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Testo del comunicato

Vedi allegato.

PHASE 3 PACRITINIB STUDY SHOWS SIGNIFICANT CLINICALLY MEANINGFUL RESULTS IN PATIENTS WITH MYELOFIBROSIS IN LATE-BREAKING SESSION AT ASCO 2015

Study demonstrates improvement in key disease measurements and disease-related symptoms with pacritinib treatment compared to best available therapy, regardless of platelet levels at the time of enrollment

Treatment resulted in improvements in severe thrombocytopenia and severe anemia, eliminating the need for blood transfusions in a quarter of patients who were transfusion dependent at the time of enrollment

CHICAGO, Ill., **May 30, 2015**—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) and Baxter International's Bioscience business (NYSE:BAX) today announced data from PERSIST-1 – a randomized Phase 3 registration-directed trial examining pacritinib for the treatment of myelofibrosis – in a late-breaking oral session at the 51st Annual Meeting of the American Society of Clinical Oncology (ASCO), May 29-June 2, 2015 in Chicago, Ill. Pacritinib is an investigational oral multikinase inhibitor with specificity for JAK2 and FLT3. Data presented at ASCO (Abstract #LBA7006) show that compared to best available therapy (exclusive of a JAK inhibitor), pacritinib therapy resulted in a significantly higher proportion of patients with spleen volume reduction and control of disease-related symptoms. Data were also selected for inclusion in the official ASCO Press Program.

"Myelofibrosis is a difficult-to-treat, rare chronic blood cancer in need of new options that can help overcome the many unique and burdensome symptoms that patients with this disease face on a regular basis, such as blood transfusions and debilitating pain and fatigue," stated Claire Harrison, M.D., Consultant Hematologist, Guy's and St. Thomas' NHS Foundation Trust, Guy's Hospital, London, United Kingdom and one of the principal investigators for PERSIST-1. "Based on data showing improvement in bone marrow function, pacritinib may have the potential to modify disease in the sickest patients as monotherapy and warrants further evaluation in combination with other potential disease-modifying agents."

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.

PERSIST-1 Findings Presented at ASCO

PERSIST-1 is a randomized (2:1), controlled Phase 3 registration-directed trial comparing the efficacy and safety of pacritinib to best available therapy (BAT) – which included a range of currently utilized off-label treatments – in 327 patients with myelofibrosis (primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis), regardless of the patients' platelet counts. At study entry, 46 percent of patients were thrombocytopenic; 32 percent of patients had platelet counts less than 100,000 per microliter (<100,000/µL); and 16 percent of patients had platelet counts less than 50,000 per microliter (<50,000/µL); normal platelet counts range from 150,000 to 450,000 per microliter. The median duration of treatment was 16.2

months in patients treated with pacritinib, compared to 5.9 months in patients treated with BAT. The majority (79 percent) of patients on the BAT arm eventually crossed over to receive pacritinib therapy.

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). These results included patients with severe or life-threatening thrombocytopenia. Data presented at ASCO show that when measuring the volume of spleen reduction, the greatest difference in treatment arms was observed in evaluable patients with the lowest platelet counts (<50,000/µL platelets) (33.3 percent with pacritinib vs 0 percent with BAT) (p=0.037).

Spleen Volume Reduction of ≥35% at Week 24 by Platelet Levels

	Pacritinib	BAT	p-value
All Platelet Levels			
ITT*	19.1% (n=220)	4.7% (n=107)	0.0003
Evaluable**	25.0% (n=168)	5.9% (n=85)	<0.0001
<**100,000/µL platelets**			
ITT	16.7% (n=72)	0% (n=34)	0.0086
Evaluable	23.5% (n=51)	0% (n=24)	0.0072
<**50,000/µL platelets**			
ITT	22.9% (n=35)	0% (n=16)	0.0451
Evaluable	33.3% (n=24)	0% (n=11)	0.0370

** ITT - primary analysis included all patients randomized. Patients who missed MRI or CT scans at baseline or at Week 24 were counted as non-responders.*
*** Evaluable - analysis included patients who had assessment at both baseline and at Week 24.*

Beyond the statistically significant reductions in spleen volume, patients treated with pacritinib also experienced a sustained improvement in myelofibrosis-associated symptoms or Total Symptom Score (TSS) as measured by the Myeloproliferative Neoplasm Symptom Assessment Form electronic diary (MPN-SAF TSS and MPN-SAF TSS 2.0). The patient-reported outcomes instrument captures in an electronic diary how a patient feels or functions in relation to their health condition or treatment, including: fatigue, concentration, early satiety/fullness, inactivity, night sweats, itching, bone pain, abdominal discomfort, weight loss, and fevers. When measuring the secondary endpoint (the proportion of patients with a 50 percent or greater reduction in TSS from baseline to Week 24), patients treated with pacritinib experienced greater improvement in their symptoms when compared to BAT, regardless of their baseline platelet counts (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).

Twenty-five percent (25%) of patients treated with pacritinib who were severely anemic and transfusion dependent – requiring at least six units of blood in the 90 days prior to study entry – became transfusion independent, compared to zero patients treated with BAT (p<0.05). Among patients with the lowest baseline platelets (<50,000/µL) who received treatment with pacritinib, a significant increase in platelet counts was observed over time compared to BAT (p=0.003) – with a 35 percent increase in platelet counts from baseline to Week 24.

The most common adverse events, occurring in 10 percent or more of patients treated 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.

"Results from PERSIST-1 add to the growing body of data showing the potential for pacritinib to address an unmet medical need that currently exists for patients with myelofibrosis, particularly patients with severely low platelet counts that result either from their disease or as a side effect from current treatment," said James A. Bianco, M.D., CTI BioPharma's President and CEO. "Based on the results observed in this trial, we are continuing to advance the broad clinical development program for pacritinib across a range of hematologic malignancies."

"PERSIST-1 is the most inclusive randomized study of patients with myelofibrosis ever conducted, as we believe it is truly representative of healthcare providers' real-world clinical experience, including patients with advanced disease, severe cytopenias, and a broad range of platelet count levels with the greatest need for effective treatment options," said David Meek, Head of Oncology at Baxter BioScience. "We look forward to advancing the clinical trial program of pacritinib for the treatment of myelofibrosis and to sharing these data with regulatory authorities."

Data will be presented today by Ruben Mesa, M.D., Deputy Director, Mayo Clinic Cancer Center, Chair of the Division of Hematology & Medical Oncology, Mayo Clinic Cancer Center in both the official ASCO Press Program (titled 'Targeted Therapy') at 8:00 a.m. CT, as well as in a late-breaking oral session at 2:37 p.m. CT.

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. The PERSIST clinical trials are intended to support a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA). 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, patients experiencing treatment-emergent thrombocytopenia on other JAK2 inhibitor therapy, or patients who are intolerant of, or whose symptoms are sub-optimally managed on other JAK2 inhibitor therapy. The FDA's Fast Track process is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need.

PERSIST-1 is a randomized (2:1), open-label, multinational Phase 3 clinical trial comparing the efficacy and safety of pacritinib with that of best available therapy (BAT), other than JAK inhibitors, 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 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 Pacritinib
Pacritinib is an 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. The kinase profile of pacritinib suggests its potential therapeutic utility in conditions such as 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.[1]

About Myelofibrosis and Myeloproliferative Neoplasms
Myelofibrosis is a one of three main types of myeloproliferative neoplasms (MPN), which are a closely related group of hematological blood cancers. The three main types of MPNs are myelofibrosis, polycethemia vera, and essential thrombocythemia.[2] 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.[3] 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.[4] 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.[5]

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., 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, and 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. 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. Neither Baxter nor CTI BioPharma undertakes to update its forward-looking statements.

1. 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.

2. MPN Research Foundation website, www.mpnresearchfoundation.org.

3. Based on Mesa R, ASH 2012 poster.

4. 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.

5. Vannucchi, A. Management of Myelofibrosis. ASH Education Book. 2011; 1:222-230.

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