June 5, 2017

bluebird bio and Celgene Corporation Announce Updated Clinical Results from Ongoing First-in-Human Multicenter Study of bb2121 Anti-BCMA CAR T Cell Therapy in Relapsed/Refractory Multiple Myeloma at ASCO Annual Meeting

- 100% of the 15 evaluable patients in active dose cohorts (doses above 50 x 10^6) achieved an objective response; overall response rate (ORR) across all cohorts (n=18) is 89% -

- 73% of evaluable patients in active dose cohorts achieved a very good partial response (VGPR) or better; 27% complete response (CR) rate across active dose cohorts -

- All patients tested for minimal residual disease (MRD) status (n=4) were found to be MRD-negative -

- No disease progression has been observed in active dose cohorts as of May 4, 2017 data cut-off; range of follow-up was 8 to 54 weeks -

- No dose-limiting toxicities have been observed -

- bluebird to host event with live webcast, Monday, June 5, 6:30 p.m. CT -

CAMBRIDGE, Mass. & SUMMIT, N.J.--(BUSINESS WIRE)-- bluebird bio, Inc. (NASDAQ:BLUE), and Celgene Corporation (NASDAQ:CELG) today announced that updated results from the ongoing CRB-401 Phase 1 clinical study of bb2121, an investigational anti-BCMA CAR T cell therapy, in 18 patients with relapsed/refractory multiple myeloma will be presented at the American Society of Clinical Oncology (ASCO) Annual Meeting in Chicago, Illinois. The objective of this Phase 1 dose-escalation study is to evaluate safety and efficacy of bb2121 and determine a recommended Phase 2 dose. bluebird bio and Celgene are jointly developing bb2121.

This Smart News Release features multimedia. View the full release here:
http://www.businesswire.com/news/home/20170605005423/en/

"It is impressive to see objective responses in all patients treated at dose levels of 150 x 10^6 CAR+ T cells or higher in such a heavily pretreated population, including those with high tumor burden. We are encouraged by the duration and depth of responses, and pleased that the safety profile remains readily manageable," said David Davidson, M.D., chief medical officer, bluebird bio. "Although these data are still early, it is encouraging that no patient in the active dose cohorts has had myeloma progression. In light of these results, we look forward to initiating the expansion phase of the CRB-401 study in the coming months."

"The heavily pretreated, relapsed/refractory patients in this study have few effective treatment options, highlighting the importance of this interim data. All patients previously underwent autologous HSCT, and received a median of 7 lines of prior therapy," said Michael Pehl, President, Hematology and Oncology for Celgene. "The consistency, depth and durability of these patients' responses coupled with a manageable safety profile is very exciting, and we believe will provide hope for patients in this setting. Efforts are underway to advance the development of bb2121 for patients with relapsed/refractory multiple myeloma."

First-in-Human Multicenter Study of bb2121 anti-BCMA CAR T Cell Therapy for Relapsed/Refractory Multiple Myeloma: Updated Results. (Abstract #3010)

Presenter: Jesus G. Berdeja, M.D., Sarah Cannon Research Institute and Tennessee Oncology, Nashville, TN

Date: Monday, June 5, 2017, 4:45-6:00 pm CT (poster discussion); 8:00-11:30 am CT

Location: Hall D1

Session Title: Poster Discussion Session: Developmental Therapeutics—Immunotherapy

The open-label Phase 1 CRB-401 study (NCT02658929) is investigating the administration of bb2121 anti-BCMA CAR T cells in patients with relapsed and/or refractory multiple myeloma. The primary endpoint of the study is incidence of adverse events (AEs) and abnormal laboratory test results, including dose-limiting toxicities (DLTs). The study also seeks to assess disease-specific response criteria including: complete response (CR), very good partial response (VGPR), and partial response (PR) according to the International Myeloma Working Group (IMWG) Uniform Response Criteria for Multiple Myeloma. The study also seeks to determine the recommended dose for further clinical trials.

Patients on study were heavily pre-treated, with a median of seven prior therapies (range: 3 - 14):

- 100% previously treated with lenalidomide and bortezomib
- 91% previously treated with pomalidomide and carfilzomib
- 71% previously treated with daratumumab
- 29% of patients were penta-refractory (bortezomib, lenalidomide, carfilzomib, pomalidomide, daratumumab)
- All patients had at least one prior autologous stem cell transplant (ASCT)

As of the May 4, 2017 data cut-off, 21 patients had been enrolled and dosed in four dose cohorts: 50 x 10^6, 150 x 10^6, 450 x 10^6 and 800 x 10^6 CAR+ T cells. All 21 dosed patients were evaluable for safety, and 18 patients have undergone their first multiple myeloma tumor restaging and were evaluable for efficacy. This study has enrolled patients at seven sites in the U.S., with an anticipated total enrollment of up to 50 patients.

Patients received a conditioning regimen of cyclophosphamide and fludarabine, followed by an infusion of bb2121 anti-BCMA CAR T cells. The CAR T cells were produced from each patient's own blood cells, which were modified using a lentiviral vector encoding the anti-BCMA CAR.

bb2121 is an investigational compound that is not approved for any use in any country.

Results, as of May 4, 2017 Data Cut-off:

Cohort	1	2	3	4
CAR+ T Cell Dose	50 x 10^6	150 x 10^6	450 x 10^6	800 x 10^6
Number of Patients Evaluable for Efficacy	3	4	8	3
Overall Response Rate in Cohort	33%	100%	100%	100%
Best Response	PD (1 patient) SD (1 patient) PR (1 patient)	CR (2 patients; 1 patient MRD negative) VGPR (1 patient MRD negative) PR (1 patient)	CR (1 patient*) VGPR (5 patients; 1 patient MRD negative) PR (2 patients; 1 patient MRD negative) *Patient died of unrelated cardio pulmonary arrest	VGPR (1 patient) PR (1 patient) CR (1 patient)
		All patients in cohorts 2, 3 and 4 with bone marrow involvement at baseline had no detectable multiple myeloma cells in their bone marrow on Day 14 or beyond. Of four patients evaluable for MRD status, all four were found to be MRD-negative.		
Median Prior Lines of Therapy	7 (range: 3-14); all patients had at least one prior autologous stem cell transplant, as well as prior exposure to a proteasome inhibitor and an immunomodulatory agent; 71% of patients had previously received daratumumab or CD38 antibody.			

Safety	15/21 (71%) of patients had CRS, mostly Grade 1 & 2; 2 patients with Grade 3 CRS that resolved within 24 hours. 4 patients received tocilizumab, 1 (Grade 2 CRS) received steroids. The most common treatment-emergent Grade 3-4 AEs in 21 infused patients include cytopenias commonly associated with cy/flu lymphodepletion, as well as Grade 3 events of hyponatraemia (n=4), cytokine release syndrome (n=2), upper respiratory infection (n=2), and syncope (n=2).

Webcast Information

bluebird bio will host a live webcast at 6:30 p.m. CT (7:30 p.m. ET) today, June 5, 2017. The live webcast can be accessed under "Calendar of Events" in the Investors and Media section of the company's website at www.bluebirdbio.com.

About bluebird bio, Inc.

With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built an integrated product platform with broad potential application to severe genetic diseases and cancer. bluebird bio's gene therapy clinical programs include its Lenti-D™ product candidate, currently in a Phase 2/3 study, called the Starbeam Study, for the treatment of cerebral adrenoleukodystrophy, and its LentiGlobin™ product candidate, currently in four clinical studies for the treatment of transfusion-dependent β-thalassemia, and severe sickle cell disease. bluebird bio's oncology pipeline is built upon the company's leadership in lentiviral gene delivery and T cell engineering, with a focus on developing novel T cell-based immunotherapies, including chimeric antigen receptor (CAR T) and T cell receptor (TCR) therapies. bluebird bio's lead oncology program, bb2121, is an anti-BCMA CAR T program partnered with Celgene. bb2121 is currently being studied in a Phase 1 trial for the treatment of relapsed/refractory multiple myeloma.

bluebird bio also has discovery research programs utilizing megaTAL/homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

bluebird bio has operations in Cambridge, Massachusetts, Seattle, Washington and Europe.

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.

About the bluebird bio-Celgene Collaboration

In March 2013, bluebird bio and Celgene entered into a collaboration to develop chimeric antigen receptor (CAR) T cell therapies to target and destroy cancer cells. In June 2015, the collaboration was amended and restated to focus on developing product candidates targeting B-cell maturation antigen (BCMA). bluebird bio and Celgene are working together on the initial, lead anti-BCMA product candidate (bb2121), and are developing next-generation anti-BCMA product candidates, including bb21217.

Forward-Looking Statements

This press release contains forward-looking statements, which are generally statements that are not historical facts, including statements regarding the potential of the bb2121 product candidate to treat relapsed/refractory multiple myeloma and future clinical development plans of the Company and Celgene. 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. Neither Celgene nor bluebird bio undertake any 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 each company's control. These risks and uncertainties include, but are not limited to, the risk that the bb2121 product candidate will not be successfully developed, approved or commercialized in relapsed/refractory multiple myeloma, or the risk that the bb2121 product candidate will be safe and efficacious in other disease settings. 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 the section entitled "Risk Factors" of the Annual Report on Form 10-K and other reports of each company filed with the Securities and Exchange Commission.

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