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

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**FORM 8-K**

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**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 14, 2014**

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**bluebird bio, Inc.**

(Exact name of registrant as specified in its charter)

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**DELAWARE**  
(State or other jurisdiction  
of incorporation)

**001-35966**  
(Commission  
File Number)

**13-3680878**  
(I.R.S. Employer  
Identification No.)

**150 Second Street**  
**Cambridge, MA**  
(Address of principal executive offices)

**02141**  
(Zip Code)

**Registrant's telephone number, including area code (339) 499-9300**

**Not Applicable**  
(Former name or former address, if changed since last report)

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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:

- 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))
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**Item 7.01 Regulation FD Disclosure**

On June 16, 2014, bluebird bio, Inc. (“bluebird”) conducted an investor webcast summarizing clinical data from its HGB-205 clinical trial that was presented at an oral presentation at the 19<sup>th</sup> European Hematology Association Congress in Milan, Italy on June 14, 2014. A copy of the presentation is being furnished as Exhibit 99.2 to this Report on Form 8-K.

The information in Item 7.01 of this Report on Form 8-K and Exhibit 99.2 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 14, 2014, issued a press release announcing clinical data from its HGB-205 clinical trial at an oral presentation at the 19<sup>th</sup> European Hematology Association Congress in Milan, Italy on June 14, 2014. The full text of bluebird’s press release regarding the announcement is filed as Exhibit 99.1 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	Press release issued by bluebird bio, Inc. on June 14, 2014.
99.2	Investor presentation provided by bluebird bio, Inc. on June 16, 2014.

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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: June 16, 2014

**bluebird bio, Inc.**

By: /s/ Jason F. Cole

Jason Cole

*Senior Vice President, General Counsel*

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**EXHIBIT INDEX**

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99.1	Press release issued by bluebird bio, Inc. on June 14, 2014.
99.2	Investor presentation provided by bluebird bio, Inc. on June 16, 2014.



## NEWS RELEASE

**bluebird bio Reports Rapid Transfusion Independence in Beta-Thalassemia Major Patients Treated with its LentiGlobin Product Candidate**

*First two patients in the HGB-205 Study achieved transfusion independence within two weeks of an autologous transplant with bluebird's lentiviral gene therapy*

CAMBRIDGE, MA, June 14, 2014 – bluebird bio, Inc. (Nasdaq: BLUE), a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic and orphan diseases, today released initial positive clinical data from its HGB-205 clinical study of its LentiGlobin BB305 product candidate in beta-thalassemia major subjects at the 19<sup>th</sup> Annual Congress of the European Hematology Association (EHA) in Milan, Italy.

“We are gratified that the improvements we introduced into the BB305 lentiviral vector design and manufacturing process appear to have translated into clinical results that we believe support the potential for our LentiGlobin BB305 gene therapy to transform the lives of patients with beta-thalassemia major,” stated David Davidson, M.D., bluebird bio’s Chief Medical Officer. “We are encouraged by the early and high-level production of corrected beta<sup>AT87Q</sup>-globin and the rapid onset of transfusion independence in these initial subjects, as well as the absence of any gene therapy related adverse events. We look forward to providing additional data from this study and our ongoing multi-center Northstar Study later this year.”

The principal investigator of the HGB-205 Study, Marina Cavazzana, M.D delivered an oral presentation at the EHA Congress entitled “Improving gene therapy for beta-thalassemia major: initial results from Study HGB-205” on June 14, 2014 at 04:15 pm CET (10:15 am EDT). The presentation included data from the prior LG001 Clinical Study and the ongoing HGB-205 Study.

Summary of the clinical data presented at EHA were:

**LG001 Clinical Study**

- Clinical update provided on two subjects treated in the prior LG001 Study (subjects 3 and 4) using the prior lentiviral HPV569 product candidate
- Subject 3 remains blood transfusion independent 72 months after being transplanted with the lentiviral HPV569 product candidate
- Subjects 3 and 4 are producing 2.7 g/dL and 0.4 g/dL of therapeutic beta<sup>A-T87Q</sup>-globin post-transplant, respectively
- No drug product related adverse events were reported in the LG001 Study.

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## **HGB-205 Clinical Study**

- Clinical data were presented on two subjects (subjects 1 and 2), both with beta-thalassemia major and the Beta E/Beta 0 genotype who were treated using the new lentiviral vector BB305
- At 4.5 months following autologous transplant subject 1 had a total hemoglobin of 10.1 g/dL of which 6.6 g/dL was therapeutic beta<sup>AT87Q</sup>-globin, and at 2 months post-transplant subject 2 had a total hemoglobin of 11.6 g/dL of which 4.2 g/dL was beta<sup>AT87Q</sup>-globin
- Subjects 1 and 2 received their last blood transfusion on day 10 and 12, respectively, post-transplant and both subjects remain blood transfusion independent
- Vector copy number in the drug product for subjects 1 and 2 were 1.5 and 2.1, respectively; multiple times higher than the drug product vector copy numbers reported in the prior LG001 Study (VCN 0.6 and 0.3 for Subjects 3 and 4, respectively)
- No drug product related adverse events were reported, and the integration site analysis performed on subject 1 at the 3-month time point showed polyclonal reconstitution.

We anticipate reporting additional data from the HGB-205 Study and from our ongoing Northstar Study in late 2014.

## **Conference Call and Webcast**

bluebird bio will host a conference call at 8:00 am EDT on Monday, June 16, 2014 to discuss the initial results from its HGB-205 Study. Investors may listen to the webcast of the conference call live on the “Calendar of Events” section of bluebird bio’s website, [www.bluebirdbio.com](http://www.bluebirdbio.com). Alternatively, investors may listen to the call by dialing: (844) 825-4408 from locations in the U.S. and (315) 625-3227 from outside the U.S. The webcast replay will be available for at least 72 hours following the call.

## **About beta-thalassemia**

Beta-thalassemia major is a rare hereditary blood disorder caused by a genetic abnormality of the beta globin gene resulting in defective red blood cells. Symptoms of beta-thalassemia include severe anemia and splenomegaly. It is estimated that about 288,000 patients with beta-thalassemia are alive, of which an estimated 15,000 live in the United States and Europe. The majority of beta-thalassemia patients have beta-thalassemia major.

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

The phase 1/2 study is designed to evaluate the safety and efficacy of LentiGlobin BB305 drug product in the treatment of subjects with beta-thalassemia major and severe sickle cell disease. The study is designed to enroll up to seven subjects. Subjects will be followed to evaluate safety and blood transfusion requirements post-transplant. In sickle cell disease patients only, efficacy will also be measured based on the number of vaso-occlusive crises or acute chest syndrome events.

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For more information on the HGB-205 Study, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) using identifier [NCT02151526](https://clinicaltrials.gov/ct2/show/study/NCT02151526).

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

bluebird bio is a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic and orphan diseases. bluebird bio has two clinical-stage programs in development. The most advanced product candidate, Lenti-D, is in a recently-initiated phase 2/3 study, the Starbeam Study, for the treatment of childhood cerebral adrenoleukodystrophy (CCALD), a rare, hereditary neurological disorder affecting young boys. The next most advanced product candidate, LentiGlobin, is currently in two phase 1/2 studies, one in the US (the Northstar Study) and one in France (HGB-205), for the treatment of beta-thalassemia major. The phase 1/2 HGB-205 study also allows enrollment of patient(s) with sickle cell disease, and bluebird bio is planning a separate U.S. sickle cell disease trial (HGB-206).

bluebird bio also has an early-stage chimeric antigen receptor-modified T cell (CAR-T) program for oncology in collaboration with Celgene Corporation.

bluebird bio has operations in Cambridge, Massachusetts and Paris, France. For more information, please visit [www.bluebirdbio.com](http://www.bluebirdbio.com)

#### ***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 potential efficacy and safety of the Company’s LentiGlobin product candidate, the Company’s plans with respect to LentiGlobin and its other product candidates and anticipated clinical and business milestones and announcements for 2014. In addition it should be noted that the data for LentiGlobin announced from the HGB-205 study at the EHA Congress are preliminary in nature and the HGB-205 trial is not completed. These data may not continue for these subjects or be repeated or observed in ongoing or future studies involving our LentiGlobin product candidate, including the HGB-205 Study, the Northstar Study or the HGB-206 study in 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 risk that the preliminary results from our clinical trials will not continue or be repeated in our ongoing clinical trials, the risk that previously conducted studies involving similar product candidates will not be repeated or observed in ongoing or future studies involving current product candidates, the risk of cessation or delay of any of the ongoing or planned clinical studies and/or our development of our product candidates, the risk of a delay in the enrollment of patients in the Company’s clinical studies, the risk that our collaboration with Celgene will not continue or will not be successful, and the risk that any one or more of our product candidates will not be successfully developed and*

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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 quarterly report on 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.

**Availability of other information about bluebird bio**

Investors and others should note that we communicate with our investors and the public using our company website ([www.bluebirdbio.com](http://www.bluebirdbio.com)), our investor relations website (<http://www.bluebirdbio.com/investor-splash.html>), including but not limited to investor presentations and FAQs, Securities and Exchange Commission filings, press releases, public conference calls and webcasts. You can also connect with us on Twitter @bluebirdbio, [LinkedIn](#) or our [YouTube](#) channel. The information that we post on these channels and websites could be deemed to be material information. As a result, we encourage investors, the media, and others interested in bluebird bio to review the information that we post on these channels, including our investor relations website, on a regular basis. This list of channels may be updated from time to time on our investor relations website and may include other social media channels than the ones described above. The contents of our website or these channels, or any other website that may be accessed from our website or these channels, shall not be deemed incorporated by reference in any filing under the Securities Act of 1933.

**Investor Relations:**

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Dan Budwick  
Pure Communications, Inc.  
(973) 271-6085





# *Making Hope A Reality*

June 16, 2014

Nasdaq : BLUE

## Forward Looking Statement

These slides and the accompanying oral presentation contain forward-looking statements and information. 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. The data for LentiGlobin are preliminary in nature and the HGB-205 trial is not completed. These data may not continue for these subjects or be repeated or observed in ongoing or future studies involving our LentiGlobin product candidate, including the HGB-205 Study, the Northstar Study or the HB-206 Study in sickle cell disease. 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 the 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.

## Summary - Key Messages

- Potential for **one-time transformative** treatments for **severe genetic and orphan diseases**
- **Encouraging clinical data** in beta-thalassemia major patients
- **Promising proof of concept data** in CCALD patients
- **Industrialized platform** across people, production, development and deployment
- **Disruptive product** platform with broad product and deal potential
- **Industry leading team** and **culture** funded for success

# bluebird Pipeline

Products	Program Area	Preclinical	Phase I/II	Phase II/III	Rights
Lenti-D	CNS Diseases				
	Childhood Cerebral ALD – Starbeam Study*				Worldwide
LentiGlobin™	Hematologic Diseases				
	b-thalassemia/SCD (France) –HGB-205 Study**				Worldwide
	b-thalassemia (U.S.) –Northstar Study**				
	Sickle Cell Disease (U.S.) –HGB-206 Study				
CAR-T Cells	Oncology				
	Hematologic/Solid Tumors				Global Celgene Collaboration
Early Pipeline	Research				
	Undisclosed				Worldwide

\* The Phase II/III Starbeam Study is our first clinical study of our current Lenti-D viral vector and product candidate.

\*\* The Phase I/II HGB-205 and Northstar Studies are our first clinical studies of our current LentiGlobin viral vector and product candidate.



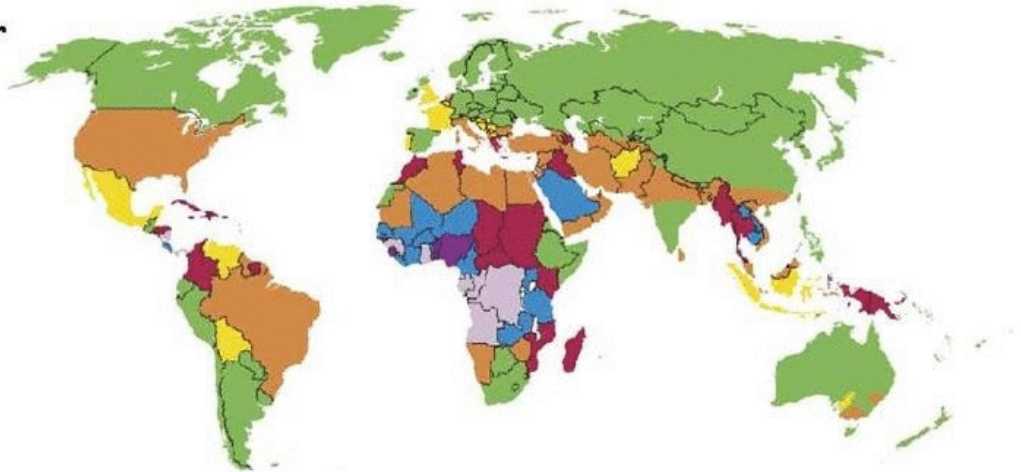
# **IMPROVING GENE THERAPY FOR $\beta$ -THALASSEMIA MAJOR: INITIAL RESULTS FROM STUDY HGB-205**

M. Cavazzana, JA Ribeil\*, E. Payen\*, F. Suarez, O. Negre, Y. Beuzard, F. Touzot, R. Cavallesco, F. Lefrere, S. Chretien, P. Bourget, F. Monpoux, C. Pondarre, B. Neven, F. Bushman, M. Schmidt, C. von Kalle, L. Sandler, S. Soni, B. Ryu, R. Kutner, G. Veres, M. Finer, S. Blanche, O. Hermine, S. Hacein-Bey-Abina, P. Leboulch

*\*these authors contributed equally*

# Epidemiology of hemoglobin disorders

Births with a pathological Hb disorder per 1,000 live births



The hemoglobinopathies are the most prevalent monogenetic disorders in the world – 7% of global population carry an abnormal hemoglobin gene

Between 300,000 – 400,00 babies are born each year with a serious hemoglobin disorder

- >40,000 with  $\beta$ -thalassemia major/HbE  $\beta$ -thalassemia
- >200,000 with sickle cell disease

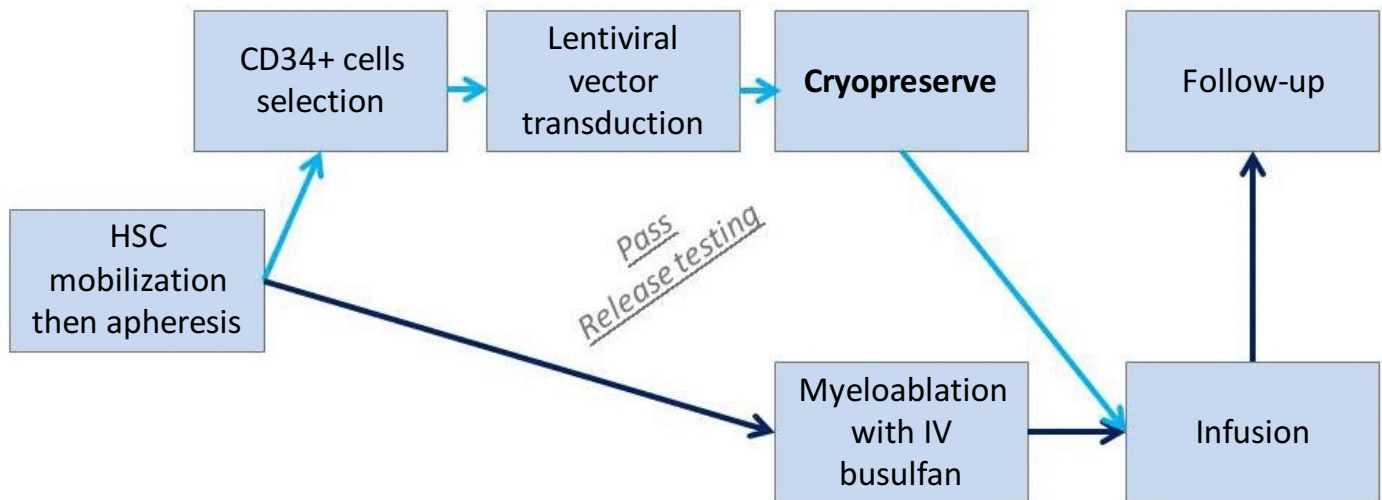
## ***β-thalassemia major is a serious disease potentially amenable to gene therapy***

- Only curative approach is allogeneic HSCT, but complicated by:
  - difficulty in finding well-matched donors
  - graft versus host disease
  - prolonged immunosuppression
- At EHA we report data from two clinical trials of *ex-vivo* gene therapy in subjects with β-thalassemia major

Study	Lentiviral vector	Current status
1 (LG001)	HPV569	Study closed, update presented today
2 (HGB-205)	BB305	Enrolling, initial results on first 2 subjects presented today



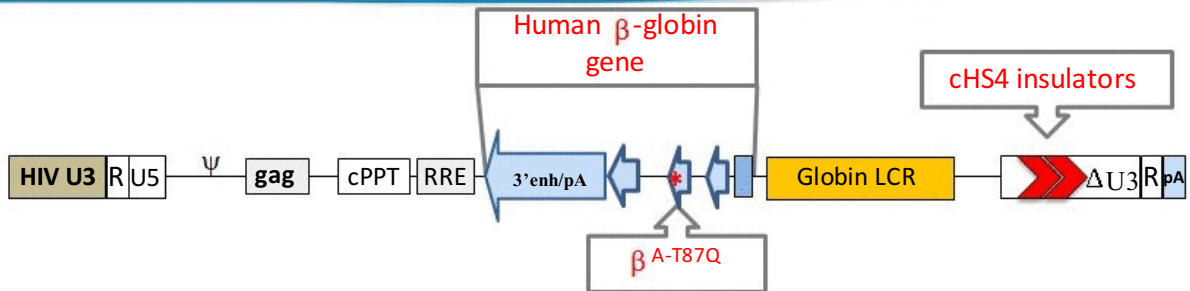
## Identical study design for both gene therapy trials



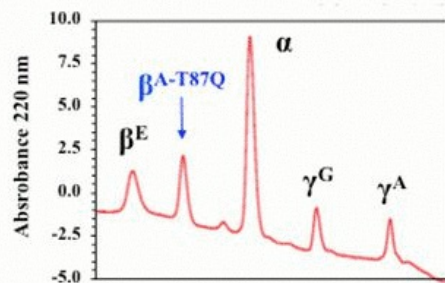
- Key eligibility:
- b-Thalassemia major (  $\geq 100$  mls pRBCs/kg/year)
  - Subjects with severe sickle cell disease are also eligible; none treated to date
- No HLA-matched sibling donor



# HPV569 lentiviral vector used in Study 1



- Globin production is under transcriptional control of an erythroid-specific promoter and enhancer



- $\beta^{A-T87Q}$ -globin allows for monitoring of protein levels produced using HPLC

## Study 1 – characteristics of included subjects

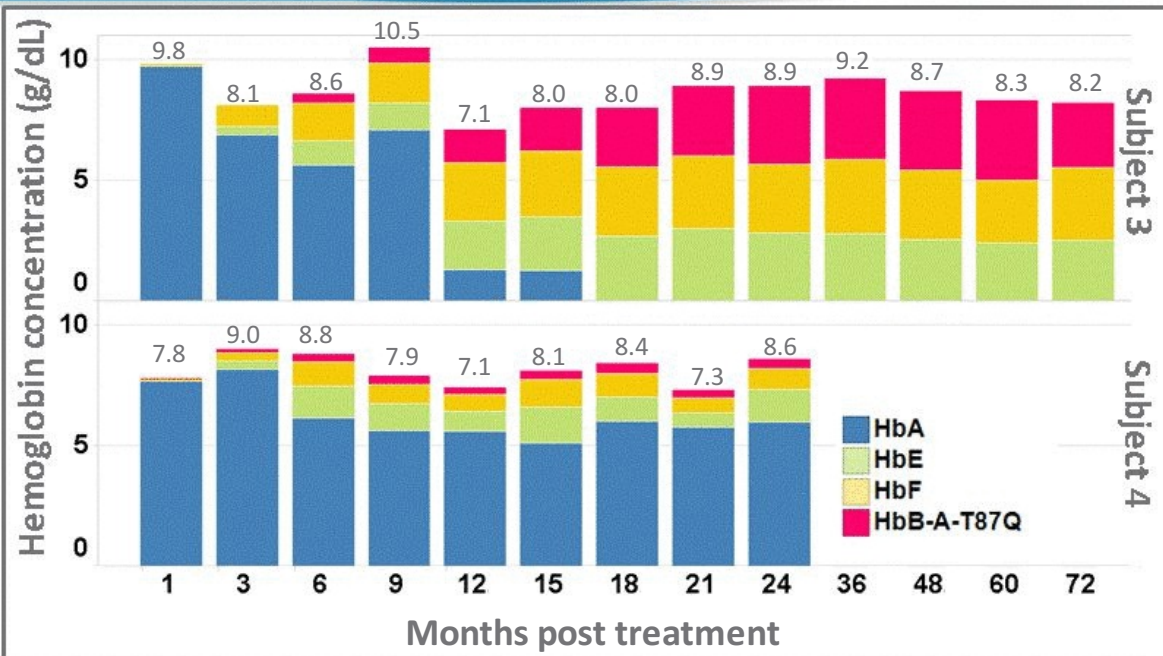
Subject	Outcome
1	Not treated
2	Low number of stem cells infused, no engraftment, received rescue cells
3	Engrafted, 6 years follow-up
4	Engrafted, 2 years follow-up

	Subject 3	Subject 4
Age	18	22
Genotype	b <sup>0</sup> /b <sup>E</sup>	b <sup>0</sup> /b <sup>E</sup>
CD34 <sup>+</sup> VCN	0.6	0.3
CD34 <sup>+</sup> cell dose (x 10 <sup>6</sup> /kg)	4.9*	4.3

\*Subject 3 source of CD34+ cells was bone marrow

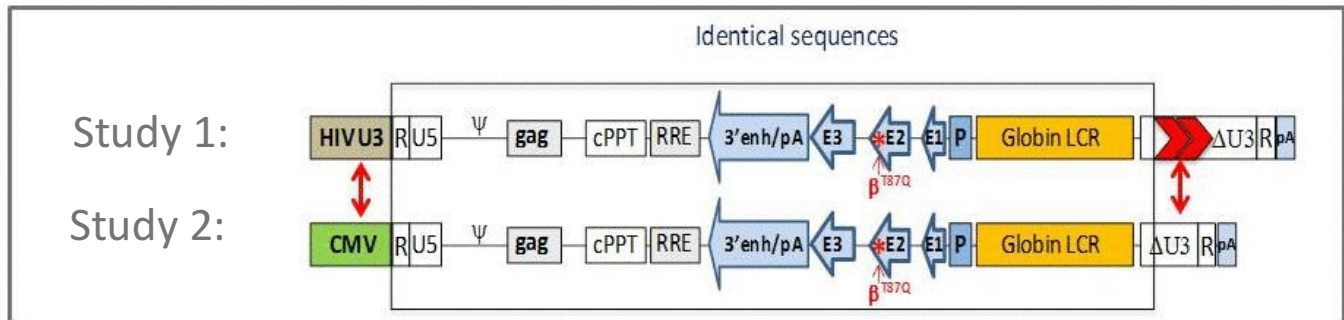
- No AEs related to drug product, including no RCL nor malignancy

## Study 1 : hemoglobin concentrations



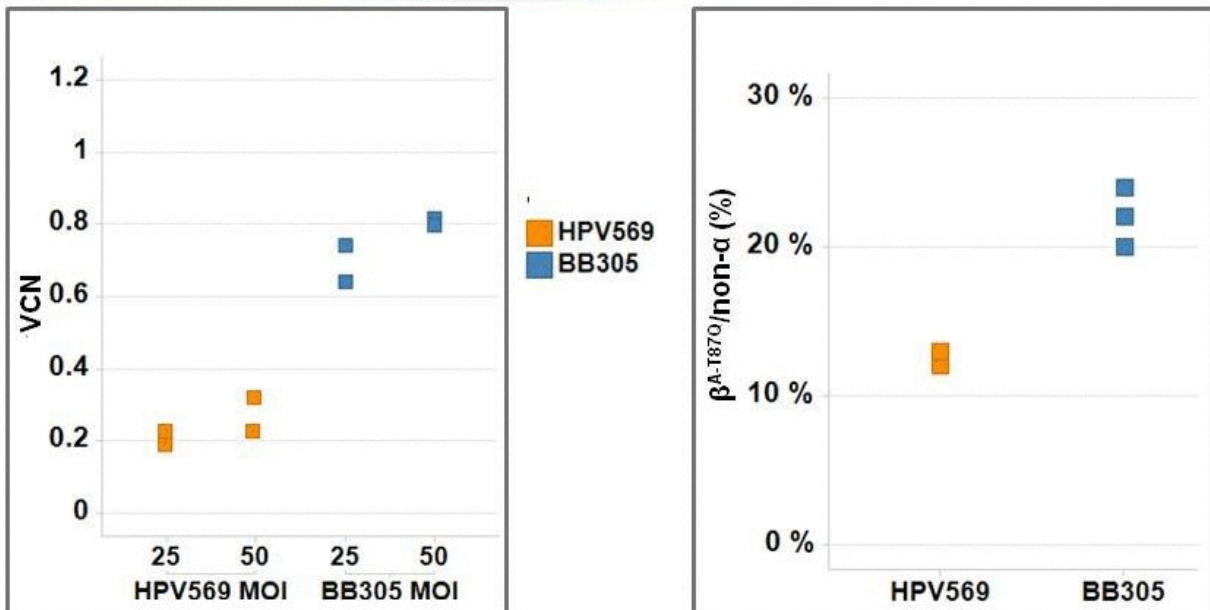
- **Subject 3:** stable levels of  $\text{b}^{\text{A-T87Q}}$ -globin beginning at Month 18, **transfusion independent by Month 12**. Producing 2.7 g/dL of  $\text{b}^{\text{A-T87Q}}$ -globin at 6 years.
- **Subject 4:** minimal levels of  $\text{b}^{\text{A-T87Q}}$ -globin post-treatment, transfusion dependent. Producing 0.4 g/dL of  $\text{b}^{\text{A-T87Q}}$ -globin at 2 years.

## BB305 lentiviral vector used in Study 2



- **Improvements made in vector design, vector manufacturing process, and drug product manufacturing, including:**
  - CMV promoter to drive vector production (*aim to increase vector titer*)
  - cHS4 insulator elements were removed (*aim to increase vector titer, potency, and stability*)

## Improvements in BB305 vector design and manufacturing process increase transduction efficiency in vitro



- Mean VCN was 2- to 3-fold higher in transduced human CD34<sup>+</sup> cells
- $\beta^{A-T87Q}$ -globin was produced at a 2-fold higher level in differentiated erythroid lineage cells

## Study 2 : characteristics of included subjects (to date)

	Subject 1	Subject 2
Age at Enrollment	18	16
Genotype	b <sup>0</sup> /b <sup>E</sup>	b <sup>0</sup> /b <sup>E</sup>
CD34 <sup>+</sup> VCN	1.5	2.1
CD34 <sup>+</sup> cell dose (x 10 <sup>6</sup> /kg)	8.9	13.6



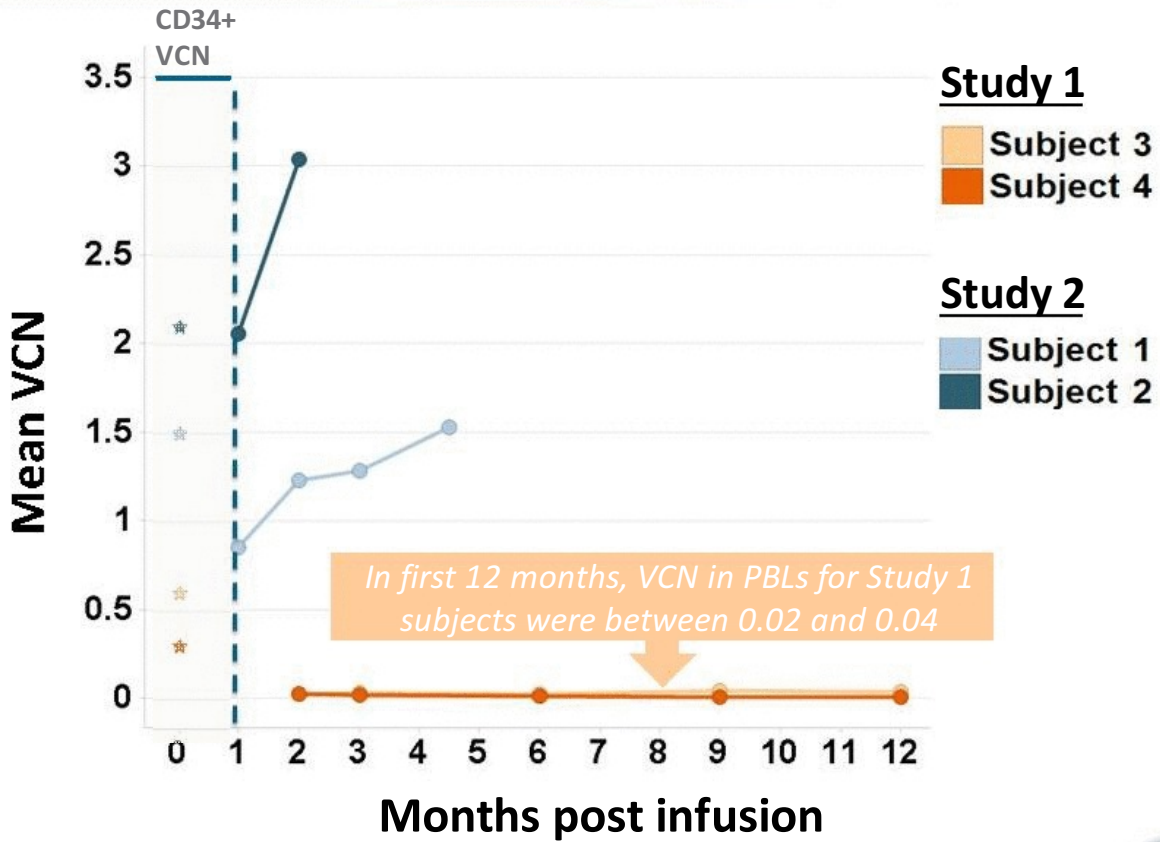
## Study 2 : Safety

	Subject 1	Subject 2
Follow up period	4.5 months	2 months
Day of neutrophil engraftment ANC > 500/ $\mu$ L	Day 13	Day 15
Day of platelet engraftment Unsupported platelet count > 20,000/ $\mu$ L	Day 17	Day 24
Non-laboratory $\geq$ Grade 3 AEs	Mucositis <sup>1</sup>	Mucositis
SAEs occurring $\geq$ Day 0	None	None
Insertion site analysis	At 3 Months: highly polyclonal (>1000), no clonal dominance	Not yet available

<sup>1</sup>Subject 1201 had an asymptomatic Grade 3 AST, ALT and GGT elevation from Days 23-90

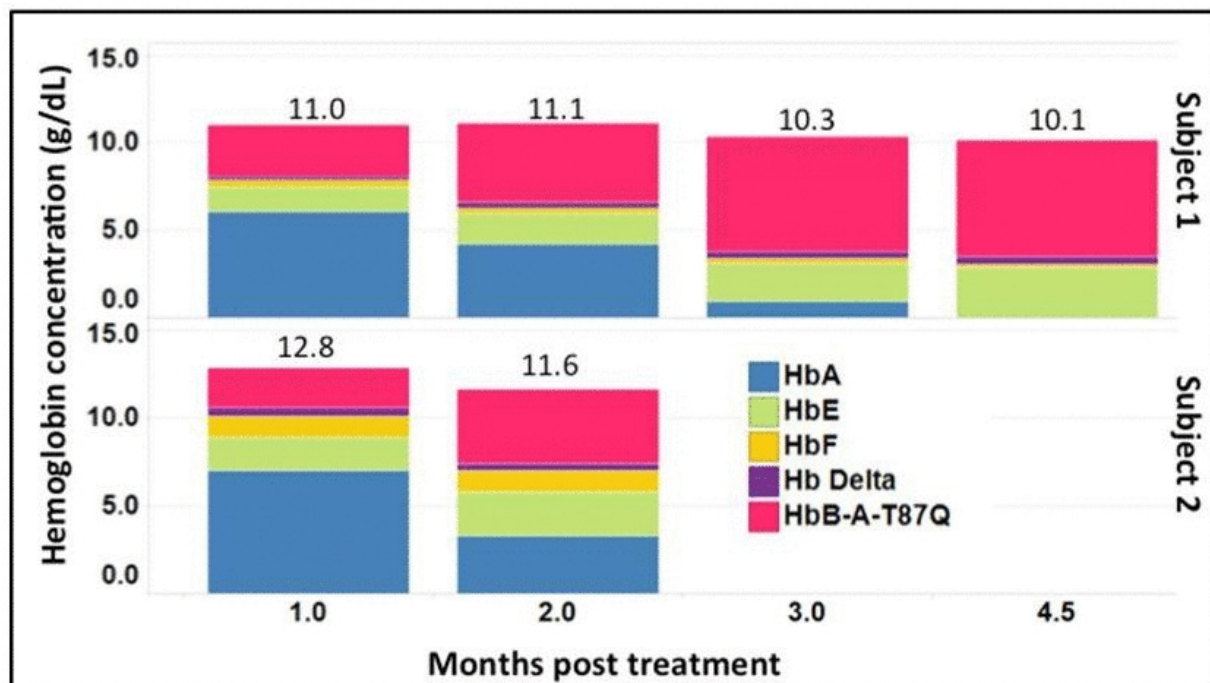
- No AEs related to drug product, including no RCL nor malignancy

# Study 2 vs. Study 1: VCN in peripheral blood leucocytes



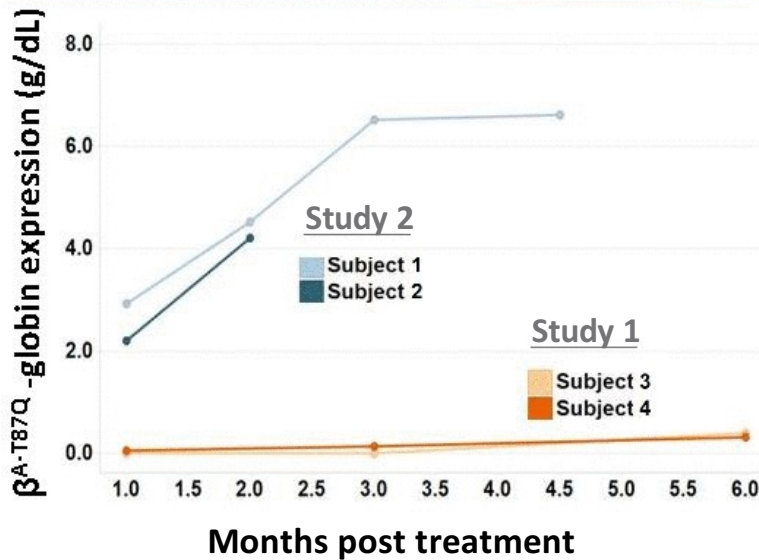


*In study 2, early and high production of  $\beta^{A-T87Q}$ -globin resulting in rapid transfusion-independence at near normal Hb levels in both patients*



- Subject 1: producing 6.6 g/dL of  $\beta^{A-T87Q}$ -globin at 4.5 months
- Subject 2: producing 4.2 g/dL of  $\beta^{A-T87Q}$ -globin at 2 months

# Kinetics of $\beta^{A-T87Q}$ expression and transfusion independence



RBC transfusion Independence			
	Subject 3	Subject 1	Subject 2
Study	1 LG001	2 HGB-205	2 HGB-205
Vector	HPV569	BB305	BB305
Day of last transfusion	Month 12	Day 10	Day 12
Duration since last transfusion	>5 years	>125 days	>48 days

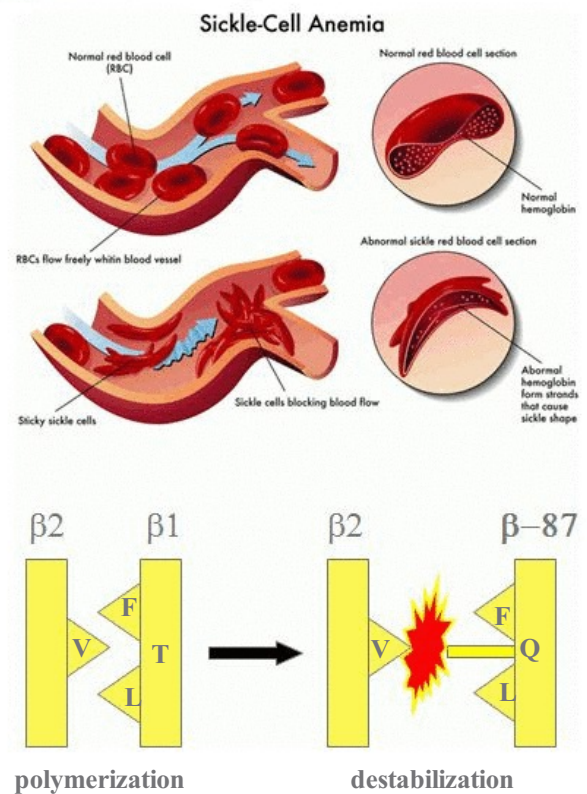
- In Study 2, rapid production of therapeutic globin (weeks as opposed to one year)
- Both subjects in Study 2 have near-normal hemoglobin levels without transfusion support (neither subject has required a transfusion post-engraftment)

## Conclusions

- BB305 lentiviral vector and improved manufacturing process produce superior transduction efficiency as compared with HPV569
- With BB305 lentiviral vector in Study 2, neither subject has received a transfusion since the second week post transplantation
  - Production of  $\beta^{A-T87Q}$ -globin has been rapid and clinically significant resulting in near-normal hemoglobin levels
- Initial safety profile is consistent with autologous transplantation, without gene-therapy related adverse events, and with polyclonal reconstitution in the first subject
- These data demonstrate that early transfusion independence (within weeks of transplantation) with near-normal levels of hemoglobin can be achieved with *ex-vivo* gene therapy using BB305 lentiviral vector in subjects with  $\beta^0/\beta^E$ -thalassemia major

# Evidence for why BB305 globin may work for sickle cell disease

- BB305 globin incorporates an anti-sickling amino acid that is found in fetal hemoglobin (glutamine at position 87)
- Anti-sickling activity of  $\beta^{A-T87Q}$ -globin has been demonstrated in a mouse model of SCD (Science 2001)
- Elevated fetal hemoglobin from hereditary persistence of fetal hemoglobin (HPFH) or treatment with hydroxurea has shown clinical benefit



## Ongoing Studies using BB305 lentiviral vector

Study	Centers	Indication	Planned subjects	Current Status
HGB-205 (trial reported today)	1 in France	b- thalassemia major and severe sickle cell disease	7	4 subjects enrolled 2 subjects treated to date
Northstar Study (HGB-204)	4 in US 1 in Australia 1 in Thailand	b- thalassemia major	15	6 subjects enrolled 1 subject treated to date
HGB-206	3-6 planned, all in US	Severe sickle cell disease	8	Open IND, pending initiation

# Recent and Upcoming News Flow

## 2013

- Signed global Oncology collaboration with Celgene
- Completed IPO
- Initiated phase II/III Starbeam Study
- Initiated two phase I/II Thal studies (Northstar & HGB-205)
- First patient transplanted in Starbeam Study
- First patient transplanted in Thal HGB-205 study

## 2014

- First patient transplanted in Northstar Study**
- File IND for sickle cell disease (SCD) study**
- Preliminary Thal HGB-205 data at EHA**
- Enroll first SCD patient in HGB-205 or HGB-206 (2014)**
- Preliminary Thal Northstar & HGB-205 data (late 2014)**
- Various clinical publications**

## 2015

- Complete enrollment of Starbeam Study
- Complete enrollment Thal Northstar & HGB-205
- Preliminary SCD data



# Q&A





# Backups



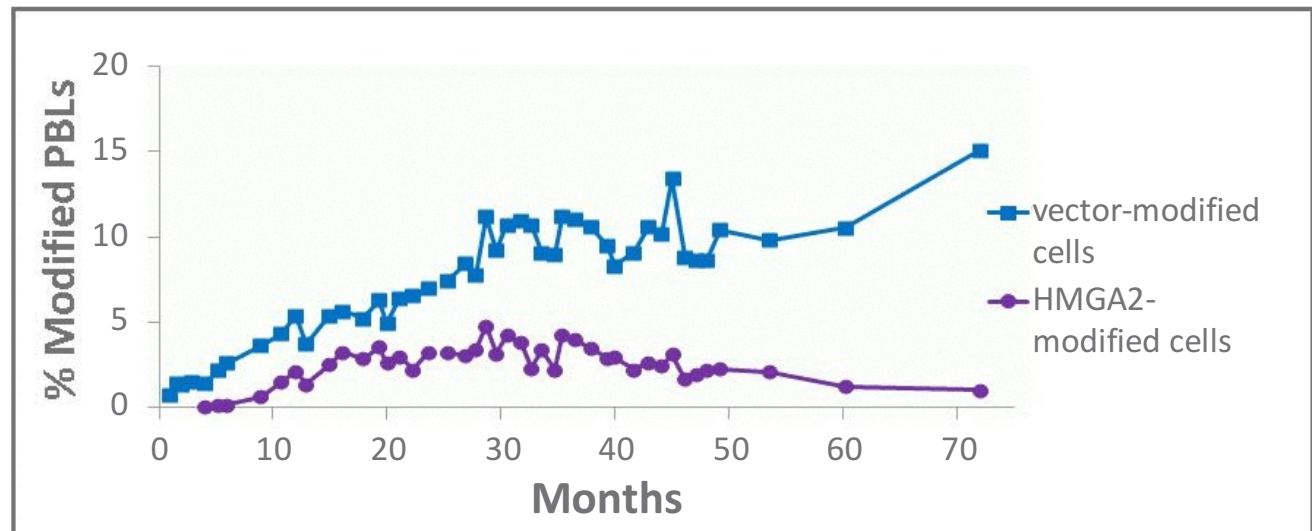


## Study 1 (LG001) : Safety

	Subject 3	Subject 4
Follow-up period	6 years	2 years
Day of neutrophil engraftment ANC > 500/ $\mu$ L	Day 27	Day 19
Day of platelet engraftment Unsupported platelet count > 20,000/ $\mu$ L	Day 40	Day 130
Non-laboratory $\geq$ Grade 3 AEs	None	Mucositis, metrorrhagia, epistaxis, mouth bleeding
SAEs occurring $\geq$ Day 0	None	Thrombocytopenia
Insertion site analysis	Multiple clones (25-50 detected at each timepoint), including HMGA2, many observed repeatedly over the following 5 years	Polyclonal (90-200 clones detected at each timepoint) reconstitution without clonal dominance at Year 1

- No AEs related to drug product, including no RCL nor malignancy

## Subject 3 (LG001) : prominence of HMGA2 clone decreasing over time



- By Year 5, SPATS2 and ZZEF1 have replaced HMGA2 as the most common clones identified by LAM-PCR
- In spite of decrease in HMGA2 clone, therapeutic effect has been maintained
- No hematological or clinical effects of the HMGA2 clone have been noted in over 6 years of follow-up