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Testo del comunicato

Vedi allegato.

CTI BIOPHARMA ANNOUNCES PUBLICATION IN *BLOOD* OF PHASE 2 RESULTS OF PACRITINIB IN PATIENTS WITH MYELOFIBROSIS

SEATTLE, Wash., March 16, 2015—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) today announced that results of a Phase 2 study of pacritinib, in patients with myelofibrosis were published in the journal *Blood*. Pacritinib is a next-generation oral JAK2/FLT3 multikinase inhibitor currently in Phase 3 development in the PERSIST program. Dr. Rami S. Komrokji, Associate Professor of Oncologic Sciences at the University of South Florida College of Medicine and Clinical Director of the Department of Malignant Hematology at Moffitt Cancer Center in Tampa, Fla., was the lead author.

Results of the Phase 2 study demonstrated that pacritinib is active in patients with myelofibrosis, resulting in spleen volume reduction, while producing substantial and prolonged improvement in disease-related symptoms without causing clinically significant myelosuppression. We believe pacritinib was well tolerated, including in patients with disease-related anemia and thrombocytopenia, with the predominant side effect being manageable gastrointestinal toxicity.

“The results of this Phase 2 study are consistent with our recently reported top-line results from the PERSIST-1 randomized Phase 3 trial with a comparable reduction in spleen volume of up to Week 24 in addition to the observation that patients on pacritinib continued to respond over time,” said James A. Bianco, M.D., President and CEO of CTI BioPharma. “Based on the results of this study and the recently announced PERSIST-1 top-line data, we believe pacritinib may provide an important therapeutic option for a broader spectrum of patients suffering from this challenging disease. We look forward to presenting additional data from PERSIST-1 at a scientific conference this year.”

“The publication of the Phase 2 results in the *Blood* journal was timely as we recently learned about the positive results from PERSIST-1 randomized Phase 3 study. Currently, myelofibrosis patients with anemia and thrombocytopenia have limited treatment options for splenomegaly and constitutional symptoms and these data show that pacritinib has potential to help patients that are sub-optimally managed on currently available treatments,” said Dr. Komrokji.

Phase 2 Study Design and Results

The multicenter, single-arm, open-label Phase 2 study evaluated the safety and efficacy of pacritinib in the treatment of patients with myelofibrosis who had clinical splenomegaly poorly controlled with standard therapies or were newly diagnosed with intermediate- or high-risk disease and not considered candidates for standard therapy. Patients were allowed to enroll irrespective of their degree of thrombocytopenia, anemia or neutropenia. A total of 35 patients were enrolled and treated with pacritinib 400 mg administered once daily in 28-day cycles. The median age of the patients was 69 years. The endpoint of the study was assessment of the spleen response rate, defined as the proportion of subjects achieving 35 percent or greater reduction in spleen volume from baseline up to Week 24 as measured by magnetic resonance imaging (MRI). Other endpoints included the proportion of patients with 50 percent or greater reduction in spleen size as determined by physical exam and the proportion of patients with 50 percent or greater reduction in total symptom score (TSS), including symptoms of

abdominal pain, bone pain, early satiety, fatigue, inactivity, night sweats and pruritus, from baseline up to Week 24.

Results showed that up to Week 24:

- 30.8 percent of evaluable patients (8/26) had 35 percent or greater reduction in spleen volume by computerized tomography (CT) or MRI scan with 42 percent of patients reaching 35 percent or greater reduction by end of treatment

- 42.4 percent of evaluable patients (14/33) achieved 50 percent or greater reduction in spleen size by physical exam

- 48.4 percent of evaluable patients (15/31) achieved 50 percent or greater reduction in TSS

The most common treatment-emergent adverse events were Grade 1-2 diarrhea (69%) and nausea (49%), which caused one patient to discontinue treatment. The study drug was discontinued in nine patients (26%) due to adverse events, of which three were deemed unrelated to study drug. There were five deaths, three of which were due to serious adverse events. Of those, one (subdural hematoma) was considered possibly related to study drug. Anemia and thrombocytopenia adverse events were reported in 12 (34.3%) and eight (22.9%) patients, respectively.

The publication, titled "Results of a Phase 2 Study of Pacritinib (SB1518), a JAK2/JAK2(V617F) Inhibitor, in Patients with Myelofibrosis," is available at http://www.bloodjournal.org/content/early/2015/03/11/blood-2013-02-484832. Preliminary data from the study were presented at the 15th Congress of the European Hematology Association in 2010 and at the American Society of Hematology 2011 Annual Meeting.

About Myelofibrosis and Myeloproliferative Neoplasms

Myelofibrosis is one of three main types of myeloproliferative neoplasms (MPNs), which are a closely related group of hematological blood cancers. The three main types of MPNs are myelofibrosis, polycythemia vera and essential thrombocytopenia.[1] Myelofibrosis is a serious and life-threatening chronic 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 in the U.S.[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.[3] The median survival for high-risk patients is less than one and a half years; median survival for myelofibrosis patients overall is approximately six years.[4]

About Pacritinib

Pacritinib is an oral multikinase inhibitor with dual activity against JAK2 and FLT3. 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 MPNs, leukemia and lymphoma. Although pacritinib suppresses the JAK2/STAT3 pathway, pacritinib does not cause myelosuppression[5] and may offer an advantage over other JAK inhibitors through effective treatment of symptoms while having less treatment-emergent thrombocytopenia and anemia than has been seen in currently approved and in development JAK inhibitors. The kinase profile of pacritinib suggests its potential therapeutic

utility in acute myeloid leukemia (AML), myelodysplastic syndrome (MDS), chronic myelomonocytic leukemia (CMML) and chronic lymphocytic leukemia (CLL) due to its potent inhibition of c-fms, IRAK1, JAK2 and FLT3.[5]

CTI BioPharma and Baxter entered into a worldwide license agreement in November 2013 to develop and commercialize pacritinib pursuant to which CTI BioPharma and Baxter will jointly commercialize pacritinib in the U.S. while Baxter has exclusive commercialization rights for all indications outside the U.S.

About CTI BioPharma Corp.
CTI BioPharma Corp. (NASDAQ and MTA: CTIC) 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 and a late-stage development pipeline, including pacritinib, CTI BioPharma's lead product candidate, which is currently being studied in a Phase 3 program 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.

Source: CTI BioPharma Corp.

Forward Looking Statements
This press release includes forward-looking statements related to pacritinib and related clinical trials conducted pursuant to a collaboration between Baxter International Inc. and CTI BioPharma Corp., 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, statements regarding expectations with respect to the potential therapeutic utility of pacritinib and the ability of pacritinib to meet unmet medical needs. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release. 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: satisfaction of regulatory and other requirements; that [top-line] results observed to date may differ from future results or that difference conclusions or considerations may qualify such results once existing data has been more fully evaluated, actions of regulatory bodies and other governmental authorities; 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 issuer's most recent filings on Form 10-K 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 website, www.mpnresearchfoundation.org.

2. Mehta J, Wang H, Iqbal SU, Mesa R. Epidemiology of myeloproliferative neoplasms in the United States. Leukemia & Lymphoma 2014; 55(3): 595-600.

3. Cervantes F, Dupriez B, Pereira A, Passamonti F, Reilly JT, Morra E, Vannucchi AM, Mesa RA, Demory J-L, Barosi G, Rumi E, Tefferi A. 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.

5. Singer J et al., ASH 2014 Abstract #1874: Comprehensive Kinase Profile of Pacritinib, a Non-Myelosuppressive JAK2 Kinase Inhibitor in Phase 3 Development in Primary and Post ET/PV Myelofibrosis.

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