

March 25, 2015



## CymaBay Therapeutics Announces U.S. Orphan Drug Designation for MBX-8025 in Homozygous Familial Hypercholesterolemia

NEWARK, CA -- (Marketwired) -- 03/25/15 -- 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 as a treatment for homozygous familial hypercholesterolemia (HoFH). MBX-8025 is a potent and selective peroxisome proliferator-activated receptor delta (PPAR $\delta$ ) agonist being evaluated in high unmet need and orphan diseases.

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 qualifies the sponsor for a potential seven years marketing exclusivity period upon approval, as well as exemption of FDA application fees and tax credits for qualified clinical trials.

"Orphan designation is an important milestone for CymaBay as we advance the development of MBX-8025 in HoFH," said Harold Van Wart, President and Chief Executive officer of CymaBay. "As a potent, selective PPAR $\delta$  agonist, we believe MBX-8025 may provide meaningful clinical benefit to patients across a number of diseases and disorders, including HoFH, primary biliary cirrhosis, severe refractory hypertriglyceridemia and nonalcoholic steatohepatitis, or NASH. As we move into a Phase 2 pilot study of MBX-8025 in HoFH in the first half of this year, we also look forward to providing additional guidance on the expansion of our development strategy into a second indication."

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. This loss of LDL-R activity results in marked elevations in the plasma levels of LDL cholesterol (LDL-C) in patients with HoFH, 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. In clinical studies of patients with mixed dyslipidemia, MBX-8025 has been shown to reduce LDL-C. Further, the Company recently announced data demonstrating LDL-C lowering activity independent of fully functional LDL-R using MBX-8025 in a preclinical model of human HoFH.

CymaBay is planning a pilot Phase 2 study of MBX-8025 in HoFH starting in the first half of 2015.

### **About MBX-8025**

MBX-8025 is a potent and selective agonist of PPAR $\delta$ , 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 PPAR $\delta$  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 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 visit [www.cymabay.com](http://www.cymabay.com).

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Source: CymaBay Therapeutics