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Results from Phase 2 Studies of Oral Ozanimod in Crohn's Disease and Ulcerative Colitis to Be Presented at World Congress of Gastroenterology at ACG2017

In the STEPSTONE open-label study, ozanimod demonstrated meaningful clinical and endoscopic improvements in patients with moderately to severely active Crohn's disease at week 12

In the TOUCHSTONE open-label extension study, ozanimod continued to demonstrate clinically meaningful results in moderately to severely active ulcerative colitis across multiple measures of disease activity through week 92

The safety and tolerability profile of ozanimod in both studies was generally consistent with previously reported studies of ozanimod

SUMMIT, N.J.--(BUSINESS WIRE)-- Celgene Corporation (NASDAQ:CELG) today announced that phase 2 data of investigational compound ozanimod in Crohn's disease and ulcerative colitis will be presented at the World Congress of Gastroenterology at ACG2017 (WCOG at ACG2017) in Orlando. The studies include new data from the STEPSTONE study in patients with moderately to severely active Crohn's disease (CD) and long-term data from the open-label extension of the TOUCHSTONE study in patients with moderately to severely active ulcerative colitis (UC). Ozanimod is an investigational, selective sphingosine 1-phosphate (S1P) 1 and 5 receptor modulator.

"The new STEPSTONE findings further validate our hypothesis of ozanimod in Crohn's disease and the latest data from the TOUCHSTONE study continue to support the potential of ozanimod to provide continued efficacy in ulcerative colitis at 92 weeks," said Terrie Curran, President, Celgene Inflammation and Immunology. "The results in both studies are highly encouraging as we execute pivotal studies of ozanimod in Crohn's disease and ulcerative colitis in hopes of advancing oral treatment options for these patients."

The STEPSTONE data presented at WCOG at ACG2017 will include the first 12-week data from the phase 2, open-label study. STEPSTONE evaluated endoscopic improvement and clinical outcomes following treatment with ozanimod 1.0 mg daily for 12 weeks in 69 patients with a mean age of 37.7 years with moderately to severely active CD. Patients with available data at baseline and week 12 were included in the analyses of the simple endoscopic score for Crohn's disease (SES-CD) matched intestinal segments (n=60) and the analysis of Crohn's disease activity index (CDAI) (n=59).

SES-CD score reductions from baseline of at least 50 percent and 25 percent were seen in 27 percent and 43 percent of patients, respectively. Patients who had shorter disease duration (disease duration ≤5 years) had a greater endoscopic response: 50 percent and 25 percent reductions were seen in 36 percent and 50 percent of patients, respectively. Patients who had lower baseline endoscopic disease activity (SES-CD ≤12) also had a greater endoscopic response: 50 percent and 25 percent reductions were seen in 30 percent and 49 percent of patients, respectively.

Reductions in mean CDAI scores from baseline were seen at week 4 (first post-baseline assessment), with further reductions through week 12. At week 12, a CDAI response (CDAI decrease ≥100) was observed in 66 percent of patients, and CDAI remission (CDAI < 150) was observed in 46 percent of patients. The mean CDAI reduction at week 12 was 130 points.

The most frequently reported adverse events (AEs) and serious AEs in patients appeared to be related to underlying CD. The most common AEs (≥5 percent) were CD flare, abdominal pain, nausea, lymphopenia, arthralgia, vomiting, increased gamma-glutamyl transpeptidase (GGT), urinary tract infection, paresthesia, anal abscess and increased alanine aminotransferase (ALT). The most common SAEs in two or more patients were CD flare, fistulizing disease, intestinal obstruction and abdominal abscess.

"Improvements in endoscopy are thought to correlate with long-term benefits for patients with Crohn's disease, a chronic condition in which patients are regularly seeking out additional options to manage their disease," said Brian G. Feagan, M.D., Robarts Clinical Trials and the University of Western Ontario in Canada. "The endoscopic, clinical improvement and the safety profile of ozanimod reported in the STEPSTONE study are encouraging and support its further development as a potential oral option for patients with Crohn's disease."

A separate presentation at WCOG at ACG2017 will report 92-week data on subjects who participated in the TOUCHSTONE open-label extension. TOUCHSTONE evaluated the efficacy and safety of 0.5 mg and 1.0 mg doses of ozanimod compared with placebo after eight weeks of treatment (induction phase) in 197 patients with moderate to severe UC. The primary endpoint was the proportion of patients in remission at week 8. Patients who achieved a clinical response at week 8 continued with their original treatment through week 32 in a maintenance phase. Previously reported results from TOUCHSTONE demonstrated statistically significant improvements in both the primary and secondary endpoints in patients who received 1.0 mg dose of ozanimod versus placebo.

TOUCHSTONE participants in all three treatment arms entered the ongoing open-label extension if they did not respond to treatment after the induction phase, relapsed during the maintenance phase or completed the maintenance phase (170 of the 197 patients). The objective of the extension phase was to evaluate the long-term efficacy and safety of daily ozanimod 1.0 mg. Efficacy data are reported as observed through week 92 and safety data includes all events through the data cut-off of March 2017.

At week 92, of the 100 patients who underwent efficacy evaluations, 91 percent had little or no active disease based on the physician global assessment (PGA 0 or 1), 97 percent had little or no blood in their stools (rectal bleeding subscore [RBS] 0 or 1) and 86 percent had no blood in the stools (RBS 0).

The safety profile at week 92 was similar to that reported in the placebo-controlled portion of the study. The most common AEs were UC flare, anemia, upper respiratory tract infection and back pain. The only SAEs in two or more patients were anemia and UC flare.

"People with ulcerative colitis need additional treatment options that can provide long-term benefit," said William Sandborn, M.D., Professor of Medicine and Chief, Division of Gastroenterology and Director, University of California San Diego Inflammatory Bowel Disease Center. "These results further support the potential of longer-term treatment with ozanimod."

About STEPSTONE

STEPSTONE is a phase 2, open-label study in patients with moderately to severely active Crohn's disease (CD). Data from the first 12 weeks examined endoscopic and clinical outcomes following treatment with ozanimod 1.0 mg daily for 12 weeks. Active Crohn's disease was defined as CDAI score 220-450 and total SES-CD of 6 or greater (or in isolated ileum disease SES-CD of 4 or greater). For the 69 enrolled patients, baseline mean age was 37.7 years, mean SES-CD was 13.3 and mean CDAI was 320. Mean CD duration was 10 years, with 54 percent of patients having had prior exposure to biologic therapy. All endoscopic assessments were read in a blinded manner by an imaging core lab. Daily electronic diary records were used to collect CD symptoms (including abdominal pain and soft/loose stool frequency). SES-CD was evaluated at baseline and week 12, and CDAI was assessed at baseline and weeks 4, 8 and 12.

About TOUCHSTONE

TOUCHSTONE is a phase 2, randomized, double-blind, placebo-controlled trial comparing the efficacy and safety of ozanimod (also known as RPC1063) with placebo in patients with moderately to severely active ulcerative colitis. A total of 197 patients were randomized and treated once daily with 1.0 mg ozanimod (n=67), 0.5 mg ozanimod (n=65) or placebo (n=65) for eight weeks (the induction phase). The primary endpoint was the proportion of patients in remission (Mayo score ≤2, no subscore > 1) at week 8. Secondary endpoints were the proportion of patients achieving clinical response (reduction in Mayo score of ≥3 and ≥30 percent with a decrease in the rectal bleeding score of ≥1 or a rectal bleeding score ≤1), proportion of patients with mucosal improvement (endoscopy score ≤1) and the change in Mayo score. Safety assessments included ECG testing, pulmonary function testing, optical coherence tomography and adverse events.

For the maintenance phase, patients who achieved a clinical response at week 8 continued with their original treatment through week 32. In the open-label extension phase, all patients (n=170) were treated with ozanimod 1.0 mg. The week 44 visit was completed by 131 patients.

About Ozanimod

Ozanimod is a novel, oral, selective, sphingosine 1-phosphate 1 and 5 receptor modulator in development for immune-inflammatory indications including relapsing multiple sclerosis, ulcerative colitis and Crohn's disease. Selective binding with S1PR1 receptors is believed to inhibit a specific subset of activated lymphocytes from migrating to sites of inflammation. The result is a reduction of circulating lymphocyte count that leads to anti-inflammatory activity. Importantly, immune surveillance is maintained.

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

About Crohn's Disease

Crohn's disease is an immune-mediated, chronic inflammatory condition of the gastrointestinal tract. Estimated to affect as many as three out of every 1,000 people in Europe and North America, the disease is becoming more common for all ethnic groups. Symptoms of Crohn's disease — including abdominal pain, diarrhea, fatigue, fever, weight loss and malnutrition — most commonly begin to appear between the ages of 13 and 30, although the disease can strike at any age. The disease may affect any part of the GI tract, from the mouth to the anus, but most commonly affects the end of the small bowel (the ileum) and the beginning of the colon. The exact cause of Crohn's disease is unknown, and there is no cure. People with Crohn's disease have a slightly reduced life expectancy.

About Ulcerative Colitis

Ulcerative colitis is a chronic, relapsing condition triggered by an abnormal, prolonged immune response that creates long-lasting inflammation and ulcers (sores) in the mucosa (lining) of the large intestine (colon). Symptoms usually develop over time, rather than suddenly. The disease can be debilitating and can sometimes lead to life-threatening complications. Ulcerative colitis is the most common form of inflammatory bowel disease worldwide. About one in every 198 people in Europe, and one in every 402 people in North America, have ulcerative colitis. In 2004, 2.1 million prescriptions were written to treat ulcerative colitis, and 716,000 ambulatory care visits were related to the disease. In 2010, there were 107,000 hospitalizations due to ulcerative colitis.

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, 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.

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