Mirum Pharmaceuticals to Present LIVMARLI™ (maralixibat) Analyses at NASPGHAN 2021 Annual Meeting

December 6, 2021

FOSTER CITY, Calif.--(BUSINESS WIRE)--Dec. 6, 2021-- Mirum Pharmaceuticals, Inc. (NASDAQ: MIRM) today announced that five posters featuring data on LIVMARLI™ (maralixibat) oral solution will be presented at the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition Annual Meeting taking place virtually December 12-18, 2021.

New LIVMARLI Analyses

Abstract #305: Response to treatment with maralixibat in Alagille syndrome is associated with improved health-related quality of life
Wednesday, December 15, 2021 from 1:30-2:15pm ET, Poster session II
By Binita M. Kamath, MBBChir, Pediatric Hepatologist, The Hospital for Sick Children (SickKids), Toronto, Ontario, Canada

Abstract #297: Maralixibat treatment response is associated with improved health-related quality of life in patients with BSEP deficiency
Wednesday, December 15, 2021 from 1:30-2:15pm ET, Poster session II
By Kathleen M. Loomes, MD, Pediatric Gastroenterologist, Division of Gastroenterology, Hepatology and Nutrition, Children's Hospital of Philadelphia

Encore presentations

Abstract #304: Pruritus intensity is associated with cholestasis biomarkers and quality of life measures after maralixibat treatment in children with Alagille syndrome
Wednesday, December 15, 2021 from 1:30-2:15pm ET during Poster session II
By Binita M. Kamath, MBBChir, Pediatric Hepatologist, The Hospital for Sick Children (SickKids), Toronto, Ontario, Canada

Abstract #548: An integrated analysis of long-term clinical safety in maralixibat-treated participants with Alagille syndrome
Friday, December 17, 2021 from 1:30-2:15pm ET, Poster session III
By Pam Vig, PhD, Head of R&D, Mirum Pharmaceuticals, Inc.

Abstract #549: Gastrointestinal tolerability of maralixibat in patients with Alagille syndrome: An integrated analysis of short- and long-term treatment
Friday, December 17, 2021 from 1:30-2:15pm ET, Poster session III
By Pam Vig, PhD, Head of R&D, Mirum Pharmaceuticals, Inc.

The abstracts are now available via the NASPGHAN website* and full presentations will be available at the start of the congress on December 12.

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 (ALGS) one year of age and older and is the only FDA-approved medication to treat cholestatic pruritus associated with ALGS. 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 (PFIC) 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.

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 three potentially registrational studies including the OHANA Phase 2b study for pregnant women with intrahepatic cholestasis of pregnancy, VISTAS Phase 2b study for adults with primary sclerosing cholangitis, and the VANTAGE Phase 2b study for primary biliary cholangitis.

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 clinical and research data, including the discovery, development and commercialization of our product candidates and technologies, and the therapeutic potential thereof, the continuation of our clinical trials, and 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.

*Abstract 305 (pages 207-208), Abstract 297 (pages 202-203), Abstract 304 (pages 206-207), Abstract 548 (page 393), Abstract 549 (pages 393-394)

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