

# Bit Market Services

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Oggetto : CTI BIOPHARMA TO SUBMIT NDA FOR  
PACRITINIB IN Q4 BASED PRIMARILY  
ON DATA FROM SINGLE PIVOTAL  
PERSIST-1 TRIAL

*Testo del comunicato*

Vedi allegato.



## **CTI BIOPHARMA TO SUBMIT NDA FOR PACRITINIB IN Q4 BASED PRIMARILY ON DATA FROM SINGLE PIVOTAL PERSIST-1 TRIAL**

- *Decision Follows Pre-NDA Meeting with the FDA to Request Accelerated Approval for Patients with Myelofibrosis and Low Platelet Counts -*
- *Accelerated Approval to Be Requested Based on Single Trial, Potentially Reducing Time to Market -*

**SEATTLE, Wash., September 23, 2015**—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) today announced its plan to submit a new drug application (NDA) to the U.S. Food and Drug Administration (FDA) following a productive pre-NDA meeting for pacritinib, an investigational oral kinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R. The company expects to submit the NDA in the fourth quarter of 2015 and to request accelerated approval for the treatment of patients with intermediate and high-risk myelofibrosis with low platelet counts of less than 50,000 per microliter (<50,000/ $\mu$ L). The NDA will be based primarily on data from the PERSIST-1 Phase 3 trial – as well as data from Phase 1 and 2 studies of pacritinib – and additional information requested by the FDA, including a separate study report and datasets for the specific patient population with low platelet counts of less than 50,000 per microliter (<50,000/ $\mu$ L) for whom there are no approved drugs. Submission of an NDA after a single Phase 3 trial under accelerated approval, instead of waiting to complete two Phase 3 trials, could potentially reduce time to market by up to 14 months.

Myelofibrosis is a rare and serious chronic blood cancer that can affect patients of all ages with a median age of 65 years, with an estimated prevalence in the United States of approximately 18,000 patients.

*“This improved timing on the plan to submit an NDA – which is supported by data after completion of the PERSIST-1 trial – will allow us the potential for making pacritinib available to patients with low platelet counts earlier than expected,” said James A. Bianco, M.D., President and CEO of CTI BioPharma. “We look forward to working with the FDA on the submission and review of this application.”*

### **PERSIST Update**

Pacritinib is currently being evaluated in two Phase 3 clinical trials, known as the PERSIST program, for patients with myelofibrosis. PERSIST-1 is a randomized, open-label, multicenter Phase 3 trial comparing the efficacy and safety of pacritinib with that of best available therapy (exclusive of a JAK inhibitor) in 327 enrolled patients with myelofibrosis (primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis), regardless of the patients’ platelet counts. PERSIST-2 is a randomized, open-label, multicenter Phase 3 clinical trial evaluating pacritinib compared to best available therapy (BAT), including the approved JAK1/JAK2 inhibitor dosed according to product label for patients with myelofibrosis whose platelet counts are less than or equal to 100,000 per microliter. In October 2013, CTI BioPharma reached agreement with the FDA on a Special Protocol Assessment (SPA) for the PERSIST-2 trial. The SPA is a written agreement between CTI BioPharma and the FDA regarding the design, endpoints and planned statistical analysis approach of the trial to be used in support of a potential NDA submission. The design of PERSIST-1 and PERSIST-2 allows for patients on the BAT arm to crossover and receive treatment with pacritinib if their disease progresses or after they achieve the 24-week measurement endpoint. Although crossover design of clinical trials may confound evaluation of survival, such designs are frequently used in cancer studies, and the FDA has approved multiple oncology drugs that utilized crossover design in Phase 3 trials. The Independent Data Monitoring Committee (IDMC) for the PERSIST program recommended patients on the best available therapy arm should not crossover to receive pacritinib due to non-statistically significant safety concerns in patients who crossover after 24 weeks, which

crossover confounds evaluation of survival. After receiving input from external independent experts and providing the FDA the PERSIST-1 data, IDMC's recommendation and correspondence, CTI BioPharma and Baxalta notified the FDA of the decision to proceed per protocol. Following a written response in lieu of a Type C meeting with the FDA, CTI BioPharma and Baxalta determined that no modifications to the ongoing trials were required. Enrollment in PERSIST-2, which is designed to enroll up to 300 patients in North America, Europe, Australia, New Zealand and Russia is continuing. Based on current timelines, PERSIST-2 enrollment is expected to be completed in the first quarter of 2016. Additional details on PERSIST-2 are available at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) or [www.PERSISTprogram.com](http://www.PERSISTprogram.com).

### **About Pacritinib**

Pacritinib is an investigational oral kinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R. In August 2014, pacritinib was granted Fast Track designation by the FDA for the treatment of intermediate and high risk myelofibrosis, including but not limited to patients with disease-related thrombocytopenia, patients experiencing treatment-emergent thrombocytopenia on other JAK2 inhibitor therapy, or patients who are intolerant of, or whose symptoms are sub-optimally managed on other JAK2 inhibitor therapy. The FDA's Fast Track process is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Pacritinib does not have regulatory approval and is not commercially available.

CTI BioPharma and Baxalta (NYSE:BXLT) are parties to a worldwide license agreement to develop and commercialize pacritinib. CTI BioPharma and Baxalta will jointly commercialize pacritinib in the U.S. while Baxalta has exclusive commercialization rights for all indications outside the U.S.

### **About CTI BioPharma**

CTI BioPharma Corp. (NASDAQ and MTA: CTIC) is a biopharmaceutical company focused on the acquisition, development, and commercialization of novel targeted therapies covering a spectrum of blood-related cancers that offer a unique benefit to patients and healthcare providers. CTI BioPharma has a commercial presence in Europe and a late-stage development pipeline, including pacritinib, CTI BioPharma's lead product candidate, which is currently being studied in a Phase 3 program for the treatment of patients with myelofibrosis. CTI BioPharma is headquartered in Seattle, Washington, with offices in London and Milan under the name CTI Life Sciences Limited. For additional information and to sign up for email alerts and get RSS feeds, please visit [www.ctibiopharma.com](http://www.ctibiopharma.com).

### **Forward Looking Statements**

This press release includes forward-looking statements related to pacritinib and related clinical trials conducted pursuant to a collaboration between CTI BioPharma Corp. and Baxalta Inc., which are within the meaning of the Safe Harbor provisions of the Private Securities Litigation Reform Act of 1995. Such statements are subject to a number of risks and uncertainties, the outcome of which could materially and/or adversely affect actual future results and the trading price of the issuers' securities. Such statements include, but are not limited to, statements regarding expectations with respect to the potential therapeutic utility of pacritinib and the prevalence of myelofibrosis in the United States, plans and intentions with respect to the submission of an NDA requesting accelerated approval in the fourth quarter of 2015 or any other regulatory filings in Europe and other locations outside the US, the ability of the PERSIST-1 and Phase 1 and Phase 2 trials and additional information about pacritinib requested by the FDA to support a potential regulatory submission on an accelerated basis or otherwise, CTI BioPharma's ability to provide the FDA with the additional data and information requested, potential for making pacritinib available to patients with significant thrombocytopenia earlier than expected and to shorten the time to market for pacritinib by 14 months, the ability to complete enrollment for PERSIST-2 by in the first quarter 2016 and the ability of pacritinib to meet unmet medical needs and future regulatory, development and commercialization plans. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release and are based on assumptions about many important factors and information currently available to us to the extent we have thus far had an opportunity to evaluate such information in light of all surrounding facts, circumstances, recommendations and analyses. A number of results

and uncertainties could cause actual results to differ materially from those in the forward-looking statements: CTI BioPharma's NDA requesting accelerated approval for pacritinib may not be accepted by the FDA; additional pre-approval trials or post-approval Risk Evaluation and Mitigation Strategy (REMS) or Post-Marketing Requirements (PMR) may be required; satisfaction of regulatory and other requirements; clinical trial results; changes in laws and regulations; product quality, product efficacy, trial design and study protocol, data integrity, dataset size, patient safety issues, the smaller population size for the specific patient population; product development risks; and other risks identified in each issuer's most recent filings on Form 10-K and other Securities and Exchange Commission filings. Except as required by law, CTI BioPharma undertakes to update its forward-looking statements.

Source: CTI BioPharma Corp.

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