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Testo del comunicato

Vedi allegato.

PACRITINIB PHASE 3 STUDY SHOWS POSITIVE RESULTS IN PATIENT REPORTED OUTCOMES MEASURING QUALITY OF LIFE IN PATIENTS WITH MYELOFIBROSIS

Significant Improvements in Symptom Score were Reported with Pacritinib, Across All Measured Symptoms, Compared to Best Available Therapy

VIENNA, Austria, June 12, 2015—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) and Baxter International's BioScience business (NYSE:BAX) today announced new patient-reported outcome (PRO) data for pacritinib – an investigational oral multikinase inhibitor with specificity for JAK2 and FLT3 – from the Phase 3 PERSIST-1 study. As recently reported at the American Society of Clinical Oncology (ASCO) annual meeting, results show a significant reduction in the Total Symptom Score (TSS) (the proportion of patients with a 50 percent or greater reduction in TSS from baseline to Week 24), and in each individual common disease-related symptom, from baseline to Week 24, in patients treated with pacritinib compared to best available therapy (exclusive of a JAK inhibitor) (BAT). These PROs, as well as other quality of life measures, will be presented at the 20th Congress of European Hematology Association (EHA) by Adam Mead, M.D., Guy's and St. Thomas' NHS Foundation Trust, Guy's Hospital, London, United Kingdom in an oral presentation on Sunday, June 14, 2015 at 12:15 CEST (abstract #LB2072). These data were also selected for inclusion in the official EHA Press Briefing which occurred today (Friday, June 12, 2015) at 08:30 CEST. As previously reported, the PERSIST-1 trial met its primary endpoint of spleen volume reduction of 35 percent or greater from baseline to Week 24 as measured by MRI/CT scan.

Myelofibrosis is a rare blood cancer associated with significantly reduced quality of life and shortened survival. 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 increases significantly. Among other complications, most patients with myelofibrosis present with enlarged spleens (splenomegaly), as well as many other potentially devastating physical symptoms such as abdominal discomfort, bone pain, feeling full after eating little, severe itching, night sweats, and extreme fatigue.

"Patient-reported outcomes are an important measure for understanding the potential benefit of a therapy on patients' lives – particularly for a disease such as myelofibrosis where the symptoms have such a tremendous impact on the quality of patients' daily lives," stated James A. Bianco, M.D., President and CEO of CTI BioPharma. "These new data from the PERSIST-1 study further support our belief, not only in the activity of pacritinib, but also the potential to positively impact patients' daily lives by relieving the symptoms that accompany myelofibrosis."

"The PERSIST-1 trial has continued to generate positive and important findings for the hematology community," said David Meek, Head of Oncology at Baxter BioScience. "We look forward to advancing the clinical trial program of pacritinib as we work to realize the full potential of this investigational compound to help patients with serious blood cancers, such as myelofibrosis."

Study Details and Findings Presented at EHA

PERSIST-1 is a randomized (2:1), controlled Phase 3 registration-directed trial comparing the efficacy and safety of pacritinib to BAT – which included a range of currently utilized off-label treatments – in 327 patients with

myelofibrosis, regardless of the patients' platelet counts. As previously reported at ASCO, the trial met its primary endpoint of spleen volume reduction (35 percent or greater reduction from baseline to Week 24 by MRI/CT scan) in the intent-to-treat (ITT) population; these results included patients with severe or life-threatening thrombocytopenia. The study also measured patient-reported outcomes (PROs), the proportion of patients with a 50 percent or greater reduction in TSS from baseline to Week 24, which have become important for approval of new therapies and was one of the secondary endpoints of the study. As previously reported, patients treated with pacritinib experienced greater improvement in their disease-related symptoms (ITT patient population: 24.5 percent of pacritinib-treated patients vs 6.5 percent of BAT-treated patients, $p<0.0001$; evaluable patient population: 40.9 percent of pacritinib-treated patients vs 9.9 percent of BAT-treated patients, $p<0.0001$).

New data presented at EHA, which included results from multiple PROs measurement tools, showed:

Myeloproliferative Neoplasm Symptom Assessment (MPN-SAF TSS and MPN-SAF TSS 2.0)
When using the MPN-SAF TSS and MPN-SAF TSS 2.0, each of the six common disease-related symptoms from the TSS results showed improvements in abdominal discomfort (46 percent improvement with pacritinib vs no improvement with BAT); bone pain (32 percent improvement with pacritinib vs 8 percent improvement with BAT); feeling of early fullness (45 percent improvement with pacritinib vs 1 percent worsening with BAT); itching (48.5 percent improvement with pacritinib vs 10 percent improvement with BAT); night sweats (69.5 percent improvement with pacritinib vs no improvement with BAT); and fatigue (27.5 percent improvement with pacritinib vs 4 percent worsening with BAT). MPN-SAF TSS and MPN-SAF TSS 2.0 are specific sets of questions patients answer daily (via electronic diary) and which are based on a questionnaire originally developed by Ruben A. Mesa, M.D., Deputy Director of the Mayo Clinic Cancer Center in Scottsdale, Arizona, USA.

Patient Global Impression of Change (PGIC)
Based on the PGIC assessment – which measures a patient's assessment of overall health on a 7-point scale ranging from "very much worse" to "very much improved" – approximately 80 percent of evaluable patients treated with pacritinib rated their condition as improved compared to approximately 20 percent with BAT.

European Organization for Research and Treatment of Cancer Quality-of-Life 30 Questionnaire (EORTC QLQ-C30)
A greater improvement was also reported by evaluable patients treated with pacritinib vs BAT across all components of the EORTC QLQ-C30 questionnaire, a well-validated measure of quality of life in cancer patients.

The most common adverse events occurring with pacritinib within 24 weeks, of any grade, were mild to moderate diarrhea (53.2 percent vs 12.3 percent with BAT), nausea (26.8 percent vs 6.6 percent with BAT), anemia (22.3 percent vs 19.8 percent with BAT), thrombocytopenia (16.8 percent vs 13.2 percent with BAT), and vomiting (15.9 percent vs 5.7 percent with BAT). Of the patients treated with pacritinib, 3 discontinued therapy and 13 patients required dose interruption (average one week) for diarrhea. Patients received a daily full dose of pacritinib over the duration of treatment. Gastrointestinal symptoms typically lasted for approximately one week and few patients discontinued treatment due to side effects. There were no Grade 4 gastrointestinal events reported.

About Pacritinib
Pacritinib is an investigational oral multikinase inhibitor with specificity for 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 myeloproliferative neoplasms, leukemia, and lymphoma.

About Myelofibrosis
Myelofibrosis is a serious and life-threatening chronic blood cancer 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. Myelofibrosis is a one of three main types of myeloproliferative neoplasms (MPN), which are a closely related group of hematological blood cancers.[1] 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[2] 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 the PERSIST Phase 3 Development Program of Pacritinib
Pacritinib is currently being evaluated in two Phase 3 clinical trials, known as the PERSIST program, for patients with myelofibrosis. **PERSIST-1** is a randomized (2:1), open-label, multinational Phase 3 clinical trial comparing the efficacy and safety of pacritinib with that of BAT, in 327 enrolled patients with primary and secondary myelofibrosis (PMF), post-polycythemia vera myelofibrosis (PPV-MF), or post-essential thrombocythemia myelofibrosis (PET-MF), without exclusion for low platelet counts. **PERSIST-2** is a randomized (2:1), open-label, multinational Phase 3 clinical trial evaluating pacritinib compared to BAT, including the approved JAK1/JAK2 inhibitor dosed according to product label for patients with myelofibrosis whose platelet counts are less than or equal to 100,000 per microliter. The trial is designed to enroll up to 300 patients in North America, Europe, Australia, New Zealand, and Russia.

Additional details are available at www.clinicaltrials.gov or www.PERSISTprogram.com.

CTI BioPharma and Baxter BioScience, which is expected to become Baxalta Incorporated in mid-2015, entered into a worldwide license agreement in November 2013 to develop and commercialize pacritinib. 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.

About Baxter International Inc.
Baxter International Inc., through its subsidiaries, develops, manufactures and markets products that save and sustain the lives of people with hemophilia, immune disorders, cancer, infectious diseases, kidney disease, trauma and other chronic and acute medical conditions. As a global, diversified healthcare company, Baxter applies a unique combination of expertise in medical devices, pharmaceuticals and biotechnology to create products that advance patient care worldwide.

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., as well as statements regarding the planned separation of Baxter's biopharmaceutical and medical products businesses, 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, the ability of the PERSIST-1 and PERSIST-2 trials to support a potential regulatory submission, the anticipated completion of enrollment, the ability of pacritinib to meet unmet medical needs and future regulatory, development and commercialization plans. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release and 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 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; 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 as well as the Form 10 filed by Baxalta Incorporated. Neither Baxter nor CTI BioPharma undertakes to update its forward-looking statements.

1. MPN Research Foundation website, 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.

Source: CTI BioPharma Corp. and Baxter International Inc.

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