Mirum Pharmaceuticals Broadens Expanded Access Program for Maralixibat in Alagille Syndrome to Europe and Australia

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- Maralixibat Expanded Access Program now available for patients with pruritus associated with Alagille syndrome in Australia and 10 countries in Europe.

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 5, 2020-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a biopharmaceutical company focused on the development and commercialization of novel therapies for debilitating liver diseases, today announced it has broadened its Expanded Access Program (EAP) for maralixibat for the treatment of patients with cholestatic pruritus associated with Alagille syndrome (ALGS) to Australia and countries in Europe. The announcement follows the Company's recent initiation of an EAP in the United States and Canada for maralixibat in the same indication.

"Maralixibat has the potential to make a meaningful difference in the treatment of Alagille syndrome and it is our goal to help ensure that eligible patients have access to maralixibat until it is available for prescribing in Europe and Australia," said Chris Peetz, president and chief executive officer at Mirum. "Continuity of treatment is critically important for children with rare diseases, like Alagille syndrome, and we hope that early access to investigational treatments such as maralixibat will help to reduce many of the debilitating symptoms associated with the disease as well as the need for liver transplantation."

Maralixibat is a novel, minimally absorbed, orally administered apical sodium dependent bile acid transporter inhibitor being evaluated for the treatment of cholestatic pruritus in patients with ALGS. Mirum initiated its rolling New Drug Application (NDA) for the treatment of cholestatic pruritus in patients with ALGS to the U.S. Food and Drug Administration (FDA) in September 2020, expects to complete the submission in the first quarter of 2021, and is preparing for a U.S. commercial launch in the second half of 2021.

About the Maralixibat 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.

The goal of Mirum's EAP is to provide access to maralixibat for the treatment of cholestatic pruritus in eligible patients with ALGS prior to regulatory approval of the medication and until maralixibat is available by prescription.

The EAP will be open to eligible patients in Australia, Austria, Belgium, Canada, France, Germany, Italy, Netherlands, Poland, Spain, Sweden, the United States, and the United Kingdom.

Requests for expanded access to maralixibat must be made by a licensed physician. Physicians and patients can learn more about the maralixibat EAP for ALGS by visiting the program website at www.ALGSEAP.com or via https://clinicaltrials.gov/ct2/show/NCT04530994. Physicians in select European countries and Australia who would like to request access for their patients can contact medicineaccess@clinigengroup.com with a reference line of "Mirum ALGS EAP request". Physicians in the United States and Canada can email MirumALGS@clinigengroup.com.

For patients with progressive familial intrahepatic cholestasis (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 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 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 severe in any chronic liver disease and is present in most affected children by the third year of life.[4]

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.

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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, Mirum's Expanded Access Program for maralixibat, 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", "expects," "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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Media:
Erin Murphy
media@mirumpharma.com

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

Source: Mirum Pharmaceuticals, Inc.