CTI BioPharma Presents Pivotal Data from Pacritinib Program at the European Hematology Association (EHA) 2022 Congress

June 10, 2022

SEATTLE, June 10, 2022 /PRNewswire/ -- CTI BioPharma Corp. (Nasdaq: CTIC) today announced two scientific poster presentations from the Company's pacritinib clinical program at the European Hematology Association (EHA) 2022 Congress, being held in Vienna, Austria, June 9-12, 2022.

"Our presentions today demonstrate that full dose pacritinib achieved higher response rates and a similar, manageable safety profile compared to lower-dose ruxolitinib in patients with myelofibrosis who have moderate or severe thrombocytopenia," said Adam Craig, President and Chief Executive Officer of CTI BioPharma. "As the commercial launch of VONJO^{TM} (pacritinib) in the U.S. continues to exceed our expectations, we are pleased to highlight VONJO's clinical value as a potential best in class treatment for patients with cytopenic myelofibrosis with platelet counts below 50 × 10^9/L."

Presentation materials will be available at ctibiopharma.com.

Retrospective Comparison of Patient Outcomes on Pacritinib Versus Ruxolitinib in Patients with Myelofibrosis and Thrombocytopenia (New)

Poster Number: P1069
Session Name: Poster session
Session Date: Friday, June 10, 2022
Presentation Time: 16:30 – 17:45 p.m. CEST (10:30 – 11:45 a.m. ET)
Presenter: Prof. Claire Harrison

Pacritinib is a novel JAK2/IRAK1 inhibitor approved by the U.S. Food and Drug Administration (FDA) for patients with myelofibrosis and thrombocytopenia. Unlike the JAK 1/2 inhibitor ruxolitinib, which must be dose-reduced or held in patients with thrombocytopenia, pacritinib has been studied at full dose regardless of platelet count. In this part of of the Phase 3 PERSIST-2 study, which focuses on retrospectively analyzing outcomes in patients treated with pacritinib versus ruxolitinib, patients were randomized 1:1:1 to pacritinib 200 mg twice daily (BID), pacritinib 400 mg once daily (QD) or best available therapy (BAT), with 45 percent of patients on BAT receiving ruxolitinib.

This analysis demonstrates overall and fatal adverse events occurred at similar rates on pacritinib versus ruxolitinib, as did bleeding events. Cardiac events occurred more commonly on pacritinib, though the difference was largely due to higher rates of grade 1 peripheral edema on pacritinib. There were lower rates of herpes zoster reactivation (n=0 vs. 1), pulmonary aspergilosis (n=1 vs. 0), deep venous thrombosis (n=0 vs. 1) and pulmonary embolism (n=1 vs. 0) on pacritinib and ruxolitinib, respectively. These results show that pacritinib, administered at the full dose of 200 mg BID, yielded higher response rates and a similar safety profile compared to lower-dose ruxolitinib, in patients with myelofibrosis who have moderate or severe thrombocytopenia.

Risk-Adjusted Safety Analysis of Pacritinib in Patients with Myelofibrosis (Encore)

Poster Number: P1068
Session Name: Poster session
Session Date: Friday, June 10, 2022
Presentation Time: 16:30 – 17:45 p.m. CEST (10:30 – 11:45 a.m. ET)
Presenter: Dr. Naveen Pemmaraju

Pacritinib is a novel JAK2/IRAK1 inhibitor that has shown significant activity in patients with myelofibrosis, including those with platelet counts <50 × 10^9/L. This safety analysis focuses on toxicities of interest for patients treated with pacritinib 200 mg twice daily (BID) and best available therapy (BAT), including ruxolitinib, on the Phase 3 PERSIST-2 and Phase 2 PAC203 studies. Because the average treatment duration was longer for patients on pacritinib 200 mg BID on PERSIST-2 and PAC203 compared to BAT on PERSIST-2, this analysis presents adverse events rates in these patients corrected for duration of exposure.

This risk-adjusted analysis demonstrates that the safety profile of pacritinib 200 mg BID is comparable to BAT. In particular, rates of bleeding were not elevated on pacritinib 200 mg BID compared to BAT, both overall and in patients with PLT <50 x 10^9/L. Rates of fatal events, thrombosis, major adverse cardiac events (MACE) and non-melanoma skin cancer were higher on ruxolitinib than pacritinib. These results indicate that pacritinb 200 mg BID may represent a full-dose therapeutic option for patients with myelofibrosis, including those with thrombocytopenia.

About VONJO (pacritinib)

Pacritinib is an oral kinase inhibitor with activity against wild type Janus Associated Kinase 2 (JAK2), mutant $JAK2^{V617F}$ form and FMS-like tyrosine kinase 3 (FLT3), which contribute to signaling of a number of cytokines and growth factors that are important for hematopoiesis and immune function. Myelofibrosis is often associated with dysregulated JAK2 signaling. Pacritinib has higher inhibitory activity for JAK2 over other family members, JAK3 and TYK2. At clinically relevant concentrations, pacritinib does not inhibit JAK1. Pacritinib exhibits inhibitory activity against additional cellular kinases (such as CSF1R and IRAK1), the clinical relevance of which is unknown.

VONJO is indicated for the treatment of adults with intermediate or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis with a platelet count below 50 × 10^9/L. This indication is approved under accelerated approval based on spleen volume reduction. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Important VONJO Safety Information

Hemorrhage:

Serious (11%) and fatal (2%) hemorrhages have occurred in VONJO-treated patients with platelet counts <100 × 10^9/L. Serious (13%) and fatal (2%) hemorrhages have occurred in VONJO-treated patients with platelet counts <50 × 10^9/L. Grade ≥3 bleeding events (defined as requiring transfusion or invasive intervention) occurred in 15% of patients treated with VONJO compared to 7% of patients treated on the control arm. Due to hemorrhage, VONJO dose-reductions, dose interruptions, or permanent discontinuations occurred in 3%, 3%, and 5% of patients, respectively.

Avoid use of VONJO in patients with active bleeding and hold VONJO 7 days prior to any planned surgical or invasive procedures. Assess platelet counts periodically, as clinically indicated. Manage hemorrhage using treatment interruption and medical intervention.

Diarrhea:

VONJO causes diarrhea in approximately 48% of patients compared to 15% of patients treated on the control arm. The median time to resolution in VONJO-treated patients was 2 weeks. The incidence of reported diarrhea decreased over time with 41% of patients reporting diarrhea in the first 8 weeks of treatment, 15% in Weeks 8 through 16, and 8% in Weeks 16 through 24. Diarrhea resulted in treatment interruption in 3% of VONJO-treated patients. None of the VONJO-treated patients reported diarrhea that resulted in treatment discontinuation. Serious diarrhea adverse reactions occurred in 2% of patients treated with VONJO compared to no such adverse reactions in patients in the control arm.

Control pre-existing diarrhea before starting VONJO treatment. Manage diarrhea with antidiarrheal medications, fluid replacement, and dose-modification. Treat diarrhea with anti–diarrheal medications promptly at the first onset of symptoms. Interrupt or reduce VONJO dose in patients with significant diarrhea despite optimal supportive care.

Thrombocytopenia:

VONJO can cause worsening thrombocytopenia. VONJO dosing was reduced due to worsening thrombocytopenia in 2% of patients with pre–existing moderate to severe thrombocytopenia (platelet count <100 × 10^9/L). VONJO dosing was reduced due to worsening thrombocytopenia in 2% of patients with pre–existing severe thrombocytopenia (platelet count <50 × 10^9/L).

Monitor platelet count prior to VONJO treatment and as clinically indicated during treatment. Interrupt VONJO in patients with clinically significant worsening of thrombocytopenia that lasts for more than 7 days. Restart VONJO at 50% of the last given dose once the toxicity has resolved. If toxicity recurs hold VONJO. Restart VONJO at 50% of the last given dose once the toxicity has resolved.

Prolonged QT interval:

VONJO can cause prolongation of the QTc interval. QTc prolongation of >500 msec was higher in VONJO-treated patients than in patients in the control arm (1.4% vs 1%). QTc increase from baseline by 60 msec or higher was greater in VONJO-treated patients than in control arm patients (1.9% vs 1%). Adverse reactions of QTc prolongation were reported for 3.8% of VONJO-treated patients and 2% of control arm patients. No cases of torsades de pointes were reported.

Avoid use of VONJO in patients with a baseline QTc of >480 msec. Avoid use of drugs with significant potential for QTc prolongation in combination with VONJO. Correct hypokalemia prior to and during VONJO treatment. Manage QTc prolongation using VONJO interruption and electrolyte management.

Major Adverse Cardiac Events (MACE):

Another Janus associated kinase (JAK)-inhibitor has increased the risk of MACE, including cardiovascular death, myocardial infarction, and stroke (compared to those treated with TNF blockers) in patients with rheumatoid arthritis, a condition for which VONJO is not indicated.

Consider the benefits and risks for the individual patient prior to initiating or continuing therapy with VONJO particularly in patients who are current or past smokers and patients with other cardiovascular risk factors. Patients should be informed about the symptoms of serious cardiovascular events and the steps to take if they occur.

Thrombosis:

Another JAK-inhibitor has increased the risk of thrombosis, including deep venous thrombosis, pulmonary embolism, and arterial thrombosis (compared to those treated with TNF blockers) in patients with rheumatoid arthritis, a condition for which VONJO is not indicated.

Patients with symptoms of thrombosis should be promptly evaluated and treated appropriately.

Secondary Malignancies:
Another JAK-inhibitor has increased the risk of lymphoma and other malignancies excluding non-melanoma skin cancer (NMSC) (compared to those treated with TNF blockers) in patients with rheumatoid arthritis, a condition for which VONJO is not indicated. Patients who are current or past smokers are at additional increased risk.

Consider the benefits and risks for the individual patient prior to initiating or continuing therapy with VONJO, particularly in patients with a known malignancy (other than a successfully treated NMSC), patients who develop a malignancy, and patients who are current or past smokers.

Risk of Infection:
Another JAK-inhibitor has increased the risk of serious infections (compared to best available therapy) in patients with myeloproliferative neoplasms. Serious bacterial, mycobacterial, fungal and viral infections may occur in patients treated with VONJO. Delay starting therapy with VONJO until active serious infections have resolved. Observe patients receiving VONJO for signs and symptoms of infection and manage promptly. Use active surveillance and prophylactic antibiotics according to clinical guidelines.

Interactions with CYP3A4 Inhibitors or Inducers:
Co-administration of VONJO with strong CYP3A4 inhibitors or inducers is contraindicated. Avoid concomitant use of VONJO with moderate CYP3A4 inhibitors or inducers.

Drug interruptions due to an adverse reaction occurred in 27% patients who received VONJO 200 mg twice daily compared to 10% of patients treated with BAT. Dosage reductions due to an adverse reaction occurred in 12% of patients who received VONJO 200 mg twice daily compared to 7% of patients treated with BAT. Permanent discontinuation due to an adverse reaction occurred in 15% of patients receiving VONJO 200 mg twice daily compared to 12% of patients treated with BAT.

Please visit http://www.ctibiopharma.com/vonjo_prescribing_information for full Prescribing Information and the Medication Guide.

About Myelofibrosis
Myelofibrosis is bone marrow cancer that results in formation of fibrous scar tissue and can lead to thrombocytopenia and anemia, weakness, fatigue and an enlarged spleen and liver. Within the United States, there are approximately 21,000 patients with myelofibrosis, 7,000 of which have severe thrombocytopenia (defined as blood platelet counts of less than 50 x10^9/L). Severe thrombocytopenia is associated with poor survival and high symptom burden and can occur as a result of disease progression or from drug toxicity with other JAK2 inhibitors, such as JAKAFI and INREBIC.

About CTI BioPharma Corp.
We are a commercial biopharmaceutical company focused on the acquisition, development and commercialization of novel targeted therapies for blood-related cancers that offer a unique benefit to patients and their healthcare providers. CTI has one FDA-approved product VONJO™ (pacritinib), a JAK2 and IRAK1, that spares JAK1. VONJO is approved for the treatment of adults with intermediate or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis with a platelet count below 50 × 10^9/L. CTI is conducting the Phase 3 PACIFICA study of VONJO in patients with myelofibrosis and severe thrombocytopenia as a post-marketing requirement.

VONJO™ is a trademark of CTI BioPharma Corp.

Forward-Looking Statements
Statements included in this press release that are not historical in nature are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. These forward-looking statements, including statements that pacritnib may be a potential best in class treatment for patients with cytopenic myelofibrosis with platelet counts below 50 × 10^9/L, are based on current assumptions that involve risks, uncertainties and other factors that may cause the actual results, events or developments to be materially different from those expressed or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to those risks more fully discussed in the section entitled "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2021 and subsequent quarterly reports on Form 10-Q. These forward-looking statements speak only as of the date hereof and we assume no obligation to update these forward-looking statements, and readers are cautioned not to place undue reliance on such forward-looking statements. "CTI BioPharma" and the CTI BioPharma logo are registered trademarks or trademarks of CTI BioPharma Corp. in various jurisdictions. All other trademarks belong to their respective owner.

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