Mirum Pharmaceuticals Announces Completion of Rolling NDA Submission for Maralixibat in Alagille Syndrome

February 1, 2021

- Maralixibat U.S. launch expected in second half of 2021, if approved

FOSTER CITY, Calif.--(BUSINESS WIRE)--Feb. 1, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today announced it has completed submission of its rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for maralixibat for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) one year of age and older. ALGS is a rare liver disease for which there are currently no approved therapies. The company is planning for the U.S. launch of maralixibat in the second half of 2021, should the medication be approved by the FDA for this indication.

The NDA submission included the results of the ICONIC study and additional supportive studies totaling 86 patients with ALGS with up to six-year follow-up. The clinical safety data submitted for maralixibat includes over 1,600 individuals treated.

“Patients, families and physicians have an urgent need for effective medications to address the severe and life-altering symptoms associated with ALGS,” said Chris Peetz, president and chief executive officer at Mirum. “This submission brings us one step closer to a potential approval of maralixibat and providing a much-needed treatment option for the patients and their families suffering with ALGS.”

Until maralixibat is approved by the FDA for prescription, maralixibat is available to eligible patients with ALGS through Mirum’s Expanded Access Program (EAP). The EAP is available in the United States, Canada, Australia and in regions throughout Europe. Requests for expanded access to maralixibat must be made by a licensed physician. Physicians and patients can learn more about the maralixibat EAP by visiting the program website at www.ALGSEAP.com or via Clinicaltrials.gov. Physicians in the United States and Canada who would like to request access for their patients can email MirumALGS@clinigengroup.com. Physicians in Europe and Australia can contact medicineaccess@clinigengroup.com with a reference line of “Mirum ALGS EAP request”.

Maralixibat was granted Rare Pediatric Disease Designation for ALGS and, as such, may qualify for receipt of a priority review voucher, if 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.

About Alagille syndrome

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 ALGS 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 severe in any chronic liver disease and is present in most affected children by the third year of life.[4]

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 with an increase in transplant-free survival. The U.S. Food and Drug Administration has granted maralixibat Breakthrough Therapy designation for the 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 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 Mirum

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. Mirum’s lead product candidate, maralixibat, is an investigational oral drug in development for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. Mirum has submitted an NDA for maralixibat in the treatment of cholestatic pruritus in patients with ALGS. Additionally, Mirum’s marketing authorization application for the treatment of pediatric patients with PFIC2 has been accepted for review (validated) by the European Medicines Agency.

Mirum is also developing volixibat, also an oral ASBT-inhibitor, in primary sclerosing cholangitis, intrahepatic cholestasis of pregnancy, and primary

biliary cholangitis. For more information, visit MirumPharma.com.

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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 regulatory approval path for maralixibat in the United States. 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,” ”may,” “anticipates,” “expects,” “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.

References

[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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