August 1, 2022

CymaBay Completes Enrollment for the RESPONSE Global Phase 3 Study Evaluating Seladelpar for Patients with Primary Biliary Cholangitis

NEWARK, Calif., Aug. 01, 2022 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ: CBAY), a clinical-stage biopharmaceutical company focused on developing therapies for liver and other chronic diseases with high unmet need, today announced the completion of enrollment for RESPONSE, a global Phase 3 study evaluating seladelpar for patients with Primary Biliary Cholangitis (PBC).

RESPONSE is CymaBay's pivotal registrational study evaluating the safety and efficacy of seladelpar for patients with PBC who have been using ursodeoxycholic acid (also known as UDCA or ursodial), but have not achieved the recommended treatment goal or who cannot tolerate UDCA. RESPONSE is a 52-week, placebo-controlled, randomized study that targeted enrollment of 180 patients recruited in over 20 countries.

"Patients with PBC are in need of new therapies that provide greater safety, efficacy and relief from symptoms which can significantly diminish the quality of their lives," said Dr. Marlyn J. Mayo, MD, Professor of Internal Medicine at the University of Texas Southwestern Medical Center and an investigator in the RESPONSE study. "RESPONSE provides an opportunity to gather the scientific evidence for seladelpar's potential to change the treatment paradigm for patients with PBC."

Dr. Dennis Kim, Chief Medical Officer of CymaBay, commented, "We are so thankful to the patients and investigators who were instrumental to achieving this important milestone. Completing study enrollment in the midst of the COVID-19 pandemic reflects the enormous level of commitment by our study sites around the world as well as PBC patients who desire better treatment options. We look forward to announcing results next year with the goal of advancing care and quality of life for patients with PBC."

About PBC
PBC is a rare, chronic inflammatory liver disease primarily affecting women (1 in 1,000) over the age of 40. PBC is characterized by impaired bile flow (known as cholestasis) and the accumulation of toxic bile acids in the liver, leading to inflammation and destruction of the bile ducts within the liver and causing increased levels of alkaline phosphatase (ALP) and total bilirubin. The most common early symptoms of PBC are itching (pruritus) and fatigue, which can be very debilitating for some patients. Progression of PBC is associated with an increased risk of liver cancer and liver-related mortality.

About RESPONSE
RESPONSE (NCT04620733) is a 52-week, placebo-controlled, randomized, global phase 3 study to evaluate the safety and efficacy of seladelpar in patients with primary biliary cholangitis (PBC). The study is being conducted in more than 20 countries over five

continents (North America, South America, Europe, Australia and Asia). Approximately 180 PBC patients were intended to be randomized to seladelpar 10 mg/day, or placebo. Patients must have 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 UDCA treatment during the study. The primary outcome measure will be the responder rate at 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 for patients with moderate to severe pruritus at baseline assessed by a numerical rating scale recorded with an electronic diary. Additional information can be found at https://www.clinicaltrials.gov/. After completing the study, patients will be able to continue treatment in **ASSURE**, an open-label, long-term study intended to collect additional long-term safety and efficacy data to support registration. Patients on placebo will be able to start seladelpar treatment in the **ASSURE** study.

About Seladelpar
Seladelpar is a first-in-class oral, selective PPARδ agonist shown to regulate critical metabolic and liver disease pathways in indications with high unmet medical need. Preclinical and clinical data support its ability to regulate genes involved in bile acids synthesis, inflammation, fibrosis and lipid metabolism, storage and transport.

About CymaBay
CymaBay Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on improving the lives of people with liver and other chronic diseases that have high unmet medical need through a pipeline of innovative therapies. Our deep understanding of the underlying mechanisms of liver inflammation and fibrosis, and the unique targets that play a role in their progression, have helped us receive breakthrough therapy designation (U.S. Food and Drug Administration), PRIority MEdicines status (European Medicines Agency) and orphan drug status (U.S. and Europe) for seladelpar, a first-in-class treatment for people with primary biliary cholangitis (PBC). Our evidence-based decision-making and commitment to the highest quality standards reflect our relentless dedication to the people, families and communities we serve. To learn more, visit www.cymabay.com and follow us on Twitter and Linkedin.

Cautionary Statements
Any statements made in this press release regarding the potential for seladelpar to treat PBC and potentially improve clinical symptoms of the disease and the potential benefits to patients, as well as the timing of the release of data from RESPONSE 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; and effects observed in trials to date that may not be repeated in the future. 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, its Quarterly Reports on Form 10-Q 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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Source: CymaBay Therapeutics, Inc.