Incyte Announces Top-Line Results from RELIEF Trial of Ruxolitinib in Patients with Polycythemia Vera

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- *Statistical significance not achieved for the primary endpoint of symptom control; positive trends in favor of ruxolitinib versus hydroxyurea observed*
- *RELIEF trial conducted in patients who were generally well-controlled on hydroxyurea but reporting continued disease-related symptoms, a different population than the pivotal Phase III RESPONSE trial*
- *RELIEF not included in, nor required for, polycythemia vera (PV) sNDA submission; data expected to be presented at an upcoming scientific meeting*
- *sNDA submitted to FDA for PV in June 2014 based on the pivotal Phase III RESPONSE trial demonstrating superior clinical benefit of ruxolitinib over best available therapy in patients resistant to, or intolerant of, hydroxyurea*

WILMINGTON, Del.--(BUSINESS WIRE)--Jul. 23, 2014-- Incyte Corporation (Nasdaq: INCY) today announced top-line results from RELIEF, a randomized, double-blind clinical trial designed to compare symptom improvement in 110 patients with polycythemia vera (PV) treated with ruxolitinib versus patients treated with hydroxyurea (HU). While positive trends were observed in favor of ruxolitinib, the trial did not achieve statistical significance for the primary endpoint as measured by the proportion of patients with ≥ 50% reduction in a defined cluster of symptoms that included tiredness, itching, muscle aches, night sweats and sweats while awake at week 16 compared to baseline. Topline results showed a 43.4% symptom response rate in the ruxolitinib arm and a 29.6% symptom response rate in the hydroxyurea arm (p=0.139). Full data from the RELIEF trial are expected to be presented at an upcoming scientific meeting.

“RELIEF was designed to provide us with additional information regarding symptom improvement and is not required for FDA approval. The patients recruited into the RELIEF study had less advanced PV compared to those in RESPONSE, our larger pivotal Phase III study which formed the basis of the sNDA, and which met the primary endpoint of improved hematocrit control and reduced spleen size in patients with uncontrolled PV,” stated Richard Levy, M.D., Executive Vice President, Chief Drug Development & Medical Officer of Incyte.

Further analyses of RELIEF are underway to evaluate what factors may have contributed to a symptom control rate for patients on stable doses of HU that was five to six times higher than that seen in the best available therapy control arm of the RESPONSE trial, and which led to an underpowering of the RELIEF trial.

RESPONSE was an open-label randomized trial of 222 patients and is the basis of Incyte’s supplemental New Drug Application (sNDA) submitted in June 2014. RESPONSE was conducted under a Special Protocol Assessment (SPA) agreement. The FDA granted Fast Track designation for ruxolitinib in PV, specifically for the treatment of patients who are resistant to or intolerant of hydroxyurea. If approved, ruxolitinib would be the first JAK1/JAK2 inhibitor available for patients with PV.

Hervé Hoppenot, President and CEO of Incyte, commented, “One in four patients with PV remain uncontrolled despite best available therapies. Such patients may have severe disease-related symptoms and elevated blood counts which put them at greater risk of cardiovascular complications such as stroke, pulmonary embolism, deep vein thrombosis and heart attack. Based on the positive and statistically significant results from the pivotal Phase III RESPONSE trial, as presented at ASCO last month, we remain confident that ruxolitinib has the potential to become an important new treatment for PV patients who are no longer responding to, or are intolerant of, hydroxyurea.”

About RELIEF (Randomized Switch Study from Hydroxyurea to Ruxolitinib for RELIEF of Polycythemia Vera Symptoms)

RELIEF is a Phase III multicenter, double-blind, double-dummy, randomized (1:1) study involving 110 PV patients. Eligible patients were required to have a confirmed diagnosis of PV according to the revised World Health Organization criteria; be on a stable dose of HU monotherapy but reporting symptoms based on the modified MPN-SAF screening symptom form; have a cytokine-related total symptom score (TSS-C: tiredness, itching, muscle aches, night sweats and sweats while awake) of ≥ 8 out of a maximum score of 50; meet at least one of the following criteria with respect to phlebotomy and splenomegaly: have had no more than 2 phlebotomies within the 6 months before screening or no palpable splenomegaly; and have a hematocrit that could be controlled within 35% to 48% (inclusive) before randomization.

The primary endpoint of the study was the proportion of subjects with ≥ 50% reduction in TSS-C, measured using a patient questionnaire, at week 16 compared to baseline.

About RESPONSE (Randomized Study of Efficacy and Safety in POlycythemia Vera with Jak INhibitor INCB018424 VerSus BEst Available Care)

RESPONSE is an open-label randomized trial of 222 patients conducted in North America, Europe, Asia, and Australia. Patients with polycythemia vera (PV) who were resistant to or intolerant of hydroxyurea (HU) were randomized 1:1 to receive ruxolitinib 10 mg twice daily or best available therapy (BAT), which was defined as investigator selected monotherapy or observation only. From week 32, patients in the BAT group could cross over to receive ruxolitinib therapy.

The primary endpoint of the study is the proportion of patients who achieved both hematocrit control without the need for phlebotomy from week 8 through 32 and a spleen volume reduction of at least 35 percent from baseline at 32 weeks. Key secondary endpoints include durable primary response and complete hematologic remission. Complete hematologic remission was defined as maintaining hematocrit control without the need for

phlebotomy, a platelet count ≤400 x 10^9/L and white blood cell count ≤10 x 10^9/L. Other secondary endpoints include safety, symptom improvement, and quality of life.

About Polycythemia Vera

Polycythemia vera (PV) is a myeloproliferative neoplasm (MPN) characterized by an overproduction of normal red blood cells, white blood cells and platelets that leads to an increased risk of thrombosis.[1-4] Erythrocytosis (elevated red blood cell mass) is the most prominent clinical manifestation of PV, distinguishing it from other MPNs.[5] PV may occur at any age but often presents later in life, with a median age at diagnosis of 60 years.[6,7] Approximately 100,000 patients in the United States are living with PV[8] and approximately 25 percent of patients with PV develop resistance to or intolerance of hydroxyurea[9,10] and are considered uncontrolled.

About Jakafi® (ruxolitinib)

Jakafi is a prescription medicine approved by the U.S. Food and Drug Administration to treat people with intermediate or high-risk myelofibrosis (MF), including primary MF, post–polycythemia vera MF and post–essential thrombocythemia MF. Jakafi is marketed by Incyte in the United States and by Novartis as Jakavi® (ruxolitinib) outside the United States.

Important Safety Information

Jakafi can cause serious side effects including:

Low blood counts: Jakafi may cause your platelet, red blood cell, or white blood cell counts to be lowered. If you develop bleeding, stop taking Jakafi and call your healthcare provider. Your healthcare provider will perform blood tests to check your blood counts before you start Jakafi and regularly during your treatment. Your healthcare provider may change your dose of Jakafi or stop your treatment based on the results of your blood tests. Tell your healthcare provider right away if you experience unusual bleeding, bruising, fatigue, shortness of breath, or a fever.

Infection: You may be at risk for developing a serious infection while taking Jakafi. Tell your healthcare provider if you develop symptoms such as chills, nausea, vomiting, aches, weakness, fever, or painful skin rash or blisters.

The most common side effects of Jakafi include dizziness and headache.

These are not all the possible side effects of Jakafi. Ask your healthcare provider or pharmacist for more information. Tell your healthcare provider about any side effect that bothers you or that does not go away.

Before taking Jakafi, tell your healthcare provider about all the medications, vitamins, and herbal supplements you are taking and all your medical conditions, including if you have an infection, have or had liver or kidney problems, are on dialysis, or have any other medical condition. Do not drink grapefruit juice while taking Jakafi.

Women should not take Jakafi while pregnant or planning to become pregnant, or if breast-feeding.

Please see the Full Prescribing Information available at www.jakafi.com, which includes a more complete discussion of the risks associated with Jakafi.

About Incyte

Incyte Corporation is a Wilmington, Delaware-based biopharmaceutical company focused on the discovery, development and commercialization of proprietary small molecule drugs, primarily in oncology. For additional information on Incyte, please visit the Company's website at www.incyte.com.

Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including without limitation statements with respect to the potential efficacy, safety and therapeutic value of, and Incyte's plans and expectations for, ruxolitinib in polycythemia vera, including the potential for ruxolitinib to become the first JAK1/JAK2 inhibitor available for patients with polycythemia vera and an important new treatment for patients with polycythemia vera who are no longer responding to or are intolerant of hyroxyurea, and the expectation to present full data from the RELIEF trial at an upcoming scientific meeting, contain predictions and estimates and are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. These forward-looking statements are based on Incyte's current expectations and subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to the efficacy or safety of ruxolitinib, the results of further analyses of trial results, the results of further research and development, the high degree of risk and uncertainty associated with drug development, clinical trials and regulatory approval processes, other market or economic factors, competitive and technological advances, and other risks detailed from time to time in Incyte's filings with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the quarter ended March 31, 2014. Incyte disclaims any intent or obligation to update these forward-looking statements.

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