JUNE 4, 2019 — SUMMIT, N.J. — Celgene Corporation (NASDAQ: CELG) today announced that data from the TRANSCEND CLL 004 and TRANSCEND NHL 001 trials studying the investigational anti-CD19 chimeric antigen receptor (CAR) T-cell therapy lisocabtagene maraleucel (liso-cel; JCAR017) in patients with B-cell blood cancers were presented at the American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago.

Updated results from the ongoing, open-label multicenter phase 1/2 TRANSCEND CLL 004 study (Abstract #7501) of liso-cel in patients with relapsed/refractory (R/R) chronic lymphocytic leukemia or small lymphocytic lymphoma (CLL/SLL) were presented in an oral presentation today. The data included safety and efficacy findings from 23 patients who received liso-cel infusion at one of two dose levels: 50 × 10^6 or 100 × 10^6 total CAR-positive T cells following lymphodepleting chemotherapy. All patients had been previously treated with ibrutinib, and more than half had received prior venetoclax. The median number of lines of prior therapy was five and 83% of patients had high-risk cytogenetic features.

In the study, 22 of 23 patients were evaluable for response. The best overall response rate was 82% (18/22), with 46% (10/22) of patients achieving complete remission with or without complete blood count recovery (CR/CRi). Of 20 patients evaluable for minimal residual disease (MRD), 75% (15/20) achieved undetectable MRD (uMRD) by blood measures (sensitivity, 10^-4) and 65% (13/20) achieved uMRD by bone marrow measures (sensitivity, 10^-4). Responses have been durable, with 83% of patients (5/6) who were in CR/CRi at six months post liso-cel infusion showing ongoing response.

“For patients who have failed the current standard of care treatments for CLL, such as ibrutinib and venetoclax, there is a need for additional treatment options,” said lead study investigator Tanya Siddiqi, M.D., City of Hope National Medical Center. “I am highly encouraged by this early data showing manageable toxicity and promising clinical activity in a heavily pretreated patient population with high-risk CLL. In this preliminary analysis, clinical responses are rapid, deep and durable when assessed by clinical and MRD criteria. We look forward to further investigation of liso-cel in CLL patients who have relapsed from or have become refractory to currently available treatment options.”

The most common treatment-emergent adverse events (TEAEs) of any grade were anemia (83%), cytokine release syndrome (CRS; 74%), thrombocytopenia (74%), neutropenia (57%), and leukopenia (48%). There were two patients with dose-limiting toxicities among the 14 patients treated at 100 × 10^6 total CAR-positive T cells: grade 4 hypertension in one patient; and grade 3 encephalopathy, grade 3 muscle weakness and grade 4 tumor lysis syndrome in the other patient. Across 23 patients evaluable for safety, TEAEs of note included grade 3 CRS...
(2/23), grade ≥ 3 neurological events (5/23), and grade ≥ 3 tumor lysis syndrome (4/23). No grade 5 CRS or neurological events occurred.

In addition to these findings from TRANSCEND CLL 004, preliminary safety and efficacy data were presented from two subgroup analyses from the ongoing, open-label multicenter phase 1 TRANSCEND NHL 001 trial evaluating liso-cel in patients with R/R B-cell non-Hodgkin’s lymphoma at one of two dose levels: 50 × 10^6 or 100 × 10^6 total CAR-positive T cells following lymphodepleting chemotherapy. The data included results from a subgroup of patients with secondary central nervous system (CNS) lymphoma (n=9) (Abstract #7515) and from patients with R/R mantle cell lymphoma (MCL; n=17) (Abstract #7516). These were highlighted in a poster discussion session on Monday, June 3.

Patients had secondary CNS lymphoma at the time of first treatment (n=7; 6 DLBCL, 1 MCL) or retreatment with liso-cel (n=2 DLBCL), and neurological events and CRS were observed in only one patient. Of the 6 DLBCL patients with CNS lymphoma at the time of first retreatment with liso-cel, 4 achieved complete responses, 2 of whom are in sustained remission at more than 8 and 17 months, respectively.

The data in patients with MCL included safety and preliminary efficacy findings for 17 treated patients. The most common grade ≥ 3 TEAEs were thrombocytopenia (41%), anemia (35%) and neutropenia (35%). Grade ≥ 3 CRS and neurological events occurred in 6% and 12% of patients, respectively. One fatal event of tumor lysis syndrome was observed. The best overall response rate across dose levels was 71% (12/17); the best complete response rate was 53% (9/17). These results are consistent with those seen in all patients treated with liso-cel in the TRANSCEND NHL 001 study.

“We are pleased to share these new data, which continue to demonstrate the potential of liso-cel in a range of B-cell cancers, at this year’s ASCO,” said Alise Reicin, M.D., President, Global Clinical Development at Celgene. “These results support our continued commitment to broadly develop CAR T-cell therapies to address the clinical needs of patients living with blood cancers.”

Liso-cel is not approved in any country.

About Liso-cel
Liso-cel is an investigational defined composition CD19-directed CAR T-cell product candidate using a 4-1BB costimulatory domain. Celgene’s lead CAR T trial, TRANSCEND NHL-001, is studying liso-cel in adult patients with relapsed or refractory diffuse large B-cell lymphoma, primary mediastinal B-cell lymphoma, follicular lymphoma Grade 3B and mantle cell lymphoma.

About Celgene’s Cell Therapies
Celgene is committed to advancing the field of immune cell therapy – from pursuing scientific breakthroughs to enabling routine clinical use – so that more patients may benefit from the research that may ultimately lead to these emerging treatments. Celgene is building a portfolio of cell therapies supported by more than 15 years of development, including several chimeric antigen receptor (CAR) T-cell agents in registrational trials across multiple disease states, and a growing early-stage pipeline that expands cell therapy targets and technologies. We are advancing cell therapies in diffuse large B-cell lymphoma, multiple myeloma and other B-cell malignancies. These efforts underscore our belief in the promise of cell therapy to redefine the way patients fight cancer and to potentially transform how these diseases are treated.
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, LinkedIn and Facebook.

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, 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; any legal proceedings are instituted against Bristol-Myers Squibb, Celgene or the combined company that 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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Celgene Corporation
Investors:
+1-908-673-9628
ir@celgene.com or

Media:
+1-908-673-2275
media@celgene.com