CTI BioPharma Announces Presentation of Data Supporting Pacritinib's Potential Benefit in Preventing Acute GVHD at the 62nd American Society of Hematology Meeting

December 6, 2020

Results from Investigator-sponsored Phase 1/2 Study of Acute GVHD Prophylaxis Following Allogeneic Hematopoietic Cell Transplantation Demonstrate Encouraging Efficacy and Safety Profile when Combining Pacritinib 100 mg BID with Standard Immunosuppressive Therapy

Phase 2 Investigator-sponsored Clinical Trial Underway to Further Evaluate Efficacy and Safety of Pacritinib 100 mg BID in Combination with Standard Immunosuppressive Therapy

SEATTLE, Dec. 6, 2020 /PRNewswire/ -- CTI BioPharma Corp. (Nasdaq: CTIC) today announced an oral presentation supporting the Company's pacritinib development program in the prevention of acute graft versus host disease (GVHD) at the 62nd American Society of Hematology (ASH) Annual Meeting & Exposition, being held virtually December 5-8, 2020. The results are from an investigator-sponsored Phase 1/2 study conducted at the Mayo Clinic and Moffit Cancer Center.

"The data presented today at ASH highlights the potential for the expanded use of pacritinib. Acute GVHD following allogeneic hematopoietic cell transplantation results in higher rates of morbidity and mortality, compromises the anti-leukemic response of the graft and occurs despite standard immunosuppressive prophylaxis, rendering it an area where new therapeutic options are needed," said Adam R. Craig, M.D., Ph.D., President and Chief Executive Officer of CTI Biopharma. "These data demonstrate that in this Phase 1/2 study, adding pacritinib to the standard prophylaxis of sirolimus and low-dose tacrolimus resulted in a significant reduction in the expected acute GVHD rates in patients within the first 100 days of therapy as compared to historical data, without compromising transplantation outcomes and without any new safety concerns. We are encouraged by these data, and look forward to further evaluating pacritinib's efficacy and safety in this indication in the on-going Phase 2 clinical trial."

All presentation materials will be available at ctibiopharma.com following the presentations.

Biological and Clinical Impact of JAK2/mTOR Blockade in GVHD Prevention: Preclinical and Phase 1 / 2 Study Results
Results from an investigator-sponsored (Mayo Clinic and Moffit Cancer Center) Phase 1/2 study of GVHD prophylaxis for allogeneic hematopoietic cell transplantation (alloHCT) using pacritinib, sirolimus, low-dose tacrolimus are being presented today, Sunday, December 6 at 10 a.m. PT in an oral presentation session.
Abstract: No. 355
Summary: This single-arm Phase 1/2 study (NCT02891603) tested the safety, pharmacodynamics and efficacy of pacritinib when administered with sirolimus plus low-dose tacrolimus (PAC/SIR/TAC) after alloHCT. A 3+3 dose escalation design was planned using three doses of pacritinib (100 mg QD, 100 mg BID, 200 mg BID) in combination with standard sirolimus and low-dose tacrolimus immunosuppressive therapy. Efficacy endpoints included acute GVHD (aGVHD) rate at day 100.

PAC 100 mg twice daily (BID), dose level 2, was selected as the recommended Phase 2 dose based on its biological activity and safety profile. Preliminary evidence of the benefit of adding pacritinib to standard immunosuppressive therapy in reducing the rates of aGVHD was reported, with a 25% rate of grade 2-4 aGVHD across all dose cohorts, and 17% at the recommended Phase 2 dose of 100 mg BID, as compared to grade 2-4 aGVHD incidence rate of 43% expected with sirolimus plus low-dose tacrolimus administered alone[1]. No compromise in alloHCT outcome or major safety concerns were observed. Futher, there was no evidence of cytopenias, impaired immune reconstitution, or cytomegalovirus (CMV) reactivation. A Phase 2 clinical trial to fully evaluate efficacy in GVHD prevention is ongoing (NCT02891603).

About Pacritinib

Pacritinib is an investigational oral kinase inhibitor with specificity for JAK2, IRAK1, and CSF1R. 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. In addition to myelofibrosis, 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 inhibition of c-fms, IRAK1, JAK2 and FLT3.

About CTI BioPharma Corp.

We are a 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. We concentrate our efforts on treatments that target blood-related cancers where there is an unmet medical need. In particular, we are focused on evaluating pacritinib, our sole product candidate currently in active late-stage development, for the treatment of adult patients with myelofibrosis. In addition, we have recently started developing pacritinib for use in the prevention of acute graft versus host disease and in hospitalized patients with severe COVID-19, in response to the COVID-19 pandemic. We are headquartered in Seattle, Washington.

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, Section 21E of the Securities Exchange Act of 1934 and the Private Securities Litigation Reform Act of 1995. These forward-looking statements 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: our ability to successfully demonstrate the safety and efficacy of pacritinib in various indications; our ability to receive regulatory approval for pacritinib pursuant to the accelerated approval pathway or at all; our planned commercialization of pacritinib; our ability to enter into potential partnerships relating to our commercial launch of pacritinib; and 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, 2019 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.

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1. Pidala et al, Oral Presentation, Abstract 355, ASH 2020.

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