

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): January 8, 2018

**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 2.02 Results of Operations and Financial Condition.**

bluebird bio, Inc. (the "Company") intends to share with investors the number of shares outstanding as of December 31, 2017, and the amount of cash, cash equivalents and marketable securities it had on hand as of December 31, 2017. Although the Company has not finalized its financial results for the twelve months ended December 31, 2017, the Company currently anticipates that its cash, cash equivalents and marketable securities were approximately \$1.6 billion as of December 31, 2017, with approximately 49.4 million shares outstanding as of December 31, 2017. This information is unaudited and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2017 and its results of operations for the twelve months ended December 31, 2017. The Company expects to announce its full results for the twelve months ended December 31, 2017 on or before March 1, 2018.

**Item 7.01 Regulation FD Disclosure.**

The Company will be conducting meetings with investors attending the 36th Annual J.P. Morgan Healthcare Conference in San Francisco beginning on January 8, 2018. As part of these meetings, the Company will deliver the slide presentation furnished to this report as Exhibit 99.1 and which is incorporated herein by reference.

See Item 2.02 above, which is incorporated by reference herein.

*The information in this report furnished pursuant to Items 2.02 and 7.01 shall not be deemed "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section. It may only be incorporated by reference in another filing under the Exchange Act or the Securities Act of 1933, as amended, if such subsequent filing specifically references the information furnished pursuant to Items 2.02 and 7.01 of this report.*

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#"><u>Investor presentation furnished by bluebird bio, Inc. on January 8, 2018.</u></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: January 8, 2018

**bluebird bio, Inc.**

By: /s/ Jason F. Cole

Jason F. Cole  
*Chief Legal Officer*



# PATH TO PATIENTS

36th Annual J.P. Morgan  
HEALTHCARE CONFERENCE

January 9, 2018

NASDAQ: BLUE

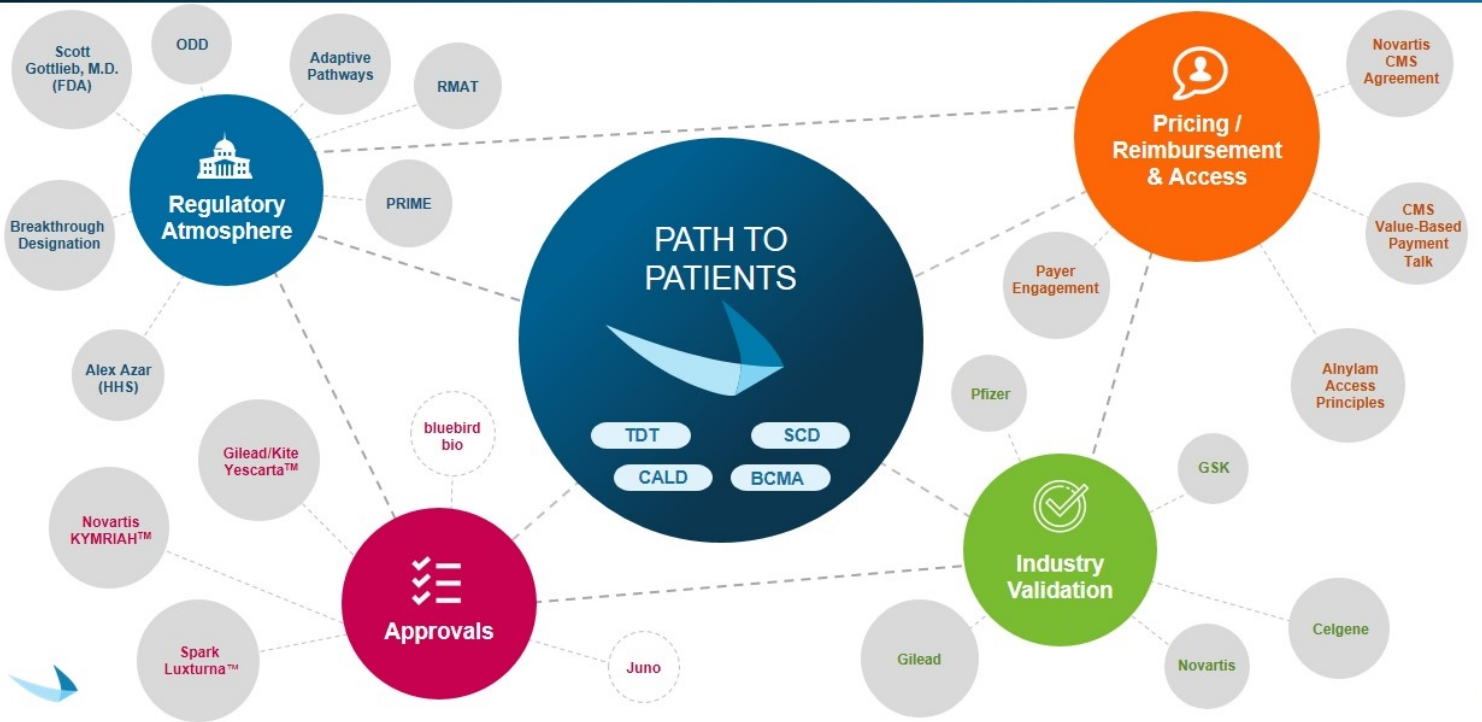


# Forward Looking Statements

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. 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.



# Healthy Ecosystem for Transformative Gene Therapy



# Our Focus. Our Imperatives.

## Execute & Deliver

Operate with discipline, urgency and healthy paranoia

## Scale & Reach

Expand organization and capabilities to bring products to patients globally

## Lead The Way

Lever product engine, capabilities and resources to solve challenges and unleash opportunities

## Stay BLUE

Beat the regression odds. Believe in the WHY and act accordingly.



# Hopes & Dreams Becoming a Reality

## HOPE

## REALITY

1993

- Genetix Founded

2009/2010

- *Science*: CALD
- *Nature*: TDT
- Restart VC Investment
- Changed Name to bluebird bio

2013/2014

- Celgene CAR T partnership
- IPO
- Acquired Genome Editing Company

2015/2016

- TDT: Breakthrough & PRIME Designation

2017

- BCMA: Breakthrough & PRIME Designation
- SCD: RMAT Designation
- *NEJM*: CALD & SCD
- Acquired Manufacturing Facility

CALD Starbeam (Oct. 2013)

TDT Northstar (March 2014)

SCD HGB-205 (Oct. 2014)

bb2121 for multiple myeloma (Feb. 2016)





# Three Regulatory Filings Anticipated by End of 2019

**LentiGlobin TDT**  
First Filing (2018)

**Lenti-D CALD**  
First Filing (2019)



**LentiGlobin SCD**  
Data-Driven Acceleration

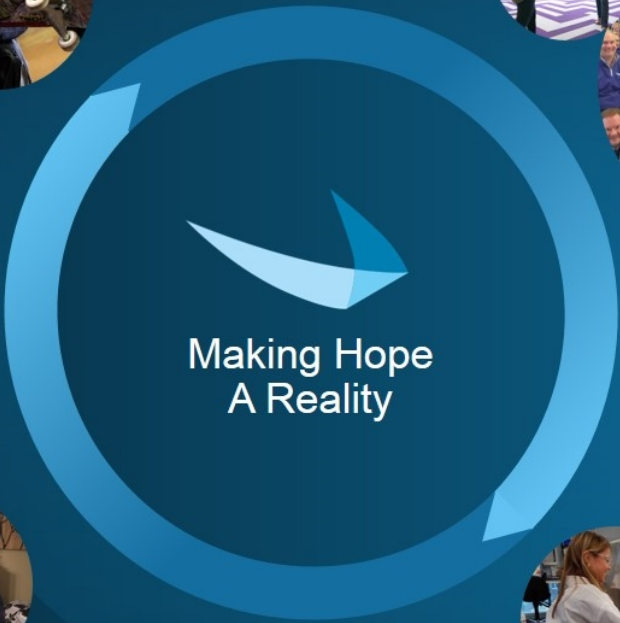
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Patient Impact

**bb2121 Multiple Myeloma**  
First Filing (2019)

**2+** Products  
on the Market

**2+** Programs Nearing  
Commercialization

**4+** Additional Programs  
in the Clinic



trueblue


bluemojo



# trueblue

OUR PATIENTS





***Ethan's family spent nearly two years trying different medications and meeting with specialists to try and resolve his symptoms. Tragically, during this period, the ravaging effects of ALD were continuing to damage Ethan's brain and adrenal glands.***

Ethan Zakes 2000 - 2011

Source: Ethan Zakes Foundation

## Cerebral Adrenoleukodystrophy

- Severe, often fatal neurological disease in boys

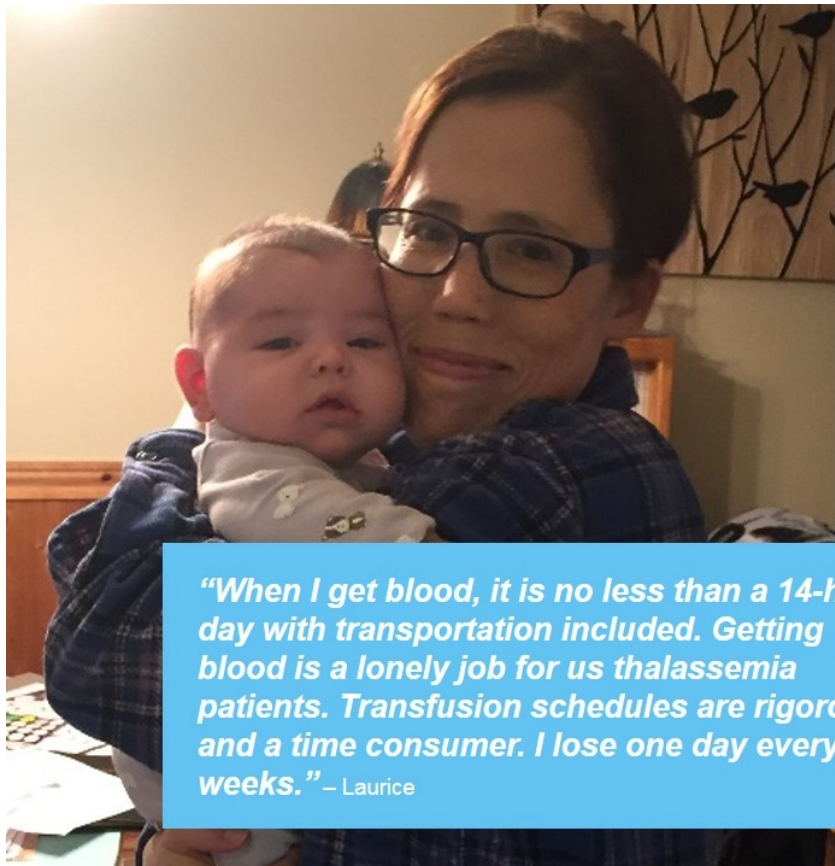
### STATUS

- 15/17 patients hit the primary endpoint so far
- Newborn screening active in 5 states<sup>1</sup>

### NEXT STEPS

- Expanding study to enroll total of 30 patients
- Anticipated filing in 2019

<sup>1</sup>Salzman, R., Kemp, S. (2017, December 06) Newborn Screening. Retrieved from <http://adrenoleukodystrophy.info/clinical-diagnosis/newborn-screening>

A photograph of a woman with dark hair and glasses, wearing a blue plaid shirt, holding a baby. The baby is wearing a white shirt and a blue blanket. The background shows a wall with a decorative pattern of leaves and branches.

***“When I get blood, it is no less than a 14-hour day with transportation included. Getting blood is a lonely job for us thalassemia patients. Transfusion schedules are rigorous and a time consumer. I lose one day every two weeks.” – Laurice***

## Transfusion-dependent $\beta$ -thalassemia

- Inherited blood disease that requires lifelong, frequent blood transfusions and iron reduction therapy

### STATUS

- Majority of patients with non- $\beta^0/\beta^0$  genotype are free of transfusions
- Refined manufacturing leading to robust increase in HbA<sup>T87Q</sup>
- 3+ years durability of effect in early studies

### NEXT STEPS

- Anticipated first regulatory filing in EU in patients with non- $\beta^0/\beta^0$  genotypes in 2018



***“I experienced my first sickle crisis requiring hospitalization at age 5. Since then I’ve endured hundreds of hospitalizations, blood transfusions and surgical procedures. Despite the devastating symptoms of sickle cell, I was determined to complete my educational goals.”- Lakiea***

*Source: Global Genes*

## Severe Sickle Cell Disease

- Severe blood disorder that leads to anemia, frequent pain crises and shortened lifespan

### STATUS

- Revised study protocol has yielded significant increase in anti-sickling hemoglobin
- Shift to plerixafor-based cell collection providing more and better cells; easier for patients

### NEXT STEPS

- Complete 206 study
- Define clinical development and regulatory path



*“When I was diagnosed and realized that there was an empty pipeline... I knew I needed to do something — not only for myself and my family, but for everyone else with this ‘orphan cancer’. I desperately wanted my daughter to remember me and thought that if I lived for five years, maybe she would have memories of her mom.”* - Kathy Giusti, Founder, MMRF

## Multiple Myeloma (BCMA)

- A lethal blood cancer that often infiltrates the bone marrow causing anemia, kidney failure, immune problems and bone fractures

### STATUS

- 94% ORR, 56% CR
- 89% VGPR or better
- Median PFS not reached with 40 weeks follow up

### NEXT STEPS

- Complete pivotal study
- Initiate studies in earlier lines
- Anticipated US and EU filings in 2019

# bluemojo

OUR PEOPLE





# Driving the Product Platform to Reality for Patients

**Make & Scale It**

**Relentlessly Learn & Innovate**

**Deliver It**

**Relentlessly Learn & Innovate**

**Value It**

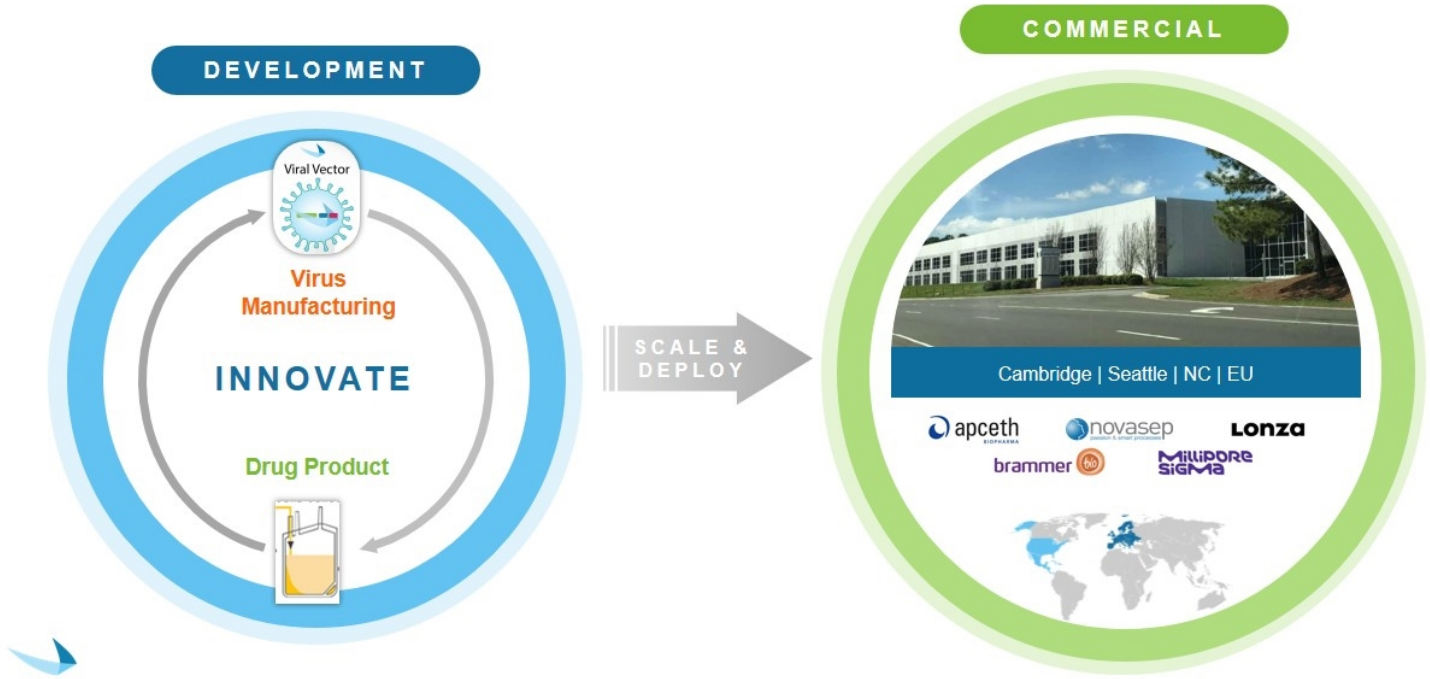
**Relentlessly Learn & Innovate**

**Lever It**

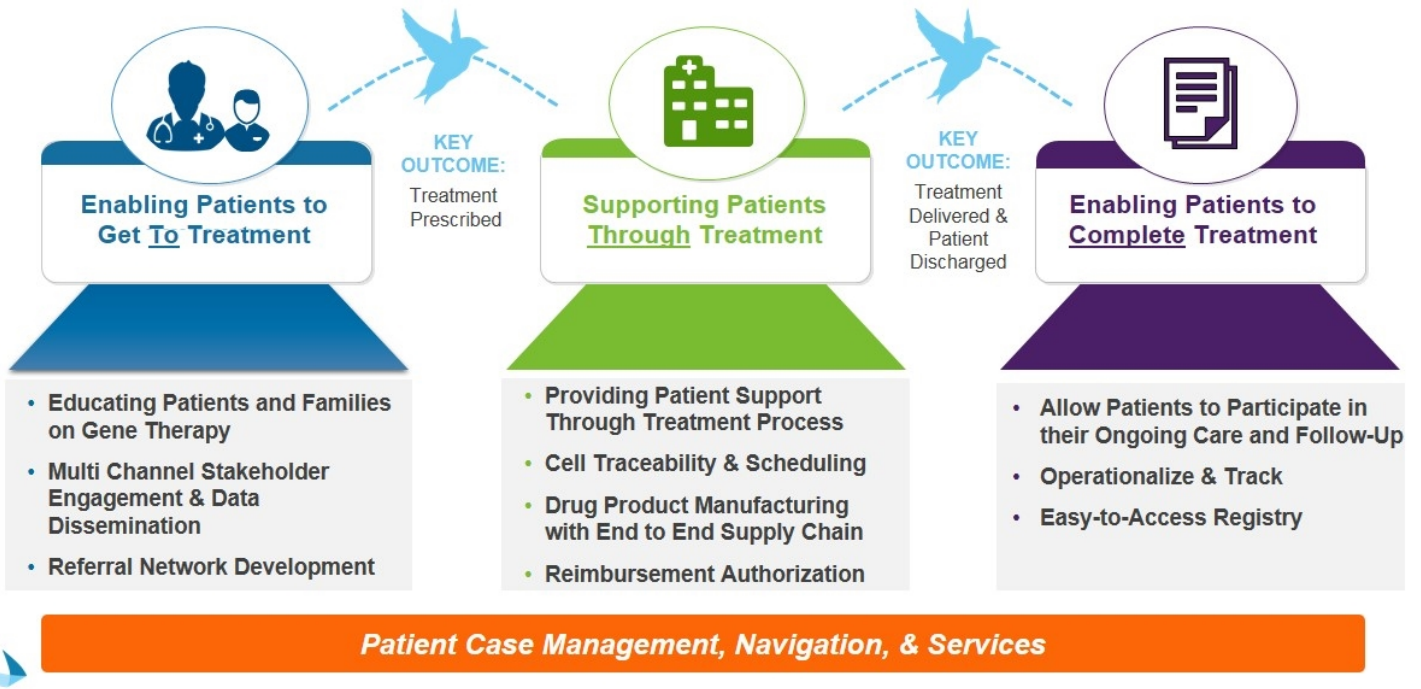
**Relentlessly Learn & Innovate**



# Make & Scale It: Focused on Transitioning from Development to Commercial



# Deliver It: The Best Possible Provider, Payer and Patient Experience



# Value It: Time to Get It Right



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**The value our products bring to patients should stand on its own for all stakeholders**



# Value It : Quick Answer is Value Based Payment Over Time

## BLUE "VALUE" PRINCIPLES

- Be focused on patient access to innovation
- Be creative and disruptive (if needed)
- Be flexible and share risk
- Be transparent and proactive with stakeholders
- Be proud
- Don't do stupid short sighted stuff!

## CONSTRAINTS & AMBITIONS

### UNMET NEED

- Heighten awareness of true unmet need in terms of impact on life expectancy and cost

### VALUE EVIDENCE

- Deliver credible and rigorous value platform arguments/data for value

### PAYMENT MODELS

- "Free Up" system to recognize value over time
- "Buy time" to prove enduring value
- Fix cost density constraint
- Fix policy constraints (e.g., best price)
- Fix "portability of cure" concern

## Innovation & Capabilities

- Viral Vector Manufacturing
- Transduction Enhancements
- Plerixafor Mobilization
- PI3ki-based BCMA manufacturing

## Partnerships & Acquisitions



## New Products & Pipeline

- bb21217 *Phase 1*
- shmiR *Phase 1*
- CAR Ts and TCRs *Preclinical*
- Gamma Delta T cells *Preclinical*
- MegaTALs *Preclinical*

# Our Quest to Constantly Innovate Continues

Product Candidates	Program Area	Preclinical	Phase 1/2	Phase 2/3	Rights/Partner
<b>Severe Genetic Diseases</b>					
Lenti-D™ Drug Product	Cerebral ALD				Worldwide
LentiGlobin® Drug Product	Transfusion-Dependent β-thalassemia			(Phase 3)	Worldwide
	Severe Sickle Cell Disease				Worldwide
BCL11a shRNA(miR)*	Severe Sickle Cell Disease				Worldwide
<b>Cancer</b>					
bb2121	Multiple Myeloma				Celgene
bb21217	Multiple Myeloma				Celgene
Undisclosed Targets	Various Indications				Worldwide
<b>Early Research</b>					
Early Pipeline	Undisclosed + Gene Editing				Worldwide

## COLLABORATORS



\*Development is led by Boston Children's Hospital



# 2018 Milestones

## BY MID YEAR\*\*

- TDT: Northstar-2 (HGB-207) Data
- MM: CRB-401 (bb2121) Data
- SCD: BCL11A shRNA Study Start

**\$1.6 Billion Cash  
Runway into 2021**

*49.4m shares outstanding as of 12/31/17*

Cash, cash equivalents and marketable securities (unaudited) as of 12/31/2017. Cash runway guidance is based on current assumptions as of the date thereof and does not include the effect of potential license and collaboration agreements, business combinations or asset acquisitions.

## BY END OF YEAR\*\*

- TDT: EMA Filing in Non- $\beta^0/\beta^0$  Genotypes
- TDT: Northstar-3 (HGB-212) Data
- SCD: HGB-206 Data
- SCD: Registration Strategy Update
- MM: Initiate 3<sup>rd</sup> Line Study\*; bb21217 Data
- CALD: Starbeam (ALD-102) Data



# Path to Patients

Three Regulatory Filings Anticipated by End of 2019



**2+** Products on the Market

**2+** Programs Nearing Commercialization

**4+** Additional Programs in the Clinic

