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CymaBay Therapeutics Announces the Initiation of a Phase 2 Study of MBX-8025 in Patients With Homozygous Familial Hypercholesterolemia

NEWARK, CA -- (Marketwired) -- 04/23/15 -- 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 homozygous familial hypercholesterolemia (HoFH). MBX-8025 is an orally administered potent and selective peroxisome proliferator-activated receptor delta (PPARδ) agonist.

HoFH is a rare, life-threatening, autosomal genetic disease characterized by loss-of-function mutations in both alleles of the LDL receptor (LDL-R) gene. The accompanying loss of LDL-R activity results in marked elevations in the plasma levels of LDL cholesterol (LDL-C), causing premature cardiovascular disease that often presents during the first decades of life and which can result in myocardial infarction, ischemic stroke and premature death. The Phase 2 trial is an open label, dose-escalation study that will target enrollment of 8 patients at sites in Europe and North America. Following enrollment, patients will initially receive a 50 mg dose of MBX-8025 once daily that will be increased first to 100 mg and eventually 200 mg of MBX-8025 over the course of 3 months.

"HoFH is a rare and serious disease associated with early morbidity and mortality," said Harold Van Wart, president and chief executive officer of CymaBay. "Because patients with HoFH do not have fully functional LDL receptors, conventional lipid lowering therapy is minimally effective. Moreover, recently approved therapies for HoFH that reduce LDL-C by mechanisms independent of the LDL-R have significant limitations and the unmet need for this disease remains high. Clinical and preclinical results for MBX-8025 indicate that it offers the potential to significantly lower LDL-C in patients with HoFH while exhibiting markedly improved tolerability over current therapies. We look forward to the outcome of this study around the end of this year."

In a clinical study conducted in patients with mixed dyslipidemia, MBX-8025 was shown to reduce LDL-C. Data from a preclinical model of human HoFH indicate that MBX-8025 lowers LDL-C in the absence of a fully functional LDL-R. The U.S. Food and Drug Administration (FDA) has granted the Company orphan drug designation for MBX-8025 for the treatment of HoFH. MBX-8025 has also received orphan designation for Fredrickson types I and V hyperlipoproteinemia.

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 has initiated a pilot clinical study evaluating the activity of MBX-8025 in patients with homozygous familial hypercholesterolemia. The U.S. Food and Drug Administration (FDA) has granted CymaBay orphan drug designation for MBX-8025 as a treatment for homozygous familial hypercholesterolemia (HoFH) and Fredrickson types I and V hyperlipoproteinemia.

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 has initiated 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 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.

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