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# CymaBay Announces Positive New 12-Week and 26-Week Results from its Ongoing Phase 2 Study of Seladelpar in Patients with Primary Biliary Cholangitis at The International Liver Congress™ 2018

- Seladelpar maintains potent anti-cholestatic and anti-inflammatory activity and appears safe and well tolerated, with no drug-induced pruritus, through 26 weeks of treatment
- Results from the extended study support the potential for improved efficacy and better tolerability over existing second-line therapy
- Results reaffirm plans for advancing to Phase 3 in the second half of 2018

NEWARK, Calif., April 11, 2018 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ:CBAY), today announced that a late-breaking poster presentation describing new data from a second interim analysis of its ongoing Phase 2 study of seladelpar in patients with primary biliary cholangitis (PBC) will be featured during The International Liver Congress™ hosted by the European Association for the Study of Liver Diseases (EASL) in Paris, France (April 11-15, 2018). Seladelpar is an orally administered, potent and selective peroxisome proliferator-activated receptor delta (PPAR $\delta$ ) agonist currently in development for PBC and nonalcoholic steatohepatitis (NASH).

The presentation, titled "**Treatment efficacy and safety of seladelpar, a selective peroxisome proliferator-activated receptor delta agonist, in primary biliary cholangitis patients: 12- and 26-week analyses of an ongoing international, randomized, dose ranging phase 2 study**" will be made by Professor Gideon Hirschfield on behalf of the study investigators and team.

These data demonstrate that in patients with PBC, seladelpar exhibits potent and sustained anti-cholestatic and anti-inflammatory efficacy over 26 weeks of administration. As of January 2018, 71 patients were exposed to at least one dose of seladelpar, of whom 53 received 12 weeks of treatment and 42 received 26 weeks of treatment. At baseline, mean alkaline phosphatase (AP) were 358, 333, and 262 U/L in the 2 mg, 5 mg, and 10 mg groups, respectively. At 12 weeks, changes in AP were -21%, -33%, and -45% in the 2 mg (N=6), 5 mg (N=25), and 10 mg (N=22) groups, respectively. After 12 weeks, dose titration was permitted for patients whose AP remained above normal and at a level where additional AP lowering had the potential to reduce the risk of disease progression. At 26 weeks, decreases in AP were similar across regimens at -45%, -43%, and -43% in the 5 mg (N=13), 5 to 10 mg titration (N=6) and 10 mg (N=19) groups, respectively. At 26 weeks, 69%, 67%, and 79% of patients across these three dose regimens, respectively, had an AP less than 1.67 times the upper limit of normal, with at least a 15% decrease in AP from baseline and normal bilirubin. Overall, 29% of patients had a normal AP at 26 weeks.

At 12 weeks, median transaminase changes were -9%, -28%, and -35% in the 2 mg, 5 mg, and 10 mg groups, respectively and decreases were maintained at 26 weeks in the 5 mg and 10 mg groups ( $\geq -40\%$ ).

Seladelpar was not associated with drug-induced pruritus. Baseline median pruritus VAS was 10 and 36 in the 5 mg and 10 mg groups, respectively, and patients in the 10 mg group experienced consistent decreases during treatment (-24% at week 26) suggesting potential anti-pruritic activity. Seladelpar was generally safe and well tolerated, with no transaminase elevation safety signal. There were 6 serious adverse events and none were deemed related to seladelpar.

Professor Hirschfield, from the University of Birmingham, UK, commented, "Seladelpar continues to demonstrate an impressive level of activity that is now sustained over 26 weeks of treatment. The decreased level of pruritus that was noted in the 10 mg group, a group that demonstrated a clinically relevant level of pruritus at baseline, is particularly intriguing and needs to be confirmed in additional studies."

"We are thrilled to have the opportunity to share new data from our development of seladelpar for patients with PBC in a late-breaking presentation at The International Liver Congress™. This is the third consecutive year in which seladelpar will be featured in the late-breaker category at one of the key international liver meetings. We have now firmly established doses of seladelpar with compelling efficacy and tolerability which we expect to further confirm in a Phase 3 study planned to start in the second half of the year," said Dr. Pol Boudes, M.D., Chief Medical Officer of CymaBay. "These data continue to support the potential for seladelpar to significantly improve treatment options for patients with PBC. We are thankful for the commitment and dedication of the patients, PBC support groups, investigators and study coordinators who are all essential to our efforts to advance seladelpar for patients with PBC."

CymaBay is also presenting new data highlighting the mechanism of action of seladelpar and its pharmacokinetic and pharmacodynamic profile in two additional posters at The International Liver Congress™. The posters titled: "**Seladelpar's mechanism of action as a potential treatment for primary biliary cholangitis and non-alcoholic steatohepatitis**" (540) and "**Pharmacokinetics and pharmacodynamics of seladelpar, a potent and selective PPAR-delta, in patients with primary biliary cholangitis**" (2463) are both being presented on April 12, 2018. Seladelpar demonstrates multiple beneficial activities that address the underlying pathophysiology of PBC and NASH. The pharmacokinetics of seladelpar in PBC patients supports a once daily dosing regimen, appears to be dose proportional and the level of exposure correlates with its pharmacodynamic activities.

Electronic copies of all three posters are available on CymaBay's website at <http://ir.cymabay.com/presentations>.

### **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 PPAR $\delta$  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 US Food and Drug Administration and the European Medicine Agency. Seladelpar also received 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 PPAR $\delta$  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 planning to advance development of seladelpar into Phase 3 for PBC and Phase 2 for NASH. Arhalofenate is a potential urate-lowering anti-flare therapy that 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 and NASH and the potential for arhalofenate to treat gout, 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](http://www.cymabay.com).

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