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Positive TOP-LINE Results from Phase 3  
PERSIST-1 Trial

*Testo del comunicato*

Vedi allegato.



FOR IMMEDIATE RELEASE

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**CTI BIOPHARMA AND BAXTER ANNOUNCE POSITIVE TOP-LINE RESULTS FROM PHASE 3 PERSIST-1 TRIAL OF PACRITINIB FOR PATIENTS WITH MYELOFIBROSIS**

*Trial Meets Primary Endpoint in Intent-to-Treat Population*

*Oral Pacritinib is the First Agent with Clinical Data Supporting its Use in Treating Patients with Myelofibrosis Irrespective of Their Platelet Count, Including Severe and Life-Threatening Thrombocytopenia (low platelets)*

*CTI BioPharma to Host Conference Call Today at 8:00 am EDT*

**SEATTLE, Wash. and DEERFIELD, Ill., March 9, 2015**—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) and Baxter International Inc. (NYSE:BAX) today announced positive top-line results for the primary endpoint from PERSIST-1, the randomized, controlled Phase 3 registration clinical trial examining pacritinib, a next generation oral JAK2/FLT3 multikinase inhibitor, for the treatment of patients with primary or secondary myelofibrosis. The PERSIST-1 trial met its primary endpoint in the intent-to-treat population with statistically significant activity observed in patients irrespective of their initial platelet count, including patients with very low platelet counts at study entry, a condition known as severe or life-threatening thrombocytopenia.

The primary endpoint of the trial was 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) when compared with physician-specified best available therapy (BAT), excluding treatment with JAK2 inhibitors. The PERSIST-1 trial demonstrated that pacritinib treatment provided a clinically and statistically significant response rate ( $p = 0.0003$ ) in spleen volume reduction in patients with myelofibrosis when compared to BAT. Importantly, the trial results also demonstrated a significant difference among patients with platelet counts of less than 100,000 per microliter and less than 50,000 per microliter, both subgroups that were

stratified at randomization. The magnitude of treatment effect was consistent with previously reported Phase 2 results, with the greatest reduction observed among the sickest patients (platelet counts <50,000 per microliter). Among 50 patients who were red blood cell (RBC) transfusion dependent at study entry ( $\geq 6$  units of RBC over 90 days pre entry), pacritinib therapy resulted in a clinically meaningful percentage of patients becoming transfusion independent compared to BAT. Seventy-nine percent (79%) of patients in the BAT arm of the study crossed over to pacritinib therapy.

The safety profile in the PERSIST-1 trial was consistent with prior Phase 2 trials. While the most common treatment emergent adverse events were diarrhea, nausea and vomiting, the incidence of grade 3 events was lower than observed in Phase 2 trials. No grade 4 gastrointestinal adverse events were reported. Three patients discontinued therapy and nine patients required dose reduction for diarrhea. Preliminary analysis suggests that very few patients discontinued treatment while on pacritinib or required a dose reduction due to treatment-related anemia or thrombocytopenia. Additional data from ongoing analyses along with top-line results from PERSIST-1 will be submitted for presentation at an upcoming scientific meeting.

“Despite the introduction of JAK2 inhibitors as effective therapies for patients with myelofibrosis, there remains a treatment gap for patients with disease-related or treatment emergent thrombocytopenia. The currently approved drug may require dose titration to less effective doses in this patient population, thus limiting our ability to effectively treat them. Results from the PERSIST-1 randomized trial demonstrate that pacritinib could address this unmet medical need,” 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. “It is encouraging to see that patients were able to receive therapeutic doses of pacritinib over a long period of time irrespective of their baseline platelet or red blood cell count while having therapeutic benefit in reduction in spleen volume and disease-related symptoms and improvement in transfusion dependency.”

“PERSIST-1 is the first randomized Phase 3 trial investigating the potential benefit of a JAK2 inhibitor across a patient population with myelofibrosis that is representative of patients that healthcare providers see and treat in clinical practice,” said James A. Bianco, M.D., CTI BioPharma’s President and CEO. “We are excited by the clinical profile demonstrated in this randomized trial with respect to benefit–risk especially for a segment of MF patients excluded from other randomized trials with JAK2 inhibitors. We are grateful for the support and commitment of the investigators, our steering committee and, most importantly, all the patients who participated in PERSIST-1. We look forward to building on the progress we have made thus far.”

“These positive top-line results illustrate the potential of this investigational treatment to become a valuable new treatment option for this challenging disease. Pacritinib is an important component of Baxter’s growing oncology portfolio, and we look forward to partnering with CTI BioPharma to share these results with physicians and discussing next steps with regulatory agencies,” said David Meek, Head of Oncology at Baxter BioScience.

### **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 therapy or patients who are intolerant to or whose symptoms are sub-optimally managed on other JAK2 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 BAT, other than JAK inhibitors, in 327 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. The primary endpoint assessed the proportion of patients achieving a 35 percent or greater reduction in spleen volume from baseline to Week 24 as measured by MRI or CT, when compared with BAT, excluding treatment with JAK2 inhibitors. After the completion of 24 weeks of treatment or disease progression, crossover from the BAT arm to pacritinib was allowed.

**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. Patients will be randomized to receive 200 mg pacritinib twice daily (BID), 400 mg pacritinib once daily (QD) or BAT. The trial is designed to enroll up to 300 patients in North America, Europe, Australia, New Zealand and Russia. In October 2013, CTI BioPharma reached agreement with the FDA on a Special Protocol Assessment (SPA) for the PERSIST-2 trial, which is a written agreement between CTI BioPharma and the FDA regarding the planned design, endpoints and statistical analysis approach of the trial to be used in support of a potential NDA submission. Under the SPA, the agreed upon co-primary endpoints are the proportion of patients achieving a 35 percent or greater reduction in spleen volume from baseline to Week 24 of treatment 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. Additional details are available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) or [www.PERSISTprogram.com](http://www.PERSISTprogram.com).

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.

### **Conference Call Information**

CTI BioPharma management will host a conference call today, March 9, 2015 at 8:00 a.m. EDT / 7:00 a.m. CDT / 5:00 a.m. PDT / 1:00 p.m. CET. Participants can access the call at 1-888-471-3843 (domestic) or +1 719-325-2376 (international). To access the live audio webcast or the subsequent archived recording, visit CTI BioPharma's website, [www.ctibiopharma.com](http://www.ctibiopharma.com). Webcast and telephone replays of the conference call will be available approximately two hours after completion of the call. Callers can access the replay by dialing 1-888-203-1112 (domestic) or +1 719-457-0820 (international). The access code for the replay is 1881293. The telephone replay will be available until Monday, March 16, 2015.

### **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 myeloproliferative neoplasms (MPNs), leukemia and lymphoma. Although pacritinib suppresses the JAK2/STAT3 pathway, pacritinib does not cause myelosuppression<sup>1</sup> 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.<sup>1</sup>

### **About Myelofibrosis and Myeloproliferative Neoplasms**

Myelofibrosis is a one of three main types of MPNs, which are a closely related group of hematological blood cancers. The three main types of MPNs are myelofibrosis, polycythemia vera, essential thrombocytopenia.<sup>2</sup>

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.<sup>3</sup> 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<sup>3</sup> and is a progressive disease with approximately 20 percent of patients eventually developing acute myeloid leukemia.<sup>4</sup> 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.<sup>5</sup>

### **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.

### **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](http://www.ctibiopharma.com).

Source: CTI BioPharma Corp. and Baxter International Inc.

### **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 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. In particular, this release addresses top-line results regarding primary endpoints, and should be evaluated together with secondary endpoints, safety and additional data once such data has been more fully analyzed and is made publicly available. 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. Neither Baxter nor CTI BioPharma undertakes to update its forward-looking statements.

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2. MPN Research Foundation website, [www.mpnresearchfoundation.org](http://www.mpnresearchfoundation.org).
3. Mehta J, Wang H, Iqbal SU, Mesa R. Epidemiology of myeloproliferative neoplasms in the United States. *Leukemia & Lymphoma* 2014; 55(3): 595-600.
4. 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.
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