CymaBay Therapeutics Announces Seladelpar Granted Breakthrough Therapy Designation by the FDA for the Treatment of Primary Biliary Cholangitis

NEWARK, Calif., Feb. 15, 2019 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ: CBAY), today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for seladelpar for the treatment of early stage primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adult patients with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA. Seladelpar is an orally administered, potent and selective peroxisome proliferator-activated receptor delta (PPARδ) agonist currently being evaluated in a global, Phase 3 registration study, ENHANCE, for patients with PBC.

Breakthrough Therapy Designation is granted by the FDA to investigational agents intended to treat a serious or life-threatening disease or condition and whose preliminary clinical evidence may demonstrate substantial improvement on at least one clinically significant endpoint over available therapy. This program was designed by the FDA to help ensure patients gain access to important new therapies through FDA approval as soon as possible.

The Breakthrough Therapy Designation of seladelpar was granted based on preliminary evidence from the ongoing Phase 2 clinical trial (CB8025-21629), which indicates that seladelpar may demonstrate substantial improvement over existing therapy based on a reduction in alkaline phosphatase (AP). Preliminary results from the trial have been recently presented at late breaking sessions of the American Association for the Study of Liver Diseases (AASLD) and the International Liver Congress (EASL).

"We are very pleased that the FDA has recognized seladelpar as a potential breakthrough therapy for the treatment of PBC, which recognizes the disease as a serious condition for which new treatments are still urgently needed," said Klara Dickinson, Chief Regulatory and Compliance Officer of CymaBay Therapeutics. "Seladelpar has now received both the FDA Breakthrough Designation and the corresponding PRIority MEdicine (PRIME) designation by the European Medicines Agency, which we believe reflects the significance of the seladelpar clinical data and the potential for seladelpar to be an improved treatment alternative for patients with PBC. We look forward to working closely with regulators to complete the seladelpar development program. These designations afford us an opportunity to accelerate the access of a new and innovative treatment option for patients with PBC."

About PBC
Primary biliary cholangitis (PBC) 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: 1 in 1000 women over the age of 40 lives with PBC.

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 U.S. Food and Drug Administration (FDA) and the European Medicine Agency (EMA). Seladelpar also received Breakthrough Therapy Designation from the FDA and PRIority MEdicine (PRIME) status from the EMA for PBC.

About ENHANCE
ENHANCE (NCT03602560) is a 52-week, placebo-controlled, randomized, Phase 3 study to evaluate the safety and efficacy of seladelpar. It will be conducted in more than 20 countries over five continents (North America, South America, Europe, Australasia and Asia). Approximately 240 PBC patients will be randomized to seladelpar 10 mg/day, seladelpar 5/10 mg/day (starting treatment at 5 mg with the possibility to escalate dose to 10 mg after 6 months), or placebo. Patients must experience an inadequate response to UDCA (defined as a serum alkaline phosphatase level ≥ 1.67 x the upper limit of normal after at least 12 months of treatment) or an intolerance to UDCA to be eligible for the study. Patients who are inadequate responders to UDCA will continue their treatment during the study, and UDCA will be provided free of charge. The primary outcome measure is the responder rate after 52 weeks. A responder is defined as a patient who achieves an alkaline phosphatase level < 1.67 x the upper limit of
normal with at least a 15% decrease from baseline and has a normal level of total bilirubin. Additional key outcomes of efficacy will compare the rate of normalization of alkaline phosphatase at 52 weeks and the level of pruritus at 6-months assessed by a numerical rating scale recorded with an electronic diary. Additional information can be found at https://www.clinicaltrials.gov/ct2/show/NCT03602560?term=seladelpar&rank=2. After completing the study, patients will be offered to continue treatment in an open label extension study. Patients on placebo will be offered to start seladelpar in the extension study.

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. Seladelpar is a potent, selective, 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, placebo-controlled, randomized, Phase 3 study to evaluate the safety and efficacy of seladelpar (ENHANCE) in patients with PBC. CymaBay is also conducting a Phase 2b proof-of-concept study of seladelpar for patients with NASH.

Cautionary Statements
The statements in this press release regarding the potential for seladelpar to treat PBC, the potential benefits to patients, CymaBay's expectations and plans regarding future clinical 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 visit www.cymabay.com.

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