News Release Details

Mirum Pharmaceuticals Initiates Rolling Submission of a New Drug Application for Maralixibat for the Treatment of Cholestatic Pruritus in Patients with Alagille Syndrome and Launches Expanded Access Program

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(Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases today announced that it has submitted the first portion of its rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for maralixibat. Maralixibat is a novel, minimally absorbed, orally administered apical sodium dependent bile acid transporter (ASBT) inhibitor being evaluated for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS). Mirum expects to complete the NDA submission in the first quarter of 2021.

Mirum also launched its Expanded Access Program (EAP) for maralixibat for the treatment of cholestatic pruritus in patients with ALGS one year of age and older. The EAP is open for registration in the United States and Canada. Through this program, physicians can request access to maralixibat for eligible patients who are not part of an ongoing clinical trial.

“We are thrilled to initiate the rolling NDA submission for maralixibat, taking us one step closer to making this medicine widely available for patients with ALGS,” said Chris Peetz, president and chief executive officer at Mirum. “We believe the results of our clinical program demonstrate the potential of maralixibat to transform the treatment of this life-threatening disease. We are also pleased to launch our Expanded Access Program for patients with ALGS in the United States and Canada and are evaluating ways in which we can make maralixibat available for patients with ALGS in other countries. Additionally, we are planning to broaden access to maralixibat through our anticipated Marketing Authorization Application submission for patients with PFIC2 in Europe later this year.”

About the NDA Submission

Maralixibat was previously granted Rare Pediatric Disease Designation for ALGS and, as such, may qualify for receipt of a priority review voucher if the NDA is approved by the FDA. Maralixibat was also granted Breakthrough Therapy Designation for the treatment of pruritus associated with ALGS in patients one year of age and older. Maralixibat was granted Orphan Drug Designation by the FDA for the treatment of patients with PFIC and ALGS in the United States.

Data from the maralixibat Phase 2 ICONIC study evaluating patients with ALGS serves as the basis of efficacy for the submission. Previously presented data from this study in November 2019 are available within the Publications and Presentations section on Mirum’s website.

Mirum expects to complete the rolling NDA submission in the first quarter of 2021 and is planning for a potential launch of maralixibat in ALGS in the second half of 2021.

About the Expanded Access Program

The EAP, sometimes referred to as “compassionate use,” provides a potential pathway for a patient with an immediately life-threatening condition or serious disease to gain access to an investigational medicine for the treatment of that disease outside of a clinical trial when no comparable or satisfactory alternative therapy options are available.

Physicians and patients can learn more about the maralixibat EAP by visiting the program website at www.ALGSEAP.com or via https://clinicaltrials.gov/ct2/show/NCT04530994. Physicians who would like to request access for their patient can contact MirumALGS@clinigengroup.com.

For patients with PFIC, access to maralixibat is possible through our MARCH Phase 3 study, which is currently open to enrollment. More information can be found at: https://pfictrial.com. Mirum plans to continually evaluate the need for expanded access to maralixibat as studies reach enrollment milestones.

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, 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.[1] In patients with ALGS, multiple organ systems may be affected by the mutation, including the liver, heart, kidneys and central nervous system.[2] 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 Alagille syndrome have a liver transplant before reaching adulthood.[3] 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)[2]. The pruritus experienced by patients with ALGS is among the most

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, progress and timing of Mirum's ongoing and planned studies for maralixibat, as well as Mirum's Expanded Access Program for maralixibat, the regulatory approval path for maralixibat and volixibat, 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.

[1]Danks, et al. Archives of Disease in Childhood 1977

[2]Johns Hopkins Medicine. hopkinsmedicine.org/health/conditions-and-diseases/Alagille-syndrome

[3]Vandriel, et al. GALA EASL 2020; Kamath, et al. Hepatology Communications 2020

[4]Elisofon, et al. Journal of Pediatric Gastroenterology and Nutrition 2010

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