Durability of Treatment Effect of Mirum Pharmaceuticals' Maralixibat for Children With Alagille Syndrome Featured in Late Breaking Session at the Liver Meeting 2019

October 21, 2019

FOSTER CITY, Calif.--(BUSINESS WIRE)--Oct. 21, 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 data on its program for the treatment of children with maralixibat will be featured in the late breaking oral presentation session during the American Association for the Study of Liver Diseases Annual Meeting (The Liver Meeting), November 8-12, 2019, in Boston.

"We are very pleased to be presenting the long-term treatment effect of maralixibat in Alagille syndrome patients from the ICONIC study, highlighting the potential impact of maralixibat on this terrible disease," said Chris Peetz, president and chief executive officer of Mirum. "In ICONIC, long-term treatment with maralixibat was associated with durability of treatment response on pruritus measures as well as serum bile acids and xanthomas. Over the course of four years maralixibat was generally well tolerated, and led to control of cholestasis and pruritus in children with Alagille syndrome."

Presentation Details:

Title: Durability of Treatment Effect with Long-Term Maralixibat in Children with Alagille Syndrome: 4-Year Safety and Efficacy Results from the ICONIC Study
Date and Time: Monday, November 11, 2019, 3:00 p.m. ET
Presenter: Emmanuel Gonzales M.D., Professor of Pediatrics, Hôpital Bicêtre
Location: Auditorium, Hynes Convention Center
Abstract Number: LO3

Abstract Summary:

Maralixibat, a minimally absorbed apical sodium-dependent bile acid transporter inhibitor, is in development for treatment of severe cholestatic diseases. ICONIC is a randomized, controlled long-term phase 2b study of maralixibat in children with Alagille syndrome. Primary results (week 48) demonstrating significant reductions in serum bile acids (sBA), pruritus and xanthomas have been presented. Results of patients who entered long-term extension are reported.

Participants reaching week 48 of ICONIC were eligible to continue maralixibat in this open-label extension. Statistically significant reductions from baseline to week 48 were observed in sBA levels, ItchRO observer score, xanthoma score and clinician scratch scale. Participants who consented to long-term extension and remain on maralixibat have been treated for a median duration of 44.5 months. In line with the 48-week results, reductions in sBA, pruritus and xanthomas remained statistically significant over the long term. Maralixibat was generally safe and well tolerated long term, with no increases in frequency or severity of adverse events.

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 results 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 1,500 individuals have received maralixibat, including more than 100 children who have received maralixibat as an investigational treatment for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). In a Phase 2 PFIC study, a genetically defined subset of BSEP (bile salt export protein) deficient, or PFIC2, patients responded to maralixibat, which led 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 throughout the studies. The most frequent adverse events were diarrhea, abdominal pain and vomiting.

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. 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, including without limitation in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2019. 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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