April 22, 2015

CymaBay Therapeutics Announces U.S. Orphan Drug Designation for MBX-8025 in Severe Hypertriglyceridemia

NEWARK, CA -- (Marketwired) -- 04/22/15 -- CymaBay Therapeutics, Inc.(NASDAQ: CBAY) today announced that the U.S. Food and Drug Administration (FDA) has granted the Company orphan drug designation for MBX-8025 to treat patients with hyperlipoproteinemia types I or V (Fredrickson classification). 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. Recently, the FDA also granted orphan drug designation for MBX-8025 to treat patients with homozygous familial hypercholesterolemia (HoFH).

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 second 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 Harold Van Wart, President and Chief Executive Officer of CymaBay. "MBX-8025 potentially offers unique benefits for the treatment of metabolic disorders including HoFH, severe hypertriglyceridemia, primary biliary cirrhosis and nonalcoholic steatohepatitis. We remain on track to initiate a Phase 2 pilot study of MBX-8025 in HoFH this quarter, and look forward to announcing further details regarding the expansion of our development strategy of MBX-8025 in the near-term."

Patients with types I and V hyperlipoproteinemias have severely elevated levels of triglycerides in the blood. Type I is characterized by deficiencies related to lipoprotein lipase (LPL) and by an elevation of chylomicron particles. Type V is characterized by an elevation in very-low-density lipoprotein (VLDL). Both types of hyperlipoproteinia are associated with an increased risk of acute and chronic pancreatitis that is severe and potentially life-threatening. The treatment goal for patients is to reduce their triglycerides, chylomicrons and VLDL to reduce the risk of severe clinical events.

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 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 high density lipoprotein, as well as decreases in hsCRP, a biomarker of cardiovascular inflammation. CymaBay is in the process of initiating a pilot clinical study evaluating the activity of MBX-8025 in patients with homozygous familial hypercholesterolemia.

About CymaBay

CymaBay Therapeutics, Inc. (NASDAQ: 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, the company's lead product candidate, 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. CymaBay's second product candidate, 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 is in the process of initiating a pilot study of MBX-8025 in patients with homozygous familial hypercholesterolemia.

Cautionary Statements

The statements in this press release, including but not limited to the statements regarding the potential of MBX-8025 in the treatment of patients with severe hypertriglyceridemia, HoFH or any other indication, the therapeutic and commercial potential of MBX-8025, the benefits of orphan drug designation, and the anticipated timing and therapeutic and commercial potential of MBX-8025 or other 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 MBX-8025 and other product candidates of CymaBay 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; 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; 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; and the market potential for CymaBay's product candidates. Additional risks relating to CymaBay are contained in CymaBay's Annual Report on Form 10-K, filed with the Securities and Exchange Commission on March 23, 2015. CymaBay disclaims any obligation to update these forward-looking statements except as required by law.

For additional information about CymaBay visitwww.cymabay.com.

Contact:
Sujal Shah
CymaBay Therapeutics, Inc.
(510) 293-8800
investors@cymabay.com

Source: CymaBay Therapeutics