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CymaBay Announces Positive Interim Results from Its Ongoing Low-Dose Phase 2 Study of Seladelpar in Patients with Primary Biliary Cholangitis

- Strong reduction in alkaline phosphatase of 39% (5 mg) and 45% (10 mg) at week 12
- No safety signal for transaminase elevation or drug-induced itch
- Potential for superior efficacy and better tolerability than existing second-line therapy
- FDA agrees to extend dosing of 5 mg and 10 mg beyond six months
- Conference call today at 8:30 a.m. ET

NEWARK, Calif., July 17, 2017 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (Nasdaq:CBAY), today announced positive interim results from its ongoing low-dose Phase 2 study of seladelpar in patients with primary biliary cholangitis (PBC), a life-threatening and life-limiting chronic cholestatic liver disease. In the first part of the study, patients at high risk of disease progression, with an inadequate response to ursodeoxycholic acid (UDCA), as characterized by a persistent elevation in alkaline phosphatase (AP), or who were intolerant to UDCA, received either 5 mg or 10 mg of seladelpar once-daily. A planned interim analysis of the first 24 patients enrolled in these two dose groups demonstrated after 12 weeks of treatment a significant AP reduction from baseline of 39% and 45% for the 5 mg and 10 mg groups, respectively. On seladelpar, 45% of patients in the 5 mg and 82% of patients in the 10 mg dose groups had AP values < 1.67 times the upper limit of normal (ULN). AP is an established surrogate marker of disease progression in PBC, and reaching a level of < 1.67 x ULN is a key component in the composite endpoint used for regulatory approval.

Alongside substantial reductions in AP, patients in both dose groups experienced decreases in other liver markers of cholestasis including gamma glutamyl transferase and total bilirubin. Seladelpar also improved metabolic and inflammatory markers with patients experiencing decreases in low-density lipoprotein-C and high sensitivity C-reactive protein.

There were no serious adverse events and no safety transaminase signal was observed at either dose. Instead, mean transaminase levels decreased over the course of treatment, further supporting seladelpar's anti-inflammatory activity. Consistent with prior studies, there was no signal for drug-induced pruritus.

After sharing preliminary results from the study, the FDA has agreed to allow continuation of seladelpar treatment beyond six months for the 5 mg and 10 mg doses.

"The data emerging from this study are impressive and support our hypothesis that lower doses of seladelpar than previously studied retain strong efficacy without raising a concern with transaminase elevations. We also see that seladelpar activity is not associated with drug-induced itch, an important benefit for patients with PBC. If these results are maintained over longer periods, we think that seladelpar could offer patients significant advantages over existing treatments," said Professor Gideon Hirschfield, Centre for Liver Research, University of Birmingham, UK.

Dr. Pol Boudes, Chief Medical Officer of CymaBay added, "We'd like to thank the investigators and their staff as well as the patients and their families for their tremendous support. These interim results support the potent anti-cholestatic and anti-inflammatory effects of seladelpar. We are particularly excited about the FDA's decision to allow dosing of seladelpar beyond six months enabling us to turn our attention towards planning the Phase 3 program."

"The clinical and regulatory progress to date represent meaningful advancement in the development of seladelpar for patients with PBC," said Sujal Shah, Interim President and CEO of CymaBay. "We are very encouraged by these results and the potential for seladelpar to improve treatment alternatives in PBC and other chronic liver diseases."

Conference Call

CymaBay will host a conference call today at 8:30 a.m. ET to discuss the interim topline results from this study. To access the live conference call, please dial 877-407-0784 from the U.S. and Canada, or 201-689-8560 internationally, Conference ID# 13666314. A slide presentation to be used in connection with the call entitled

“Seladelpar Interim Data – Phase 2 Low Dose Study in PBC” has been posted on CymaBay’s website and can be accessed at http://ir.cymabay.com/presentations. To access the live and subsequently archived webcast of the conference call, go to the Investors section of the company's website at http://ir.cymabay.com/events.

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 live 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 Nonalcoholic steatohepatitis (NASH). For PBC, seladelpar has received an orphan designation from the US Food and Drug Administration and the PRIority MEdicine (PRIME) status from the European Medicine Agency.

About CymaBay

CymaBay Therapeutics, Inc. (CBAY) 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 the autoimmune liver disease, PBC and NASH. A Phase 2 study of seladelpar established proof of concept in PBC. CymaBay is currently conducting a second Phase 2 study of seladelpar in PBC in order to support dose selection for Phase 3. Arhalofenate is a potential urate-lowering anti-flare therapy that has completed five Phase 2 studies in subjects with gout. Arhalofenate has been found to reduce painful flares in joints while at the same time lowering serum uric acid by promoting excretion of uric acid by the kidney. This dual action addresses both the signs and symptoms of gout while managing the underlying pathophysiology of hyperuricemia. Arhalofenate has been licensed in the U.S. to Kowa Pharmaceuticals America, Inc. CymaBay retains full development and commercialization rights for arhalofenate outside the U.S.

Cautionary Statements

The statements in this press release regarding the potential for seladelpar to treat PBC, the potential benefits to patients with PBC, and the expectations regarding future 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 which 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 Quarterly Report on Form 10-Q, 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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