Mirum Pharmaceuticals Sells Rare Pediatric Disease Priority Review Voucher

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FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 17, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a leader in rare liver disease, today announced that it has entered into a definitive agreement to sell its Rare Pediatric Disease Priority Review Voucher ("PRV") for $110 million.

The PRV was granted by the U.S. Food and Drug Administration in September 2021 with the approval of LIVMARLI™ (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older.

Pursuant to the agreement, Mirum will receive a payment of $110 million upon the closing of the transaction, which is subject to customary closing conditions and is expected to occur following the expiration of the applicable U.S. antitrust clearance requirements.

Jefferies LLC acted as the exclusive financial advisor to Mirum for this transaction.

About LIVMARLI™ (maralixibat) oral solution

LIVMARLI™ (maralixibat) oral solution is an orally administered, once-daily, ileal bile acid transporter (IBAT) inhibitor approved by the U.S. Food and Drug Administration for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older and is the only FDA-approved medication to treat cholestatic pruritus associated with Alagille syndrome. For more information, please visit LIVMARLI.com.

LIVMARLI is currently being evaluated in late-stage clinical studies in other rare cholestatic liver diseases including progressive familial intrahepatic cholestasis and biliary atresia. LIVMARLI has received Breakthrough Therapy designation for ALGS and PFIC type 2 and orphan designation for ALGS, PFIC and biliary atresia. To learn more about ongoing clinical trials with LIVMARLI, please visit Mirum's clinical trials section on the company's website.

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]

IMPORTANT SAFETY INFORMATION

LIVMARLI can cause serious side effects, including:

Changes in liver tests. Changes in certain liver tests are common in patients with Alagille syndrome and can worsen during treatment with LIVMARLI. These changes may be a sign of liver injury and can be serious. Your healthcare provider should do blood tests before starting and during treatment to check your liver function. Tell your healthcare provider right away if you get any signs or symptoms of liver problems, including nausea or vomiting, skin or the white part of the eye turns yellow, dark or brown urine, pain on the right side of the stomach (abdomen) or loss of appetite.

Stomach and intestinal (gastrointestinal) problems. LIVMARLI can cause stomach and intestinal problems, including diarrhea, stomach pain, and vomiting during treatment. Tell your healthcare provider right away if you have any of these symptoms more often or more severely than normal for you.

A condition called **Fat Soluble Vitamin (FSV) Deficiency** caused by low levels of certain vitamins (vitamin A, D, E, and K) stored in body fat. FSV deficiency is common in patients with Alagille syndrome but may worsen during treatment. Your healthcare provider should do blood tests before starting and during treatment.

Other common side effects reported during treatment were bone fractures and gastrointestinal bleeding.

Prescribing information

About Mirum Pharmaceuticals, Inc.

Mirum Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to transforming the treatment of rare liver diseases. Mirum's approved medication is LIVMARLI™ (maralixibat) oral solution which is approved in the U.S. for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older.

Mirum's late-stage pipeline includes two investigational treatments for debilitating liver diseases affecting children and adults. Maralixibat (LIVMARLI), an oral ileal bile acid transporter (IBAT) inhibitor, is currently being evaluated in clinical trials for pediatric liver diseases and includes the MARCH Phase 3 study for progressive familial intrahepatic cholestasis (PFIC) and the EMBARK Phase 2b study for patients with biliary atresia. In addition, Mirum has an expanded access program open in Canada, Australia, the UK and several countries in Europe for eligible patients with Alagille

syndrome.

Mirum has submitted a Marketing Authorization Application to the European Medicines Agency for maralixibat for the treatment of cholestatic liver disease in patients with Alagille syndrome.

Mirum's second investigational treatment, volixibat, also an oral IBAT inhibitor, is being evaluated in two registrational studies including the OHANA Phase 2b study for pregnant women with intrahepatic cholestasis of pregnancy and the VISTAS Phase 2b study for adults with primary sclerosing cholangitis. Mirum is planning to launch a Phase 2b study in primary biliary cholangitis later this year.

To augment its pipeline in cholestatic liver disease, Mirum has acquired the exclusive option to develop and commercialize gene therapy programs VTX-803 and VTX-802 for PFIC3 and PFIC2, respectively, from Vivet Therapeutics SAS, following preclinical evaluation and investigational new drug-enabling studies.

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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 closing of the PRV sale and Mirum's receipt of the proceeds from the proposed sale. 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 "will," "could," "would," "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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