Celgene Announces Interim Topline Data from Trial of Investigational Oral GED-0301 in Patients with Active Crohn's Disease

Data show endoscopic improvement and clinical response and remission at week 12

Safety and tolerability consistent with previous studies

Results expected to be presented at upcoming medical meetings

SUMMIT, N.J.--(BUSINESS WIRE)-- Celgene Corporation (NASDAQ:CELG) today announced interim topline data from a randomized, double-blind, multicenter, exploratory phase 1b study evaluating the effects of oral GED-0301 (mongersen) on both endoscopic and clinical outcomes in patients with active Crohn's disease.

The trial, CD-001, is an ongoing study evaluating three different treatment regimens of GED-0301 in a 12-week treatment phase, followed by an observation phase up to 52 weeks (off treatment). The primary objective of the study is to explore the effect of GED-0301 on endoscopic outcomes. The trial enrolled a total of 63 patients across multiple countries.

The study was designed to further enhance the understanding of GED-0301 activity in a difficult-to-treat, moderate-to-severe patient population. This population was more diverse than prior GED-0301 studies and included patients with endoscopically confirmed mucosal damage at entry and those who had previous surgeries. The study also included both biologic exposed and biologic naïve patients as well as patients with a diagnosis of ileitis, ileocolitis or colitis.

Topline data from CD-001 show that in a proportion of patients treated with oral GED-0301 there was endoscopic improvement (defined as a 25 percent improvement from baseline) and clinical response and remission across all treatment groups at week 12. Findings to date reveal no new safety signals and tolerability is consistent with earlier studies.

"Given the high unmet need in Crohn's disease, we are pleased that oral GED-0301 showed both endoscopic improvements and clinically meaningful responses and remission at an early timepoint in this study," said Scott Smith, President of Celgene Inflammation and Immunology. "These data are particularly encouraging for several reasons, including the difficult-to-treat patient population evaluated in the trial."

"At this early 12-week timepoint, we're looking at the proportion of patients who had a 25 percent or greater endoscopic improvement, suggesting mucosal healing is underway in these patients," said Dr. William Sandborn, M.D., Professor of Medicine and Chief, Division of Gastroenterology and Director, University of California San Diego Inflammatory Bowel Disease Center. "These data support the notion that GED-0301, a potential first-in-class oral antisense therapy, may target an underlying cause of Crohn's disease, rather than simply improving symptoms."

Full data from the 12-week timepoint have been submitted for presentation at an upcoming scientific meeting later this year. The CD-001 study is ongoing until all patients complete the observation phase. Data from the observation portion of the trial are expected in 2017. The Phase III registration program is ongoing.

About CD-001

CD-001 is a phase 1b randomized, double-blind, multicenter, exploratory study evaluating the effects of oral GED-0301 on endoscopic and clinical outcomes in patients with active Crohn's disease. A total of 63 patients were randomized in a 1:1:1 ratio to receive one of three treatment regimens in a 12-week treatment phase: GED-0301 160 mg once daily for 12 weeks; GED-0301 160 mg once daily for eight weeks followed by four weeks of placebo; or GED-0301 160 mg once daily for four weeks followed by eight weeks of placebo. This treatment phase was followed by an off-treatment observation phase for up to 52 weeks. Eligible patients can also enter an extension phase (on treatment) for an additional 24 weeks.

About GED-0301
The investigational oral antisense therapy GED-0301 is an oligonucleotide that is designed to target the messenger RNA (mRNA) for Smad7, thereby reducing Smad7 protein levels. In patients with Crohn's disease, abnormally high levels of Smad7 interfere with TGF-β1 anti-inflammatory pathways in the gut, leading to increased inflammation. GED-0301 is designed to act locally and is thought to reduce Smad7 levels with negligible systemic exposure.

GED-0301 (mongersen) 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 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](http://www.celgene.com). Follow Celgene on Social Media: [@Celgene](https://twitter.com/Celgene), [Pinterest](https://www.pinterest.com/celgene/), [LinkedIn](https://www.linkedin.com/company/celgene-corporation), [Facebook](https://www.facebook.com/celgene) and [YouTube](https://www.youtube.com/celgene).

**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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