U.S. FDA GRANTS PRIORITY REVIEW FOR FEDRATINIB NEW DRUG APPLICATION IN MYELOFIBROSIS

U.S. Food & Drug Administration sets Prescription Drug User Fee Act action date for Sept. 3, 2019

March 5, 2019 – SUMMIT, N.J. – Celgene Corporation (NASDAQ:CELG) today announced the U.S. Food and Drug Administration (FDA) has accepted the company’s New Drug Application (NDA) for fedratinib and granted a Priority Review. Fedratinib is a highly selective JAK2 inhibitor intended for the treatment of patients with myelofibrosis, a serious bone marrow disorder that disrupts the body’s normal production of blood cells.1 Under the Prescription Drug User Fee Act, the FDA has set its action date as Sept. 3, 2019.

“The acceptance of the NDA and granting of Priority Review for fedratinib represent the first potential new treatment option after many years for patients affected by myelofibrosis.” said Jay Backstrom, M.D., Chief Medical Officer for Celgene. “Patients with myelofibrosis, including the number who are ineligible for or failed existing therapy continues to increase, representing a well-defined unmet medical need. We believe fedratinib can play an important role in the treatment of myelofibrosis and we look forward to working with the FDA as the review process advances.”

The NDA for fedratinib is based on results from a randomized, placebo-controlled, phase 3 trial (JAKARTA) in patients with primary or secondary myelofibrosis, as well as a single-arm, open-label phase 2 trial (JAKARTA2) in patients with primary or secondary myelofibrosis previously exposed to ruxolitinib, the only FDA-approved treatment for the disease. Results of these two trials have been previously published in peer-reviewed journals. The FDA has also provided fedratinib Orphan Drug designation for the treatment of secondary and primary myelofibrosis.

Celgene also plans to evaluate fedratinib in combination with luspatercept.

Fedratinib is an investigational compound that is not approved for any use in any country.

About JAKARTA
JAKARTA is a pivotal phase 3, multicenter, randomized, double-blind, placebo-controlled trial evaluating the efficacy of daily oral doses (400 mg or 500 mg) of fedratinib compared with placebo in patients with intermediate-2 or high-risk primary myelofibrosis, post-polycythemia vera myelofibrosis or post-essential thrombocythemia myelofibrosis with splenomegaly. The study included 289 subjects across 94 sites in 24 countries.

The primary endpoint was spleen response rate, defined as the proportion of patients who had a reduction in spleen volume of at least 35% after six one-month treatment cycles with a magnetic resonance imaging (MRI) or computerized tomography (CT) scan four weeks later. Secondary endpoints included symptom response rate, defined as the proportion of patients with a 50% or greater reduction in Total Symptom Score after six one-month treatment cycles as measured by the modified Myelofibrosis Symptoms Assessment Form (MFSAF) v2.0 diary.
About JAKARTA2
JAKARTA2 is a phase 2, multicenter, open-label, single-arm trial evaluating the efficacy of a once daily dose of fedratinib (400 mg starting dose) in patients previously treated with ruxolitinib and with a diagnosis of intermediate-1 with symptoms, intermediate-2 or high-risk primary myelofibrosis, post-polycythemia vera myelofibrosis or post-essential thrombocytopenia myelofibrosis. The study included 97 subjects across 40 sites in 10 countries.

The primary endpoint was spleen response rate, defined as the proportion of patients who had a reduction in spleen volume of at least 35% as measured by MRI or CT scan after six one-month treatment cycles. Secondary endpoints included symptom response rate, defined as the proportion of patients with a 50% or greater reduction in Total Symptom Score after six one-month treatment cycles as measured by the modified Myelofibrosis Symptoms Assessment Form (MFSAF) v2.0 diary.

FDA placed a clinical hold on the fedratinib program in November 2013 after potential cases of Wernicke’s encephalopathy were reported among subjects (approximately 1%) in clinical trials. The FDA removed the clinical hold in August 2017.

About Fedratinib
Fedratinib is an oral kinase inhibitor with activity against wild type and mutationally activated Janus Associated Kinase 2 (JAK2) and FMS-like tyrosine kinase 3 (FLT3). Fedratinib is a JAK2-selective inhibitor with higher potency for JAK2 over family members JAK1, JAK3 and TYK2. Abnormal activation of JAK2 is associated with myeloproliferative neoplasms (MPNs), including myelofibrosis and polycythemia vera. In cell models expressing mutationally active JAK2 or FLT3, fedratinib reduced phosphorylation of signal transducer and activator of transcription (STAT3/5) proteins, inhibited cell proliferation, and induced apoptotic cell death. In mouse models of JAK2V617F-driven myeloproliferative disease, fedratinib blocked phosphorylation of STAT3/5, increased survival and improved disease-associated symptoms, including reduction of white blood cells, hematocrit, splenomegaly, and fibrosis.

About Myelofibrosis
Myelofibrosis is a serious and rare bone marrow disorder that disrupts the body’s normal production of blood cells. Bone marrow is gradually replaced with fibrous scar tissue, which limits the ability of the bone marrow to make red blood cells. The disorder can lead to anemia, weakness, fatigue and swelling of the spleen and liver, among other symptoms. Myelofibrosis is classified as a myeloproliferative neoplasm, a group of rare blood cancers that derive from blood-forming stem cells. In the U.S. myelofibrosis occurs in 1.5 of every 100,000 people each year. Both men and women are affected and, while the disease can affect people of all ages, the median age at diagnosis ranges from 60 to 67 years.

About Celgene
Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through next-generation solutions in protein homeostasis, immuno-oncology, epigenetics, immunology and neuro-inflammation. For more information, please visit www.celgene.com. Follow Celgene on Social Media: @Celgene, LinkedIn and Facebook.

Forward-Looking Statements
This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. Celgene undertakes no obligation to update any forward-looking
statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond each company's control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors, many of which are discussed in more detail in the Annual Report on Form 10-K and other reports of each company filed with the Securities and Exchange Commission, including factors related to the proposed transaction between Bristol-Myers Squibb and Celgene, such as, but not limited to, the risks that: management’s time and attention is diverted on transaction related issues; disruption from the transaction makes it more difficult to maintain business, contractual and operational relationships; pending legal proceedings or any future litigation instituted against Bristol-Myers Squibb, Celgene or the combined company could delay or prevent the proposed transaction; and Bristol-Myers Squibb, Celgene or the combined company is unable to retain key personnel.

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