November 10, 2015

CymaBay Therapeutics Announces the Initiation of a Phase 2 Study of MBX-8025 in Patients With Primary Biliary Cholangitis/Cirrhosis

NEWARK, Calif., Nov. 10, 2015 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (Nasdaq:CBAY), a clinical-stage biopharmaceutical company developing therapies to treat metabolic diseases with high unmet medical need, today announced the initiation of a Phase 2 study of MBX-8025 in patients with primary biliary cholangitis. MBX-8025 is an orally administered potent and selective peroxisome proliferator-activated receptor delta (PPARδ) agonist.

Primary biliary cholangitis (PBC), formerly referred to as primary biliary cirrhosis, is an orphan autoimmune disease affecting primarily women over the age of 40. Patients with PBC often experience fatigue and pruritus. The disease is characterized by inflammation and the immune-mediated destruction of the small intrahepatic bile ducts resulting in the reduction or stoppage of bile flow, a condition referred to as cholestasis. The accompanying build-up of bile acids leads to chronic liver inflammation and fibrosis that may progress to cirrhosis and hepatic failure. The only approved drug for PBC is ursodiol. However, approximately 40% of patients do not respond to ursodiol adequately and would benefit from a newer therapy.

"I am pleased to report that we initiated a placebo-controlled, dose-ranging Phase 2 study for MBX-8025 in patients with PBC," said Harold Van Wart, Ph.D., President and Chief Executive Officer of CymaBay. In this study, patients who have had an inadequate response to ursodiol will be enrolled and randomized to receive either placebo or MBX-8025 (either 50 mg or 200 mg) for 12 weeks. The primary endpoint will be the change in alkaline phosphatase, a parameter that has been used in prior clinical studies with PBC and which is believed to reflect the status of the disease. A variety of secondary outcomes will also be studied. The study is designed to enroll approximately 75 patients in the U.S., U.K., Canada, Germany and Poland and is expected to be completed around the end of 2016.

"PBC is a rare and serious liver disease for which there remains a significant unmet medical need," said Dr. Keith D. Lindor, M.D., Executive Vice Provost and Dean, College of Health Solutions, Arizona State University. "MBX-8025 is a potent, selective PPARδ agonist that has shown improvements in markers of biliary health in an earlier clinical study of patients with mixed dyslipidemia and we hope it will also be seen in patients with PBC."

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. MBX-8025 has shown favorable effects on lipid and other metabolic parameters in a Phase 2 study in patients with mixed dyslipidemia. Treatment effects observed include lowering of LDL-C with selective depletion of pro-atherogenic dense LDL-C particles, decreases in triglycerides and increases in HDL, as well as decreases in hsCRP, a biomarker of cardiovascular and systemic inflammation. MBX-8025 also decreased levels of alkaline phosphatase and gamma glutamyl transferase, which are markers of biliary health. CymaBay has initiated a pilot Phase 2 clinical study evaluating the activity of MBX-8025 in patients with homozygous familial hypercholesterolemia (HoFH). The U.S. Food and Drug Administration (FDA) has granted the Company orphan drug designation for MBX-8025 as a treatment for HoFH and Fredrickson types I and V hyperlipoproteinemia. CymaBay has also initiated a Phase 2 study in patients with primary biliary cholangitis.

About CymaBay

CymaBay Therapeutics, Inc. (CBAY) is a clinical-stage biopharmaceutical company developing therapies to treat metabolic diseases with high unmet medical need, including serious rare and orphan disorders. Arhalofenate has shown two therapeutic actions in a single drug in multiple Phase 2 gout studies. In gout patients, arhalofenate is intended to prevent painful flares in joints while at the same time promoting excretion of uric acid by the kidney, thereby addressing both the signs and symptoms of gout and the hyperuricemia that is the root cause of the disease. 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 two ongoing clinical studies for MBX-8025 including a pilot Phase 2 study in patients with homozygous familial hypercholesterolemia and

a Phase 2 study in patients with primary biliary cholangitis.

Cautionary Statements

The statements in this press release, including those statements regarding the structure and conduct of clinical trials, future performance of CymaBay's product candidates, the potential of MBX-8025 to treat primary biliary cholangitis, the therapeutic and commercial potential of arhalofenate and MBX-8025, and the therapeutic and commercial potential of the product candidates of CymaBay Therapeutics, Inc., are forward looking statements that are subject to risks and uncertainties. Actual results and the timing of events regarding the further development of arhalofenate and MBX-8025 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 arhalofenate and MBX-8025; effects observed in trials to date 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; the ability of CymaBay to attract funding partners or collaborators with development, regulatory and commercialization expertise; 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 Quarterly Report on Form 10-Q, filed with the Securities and Exchange Commission on August 11, 2015. CymaBay disclaims any obligation to update these forward-looking statements except as required by law.

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

CONTACT: Sujal Shah
CymaBay Therapeutics, Inc.
(510) 293-8800
Investors@CymaBay.com

Hans Vitzthum
LifeSci Advisors, LLC
212-915-2568
Hans@LifeSciAdvisors.com

Source: CymaBay Therapeutics, Inc.