November 16, 2017

# Celgene Corporation and bluebird bio Announce bb2121 Anti-BCMA CAR-T Cell Therapy Has Been Granted Breakthrough Therapy Designation from FDA and Prime Eligibility from EMA for Relapsed and Refractory Multiple Myeloma

*Designations based on preliminary clinical data from ongoing phase I study of bb2121 in heavily pre-treated multiple myeloma*

SUMMIT, N.J. & CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Celgene Corporation (NASDAQ:CELG) and bluebird bio, Inc. (NASDAQ:BLUE) today announced that bb2121, a chimeric antigen receptor T-cell (CAR-T) therapy targeting b-cell maturation antigen (BCMA) in previously treated patients with multiple myeloma, has been granted Breakthrough Therapy Designation (BTD) by the U.S. Food and Drug Administration (FDA) and PRIority MEdicines (PRIME) eligibility by the European Medicines Agency (EMA).

BTD designation and PRIME eligibility for bb2121 were based on preliminary clinical data from the ongoing phase 1 study CRB-401. Updated data from CRB-401 is scheduled to be presented at the 59$^{th}$ annual meeting of the American Society of Hematology in Atlanta during an oral presentation on Dec. 11.

"Receiving Breakthrough Therapy Designation and PRIME eligibility for bb2121 further underscores the potential of this novel cellular immunotherapy approach to multiple myeloma treatment," said Jay Backstrom, M.D., Chief Medical Officer and Head of Global Regulatory Affairs for Celgene. "We will work closely with these agencies as we accelerate development of bb2121, a novel technology and therapy for patients with multiple myeloma."

"Despite recent advances, multiple myeloma remains an incurable disease, and heavily pretreated patients have limited therapeutic options," said David Davidson, M.D., Chief Medical Officer for bluebird bio. "Early data suggest that treatment with bb2121 has the potential to induce durable responses in this patient population. It is encouraging for both the FDA and EMA to identify bb2121 as a candidate for accelerated development as we continue our work with Celgene to bring this therapy to patients in need of new options."

Breakthrough Therapy Designation is intended to expedite the development and review of drugs that are intended to treat serious or life-threatening conditions. The criteria for breakthrough therapy designation require preliminary clinical evidence that demonstrates the drug may have substantial improvement on at least one clinically significant endpoint over available therapy.

PRIME is a program launched by the EMA to enhance support for the development of medicines that target an unmet medical need. This voluntary program is based on enhanced interaction and early dialogue with developers of promising medicines, to optimize development plans and speed up evaluation so these medicines can reach patients earlier. The program focuses on medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. These medicines are considered priority medicines by EMA. To be accepted for PRIME, a medicine must show its potential to benefit patients with unmet medical needs based on early clinical data.

**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 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

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

**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's lead oncology program, bb2121, is an anti-BCMA CAR T program currently being studied in a Phase 1 trial for the treatment of relapsed/refractory multiple myeloma. bluebird bio and Celgene are also working together on a second anti-BCMA CAR T program, bb21217.

**Forward-Looking Statements**

*This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Such forward-looking statements include those regarding the potential benefits of, and plans relating to the collaboration between bluebird bio and Celgene; the potential of bb2121 as a therapeutic drug; and the benefit of each company's strategic plans and focus. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Such statements are subject to numerous important factors, risks and uncertainties that may cause actual events or results to differ materially from current expectations and beliefs. For example, there can be no guarantee that any product candidate will be successfully developed or complete necessary preclinical and clinical phases, or that development of any of product candidates will successfully continue. There can be no guarantee that any positive developments will result in stock price appreciation. Management's expectations and, therefore, any forward-looking statements in this press release could also be affected by risks and uncertainties relating to a number of other important factors, including: results of clinical trials and preclinical studies, including subsequent analysis of existing data and new data received from ongoing and future studies; the content and timing of decisions made by the U.S. FDA and other regulatory authorities, investigational review boards at clinical trial sites and publication review bodies; the ability to obtain and maintain requisite regulatory approvals and to enroll patients in planned clinical trials; unplanned cash requirements and expenditures; competitive factors; the ability to obtain, maintain and enforce patent and other intellectual property protection for any product candidates; the ability to maintain key collaborations; and general economic and market conditions. These and other risks are described in greater detail under the caption "Risk Factors" included in each company's public filings with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and neither company has any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.*

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