

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549**

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): June 5, 2017

bluebird bio, Inc.

(Exact name of Registrant as Specified in Its Charter)

DELAWARE

(State or Other Jurisdiction
of Incorporation)

**60 Binney Street,
Cambridge, MA**

(Address of Principal Executive Offices)

001-35966

(Commission File Number)

13-3680878

(IRS Employer
Identification No.)

02142

(Zip Code)

Registrant's Telephone Number, Including Area Code: (339) 499-9300

Not Applicable

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 7.01 Regulation FD Disclosure

On June 5, 2017, bluebird bio, Inc. (“bluebird”) will be conducting meetings with investors attending the American Society of Clinical Oncology Annual Meeting. As part of these meetings, bluebird will deliver the slide presentation furnished to this report as Exhibit 99.1 and which is incorporated by reference herein.

See Item 8.01 below, which is incorporated by reference herein.

The information in Item 7.01 of this Report on Form 8-K and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events

On June 5, 2017, bluebird issued a press release announcing updated clinical data from its anti-BCMA CAR T cell therapy, being presented at the American Society of Clinical Oncology Annual Meeting on June 5, 2017. The full text of bluebird’s press release regarding the announcement is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	Investor presentation provided by bluebird bio, Inc. on June 5, 2017
99.2	Press release issued by bluebird bio, Inc. on June 5, 2017

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: June 5, 2017

bluebird bio, Inc.

By: /s/ Jason F. Cole

Jason F. Cole

Chief Legal Officer

EXHIBIT INDEX

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bluebird**bio**[®]

bb2121 Investor & Analyst Event

June 5, 2017

CONFIDENTIAL

NASDAQ: BLUE

Forward Looking Statements

These slides and the accompanying oral presentation contain forward-looking statements and information relating to bluebird bio, its product candidate bb2121 and oncology research and development plans. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward-looking statements. For example, all statements we make regarding the initiation, timing, progress and results of our preclinical and clinical studies and our research and development programs, our ability to advance product candidates into, and successfully complete, clinical studies, and the timing or likelihood of regulatory filings and approvals are forward looking. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our most recent quarterly report on Form 10-Q, as well as our subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Agenda

Introduction	Manisha Pai, Nick Leschly
bluebird Oncology Franchise	Philip Gregory, D.Phil.
CRB-401 Study Design and Clinical Overview	M. Travis Quigley
CRB-401 Data	Jesus Berdeja, M.D.
Closing	David Davidson, M.D.
Q&A	bluebird bio management Jesus Berdeja, M.D., principal investigator Michael Pehl, president, hematology & oncology, Celgene



Welcome

Nick Leschly, chief bluebird



Our Vision: Make Hope a Reality



OUR PATIENTS



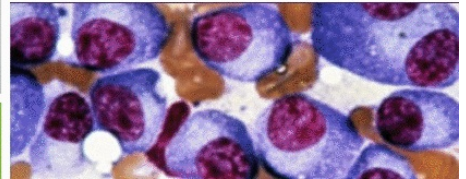
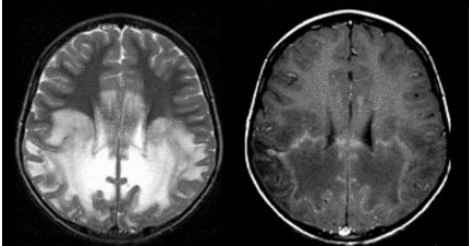
BLUE MOJO



TRUE BLUE



OUR PEOPLE



Advancing Multiple Programs in Parallel



Our Strategic Intent



bluebird Oncology Franchise

Philip Gregory, D.Phil., chief scientific officer



Pipeline

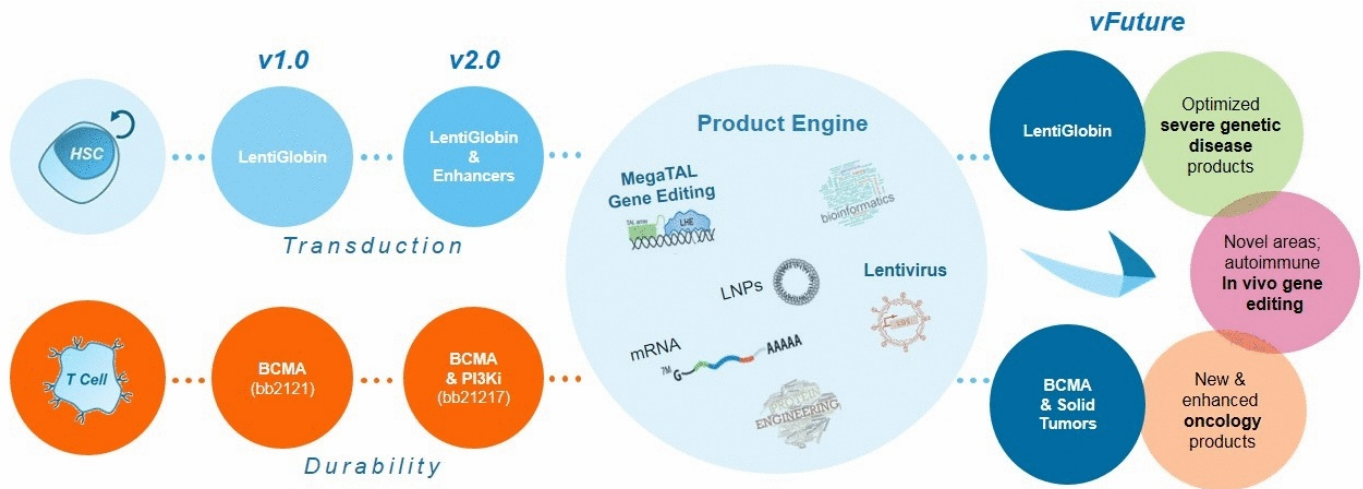
Product Candidates	Program Area	Preclinical	Phase 1/2	Phase 2/3	Rights/Partner
	CNS Diseases				
Lenti-D™ Drug Product	Cerebral ALD				Worldwide
	Rare Hemoglobinopathies				
LentiGlobin® Drug Product	Transfusion-Dependent β-thalassemia				Worldwide
	Severe Sickle Cell Disease				Worldwide
	BCMA				
bb2121	Multiple Myeloma				Celgene
bb21217	Multiple Myeloma				Celgene
	Oncology				
HPV-16 E6 TCR	HPV-associated Cancers				Kite Pharma
Viromed Target	Undisclosed				Worldwide excluding Korea
Medigene Targets	Undisclosed				Worldwide
	Research				
Early Pipeline	Undisclosed + Gene Editing				Worldwide

COLLABORATORS



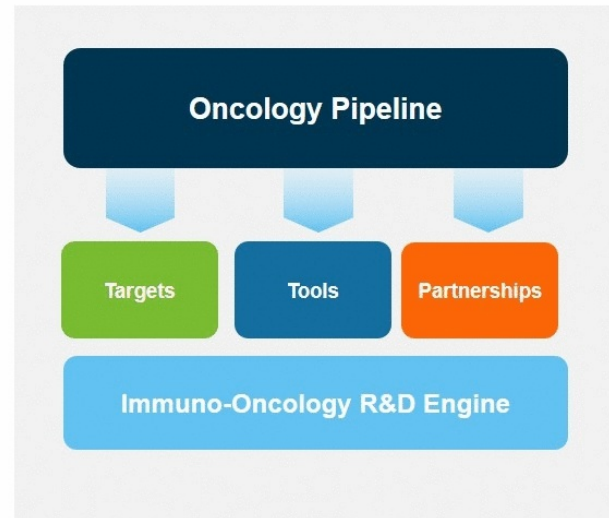
NASDAQ: BLUE

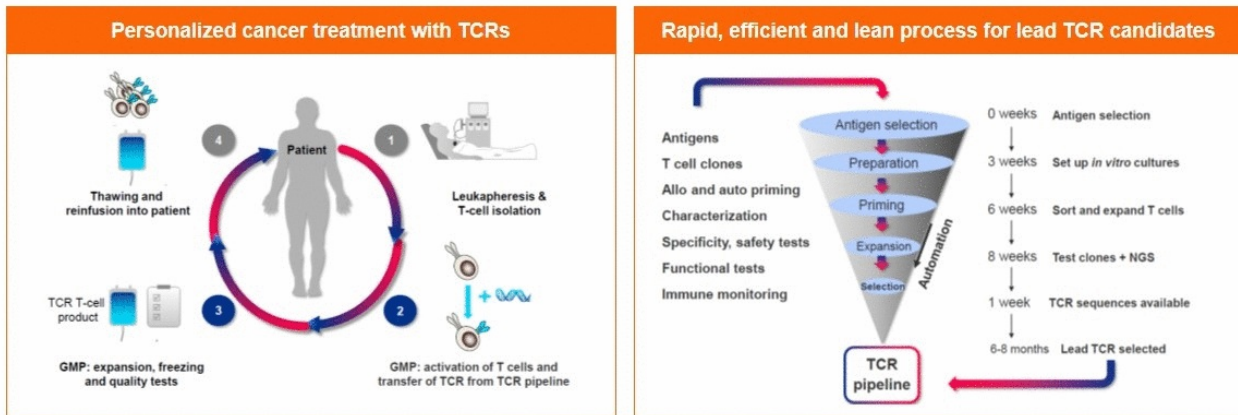
Good is Never Good Enough for Patients: bluebird Toolbox Strategy



Building a Translational Oncology Products Company: The Right Approach for the Right Targets

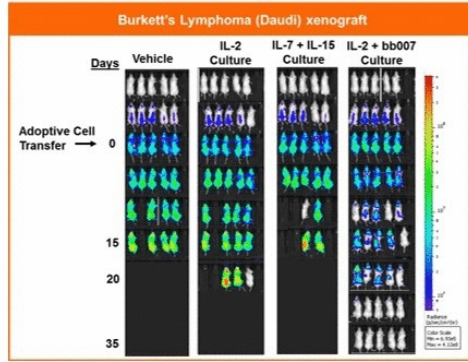
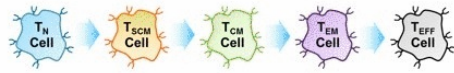
- Access to targets: both CARs and TCRs
- Optimization: gene editing; manufacturing enhancements; on/off switches; product and technology combinations
- Internal focus on bluebird strengths coupled with academic and industry collaborations to benefit from outside strengths
- Building a broad and diversified pipeline





- TCR technology provides T cell redirection to intracellular antigens not addressable by CARs
- Medigene partnership provides bluebird access to TCR therapeutic candidates against four targets

Example 1: bb21217 – making younger T cells



Example 2: megaTALs – driving site specific CAR insertion

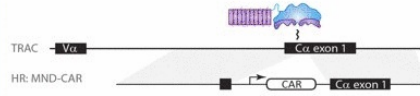
Molecular Therapy
Methods & Clinical Development
Original Article



Homology-Directed Recombination for Enhanced Engineering of Chimeric Antigen Receptor T Cells

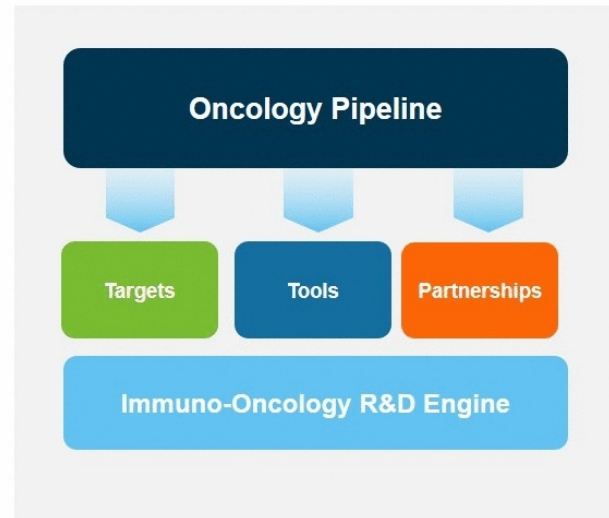
Malika Hale,^{1,2} Baekseung Lee,^{1,2} Yuchi Homaker,^{1,2,3} Wai-Hang Leung,^{1,2} Alexandra E. Grier,^{1,2} Holly M. Jacobs,¹ Karen Sommer,^{1,2} Jaya Sahni,^{1,2} Shaun W. Jackson,^{1,2} Andrew M. Scharnberg,^{1,2,4,5} Alexander Astrakhan,¹ and David J. Rawlings^{1,2,4,5}

¹Center for Immunology and Immunotherapy, Seattle Children's Research Institute, Seattle, WA 98101, USA; ²Program for Cell and Gene Therapy, Seattle Children's Research Institute, Seattle, WA 98101, USA; ³Medford Inc, Seattle, WA 98101, USA; ⁴Department of Pediatrics, University of Washington, Seattle, WA 98101, USA; ⁵Department of Immunology, University of Washington, Seattle, WA 98101, USA



Building a Translational Oncology Products Company: The Right Approach for the Right Targets

- Access to targets: both CARs and TCRs
- Optimization: gene editing; manufacturing enhancements; on/off switches; product and technology combinations
- Internal focus on bluebird strengths coupled with academic and industry collaborations to benefit from outside strengths
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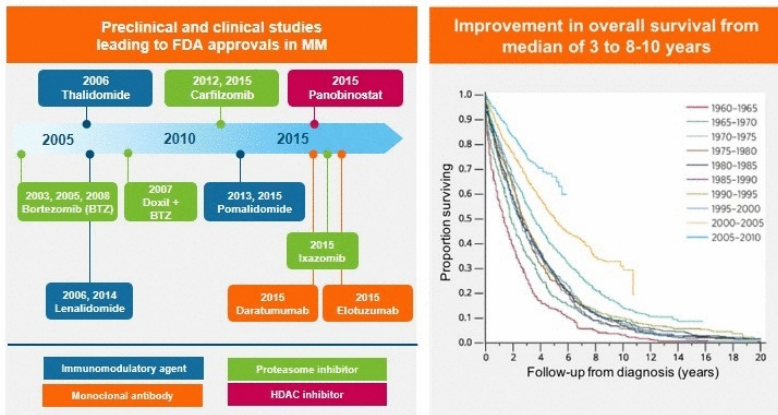
CRB-401 Study Design and Clinical Overview

M. Travis Quigley

BCMA program leader & senior director, clinical development



Despite Progress in Multiple Myeloma, There Remains a Need for New Therapies



CCR Focus

AAGR

Kenneth C. Anderson *Clin Cancer Res* 2016;22:5419-5427

Clinical efficacy of daratumumab monotherapy in patients with heavily pretreated relapsed or refractory multiple myeloma

Saad Z. Usmani,¹ Brendan M. Weiss,² Torben Plesner,³ Nizar J. Bahis,⁴ Andrew Belch,⁵ Sagar Lonial,⁶ Henk M. Lokhorst,⁷ Peter M. Voorhees,⁸ Paul G. Richardson,⁹ Ajai Chari,¹⁰ A. Kate Sasser,¹¹ Amy Axel,¹¹ Huaibao Feng,¹² Clarissa M. Uhlar,¹¹ Jianping Wang,¹¹ Imran Khan,¹² Tahamtan Ahmadi,¹¹ and Hareth Nah¹³

“Despite the availability of these classes of drugs for the treatment of MM, a recent analysis of patients with relapsed and refractory MM (RRMM) who were double refractory to a PI and an IMiD or had relapsed after ≥ 3 prior lines of therapy, including the novel agents pomalidomide (third-generation IMiD) and carfilzomib (second-generation PI), showed a median overall survival (OS) of 8 months.”

Usmani, *Blood* 2016

Current U.S. Standard of Care in 3rd/4th Line Multiple Myeloma

Current U.S. Standards of Care		
	Pomalyst and dex. (Pomalyst Product Monograph)	Daratumumab (Lancet 2016, Lonial, S)
N	452	106
Inclusion Criteria	<ul style="list-style-type: none"> • ≥2 prior therapies (including REVLIMID and bortezomib) • Relapsed and refractory multiple myeloma • Disease progression on or within 60 days of last therapy 	<ul style="list-style-type: none"> • Previously treated with at least three lines of therapy (including proteasome inhibitors and immunomodulatory drugs), or were refractory to both proteasome inhibitors and immunomodulatory drugs
Prior Tx	5 (2-14)	5 (2-14)
CR Rate (%)	<1%	~3%
ORR (%)	23.5%	29%
PFS (mos)	3.6 months	3.7 months

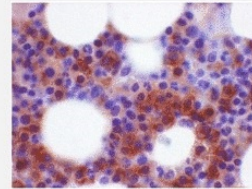
- The existing SOC outcomes for 3rd/4th line are limited in efficacy and durability leaving a need for new options for patients in need of better results
- Deep MRD negative responses are desirable in earlier lines of therapy and have demonstrated a benefit in long term outcomes

BCMA – A Promising Target in Multiple Myeloma

Despite the availability of various approved therapies, including proteasome inhibitors, IMiDs and, more recently, anti-CD38 antibodies, multiple myeloma remains an incurable disease.

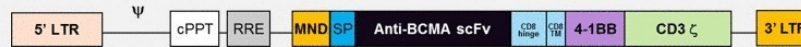
B-Cell Maturation Antigen (BCMA)

- BCMA is member of the TNF receptor superfamily expressed nearly universally on multiple myeloma cells with expression largely restricted to plasma cells and some mature B cells
- Initial proof of anti-BCMA activity has been demonstrated using T cells transduced with a gamma-retroviral vector encoding an anti-BCMA CAR with a CD28 costimulatory domain, but significant cytokine release syndrome occurred in patients with high disease burden (*Ali et al., Blood 2016*)

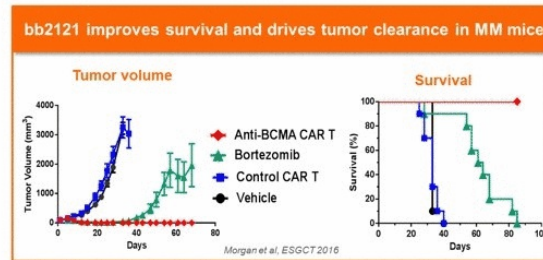
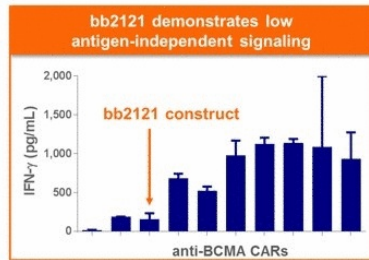


Multiple myeloma cells
expressing BCMA
(brown color = BCMA protein)

bb2121: Anti-BCMA Chimeric Antigen Receptor T Cell Product Candidate



- bb2121 is a second-generation CAR construct targeting BCMA, consisting of autologous T cells transduced with a lentiviral vector encoding a novel CAR incorporating an anti-BCMA scFv, a 4-1BB costimulatory motif to promote proliferation and persistence, and a CD3-zeta T cell activation domain
- Construct demonstrated potent preclinical *in vivo* activity with low tonic signaling



CRB-401 Phase 1 Study in Relapsed / Refractory Multiple Myeloma

CRB-401 Open-label Phase 1 Clinical Study of bb2121

- Objectives: Determine preliminary safety and efficacy and recommended phase 2 dose
- N = 50 patients, standard 3 + 3 dose escalation + expansion cohort
- Eligibility:
 - Relapsed / refractory MM with ≥ 3 prior lines of therapy (including PI and IMiD), or double refractory
 - Measurable disease
 - $\geq 50\%$ BCMA expression

9 U.S. Clinical Sites, 1 Centralized Manufacturing Site



CRB-401 Data

Jesus Berdeja, M.D.

Sarah Cannon Research Institute & Tennessee Oncology

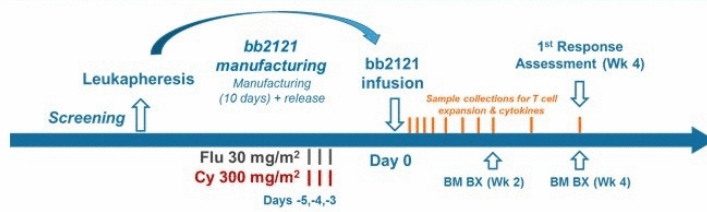


First-in-Human Multicenter Study of bb2121 anti-BCMA CAR T Cell Therapy for Relapsed/Refractory Multiple Myeloma: Updated Results

Jesus G. Berdeja, MD¹, Yi Lin, MD, PhD², Noopur Raje, MD³, Nikhil Munshi, MD⁴, David Siegel, MD, PhD⁵, Michaela Liedtke, MD⁶, Sundar Jagannath, MD⁷, Marcela Maus, MD, PhD³, Ashley Turka⁸, Lyh Ping Lam⁸, Kristen Hege, MD⁹, Richard Morgan, PhD⁸, M. Travis Quigley⁸, and James N. Kochenderfer, MD¹⁰

1-Sarah Cannon Research Institute and Tennessee Oncology, Nashville, TN; 2-Mayo Clinic, Rochester, MN; 3-Massachusetts General Hospital Cancer Center, Boston, MA; 4-Dana Farber Cancer Institute, Boston, MA; 5-Hackensack University Medical Center, Hackensack, NJ; 6-Stanford University Medical Center, Palo Alto, CA; 7-Mt. Sinai Medical Center, New York, NY; 8-bluebird bio, Inc., Cambridge, MA; 9-Celgene, San Francisco, CA; 10-Experimental Transplantation and Immunology Branch, National Cancer Institute/National Institutes of Health, Bethesda, MD

CRB-401 Open-label Phase 1 Clinical Study of bb2121

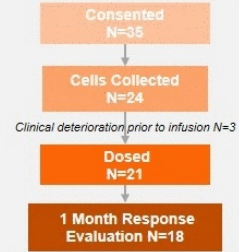


3 + 3 Dose Escalation of CAR + T Cells



- CRB-401 is a phase 1 dose-escalation and dose response study in relapsed / refractory MM
- Objectives: Determine preliminary safety and efficacy and recommended phase 2 dose
- 50 patients planned, standard 3 + 3 dose escalation followed by expansion cohort
- Key eligibility criteria
 - Relapsed / refractory MM with ≥ 3 prior lines of therapy (including PI and IMiD), or double refractory
 - Measurable disease
 - $\geq 50\%$ BCMA expression by IHC
 - Adequate bone marrow (ANC $\geq 1,000$, platelet count $\geq 50,000$), adequate renal and hepatic function

Study Status



* bb2121 Successfully manufactured for all patients collected

Baseline Demographics, Clinical Characteristics and Treatment History

21 patients have received bb2121 as of the data cut-off of May 4, 2017. Median follow-up is 15.4 weeks (range 1.4 to 54.4).

Demographics and Clinical Characteristics		
Parameter	Statistic	N=21 Dosed Patients
Age years	Median (range)	58 (37-74)
Male gender	N (%)	13 (62%)
Time since diagnosis (years)	Median (range)	5 (1-16)
ECOG ¹ = 0	N (%)	10 (48%)
ISS ² Stage		
I	N (%)	6 (29%)
II		11 (52%)
III		4 (19%)
High-risk cytogenetics (del17p, t(4;14), t(14;16), 1q, del 13)	N (%)	14 (67%)

¹ ECOG: Eastern Cooperative Oncology Group Performance Score

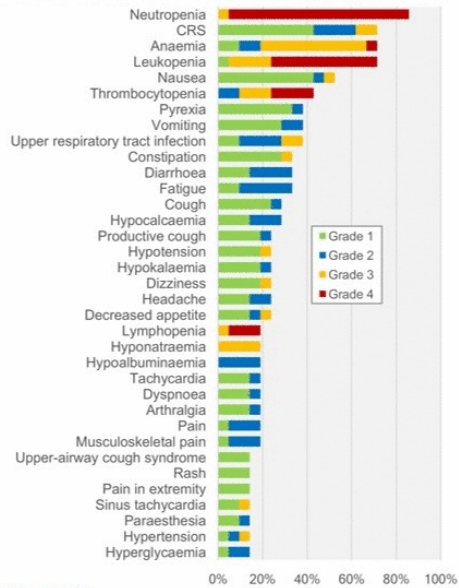
² ISS: International Staging System

³ SCT: Stem Cell Transplant

MM Treatment History		
Parameter	Statistic	N=21 Dosed Patients
Prior lines of therapy	Median (range)	7 (3-14)
Prior autologous SCT ³	N (%)	21 (100%)
Prior Therapies	Exposed	Refractory
Bortezomib	100%	67%
Carfilzomib	91%	57%
Lenalidomide	100%	86%
Pomalidomide	91%	71%
Daratumumab	71%	48%
Cumulative Exposure	Exposed	Refractory
Bort / Len	100%	67%
Bort / Len / Car	91%	48%
Bort / Len / Pom	91%	57%
Bort / Len / Car / Pom	86%	43%
Bort / Len / Car / Pom / Dara	71%	29%

bb2121 Generally Well Tolerated

Treatment-Emergent Adverse Events in >2 Patients (N=21 Patients Dosed)



• No dose-limiting toxicities (DLTs) observed as of data cut-off

• Cytopenias related to Cy/Flu lymphodepletion

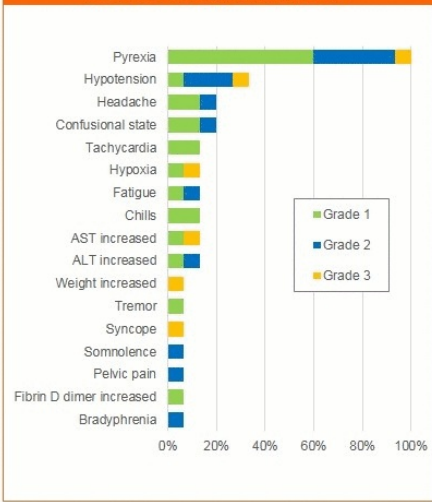
• 1 unrelated death due to cardio pulmonary arrest in a patient with an extensive cardiac history, the event occurred over 4 months after bb2121 infusion. The patient had achieved a stringent CR at 1 month and remained in remission at time of event

• 11 patients experienced 1 or more SAEs. SAEs occurring in more than 1 patient were CRS* Grade 1-2 that required hospitalization per protocol (N=4) and pyrexia (N=2)

*CRS uniformly graded according to Lee et al., Blood 2014;124:188-195

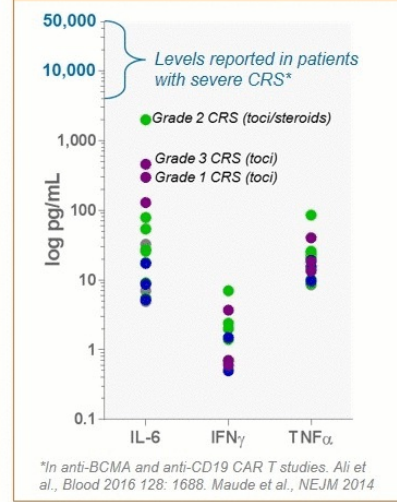
Cytokine Release Syndrome Readily Manageable

Reported CRS-Related Symptoms
In 15/21 treated patients with CRS

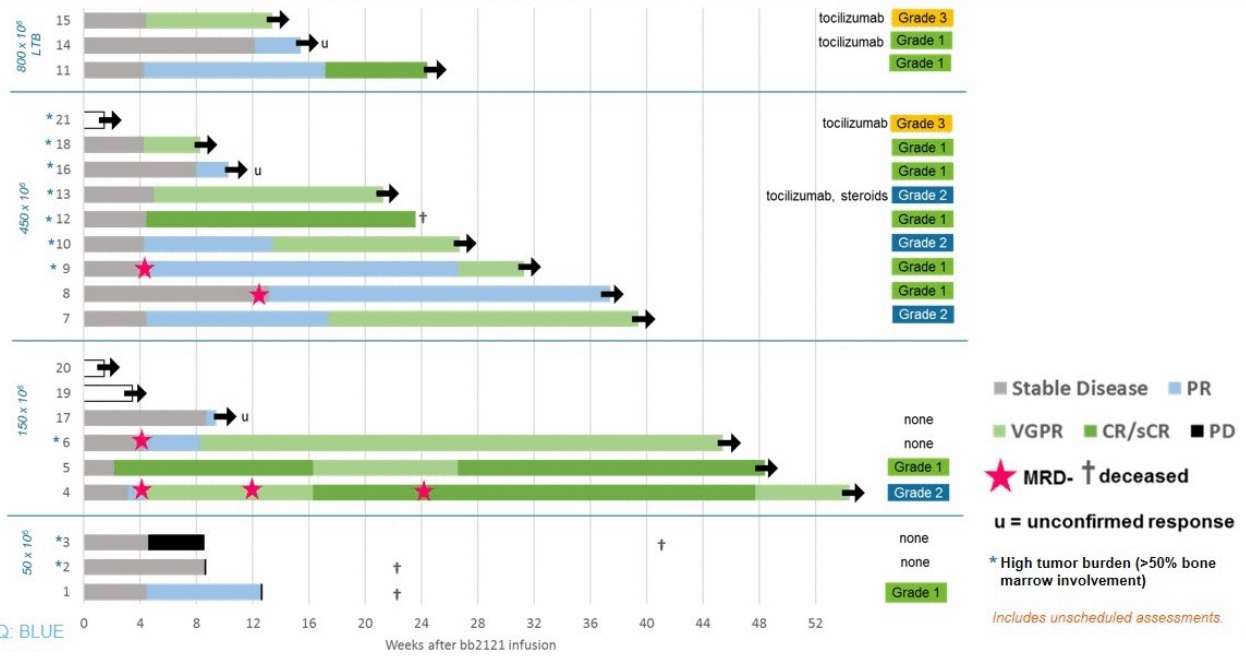


- **15/21 (71%) with cytokine release syndrome (CRS)**
 - 2 patients with Grade 3 CRS that resolved in 24 hours
 - 4 patients received tocilizumab, 1 (Grade 2 CRS) with steroids
 - CRS grade does not appear related to tumor burden
- **CRS-related symptoms mostly Grade 1-2**
- **No Grade 3/4 neurotoxicity**

Peak Cytokine Levels



All Patients in Active Dose Cohorts Achieved an Objective Response, Duration up to 54 Weeks



NASDAQ: BLUE

Clinical Response: Time to Response and MRD

Response Rates and Timing

Efficacy Parameter	% (95% CI)
ORR all doses	89% (65-99)
ORR (> 50 x 10 ⁶ CAR+ cells)	100% (78.2-100)
≥VGPR (> 50 x 10 ⁶ CAR+ cells)	73%
CR rate (> 50 x 10 ⁶ CAR+ cells)	27%
	<i>Median (range)</i>
Time to First Response (days)	31 (15-92)
Time to Best Response (days)	59.5 (15-186)
Duration of Response (days, as of data cut-off)	134+ (7-361)

ORR: overall response rate among patients evaluable for clinical response

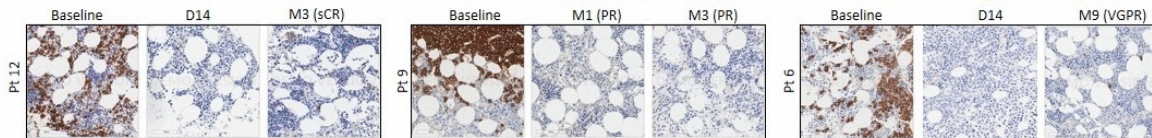
Assessment of Minimal Residual Disease (MRD)[†]

Patient ID	Month 1	Month 3	Month 6
4	Negative 10⁻⁵	Negative 10⁻⁴ (10 ⁻⁵ undetermined) (10 ⁻⁶ undetermined)	Negative 10⁻⁴ & 10⁻⁵ Positive 10 ⁻⁶
6	Negative 10⁻⁵	<i>failed QC</i>	N/A
8	N/A	Negative 10⁻⁴ and 10⁻⁵ (10 ⁻⁶ undetermined)	N/A
9	Negative 10⁻⁴ & 10⁻⁵ (10 ⁻⁶ undetermined)	Negative at 10⁻⁴ & 10⁻⁵ (10 ⁻⁶ undetermined)	N/A

^{*}Pt 5 and Pt 7 had no clone identified at baseline; Pt 10 Month 1 failed QC
[†]MRD assessed using next-gen sequencing immunoSEQ, Adaptive, Inc.

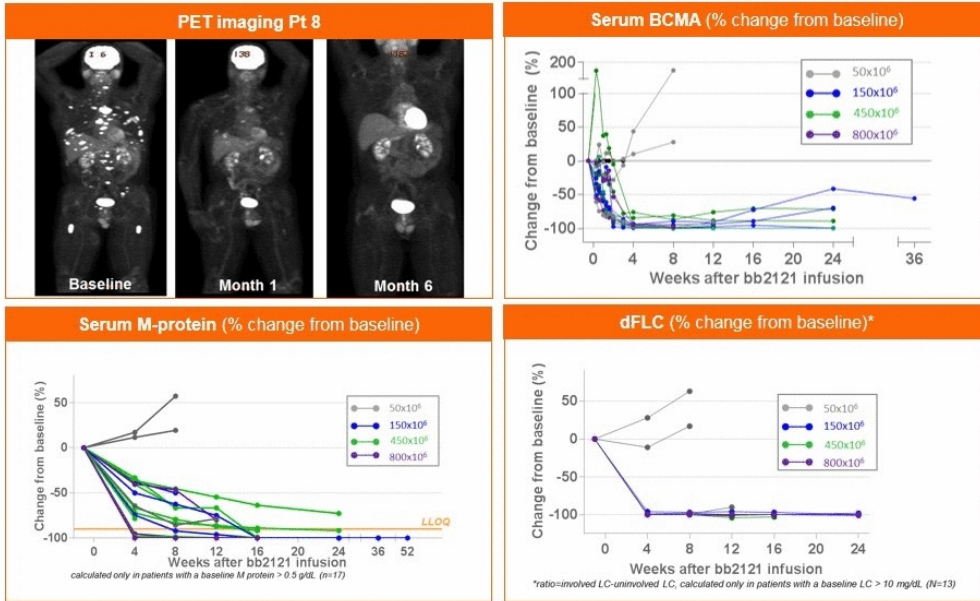
- Durable responses in all evaluable subjects at doses > 50 x 10⁶ CAR+ cells
- 4 of 4 evaluable patients are MRD negative at 10⁻⁵ sensitivity level

Clearance of Myeloma in the Bone Marrow by IHC (CD138+ cells) as Early as Day 14

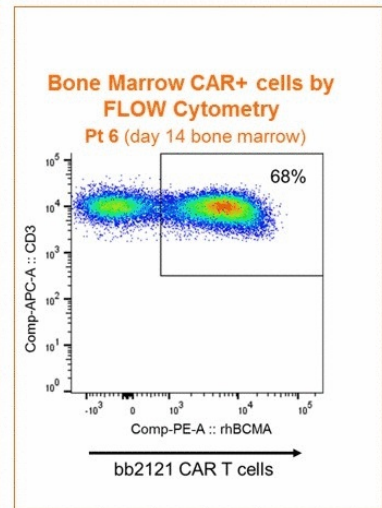
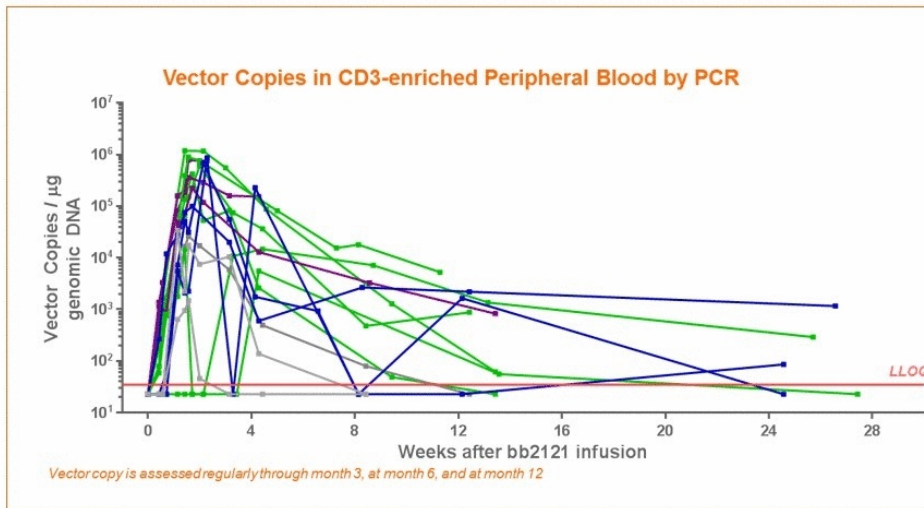


NASDAQ: BLUE

Tumor Response Kinetics: Rapid Clearance of PET Uptake, sBCMA and sFLC; Slower Clearance of M-protein




Persistence of CAR T Cells in Peripheral Blood up to 24 Weeks



Summary

- **bb2121 has induced durable and deepening responses in a heavily pre-treated population with relapsed/refractory multiple myeloma, including:**
 - 100% ORR, 73% VGPR or better, 27% CR (at doses $> 50 \times 10^6$)
 - MRD negative results in all evaluable patients (N=4)
 - No disease progression in patients treated with doses $> 50 \times 10^6$, with 1 patient past 1 year and 8 patients past 6 months
- **To date, the safety profile of bb2121 has been manageable through doses as high as 800×10^6**
 - The 2 reported events of grade 3 CRS resolved within 24 hours
 - No grade 3/4 neurotoxicity reported
- **These results will inform identification of the dose(s) to bring forward into the expansion phase of the study in future development**



Closing

David Davidson, M.D., chief medical officer

Key Takeaways

As of May 4 Data Cut-off

Patient Population

- ✓ Heavily pre-treated; all had prior auto transplant, median of 7 prior lines of therapy

Safety Profile

- ✓ No DLTs; toxicity readily manageable, CRS largely grade 1/2, two Grade 3 CRS resolved within 24 hours with tocilizumab

Responses

- ✓ 100% response rate in active dose cohorts (above 50×10^6)
 - ✓ 27% complete response rate
 - ✓ 73% VGPR or better
 - ✓ All patients tested for MRD status (N=4) were found to be MRD-negative

Durability

- ✓ No patients with clinical progression in active dose cohorts (above 50×10^6)



Q&A

bluebirdbio

Nick Leschly, chief bluebird

David Davidson, M.D., chief medical officer

Philip Gregory, D.Phil., chief scientific officer

M. Travis Quigley, BCMA program leader & senior director, clinical development

Jesus Berdeja, M.D., Sarah Cannon Research Institute & Tennessee Oncology

Michael Pehl, president, hematology & oncology, Celgene



Exhibit 99.2

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 American Society of Clinical Oncology (ASCO) Annual Meeting

- *100% of the 15 evaluable patients in active dose cohorts (doses above 50 x 10⁶) 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. and Summit, N.J., June 5, 2017 – [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.

“It is impressive to see objective responses in all patients treated at dose levels of 150 x 10⁶ 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 10⁶, 150 x 10⁶, 450 x 10⁶ and 800 x 10⁶ 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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