

CTI BioPharma Begins Patient Enrollment in PACIFICA Pivotal Phase 3 Trial of Pacritinib in Myelofibrosis Patients With Severe Thrombocytopenia

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- Company Expects to Report Topline Data in Mid-2021 -

SEATTLE, Oct. 1, 2019 /PRNewswire/ -- [CTI BioPharma Corp.](http://www.ctibio.com) (Nasdaq: CTIC) today announced that it has initiated patient enrollment in the PACIFICA pivotal Phase 3 trial of its investigational myelofibrosis treatment candidate, pacritinib. The PACIFICA trial will compare the safety and efficacy of 200 mg of pacritinib administered twice daily (BID) to Physician's Choice in 180 adult myelofibrosis patients with severe thrombocytopenia (platelet counts of less than 50,000 per microliter).

"Initiation of the PACIFICA Phase 3 trial is an important step forward for the company and the pacritinib development program," said Adam R. Craig, M.D., Ph.D., President and Chief Executive Officer of CTI BioPharma. "An estimated one-third of patients with myelofibrosis are severely thrombocytopenic – a population with limited therapeutic options and poor survival, thereby making this disease setting a very important area of unmet medical need. Moving forward, successful trial execution is our primary focus, and with patient enrollment now underway, we expect to report topline results in mid-2021."

The PACIFICA trial is a randomized, active-comparator trial designed to evaluate the safety and efficacy of 200 mg of pacritinib administered twice daily (BID) compared to Physician's Choice in 180 myelofibrosis patients with severe thrombocytopenia (platelet counts of less than 50,000 per microliter). Patients will be randomized in a ratio of 2:1 between pacritinib and Physician's Choice, which may include steroids, thalidomide or lenolidamide, hydroxyurea or low-dose ruxolitinib. The primary endpoint of the trial is the percentage of patients who achieve at least 35% reduction in spleen volume at 24 weeks. Dr. Srdam Verstovsek, Professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, and Dr. John Mascarenhas, Associate Professor of Medicine Myeloproliferative Disorders Program, Tisch Cancer Institute, Mount Sinai School of Medicine, will be co-principal investigators in the PACIFICA trial. Professor Claire Harrison, Professor of Medicine, Guy's and St Thomas' NHS Foundation Trust, London, will chair the trial's Steering Committee.

The PACIFICA trial initiation follows the Type B, End-of-Phase-2a meeting with the U.S. Food and Drug Administration ("FDA" or "the Agency") held in July 2019 and the FDA's acceptance of an amendment to the company's PAC203 trial protocol, which has enabled a rapid transition to the PACIFICA Phase 3 trial. Results from the randomized, open-label Phase 2 PAC203 dose-finding trial are expected to be presented at a scientific conference before the end of 2019. For more information on the PACIFICA Phase 3 trial, please go to PACIFICA-trial.com.

The company's previously conducted Phase 3 PERSIST program consisted of the PERSIST-1 trial, which included a broad set of patients without limitations on platelet counts, and the PERSIST-2 trial, which was conducted in patients with low platelet counts. An ad-hoc analysis of pooled data from PERSIST-1 and PERSIST-2 evaluated results from patients with platelet counts of less than 50,000 per microliter and showed that 23% (n=104) of patients administered pacritinib had a $\geq 35\%$ spleen volume reduction (SVR), compared to 2% (n=48) (p=0.0007) given the best available therapy, which in the PERSIST-1 trial excluded JAK2 inhibitors and in the PERSIST-2 trial included the approved JAK2 inhibitor, ruxolitinib. The most common treatment-emergent adverse events of any grade occurring in 20% or more of patients treated with pacritinib within 24 weeks during the PERSIST-1 and PERSIST-2 trials were gastrointestinal (generally manageable diarrhea, nausea and vomiting) and hematologic (anemia and thrombocytopenia).

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 anemia, 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 18,000 people.¹ 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.² 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.^{3,4} There are currently no approved therapies available to treat myelofibrosis patients with severe thrombocytopenia, or patients who have failed ruxolitinib treatment, thereby making this a significant unmet medical need.

About Pacritinib

Pacritinib is an investigational oral kinase inhibitor with specificity for JAK2, FLT3, 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.

CTI BioPharma Corp. is 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. In particular, we are focused on evaluating pacritinib for the treatment of adult patients with myelofibrosis. CTI BioPharma is headquartered in Seattle, Washington.

Forward-Looking Statements

This press release contains 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 include statements regarding our expectations regarding: the effectiveness of, and potential changes to, the PACIFICA Phase 3 trial design; the timing of, and results from, clinical trials and other development activities related to pacritinib, including the anticipated PACIFICA Phase 3 trial and its related protocol; the potential efficacy, safety profile, future development plans, addressable market, regulatory success and commercial potential of pacritinib; the anticipated timing of regulatory submissions and interactions; our ability to successfully develop and achieve milestones in the development of

pacritinib; and the anticipated benefits of pacritinib. 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, many of which are beyond our control, include, but are not limited to: clinical trials may not demonstrate safety and efficacy of pacritinib; 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 based on the results of such trial, previously identified FDA concerns regarding safety and dosing limitations or otherwise; pacritinib may fail in development, may not receive required regulatory approvals, or may be delayed to a point where it is not commercially viable; our assumptions regarding our planned expenditures and sufficiency of our cash to fund operations may be incorrect; we may not achieve additional milestones in our pacritinib development program; the impact of competition; the impact of expanded product development and clinical activities on operating expenses; adverse conditions in the general domestic and global economic markets; as well as the other risks identified in our filings 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.

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¹ Company estimates based on internal company research

² Masarova et al., *Eur J Haematol.* 2017

³ Newberry, *Blood* 2017

⁴ Mehra, et al. ASH 2016 poster, Abstract 4769

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