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Testo del comunicato

Vedi allegato.

PRECLINICAL DATA PRESENTED AT AACR INDICATE PACRITINIB'S POTENTIAL TO ERADICATE THERAPY-RESISTANT LEUKEMIA STEM CELLS RESIDING IN BONE MARROW MICROENVIRONMENT

Additional pacritinib data also presented at AACR

SEATTLE, April 20, 2016—CTI BioPharma Corp. (CTI) (NASDAQ and MTA:CTIC) today announced findings from an investigator-sponsored preclinical study indicating that pacritinib, an inhibitor of JAK2, FLT3, IRAK1 and CSF1R, may be effective in reducing survival of myelofibrosis and acute myeloid leukemia (AML) repopulating cells. Further, this study also demonstrated that the combination of pacritinib at low nanomolar concentrations with dasatinib may eliminate self-renewing leukemia stem cells in blast crisis of chronic myeloid leukemia *(*CML) with minimal toxicity toward normal progenitors. In myeloid leukemias, these leukemic stem cells can evade initial treatment and hide within the bone marrow microenvironment, develop resistance to current therapies, self-renew and eventually cause relapse.

These findings were presented by Larissa Balaian, Ph.D. from the Moores Cancer Center, University of California San Diego in a poster presentation (abstract #3338) titled: "Pacritinib reduces human myeloid leukemia stem cell maintenance in a defined niche," during the American Association of Cancer Research (AACR) Annual Meeting held April 16-20 in New Orleans, LA.

"The potential ability for pacritinib to eradicate therapy resistant leukemia stem cells in relapse AML as a single-agent, as well as eliminate self-renewing stem cells in CML, when used in combination with standard of care therapy, demonstrates that targeting niche-dependent signaling with pacritinib could represent a new approach to treating patients with refractory acute myeloid leukemia and blast crisis of CML," said Dr. Balaian.

Additional data being presented at the meeting include:

A poster (abstract #2602) titled: "The nonclinical toxicology profile of pacritinib, a JAK2/FLT3 inhibitor with no dose-limiting clinical myelosuppression." In this poster, CTI BioPharma researchers presented data from studies of pacrinitib in nonclinical models that were evaluated in comparison to publicly available information for the currently approved JAK inhibitors. The nonclinical toxicology profile findings showed that pacritinib is unique for its mild myelosuppressive effects in the nonclinical studies. Of interest, only pacritinib was not associated with increased opportunistic infections in the long-term toxicology studies.

A poster (abstract #1609) titled: "Investigation of absorption, metabolism, excretion, and mass balance of [14C]-pacritinib in healthy subjects: a phase 1 study." In this poster, CTI BioPharma researchers investigated clearance pathways, excretion, pharmacokinetics and recovery of pacritinib's major metabolites in healthy volunteers. Intact pacritinib was minimally excreted in urine and feces while most radioactivity was recovered as metabolites in feces, suggesting extensive biliary clearance and hepatic metabolism of pacritinib. No dose adjustments are anticipated to be required for patients with renal impairment.

The foregoing summaries of such reported findings and posters are not complete and are qualified in their entirety by reference to the referenced posters. These and other poster presentations are available in the publication section of the CTI BioPharma website at ctibiopharma.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 (low platelet counts); patients experiencing treatment-emergent thrombocytopenia on other JAK2 inhibitor therapy; or patients who are intolerant of, or whose symptoms are not well controlled (sub-optimally managed) on other JAK2 therapy. Clinical studies for pacritinib are currently subject to a full clinical hold issued by the U.S. Food and Drug Administration in February 2016. The Company is in the process of responding to the full clinical hold by working through the FDA's recommendations prior to requesting a meeting with them. In March 2016, the FDA expressed interest in allowing patients who were receiving benefit from pacritinib treatment at the time the clinical hold was imposed to submit requests to the FDA to resume pacritinib treatment under a Single Patient IND (SPI) program on a case-by-case basis. The Company is working with investigators in submitting SPI requests to the FDA. Separately, the FDA has informed clinical investigators that emergency requests may be submitted to the FDA for individual patient Expanded Access to pacritinib. Expanded Access, sometimes called "compassionate use," is the use outside of a clinical trial of an investigational medical product. Pacritinib does not have regulatory approval and is not commercially available.

CTI BioPharma and Baxalta Incorporated 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 Corp.

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 with respect to PIXUVRI® and a late-stage development pipeline, including pacritinib 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.

Forward Looking Statements

This press release includes forward-looking statements, 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, expectations with respect to the potential therapeutic utility of pacritinib and patients' ability to obtain pacritinib treatment under the SPI program or the Expanded Access program. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release. In particular, this release addresses select clinical trial data and results, and should be evaluated together with information regarding primary and secondary endpoints, safety and additional data once such data has been more fully analyzed and is made publicly available. The statements 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 fully and carefully 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, including: with respect to the SPI program and the Expanded Access program, the possibility that the FDA may not ultimately permit dosing with pacritinib under the programs, may impose conditions that make it difficult to implement on reasonable terms or at all, and could modify, suspend or terminate the programs at any time; satisfaction of

regulatory and other requirements; that trial, study and model results observed to date may differ from future results or that difference conclusions or considerations may qualify such results once existing data has been more fully evaluated, actions of regulatory bodies and other governmental authorities; other clinical trial results; changes in laws and regulations; product quality, product efficacy, study protocol, data integrity or patient safety issues; product development risks; and other risks identified in each issuer's most recent filings on Forms 10-K and 10-Q and other Securities and Exchange Commission filings. CTI BioPharma does not undertake to update such forward-looking statements.

Source: CTI BioPharma Corp.

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