CTI BioPharma Initiates Rolling Submission of New Drug Application (NDA) for Pacritinib in Myelofibrosis Patients with Severe Thrombocytopenia

October 13, 2020

- Completion of Submission Expected in First Quarter 2021 -

- Pre-Commercialization Activities are Underway -

SEATTLE, Oct. 13, 2020 /PRNewswire/ -- CTI BioPharma Corp. (Nasdaq: CTIC) today announced that it has commenced a rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration ("FDA" or "the Agency") seeking approval of pacritinib as a treatment for myelofibrosis patients with severe thrombocytopenia (platelet counts less than 50 x 10^9/L). CTI has previously announced the results of a recent Pre-NDA meeting with FDA where agreement was reached on an NDA submission package based upon available data from the completed Phase 3 PERSIST-1 and PERSIST-2, and the Phase 2 PAC203 trials.

"Today we are pleased to announce the start of a rolling NDA submission that seeks to address the important unmet medical need of myelofibrosis patients with severe thrombocytopenia, a population that includes both front-line treatment-naïve patients and patients with prior exposure to JAK2 inhibitors," said Adam R. Craig, M.D., Ph.D., President and Chief Executive Officer of CTI Biopharma. "We have started pre-commercial activities and are planning for a commercial launch in 2021, subject to priority review."

About Myelofibrosis and Severe Thrombocytopenia

Myelofibrosis is a type of bone marrow cancer that results in formation of fibrous scar tissue and can lead to severe thrombocytopenia and anemia, weakness, fatigue and enlarged spleen and liver. Patients with severe thrombocytopenia are estimated to make up more than one-third of patients treated for myelofibrosis, or approximately 17,000 people in the United States and Europe. Severe thrombocytopenia, defined as blood platelet counts of less than 50,000 per microliter, has been shown to result in overall survival rates of just 15 months. Thrombocytopenia in patients with myelofibrosis is associated with the underlying disease but has also been shown to correlate with treatment with ruxolitinib, which can lead to dose reductions, and as a result, may potentially reduce clinical benefit. Survival in patients who have discontinued ruxolitinib therapy is further compromised, with an average overall survival of seven to 14 months. Myelofibrosis patients with severe thrombocytopenia have limited treatment options, creating a significant area of unmet medical need.

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 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 and 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; our ability to complete a rolling NDA for pacritinib in the timeline currently anticipated; 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. "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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