Mirum Pharmaceuticals and Takeda Enter into Exclusive Licensing Agreement to Develop and Commercialize Maralixibat for Rare Pediatric Liver Diseases in Japan

September 21, 2021

Partnership leverages Takeda's leadership in rare disease, gastroenterology, and hepatology to advance maralixibat in a major market

FOSTER CITY, Calif. & OSAKA, Japan--(BUSINESS WIRE)--Sep. 21, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) and Takeda Pharmaceutical Company Limited (TSE:4502/NYSE:TAK) announced that the companies have entered into an exclusive licensing agreement for the development and commercialization of maralixibat in Japan for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia (BA). Maralixibat, an investigational, orally administered medication, is being evaluated globally in ALGS, PFIC, and BA.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20210921005502/en/

Under the terms of the agreement, Takeda will be responsible for regulatory approval and commercialization of maralixibat in Japan. Takeda will also be responsible for development, including conducting clinical studies in cholestatic indications.

"Takeda is a leading global biopharmaceutical company with extensive experience in development and commercialization of novel therapies to treat rare diseases as well as gastroenterology and hepatology, making them an ideal partner as we look to accelerate the delivery of maralixibat to children living with rare liver diseases in Japan," said Chris Peetz, president and chief executive officer of Mirum. "As we approach potential commercialization in the United States and complete the recent filing for Alagille syndrome in Europe, our goal is to partner with top companies outside of North America and Europe to ensure global reach for patients with these terrible diseases. We are excited for Takeda to engage in the development of maralixibat and collaborate in our effort to advance this potentially life-changing therapy."

"There is a significant unmet medical need for a treatment to help patients with cholestatic diseases such as ALGS and PFIC in Japan and developing novel treatment for those patients suffering from rare liver diseases is a top priority for Takeda's global R&D strategy," said Dr. Naoyoshi Hirota, general manager of Takeda development center Japan. "This agreement reinforces Takeda's commitment to developing highly differentiated medicines to improve the health and quality of life of patients."

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. The NDA is currently under priority review with a PDUFA, or FDA decision date, of September 29, 2021. Mirum also recently submitted a Marketing Authorization Application to the European Medicines Agency for maralixibat for the treatment of cholestatic liver disease in patients with ALGS.

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 effects. 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. 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 shown to have a tolerable safety profile in the studies. The most frequent treatment-related adverse events were diarrhea and abdominal pain. Maralixibat has been studied extensively and its safety database represents the largest database for an ASBT inhibitor.

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 further information about maralixibat's ongoing studies in pediatric liver disease, please visit the study websites: Phase 3 MARCH study for PFIC and Phase 2b EMBARK study for biliary atresia.

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 for the treatment of cholestatic pruritus in patients with ALGS in the U.S. The NDA has been accepted for priority review by the FDA with a PDUFA action date of September 29, 2021. Additionally, Mirum has submitted a marketing authorization application for maralixibat to the European Medicines Agency for the treatment of cholestatic liver disease for patients with ALGS. 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 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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About Takeda Pharmaceutical Company Limited

Takeda Pharmaceutical Company Limited (TSE: 4502/NYSE: TAK) is a global, values-based, R&D-driven biopharmaceutical leader headquartered in Japan, committed to discover and deliver life-transforming treatments, guided by our commitment to patients, our people and the planet. Takeda focuses its R&D efforts on four therapeutic areas: Oncology, Rare Genetics and Hematology, Neuroscience, and Gastroenterology (GI). We also make targeted R&D investments in Plasma-Derived Therapies and Vaccines. We are focusing on developing highly innovative medicines that contribute to making a difference in people's lives by advancing the frontier of new treatment options and leveraging our enhanced collaborative R&D engine and capabilities to create a robust, modality-diverse pipeline. Our employees are committed to improving quality of life for patients and to working with our partners in health care in approximately 80 countries and regions.

For more information, visit https://www.takeda.com.

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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 potential development and commercialization by Takeda of maralixibat in Japan for various indications, Mirum and Takeda's receipt of revenue in connection with the license agreement with Takeda, as well as the regulatory approval pathway for maralixibat. 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.

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