CELGENE PRESENTS DATA FROM A PHASE 1/2 CLINICAL STUDY OF IBERDOMIDE IN COMBINATION WITH DEXAMETHASONE IN PATIENTS WITH RELAPSED AND REFRACTORY MULTIPLE MYELOMA AT ASCO 2019

Investigational treatment demonstrates favorable early safety and efficacy data in patients who had received a median five prior treatments including immunomodulatory agents, proteasome inhibitors and anti-CD38 agents


The results included preliminary safety and efficacy data from the ongoing multicenter, open-label, dose-escalation study, which aims to determine the maximum tolerated dose and the recommended phase 2 dose of iberdomide in combination with dexamethasone. Iberdomide is Celgene’s proprietary cereblon E3 ligase modulator (CELMoD®) compound with enhanced tumoricidal and immune stimulatory effects demonstrated in preclinical studies. The phase 1/2 study is expected to enroll approximately 300 participants.

“Nearly half a million people globally are affected by multiple myeloma, a cancer of plasma cells. The management of patients with late relapsed or refractory multiple myeloma continues to be challenging due to the complex nature of the disease’s pathophysiology. Despite the introduction of newer agents, patients continue to experience disease relapse therefore new therapeutic options are needed for patients who have failed multiple prior treatments,” said Sagar Lonial, MD, Chief Medical Officer at Winship Cancer Institute of Emory University. “The early data on iberdomide in combination with dexamethasone in these heavily pretreated patients show promising activity, and we look forward to advancing our understanding of this combination’s potential in this patient population.”

As of April 2019, 66 patients at a median age of 65 received the iberdomide plus dexamethasone combination, with iberdomide being administered in 8 incremental doses ranging from 0.3 mg to 1.3 mg. Escalating doses of iberdomide were given on days 1 through 21 in combination with dexamethasone (40 mg; 20 mg in patients older than 75) on days 1, 8, 15, and 22 of each 28-day cycle. Patients had a median of five prior multiple myeloma treatment regimens, which could have included stem cell transplant, immunomodulatory drugs including lenalidomide and pomalidomide, proteasome inhibitors and daratumumab.

Grade 3-4 adverse events (AE) reported included neutropenia (29%), infection (26%), anemia (24%), thrombocytopenia (12%), pulmonary embolism (1.5%) and peripheral sensory neuropathy (1.5%). Six patients (9%) discontinued treatment due to adverse events.
Of the 66 patients who received the iberdomide plus dexamethasone combination, 59 were evaluable for response. The overall response rate was 32% (19/59), with 29% (17/59) achieving a partial response and two patients achieving a very good partial response.

Patients (n=51) who were refractory to IMiD® compounds, which included lenalidomide and pomalidomide, had an overall response rate of 35% (18/51) with 33% (17/51) of patients achieving a partial response and one patient achieving a very good partial response. Further, patients who were refractory to both daratumumab and pomalidomide (n=27) had an overall response rate of 29% (8/27), with 25% (7/27) achieving a partial response and one patient achieving a very good partial response. Maximum tolerated dose and the recommended phase 2 dose have not yet been determined.

“While we have made tremendous progress in treating multiple myeloma, there is still a significant need for new options to address the heterogeneous nature of the disease. We are focused on exploring the potential of our next generation CELMoD compounds to help fill this gap,” said Dr. Alise Reicin, President, Global Clinical Development for Celgene. “The preliminary clinical activity and favorable safety data observed with the combination of iberdomide and dexamethasone are encouraging, particularly in patients who have failed multiple lines of therapy including patients who were refractory to lenalidomide, pomalidomide and/or daratumab.”

The phase 1/2 study is also evaluating iberdomide as monotherapy and in combination with daratumumab or bortezomib or carfilzomib. Iberdomide is investigational and has not been approved in any country.

**About Iberdomide (CC-220)**

Iberdomide is an investigational cereblon E3 ligase modulator (CELMoD®) compound that induces degradation of transcription factors Aiolos and Ikaros, thereby inhibiting growth of myeloma cells in vitro. In pre-clinical models, iberdomide has demonstrated ability to destroy tumor cells, stimulate an immune response, overcome resistance to immunomodulatory drugs, and synergize with dexamethasone, daratumumab and bortezomib.

**About CC-220-MM-001**

The open-label phase 1/2 CC-220-MM-001 dose escalation study (NCT02773030) seeks to determine the maximum tolerated dose and recommended phase 2 dose of iberdomide (CC-220) as monotherapy and in combination with dexamethasone, as well as further evaluate safety and preliminary efficacy in patients with relapsed/refractory multiple myeloma. The study consists of a dose-escalation portion (Part 1) as well as an expansion of these two cohorts at the recommended phase 2 dose to further evaluate safety and estimate preliminary efficacy (Part 2). The study also seeks to establish the maximum tolerated dose and recommended phase 2 dose of iberdomide when administered in combination with daratumumab or bortezomib or carfilzomib.

**About Multiple Myeloma**

Multiple myeloma is a life-threatening blood cancer that is characterized by tumor proliferation and suppression of the immune system. It is a rare but deadly disease – more than 32,000 new cases will be diagnosed in the United States in 2019, and approximately 13,000 deaths will occur in 2019, representing about 2.1% of all cancer-related deaths. The typical multiple myeloma disease course includes periods of symptomatic myeloma followed by periods of remission, and eventually, the disease becomes refractory (nonresponsive).
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; legal proceedings are 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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