June 14, 2017

Phase 2a Safety and Efficacy Data Support Further Development of Oral CC-220 in Patients with Lupus

Data showed positive trend in multiple disease measures versus placebo

Results presented at the Annual European Congress of Rheumatology (EULAR) 2017

BOUDRY, Switzerland--(BUSINESS WIRE)-- Celgene International Sàrl, a wholly owned subsidiary of Celgene Corporation (NASDAQ:CELG), today announced results from the phase 2a SLE-001 trial evaluating CC-220, the Company's investigational, oral immunomodulatory compound, at the Annual European Congress of Rheumatology (EULAR) 2017 in Madrid. A trend toward greater improvement with CC-220 treatment compared with placebo in multiple measures of disease activity, as measured by standard scores employed in clinical trials, was observed in patients with systemic lupus erythematosus (SLE), commonly known as lupus.

"While the outlook for patients with lupus has improved over the last 75 years, treatment options remain limited," said Dr. Richard Furie, chief of Rheumatology at Northwell Health in New York. "New treatment options are greatly needed for people who are dealing with this often disabling autoimmune condition."

The SLE-001 study randomized 42 patients who were diagnosed with lupus at least six months before and had a baseline SELENA-SLEDAI score greater than or equal to four. Participants were randomized to one of four escalating doses of CC-220 (CC-220 0.3 mg every other day, 0.3 mg once daily, 0.3 mg alternating with 0.6 mg once daily and 0.6 mg once daily) or placebo for 12 weeks, followed by 12 weeks of observational follow-up or long-term extension. The study evaluated safety, tolerability and pharmacokinetics. Efficacy endpoints, including changes in Cutaneous Lupus Area and Severity Activity Index (CLASI) and SELENA-SLEDAI score, were exploratory.

The most common adverse events (AEs) were nausea, diarrhea and maculopapular rash. Serious AEs were reported in two patients in the two highest CC-220 doses combined (pneumonia in both cases) and in two patients in the placebo group. Five patients in the two highest CC-220 dose groups and one patient in the placebo group discontinued due to AEs. No opportunistic infections or other systemic infections were reported in any CC-220 dose groups.

At day 85, reductions in skin-specific disease activity, as measured by CLASI, ranged from -4.3 to -7.8 in the CC-220 treatment groups, while a CLASI score increase of 0.4 was seen in the placebo group. Mean reductions in CLASI exceeded the minimal clinically important difference of -4.0 in the CC-220 groups. Additionally, more patients receiving CC-220 had at least a 4-point reduction in SELENA-SLEDAI score—an index used to assess lupus disease activity across 24 different disease descriptors (between 22.2 percent and 50.0 percent for CC-220 vs. 12.5 percent for placebo). A trend toward greater improvement in tender joint count and swollen joint count was seen in the CC-220 treatment groups compared with the placebo group at day 85. A trend toward improvement in the Physician's Global Assessment score was also seen at day 85 in the CC-220 groups (ranging from -0.5 to -0.9) compared with the placebo group (0.0). All data are as observed. The low number of study patients and some variability in baseline disease characteristics across treatment groups limit the interpretability of a dose response.

"Celgene is committed to addressing immunological diseases with serious unmet needs and investigating compounds that we believe could have the potential to improve the lives of patients. We're excited by the possibility that CC-220 may offer a novel mechanism to address lupus, a complex disease that has few effective treatment options," said Terrie Curran, President, Celgene Inflammation & Immunology. "Our work with CC-220 will also help to diversify and deepen our Inflammation and Immunology franchise as we continue to advance CC-220 in the clinic."

CC-220 is not approved for use in any indication in any country.

About CC-220

CC-220 is a novel, oral immunomodulatory compound that binds to and modulates cereblon, a component of the E3 ubiquitin ligase complex. CC-220 reduces levels of the transcription factors Ikaros and Aiolos, which are associated with an

increased risk of lupus. CC-220 is currently being studied in a phase 2 dose-finding study for lupus. It is also being studied in multiple myeloma.

About Systemic Lupus Erythematosus[1,2,3]

Systemic lupus erythematosus (SLE), commonly known as lupus, is an autoimmune disease in which the immune system creates antibodies that attack the body's healthy cells and tissues, including joints, skin, kidneys, heart, lungs, blood vessels and brain. Lupus affects five million people, mostly women, worldwide.

Symptoms of lupus may vary, but the most common include fatigue, pain or swelling in joints, muscle pain, skin rashes, hair loss, seizures, sun sensitivity, ulcers and fever as well as lung, kidney and heart problems. The disease may have periods without symptoms (known as remission) alternating with periods of disease flares. There is no cure for lupus, but medical interventions and lifestyle changes can help control it.

About Celgene

Celgene International Sàrl, located in Boudry, 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 www.celgene.com. Follow Celgene on Social Media: @Celgene, Pinterest, LinkedIn, Facebook and YouTube.

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 U.S. Securities and Exchange Commission.

References

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