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Testo del comunicato

Vedi allegato.

LONG-TERM FOLLOW UP DATA FOR PACRITINIB PRESENTED AT 2016 ASCO ANNUAL MEETING

-72 week follow up of patients with myelofibrosis, including patients with baseline thrombocytopenia, show pacritinib treatment led to durable reductions in spleen volume and symptom burden-

SEATTLE, June 7, 2016–CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA:CTIC) today announced long-term safety and efficacy results from the pivotal Phase 3 PERSIST-1 trial evaluating pacritinib versus best available therapy, excluding treatment with JAK2 inhibitors (BAT), in patients with myelofibrosis. As previously reported, the PERSIST-1 trial met its primary endpoint in the intent-to-treat population with statistically significant reduction in spleen volume when compared to patients receiving BAT. The results represent an update on the efficacy and safety for all patients regardless 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 most frequently occurring adverse events with pacritinib were gastrointestinal events and incidence decreased over time. For patients crossing over to receive pacritinib treatment (84 percent of BAT patients), less than 5 percent of patients had diarrhea with only one patient experiencing grade 3. Patients in the BAT arm that crossed over to receive pacritinib treatment had a similar rate of events as patients initially randomized to BAT or pacritinib. A planned analysis of the study up to 72 weeks demonstrated treatment with pacritinib led to durable reductions in spleen volume and symptom burden, two key measures of disease control, in patients with myelofibrosis, including patients with low platelets at baseline (less than 50,000 per microliter and less than 100,000 per microliter). Patients who crossed over to pacritinib from BAT experienced similar reductions in spleen volume and symptom burden as patients initially randomized to pacritinib, including patients with low platelets. Pacritinib is an investigational oral kinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R, which are kinases found to be involved in the growth and spread of myelofibrosis and other blood-related cancers.

"From the time a patient is diagnosed with myelofibrosis, the incidence of disease-related thrombocytopenia increases with time," 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. "Patients with thrombocytopenia have significantly greater symptom burden, distinct clinical characteristics and shorter overall survival. There is currently a significant unmet need for patients with myelofibrosis who are unable to tolerate or control their disease on other treatments due to low platelet counts.

Dr. Harrison added, "It is encouraging to see that patients with baseline thrombocytopenia who were treated with pacritinib, had stable mean platelet counts and hemoglobin levels through the end of treatment, and that some patients with very low platelets increased their platelet counts while receiving pacritinib treatment. In this intermediate- to high-risk patient group, pacritinib was generally well tolerated."

Myelofibrosis is a rare, but serious and life-threatening chronic leukemia that disrupts the normal production of blood cells and results in scarring of the bone marrow, limiting the ability to produce new blood cells and prompting the spleen and other organs to take over this function. The disease often leads to an enlarged spleen and lower than normal counts of blood cells – including red blood cells and platelets, which are essential for blood clotting. Frequent causes of death among patients with myelofibrosis include leukemic transformation (31 percent), disease progression (18 percent), and thrombosis and cardiovascular complications (13 percent) [1].

Below are highlights presented at the 52nd Annual Meeting of the American Society of Clinical Oncology (ASCO) being held June 3–June 7, 2016 in Chicago, Ill. The poster presentations are available at www.ctibiopharma.com.

Highlights of Data Presented

- Mesa, R, et al. *Pacritinib (PAC) vs. best available therapy (BAT) in myelofibrosis (MF): 72 week follow-up of the phase III PERSIST-1 trial*. Abstract #7065

  In this poster, an update on efficacy and safety of the PERSIST-1 clinical trial up to Week 72 is provided for all patients in the study (pacritinib n=220; BAT n=107), regardless of platelet count. Ninety patients (84 percent) randomized to BAT crossed over to receive pacritinib at a median of 27.2 weeks.

  Responses to pacritinib were durable and rates of 35 percent or greater spleen volume reduction (SVR) were maintained from Weeks 24 to 72 (25 percent vs. 24 percent). Patients who crossed over to receive pacritinib achieved similar responses to those receiving initial treatment with pacritinib. Overall survival, although not a primary or secondary endpoint of the trial, did not show a statistically significant difference between the pacritinib and BAT arms. This was primarily due to an imbalance between the two arms, with higher risk patients in the pacritinib arm vs. BAT. The result was potentially confounded by a high percentage of patients who crossed over at Week 24 to receive pacritinib therapy. Pacritinib-treated patients who achieved SVR greater or equal to 20 percent had statistically significant longer overall survival vs. patients who did not achieve SVR at this level.

  The most frequently occurring adverse events with pacritinib were gastrointestinal events and the incidence decreased over time. Incidence of grade 3/4 treatment-emergent diarrhea with initial pacritinib treatment was highest in Weeks 1-8 (3 percent) and decreased in Weeks 8-16 (1.4 percent), Weeks 16-24 (1.5 percent) and in the final period analyzed, Weeks 64-72 (0.9 percent). Up to 24 weeks, prior to the majority of patients in the BAT arm crossing over, there was no statistically significant difference in the incidence of cardiac and bleeding adverse events between the pacritinib and BAT arms; following crossover to pacritinib, BAT patients had a similar rate of all grades of adverse events. The incidence of all grade cardiac AEs was similar between pacritinib and BAT arms between Weeks 1 and 24; incidence of cardiac AEs was greater for pacritinib between Weeks 24 and 72 vs. BAT, but was low overall (5 percent or less at any time). Among all patients, incidence of grade 3/4 bleeding events was 3 percent or less during any 8-week time interval.

- Harrison, C, et al. *Pacritinib (PAC) vs. best available therapy (BAT) in myelofibrosis (MF): Outcomes in patients (pts) with baseline (BL) thrombocytopenia*. Abstract #7011

  This analysis examines outcomes up to 72 weeks among patients in the PERSIST-1 trial with baseline platelets less than 100,000 per microliter treated with pacritinib vs. BAT (pacritinib, n=72; BAT, n=34). For patients receiving pacritinib, a median duration of spleen volume reduction (SVR) of 35 percent or greater was 48 weeks for patients with baseline platelets less than 50,000 per microliter and 57 weeks for patients with baseline platelets less than 100,000 per microliter. For BAT-treated patients who crossed over to receive pacritinib before or after Week 24, duration of SVR was 73 weeks for patients with baseline platelets less than 50,000 per microliter and 46 weeks for patients with baseline platelets less than 100,000 per microliter. At Week 36, 46 percent (6/13) of evaluable pacritinib-treated patients with baseline platelets less than 50,000 per microliter and 48 percent (12/28) of evaluable pacritinib-treated patients with baseline platelets less than 100,000 per microliter achieved 50 percent or greater reduction in Total Symptom Score (TSS). This is an increase from 32 percent and 42 percent, respectively, at Week 24.

  Patients treated with pacritinib had stable mean platelet counts and hemoglobin levels through Week 72 and increased platelet counts in patients with platelets less than 50,000 per microliter. At 24 weeks, bleeding events occurred at a similar rate in pacritinib-and BAT-treated patients; following crossover to pacritinib, BAT patients had a similar rate of events. Among patients with baseline thrombocytopenia treated with pacritinib, mean hemoglobin levels remained stable through Week 72. Due to BAT crossover to pacritinib at Week 24, there were no evaluable patients with baseline thrombocytopenia in the BAT arm beyond Week 36. These data suggest pacritinib treatment led to durable reductions in spleen volume and symptom burden.

- Harrison, C, et al. *Outcomes in patients with myelofibrosis and RBC-transfusion dependence in the phase III PERSIST-1 study of pacritinib vs. best available therapy*. Abstract #7066

In this poster, pacritinib-treated patients demonstrated clinically meaningful reductions in spleen volume independent of RBC-transfusion independence (RBC-TI). Among pacritinib-treated patients, 16 percent (36/220) were RBC-transfusion dependent (RBC-TD) at baseline. Eighteen (18) patients were RBC-TD at the time of crossover.

Twenty-two percent (12/54) of RBC-TD patients treated with pacritinib either from study start or after crossover achieved RBC-TI during the course of the study. During the course of the study, 25 percent (9/36) of RBC-TD patients treated with pacritinib at the start of the study achieved RBC-TI vs. 0 percent (0/16) patients treated with BAT (p=0.043). Seventeen percent (3/18 patients) of RBC-TD patients at the time of crossover achieved RBC-TI during the crossover period. Pacritinib treatment was associated with improved patient outcomes for those with baseline RBC-TD.

- Mesa, R, et al. *Pacritinib (PAC) vs. best available therapy (BAT) in myelofibrosis (MF): Long-term follow-up of patient-reported outcomes (PROs) in the phase III PERSIST-1 trial.* Abstract #7067

  Patient Reported Outcomes (PRO) data at 24 weeks was previously reported and the poster presented today provided an update at Week 48. The Myeloproliferative Neoplasm Symptom Assessment Form (MPN-SAF) is a PRO assessment tool designed to measure myelofibrosis-related symptom burden resulting in a TSS based on how the patient feels or functions in relation to six common symptoms. The proportion of patients achieving a TSS reduction of 50 percent or greater improved from Weeks 24 to 48 and was greater than observed with BAT showing that reduction in symptoms continued to increase over time. In patients who crossed over from BAT to pacritinib, reductions in TSS improved from Week 24 to Week 36. Mean percentage reductions in common symptoms measured in both MPN-SAF versions at Week 48 were greater than those observed at Week 24 among patients treated with pacritinib.

About PERSIST-1

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 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. 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. At study entry, 62 percent of patients had primary myelofibrosis; 46 percent of patients were thrombocytopenic; 32 percent of patients had baseline platelet counts less than 100,000 per microliter; and 16 percent of patients had platelet counts less than 50,000 per microliter; normal platelet counts range from 150,000 to 450,000 per microliter. The design of PERSIST-1 allowed for patients on the BAT arm to crossover and receive treatment with pacritinib if their disease progresses or after they achieve the 24-week measurement endpoint. Although crossover design of clinical trials may confound evaluation of survival, which is a tertiary endpoint of PERSIST-1, such designs are frequently used in cancer studies. 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 of patients (84 percent) on the BAT arm eventually crossed over to receive pacritinib therapy.

About Pacritinib

Pacritinib is an investigational oral kinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R. 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 for pacritinib are currently subject to a full clinical hold issued by the U.S. Food and Drug Administration in February 2016. A second Phase 3 study, known as PERSIST-2, is evaluating pacritinib in a subset of patients with myelofibrosis whose platelet counts are 100,000 per microliter or less. Although not all patients enrolled reached the 24-week endpoint prior to the full clinical hold on pacritinib, approximately two thirds of the enrolled patients reached or exceeded the 24-week endpoint evaluation. Therefore, these patients will contribute to the evaluation of the study endpoints. Based on the assumptions of

the design, CTI BioPharma believes there is sufficient power to reach statistical significance of the primary objectives. Top-line results from the PERSIST-2 Phase 3 trial of pacritinib are expected in the third quarter of 2016.

CTI BioPharma and Baxalta Incorporated (now part of Shire plc) are parties to a worldwide license agreement to develop and commercialize pacritinib. CTI BioPharma and Baxalta will jointly commercialize pacritinib in the U.S. while Baxalta 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 hematological blood cancers. The three main types of MPNs are myelofibrosis, polycethemia vera and essential thrombocythemia.[2]

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.[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 AML.[4] The median survival for high-risk myelofibrosis patients is less than one and a half years, while the median survival for patients with myelofibrosis overall is approximately six years.[5]

About CTI BioPharma

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 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 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, and expectations for the future availability of top-line results from the PERSIST-2 Phase 3 trial of pacritinib. 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 select 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. 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. Cervantes, F. *Blood* 2009;113:2895-2901
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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