Study to Evaluate Dose and Schedule for Oral Epigenetic Therapy CC-486 in Lower-Risk MDS Presented at ASH

ABSTRACT #424

BOUDRY, Switzerland--(BUSINESS WIRE)--Dec. 10, 2012-- Celgene International Sàrl, a subsidiary of Celgene Corporation (NASDAQ: CELG), today announced that data from a phase I study of oral, epigenetic therapy CC-486 (oral azacitidine) in patients with lower-risk myelodysplastic syndromes (MDS) was presented at the 54th Annual Meeting of the American Society of Hematology in Atlanta, GA.

In the study, patients with lower-risk (IPSS Low or INT-1) MDS who were red blood cell transfusion-dependent and/or thrombocytopenic were enrolled and sequentially assigned to receive oral azacitidine 300 mg once daily for either 14 or 21 days of each 28-day cycle. Patients were assessed for safety and hematologic response.

At study entry, 40% of patients had received no prior MDS treatment (outside of transfusions), 45% had received erythropoiesis-stimulating agents, and 15% had received white blood cell growth factors. At the time of the analysis, 53 patients had been enrolled.

After a median seven treatment cycles for the 14-day treatment arm and five for the 21-day treatment arm, the most frequent grade 3/4 hematologic adverse events in the 14-day arm were anemia (11.5%), thrombocytopenia (11.5%) and neutropenia (7.7%), and in the 21-day arm were neutropenia (18.5%), febrile neutropenia (11.1%) and anemia (7.4%). The most frequent grade 3/4 non-hematologic adverse events were pneumonia (15.4%), vomiting (7.7%), diarrhea (7.7%) and cellulitis (7.7%) in the 14-day arm, and diarrhea (11.1%) and vomiting (7.4%) in the 21-day arm. At data cut-off, 36 patients had discontinued treatment, including eight who discontinued due to adverse events. Three patients in the 21-day arm and two patients in the 14-day arm received reduced doses of oral azacitidine (200 mg) due to adverse events.

The overall response rate for patients in the 14-day arm was 42.3% (11/26) and 37.0% (10/27) in the 21-day arm. Additionally, the percentage of patients showing any hematologic improvement was 26.9% (7/26) in the 14-day arm and 29.6% (8/27) in the 21-day arm. The percentage of patients who sustained RBC transfusion independence for 56 days was 53.5% (8/15) in the 14-day arm and 40.0% (6/15) in the 21-day arm. The percentage of patients who sustained RBC transfusion independence for 84 days was 20% (3/15) in the 14-day arm and 33.3% (5/15) in the 21-day arm.

Based on these and other early-stage data evaluating CC-486, Celgene plans to initiate two phase III studies (QUAZAR program) evaluating this oral agent in lower-risk MDS and acute myeloid leukemia by the end of 2012.

These results are from an investigational study. CC-486 is not approved for any indication.

About Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) are a group of hematologic malignancies that affect approximately 300,000 people worldwide. Myelodysplastic syndromes occur when blood cells remain in an immature or “blast” stage within the bone marrow and never develop into mature cells capable of performing their necessary functions. Eventually, the bone marrow may be filled with blast cells, suppressing normal cell development. According to the American Cancer Society, 10,000 to 20,000 new cases of MDS are diagnosed each year in the United States, with median survival rates ranging from approximately six months to six years for the different classifications of MDS. MDS patients must often rely on blood transfusions to manage symptoms of anemia and fatigue and may develop life-threatening iron overload and/or toxicity from frequent transfusions, thus underscoring the critical need for new therapies targeting the cause of the condition rather than simply managing its symptoms.

About Celgene International Sàrl

Celgene International Sàrl, located in Boudry, in the Canton of Neuchâtel, Switzerland, is a wholly owned subsidiary and international headquarters of Celgene Corporation. Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global pharmaceutical company engaged primarily in the discovery, development and commercialization of innovative
therapies for the treatment of cancer and inflammatory diseases through gene and protein regulation. For more information, please visit the Company's website at www.celgene.com.

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. 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 Securities and Exchange Commission.

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