

**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): December 10, 2017**

**bluebird bio, Inc.**

(Exact name of Registrant as Specified in Its Charter)

**DELAWARE**

(State or Other Jurisdiction  
of Incorporation)

**001-35966**

(Commission File Number)

**13-3680878**

(IRS Employer  
Identification No.)

**60 Binney Street,  
Cambridge, MA**  
(Address of Principal Executive Offices)

**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 8.01 Other Events**

On December 10, 2017, bluebird bio, Inc. (“bluebird”) issued a press release announcing data presented at the 58<sup>th</sup> Annual Meeting of the American Society of Hematology (“ASH”) from the CRB-401 clinical study of the bb2121 product candidate in patients with relapsed/refractory multiple myeloma.

Also on December 10, 2017, bluebird issued a press release announcing data presented at ASH from the Northstar (HGB-204) and Northstar-2 (HGB-207) clinical studies of the LentiGlobin product candidate in patients with transfusion-dependent beta-thalassemia, and a press release announcing data presented at ASH from the HGB-206 clinical study of the LentiGlobin product candidate in patients with severe sickle cell disease.

On December 11, 2017, bluebird issued a press release announcing data presented at ASH from the HGB-205 clinical study of the LentiGlobin product candidate in patients with severe sickle cell disease and transfusion-dependent beta-thalassemia.

The full text of bluebird’s press releases regarding these announcements are filed as Exhibits 99.1, 99.2, 99.3, and 99.4 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	<a href="#">Press release issued by bluebird bio, Inc. on December 10, 2017 regarding data from the CRB-401 clinical study</a>
99.2	<a href="#">Press release issued by bluebird bio, Inc. on December 10, 2017 regarding data from the HGB-204 and HGB-207 clinical studies</a>
99.3	<a href="#">Press release issued by bluebird bio, Inc. on December 10, 2017 regarding data from the HGB-206 clinical study</a>
99.4	<a href="#">Press release issued by bluebird bio, Inc. on December 11, 2017 regarding data from the HGB-205 clinical study</a>

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**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: December 11, 2017

**bluebird bio, Inc.**

By: /s/ Jason F. Cole

Jason F. Cole

*Chief Legal Officer*



**Celgene Corporation and bluebird bio Announce Updated Results from Ongoing Multicenter Phase 1 Study of bb2121 Anti- BCMA CAR T Cell Therapy in Patients with Late Stage Relapsed/Refractory Multiple Myeloma at ASH Annual Meeting**

*Overall response rate (ORR) of 94% in patients in active dose cohorts (doses above  $50 \times 10^6$  CAR + T-cells)*

*Complete Response (CR) rate of 56% observed in patients in active dose cohorts*

*Median progression free survival (PFS) not reached with median follow up of 40 weeks in active dose cohorts*

*Nine of 10 (90%) of patients evaluable for minimal residual disease (MRD) status were found to be MRD-negative*

**Summit, N.J. and Cambridge, Mass., December 10, 2017** – Celgene Corporation (NASDAQ: CELG) and bluebird bio, Inc. (Nasdaq: BLUE) today announced that updated results from the ongoing CRB-401 Phase 1 clinical study of bb2121, an investigational anti-B-cell maturation antigen (BCMA) CAR T cell therapy, in 21 patients with late-stage relapsed/refractory multiple myeloma will be presented in an oral presentation at the American Society of Hematology (ASH) Annual Meeting in Atlanta, Georgia.

The objective of this Phase 1 dose-escalation study is to evaluate safety and efficacy of bb2121 and determine a recommended Phase 2 dose.

“Celgene has a longstanding commitment to patients with multiple myeloma through our extensive research efforts in this deadly blood cancer,” said Nadim Ahmed, President, Hematology and Oncology for Celgene. “Looking ahead, we see BCMA as an important target in this disease and we believe bb2121 has the potential to create significant impact on the treatment approach and outcomes for these patients.”

“The growing body of bb2121 clinical data are building a compelling story, further supporting the importance of the therapy’s unique features,” said Dave Davidson, M.D., chief medical officer, bluebird bio. “The responses achieved in this relapsed and refractory patient population, combined with the generally tolerable safety profile, reinforce the potential role of bb2121 as a groundbreaking CAR T therapy in multiple myeloma.”

**Durable clinical responses in heavily pretreated patients with relapsed/refractory multiple myeloma: Updated results from a multicenter study of bb2121 anti-BCMA CAR T cell therapy (Abstract #740)**

**Presenter:** James Kochenderfer, M.D., the Center for Cancer Research at the National Cancer Institute in Bethesda, Maryland

**Date:** Monday, December 11, 3:00 pm (Oral presentation)

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**Location:** Hall C1 (Georgia World Congress Center)

**Session Title:** Myeloma: Therapy, excluding Transplantation I

The open-label Phase 1 CRB-401 study (NCT02658929) is evaluating the preliminary safety and efficacy of bb2121 anti-BCMA CAR T cell in patients with relapsed and/or refractory multiple myeloma. The study also evaluated the recommended dose of bb2121 for future studies.

Patients on study were heavily pre-treated, with a median of 7 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 October 2, 2017 data cut-off, 21 patients had been enrolled and dosed in the dose-escalation phase of the study, in four dose cohorts: 50 x 10<sup>6</sup>, 150 x 10<sup>6</sup>, 450 x 10<sup>6</sup> and 800 x 10<sup>6</sup> CAR+ T cells. This multi-center study has enrolled patients at nine sites in the U.S with central manufacturing performed at Celgene.

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 proprietary lentiviral vector encoding the anti-BCMA CAR.

**Results in the active dose cohorts (150 x 10<sup>6</sup>, 450 x 10<sup>6</sup> and 800 x 10<sup>6</sup> CAR+ T cells; N=18) were:**

- Median follow-up was 40 weeks (range: 6.6-69)
- 17/18 (94%) patients achieved an objective response
- 16/18 (89%) patients achieved at least a very good partial response (VGPR)
- 10/18 (56%) patients achieved a complete response (CR, N = 7), or unconfirmed complete response (N = 3)
- 9 of 10 patients who were evaluable for MRD status were found to be MRD-negative
- Median PFS has not been reached in the active dose cohorts. The PFS at 6 months and 9 months was 81% and 71%, respectively.
- Three patients in the dose-escalation who responded to therapy subsequently experienced disease progression.

In the dose-escalation phase, 15/21 (71%) of patients had cytokine release syndrome (CRS), mostly Grade 1 & 2, with 2 patients experiencing Grade 3 CRS (9%). Four patients received tocilizumab, 1 (Grade 2 CRS) received steroids and in each case the CRS resolved within 24 hours. The most common treatment-emergent Grade 3-4 AEs in 21 infused patients were cytopenias commonly associated with lymphodepleting chemotherapy including neutropenia (86%), anemia (57%) and thrombocytopenia (43%). There were two deaths in the active cohorts at 22 and 69 weeks following infusion, respectively. The first was due to cardiac arrest and the second was due to myelodysplastic syndrome; both subjects were in a myeloma CR at their last

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study assessment prior to death. Based on the findings during dose escalation, a dose expansion phase of 12 subjects has started testing doses between 150-450 x 10<sup>6</sup> CAR+ T cells. In the dose expansion phase, one patient treated at the 450 x 10<sup>6</sup> CAR+ T cells dose experienced Grade 4 neurotoxicity including focal cerebral edema and subarachnoid hemorrhage. This patient had a high tumor burden, and a history of subarachnoid hemorrhage. The event was successfully managed, and the patient remains in the response group. No other Grade 3/4 neurotoxicity was observed in the escalation or expansion cohort.

“To see these types of responses after one treatment with bb2121 in a heavily pre-treated patient population is very promising, and we are hopeful that CAR T therapy with bb2121 may become an important therapy in the fight against multiple myeloma, which remains an insidious and incurable disease,” said James Kochenderfer, M.D., the Center for Cancer Research at the National Cancer Institute in Bethesda, Maryland and a primary investigator in the study.”

bb2121 is an investigational compound that is not approved for any use in any country. bb2121 recently received Breakthrough Therapy Designation from the U.S. FDA and PRIME eligibility from the EMA. Celgene has also sponsored an open-label, single-arm phase 2 study (KarMMA), which is open to recruitment, to evaluate bb2121 further in patients with relapsed/refractory multiple myeloma. (NCT03361748)

### **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](#), [Pinterest](#), [LinkedIn](#), [Facebook](#) and [YouTube](#).

### **About bluebird bio, Inc.**

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 five clinical studies for the treatment of transfusion-dependent β-thalassemia, also known as β-thalassemia major, 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 programs, bb2121 and bb21217, are anti-BCMA CAR T programs partnered with Celgene. bb2121 and bb21217 are each currently being studied in Phase 1 trials for the treatment of relapsed/refractory multiple myeloma. bluebird bio also has discovery research programs utilizing megaTALs/homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

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

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LentiGlobin and Lenti-D are trademarks of bluebird bio, Inc.

## **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.*

*Hyperlinks are provided as a convenience and for informational purposes only. Neither Celgene nor bluebird bio bears responsibility for the security or content of external websites or websites outside of their respective control.*

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For bluebird bio  
Investors & Media  
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**bluebird bio Presents New Data from Clinical Studies of LentiGlobin™ Gene Therapy in Transfusion-Dependent  $\beta$ -Thalassemia at American Society of Hematology Annual Meeting**

– Majority of patients in the Phase 3 Northstar-2 (HGB-207) study show higher *in vivo* vector copy numbers and hemoglobin levels; 5/6 patients producing >6g HbA<sub>T87Q</sub> at 3 months –

– 90% patients with non- $\beta^0/\beta^0$  genotypes free of transfusions in Phase 1/2 Northstar (HGB-204) study after up to three-year follow up, demonstrating durable treatment effect in TDT –

– Company to hold webcast today,  
December 10, at 8:30 p.m. ET –

**Atlanta, GA, December 10, 2017** – bluebird bio, Inc. (Nasdaq: BLUE), a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic diseases and T cell-based immunotherapies for cancer, today announced data from two studies of its LentiGlobin gene therapy product candidate in patients with transfusion-dependent  $\beta$ -thalassemia (TDT). Data from the Northstar (HGB-204) and Northstar-2 (HGB-207) studies were presented today at the 59<sup>th</sup> Annual Meeting of the American Society of Hematology (ASH) by Janet Kwiatkowski, M.D., MSCE, of Children’s Hospital of Philadelphia, and Mark C. Walters, M.D., of UCSF Benioff Children’s Hospital, respectively.

“Addressing the underlying genetic cause of TDT to restore production of functional hemoglobin can potentially eliminate or reduce the need for chronic blood transfusions in people with this disease, which we expect will reduce the risk of iron overload and associated long-term complications of TDT, and may allow cessation of chelation therapy,” said Dave Davidson, chief medical officer, bluebird bio. “Northstar-2 is the first clinical trial to use our refined manufacturing process for LentiGlobin drug product. Early data from this study demonstrates consistently higher *in vivo* vector copy numbers and HbA<sub>T87Q</sub> hemoglobin levels, potentially enabling patients to consistently achieve near-normal or normal total hemoglobin levels. It is important to demonstrate the long-term benefit of gene therapy, and follow-up data of up to three years from the first Northstar study show that nearly all patients with non- $\beta^0/\beta^0$  genotypes were transfusion-free. We are engaged with the regulatory authorities in the context of the Breakthrough Designation from FDA, and PRIME and Adaptive Pathways from EMA, and look forward to submitting these data to seek marketing approval for LentiGlobin in TDT.”

“People with transfusion-dependent thalassemia need regular blood transfusions to survive, but chronic transfusions lead to unavoidable iron overload that can result in

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multi-organ damage and shortened life span. Eliminating or reducing the need for transfusions can reduce the risk of these long-term complications,” said Janet L. Kwiatkowski, MD, MSCE, Director of the Thalassemia Program at the Children’s Hospital of Philadelphia and Associate Professor of Pediatrics at the Perelman School of Medicine of the University of Pennsylvania, and a primary investigator of the Northstar and Northstar-2 studies. “The growing body of data from the Northstar studies indicate LentiGlobin gene therapy may enable transfusion independence for the majority of patients with non- $\beta^0/\beta^0$  genotypes – and that this effect has been durable during the 3 years of follow-up.”

**Clinical Outcomes up to 3 Years Following LentiGlobin Gene Therapy for Transfusion-Dependent  $\beta$ -Thalassemia in the Northstar HGB-204 Study (Oral Abstract #360)**

**Presenter:** Janet Kwiatkowski, M.D., MSCE, Children’s Hospital of Philadelphia, Philadelphia, PA

**Date and Time:** Sunday, December 10 at 10:45 a.m.

**Location:** Building B, Level 2, B213-B214

The Northstar study is an open-label, single-dose, international, multi-center Phase 1/2 study designed to evaluate the efficacy and safety of LentiGlobin for the treatment of patients with TDT. The study has completed its treatment phase and 18 patients with TDT (eight with  $\beta^0/\beta^0$  and 10 with non- $\beta^0/\beta^0$  genotypes) received LentiGlobin drug product (DP). Results as of September 21, 2017 include:

- All 18 patients have  $\geq 18$  months follow up, with 10 completing two-year analysis. Three patients have three years of follow up (median follow-up: 27.4 months; min-max: 17.5-36.5 months).
  - Nine of ten patients with non- $\beta^0/\beta^0$  genotypes were free from chronic transfusions for a median of 29 months (range: 14.7-33.1 months).
  - Patients with non- $\beta^0/\beta^0$  genotypes who were able to achieve freedom from chronic transfusions had HbA<sup>T87Q</sup> concentrations of 3.6-9.3.
  - The one patient with a non- $\beta^0/\beta^0$  genotype who still required periodic transfusions was treated with LentiGlobin with a VCN in the lower range (VCN: 0.3 copies/diploid genome).
  - Two of eight patients with  $\beta^0/\beta^0$  genotypes have not received a transfusion in more than a year (16.7 months and 15.7 months). At the patients’ last study visits (Month 36 and Month 18, respectively), total hemoglobin levels were 10.2 and 10.3 g/dL and HbA<sup>T87Q</sup> levels were 9.7 and 7.0 g/dL, respectively.
  - Clinically meaningful reductions in transfusion volume and frequency were observed in five of the six patients with  $\beta^0/\beta^0$  genotypes who have continued to receive transfusions.
  - For the 18 study participants, the median DP vector copy number (VCN) was 0.7 (range: 0.3-1.5) copies/diploid genome, the median cell dose was 8.1 (range: 5.2-18.1)  $\times 10^6$  CD34+ cells/kg, and the proportion of transduced CD34+ cells was 17-58 percent.
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- The safety profile of LentiGlobin DP continues to be consistent with myeloablative conditioning with single-agent busulfan. No Grade 3 or higher DP-related adverse events (AEs) have been observed, and there is no evidence of clonal dominance.
- All study participants remain enrolled in the trial, and there have been no reports of graft versus host disease (GVHD).

**Results from the HGB-207 (Northstar-2) Trial: A Phase 3 Study to Evaluate Safety and Efficacy of LentiGlobin Gene Therapy for Transfusion-Dependent  $\beta$ -thalassemia (TDT) in Patients with non- $\beta^0/\beta^0$  Genotypes (Oral Abstract #526)**

**Presenter:** Mark C. Walters, M.D., UCSF Benioff Children's Hospital, Oakland, Calif.

**Date and Time:** Sunday, December 10 at 5:15 p.m.

**Location:** Building C, Level 1, C101 Auditorium

The Northstar-2 study is an ongoing, open-label, single-dose, international, multicenter Phase 3 study designed to evaluate the efficacy and safety of LentiGlobin for the treatment of patients with TDT and non- $\beta^0/\beta^0$  genotypes. As of December 1, 2017, drug product had been manufactured for 10 patients. The median LentiGlobin DP VCN these patients received was 3.3 (range: 2.4-5.4) copies/diploid genome) compared to a median DP VCN of 0.7 (range: 0.3-1.5) copies/diploid genome in the Phase 1/2 Northstar study. Results in treated patients, ages 15 to 24 years, include:

- Seven patients had been infused with LentiGlobin as of October 13, 2017. The median follow-up was 3 months (range: 1-9 months).
- All three patients who have  $\geq 6$  months follow-up are transfusion-free, and 2/3 have achieved or are approaching a normal total hemoglobin level (up to 12.5 g/dl total Hb; range in three patients: 8.4 – 12.5) without transfusions (up to 10.2 g/dL vector-derived HbAT87Q).
- Five of six patients treated in the study with  $\geq 3$  months follow-up data available as of December 1, 2017 are making at least 6 g/dL of HbAT87Q.
- The safety profile of LentiGlobin to date is similar to that observed in the Northstar study, and consistent with myeloablative conditioning with single-agent busulfan. No DP-related AEs have been observed.
- All study participants remain enrolled in the trial, and there have been no reports of graft failure or graft versus host disease (GVHD).

**Webcast Information**

bluebird bio will host a webcast at 8:30 p.m. ET on Sunday, December 10, 2017. The webcast can be accessed under "Calendar of Events" in the Investors and Media section of the company's website at [www.bluebirdbio.com](http://www.bluebirdbio.com).

**About TDT**

Transfusion-dependent  $\beta$ -thalassemia (TDT) is a severe genetic disease characterized by reduced or absent hemoglobin levels that results in severe anemia and ineffective red blood cell production. Supportive care for people with TDT consists of a lifelong regimen of chronic blood transfusions to enable survival and suppress symptoms of the disease,

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and iron chelation therapy to manage iron overload that results from the transfusions. Despite the availability of supportive care, many people with TDT experience serious complications and organ damage due to underlying disease and iron overload.

Allogeneic hematopoietic stem cell transplant (HSCT) is currently the only available option to address the underlying genetic cause of TDT, though it carries significant risks. Complications of allogeneic HSCT include a risk of treatment-related mortality, graft failure, graft-versus-host disease (GvHD) and opportunistic infections, particularly in patients who undergo non-sibling matched allogeneic HSCT.

#### **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 five clinical studies for the treatment of transfusion-dependent  $\beta$ -thalassemia, also known as  $\beta$ -thalassemia major, 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 programs, bb2121 and bb21217, are anti-BCMA CAR T programs partnered with Celgene. bb2121 and bb21217 are each currently being studied in Phase 1 trials for the treatment of relapsed/refractory multiple myeloma. bluebird bio also has discovery research programs utilizing megaTALs/homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

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

LentiGlobin and Lenti-D are trademarks of bluebird bio, Inc.

#### **Forward-Looking Statements**

*This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the Company's research, development, manufacturing and regulatory approval plans for its LentiGlobin product candidate to treat transfusion-dependent  $\beta$ -thalassemia, including statements whether the manufacturing process changes for LentiGlobin will improve outcomes of patients with transfusion-dependent  $\beta$ -thalassemia. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risks that the preliminary positive efficacy and safety results from our prior and ongoing clinical trials of*

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*LentiGlobin will not continue or be repeated in our ongoing, planned or expanded clinical trials of LentiGlobin, risks that the current or planned clinical trials of LentiGlobin will be insufficient to support regulatory submissions or marketing approval in the US and EU, the risk of a delay in the enrollment of patients in our clinical studies, and the risk that any one or more of our product candidates, including our bb2121 product candidate, will not be successfully developed, approved or commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled “Risk Factors” in our most recent Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and bluebird bio undertakes no duty to update this information unless required by law.*

**Contact:**

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Investors & Media

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**bluebird bio Announces Updated Clinical Results from Ongoing Phase 1 Multicenter Study of LentiGlobin™ Gene Therapy in Severe Sickle Cell Disease at American Society of Hematology (ASH) Annual Meeting**

- Promising early results from two patients treated under amended study protocol and with refined manufacturing process show 51% and 28% anti-sickling HbA<sup>T87Q</sup> at six and nine months, respectively, exceeding levels seen previously in the HGB-206 study –*
- Plerixafor mobilization and apheresis cell collection for LentiGlobin manufacture now implemented in the study, first patient treated with this method had a peripheral VCN of 2.5 copies/diploid genome at month 1 –*
- Company to hold webcast today, December 10, 8:30 p.m. ET –*

**Atlanta, GA, December 10, 2017** – [bluebird bio, Inc.](#) (Nasdaq: BLUE), a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic diseases and T cell-based immunotherapies for cancer, today announced that updated clinical results from HGB-206, the company’s ongoing Phase 1 multicenter study of its LentiGlobin gene therapy product candidate in patients with severe sickle cell disease (SCD), will be discussed in an oral presentation during the 59th Annual Meeting of the American Society of Hematology (ASH). In addition, a poster on the feasibility and potential benefits of plerixafor-mediated peripheral blood stem cell collection and drug product (DP) manufacturing in patients with SCD was presented yesterday at ASH.

“The promising early results from the first two patients treated under the amended HGB-206 study protocol indicate that the manufacturing and patient management changes we implemented may have a meaningful impact on patient outcomes,” said Dave Davidson, chief medical officer, bluebird bio. “These two patients have maintained higher levels of gene-marked cells in the blood following treatment compared to the previous patients in HGB-206. This improvement corresponds with increased production of the anti-sickling hemoglobin, HbA<sup>T87Q</sup>, made from LentiGlobin. We are hopeful that this high-level expression of HbA<sup>T87Q</sup> will lead to a sustained clinical benefit for these patients. The next group of patients in the study will be treated using LentiGlobin made from stem cells obtained from plerixafor-mobilized peripheral blood. Plerixafor mobilization in place of direct bone marrow harvest is less burdensome for patients, and our results suggest that this approach may be able to obtain a greater quantity of higher quality cells.”

**Interim Results from a Phase 1/2 Clinical Study of LentiGlobin Gene Therapy for Severe Sickle Cell Disease (Oral Abstract #527)**

**Presenter:** Julie Kanter, M.D., Medical University of South Carolina, Charleston, SC

**Date & Time:** Sunday, December 10 at 5:30 p.m.

**Location:** Bldg C, Level 1, C101 Auditorium

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“People with sickle cell disease have a genetic disease that causes the protein in red blood cells, called hemoglobin, to be misshapen. As a result of this abnormal hemoglobin, many affected individuals live with low blood counts and severe, recurrent pain crises that lead to organ damage and shortened life spans,” said Dr. Kanter. “It is also a disease that has been historically under-researched and under-resourced, with few treatment options beyond pain management. These early results with the revised study protocol indicate that gene therapy with LentiGlobin may allow people with SCD to produce substantial levels of normal, anti-sickling, adult hemoglobin. We are hopeful about the possibility that this could substantially reduce the painful and damaging crises that are a hallmark of this disease, potentially allowing patients to live longer, healthier lives.”

HGB-206 is an ongoing, open-label study designed to evaluate the safety and efficacy of LentiGlobin DP in the treatment of adults with severe SCD. Patients in this study are divided into three cohorts: A, B and C. Patients in Group A were treated under the original study protocol. Patients in Group B were treated under an amended study protocol that included changes intended to increase DP vector copy number (VCN) and improve engraftment of gene-modified stem cells. Patients in both Group A and B had DP made from stem cells collected using bone marrow harvest. Patients in Group C are also treated under the amended study protocol, but receive LentiGlobin made from stem cells collected from peripheral blood after mobilization with plerixafor rather than via bone marrow harvest. As of November 30, 2017, ten patients had been treated in the study and follow-up data were available on nine patients from groups A and B, with a median of 21 (6-27) months since transplantation. Key results include:

	Group A N=7 Median (min-max)	Group B N=2	
		Patient 1312	Patient 1313
Transduced CD34+ cells (%)	25 (8-42)	95 <sup>1</sup> , 90 <sup>1</sup>	46, 83 <sup>1</sup>
Drug product Cell Dose (x10 <sup>6</sup> CD34+ cells)	2.1 (1.6-5.1)	3.2	2.2
Drug product VCN (copies per diploid genome)	0.6 (0.3-1.3)	2.9 <sup>1</sup> , 5.0 <sup>1</sup>	1.4, 3.3 <sup>1</sup>
VCN in peripheral blood (copies per diploid genome at last measurement)	0.1 (0.1-0.2)	2.5 (M6)	0.5 (M9)
HbAT87Q (g/dL at last measurement)	0.7 (0.5-2.0)	6.4 (M6)	3.0 (M9)
HbAT87Q (% of total, at last measurement)	7.9 (5.3-18.2)	51% (M6)	28% (M9)

<sup>1</sup> LentiGlobin DP manufactured using refined process

- Both patients in Group B were treated with two DP lots. Information from each of these LentiGlobin DP lots is reflected in the chart above.
  - Patient 1313 received LentiGlobin manufactured using a combination of the original and the refined manufacturing processes.
  - Patient 1312 received LentiGlobin manufactured entirely using the refined manufacturing process.
- LentiGlobin DP has been manufactured for four patients in Group C:
  - Median transduced CD34+ cells: 80%
  - Median DP cell dose: 6.9 x10<sup>6</sup> CD34+ cells
  - Median DP VCN (copies per diploid genome): 3.3
- The first patient treated with LentiGlobin (Group C) made using plerixafor-mobilized stem cells had a VCN in peripheral blood of 2.5 at one month.
- The toxicity profile observed from drug product infusion to latest follow-up was generally consistent with myeloablative conditioning with single-agent busulfan.

**Successful Plerixafor-Mediated Mobilization, Apheresis, and Lentiviral Vector Transduction of Hematopoietic Stem Cells in Patients with Severe Sickle Cell Disease (Poster Abstract #990)**

**Presenter:** John Tisdale, M.D., National Heart, Lung and Blood Institute (NHLBI), Bethesda, MD

**Date & Time:** Saturday, December 9 at 5:30 p.m.

**Location:** Bldg A, Level 1, Hall A2

“Historically, harvesting stem cells from people with SCD required bone marrow harvest, a painful approach for obtaining cells that often yields a suboptimal dose level and cell quality,” said Dr. Tisdale. “The data we presented at ASH suggest that not only is this new approach using plerixafor mobilization generally tolerable for patients, but it may enable us to obtain a larger cell dose with a higher concentration of primitive stem cells. Cells with this primitive phenotype are more likely to become long-term sources of gene-modified red blood cells. We believe that providing more primitive hematopoietic stem cells that carry more copies of the gene therapy vector may be critical to realizing the full promise of gene therapy for people with SCD, and we look forward to getting more data on this new cohort of patients in the coming months.”

**Results as of November 30, 2017:**

	Bone Marrow Harvest	Plerixafor
Number of Patients	9 (26 BMHs)	7 (10 mobilization cycles)



Adverse Events	17 Grade 3 AEs following BMH in 5 patients, 4 were SAEs (1 procedural pain, 3 SCD pain crisis)	5 Grade 3 events included 2 non-serious (hypomagnesemia and non-cardiac chest pain) and 3 SAEs (1 patient each) of SCD pain crisis
CD34+ cells collected per harvest, median (min-max) cells/kg	5.0 (0.3-10.8) x 10 <sup>6</sup>	10.4 (5.1-20.0) x 10 <sup>6</sup>

#### Webcast Information

bluebird bio will host a webcast at 8:30 p.m. ET today, December 10, 2017. The webcast can be accessed under "Calendar of Events" in the Investors and Media section of the company's website at [www.bluebirdbio.com](http://www.bluebirdbio.com).

#### About SCD

Sickle cell disease (SCD) is an inherited disease caused by a mutation in the beta-globin gene, that produces  $\beta^S$ -globin. High levels of Hb<sup>S</sup> in patients with SCD are responsible for the characteristic chronic anemia, vaso-occlusive crises, and other acute and chronic manifestations of SCD which lead to significant morbidity and early mortality.

Where adequate medical care is available, common treatments for patients with SCD largely revolve around prevention of infection and management and prevention of acute sickling episodes. Chronic management may include hydroxyurea and, in certain cases, chronic transfusions. Allogeneic hematopoietic stem cell transplant (HSCT) is currently the only available option to address the underlying genetic cause of SCD, though it carries significant risk. Complications of allogeneic HSCT include a risk of treatment-related mortality, graft failure, graft versus host disease (GvHD) and opportunistic infections, particularly in patients who undergo non-sibling-matched allogeneic HSCT.

#### 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 five clinical studies for the treatment of transfusion-dependent  $\beta$ -thalassemia, also known as  $\beta$ -thalassemia major, 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 programs, bb2121 and bb21217, are anti-BCMA CAR T programs partnered with Celgene. bb2121 and bb21217 are each currently being studied in Phase 1 trials for the treatment of relapsed/refractory multiple myeloma. bluebird bio also has discovery research programs utilizing megaTALs/homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

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



LentiGlobin and Lenti-D are trademarks of bluebird bio, Inc.

### **Forward-Looking Statements**

*This release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements whether the manufacturing process changes for LentiGlobin will improve outcomes of patients with transfusion-dependent  $\beta$ -thalassemia and severe sickle cell disease, whether the planned changes to the HGB-206 clinical trial protocol, including plerixafor mobilization, will improve outcomes in patients with severe sickle cell disease. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risks that the preliminary positive efficacy and safety results from our prior and ongoing clinical trials of LentiGlobin will not continue or be repeated in our ongoing, planned or expanded clinical trials of LentiGlobin, the risks that the changes we have made in the LentiGlobin manufacturing process or the HGB-206 clinical trial protocol will not result in improved patient outcomes, risks that the current or planned clinical trials of LentiGlobin will be insufficient to support regulatory submissions or marketing approval in the US and EU, the risk of a delay in the enrollment of patients in our clinical studies, and the risk that any one or more of our product candidates, including our bb2121 product candidate, will not be successfully developed, approved or commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled “Risk Factors” in our most recent Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and bluebird bio undertakes no duty to update this information unless required by law.*

### **Contact:**

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**bluebird bio Presents Updated Data from HGB-205 Study of LentiGlobin™ Gene Therapy in Patients with Severe Sickle Cell Disease and Transfusion-Dependent  $\beta$ -Thalassemia at American Society of Hematology Annual Meeting**

- Two of three patients with severe sickle cell disease show >45 percent total anti-sickling hemoglobin levels and significant clinical improvement; all three show rising trajectory of HbA<sup>T87Q</sup> production in first six months post-drug product infusion –*
- Ongoing transfusion independence up to 3.8 years in patients with transfusion-dependent  $\beta$ -thalassemia; three of four patients have normal or near-normal total hemoglobin levels –*
- Safety profile of LentiGlobin remains consistent with that of myeloablative conditioning with single-agent busulfan –*

**Atlanta, December 11, 2017** – bluebird bio, Inc. (Nasdaq: BLUE), a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic diseases and T cell-based immunotherapies for cancer, announced updated data from the ongoing HGB-205 clinical study of its LentiGlobin gene therapy product candidate in patients with severe sickle cell disease (SCD) and transfusion-dependent  $\beta$ -thalassemia (TDT). The findings will be presented today in a poster session at the 59<sup>th</sup> Annual Meeting of the American Society of Hematology (ASH).

“People with SCD and TDT experience serious complications and organ damage as a result of their disease and complications from chronic blood transfusions. Addressing the underlying genetic causes of these diseases has the potential to dramatically improve patient outcomes,” said Dave Davidson, M.D., chief medical officer, bluebird bio. “All three patients with severe SCD in the HGB-205 study showed a steady increase in HbA<sup>T87Q</sup> production in the first six months following LentiGlobin therapy, with the longest-treated patient showing stable hemoglobin levels over two and a half years. All four patients with TDT are transfusion-free following therapy, up to almost four years in the first patient treated. The durable treatment effects observed to date in this study are encouraging, particularly given the manufacturing process improvements that we implemented across our subsequent clinical studies of LentiGlobin, and additional changes to the HGB-206 study protocol that we hope will further improve outcomes for patients with SCD.”

These data will be presented by Marina Cavazzana, M.D., Ph.D., Professor of Medicine at Paris Descartes University and Research Director at the Centre for Clinical Research in Biotherapy, Necker Hospital, and at the Institute of Genetic Diseases, Imagine, Paris, France. Professor Cavazzana is the primary investigator of the HGB-205 study.

“All seven patients in this study continue to experience notable clinical improvement. Since being treated with LentiGlobin therapy, the four patients with TDT have been free of chronic transfusions with near normal and stable levels of total hemoglobin,” said Professor Cavazzana.

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“While progress has been made with medications to treat SCD and TDT, we are in need of better options for our patients. This study suggests that LentiGlobin has the potential to be a transformational one-time therapy for people with SCD and TDT.”

**Longer Term Follow-up on the First Patients with Severe Hemoglobinopathies Treated with LentiGlobin Gene Therapy (Poster Abstract #4609)**

**Presenter:** Marina Cavazzana, M.D., Ph.D. Necker-Enfants Malades Hospital, Paris, France

**Poster Session Date & Time:** Monday, December 11 at 6:00 p.m.

**Location:** Building A, Level 1, Hall A2

HGB-205 is an ongoing, open-label, single-center Phase 1/2 study designed to evaluate the safety and efficacy of LentiGlobin drug product (DP) in the treatment of patients with severe SCD and TDT. The study enrolled three patients with severe SCD and four patients with TDT, who have undergone infusion with LentiGlobin DP. Results as of September 20, 2017 include:

**SCD:**

- All three treated patients showed rising HbA<sup>T87Q</sup> levels in the first six months.
- Patient 1204 was 13 years old at study enrollment. At last follow-up (35.2 months), this patient had a total hemoglobin of 12.4 g/dL, of which 6.1 g/dL was HbA<sup>T87Q</sup> (52 percent anti-sickling Hb). HbA<sup>T87Q</sup> concentration in this patient has remained stable since approximately nine months post-infusion. The patient continues to show marked clinical improvement.
- Patient 1207 was 16 years old at study enrollment. At last follow-up (8.9 months), this patient had a total hemoglobin of 10.0 g/dl, of which 0.7 g/dl was HbA<sup>T87Q</sup> (14 percent anti-sickling Hb). This patient had a pre-treatment history of frequent episodes of vaso-occlusive crisis (VOC) and acute chest syndrome (ACS) despite hydroxyurea prior to beginning regular transfusions. Patient 1207 had episodes of ACS and hospitalization at six and eight months post-treatment, and received three transfusions.
- Patient 1208 was 21 years old at study enrollment. At last follow-up (6.0 months), this patient had a total hemoglobin of 10.6 g/dL, of which 2.7 g/dL was HbA<sup>T87Q</sup> (46 percent total anti-sickling Hb). This patient had a pre-treatment history of frequent episodes of VOCs and ACS prior to beginning regular transfusions, and was still symptomatic while receiving regular transfusions. Following LentiGlobin treatment, Patient 1208 has had no episodes of VOCs or ACS (with six months follow-up).

**TDT:**

- All four patients with TDT have remained free of chronic transfusions since shortly after receiving LentiGlobin DP.
- Patient 1201 ( $\beta^0/\beta^E$  genotype) has been free of transfusions for 45.2 months with total hemoglobin of 10.1 g/dL, of which 6.7 g/dL was HbA<sup>T87Q</sup>.
- Patient 1202 ( $\beta^0/\beta^E$  genotype) has been free of transfusions for 40.1 months with total hemoglobin of 12.9 g/dL, of which 10.1 g/dL was HbA<sup>T87Q</sup>.
- Patient 1206 ( $\beta^0/\beta^E$  genotype) has been free of transfusions for 23.8 months with total hemoglobin of 11.1 g/dL, of which 8.0 g/dL was HbA<sup>T87Q</sup>.

- Patient 1203, who is homozygous for the severe  $\beta^+$  mutation IVS1-110, has been free of transfusions for 20.9 months with total hemoglobin of 8.7 g/dL, of which 6.7 g/dL was HbA<sup>T87Q</sup>.
- Three of four patients (1201, 1202 and 1206) were able to begin therapeutic phlebotomy. Patient 1202 subsequently discontinued iron chelation and phlebotomy.
- The safety profile of LentiGlobin DP continues to be consistent with myeloablative conditioning with single-agent busulfan. No DP-related adverse events have been observed, and there is no evidence of clonal dominance.

### **About SCD**

Sickle cell disease (SCD) is an inherited disease caused by a mutation in the beta-globin gene, that produces  $\beta^S$ -globin. High levels of Hb<sup>S</sup> in patients with SCD are responsible for the characteristic chronic anemia, vaso-occlusive crises, and other acute and chronic manifestations of SCD which lead to significant morbidity and early mortality.

Where adequate medical care is available, common treatments for patients with SCD largely revolve around prevention of infection and management and prevention of acute sickling episodes. Chronic management may include hydroxyurea and, in certain cases, chronic transfusions. Allogeneic hematopoietic stem cell transplant (HSCT) is currently the only available option to address the underlying genetic cause of SCD, though it carries significant risk. Complications of allogeneic HSCT include a risk of treatment-related mortality, graft failure, graft-versus-host disease (GvHD) and opportunistic infections, particularly in patients who undergo non-sibling-matched allogeneic HSCT.

### **About TDT**

Transfusion-dependent  $\beta$ -thalassemia (TDT) is a severe genetic disease characterized by reduced or absent hemoglobin levels that results in severe anemia and ineffective red blood cell production. Supportive care for people with TDT consists of a lifelong regimen of chronic blood transfusions to enable survival and suppress symptoms of the disease, and iron chelation therapy to manage iron overload that results from the transfusions. Despite the availability of supportive care, many people with TDT experience serious complications and organ damage due to underlying disease and iron overload.

Allogeneic HSCT is currently the only available option to address the underlying genetic cause of TDT, though it carries significant risks. Complications of allogeneic HSCT include a risk of treatment-related mortality, graft failure, GvHD and opportunistic infections, particularly in patients who undergo non-sibling matched allogeneic HSCT.

### **About the HGB-205 Study**

HGB-205 is an ongoing, open-label Phase 1/2 study designed to evaluate the safety and efficacy of LentiGlobin in the treatment of subjects with TDT and SCD. The study enrolled seven subjects who will be followed to evaluate safety and transfusion requirements post-transplant. Among patients with sickle cell disease only, efficacy will also be measured based on the number of vaso-occlusive crises or acute chest syndrome events. For more information on the HGB-205 study, please visit [clinicaltrials.gov](https://clinicaltrials.gov) using identifier NCT02151526.

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## **Forward-Looking Statements**

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**Investors & Media**

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