# Mirum Pharmaceuticals Announces New Data Being Presented in Late-Breaker Oral and Poster Presentations at the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD)

November 2, 2020

- *Late-breaker oral presentation features long-term data from Phase 2 ITCH and IMAGINE II studies evaluating maralixibat in pediatric patients with Alagille syndrome*

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 2, 2020-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases today announced that new data from the company's maralixibat and volixibat studies will be presented at The Liver Meeting Digital Experience™, the Annual Meeting of the American Association for the Study of Liver Diseases (AASLD), taking place November 13-16, 2020.

***Late-breaker Oral Presentation***

**L05: Preliminary Analysis of ITCH and IMAGINE II – Outcome of long-term administration of maralixibat in children with Alagille syndrome**

- Presented by Benjamin Shneider, M.D. on November 15, 2020 during the 5:30-7:00 p.m. ET session.

***Poster Presentations***

**Abstract #1221: A Phase 1 dose-ranging study assessing fecal bile acid excretion by volixibat, an apical sodium-dependent bile acid transporter inhibitor, and coadministration with loperamide**

**Abstract #341: Pruritus intensity is associated with cholestasis biomarkers and quality of life measures after maralixibat treatment in children with Alagille syndrome**

**Abstract #1792: Natural variability of pruritus in Alagille syndrome; an analysis from the ICONIC study utilizing the Itch Reported Outcome Observer (ItchRO[Obs]) tool**

All posters will be available at the start of the congress on November 13, 2020 and available throughout the duration of the meeting. Abstracts are available via Hepatology on the AASLD website.

**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. The FDA has granted maralixibat Breakthrough Therapy designation for 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 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 Volixibat**

Volixibat is an 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 400 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 Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. The company has initiated a rolling NDA submission for maralixibat in 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. 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 and progress of Mirum's ongoing and planned studies for maralixibat and volixibat, and the regulatory approval path for 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 "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, 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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Media Contact:
Erin Murphy
media@mirumpharma.com

Investor Contact:
Ian Clements, Ph.D.
ir@mirumpharma.com

Source: Mirum Pharmaceuticals, Inc.