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

March 31, 2021

- Commercial Preparations Underway to Support Potential Approval and Launch of Pacritinib in the United States in 2021 -

SEATTLE, March 31, 2021 /PRNewswire/ -- CTI BioPharma Corp. (Nasdaq: CTIC) today announced that it has completed a rolling New Drug Application ("NDA") submission to the U.S. Food and Drug Administration ("FDA") seeking approval of pacritinib as a treatment for myelofibrosis patients with severe thrombocytopenia (platelet counts less than 50 x 10^9/L). CTI had previously announced the results of a 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 trials and the Phase 2 PAC203 trials.

"The completion of the pacritinib NDA submission is the result of many years of clinical research and a collaborative and constructive dialogue with the FDA on how pacritinib could address the unmet medical need of myelofibrosis ("MF") patients with severe thrombocytopenia. MF patients with severe thrombocytopenia experience poor treatment outcomes, primarily due to their severely cytopenic disease and the significant limitations of approved therapies," said Adam R. Craig, M.D., Ph.D., President and Chief Executive Officer of CTI BioPharma. "CTI has initiated pre-commercialization activities and has completed the hiring of a commercial leadership team. Assuming a successful priority review of the NDA, we are preparing for a commercial launch of pacritinib before the end of 2021. We look forward to providing updates on the NDA and our commercialization plans over the coming months."

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 cytopenias, including thrombocytopenia and anemia, as well as weakness, fatigue and an 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 development, for the treatment of adult patients with myelofibrosis. In addition, are developing pacritinib for use 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 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 conduct and complete clinical trials in our currently anticipated timeframes; our ability to successfully demonstrate the safety and efficacy of pacritinib; our expectations regarding the completion and outcome of our PACIFICA Phase 3 trial and our PRE-VENT Phase 3 trial; the risk that the FDA may determine that the benefit/risk profile of pacritinib at the dose selected for the PACIFICA Phase 3 trial does not support approval; the risk that the FDA may determine that the benefit/risk profile of pacritinib in the PRE-VENT Phase 3 trial does not support approval or requires additional clinical data for approval; the risk that pacritinib may fail in its development through our PACIFICA and PRE-VENT trial; our ability to receive regulatory approval for pacritinib pursuant to the accelerated approval pathway or at all; the risk that pacritinib may be delayed to a point where it is not commercially viable; the accuracy of our assumptions regarding our planned expenditures and sufficiency of our cash to fund operations; risks and uncertainties related to the COVID-19 pandemic as it relates to our operations and ongoing clinical trials; 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, 2020 and subsequent reports filed with the Securities and Exchange Commission. 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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