Portola Pharmaceuticals Presents New Interim Data at the 61st ASH Meeting on its Oral SYK/JAK Inhibitor Cerdulatinib in Heavily Pre-Treated Patients with Relapsed/Refractory Follicular Lymphoma

- Interim Phase 2a Data Demonstrate Improved Overall Response Rate in Combination with Rituximab -

- 48% Overall Response Rate Observed in Cerdulatinib Only; 76% Overall Response Rate Observed with Cerdulatinib in Combination with Rituximab -

SOUTH SAN FRANCISCO, Calif., Dec. 9, 2019 /PRNewswire/ -- 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 relapsed/refractory follicular lymphoma (FL) receiving cerdulatinib alone or in combination with rituximab. The data will be presented today during a poster session at the 61st American Society of Hematology (ASH) Annual Meeting in Orlando (December 7-10).

Data included safety and efficacy findings as of November 2019 for 42 patients who received single agent cerdulatinib at 30 mg twice daily (with the exception of two patients who initiated treatment at 35 mg) and 21 patients who received cerdulatinib at 30 mg twice daily in combination with a standard dosing regimen of rituximab. The number of prior treatment regimens including anti-CD20 antibody, bendamustine and other alkylating agents, and PI3K inhibitors ranged from one to 10, with a median of three.

Among the 42 patients in the cerdulatinib-only cohort, the overall response rate (ORR) was 48%; 7 patients (17%) achieved a complete response (CR), 13 patients (31%) achieved a partial response (PR) and 10 patients (24%) achieved stable disease (SD). To date, 16 of the 42 patients (38%) in the cerdulatinib-only cohort have been on study drug for at least 10 months.

Among the 21 patients evaluated for efficacy in the cerdulatinib and rituximab combination cohort, the ORR was 76%; 5 patients (24%) achieved a CR, 11 patients (52%) achieved a PR and 5 patients (24%) achieved SD. Of the 11 patients in this combination cohort who have been on one to three prior therapies, the ORR was 91% with a complete response rate of 36%.

Cerdulatinib was generally well-tolerated and the safety profile appeared similar in both the cerdulatinib-only and rituximab combination cohorts. The most common adverse events (AEs) occurring in ≥5% of all evaluable study patients were lipase increase (27%), neutropenia (18%), diarrhea (13%) and amylase increase (9%). The most common AEs in the combination cohort included lipase increase (32%), neutropenia (22%) and diarrhea (14%). The lipase and amylase changes were generally asymptomatic and not associated with pancreatitis. Additionally, there was no emergence of late-stage colitis, cardiac or liver abnormalities, or other evidence of cumulative toxicity.

"These interim results demonstrate that cerdulatinib provides sustained clinical activity and good tolerability in patients with relapsed/refractory follicular lymphoma, and that the tumor response to both monotherapy and combination therapy appears to deepen over time," said Paul Hamlin, M.D., medical director for the David H. Koch Center for Cancer Care at Memorial Sloan Kettering Cancer Center. "It is encouraging that the combination of cerdulatinib and rituximab achieved an improved objective response rate (ORR) in heavily pre-treated patients compared to prior interim data, indicating it may have potential as a second-line therapy. We look forward to continuing the study and exploring an optimized dose of cerdulatinib and rituximab in this setting."

"Cerdulatinib is the most advanced SYK/JAK inhibitor of its kind in development for oncology, and has demonstrated its potential to inhibit two key survival pathways in Non-Hodgkin Lymphoma," said Jeff Myers, Portola's interim chief medical officer. "The data presented at ASH continues to demonstrate its safety and efficacy across a range of malignancies. Specific to follicular lymphoma, we are excited that the updated data showed an improved ORR with greater anti-tumor activity in combination with rituximab, and we look forward to exploring lower doses for potential use in the second-line setting."

ASH Poster Session Details – Monday, December 9, 2019, at 6:00 p.m. EST

Title:	Rapid and Durable Responses with the SYK/JAK Inhibitor Cerdulatinib in a Phase 2 Study in Relapsed/Refractory Follicular Lymphoma—Alone or in Combination with Rituximab
Session:	623. Mantle Cell, Follicular, and Other Indolent B-Cell Lymphoma—Clinical Studies: Poster III
Presenter:	Paul A. Hamlin, M.D., David H. Koch Center for Cancer Care at Memorial Sloan Kettering Cancer Center
Location:	Hall B, Level 2 (Orange County Convention Center)

About the Phase 2a Study

The Phase 2a, open-label study was designed to assess the safety and efficacy of cerdulatinib in patients with relapsed/refractory FL (alone or in combination with rituximab), small lymphocytic lymphoma (SLL) and specific subtypes of T-cell Non-Hodgkin Lymphoma, including PTCL, AITL and CTCL.

Tumor response in the two cohorts evaluating patients with relapsed/refractory FL was assessed by Lugano classification, with treatment continued until disease progression or unacceptable toxicity. Tumor response assessments were performed at the end of cycle two and every three cycles thereafter.

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.

The U.S. Food and Drug Administration granted cerdulatinib Orphan Drug Designation for the treatment of PTCL in September 2018.

About Portola Pharmaceuticals, Inc.

Portola Pharmaceuticals is a global, 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 conditions. The Company's first two commercialized products are Andexxa® [coagulation factor Xa (recombinant), inactivated-zhzo], marketed in Europe as Ondexxya® (andexanet alfa), and Bevyxxa® (betrixaban). The company also is advancing cerdulatinib, a SYK/JAK inhibitor being developed for the treatment of hematologic cancers. Founded in 2003 in South San Francisco, California, Portola has operations in the United States and Europe.

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, statements regarding the anticipated development plan for cerdulatinib. Risks that contribute to the uncertain nature of the forward-looking statements include: the risk of unfavorable results from additional clinical trials involving cerdulatinib; our ability to successfully execute on our development strategy; the risk of unfavorable regulatory developments; our expectation that we will incur losses for the foreseeable future and will need additional funds to finance our operations; the effects of competition; the accuracy of our estimates regarding expenses and capital requirements; our ability to successfully market Andexxa; our ability to obtain and maintain intellectual property protection for our product candidates; our ability to retain key scientific or management personnel; and general market conditions. 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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