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CymaBay Therapeutics Announces Successful Completion of Scientific Advice Discussions with the European Medicines Agency for Arhalofenate

Agreement on Phase 3 Program to Capture Unique Dual Actions of Arhalofenate

NEWARK, Calif., Jan. 05, 2017 (GLOBE NEWSWIRE) -- CymaBay Therapeutics, Inc. (NASDAQ:CBAY), a clinical-stage biopharmaceutical company developing therapies to treat specialty and orphan diseases with high unmet medical need, today announced that it has successfully concluded its scientific advice discussions with the European Medicines Agency (EMA) on the Phase 3 development program for arhalofenate. Arhalofenate is a novel dual-acting product candidate for the treatment of gout that both lowers serum uric acid (sUA) and reduces gout flares. It is the first compound in a new class of gout therapy that the CymaBay refers to as Urate Lowering Anti-Flare Therapy (ULAFT). CymaBay licensed rights to arhalofenate in the U.S. to Kowa Pharmaceuticals America, Inc. but retains rights to develop and commercialize arhalofenate outside the U.S.

CymaBay reached agreement with the EMA on all of the key elements of the planned Phase 3 program which is very similar to that agreed upon with the Food and Drug Administration (FDA) last year. It will include two pivotal studies of arhalofenate in combination with febuxostat (40 mg) in patients with chronic gout and a third study in combination with febuxostat (80 mg) in subjects with tophaceous gout, a more advanced form of the disease.

Agreement was reached on the efficacy endpoints for the two separate clinical actions of arhalofenate. The sUA lowering will be assessed as responder rates for patients achieving the targets of <6 and <5 mg/dL for chronic and tophaceous gout, respectively. The data could support an indication for the management of hyperuricemia associated with gout in combination with febuxostat in these two patient populations. Flare data will be collected with an electronic diary and assessed using the same flare definition successfully used in the Phase 2 program. These data could support an indication for flare prophylaxis. In total, these studies will enroll approximately 1300 patients intended to receive treatment for at least 6 months. Approximately half of these patients will receive arhalofenate and will enter into an extension phase for an additional 6 months to collect safety information.

The lowest dose at which febuxostat (Adenuric®) is approved in Europe is 80 mg. In order to provide information on the relative effectiveness of the combination of arhalofenate and febuxostat (40 mg), a fourth study in subjects with chronic gout will be conducted comparing flare rate of this combination with that of febuxostat (80 mg) alone. The duration of this study would be 6 months and will enroll approximately 150 subjects.

“We are very pleased with the positive outcome of our discussions with the EMA on the registration program for arhalofenate,” commented Harold Van Wart, Ph.D., Chief Executive Officer of CymaBay. “The close alignment on the Phase 3 program requirements between the EMA and FDA, with only one small additional study required for Europe, is a very positive outcome. This additional clarity on the development program will also help us move forward with our partnering discussions for Europe.”

About Arhalofenate

Arhalofenate is an oral, once-daily dual-acting drug candidate for the treatment of gout that both lowers serum uric acid (sUA) and suppresses flares. It is the first compound in a new class of gout therapy that the Company refers to as Urate Lowering Anti-Flare Therapy (ULAFT).

Arhalofenate lowers sUA by blocking the reabsorption of uric acid in the proximal tubules of the kidney by inhibiting a renal uric acid transporter called URAT1. This leads to the excretion of uric acid into the urine. Arhalofenate produces its uricosuric effect gradually and appears to have a favorable overall and renal safety profile in studies completed to date in over 1,100 patients. The sUA lowering of arhalofenate is complementary and additive to that produced by the xanthine oxidase inhibitor febuxostat, which works by blocking the production of uric acid. The anti-flare activity of arhalofenate is attributable to the suppression of the urate crystal-induced production of IL-1β in gouty joints.

Current treatment guidelines for gout recommend the use of urate lowering therapies (ULTs) to reverse hyperuricemia in order to remove deposits of pro-inflammatory urate crystals. The minimal goal of this treatment is to reduce sUA levels to below 6 mg/dL. The goal for patients with a more advanced form of the disease called tophaceous gout is <5 mg/dL. Many patients treated with currently marketed xanthine oxidase inhibitors (allopurinol or febuxostat) alone do not reach these goals. In previously published studies, arhalofenate in combination with febuxostat has been shown to significantly increase the number of patients achieving their sUA goals.

Paradoxically, the initiation of ULT triggers an increased risk of gout flares for the first six months or more. The anti-inflammatory activity of arhalofenate has been shown in clinical studies to suppress flares, making it uniquely suited for the treatment of gout.

About CymaBay

CymaBay Therapeutics, Inc. (CBAY) is a clinical-stage biopharmaceutical company focused on developing therapies to treat metabolic diseases with high unmet medical need, including serious rare and orphan disorders. Seladelpar is a potent, selective, orally active PPARδ agonist. A Phase 2 study of seladelpar in patients with mixed dyslipidemia established that it has an anti-atherogenic lipid profile. CymaBay has completed Phase 2 studies for seladelpar in subjects with primary biliary cholangitis and homozygous familial hypercholesterolemia, establishing proof-of-concept in both indications. Arhalofenate, CymaBay's other product candidate, 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 future performance of CymaBay's product candidates are forward looking statements that are subject to risks and uncertainties. Actual results and the timing of events regarding the further development of CymaBay's product candidates, including but not limited to the expected Phase 3 trial of Arhalofenate and the anticipated resulting label, 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 of seladelpar and arhalofenate; 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 Annual Report on Form 10-K and Quarterly Report 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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