



U.S. FDA Approves LIVMARLI (maralixibat) as the First and Only Approved Medication for the Treatment of Cholestatic Pruritus in Patients with Alagille Syndrome One Year of Age and Older

September 29, 2021

Mirum to host an investor call on September 29, 2021 at 4:30 p.m. ET

FOSTER CITY, Calif.--(BUSINESS WIRE)--Sep. 29, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a leader in rare liver disease, today announced that the U.S. Food and Drug Administration (FDA) has approved LIVMARLI™ (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) one year of age and older. LIVMARLI, a minimally absorbed ileal bile acid transporter (IBAT) inhibitor, is the first and only FDA-approved medication in this rare liver disease which affects 2,000 to 2,500 children in the United States. LIVMARLI is now available for prescribing. In conjunction with the approval, Mirum received a rare pediatric disease priority review voucher.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20210929005810/en/>



www.livmarli.com

“Children with Alagille syndrome suffer from cholestatic pruritus, which is serious, unremitting, and debilitating. Their sleep is disrupted, and they endure bleeding and scarring of the skin due to unrelenting scratching,” said Binita M. Kamath, MBBChir, Pediatric Hepatologist, The Hospital for Sick Children (SickKids), Toronto, Ontario, Canada. “There have been no approved treatments to date for cholestatic pruritus in Alagille syndrome, and many children ultimately require major surgical interventions such as liver

transplantation for refractory pruritus. The approval of LIVMARLI signifies a meaningful shift in the treatment paradigm for Alagille syndrome and provides hope for the many families who have lived with persistent itch for far too long.”

ALGS is a rare genetic disorder caused by abnormalities in bile ducts that can lead to progressive liver disease. Malformed or reduced bile ducts cause cholestasis, the accumulation of bile acids in the liver, which leads to inflammation and liver injury, and prevents the liver from working properly. Cholestasis in ALGS is associated with pruritus which is among the most common indications for liver transplant in ALGS.

The approval of LIVMARLI is based on the pivotal ICONIC study as well as five years of data from supportive studies resulting in a robust body of evidence in 86 patients with ALGS. Data from ICONIC demonstrated statistically significant reductions in pruritus, one of the most common and arduous symptoms associated with the disease, which was maintained through four years.

“Today is a great day for the Alagille syndrome community with the approval of a much-needed new treatment option to address one of the most debilitating effects of this disease,” said Chris Peetz, president and chief executive officer of Mirum. “We are grateful to the patients, families, and healthcare professionals who advanced the research and participated in the LIVMARLI clinical studies. Today is also a landmark day for Mirum as we take steps forward in developing potentially life-changing medicines for rare liver disease.”

“We have had the pleasure of being part of and closely following the clinical progress of LIVMARLI in many ways. Since the first study’s initiation more than a decade ago, we have dreamed of today, seeing LIVMARLI receive FDA approval, marking an incredibly meaningful milestone for the ALGS community,” said Roberta Smith, president, Alagille Syndrome Alliance and an ALGS mom. “Until now, patients have had limited-to-no treatment options to address the severe and unrelenting itch that significantly impacts both patients and their families. Additionally, because pruritus associated with ALGS greatly impacts caregivers, having a strong support program like Mirum Access Plus to reduce the strain on families is so important. The ALGS community has been waiting for a long time for a treatment and we’re so pleased that LIVMARLI is now available in the United States.”

Mirum Access Plus (MAP)

LIVMARLI will be accessible to patients with a prescription through Mirum Access Plus (MAP), the company’s patient support services program and single-source specialty pharmacy. The MAP program has fully dedicated and experienced coordinators who will work with healthcare providers and families to provide insurance coverage and access support, as well as help with financial support options for LIVMARLI. A dedicated Navigator team will also provide health education and will connect families to resources and tools to support their disease. MAP staff are available Monday through Friday, 8:00 a.m. to 8:00 p.m. ET by calling 855-MRM-4YOU (1-855-676-4968) or more information, visit www.livmarli.com.

Patients Enrolled in the U.S. Expanded Access Program

For patients who are currently enrolled in the U.S. Expanded Access Program (EAP), MAP coordinators will assist patients who wish to continue on LIVMARLI with the conversion to prescription LIVMARLI. The EAP will remain open for eligible patients with ALGS in Australia, Canada, the UK and several countries in Europe until LIVMARLI is approved in the respective country. For more information about the maralixibat EAP, visit algseap.com.

Investor Conference Call

Mirum will host a conference call to discuss LIVMARLI's approval in further detail today, September 29, at 4:30 p.m. ET. The live webcast and archived event will be available within the [Events section](#) on Mirum's website. To participate in the live call, dial (844) 200-6205 (toll free) or (646) 904-5544 (international) and use the passcode 986568.

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. For more information, please visit LIVMARLI.com.

LIVMARLI is the only FDA-approved medication to treat cholestatic pruritus associated with Alagille syndrome.

LIVMARLI is currently being evaluated in late-stage clinical studies in other rare cholestatic liver diseases including progressive familial intrahepatic cholestasis and biliary atresia, of which both have received Breakthrough Therapy designation and Orphan Drug designation. 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

commercialization plans and expectations for commercializing LIVMARLI in the United States, estimates of the number of patients impacted by ALGS and who are appropriate for treatment with LIVMARLI, the potential benefits or competitive position of LIVMARLI, the timing of ongoing and planned clinical trials and the regulatory approval process of maralixibat in other indications and jurisdictions and of volixibat. 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.

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

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

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

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

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