Informazione Regolamentata n. 0696-147-2015	Data/Ora Ricezione 23 Novembre 2015 07:21:25	MTA

Societa'	:	CTI BIOPHARMA
Identificativo Informazione Regolamentata	:	66124
Nome utilizzatore	:	CELLN02 - Bell
Tipologia	:	IROS 15
Data/Ora Ricezione	:	23 Novembre 2015 07:21:25
Data/Ora Inizio Diffusione presunta	:	23 Novembre 2015 07:45:08
Oggetto	:	CTI BioPharma Initiates Rolling Submission of U.S. New Drug Application for Pacritinib for the Treatment of Patients with Myelofibrosis

Testo del comunicato

Vedi allegato.

CTI BioPharma Initiates Rolling Submission of U.S. New Drug Application for Pacritinib for the Treatment of Patients with Myelofibrosis

-- Completion of NDA Submission Expected Before the End of 2015 –

SEATTLE, Wash., November 23, 2015—CTI BioPharma Corp. (CTI BioPharma) (NASDAQ and MTA: CTIC) announced the initiation of its rolling new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for pacritinib, an investigational oral kinase inhibitor with specificity for JAK2, FLT3, IRAK1 and CSF1R. As part of the application, CTI BioPharma and its partner, Baxalta Incorporated (Baxalta), are seeking accelerated approval and priority review for 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). If approved for the requested indication, pacritinib would be the first JAK2 inhibitor approved for the treatment of patients with myelofibrosis with platelet counts of less than 50,000/µL – a specific patient population for which there are currently no approved drugs. The rolling NDA allows completed portions of an NDA to be submitted and reviewed by the FDA on an ongoing basis. CTI BioPharma and Baxalta plan to complete the submission before the end of 2015.

Myelofibrosis (a type of myeloproliferative neoplasm) is a rare, but 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. Myelofibrosis is associated with significantly reduced quality of life and shortened survival, can affect patients of all ages (with a median affected age being 65 years) and an estimated prevalence in the United States of approximately 18,000 patients.

“We believe the initiation of the rolling NDA submission represents a major step forward toward potentially offering pacritinib as a next generation JAK2/FLT3 inhibitor to patients with this rare and chronic type of blood cancer,” said James A. Bianco, M.D., President and CEO of CTI BioPharma. “We are excited to have achieved this milestone and look forward to working with the FDA during the review process, with the goal of bringing this important treatment to market – which we hope will fill an unmet need for many patients whose lives are profoundly impacted by myelofibrosis.”

The submission includes data from the PERSIST-1 Phase 3 trial – as well as data from Phase 1 and 2 studies of pacritinib. 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. 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 another JAK2 therapy; or patients who are intolerant to or whose symptoms are not well controlled (or sub-optimally managed) on another JAK2 therapy.

About Myelofibrosis and Myeloproliferative Neoplasms

Myelofibrosis is a 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.

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 acute myeloid leukemia.[3] The median survival for high-risk patients is less than one and a half years; median survival for myelofibrosis patients overall is approximately six years.[4]

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 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 related to pacritinib and related clinical trials conducted pursuant to a collaboration between CTI BioPharma and Baxalta, 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, the expected completion of the NDA submission for pacritinib before the end of 2015, the potential to reduce time to market for pacritinib by up to 14 months in the event of an FDA accelerated approval scenario, the prevalence of MPNs and myelofibrosis, the percentage of myelofibrosis patients that develop AML, the mortality rates associated with myelofibrosis, the ability of the PERSIST-1 and Phase 1 and Phase 2 trials and any additional information about pacritinib requested by the FDA to support a potential regulatory submission on an accelerated basis or otherwise 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: 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, dataset size or patient safety issues; the smaller population size for the specific patient population, product development risks; and other risks identified in the respective issuer's most recent filings on Form 10-K and other Securities and Exchange Commission filings. CTI BioPharma does not undertake to update such forward-looking statements.

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.

Source: CTI BioPharma Corp.

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