Mirum Pharmaceuticals and CANbridge Pharmaceuticals Enter into Exclusive Licensing Agreement to Develop and Commercialize Maralixibat in Greater China for Rare Liver Diseases

April 29, 2021

- CANbridge to lead development and commercialization in China; expands rare disease pipeline

- Mirum is entitled to receive up to an aggregate of $120.0 million in upfront and milestone payments, and significant double-digit tiered royalties

FOSTER CITY, Calif. & BEIJING--(BUSINESS WIRE)--Apr. 29, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) and CANbridge Pharmaceuticals, Inc., announced today that they have entered into a licensing agreement, pursuant to which CANbridge has agreed to develop and commercialize maralixibat in Greater China (China, Hong Kong, Macau and Taiwan). Maralixibat, an investigational, orally administered medication, is being evaluated in Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia (BA).

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Maralixibat targets the apical sodium dependent bile acid transporter (ASBT) ultimately resulting in lower levels of bile acid systemically, which could mediate liver damage. The U.S. Food and Drug Administration (FDA) has accepted a New Drug Application (NDA) for maralixibat for the treatment of cholestatic pruritus in patients with ALGS under priority review. The European Medicines Agency is reviewing maralixibat for the treatment of PFIC2. Mirum has commenced a global Phase 2b maralixibat study (EMBARK) for the treatment of BA.

Under the terms of the licensing agreement, CANbridge has obtained the exclusive right to develop and commercialize maralixibat within the Greater China regions for ALGS, PFIC, and BA. In exchange, Mirum is entitled to receive an $11.0 million upfront payment, R&D funding, and up to $109.0 million for the achievement of future regulatory and commercial maralixibat milestones, with significant double-digit tiered royalties based on product net sales.

In collaboration with Mirum, CANbridge has agreed to oversee Mirum's clinical study sites in China, with the goal of accelerating enrollment of the global Phase 2b EMBARK study, which was recently initiated for patients with BA. CANbridge would also have the right to manufacture maralixibat in Greater China under certain conditions.

"Maralixibat has the potential to be a transformative medication for certain cholestatic liver diseases and our goal is to ensure its availability to patients globally," said Chris Peetz, president and chief executive officer at Mirum. "CANbridge is a leading rare disease company in China and with their track record of commercial success, we believe they will be a strategic partner to accelerate the global launch of maralixibat, if approved."

"We are thrilled to add maralixibat to our rare disease portfolio, as we expand into liver diseases and strengthen our pipeline with a late-stage asset in indications for which there are no approved treatments," said James Xue, Ph.D., Founder, Chairman and CEO of CANbridge Pharmaceuticals, Inc. "We are looking forward to participating in the global development of maralixibat in BA by supporting the China sites for the global Phase 2b EMBARK study, in collaboration with Mirum, and to working closely with Mirum to bring this treatment to patients and families in Greater China, where the need is great."

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 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 CANbridge Pharmaceuticals Inc.

CANbridge Pharmaceuticals Inc. is a biopharmaceutical company accelerating development and commercialization of treatments for orphan diseases and rare cancers to address unmet medical needs.

CANbridge has a global partnership with WuXi Biologics to develop and commercialize proprietary therapeutics for the treatment of rare genetic diseases. In greater China, where it is a recognized leader in rare diseases, CANbridge has an exclusive licensing agreement to commercialize Hunterase®, an enzyme replacement therapy for the treatment of Hunter syndrome (also known as mucopolysaccharidosis type II), developed by GC Pharma and marketed in more than 12 countries worldwide. CANbridge also has entered into a strategic collaboration and licensing agreement with LogicBio Therapeutics to develop, manufacture and commercialize gene therapy candidates for treatments for Fabry and Pompe diseases. CANbridge also has a collaborative agreement with the Horae Gene Therapy Center at UMass Medical School for the research and development of

gene therapies to treat rare genetic diseases.

For more on CANbridge Pharmaceuticals Inc., please go to: www.canbridgepharma.com.

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.

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 CANbridge of maralixibat in Greater China, Mirum's receipt of milestone and royalty revenue in connection with the license agreement with CANbridge, and 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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