Portola Investor Room

New Interim Phase 2a Study Results Demonstrate Broad Clinical Activity of Portola Pharmaceuticals' Oral SYK/JAK Inhibitor Cerdulatinib

– Differentiated SYK-JAK Inhibitor Shows Efficacy in Patients with Heavily Pre-Treated B- and T-Cell Malignancies –

– New Signal in AITL; Five Complete Responses for a Complete Response Rate of 71 Percent –

– Data to be Presented Monday, June 4, 2018 at ASCO and Friday, June 15, 2018 at EHA –

SOUTH SAN FRANCISCO, Calif., June 04, 2018 (GLOBE NEWSWIRE) -- Portola Pharmaceuticals, Inc.® (Nasdaq:PTLA) today announced new interim results from the Company's ongoing Phase 2a study of cerdulatinib, an investigational, oral SYK/JAK inhibitor, in patients with specific subtypes of B-cell and T-cell Non-Hodgkin Lymphoma (NHL), including relapsed/refractory follicular lymphoma (FL) and peripheral T-cell lymphoma (PTCL), and chronic lymphocytic lymphoma/small lymphocytic lymphoma (CLL/SLL). The data will be presented today by Paul Hamlin, M.D., medical director for the David H. Koch Center for Cancer Care at Memorial Sloan Kettering Cancer Center, during a Poster Discussion Session at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago (June 1-5). Data from this ongoing study also will be presented during a Poster Presentation Session at the 23rdCongress of the European Hematology Association (EHA) in Stockholm (June 14-17).

Among the 114 patients enrolled across five cohorts, 101 were evaluable as of May 4, 2018. The objective response rate (ORR) across all tumor types was 47 percent, with demonstration of clinical activity across tumor types and a new signal in the PTCL and cutaneous T-cell lymphoma (CTCL) cohorts.

Seven of the 20 patients in the PTCL cohort achieved a complete response (CR), including:

- Five out of seven with angioimmunoblastic T-cell lymphoma (AITL), for an ORR of 71 percent.
- Two out of eight patients with PTCL not otherwise specified (PTCL-NOS), for an ORR of 25 percent.

Five of the seven responding PTCL patients remain on study drug, including one at 12+ months and one at 9+ months. Of the remaining two patients, one received a bone marrow transplant after achieving CR and one discontinued due to Grade 3 colitis. All patients were previously on at least three prior therapies, including belinostat, pralatrexate and romidepsin. Additionally, data demonstrate an initial signal in CTCL, with the first patient enrolled achieving a CR.

Cerdulatinib also showed consistent activity among the 35 patients with FL, with an ORR of 46 percent and a median duration of response of eight months or more. Among the 28 patients with CLL/SLL, the ORR was 61 percent. All patients in these cohorts were previously on at least three prior therapies.

Cerdulatinib was generally well-tolerated. The most common serious adverse events occurring in ≥10 percent of patients were: lipase increase (18 percent), neutropenia (17 percent) and pneumonia/lung infection (11 percent). Additionally, five deaths due to sepsis or septic shock (three of which were concomitant with pneumonia) were considered related to study drug. These occurred primarily in patients with CLL/SLL.

"Cerdulatinib continues to demonstrate promising results across a wide range of B- and T-cell malignancies, including early indications of the potential for durable responses," said Dr. Hamlin. "The new signals in relapsed/refractory PTCL and CTCL are particularly compelling when you consider the limited treatment options for patients that fail front-line therapy. I am encouraged by these data and the potential of cerdulatinib to provide a significant clinical benefit to a group of patients with limited treatment options."

"These interim results provide evidence for cerdulatinib's unique mechanism of action of possibly disrupting two key cell signaling pathways, and its potential to control relapsed/refractory B-cell and T-cell malignancies in combination with standard and investigational therapies," said John Curnutte, M.D., Ph.D., executive vice

president, research and development of Portola. “We look forward to continuing discussions with the U.S. Food and Drug Administration regarding next steps for the development of cerdulatinib, including the potential for an accelerated approval pathway in the U.S. for certain tumor subtypes.”

ASCO Poster Session Details

Monday, June 4, 2018

- The Dual SYK/JAK Inhibitor Cerdulatinib Demonstrates Rapid Tumor Responses in a Phase 2 Study in Patients with Relapsed/Refractory B- and T-Cell Non-Hodgkin Lymphoma (NHL) (Abstract #7511) (Poster Board #148)
    - *Poster Session:* 8:00 a.m. – 11:30 a.m. CT (Hall A, McCormick Place)
    - *Poster Discussion Session:* 1:15 – 2:30 p.m. CT (E450, McCormick Place)

EHA Poster Presentation Details

Friday, June 15, 2018 from 17:30 p.m. – 19:00 p.m. CEST

- The Novel SYK/JAK Inhibitor Cerdulatinib Demonstrates Good Tolerability and Clinical Response in a Phase 2a Study in Relapsed/Refractory Peripheral T-Cell Lymphoma
  (Abstract #PF261)

- The Dual SYK/JAK Inhibitor Cerdulatinib Demonstrates Rapid Tumor Responses in a Phase 2 Study in Patients with Relapsed/Refractory B- and T-Cell Non-Hodgkin Lymphoma (NHL)
  (Abstract #PF437)

- *Preclinical Data:* JAK/SYK Inhibition is Vital to Prevent B-Cell Receptor Signaling and its Regulation by the Tumour Microenvironment in Chronic Lymphocytic Leukemia
  (Abstract #PF321)

Saturday, June 16, 2018 from 17:30 p.m. – 19:00 p.m. CEST

- *Preclinical Data:* Cerdulatinib Synergises with BCL-2 and MCL-1 Inhibitors to Induce Superior Cell Death in Chronic Lymphocytic Leukemia (Abstract #PS1067)

About Cerdulatinib
Cerdulatinib is an investigational oral, dual spleen tyrosine kinase (SYK) and janus kinase (JAK) inhibitor that uniquely inhibits two key cell signaling pathways implicated in certain hematologic malignancies and autoimmune diseases. There is a strong rationale for inhibiting both SYK (B-cell receptor pathway) and JAK (cytokine receptors) in B-cell malignancies where both targets have been shown to promote cancer cell growth and survival. In addition, pre-clinical data suggest an important role for SYK and JAK in peripheral T-cell lymphoma (PTCL) tumor survival.

Cerdulatinib is being developed to treat patients with follicular lymphoma (FL), PTCL and other hematologic cancers, specifically those who have relapsed or who have not responded to prior therapies.

About Portola Pharmaceuticals, Inc.
Portola Pharmaceuticals is a commercial-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics that could significantly advance the fields of thrombosis and other hematologic diseases. The Company’s two FDA-approved medicines are Bevyxxa® (betrixaban), the first and only oral, once-daily Factor Xa inhibitor, and Andexxa® [coagulation factor Xa (recombinant), inactivated-zhzo], the first and only antidote for the Factor Xa inhibitors rivaroxaban and apixaban. The company also is advancing cerdulatinib, a SYK/JAK inhibitor for the treatment of hematologic cancers.

Forward-Looking Statements

This announcement contains forward-looking statements, including statements regarding next steps for the development of cerdulatinib, including the potential for an accelerated approval pathway in the U.S. for certain tumor subtypes. Risks that contribute to the uncertain nature of the forward-looking statements include: failure to obtain FDA agreement to an accelerated or other regulatory approval pathway, 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. Risks and uncertainties relating to Portola Pharmaceuticals and its business can be found in the "Risk Factors" section of Portola Pharmaceuticals' Annual Report on Form 10-K for 2017, which was filed with the SEC on March 1, 2018, as updated by subsequent periodic reports filed by Portola with the SEC, including Quarterly Reports on Form 10-Q and Current Reports on Form 8-K which are deemed "filed" with the SEC. Portola Pharmaceuticals undertakes no duty or obligation to update any forward-looking statements contained in this release as a result of new information, future events or changes in Portola Pharmaceuticals' expectations.

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Source: Portola Pharmaceuticals, Inc.

https://investors.portola.com/2018-06-04-New-Interim-Phase-2a-Study-Results-Demonstrate-Broad-Clinical-Activity-of-Portola-Pharmaceuticals-Oral-SYK-JAK-Inhibitor-Cerdulatinib