CymaBay Therapeutics Presents New Findings that Seladelpar Treatment of PBC Patients for Two Years Predicts Improved Transplant-Free Survival

NEWARK, Calif., May 23, 2022 (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 that results from new analyses of clinical studies of seladelpar were presented during Digestive Disease Week® (DDW).

An oral presentation was made during the AALSD Presidential Plenary session of DDW titled “Seladelpar Treatment of Patients With Primary Biliary Cholangitis (PBC) For 2 Years Improves the GLOBE PBC Score and Predicts Improved Transplant-Free Survival” by Dr. Bettina Hansen, PhD, Associate Professor of Biostatistics at the University of Toronto, Toronto, CA and Erasmus University Medical Center, Rotterdam, NL. Data were presented demonstrating that seladelpar treatment throughout 2 years resulted in a decrease in PBC GLOBE score and predicted improved transplant-free survival. The GLOBE score is a validated risk assessment tool providing an estimate of transplant-free survival for patients with PBC. A GLOBE score above > 0.3 has significant risk for needing a liver transplant or death, whereas a score ≤ 0.3 has a risk that can’t be distinguished from a matched population. In this analysis, the GLOBE score was used to measure response to seladelpar treatment for predicting long-term survival outcomes for patients with PBC.

Patients with PBC having an incomplete response or intolerance to UDCA (defined as alkaline phosphatase (ALP) ≥ 1.67xULN) had completed an open-label one-year phase 2 study of daily oral seladelpar therapy (NCT: 02955602). After 1 year, patients were eligible for an open label long-term study (NCT: 03301506). Treatment of 50 patients with oral seladelpar 5 mg or 10 mg daily for 2 years resulted in a mean (SD) change from baseline in GLOBE score of -0.417 (0.269), resulting in a corresponding hazard ratio of 0.66 for transplantation or death compared to no prior treatment (baseline). The improvement in GLOBE score and predicted survival did not depend on age. However, an analysis of subpopulations of high risk patients by GLOBE score (> 0.3) revealed that while patients of all ages improved, the younger patients tended to have numerically greater improvements, although these differences did not achieve significance.

“These results demonstrate that seladelpar treatment over 2 years resulted in a sustained and progressive improvement in GLOBE score and predicted survival outcomes for patients with PBC, which is especially important for patients with high risk GLOBE scores. The age related pattern in high risk patients suggested by our analysis points toward the need to
examine earlier interventions, especially in younger patients, as a strategy to improve survival," said Dr. Bettina Hansen.

A second clinical presentation titled “Efficacy, Safety, and Tolerability of Seladelpar in Patients With Compensated Liver Cirrhosis Due to Primary Biliary Cholangitis (PBC): A Pooled Analysis of Phase 2 and Phase 3 Studies” was delivered by Dr. Stuart Gordon, MD, Professor of Medicine at Wayne State University and Director of the Division of Hepatology and GI Research at Henry Ford Health System. The results from a pooled analysis of Phase 2 and Phase 3 studies (n=384) treating compensated cirrhotic patients (n=53) with daily oral seladelpar at 5 and 10 mg for 3 months were presented as a poster. At 3 months, seladelpar 5 and 10 mg treatment led to reductions in ALP of -31% and -41%, respectively, with a -2.6% change in placebo-treated patients. A change in ALT of up to -32% was observed (10 mg seladelpar group) while bilirubin, platelets, albumin, and INR remained stable. Seladelpar appeared safe and well-tolerated. Efficacy, safety, and tolerability of seladelpar in PBC patients with compensated cirrhosis were comparable to that of non-cirrhotic patients.

A third clinical presentation titled “Treatment With Seladelpar in Patients With Primary Biliary Cholangitis (PBC) and Prior Experience With Obeticholic Acid (OCA) or Fibrates” authored by Dr. Aliya Gulamhusein, MD, Assistant Professor and Clinical Investigator, Toronto Centre for Liver Disease, University of Toronto, highlighted the efficacy after 3 months of treatment of seladelpar in patients in Phase 2 and Phase 3 studies that had prior experience with OCA, fibrates, or both. The composite endpoint (ALP <1.67×ULN, ≥15% ALP decrease from baseline, and normal total bilirubin) was met in 79% of those receiving seladelpar 10 mg versus 8% of those taking placebo. Similarly, ALP normalized in 21% of patients in the 10 mg group and in no patients receiving placebo. Mean percentage changes after 3 months in ALP and ALT for patients taking seladelpar 10 mg were -45% and -21%, respectively, compared to -9%, and -7%, respectively, for placebo. Seladelpar appeared to be safe, well tolerated and showed meaningful and dose dependant improvement in biochemical markers of cholestasis.

Dr. Dennis Kim, Chief Medical Officer of CymaBay Therapeutics, commented, “The centerpiece nature of these presentations underscore the high quality scientific and collaborative work we have been engaged in with our academic partners and investigators. These presentations also further our collective understanding of PBC as a disease and the potential key role seladelpar can play in improving the lives of PBC patients, with respect to hepatic health, as well as quality of life. We look forward to completing our Phase 3 Clinical Program with seladelpar in PBC and reporting out what we hope will be promising efficacy and safety results next year.”

DDW Presidential Plenary Presentation:
May 22<sup>nd</sup> 8:00 AM PST
323:
1“Seladelpar Treatment of Patients With Primary Biliary Cholangitis (PBC) For 2 Years Improves the GLOBE PBC Score and Predicts Improved Transplant-Free Survival”
Bettina E. Hansen, Elaine Watkins, Ke Yang, Yun-Jung Choi, Charles A. McWherter, Gideon M. Hirschfield, for the Seladelpar Long-Term Study Investigators

Clinical Poster Presentations:
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 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 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.

Public Relations Contact:

Glenn Silver
Lazar-FINN Partners
(973) 818-8198
Glenn.silver@finnpartners.com

Investor Relations Contact:

Hans Vitzthum
LifeSci Advisors, LLC
(617) 430-7578
Hans@LifeSciAdvisors.com

Source: CymaBay Therapeutics, Inc.