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CymaBay Therapeutics Announces IND to Commence a Clinical Study of Seladelpar to Treat Primary Sclerosing Cholangitis

NEWARK, Calif., June 18, 2019 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ: CBAY), a biopharmaceutical company focused on developing and providing access to innovative therapies for patients with liver and other chronic diseases, today announced FDA clearance of the company's Investigational New Drug Application (IND) for seladelpar to treat primary sclerosing cholangitis (PSC). The company intends to initiate a Phase 2 study to evaluate the safety, tolerability, and efficacy of seladelpar in patients with PSC in the third quarter.

PSC is a rare, chronic cholestatic liver disease that is characterized by diffuse inflammation and fibrosis of the bile ducts. The disease predominantly affects the medium to large-sized bile ducts inside and outside the liver and is manifested by ongoing ductal destruction leading to cholestasis, advanced fibrosis, and cirrhosis. There are currently no FDA-approved treatments for PSC. Seladelpar is an orally administered, potent and selective peroxisome proliferator-activated receptor delta (PPARδ) agonist that is also in development for primary biliary cholangitis (PBC) and nonalcoholic steatohepatitis (NASH). In clinical studies, seladelpar has demonstrated anti-cholestatic and anti-inflammatory activity, suggesting the potential of seladelpar as a therapeutic option for the treatment of PSC.

The planned Phase 2 study will be a randomized, placebo-controlled, dose-ranging study that will enroll approximately 100 patients at 60 sites globally. Seladelpar at doses of 5, 10, and 25 mg once daily will be studied versus placebo in a 1:1:1:1 randomization. The primary efficacy outcome will be the relative change in alkaline phosphatase (AP) from baseline at 24 weeks.

"PSC patients currently have significant unmet need for effective treatment options. I am encouraged by the effects of seladelpar observed in the PBC clinical program, such as the anti-cholestatic and anti-inflammatory activity as well as the favorable safety and tolerability profiles, which suggest a potential benefit for PSC patients," said Dr. Cynthia Levy, Division of Hepatology, University of Miami.

Dr. Pol Boudes, Chief Medical Officer of CymaBay, commented, "The initiation of a Phase 2 study of seladelpar in PSC is a significant step forward in addressing the high unmet need that exists for PSC patients. As we continue to progress the Phase 3 development of seladelpar in PBC, it is important to expand the potential indications where the drug may have clinical activity. We are eager to commence this dose-ranging study to further evaluate our hypothesis that seladelpar can improve the clinical course for patients with PSC."

About PSC

Primary Sclerosing Cholangitis (PSC) is a chronic cholestatic liver disease that is characterized by diffuse inflammation and fibrosis of the bile ducts. The ongoing destruction of the bile ducts can lead to cholestasis, advanced fibrosis, and cirrhosis. Disease

progression will eventually lead to liver failure with consequent complications such as portal hypertension and increased risk of malignancy, including hepatocellular carcinoma (HCC) and cholangiocarcinoma. Other clinical symptoms of PSC include fatigue and pruritus. Males are affected twice as often as females and an estimated 70% of PSC patients have concomitant inflammatory bowel disease, particularly ulcerative colitis.

About Seladelpar
Seladelpar is a potent, selective, orally active PPARδ agonist that is in development for the treatment of the liver diseases PBC and NASH. For PBC, seladelpar has received an orphan designation from the US Food and Drug Administration (FDA) and the European Medicine Agency (EMA). Seladelpar also received Breakthrough Therapy Designation from the FDA and PRIority MEdicine status from the EMA for PBC.

About CymaBay
CymaBay Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on developing therapies for liver and other chronic diseases with high unmet medical need. CymaBay's lead development candidate, seladelpar, is a potent, selective and orally active PPARδ agonist currently in development for the treatment of patients with primary biliary cholangitis (PBC), an autoimmune liver disease, and with nonalcoholic steatohepatitis (NASH). Two Phase 2 studies of seladelpar established proof-of-concept in PBC. CymaBay is currently enrolling patients in a global, Phase 3 registration study of seladelpar for PBC. This study is a 52-week, plac**E**bo-co**N**trolled, randomized, p**HA**se 3 study to evaluate the safety a**N**d effi**C**acy of s**E**ladelpar (ENHANCE) in patients with PBC. CymaBay is also conducting a Phase 2b proof-of-concept study of seladelpar in patients with NASH.

Cautionary Statements
The statements in this press release regarding the potential for seladelpar to treat PBC, PSC and NASH, the potential benefits to patients, CymaBay's expectations and plans regarding future clinical trials, including the timing of future trials, and CymaBay's ability to fund current and planned clinical trials are forward looking statements that are subject to risks and uncertainties. Actual results and the timing of events regarding the further development of seladelpar 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; effects observed in trials to date that 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 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 visitwww.cymabay.com.
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