Informazione Regolamentata n. 0696-73-2015	Data/Ora Ricezione 15 Giugno 2015 07:42:55	MTA

Societa'	:	CTI BIOPHARMA
Identificativo Informazione Regolamentata	:	59717
Nome utilizzatore	:	CELLN01 - Villa
Tipologia	:	IRAG 10
Data/Ora Ricezione	:	15 Giugno 2015 07:42:55
Data/Ora Inizio Diffusione presunta	:	15 Giugno 2015 07:57:55
Oggetto	:	CTI BIOPHARMA:TOSEDOSTAT ACHIEVES PRIMARY ENDPOINT IN PHASE 2 STUDY IN ELDERLY PATIENTS WITH AML

Testo del comunicato

Vedi allegato.

TOSEDOSTAT IN COMBINATION WITH LOW DOSE CYTARABINE ACHIEVES PRIMARY ENDPOINT IN PHASE 2 STUDY IN ELDERLY PATIENTS WITH AML

Responders Achieve 45 Percent Complete Remission Rate, Nearly Double the Upper Limit of Expected Results; 55 Percent of Responding Patients Remain in Remission for a Median of 319 Days

Data Presented at the 20th Congress of the European Hematology Association, June 11-14, 2015

VIENNA, Austria, **June 15, 2015**—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) today announced findings from an investigator-sponsored Phase 2 trial in patients with either primary (de novo) acute myeloid leukemia (AML) or AML that has evolved from myelodysplastic syndrome (MDS). Results showed the combination of tosedostat with low dose cytarabine/Ara-C (LDAC) resulted in an overall response rate (ORR) of 54 percent in elderly patients with AML – with 45 percent of patients achieving durable complete responses (CR). These final results were presented by Dr. Giuseppe Visani, Director of Hematology and Stem Cell Transplant Center at AORMN, Pesaro, Italy in a poster session (abstract #P564) during the 20th Congress of the European Hematology Association (EHA), June 11-14, 2015 in Vienna, Austria.

AML is the most common acute leukemia affecting adults, and its incidence increases with age. AML may develop from the progression of other diseases, such as MDS, which is a blood cancer that also affects the bone marrow and leads to a decrease in circulating red blood cells. Tosedostat is a potential first-in-class selective inhibitor of aminopeptidases, which are required by tumor cells to provide amino acids necessary for growth and tumor cell survival.

“Both the types and length of responses in this trial with tosedostat are very encouraging – particularly given the limited options and poor outcomes historically observed in elderly patients with acute myeloid leukemia, either de novo or secondary after myelodysplastic syndrome,” said Dr. Visani. “Importantly, through this study we have also identified potential biomarkers that may help identify high-risk patients in which we are more likely to see these clinically meaningful results – the findings of which are quite compelling and warrant further study.”

Findings Presented at EHA

Final results presented at EHA show that responding patients had a significant improvement in overall survival based on response rates compared to non-responding patients (p=0.018). In the intent-to-treat population (ITT), the ORR was 54 percent – with CR observed in 45 percent of patients (n=15/33). In the responding patients, the median time for achieving best response was 74 days (range: 22-145 days) and 55 percent (n=10/18) were still in remission after a median follow-up of 319 days. Safety analysis show that tosedostat in combination with LDAC was generally well tolerated. The primary adverse events observed were pneumonitis (12 percent), cardiac (6 percent), brain hemorrhage (3 percent), and asthenia (3 percent).

One of the secondary endpoints was to identify possible biomarkers associated with sensitivity and/or drug resistance. A gene expression profile (GEP) was performed on purified AML cells obtained from 29 patients. Analysis of these patient cells identified a molecular signature associated with clinical response (CR vs. no CR). Based on the differentially expressed genes (n=212), samples were divided according to either CR or no CR. Results showed that these differentially expressed genes were associated with relevant biological functions and

pathways – including B-catenin (beta-catenin), TNFA-NFkB, ERB2, inflammatory response, and epithelial-mesenchimal transition – and showed that the achievement of a CR could be efficiently predicted by GEP.

"The results observed with tosedostat in acute myeloid leukemia add to a growing body of data showing the anti-tumor activity of this aminopeptidase inhibitor and the potential of using this approach to treat blood-related cancers," Alan K. Burnett, M.D., Global Lead for Myeloid Diseases at CTI BioPharma. "Based on the clinical data observed to date, we are advancing the development for tosedostat including the potential for a Phase 3 registrational study for primary acute myeloid leukemia or acute myeloid leukemia that evolves from myelodysplastic syndrome."

About the Study Design
The Phase 2 multicenter clinical trial was designed to assess tosedostat (orally once-daily) in combination with intermittent LDAC (twice daily) in 33 elderly patients (median age = 75 years) with either primary AML or secondary AML after MDS. Courses of LDAC were repeated every four weeks for up to eight cycles in the absence of disease progression or unacceptable toxicity. The primary objective was to exceed the upper limit of institutional expected CR rates (P_0=10%, P_1= 25%, α=0.05, 1-β=80%); secondary objectives include safety and toxicity, stable disease, overall survival, and progression-free survival as well as the identification of a possible biomarker associated with sensitivity and/or disease resistance through global gene expression profiling (GEP, Affymetrix Transciptome Array 2.0).

The poster for Abstract #P564 – "Tosedostat plus low dose cytarabine induces a high rate of responses that can be predicted by genetic profiling in AML: Final results of a Phase II multicenter study" – is available at www.ctibiopharma.com.

About Tosedostat
Tosedostat is an oral aminopeptidase inhibitor that has demonstrated anti-tumor responses in blood-related cancers and solid tumors in Phase 1-2 clinical trials. Tosedostat is currently being evaluated in multiple Phase 2 clinical trials for the treatment of patients with AML or high-risk MDS and are intended to inform the design of a Phase 3 registration study to support potential regulatory approval. Tosedostat is not approved or commercially available.

About Acute Myeloid Leukemia and Myelodysplastic Syndrome
AML is a cancer characterized by the rapid growth of abnormal white blood cells that accumulate in the bone marrow and interfere with the production of normal blood cells. AML is the most common acute leukemia affecting adults, and its incidence increases with age.[1] The symptoms of AML are caused by the replacement of normal bone marrow with leukemic cells, which causes a drop in red blood cells, platelets, and normal white blood cells, leading to infections and bleeding. AML progresses rapidly and is typically fatal within weeks or months if left untreated. In 2015, approximately 20,830 new cases of AML are expected to be diagnosed in the United States and an estimated 10,460 are expected to die from the disease.[2] While AML may occur at any age, adults at least 60 years of age are more likely than younger people to develop the disease.[2] Although a substantial proportion of younger individuals who develop AML can be cured, AML in the elderly typically responds poorly to standard therapy with few complete remissions.

AML may develop from the progression of other diseases, such as MDS. MDS are a group of diverse bone marrow disorders in which the bone marrow does not produce enough healthy blood cells. MDS is often referred to as a "bone marrow failure disorder."

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 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.

Forward Looking Statements

This press release includes forward-looking statements 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 CTI BioPharma's securities. Such statements include, but are not limited to, statements regarding CTI BioPharma's expectations with respect to the development of CTI BioPharma and its product and product candidate portfolio, the potential opportunity for tosedostat to meet a current unmet medical need in the treatment of patients with AML, potentially starting a Phase 3 registration-directed study to evaluate tosedostat and regulatory submission for tosedostat, CTI BioPharma's ability to achieve its articulated 2015 business and financial plan, goals, objectives and projections, CTI BioPharma's projected revenues and non-GAAP operating loss and the expectations and assumption on which they are based. In particular, this release addresses the final results regarding the above discussed study, and should be evaluated together with 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. Risks that contribute to the uncertain nature of the forward-looking statements include, among others, risks associated with the biopharmaceutical industry in general and with CTI BioPharma and its product and product candidate portfolio in particular including, among others, risks associated with the following: that CTI BioPharma cannot predict or guarantee the pace or geography of enrollment of its clinical trials, that CTI BioPharma cannot predict or guarantee the outcome of preclinical and clinical studies, that final results observed to date may differ from future results or that different conclusions or considerations may qualify such results once existing data has been more fully evaluated, clinical trial results, that CTI BioPharma may not obtain favorable determinations by other regulatory, patent and administrative governmental authorities, that CTI BioPharma may experience delays in the commencement of preclinical and clinical studies, risks related to the costs of developing tosedostat and CTI BioPharma's other product candidates, and other risks, including, without limitation, competitive factors, technological developments, that CTI BioPharma may not be able to sustain its current cost controls or further reduce its operating expenses, that CTI BioPharma may not achieve previously announced goals, contractual milestones and objectives as or when projected, that CTI BioPharma's average net operating burn rate may increase, that CTI BioPharma will continue to need to raise capital to fund its operating expenses, but may not be able to raise sufficient amounts to fund its continued operation as well as other risks listed or described from time to time in CTI BioPharma's most recent filings with the SEC on Forms 10-K, 10-Q and 8-K. Except as required by law, CTI BioPharma does not intend to update any of the statements in this press release upon further developments.

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[1] The Leukemia and Lymphoma Society, Acute Myeloid Leukemia, Rev. 2011. http://tinyurl.com/d72ycja. Accessed June 2015

[2] American Cancer Society, Cancer Facts & Figures 2015. Available at http://www.cancer.org/research/cancerfactsstatistics/cancerfactsfigures2015/. Accessed June 2015

Source: CTI BioPharma Corp.

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