New LIVMARLI™ (maralixibat) Data to be Showcased at AASLD The Liver Meeting® 2021

November 1, 2021

- LIVMARLI (maralixibat) late-breaker oral presentation on a six-year event-free survival analysis in Alagille syndrome compared to a natural history cohort; selected for Best of the Liver Meeting

- Second late-breaker presentation to highlight predictors of event-free survival in patients treated with maralixibat.

FOSTER CITY, Calif.--(BUSINESS WIRE)--Nov. 1, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today announced that new data will be presented at the American Association for the Study of Liver Disease annual congress, The Liver Meeting®, November 12-15, 2021.

Highlights of the meeting will take place during two late-breaker sessions where new analyses from LIVMARLI™ (maralixibat) oral solution clinical studies will be presented. The late-breaker oral presentation will provide real world evidence analytics on a six-year event-free survival analysis in patients with Alagille syndrome (ALGS) treated with maralixibat compared with a natural history control cohort from the Global Alagille Alliance Study (GALA) database. The second late-breaker presentation is a poster which will highlight an analysis of predictors of long-term event-free survival and transplant-free survival in patients with ALGS enrolled in three long-term maralixibat clinical trials.

All abstracts are now available on the AASLD website and in the October supplement of *HEPATOLOGY.*

LATE-BREAKER PRESENTATIONS
Oral session
Abstract LO: Application of real-world evidence analytics: A 6-year event-free survival analysis in Alagille syndrome of the GALA clinical research database and maralixibat treated patients
Bettina E. Hansen, PhD and Binita M. Kamath, MBBChir, on behalf of the GALA study group; presented by Bettina Hansen, Senior Biostatistician, Toronto Centre for Liver Disease, Scientist, Toronto General Hospital Research Institute & Associate Professor, Institute of Health Policy, Management and Evaluation, University of Toronto
Monday, November 15, 2021 – 12:30 p.m. ET
This abstract has been selected as a part of the Best of the Liver Meeting series.

Poster session
Abstract LP16: Predictors of 6-Year event-free survival in patients with Alagille syndrome treated with maralixibat, an IBAT inhibitor
Ronald J. Sokol, MD, FAASLD, Professor of Pediatrics and Vice Chair of Clinical and Translational Research in the Department of Pediatrics at the University of Colorado School of Medicine (SOM) and Children's Hospital Colorado

POSTER PRESENTATIONS

Abstract 1955: Healthcare resource utilization in patients with Alagille syndrome
Noelle Ebel, MD, Clinical Assistant Professor, Director of the Alagille Syndrome Program, and Associate Program Director for the Transplant Hepatology Fellowship at Stanford University

Abstract 1946: Cost of pediatric liver transplant among commercial and Medicaid insured patients
Tamir Miloh, MD, Professor of Pediatrics and Medical Director of Pediatric Transplant Hepatology, Miami Transplant Institute

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, 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

This press release includes forward-looking statements pertaining to the Company's planned participation at a scientific conference, which may include discussion of the Company's business and operations, including the discovery, development and commercialization of our product candidates and technologies, and the therapeutic potential thereof, the success of our collaborations with partners and any potential future collaborations. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. Applicable risks and uncertainties include those relating to our preclinical research and clinical programs and other risks identified under the heading "Risk Factors" included in our most recent Form 10-Q and Form 10-K filings and in other future filings with the SEC. The forward-looking statements contained in this press release reflect Mirum's current views with respect to future events, and Mirum does not undertake and specifically disclaims any obligation to update any forward-looking statements.

The Liver Meeting® is a registered trademark of the American Association for the Study of Liver Diseases.

[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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