CTI BioPharma Corp. Logo

CTI BioPharma Announces Outcome From End-of-Phase-2a Meeting With U.S. Food and Drug Administration (FDA) Regarding Pacritinib for Treatment of Myelofibrosis

July 18, 2019

- Company Plans to Advance Pacritinib 200 mg Twice-Daily (BID) Dose to Phase 3 for Patients With Severe Thrombocytopenia -

- Company Plans to Initiate PACIFICA Phase 3 Trial in Third Quarter of 2019 -

SEATTLE, July 18, 2019 /PRNewswire/ -- CTI BioPharma Corp. (Nasdaq: CTIC) ("CTI" or "the Company") today announced the outcome of a Type B, End-of-Phase-2a meeting with the U.S. Food and Drug Administration ("FDA" or "the Agency") for the continued development of its investigational myelofibrosis treatment candidate, pacritinib. Following this meeting, CTI plans to evaluate 200 mg of pacritinib administered twice daily (BID) in 180 patients with myelofibrosis and severe thrombocytopenia. The Company plans to initiate the Phase 3 PACIFICA study in the third quarter of 2019.

"We are pleased to be able to move the pacritinib program forward and are now in the process of finalizing an amendment to the PAC203 protocol, which the FDA will review, to allow a transition to the new PACIFICA Phase 3 study, in which we plan to compare the 200 mg BID dose of pacritinib to Physician's Choice in myelofibrosis patients with severe thrombocytopenia, an important unmet medical need," said Adam R. Craig, M.D., Ph.D., President and Chief Executive Officer of CTI BioPharma. "We anticipate initiating the trial in the third quarter, which would put us on track for topline Phase 3 data in mid-2021."

The randomized, open-label Phase 2 PAC203 dose-finding study was designed to evaluate the safety and efficacy of three dosing regimens of oral pacritinib in 150 patients with myelofibrosis. The Company expects topline safety and efficacy results from the Phase 2 portion of the PAC203 study in the third quarter of 2019 and is targeting presentation of the Phase 2 results at a scientific conference before the end of 2019.

The previous Phase 3 PERSIST program consisted of the PERSIST-1 trial, which was conducted in 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 ≥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).

The Company is currently amending the protocol for the ongoing Phase 2 PAC203 study to include the new PACIFICA Phase 3 portion, in which CTI intends to compare the safety and efficacy of 200 mg of pacritinib administered twice daily to Physician's Choice in 180 myelofibrosis patients with severe thrombocytopenia (platelet counts of less than 50,000 per microliter) at approximately 120 sites worldwide. Patients will be randomized in a ratio of 2:1 between pacritinib and Physician's Choice. The primary endpoint of the trial is the percentage of patients who achieve at least 35% reduction in spleen volume at week 24. 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 study. In addition, Professor Claire Harrison, Professor of Medicine, Guy's and St Thomas' NHS Foundation Trust, London, will chair the study's Steering Committee.

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, currently the only approved treatment for myelofibrosis, 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 development and commercialization of 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 about: our ability to commence the PACIFICA Phase 3 trial of pacritinib; the anticipated trial design and enrollment of the PACIFICA Phase 3 trial; 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, including FDA review of the amended PACIFICA Phase 3 protocol; 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; we may not achieve additional milestones in our proprietary or partnered programs; 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.

[1] Company estimates based on: July 2018 Kantar Health Myelofibrosis Demand Market Research Global Report; SmartAnalyst Quantitative Web survey (N=120); 2015 ZS Pacritinib MD Treatment Flow US/EU5; IMS Jakafi Prescription data Q3 2014; and 2017 Kantar Health MF Opportunity Assessment.

[2] Masarova et al., *Eur J Haematolol*. 2017

[3] Newberry, *Blood* 2017

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

CTI BioPharma Investor Contacts:
Maeve Conneighton/Maghan Meyers
+212-600-1902
cti@argotpartners.com

View original content to download multimedia:http://www.prnewswire.com/news-releases/cti-biopharma-announces-outcome-from-end-of-phase-2a-meeting-with-us-food-and-drug-administration-fda-regarding-pacritinib-for-treatment-of-myelofibrosis-300887147.html

SOURCE CTI BioPharma Corp.