Informazione Regolamentata n. 0696-163-2015	Data/Ora Ricezione 06 Dicembre 2015 18:43:24	MTA

Societa'	:	CTI BIOPHARMA
Identificativo Informazione Regolamentata	:	66680
Nome utilizzatore	:	CELLN02 - Bell
Tipologia	:	IRAG 10
Data/Ora Ricezione	:	06 Dicembre 2015 18:43:24
Data/Ora Inizio Diffusione presunta	:	06 Dicembre 2015 22:45:06
Oggetto	:	CTI BioPharma Corp.: ANALYSIS OF PHASE 2 TOSEDOSTAT IN COMBINATION WITH LOW DOSE CYTARABINE SHOWS HIGH RATES OF RESPONSE IN ELDERLY PATIENTS WITH AML

Testo del comunicato

Vedi allegato.

ANALYSIS OF PHASE 2 TOSEDOSTAT IN COMBINATION WITH LOW DOSE CYTARABINE SHOWS HIGH RATES OF RESPONSE IN ELDERLY PATIENTS WITH AML

Data presented at 57th American Society of Hematology (ASH 2015) Annual Meeting & Exposition

ORLANDO, **December 6, 2015**—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) today announced an update on results from an investigator-sponsored Phase 2 trial of tosedostat – the company's investigational oral selective aminopeptidase inhibitor – in elderly patients with either primary (de novo) acute myeloid leukemia (AML) or secondary AML. The data showed a complete response (CR) of 48.5 percent with tosedostat in combination with low dose cytarabine/Ara-C (LDAC) – 33 percent of these patients were still responding after a median of 506 days. Results were presented in an oral presentation by Dr. Giuseppe Visani, Director of Hematology and Stem Cell Transplant Center at AORMN, Pesaro, Italy at ASH 2015 (Abstract#329).

AML is the most common acute leukemia affecting adults, and its incidence increases with age while its treatment becomes increasingly unsatisfactory – particularly in patients over age 70 and in those with co-morbid conditions that increase the risk of intensive chemotherapy. New approaches that allow meaningful leukemic control with acceptable side effects are needed. In this study, treatment with tosedostat, added to conventional therapy with low dose cytarabine demonstrated a higher response rate and longer survival than would be expected by cytarabine alone. Of great interest, preliminary data using a gene array panel suggest that it might be possible to predict on the basis of a blood test, which patients are mostly likely to benefit from tosedostat.

"The additional findings from this Phase 2 study continue to show the potential for tosedostat in combination with LDAC to produce clinically meaningful results for a patient population that is in real need of additional treatment options," said Dr. Visani. "We look forward to performing a validation analysis to confirm the ability of this preliminary gene expression profiling for identifying potential responders to tosedostat, as we believe we have identified biomarkers where the achievement of these impression responses could be efficiently predicted."

"We are particularly excited about the preliminary results of the gene array panel in this study because being able to predict a patient's likelihood of responding to tosedostat would help physicians personalize a patient's treatment plan, potentially reducing unnecessary treatments and side effects with the goal of improving the overall patient experience," said James A. Bianco, M.D., President & CEO of CTI BioPharma. "These data support our commitment to moving the tosedostat development program forward in AML."

Additional Study Details

As previously reported, the study met the primary endpoint with an overall response rate (ORR) of 54.6 percent (n=18/33) in the intent-to-treat population (ITT). At ASH, the new findings presented showed – in patients receiving tosedostat in combination with LDAC – a CR rate of 48.5 percent (n=16/33) and that the median time for achieving best response was 74 days (range: 22-145 days) with 33 percent still in remission (or experiencing a CR) after a median follow-up of 506 days.

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 a subset of 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-mesenchymal transition pathways – and suggested that the achievement of a CR could be efficiently predicted by GEP. A validation analysis is currently being conducted on additional patients in order to confirm the ability of GEP to identify potential responders to tosedostat.

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).

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. 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).

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, which 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
AML is the most common acute leukemia affecting adults, and its incidence increases with age. In older patients, AML may occur de novo or secondary to prior anti-cancer therapy, or from progression of other diseases, such as myelodysplasia. 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.

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 with respect to PIXUVRI® 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 therapeutic potential of tosedostat, including to meet a current unmet medical need in the treatment of patients with AML (and in particular, with respect to older patients and patients who relapse following standard therapies), potentially starting a Phase 3 registrational trial in 2016, prevalence of AML and mortality rates associated with AML. Investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this release. A number of results and uncertainties could cause actual results to differ materially from those in the forward-looking statements: including, among others: satisfaction of regulatory and other requirements; pre-clinical and 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 the CTI BioPharma's most recent filings on Form 10-K and other Securities and Exchange Commission filings. CTI BioPharma does not undertake to update its forward-looking statements.

Source: CTI BioPharma Corp.

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