Four-Year Phase 3 Data Analysis Shows Durability of Response of Jakafi® (ruxolitinib) in Patients with Polycythemia Vera

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- Long-term data reinforce Jakafi® (ruxolitinib) as an effective long-term treatment option for patients with polycythemia vera (PV) who have had an inadequate response to or are intolerant of hydroxyurea

- Overall safety profile of Jakafi remained consistent with previously-reported 80-week RESPONSE data

WILMINGTON, Del.--(BUSINESS WIRE)--Dec. 10, 2017-- Incyte Corporation (Nasdaq:INCY) today announced new 208-week (4-year) follow-up data from the ongoing, global, multi-center, open-label Phase 3 RESPONSE study of Jakafi® (ruxolitinib) comparing the efficacy and safety of Jakafi with best available therapy (BAT) in patients with polycythemia vera (PV) who are resistant to or intolerant of hydroxyurea (HU). The pre-planned data analysis showed a durable primary response to Jakafi in patients with PV who are resistant to or intolerant of HU and the overall safety profile for Jakafi remained consistent with previously reported 80-week RESPONSE data.[1] The results were shared in an oral presentation today at the 59thAmerican Society of Hematology (ASH) Annual Meeting 2017 in Atlanta, Georgia.

“With 30 months of additional follow-up, the four-year RESPONSE data analysis presented today at ASH further reinforces the potential of Jakafi as a long-term option for patients with PV,” said Peg Squier, M.D., Ph.D., Head of U.S. Medical Affairs at Incyte. “Given the few treatment options available to treat this chronic and progressive blood cancer, these long-term safety and efficacy data are meaningful to patients with uncontrolled PV.”

The 80-week follow-up results from RESPONSE confirmed that among patients who initially responded to Jakafi treatment, the probability of maintaining primary and hematocrit (Hct) responses for ≥ 80 weeks was 92% and 89%, respectively, and hence Jakafi could be an effective long-term treatment option for patients with PV who are HU-resistant or intolerant.

At the week 208 analysis, the overall long-term safety profile remained consistent with the 80-week data analysis and the response was durable. In both the Jakafi arm and the crossover population, around 30% of patients completed the study treatment and 37% of patients were still receiving treatment.

“These are clinically relevant long-term safety and efficacy results, and further support the use of Jakafi in PV patients who have an inadequate response to or are intolerant of hydroxyurea,” said Srdan Verstovsek, M.D., Ph.D., medical oncologist and professor, Department of Leukemia at The University of Texas MD Anderson Cancer Center, Houston, Texas.

About the RESPONSE Trial

RESPONSE is an ongoing, global, multi-center, open-label, Phase 3 trial comparing the efficacy and safety of Jakafi® (ruxolitinib) with BAT in 222 patients (Jakafi, 110; BAT, 112) with PV who are resistant to or intolerant of hydroxyurea (HU).[2]

The primary response was a composite endpoint of the proportion of patients who achieved both hematocrit (Hct) control (defined as no phlebotomy eligibility from week 8 through week 32, with no more than 1 post-randomization phlebotomy eligibility up to week 8) and a spleen volume reduction of at least 35% from baseline at week 32. Phlebotomy eligibility was defined as an Hct >45% and at least 3 percentage points greater than baseline or an Hct >48%. Patients randomized to BAT could crossover (CO) to ruxolitinib at week 32 if they did not meet the primary endpoint, or after week 32 in case of disease progression (PBT eligibility, splenomegaly progression, or both).[2]

The primary endpoint of the RESPONSE study was achieved, demonstrating that Jakafi was superior to BAT at controlling Hct and reducing spleen volume at week 32.[2] The 80-week follow-up results from RESPONSE have been published previously and confirmed that ruxolitinib could be an effective long-term therapy option for HU-resistant/intolerant (R/I) patients with PV.[3]

Durability of the primary response, overall clinicohematologic (CLHM) response (defined as Hct control, platelet count ≤ 400 × 10^9/L, white blood cell count ≤ 10 × 10^9/L, and spleen volume reduction ≥ 35% by imaging), as well as long-term safety were updated at week 208.[1]

At week 208, the Kaplan-Meier (KM) estimate of duration of primary response was 0.73 (95% CI: 0.49, 0.87), and the KM estimate of duration of absence of PBT eligibility was 0.73 (95% CI: 0.60, 0.83). The KM estimate of duration of at least 35% reduction in spleen volume was 0.86 (95% CI: 0.61, 0.95). Median duration of primary and CLHM responses has not been reached.[1]

Out of the 70 patients (63.6%) in the Jakafi arm who achieved an overall CLHM response at week 32, 21 had progressed by week 208. The KM estimate of duration of complete hematological remission (defined as Hct control, platelet count ≤ 400 × 10^9/L, and white blood cell count ≤ 10 × 10^9/L) at 208-weeks was 0.54 (95% CI: 0.31, 0.72). RESPONSE data also demonstrated that the KM estimate for overall survival at 5-years was 90.6% (95% CI: 80.1, 95.7) for patients treated with Jakafi compared to 87.7% (95% CI: 74.8, 94.3) for patients treated with BAT.[1]

At the week 208 analysis, 41 patients (37%) originally randomized to the Jakafi arm were still receiving therapy (median exposure, 225 weeks) versus no patients on BAT (median exposure, 34 weeks). Among patients in the Jakafi arm, 29% completed the treatment as per protocol. Of the 98 patients who crossed over to Jakafi after week 32, 38% remained on Jakafi (median exposure, 189 weeks) and 31% completed treatment. Other main reasons for the study drug discontinuations (Jakafi + CO patients) were disease progression (11% + 8%), patient decision (6% + 6%), and adverse events (14% + 14%).[1]

The most common adverse events in the Jakafi randomized arm (week 208 vs week 80) per 100 patient-years of exposure were anemia (9.3 vs 13.2), pruritus (7.3 vs 9.7), diarrhea (7.1 vs 9.7), headache (6.1 vs 10.5), arthralgia (5.9 vs 6.1), increased weight (5.6 vs 7.5) and muscle spasms (5.4 vs

7.9).

The 208-week results (Abstract #322) were presented as a part of an oral session (#634) on Sunday, December 10, 2017, 7:30-9:00 AM Eastern Time (8:15 AM), Building C, Level 2, C208-C210.

About Polycythemia Vera (PV)

Polycythemia vera (PV) is a myeloproliferative neoplasm (MPN) and is typically characterized by elevated hematocrit, the percent volume of red blood cells in the blood, which can lead to a thickening of the blood and an increased risk of blood clots. An elevated white blood cell and/or platelet count may also be present.[4] Patients with PV who fail to consistently maintain appropriate hematocrit levels have a four times higher risk of major thrombosis (blood clots) or cardiovascular death.[5] Patients with PV can also suffer from an enlarged spleen and a significant symptom burden which may be attributed to thickening of the blood and lack of oxygen to parts of the body.[6] Signs and symptoms of PV commonly include fatigue, itching, night sweats, bone pain, fever, and unexplained weight loss.[7]

Approximately 100,000 patients in the U.S. are living with PV.[8] Current standard treatment for PV is phlebotomy (the removal of blood from the body) plus aspirin. When phlebotomy can no longer control PV, chemotherapy such as hydroxyurea, or interferon, is utilized in high-risk patients.[9,10] Approximately one in four patients with PV are considered uncontrolled[11,12] because they have an inadequate response to or are intolerant of hydroxyurea, the most commonly used chemotherapeutic agent for the treatment of PV.

About Jakafi® (ruxolitinib)

Jakafi is a first-in-class JAK1/JAK2 inhibitor approved by the U.S. Food and Drug Administration for treatment of people with polycythemia vera (PV) who have had an inadequate response to or are intolerant of hydroxyurea.

Jakafi is also indicated for treatment of 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. Jakafi is a registered trademark of Incyte Corporation. Jakavi is a registered trademark of Novartis AG in countries outside the United States.

Important Safety Information

Jakafi can cause serious side effects, including:

Low blood counts: Jakafi® (ruxolitinib) 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 develop or have worsening symptoms such as unusual bleeding, bruising, tiredness, shortness of breath, or a fever.

Infection: You may be at risk for developing a serious infection during treatment with Jakafi. Tell your healthcare provider if you develop any of the following symptoms of infection: chills, nausea, vomiting, aches, weakness, fever, painful skin rash or blisters.

Skin cancers: Some people who take Jakafi have developed certain types of non-melanoma skin cancers. Tell your healthcare provider if you develop any new or changing skin lesions.

Increases in Cholesterol: You may have changes in your blood cholesterol levels. Your healthcare provider will do blood tests to check your cholesterol levels during your treatment with Jakafi.

The most common side effects of Jakafi include: low platelet count, low red blood cell counts, bruising, dizziness, headache.

These are not all the possible side effects of Jakafi. Ask your pharmacist or healthcare provider 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 tuberculosis (TB), or have been in close contact with someone who has TB, have or had hepatitis B, have or had liver or kidney problems, are on dialysis, had skin cancer or have any other medical condition. Take Jakafi exactly as your healthcare provider tells you. Do not change or stop taking Jakafi without first talking to your healthcare provider. Do not drink grapefruit juice while on Jakafi.

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

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

About Incyte

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

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Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding the potential for Jakafi to be a long-term treatment for patients with PV, contain predictions, estimates and other forward-looking statements. These forward-looking statements are based on the Company's current expectations and are subject to risks and uncertainties that may cause actual results to differ materially, including

unanticipated developments and the risks related to the efficacy or safety of the Company's development pipeline, 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 and competitive and technological advances; and other risks detailed from time to time in the Company's reports filed with the Securities and Exchange Commission, including its Form 10-Q for the quarter ended September 30, 2017. Incyte disclaims any intent or obligation to update these forward-looking statements.

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