Portola Pharmaceuticals Presents Interim Phase 2a Safety and Efficacy Data for Cerdulatinib at the International Congress of Malignant Lymphoma

Oral, Dual SYK/JAK Inhibitor Provides Opportunity for Differentiated Activity in B-cell and other Hematological Malignancies

SOUTH SAN FRANCISCO, Calif., June 15, 2017 (GLOBE NEWSWIRE) -- Portola Pharmaceuticals Inc.® (Nasdaq:PTLA) today announced the presentation of interim data from a Phase 2a study evaluating cerdulatinib in patients with relapsed/refractory B-cell malignancies. Cerdulatinib is an investigational oral, dual SYK/JAK kinase inhibitor for the treatment of relapsed/refractory B-cell and other hematological malignancies, specifically in patients who have not responded to prior therapies. The data were presented by Paul Hamlin, M.D., chief of the Medical Oncology Service at Memorial Sloan Kettering Basking Ridge during an oral presentation given earlier today at the International Congress on Malignant Lymphoma (ICML) in Lugano, Switzerland.

The interim results presented at ICML demonstrated evidence of clinical activity in patients with relapsed/refractory (r/r) B-cell malignancies. To date, overall response rates are as follows:

- 12 out of 18 (67%) partial responses (PRs) in patients with r/r CLL/SLL
- 5 out of 9 (56%) PRs in patients with r/r FL
- 1 out of 7 (14%) PRs in patients with other r/r iNHL (marginal zone lymphoma and Waldenstrom macroglobulinemia)
- A complete response (CR) was seen in the first r/r peripheral T cell lymphoma (PTCL) patient evaluated in the study

Results also showed that cerdulatinib was generally well-tolerated in these heavily pre-treated patients (at target drug levels). However, three patients at 35 mg BID achieved higher than expected drug concentrations and had severe adverse events (SAEs) including two grade 5 infections and one case of grade three pancreatitis. The dose was subsequently reduced to 30 mg BID and a PK monitoring strategy was implemented. This has resulted in an improved PK and safety profile without compromising clinical activity.

“While we continue to focus our efforts on gaining regulatory approval of our two lead programs, betrixaban and AndexXa® (andexanet alfa), we are pleased that our third molecule, cerdulatinib, continues to show promising results in hematologic cancers — an area of great unmet need,” said John Curnutte, M.D., Ph.D., executive vice president, research and development of Portola. “We believe these results of our Phase 2a trial further validate cerdulatinib’s potential to control relapsed/refractory B-cell malignancies by a differentiated mechanism of action, inhibiting two key cell signaling pathways that promote cancer. We anticipate an update on the ongoing study and a decision regarding future development by the end of 2017.”

“Patients with relapsed/refractory CLL and NHL are difficult to treat and have limited options after failing standard therapy,” said Dr. Hamlin. “Presently, there are few effective treatments for patients who have failed prior therapies. The interim results of this clinical trial are very encouraging, and cerdulatinib could represent an important treatment option for these patients if confirmed in further trials.”

Additional detail on the interim Phase 2a data will be provided at the upcoming European Hematology Association (EHA) 22nd Annual Congress from June 22-25, 2017.

About the Phase 1/2a Study

The open-label, multicenter, Phase 1/2a proof-of-concept study assessed the safety, pharmacokinetics, pharmacodynamics and clinical activity of oral cerdulatinib in patients with CLL and NHL. In the multi-dose, dose-escalation Phase 1 part of the study, cerdulatinib was administered orally once or twice daily in sequential dose cohorts at increasing dose levels until the maximum tolerated dose was identified. The clinical expansion

cohorts in the Phase 2a part of the study are evaluating the safety and efficacy of cerdulatinib in cancer types identified based on results from the dose-escalation phase of the study. Up to 40 patients each will be enrolled in the clinical expansion cohorts including patients with relapsed/refractory chronic lymphocytic leukemia (CLL)/ small lymphocytic leukemia (SLL), and indolent lymphomas such as follicular lymphoma (FL) and peripheral T cell lymphoma (PTCL).

About Cerdulatinib

Cerdulatinib is an oral, dual Syk-JAK inhibitor with a unique mechanism of action. It inhibits two key signaling pathways that promote cancer cell growth in certain hematologic malignancies: the B-cell receptor pathway via Syk and key cytokine receptors via JAK. With its dual pathway mechanism, cerdulatinib may be more effective in specific patients than a single pathway agent, such as those resistant to current therapies or those with known heterogeneous cellular mutations. Preclinical data suggested that cerdulatinib may have anti-tumor activity in patients who did not adequately respond to, or relapsed on, other treatments due to defined mutations.

About Portola Pharmaceuticals, Inc.

Portola Pharmaceuticals is a biopharmaceutical company developing product candidates that could significantly advance the fields of thrombosis and other hematologic diseases. The Company is advancing three programs, including betrixaban, an oral, once-daily Factor Xa inhibitor; AndexXa (andexanet alfa), a recombinant protein designed to reverse the anticoagulant effect in patients treated with an oral or injectable Factor Xa inhibitor; and cerdulatinib, a SYK/JAK inhibitor in development to treat hematologic cancers. Portola's partnered program is focused on developing selective SYK inhibitors for inflammatory conditions. For more information, visit www.portola.com and follow the Company on Twitter @Portola_Pharma.

Forward-looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, cerdulatinib’s potential as a treatment for hematologic cancers and the potential of Portola’s other product candidates. Risks that contribute to the uncertain nature of the forward-looking statements include: failure to obtain FDA and/or EMA approval for one or more of our product candidates, regulatory developments in the United States and foreign countries; our expectation that we will incur losses for the foreseeable future and will need additional funds to finance our operations; the accuracy of our estimates regarding our ability to initiate and/or complete our clinical trials and the timing and expense of these trials; the results of our clinical trials related to the efficacy and safety of our product candidates; our potential inability to manufacture our product candidates on a commercial scale in a timely or cost-efficient manner; the accuracy of our estimates regarding expenses and capital requirements; our ability to successfully build a hospital-based sales force and commercial infrastructure; our ability to obtain and maintain intellectual property protection for our product candidates; and our ability to retain key scientific or management personnel. These and other risks and uncertainties are described more fully in our most recent filings with the Securities and Exchange Commission, including our most recent quarterly report on Form 10-Q. All forward-looking statements contained in this press release speak only as of the date on which they were made. We undertake no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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