Mirum Pharmaceuticals Receives Notice of Allowance for Methods of Use Patent for Maralixibat in Alagille Syndrome

April 26, 2021

- Methods of use patent expected to provide patent protection through 2040.

FOSTER CITY, Calif.--(BUSINESS WIRE)--Apr. 26, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today announced that it has received a Notice of Allowance from the United States Patent and Trademark Office (USPTO) for a patent application No. 16/994,368. The allowed patent application covers methods of treating Alagille syndrome using maralixibat, an investigational treatment being evaluated for patients with Alagille syndrome, a rare and life-threatening cholestatic liver disease. The patent application is expected to issue in 2021 and will provide patent protection in the United States of the claimed methods of use of maralixibat into 2040.

Mirum has submitted a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for maralixibat for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older. In March 2021, the NDA was accepted for priority review by the FDA and issued a PDUFA date of September 29, 2021. Maralixibat is also being evaluated in clinical studies for the treatment of patients with progressive familial intrahepatic cholestasis (PFIC) and biliary atresia, both of which are also cholestatic liver diseases.

A notice of allowance is a written notification issued after the USPTO makes a determination that a patent can be granted from an application. The majority of patent applications that receive a notice of allowance will proceed to issue as a U.S. patent. However, a notice of allowance is not a guarantee of patent issuance.

About Maralixibat

Maralixibat is a novel, minimally absorbed, orally administered investigational drug being evaluated in several rare cholestatic liver diseases. Maralixibat inhibits the apical sodium dependent bile acid transporter (ASBT), resulting in more bile acids being excreted in the feces, leading to lower levels of bile acids systemically, thereby potentially reducing bile acid mediated liver damage and related effects and complications. More than 1,600 individuals have received maralixibat, including more than 120 children who have received maralixibat as an investigational treatment for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). In the ICONIC Phase 2b ALGS clinical trial, patients taking maralixibat had significant reductions in bile acids and pruritus compared to placebo, as well as reduction in xanthomas and accelerated growth long-term. In a Phase 2 PFIC study, a genetically defined subset of BSEP deficient (PFIC2), patients responded to maralixibat with an increase in transplant-free survival. The U.S. Food and Drug Administration has granted maralixibat Breakthrough Therapy designation for the treatment of pruritus associated with ALGS in patients one year of age and older and for PFIC2. Maralixibat was generally well-tolerated throughout the studies. The most frequent treatment-related adverse events were diarrhea and abdominal pain. Until maralixibat is approved and available for prescribing, the medication is available to patients with ALGS through Mirum's expanded access program. For more information, please visit ALGSEAP.com. For more information about the Phase 3 study for maralixibat in pediatric patients with PFIC, visit PFICtrial.com.

About Mirum Pharmaceuticals, Inc.

Mirum Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of a late-stage pipeline of novel therapies for debilitating liver diseases. Mirum's lead product candidate, maralixibat, is an investigational oral drug in development for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. Mirum has submitted an NDA for maralixibat in the treatment of cholestatic pruritus in patients with ALGS. The NDA has been accepted for priority review by the FDA with a PDUFA action date of September 29, 2021. Additionally, Mirum's marketing authorization application for the treatment of pediatric patients with PFIC2 has been accepted for review (validated) by the European Medicines Agency. Mirum is also developing volixibat, also an oral ASBT-inhibitor, in primary sclerosing cholangitis, intrahepatic cholestasis of pregnancy, and primary biliary cholangitis. For more information, visit MirumPharma.com.

To augment its pipeline in cholestatic liver disease, Mirum has acquired the exclusive option to develop and commercialize two gene therapy programs, VTX-803 and VTX-802 for PFIC3 and PFIC2, respectively, from Vivet Therapeutics, following preclinical evaluation and IND/CTA-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 date Mirum's patent application is expected to issue and the duration of such patent protection in the United States. 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," "expects," "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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Media:
Erin Murphy
media@mirumpharma.com

Investors:
Ian Clements, Ph.D.
ir@mirumpharma.com

Source: Mirum Pharmaceuticals, Inc.