CTI BioPharma Announces Enrollment of First Patient in COVID-19 PRE-VENT Phase 3 Clinical Trial

June 1, 2020

- Company Expects to Report Topline Data by the End of 2020 -

SEATTLE, June 1, 2020 /PRNewswire/ -- CTI BioPharma Corp. (Nasdaq: CTIC) today announced that it has enrolled the first patient in the Phase 3 PRE-VENT trial (NCT04404361) of pacritinib in hospitalized patients with severe COVID-19. PRE-VENT, a randomized, double-blind, placebo-controlled multicenter study, will compare pacritinib plus standard of care versus placebo and standard of care in 358 hospitalized patients with severe COVID-19, including patients with and without cancer. The primary endpoint of the trial will assess the proportion of patients who progress to invasive mechanical ventilation and/or extracorporeal membrane oxygenation or die by Day 28.

"Initiation of patient enrollment in the PRE-VENT Phase 3 trial is an important step for CTI as we work towards providing a new therapeutic option for patients with severe COVID-19," said Adam R. Craig, M.D., Ph.D., President and Chief Executive Officer of CTI BioPharma. "As a multi-kinase inhibitor, pactrinib has the potential to reduce the effects of the cytokine storm that occurs with the novel coronavirus infection, an inflammatory response that frequently leads to respiratory failure and need for mechanical ventilation."

"Preventing COVID-19 patients from developing the severe inflammatory response that leads to respiratory failure is critical as the medical community works to ameliorate the effects of the ongoing pandemic," said John Mascarenhas, M.D., Associate Professor, Medicine, Hematology and Medical Oncology, Tisch Cancer Institute, Icahn School of Medicine at Mount Sinai, New York and Chief Investigator of the PRE-VENT Study. "Because the cytokine storm occurs when multiple inflammatory cytokines are activated, including IL-1 and IL-6, a multi-kinase inhibitor such as pacritinib has the potential to inhibit the inflammatory response and improve patient outcomes."

Cytokine storm is a pathological immune reaction that can be triggered by viral infection and can lead to serious complications, including acute respiratory distress syndrome (ARDS). Multiple inflammatory cytokines are upregulated in patients with severe COVID-19, including IL-1 and IL-6, and some patients have evidence of over-active macrophage activation. As a JAK2/IRAK-1 inhibitor, pacritinib may ameliorate the effects of cytokine storm via inhibition of IL-6 and IL-1 signaling. Furthermore, as a CSF-1R inhibitor, pacritinib may mitigate effects of macrophage activation syndrome. Of particular importance in this indication, pacritinib has not been associated with increased risk in infections in prior randomized studies, likely because it does not have inhibitory effects on JAK1.

About PRE-VENT
PRE-VENT is expected to enroll 358 patients randomized 1:1 to receive pacritinib (400 mg once daily on Day 1, then 200 mg twice daily from Day 2 to Day 14) + SOC or placebo + SOC. Assigned treatment will continue for up to Day 14 or until the patient experiences intolerable adverse events, withdraws consent, initiates another investigational therapy, or until the study is terminated. Assigned therapy may be given for an additional 7 days (for a total of 21 days) at the discretion of the investigator. In the event of hospital discharge, patients will complete treatment with the assigned therapy on an outpatient basis. Severe COVID-19 will be defined as an oxygen saturation (SO2) ≤93% on room air at sea level, respiratory frequency >30 breaths per minute, ratio of arterial partial pressure of oxygen to fraction of inspired oxygen (PaO2/FiO2) <300, or lung infiltrates >50% on radiographic imaging.

The primary endpoint is the effect of treatment on the proportion of patients who require invasive mechanical ventilation and/or extracorporeal membrane oxygenation, or die by Day 28. An interim analysis will be conducted for futility after 154 patients have been enrolled. Safety will be assessed through 30 days of follow-up after the last dose of study treatment and assessed by the cumulative incidence, severity and seriousness of treatment-emergent AEs, drug discontinuations, laboratory values, and clinical assessments.

Further information on the PRE-VENT study can be found at https://clinicaltrials.gov/ct2/show/NCT04404361?term=NCT04404361&draw=2&rank=1.

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 inflammatory and immune-mediated disorders such acute graft-versus-host disease (aGVHD) and hemophagocytic lymphohistiocytosis (HLH).

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. 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 the PACIFICA Phase 3 trial and the 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 development; our ability to submit an 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; 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, 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.

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-enrollment-of-first-patient-in-covid-19-pre-vent-phase-3-clinical-trial-301068348.html

SOURCE CTI BioPharma Corp.