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Mirum Announces Label Expansion for LIVMARLI in the United States to Include Infants Three Months of Age and Older

- LIVMARLI immediately available for prescribing for patients three months of age and older

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FOSTER CITY, Calif.--(BUSINESS WIRE)--Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today announced that the U.S. Food and Drug Administration (FDA) has approved a reduction in age from one year to three months for LIVMARLI® (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS). The label expansion was based on data from the RISE study which characterized the safety and tolerability of LIVMARLI in infants under one year of age with ALGS.

“The vast majority of patients are diagnosed with ALGS before one year of age. The availability of LIVMARLI will offer an opportunity to introduce a treatment at the beginning of their ALGS journey with the goal of reducing serum bile acids and alleviating the unrelenting burden caused by pruritus,” commented Chris Peetz, president and chief executive officer at Mirum. “We are grateful to the patients and families who participated in the RISE study and made this important label expansion possible.”

LIVMARLI is currently approved for the treatment of cholestatic pruritus in patients with ALGS in the U.S. (three months and older) and in Europe (two months and older). Additional information about LIVMARLI can be found on the product website, www.livmarli.com. Mirum has also submitted an sNDA in the United States for the treatment of cholestatic pruritus in patients with progressive familial intrahepatic cholestasis.

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) three months 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 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 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 gastrointestinal bleeding and bone fractures.

US Prescribing Information

EU SmPC

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 three months of age and older, and in Europe for the same indication in patients two months of age and older.

Mirum's late-stage pipeline includes two investigational treatments for debilitating liver diseases affecting children and adults. LIVMARLI, an oral ileal bile acid transporter (IBAT) inhibitor, is currently being evaluated in clinical trials for pediatric liver diseases and includes the EMBARK Phase 2b clinical trial for patients with biliary atresia. In addition, Mirum has an expanded access program open across multiple countries for eligible patients with ALGS and PFIC.

Mirum's second investigational treatment, volixibat, an oral IBAT inhibitor, is being evaluated in two potentially registrational studies including the VISTAS Phase 2b clinical trial for adults with primary sclerosing cholangitis and the VANTAGE Phase 2b clinical trial for adults with primary biliary cholangitis.

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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 expectations for commercializing LIVMARLI in United States with an expanded label, the expectations regarding the timing of diagnosis for the expanded patient population and Mirum's ability to provide LIVMARLI to that population, and the potential benefits of LIVMARLI in the expanded patient population. 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," "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.

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