CELGENE UPDATED ANALYSIS OF JAKARTA2 FEDRATINIB STUDY SHOWS CLINICALLY MEANINGFUL RESPONSES IN PATIENTS PREVIOUSLY TREATED FOR MYELOFIBROSIS WITH RUXOLITINIB

JUNE 3, 2019 — SUMMIT, N.J. — Celgene Corporation (NASDAQ: CELG) announced an updated analysis of data from the Phase 2 JAKARTA2 clinical study demonstrating clinically meaningful response rates with investigational fedratinib in patients with myelofibrosis previously treated with ruxolitinib. This updated analysis of fedratinib employed intent-to-treat (ITT) principles and utilized a narrower definition of ruxolitinib relapsed, refractory, or intolerant patients. Results were shared in a poster presentation today at the 2019 American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago.

In the ITT population (n=97), the proportion of patients who exhibited a 35% or greater reduction in spleen volume at end of cycle 6 was 31% (95% CI 22, 41). Of these 97 patients, 79 (81%) met the narrower criteria for ruxolitinib resistance or intolerance. In this cohort, the proportion of patients who exhibited a 35% or greater reduction in spleen volume at end of cycle 6 was 30% (95% CI 21, 42), consistent with the response rate observed in the ITT population. In addition, the proportion of patients who exhibited a 50% or greater symptom response rate was 27% in both the ITT population (95% CI, 18, 37) and the patients in the analysis of the narrower criteria (95% CI, 17, 39).

The most common grade 3–4 hematologic abnormalities were anemia (46%) and thrombocytopenia (24%). Most common non-hematologic treatment-emergent adverse events (TEAEs) in all treated patients were diarrhea (62%), nausea (56%), vomiting (41%), and constipation (21%).

“Myelofibrosis is a serious and rare bone marrow disorder for which there is only one currently approved treatment option,” said Claire N. Harrison, M.D., Deputy Clinical Director of Cancer and Haematology at a NHS Foundation Trust Hospital in London. “Patients may become intolerant or resistant to the therapy. These updated results show meaningful reductions in spleen volume and symptoms and reinforce the potential of fedratinib in these difficult-to-treat patients who no longer receive benefit from ruxolitinib.”

“Fedratinib has the potential to be the first new treatment option since 2011 for patients with myelofibrosis,” said Dr. Alise Reicin, President, Global Clinical Development, for Celgene. “Patients with myelofibrosis who are relapsed, refractory or intolerant to ruxolitinib represent a population of particularly high unmet medical need and we are committed to bringing this important treatment option forward.”

About JAKARTA2
JAKARTA2 is a Phase 2, multicenter, open label, single-arm trial that evaluated 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 thrombocythemia myelofibrosis. The study included 97 subjects across 40 sites in 10 countries.

The primary endpoint was spleen volume 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.

In August 2017, the FDA removed the clinical hold on the fedratinib development program that it had placed in 2013, following potential cases of Wernicke's encephalopathy (WE) being reported in eight out of 877 patients receiving one or more doses (less than 1% of treated patients). WE is a neurological condition induced by vitamin B1 deficiency that manifests itself in the form of paralysis of one or more extraocular muscles, lack of muscle coordination and confusion. Rates of WE range from 0.8% to 2.8% of the general population, as determined by autopsy studies, however the incidence in MPN patients is reportedly three times greater.1

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, 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, improved survival and disease-associated signs (including white blood cell counts, hematocrit, splenomegaly, and fibrosis).

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

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.2 The disorder can lead to anemia, weakness, fatigue and swelling of the spleen and liver, among other symptoms.2 Myelofibrosis is classified as a myeloproliferative neoplasm, a group of rare blood cancers that derive from blood-forming stem cells.3 In the U.S. myelofibrosis occurs in 1.5 of every 100,000 people each year,4 and between 16,000 and 18,500 people are living with the disease.5 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.6,7

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: Twitter, Pinterest, LinkedIn, Facebook and YouTube.
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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. We undertake 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 our 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 our Annual Report on Form 10-K and our other reports filed with the U.S. 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 make it more difficult to maintain business, contractual and operational relationships; any legal proceedings are instituted against Bristol-Myers Squibb, Celgene or the combined company that 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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