Seladelpar Granted Revised Breakthrough Therapy Designation for the Treatment of Primary Biliary Cholangitis Including Pruritus in Patients Without Cirrhosis or With Compensated Cirrhosis

NEWARK, Calif., Oct. 23, 2023 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ: CBAY), a biopharmaceutical company focused on innovative therapies for patients with liver and other chronic diseases, today announced that the U.S. Food and Drug Administration (FDA) has revised the originally granted Breakthrough Therapy Designation for seladelpar to now reflect treatment of primary biliary cholangitis (PBC) including pruritus in adults without cirrhosis or with compensated cirrhosis (Child Pugh A). Seladelpar is the only potent, selective, orally active PPARδ agonist, or delpar, with phase 3 results demonstrating a statistically significant improvement in PBC-related cholestatic pruritus.

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 FDA’s original Breakthrough Therapy Designation for seladelpar was granted in 2019 and was based on preliminary results from a Phase 2 clinical trial (CB8025-21629), which showed that seladelpar was associated with a substantial reduction in serum alkaline phosphatase (ALP). The updated Breakthrough Therapy Designation of seladelpar was granted based on additional evidence provided to the Agency, which supports that seladelpar may provide substantial improvement over existing therapy based on a reduction in alkaline phosphatase (ALP) and pruritus in patients without cirrhosis or with compensated cirrhosis.

“The decision by the FDA to grant Breakthrough Therapy designation emphasizes the significant impact of pruritus on day-to-day functioning for people living with PBC and underscores the potential of seladelpar to help fill a critical need for a treatment that both significantly reduces markers of cholestasis and pruritus in patients with PBC, including those with compensated cirrhosis,” said Klara Dickinson, Chief Regulatory and Compliance Officer of CymaBay Therapeutics. “A Breakthrough Therapy designation is accompanied by the benefit to submit the NDA on a rolling basis. We look forward to working closely with the FDA during the review process and remain focused on bringing seladelpar to patients with PBC as quickly as possible.”

About PBC
PBC is a rare, chronic inflammatory liver disease primarily affecting women (1 in 1,000...
women over the age of 40 or about 130,000 total people in the US). 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 ALP and total bilirubin. The most common early symptoms of PBC are pruritus (itching) and fatigue, which can be debilitating for some patients. Progression of PBC is associated with an increased risk of liver-related mortality.

About Seladelpar
Seladelpar, an investigational treatment for people with PBC, is a first-in-class oral, selective peroxisome proliferator-activated receptor (PPAR) delta agonist, or delpar, 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 acid 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 investigational treatment for people with 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 X (formerly 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 or outcomes of the disease, the potential benefits to patients and the future filing and commercialization plans of CymaBay 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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