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CymaBay Therapeutics Announces U.S. Orphan Drug Designation for MBX-8025 for the Treatment of Primary Biliary Cholangitis

NEWARK, Calif., Nov. 10, 2016 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ:CBAY), a clinical-stage biopharmaceutical company focused on developing therapies for indications with high unmet medical need, including rare and orphan diseases, announced that the U.S. Food and Drug Administration (FDA) has granted the Company orphan drug designation for MBX-8025 to treat patients with primary biliary cholangitis (PBC). MBX-8025 is a potent and selective peroxisome proliferator-activated receptor delta (PPARδ) agonist being evaluated by CymaBay in high unmet need and orphan diseases. The FDA has also granted orphan drug designation for MBX-8025 to treat patients with homozygous familial hypercholesterolemia (HoFH) and hyperlipoproteinemia types I or V (Fredrickson classification).

Orphan drug designation was created to encourage the development of products that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions. Among other benefits, the designation potentially qualifies the sponsor for seven years marketing exclusivity period upon approval, as well as exemption from FDA application fees, and tax credits for qualified clinical trials.

"This orphan drug designation is a validation of the company's development strategy for MBX-8025 in which we are targeting indications with high unmet need and potentially expedited approval pathways," said Dr. Harold Van Wart, President and Chief Executive Officer of CymaBay. "MBX-8025 potentially offers unique benefits for the treatment of PBC and we remain on track to initiate a follow on Phase 2 study of MBX-8025 in PBC this quarter."

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 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 PBC, HoFH and Fredrickson types I and V hyperlipoproteinemia.

About CymaBay

CymaBay Therapeutics, Inc. (CBAY) is a clinical-stage biopharmaceutical company developing therapies to treat diseases with high unmet medical need, including serious rare and orphan disorders. MBX-8025 is a potent, selective, orally active PPARδ agonist. CymaBay has recently completed a Phase 2 study of MBX-8025 in patients with primary biliary cholangitis as well as a pilot Phase 2 study in patients with homozygous familial hypercholesterolemia, establishing proof-of-concept in both indications. Previously, a Phase 2 study of MBX-8025 in patients with mixed dyslipidemia established that it has an anti-atherogenic lipid profile. Arhalofenate, CymaBay's other product candidate, is a potential Urate-Lowering Anti-Flare Therapy that has completed five Phase 2 studies in gout patients. Arhalofenate has been found to reduce painful flares in joints while at the same time 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, 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 or any other indication, the benefits of orphan drug designation, the therapeutic and commercial potential of CymaBay's product candidates, and any of the targeted indications for the potential future development or commercialization 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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