Inc.

Media Contact:
Heidi Chokeir, Ph.D.
Canale Communications
619-203-5391
heidi@canalecomm.com

Investor Contact:
Ian Clements, Ph.D.
650-667-4085
ir@mirumpharma.com