Informazione Regolamentata n. 0696-162-2015	Data/Ora Ricezione 05 Dicembre 2015 15:18:30	MTA

Societa'	:	CTI BIOPHARMA
Identificativo Informazione Regolamentata	:	66679
Nome utilizzatore	:	CELLN02 - Bell
Tipologia	:	IRAG 10
Data/Ora Ricezione	:	05 Dicembre 2015 15:18:30
Data/Ora Inizio Diffusione presunta	:	05 Dicembre 2015 15:45:09
Oggetto	:	CTI BioPharma Corp.: ANALYSIS OF PIVOTAL PHASE 3 PATIENT OUTCOMES BY SUBGROUPS SHOWS TREATMENT WITH PACRITINIB RESULTED IN CONSISTENT RATES OF REDUCTI

Testo del comunicato

Vedi allegato.

ANALYSIS OF PIVOTAL PHASE 3 PATIENT OUTCOMES BY SUBGROUPS SHOWS TREATMENT WITH PACRITINIB RESULTED IN CONSISTENT RATES OF REDUCTION IN SPLEEN VOLUME AND SYMPTOM BURDEN

Results support the effectiveness of pacritinib across intermediate or high-risk myelofibrosis patient subgroups analyzed

ORLANDO, December 5, 2015 – CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA:CTIC) and Baxalta Incorporated (Baxalta) (NYSE:BXLT) today announced results from a new analysis of the pivotal Phase 3 trial, PERSIST-1, evaluating pacritinib versus best available therapy, excluding treatment with JAK2 inhibitors (BAT), in patients with myelofibrosis. Data examining patient outcomes across baseline demographic factors that are associated with prognosis – including age, baseline hemoglobin, baseline platelet count, ECOG status, JAK2 mutation status and red blood cell transfusion dependency – showed that treatment with pacritinib resulted in consistent rates of spleen volume reduction and control of disease-related symptoms across all intermediate or high-risk myelofibrosis subgroups. These findings were presented by Alessandro M. Vannucchi, M.D., associate professor of Hematology, University of Florence, Italy, during an oral presentation at the 57th American Society of Hematology (ASH 2015) Annual Meeting & Exposition in Orlando (Abstract #58).

Pacritinib is an investigational oral multikinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R, which are kinases found to be involved in the growth and spread of myelofibrosis and other blood-related cancers such as acute myeloid leukemia (AML).

"Reducing the burden of myelofibrosis-related symptoms is an important goal of treatment. However, for patients diagnosed with this rare blood cancer, there are limited therapeutic options – a gap that is even more significant for patients with low platelet counts," said Prof. Vannucchi. "These data presented at ASH 2015 are important and clinically meaningful as they demonstrate pacritinib's potential to achieve treatment goals across intermediate or high-risk patients with myelofibrosis, regardless of baseline characteristics including starting platelet count."

Myelofibrosis is a rare blood cancer associated with significantly reduced quality of life and shortened survival. Most patients with the disease present with enlarged spleens (splenomegaly), as well as many other potentially devastating physical symptoms such as abdominal discomfort, bone pain, feeling full after eating little, severe itching, night sweats and extreme fatigue.

"The results from this analysis add to the growing body of data for pacritinib suggesting it is a unique JAK inhibitor with a differentiated efficacy and safety profile that is not limited by the baseline characteristics of patients with myelofibrosis," said James Bianco, M.D., President and Chief Executive Officer, CTI BioPharma. "We believe pacritinib has the potential to fill a gap that exists for many patients whose lives are profoundly impacted by myelofibrosis, particularly those patients with low platelet counts."

"We are developing pacritinib with particular focus on targeting the underlying biology of myelofibrosis to improve the treatment landscape for patients with this underserved, progressive disease, including those in intermediate and high-risk subgroups," said David Meek, Executive Vice President and President, Oncology at Baxalta. "We look forward to working with worldwide regulatory authorities to advance treatment options for all patients with myelofibrosis as we begin our registration submissions for pacritinib in the coming months."

About the Subgroup Analysis
Findings presented at ASH 2015 were based on the analysis of baseline patients' characteristics from PERSIST-1, a randomized Phase 3 registration-directed trial comparing the efficacy and safety of pacritinib to BAT that included a broad range of currently utilized treatments. As previously reported, the trial met its primary endpoint of spleen volume reduction (35 percent or greater from baseline to Week 24 by MRI/CT scan) in the intent-to-treat population.

The subgroup analysis discussed above assessed results observed in patients achieving 35 percent or greater spleen volume reduction from baseline or a decrease of 50 percent or more in Total Symptom Score (TSS) by baseline characteristics or risk factors, including initial platelet count, JAK2V617F mutation status, red blood cell transfusions and bone pain. Findings showed that results (from the primary analysis) were consistent across all subgroups evaluated. Achievement of 35 percent or greater spleen volume reduction was independent of most risk factors assessed and a 50 percent or more decrease in TSS was independent of characteristics evaluated, except bone pain score greater than three at baseline.

The most common adverse events in the pacritinib arm vs. BAT that showed more than 5 percent difference were diarrhea (57 percent vs. 12 percent), nausea (29 percent vs. 6 percent) and vomiting (19 percent vs. 5 percent). No Grade 4 gastrointestinal events were reported.

Additional Pacritinib Data Presented at ASH
Also presented today were patient-reported outcome data that examined the relationship between myelofibrosis-associated symptoms (based on the TSS) and changes in splenomegaly and health-related quality of life (HRQoL) outcomes in the PERSIST-1 overall patient population and in patients with baseline thrombocytopenia. The analysis showed TSS response was associated with improvements in spleen volume response and perceived Overall Health State; this trend was also observed in patients with low baseline platelet counts (<50,000/μL and <100,000/ μL). In all patient populations analyzed, TSS response was significantly associated with improvements in fatigue, a major contributor to poor HRQoL in patients with myelofibrosis. Significant improvements in social functioning, appetite loss and insomnia were also observed in patients with baseline thrombocytopenia. These data were presented in a poster presentation by Ruben Mesa, M.D., Chair, Hematology and Medical Oncology Division, Mayo Clinic, Scottsdale, AZ (Abstract #1609).

About PERSIST-1
PERSIST-1 is a randomized (2:1), controlled Phase 3 registration-directed trial comparing the efficacy and safety of pacritinib to BAT – which included a broad range of currently utilized treatments – in 327 patients with myelofibrosis (primary myelofibrosis, post-polycythemia vera myelofibrosis or post-essential thrombocythemia myelofibrosis), regardless of the patients' platelet counts. At study entry, 46 percent of patients were thrombocytopenic; 32 percent of patients had platelet counts less than 100,000 per microliter (<100,000/μL); and 16 percent of patients had platelet counts less than 50,000 per microliter (<50,000/μL); normal platelet counts range from 150,000 to 450,000 per microliter. The median duration of treatment was 16.2 months in patients treated with pacritinib, compared to 5.9 months in patients treated with BAT. The majority (79 percent) of patients on the BAT arm eventually crossed over to receive pacritinib therapy.

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 U.S. Food and Drug Administration (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.

CTI BioPharma and Baxalta 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.

The companies recently announced the initiation of a rolling new drug application (NDA) to the FDA for pacritinib. The companies are seeking accelerated approval and priority review of pacritinib for the treatment of patients with intermediate and high-risk myelofibrosis with low platelet counts of less than 50,000 per microliter (<50,000/μL).

About Myelofibrosis and Myeloproliferative Neoplasms
Myelofibrosis is one of three main types of myeloproliferative neoplasms (MPN), which are a closely related group of hematological blood cancers. The three main types of MPNs are myelofibrosis, polycethemia vera and essential thrombocythemia.[1] Myelofibrosis is a serious and life-threatening chronic bone marrow disorder caused by the accumulation of malignant bone marrow cells that triggers an inflammatory response and scars the bone marrow. The replacement of bone marrow with scar tissue limits its ability to produce red blood cells, prompting the spleen and liver to take over this function. Symptoms that arise from this disease include enlargement of the spleen, anemia, extreme fatigue and pain. As the disease progresses, the body slows production of important blood cells, and within one year of diagnosis, the incidence of disease-related thrombocytopenia (very low blood platelet counts), anemia and red blood cell transfusion requirements increase significantly.

The estimated prevalence of MPNs suggest there are approximately 300,000 people living with the disease in the U.S., of which myelofibrosis accounts for approximately 18,000 patients.[2] In Europe, there is a wide variation of prevalence observed across data sources. Myelofibrosis has a median age of 64 at the time of diagnosis[2] and is a progressive disease with approximately 20 percent of patients eventually developing AML.[3] The median survival for high-risk myelofibrosis patients is less than one and a half years, while the median survival for myelofibrosis patients overall is approximately six years.[4]

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, 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.

About Baxalta
Baxalta Incorporated (NYSE: BXLT) is a $6 billion global biopharmaceutical leader developing, manufacturing and commercializing therapies for orphan diseases and underserved conditions in hematology, oncology and immunology. Driven by passion to make a meaningful impact on patients' lives, Baxalta's broad and diverse pipeline includes biologics with novel mechanisms and advanced technology platforms such as gene therapy. The Baxalta Global Innovation and R&D Center is located in Cambridge, Massachusetts. Launched in 2015 following separation from Baxter International, Baxalta's heritage in biopharmaceuticals spans decades. Baxalta's therapies are available in more than 100 countries and it has advanced biological manufacturing operations across 12 facilities, including state-of-the-art recombinant production and plasma fractionation. Headquartered in Northern Illinois, Baxalta employs 16,000 employees worldwide.

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, including pacritinib's potential to achieve treatment goals across patients with myelofibrosis, regardless of baseline characteristics, such as starting platelet count and in particular, its potential to reduce spleen volume and symptom burden and improve HRQoL, expectations to submit regulatory submissions in the coming months, the estimated prevalence of MPNs, myelofibrosis and myelofibrosis patients that develop AML and the survival rates for patients with myelofibrosis. 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: satisfaction of regulatory and other requirements; that trial 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. Neither CTI BioPharma nor Baxalta undertakes to update its forward-looking statements.

Source: CTI BioPharma and Baxalta Incorporated

1. MPN Research Foundation website, www.mpnresearchfoundation.org.
2. Based on Mesa R, ASH 2012 poster.
3. Cervantes F, et al., New prognostic scoring system for primary myelofibrosis based on a study of the International Working Group for Myelofibrosis Research and Treatment. *Blood*. 2009; 113:2895-2901.
4. Vannucchi, A. Management of Myelofibrosis. ASH Education Book. 2011; 1:222-230.

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