



Mirum Pharmaceuticals to Present Maralixibat Data and Host Symposium at the NASPGHAN Annual Meeting 2020

October 26, 2020

FOSTER CITY, Calif.--(BUSINESS WIRE)--Oct. 26, 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 data from the Company's maralixibat studies will be presented at the North American Society for Pediatric, Gastroenterology, Hepatology and Nutrition (NASPGHAN) Annual Meeting 2020, being held November 1-7, 2020.

Maralixibat presentations are as follows:

Oral Presentation

Abstract 243: Durability of treatment effect with long-term maralixibat use in children with Alagille syndrome: 4-year safety and efficacy results from the ICONIC study

November 2, 2020 from 3:00-4:30 p.m. ET (oral presentation at 4:02 p.m. ET)

Poster Presentation

Abstract 646: Genotype and dose-dependent response to maralixibat in patients with bile salt export pump deficiency

November 6, 2020 from 11:00 a.m. – 1:00 p.m. ET

The abstracts from the meeting are available on the Journal of Pediatric Gastroenterology and Nutrition [website](#).

Virtual Symposium

"Solving the Puzzle of Pediatric Cholestasis"

November 2, 2020 – 4:30-6:00 p.m. ET

Mirum will be sponsoring a virtual symposium led by Dr. Philip Rosenthal with presentations on Alagille syndrome (ALGS), Progressive Familial Intrahepatic Cholestasis (PFIC) and Biliary Atresia (BA), by Dr. Binita Kamath and Dr. Richard Thompson. To attend the symposium, you must be [registered](#) for NASPGHAN. Once registered, the congress will email an invitation to the event.

"We are pleased to share data from our maralixibat studies at the NASPGHAN meeting as it provides a forum for scientific exchange with leaders in the pediatric gastroenterology and hepatology community who treat children with rare diseases like ALGS and PFIC," said Chris Peetz, president and chief executive officer of Mirum. "The data demonstrate long-term treatment success of more than five years for patients with pruritus associated with ALGS, based on findings from the ICONIC study. Also being shared are data highlighting five-year transplant-free survival in patients with PFIC2 who responded to maralixibat. These data underscore our confidence in maralixibat to be a medicine that has the potential to change the treatment paradigms for ALGS and PFIC2."

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 eligible 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

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 of 2020.

The company is also developing volixibat, 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 and progress of Mirum’s studies for maralixibat, as well as the regulatory approval path for maralixibat, and the potential launch of maralixibat, if approved. 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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