CTI BIOPHARMA ANNOUNCES TOP-LINE RESULTS FROM PERSIST-2 PHASE 3 TRIAL OF PACRITINIB FOR HIGH-RISK PATIENTS WITH ADVANCED MYELOFIBROSIS

Trial demonstrates statistically significant improvement in spleen volume reduction (SVR) with pacritinib compared to best available therapy (BAT), including ruxolitinib

Oral pacritinib is the only JAK2 inhibitor evaluated in a randomized clinical trial in patients with thrombocytopenia (<100,000 platelets) to demonstrate a significant improvement in SVR, including in patients who had inadequate responses on marketed anti-JAK2 or failed prior anti-JAK2

SEATTLE, August 29, 2016–CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) today announced top-line results from PERSIST-2, a randomized, controlled Phase 3 clinical trial comparing pacritinib, an investigational oral multikinase inhibitor, with physician-specified best available therapy (BAT), including ruxolitinib, for the treatment of patients with myelofibrosis whose platelet counts are less than 100,000 per microliter -- a patient population with high-risk advanced disease. Three hundred eleven (311) patients were enrolled in the study, which formed the basis for the safety analysis. Two hundred twenty-one (221) patients who had a chance to reach Week 24 (the primary analysis time point) at the time the clinical hold was imposed and constituted the intent-to-treat (ITT) analysis population utilized for the evaluation of efficacy. Preliminary results demonstrated that the PERSIST-2 trial met one of the co-primary endpoints showing a statistically significant response rate in spleen volume reduction (SVR) in patients with myelofibrosis treated with pacritinib compared to BAT, including the approved JAK2 inhibitor ruxolitinib ($p<0.01$). Although the PERSIST-2 trial did not meet the other co-primary endpoint of greater than 50 percent reduction in Total Symptom Score (TSS), the preliminary analysis approached marginal significance compared to BAT ($p=0.0791$).

"Unlike patients with myelofibrosis who have normal baseline platelet counts where median survival is reported at 88 months, we recently reported from our institution's experience that patients with severe thrombocytopenia (low platelets) had a median survival of about 14 months," said Srdan (Serge) Verstovsek, M.D., Ph.D., Director, Clinical Research Center for MPNs at the University of Texas MD Anderson Cancer Center and principal investigator for the PERSIST-2 Phase 3 clinical trial of pacritinib. "These patients represent up to 30 percent of all myelofibrosis patients and an unmet medical need. Data from the PERSIST-2 prospective randomized, controlled trial is encouraging because we need an effective therapy to treat the most challenging patients with low platelet counts we see in our practice."

The co-primary endpoints of the trial were the proportion of patients achieving a 35 percent or greater reduction in spleen volume from baseline to Week 24 as measured by magnetic resonance imaging (MRI) or computerized tomography (CT) and the proportion of patients achieving a Total Symptom Score (TSS) reduction of 50 percent or greater using MPN-SAF TSS 2.0 diary from baseline to Week 24.

The most common treatment emergent adverse events for pacritinib were generally manageable diarrhea, nausea and vomiting. The incidence of cardiac and bleeding adverse events (all grades and grade 3-4 including deaths) were similar across the arms. Overall mortality rates were comparable between arms. Additional data from ongoing analyses along with top-line results from PERSIST-2 will be submitted for presentation at an upcoming scientific meeting.

Myelofibrosis is associated with significantly reduced quality of life and shortened survival. Spleen enlargement (splenomegaly) is a common and debilitating symptom of myelofibrosis. As the disease progresses, the body slows production of important blood cells and within one year of diagnosis the incidence of disease-related thrombocytopenia (very low blood platelet counts), severe anemia and red blood cell transfusion requirements increase significantly.

"Having analyzed data from two Phase 3 trials with the only JAK inhibitor to be studied in severely thrombocytopenic patients, including patients on JAK2 therapy or those who failed prior JAK2, we are encouraged by pacritinib's clinical profile in this difficult-to-treat group of patients with myelofibrosis," said James A. Bianco, M.D., President and Chief Executive Officer, CTI BioPharma. "We are grateful for the support and commitment of the investigators, our steering committee and, most importantly, all the patients who participated in PERSIST-2."

About PERSIST-2

PERSIST-2 was a randomized (1:1:1), controlled, open-label, multinational Phase 3 clinical trial evaluating pacritinib compared to best available therapy (BAT), including the approved JAK1/JAK2 inhibitor ruxolitinib, for patients with myelofibrosis whose platelet counts were less than or equal to 100,000 per microliter (≤100,000/μL). Three hundred eleven (311) patients were randomized to receive 200 mg pacritinib twice daily (BID), 400 mg pacritinib once daily (QD) or BAT. Clinical studies for pacritinib are currently subject to a full clinical hold issued by the U.S. Food and Drug Administration (FDA) in February 2016. The study was originally designed to enroll, and the Special Protocol Assessment (SPA) requires enrollment of 300 patients to evaluate the study objectives. Two hundred twenty-one (221) patients were enrolled at least 24 weeks prior to the full clinical hold and thus were potentially evaluable for efficacy. These patients were the population used to evaluate the study efficacy endpoints. The co-primary endpoints, originally agreed upon under the SPA, were the percentage of patients achieving a 35 percent or greater reduction in spleen volume measured by MRI or CT scan from baseline to Week 24 of treatment and the percentage of patients achieving a Total Symptom Score (TSS) reduction of 50 percent or greater using seven key symptoms as measured by the modified Myeloproliferative Neoplasm Symptom Assessment (MPN-SAF TSS 2.0) diary from baseline to Week 24. The primary objective of the study was to compare pooled pacritinib arms vs BAT. As a result of the full clinical hold on pacritinib, the SPA agreement is no longer in effect for PERSIST-2 and CTI BioPharma is no longer entitled to the benefit of the SPA.

About the Phase 3 Development Program of Pacritinib

Pacritinib was evaluated in two Phase 3 clinical trials, known as the PERSIST program, for patients with myelofibrosis, with one trial in a broad set of patients without limitations on platelet counts, the PERSIST-1 trial; and the other in patients with low platelet counts, the PERSIST-2 trial. In August 2014, pacritinib was granted Fast Track designation by the FDA for the treatment of intermediate and high risk myelofibrosis including, but not limited to, patients with disease-related thrombocytopenia (low platelet counts); patients experiencing treatment-emergent thrombocytopenia on other JAK2 inhibitor therapy; or patients who are intolerant of, or whose symptoms are not well controlled (sub-optimally managed) on other JAK2 therapy.

Clinical studies under the CTI BioPharma investigational new drug (IND) for pacritinib are currently subject to a full clinical hold issued by the U.S. Food and Drug Administration in February 2016.

PERSIST-1 was a randomized (2:1), controlled, open-label, multinational Phase 3 trial evaluating the efficacy and safety of pacritinib compared to BAT, excluding JAK2 inhibitors, which included a broad range of currently utilized treatments – in 327 patients with myelofibrosis (primary myelofibrosis, post-polycythemia vera myelofibrosis or post-essential thrombocythemia myelofibrosis), regardless of the patients' platelet counts. The study included patients with severe or life-threatening thrombocytopenia. Patients were randomized to receive 400 mg pacritinib once daily or BAT, excluding JAK2 inhibitors. As previously reported, the trial met its primary endpoint of spleen volume reduction (35 percent or greater from baseline to Week 24 by MRI/CT scan) in the intent-to-treat population (ITT).

About Pacritinib

Pacritinib is an investigational oral kinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R. The JAK family of enzymes is a central component in signal transduction pathways, which are critical to normal blood cell growth and development, as well as inflammatory cytokine expression and immune responses. Mutations in these kinases have been shown to be directly related to the development of a variety of blood-related cancers, including myeloproliferative

neoplasms, leukemia and lymphoma. In addition to myelofibrosis, the kinase profile of pacritinib suggests its potential therapeutic utility in conditions such as acute myeloid leukemia, or AML, myelodysplastic syndrome, or MDS, chronic myelomonocytic leukemia, or CMML, and chronic lymphocytic leukemia, or CLL, due to its inhibition of c-fms, IRAK1, JAK2 and FLT3.

CTI BioPharma and Shire are parties to a worldwide license agreement to develop and commercialize pacritinib. CTI BioPharma and Shire will jointly commercialize pacritinib in the U.S. while Shire has exclusive commercialization rights for all indications outside the U.S.

About Myelofibrosis and Myeloproliferative Neoplasms

Myelofibrosis is one of three main types of myeloproliferative neoplasms (MPN), which are a closely related group of progressive blood cancers. The three main types of MPNs are primary myelofibrosis (PMF), polycethemia vera (PV) and essential thrombocythemia (ET).[1]

Myelofibrosis is a serious and life-threatening bone marrow disorder caused by the accumulation of malignant bone marrow cells that triggers an inflammatory response and scars the bone marrow. The replacement of bone marrow with scar tissue limits its ability to produce red blood cells, prompting the spleen and liver to take over this function. Symptoms that arise from this disease include enlargement of the spleen, anemia, extreme fatigue, and pain.

The estimated prevalence of MPNs suggest there are approximately 300,000 people living with the disease in the U.S., of which myelofibrosis accounts for approximately 18,000 patients.[2] In Europe, there is a wide variation of prevalence observed across data sources. Myelofibrosis has a median age of 64 at the time of diagnosis[3] and is a progressive disease with approximately 20 percent of patients eventually developing acute myeloid leukemia (AML).[4] The median survival for high-risk myelofibrosis patients is less than 1.5 years, while the median survival for patients with myelofibrosis overall is approximately 6 years.[4]

About CTI BioPharma

CTI BioPharma Corp. is a biopharmaceutical company focused on the acquisition, development and commercialization of novel targeted therapies covering a spectrum of blood-related cancers that offer a unique benefit to patients and healthcare providers. CTI BioPharma has a commercial presence in Europe with respect to PIXUVRI® and a late-stage development pipeline, including pacritinib for the treatment of patients with myelofibrosis. CTI BioPharma is headquartered in Seattle, Washington, with offices in London and Milan under the name CTI Life Sciences Limited. For additional information and to sign up for email alerts and get RSS feeds, please visit www.ctibiopharma.com.

Forward-Looking Statements

This press release includes forward-looking statements, which are within the meaning of the Safe Harbor provisions of the Private Securities Litigation Reform Act of 1995. Such statements are subject to a number of risks and uncertainties, the outcome of which could materially and/or adversely affect actual future results and the trading price of the issuers' securities. Such statements include, but are not limited to, expectations with respect to our ability to be able to interpret clinical trial data and results despite not satisfying the pre-specified minimum evaluable patient goal and expectations with respect to the potential therapeutic utility of pacritinib, including pacritinib's potential to achieve treatment goals across patients with myelofibrosis, regardless of baseline characteristics, such as starting platelet count and in particular, its potential to reduce spleen volume and symptom burden and improve HRQoL. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release. In particular, this press release addresses select preliminary clinical trial data and results, and should be evaluated together with information regarding primary and secondary endpoints, safety and additional data once such data has been more fully analyzed and is made publicly available. In addition, meaningful interpretation of PERSIST-2 may not be possible because the pre-specified minimum evaluable patient goal was not met. The statements are based on assumptions about many important factors and information currently available to us to the extent we have thus far had an opportunity to fully and carefully

evaluate such information in light of all surrounding facts, circumstances, recommendations and analyses. A number of results and uncertainties could cause actual results to differ materially from those in the forward-looking statements, including: satisfaction of regulatory and other requirements; that trial results observed to date may differ from future results or that different conclusions or considerations may qualify such results once existing data has been more fully evaluated; actions of regulatory bodies and other governmental authorities; other clinical trial results; changes in laws and regulations; product quality, product efficacy, study protocol, data integrity or patient safety issues; product development risks; and other risks identified in each of the issuer's most recent filings on Forms 10-K and 10-Q and other Securities and Exchange Commission filings. Except as required by law, CTI Biopharma does not intend to update any of the statements in this press release upon further developments.

1. MPN Research Foundation. Accessed August 2016. Available at www.mpnresearchfoundation.org.
2. Based on Mesa R, ASH 2012 poster.
3. Cervantes F, et al., New prognostic scoring system for primary myelofibrosis based on a study of the International Working Group for Myelofibrosis Research and Treatment. *Blood*. 2009; 113:2895-2901.
4. Vannucchi, A. Management of Myelofibrosis. ASH Education Book. 2011; 1:222-230.

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CTI BioPharma Contacts:
Monique Greer
+1 206-272-4343
mgreer@ctibiopharma.com

Ed Bell
+1 206-272-4345
ebell@ctibiopharma.com