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CymaBay Therapeutics Announces that MBX-8025 has Received European Medicines Agency PRIority MEdicines (PRIME) Designation for the Treatment of Primary Biliary Cholangitis

NEWARK, Calif., Oct. 20, 2016 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ:CBAY), a clinical-stage biopharmaceutical company focused on developing therapies to treat metabolic diseases with high unmet medical need, today announced that MBX-8025 has been granted the PRIority MEdicine (PRIME) designation by the European Medicines Agency (EMA) for the treatment of primary biliary cholangitis (PBC) in patients who do not tolerate or respond to ursodeoxycholic acid (UDCA) or do not respond to combination UDCA / obeticholic acid treatment. MBX-8025 is an orally administered potent and selective peroxisome proliferator-activated receptor delta (PPARδ) agonist currently in Phase 2 clinical development.

The PRIME program has been launched by the EMA Committee for Medicinal Products for Human Use (CHMP) to enhance support for the development of medicines that target an unmet medical need with a focus on medicines that may offer a major therapeutic advantage over existing treatments. This program is designed to provide enhanced interaction and early dialogue between developers of promising medicines and the EMA in order to optimize development plans, speed up evaluation and, thereby, help patients benefit as early as possible from therapies that may significantly improve their condition or quality of life.

“Patients will be delighted to hear that the EMA has recognized, in an ongoing manner, the unmet need for new therapies for patients with PBC. In particular, this is of note to those individuals not currently getting the optimal benefit from presently available drugs such as UDCA or obeticholic acid,” said Dr. Gideon Hirschfield, from the Centre for Liver Research, University of Birmingham. “PRIME designation of MBX-8025 reinforces the interest and potential seen for this agent going forward, providing CymaBay with high level guidance and pathway management from early phase trials through to drug approval.”

“We are very pleased that the CHMP has recognized MBX-8025 as a potential breakthrough therapy for the treatment of PBC,” said Harold Van Wart, President and Chief Executive Officer of CymaBay Therapeutics. “Participation in the PRIME program increases the opportunities for interaction and dialogue with the EMA and will allow us to optimize the development program, potentially accelerating the access of MBX-8025 to patients with PBC."

About PRIME

PRIME is a program launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need and have the potential to bring a major therapeutic advantage to patients. Through PRIME, the EMA offers early and proactive support to medicine developers to optimize the generation of robust data on a medicine’s benefits and risks and enable accelerated assessment of medicines applications. The designation provides appointment of a rapporteur, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review earlier in the application process. The scheme focuses on medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. These medicines are considered priority medicines by EMA. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data.

About MBX-8025

MBX-8025 is a potent and selective agonist of PPARδ, a nuclear receptor important for lipid transport, storage and metabolism in liver and muscle. In a Phase 2 study in subjects with mixed dyslipidemia, MBX-8025 decreased LDL-C, triglycerides and high sensitivity CRP, a biomarker of inflammation. MBX-8025 also decreased alkaline phosphatase and gamma glutamyl transferase, two key markers of cholestasis. In a recently completed Phase 2 study in subjects with primary biliary cholangitis (PBC), MBX-8025 decreased markers of cholestasis and

inflammation without appearing to cause pruritus while also lowering LDL-C. CymaBay has also completed a pilot Phase 2 clinical study showing that MBX-8025 lowers LDL-C in patients with homozygous familial hypercholesterolemia (HoFH). The U.S. Food and Drug Administration (FDA) has granted CymaBay orphan drug designation for MBX-8025 as a treatment for HoFH and Fredrickson types I and V hyperlipoproteinemia.

About PBC

Primary biliary cholangitis (PBC), formerly known as primary biliary cholestasis, is a serious and potentially life threatening autoimmune disease of the liver characterized by impaired bile flow (cholestasis) and accumulation of toxic bile acids. There is an accompanying inflammation and destruction of the intrahepatic bile ducts which can progress to fibrosis, cirrhosis and liver failure. Other clinical symptoms of PBC include fatigue and pruritus, which can be quite disabling in some patients. PBC is primarily a disease of women, afflicting approximately one in 1,000 over the age of 40.

About CymaBay

CymaBay Therapeutics, Inc. (CBAY) is a clinical-stage biopharmaceutical company focused on developing therapies to treat metabolic diseases with high unmet medical need, including serious rare and orphan disorders. MBX-8025 is a potent, selective, orally active PPARδ agonist. A Phase 2 study of MBX-8025 in patients with mixed dyslipidemia established that it has an anti-atherogenic lipid profile. CymaBay has completed Phase 2 studies for MBX-8025 in subjects with primary biliary cholangitis and homozygous familial hypercholesterolemia, establishing proof-of-concept in both indications. Arhalofenate, CymaBay's other product candidate, is a potential Urate-Lowering Anti-Flare Therapy that has completed five Phase 2 studies in subjects with gout. Arhalofenate has been found to reduce painful flares in joints while at the same time lowering serum uric acid by promoting excretion of uric acid by the kidney. This dual action addresses both the signs and symptoms of gout while managing the underlying pathophysiology of hyperuricemia.

Cautionary Statements

The statements in this press release regarding the potential future performance of CymaBay's product candidates, are forward looking statements that are subject to risks and uncertainties. Actual results and the timing of events regarding the further development of CymaBay's product candidates could differ materially from those anticipated in such forward-looking statements as a result of risks and uncertainties, which include, without limitation, risks related to: the success, cost and timing of any of CymaBay's product development activities, including clinical trials of MBX-8025 and arhalofenate; effects observed in trials to date which may not be repeated in the future; any delays or inability to obtain or maintain regulatory approval of CymaBay's product candidates in the United States or worldwide; and the ability of CymaBay to obtain sufficient financing to complete development, regulatory approval and commercialization of its product candidates in the United States and worldwide. Additional risks relating to CymaBay are contained in CymaBay's filings with the Securities and Exchange Commission, including without limitation its most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q and other documents subsequently filed with or furnished to the Securities and Exchange Commission. CymaBay disclaims any obligation to update these forward-looking statements except as required by law.

For additional information about CymaBay visit www.cymabay.com.

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