

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, 2019

**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 8.01 Other Events.**

On January 8, 2019, bluebird bio, Inc. conducted an investor presentation at the 37<sup>th</sup> Annual J.P. Morgan Healthcare Conference in San Francisco, California. A copy of the presentation 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

Exhibit No.	Description
99.1	<a href="#">Investor presentation provided by bluebird bio, Inc. on January 8, 2019.</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, 2019

**bluebird bio, Inc.**

By: /s/ Jason F. Cole

Jason F. Cole  
*Chief Legal Officer*



# READY TO RECODE

37th Annual J.P. Morgan  
Healthcare Conference

January 8, 2019

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.

# WE RECODE FOR LIFE



## RADICAL CARE

We care in a way that's intense and truly sets us apart.



## THIS IS PERSONAL

Gene therapy is about saving lives one person at a time. And we are, each of us, personally all in.



## PIONEERS WITH PURPOSE

We're exploring new frontiers for the sake of patients.

# We LIVE By Our Non-negotiables

true blue | b colorful • b cooperative • b yourself



# Our 2022 Vision -- Just Got BOLDER

**LentiGlobin TDT**  
2019 EU Potential Approval  
2020 U.S. Potential Approval

**Lenti-D CALD**  
2020 Potential Approval

**LentiGlobin SCD**  
2022 Potential Filing/Approval

**bb2121 Multiple Myeloma**  
2020 Potential Approval

**2022**  
THE GENE THERAPY  
PRODUCTS COMPANY

$\infty$   
Patient Impact

**4** Products  
on the Market

**5+** Clinical Programs

**1-2** INDs per year starting in 2020

# UNPRECEDENTED OPPORTUNITY

Anticipated research, development, regulatory and commercial milestones



# RECODE FOR LIFE

1

**RECODE**  
THE SCIENCE

2

**RECODE**  
THE SYSTEMS

3

**RECODE**  
THE STATUS QUO

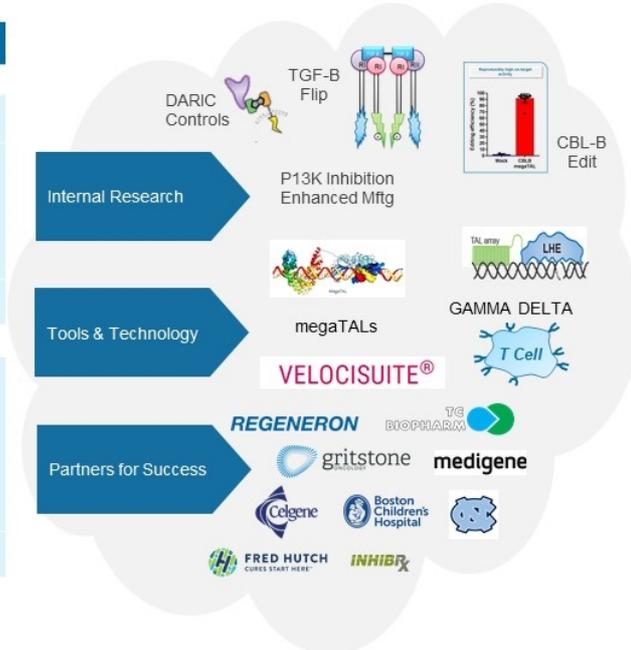
# RECODE THE SCIENCE: R&D with SOUL

## WHAT YOU SEE

PRODUCT CANDIDATES	PROGRAM AREA	PRECLINICAL	PHASE 1/2	PHASE 2/3	RIGHTS/PARTNER
<b>Severe Genetic Diseases</b>					
Lenti-D™ Drug Product	Cerebral Adrenoleukodystrophy				Worldwide
	Transfusion-Dependent β-Thalassemia Non-β <sup>0</sup> /β <sup>0</sup>				Worldwide
LentiGlobin™ Drug Product	Transfusion-Dependent β-Thalassemia β <sup>0</sup> /β <sup>0</sup>				
	Sickle Cell Disease		**		
BCL11a shRNA (miR)*	Sickle Cell Disease				Worldwide
<b>Cancer</b>					
bb2121	Multiple Myeloma Fourth Line				Celgene
	Multiple Myeloma Third Line**				
	Multiple Myeloma Second Line**				
	Multiple Myeloma First Line**				
bb21217	Multiple Myeloma Fourth Line				Celgene

\*Development is led by Dana-Farber/Boston Children's Cancer and Blood Disorders Center  
 \*\*Planned studies

## WHAT YOU DON'T SEE



# Anti-Pure Play Principles - What Do We Mean?

## RECODING TRADITIONAL R&D



# Our Philosophy Applied in a Tumor Microenvironment

## TUMOR TARGET

**REGENERON**



1

## POTENCY ENHANCEMENT

**PREGENEN**



3

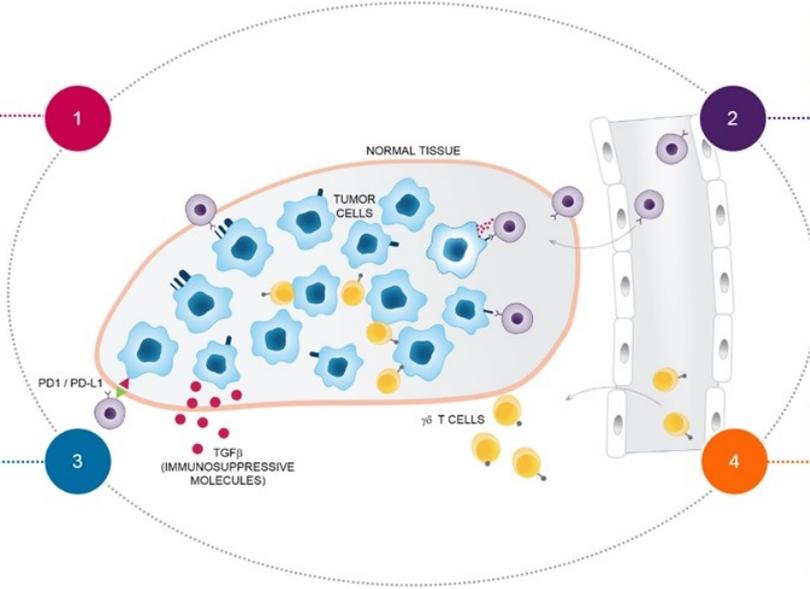
## BINDER TECHNOLOGY

**REGENERON**

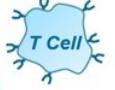
**medigene** **INHIBIX**



2



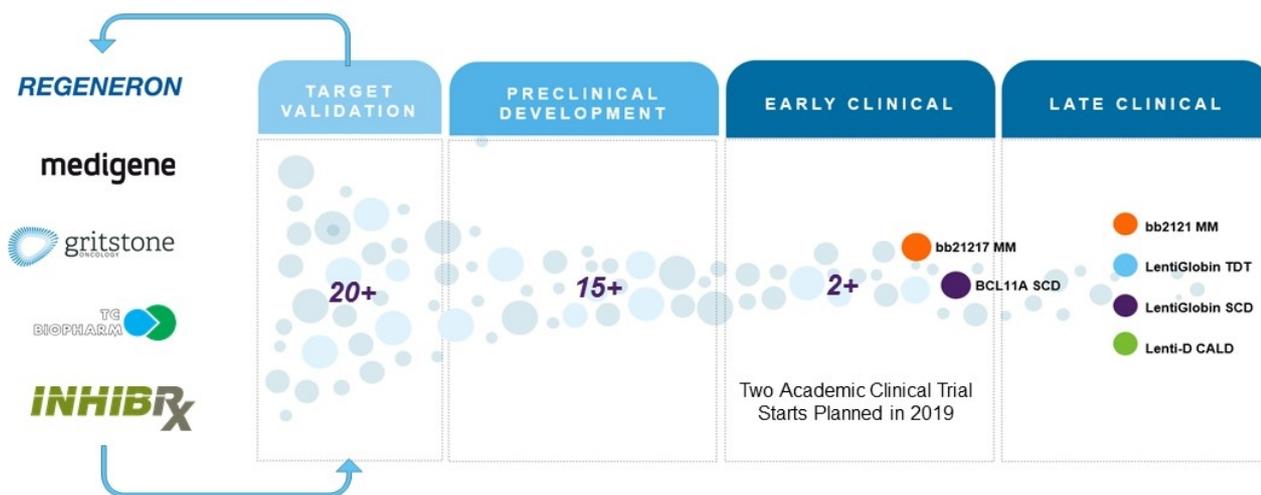
## T CELL TYPES / ALLO



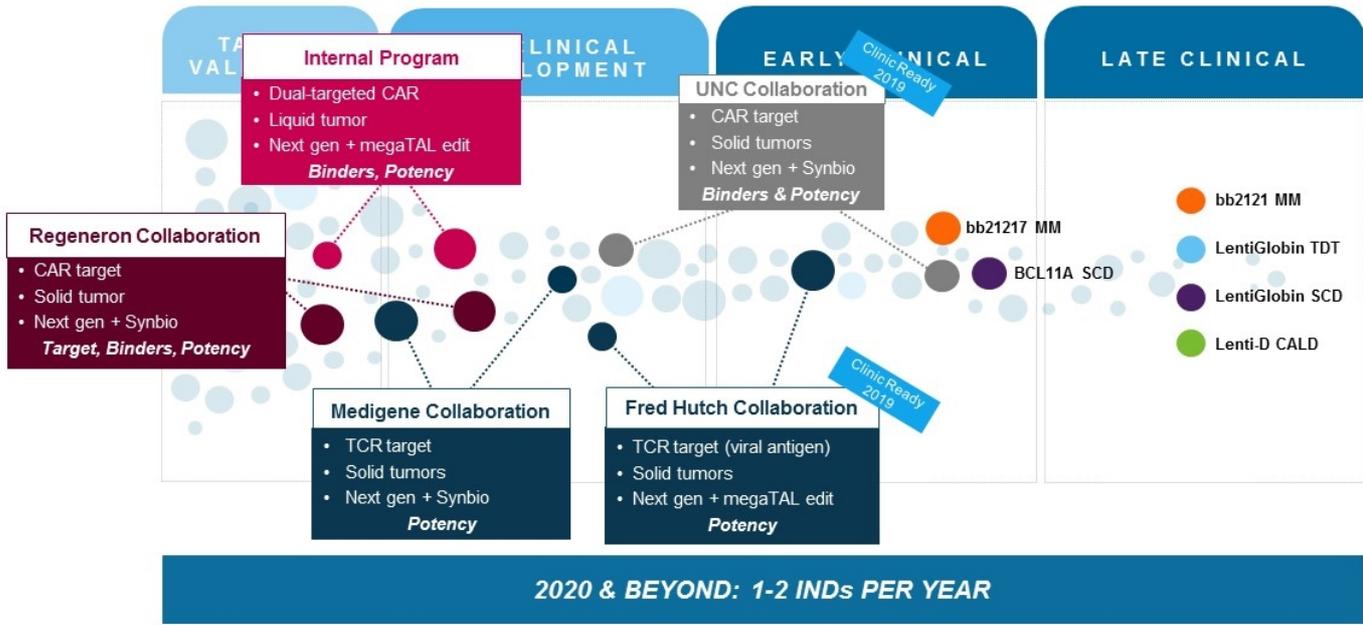
P13K Inhibition  
Enhanced Mftg

4

# Oncology Pipeline Enabled by Our Partners and Our Core Technologies



# Research Strategy Yielding Emerging Oncology Pipeline



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# RECODE THE SYSTEMS: ANYTHING BUT TRADITIONAL



**Novel  
Science/Medicine**



**Drug Product  
Production**



**Bold & Balanced  
Access Model**



**Vector  
Production**



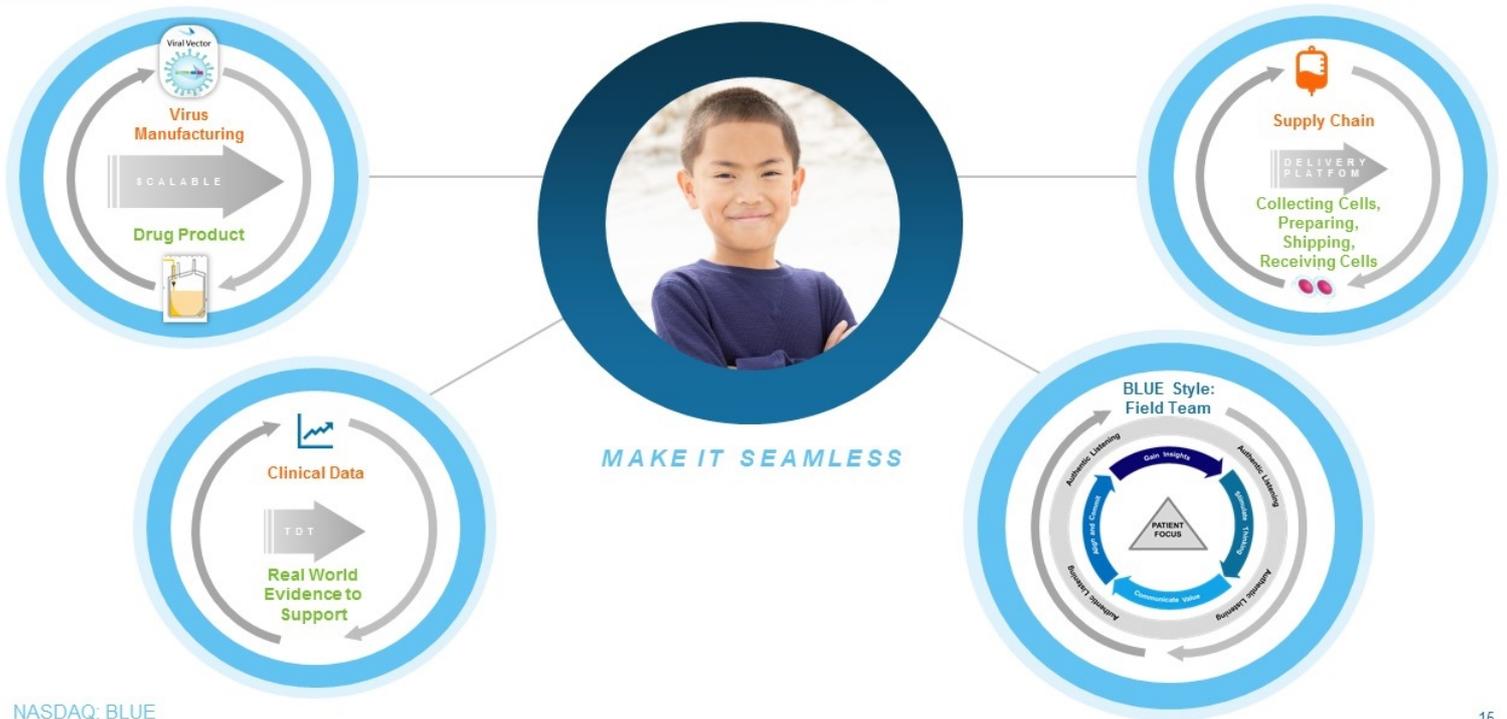
**Supply Chain &  
Patient Management**

UNKNOWN  
IMPACT

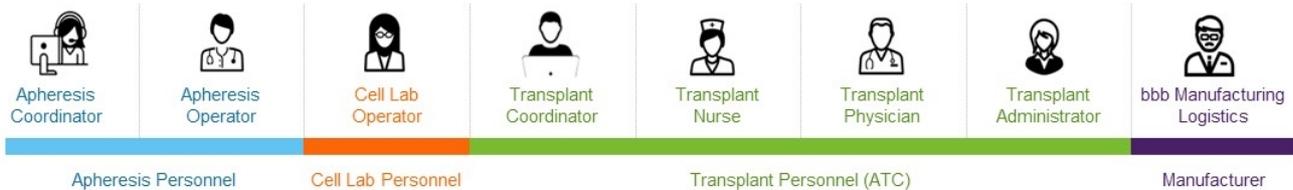
OUR GOAL - MAKE IT SEAMLESS TO ALL STAKEHOLDERS

ONE-TIME  
POTENTIALLY  
CURATIVE

# Platform Is Gearing Up for Launch



# Preparing to Serve Patients in Europe in 2019



## Launch Expectations:

1. Optimal patient experience through a seamless delivery network
2. Steady country by country launch with progressive build
3. Get the model right for long term success
4. Advance value-based payment over time reimbursement



### 1 Drug Product Manufacturing

Munich, Germany

&

### 9 Qualified Treatment Centers at 2019 Launch

- 3 - Germany
- 4 - Italy
- 2 - UK
- 4 - France (in 2020)\*

# RECODE FOR LIFE

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THE SCIENCE

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**RECODE**  
THE STATUS QUO

**Xconomy**

“Can Pay-For-Results Drug Pricing Work?”

**STAT**

“How much would you pay for the miracle of gene therapy?”

**Forbes**

“Value-Based Pricing And Reimbursement: Still More Promise Than Reality”

**BioPharma DIVE**

“Gene therapy could be cost effective in SMA, but not at \$4M”

January 8, 2019

**THE WALL STREET JOURNAL.**

**“Biotech Proposes Paying for Pricey  
Drugs by Installment”**

System NOT setup for one-time potentially curative treatments

Focus is on price, NOT on value

Focus is on difficulties and barriers, NOT solutions

Growing alignment on value & outcome based payment models

## BLUEBIRD OPPORTUNITY

- **The Will To Recode**
- **One-Time Treatment**
- **Potentially Curative**
- **Product Engine**
- **Unconstrained**

# Keep It Simple, Keep It Focused on the **Patient** & the Long Term

## BLUE VALUE PRINCIPLES

- Focus on patient innovation and access
- Creative and disruptive
- Flexible and share risk
- Transparent, proud and proactive
- Don't do silly short sighted stuff



**Unapologetically** fund & reward innovation that matters

**Focus** on **real value** delivered to the patient & system

**Don't truncate value** because it's a one-time potentially curative treatment

**Don't price** at what you can get away with or what the market can bear

## Our Goal Is to Simultaneously Achieve Five Key Objectives

1

**BLUE** has **CONVICTION** in the **VALUE** of the **LIFELONG** transformative benefit that our therapies may bring to patients, physicians, caregivers, healthcare systems and society at large

2

**BLUE** is willing to **SHARE** the **RISK** of uncertainty to **PROVE** the life long value of its therapies

3

**BLUE** wants to make its treatments **AFFORDABLE** so **PATIENTS (and SYSTEM)** can actually

4

**REALIZE** the **VALUE** of its therapies

5

**BLUE** wants to be a **CATALYST** for **CHANGE** to establish a **SUSTAINABLE MODEL** for pricing & reimbursement of gene therapies

# Approach – VALUE-BASED PAYMENT Over Time Based on OUTCOME

## OBJECTIVE

## STRATEGIC APPROACH

1

**FAIR VALUE  
RECOGNITION**

- ✓ Lifetime cost-time effectiveness timeframe
- ✓ Base value only on patient QOL and Life Extension

2

**SHARED  
RISK**

- ✓ Pay **ONLY IF** the treatment works
- ✓ Put **UP TO 80%** of the price at risk based on success

3

**PER PATIENT  
AFFORDABILITY**

- ✓ Spread payments over **UP TO A FIVE YEAR** period
- ✓ **NO PRICE INCREASES** above CPI

4

**HEALTH SYSTEM  
AFFORDABILITY**

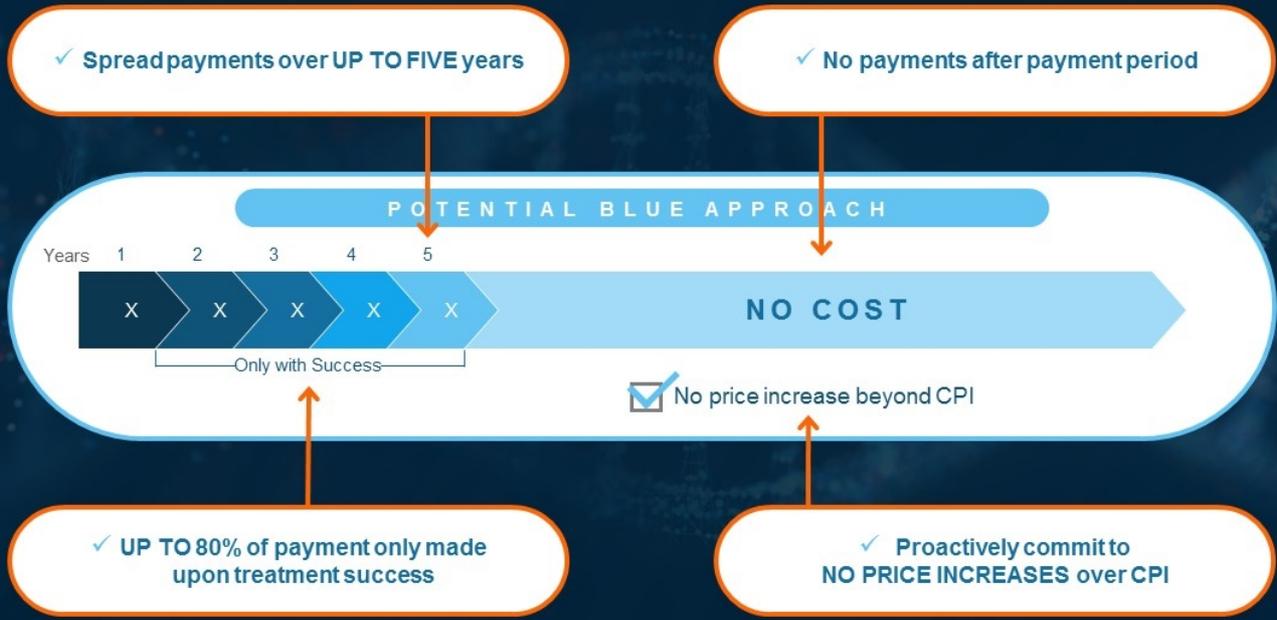
- ✓ **NO COST** after payment period (vs. for life)

# Payment Model – Patient and System Friendly



\*Ranges based on 2018 U.S. list prices from a sampling of seven chronic rare disease therapies.

# Payment Model – Sharing Risk & Value with the System



# Not the Easy Path but the Challenges Are Solvable



## TRACKABILITY?

- **Challenge:** How do you define and track outcomes?
- **Possible Solutions:** Payer to use claims and/or registry data

## PORTABILITY?

- **Challenge:** What happens when patient changes payer?
- **Possible Solutions:** Address in contract negotiation, mutual recognition strategy across payers, etc.

## BEST PRICE?

- **Challenge:** How do you handle Medicaid "Best Price" rules?
- **Possible Solutions:** Innovative federal or state pilot programs or waivers

# VALUE-BASED PAYMENT Over Time Based on OUTCOMES

## OBJECTIVE

## STRATEGIC APPROACH

1

**FAIR VALUE  
RECOGNITION**



- ✓ *Lifetime cost-time effectiveness timeframe*
- ✓ *Base value only on patient QOL and Life Extension*

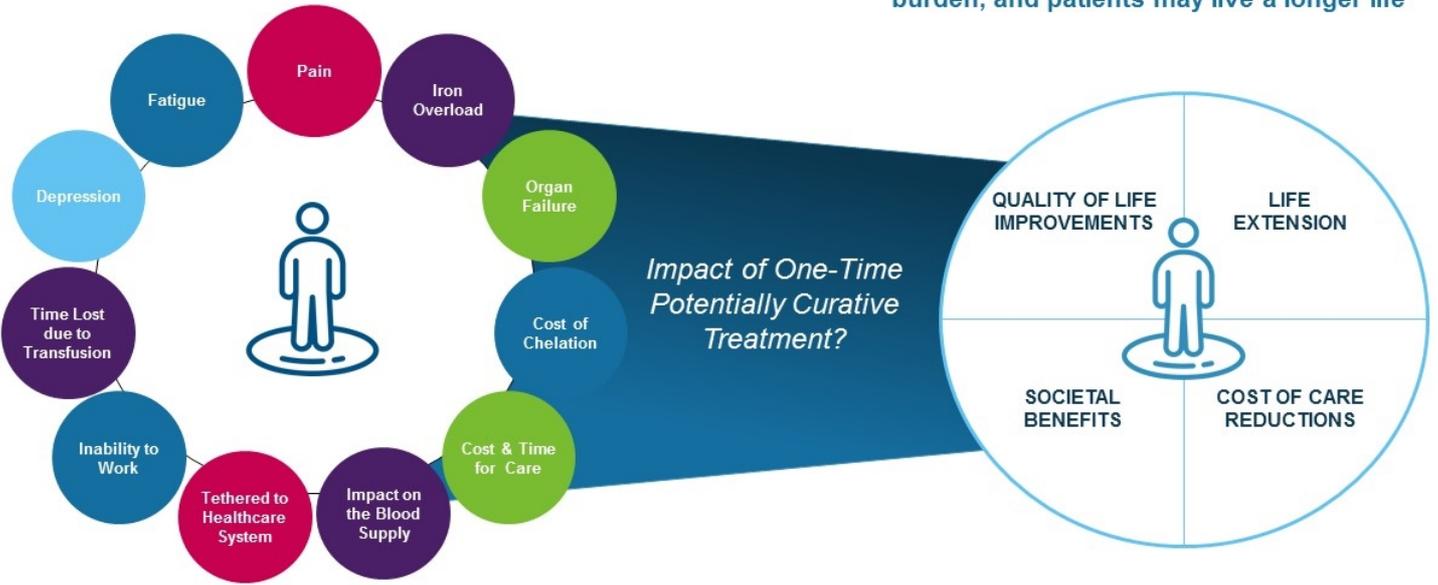
***What is the value that LentiGlobin can bring to  
TDT patients, payers, system and society?***

***AND how can it be fairly measured?***

# What Value Can LentiGlobin Bring to TDT Patients, Payers and System?

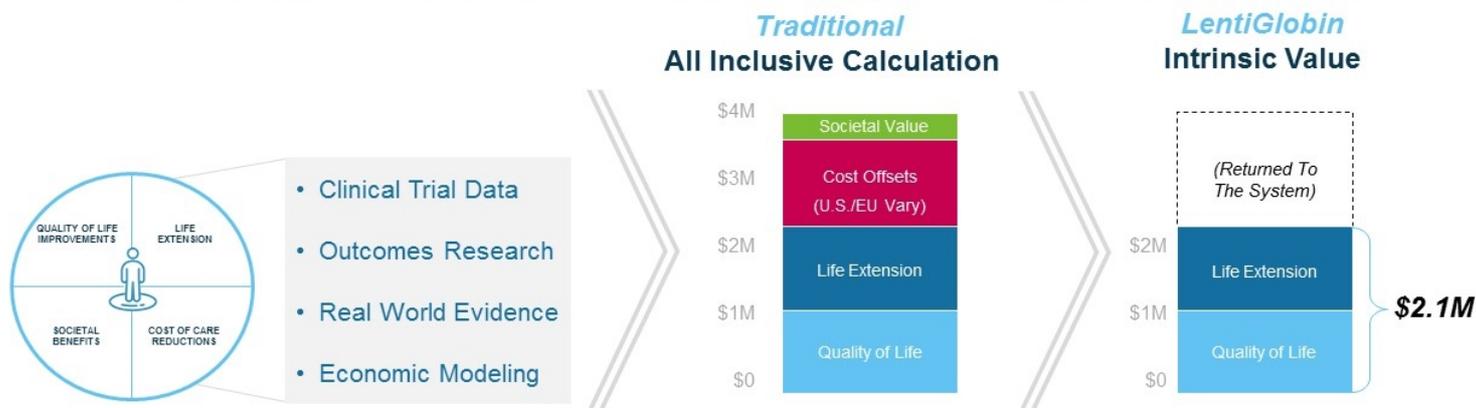
Disease and treatment-related burden builds up over time and can even lead to early death

LentiGlobin, if successful, has the potential to relieve patients of their daily disease and treatment-related burden, and patients may live a longer life



# Cost Effective Analysis Focused on Actual Patient Value: QOL and Life Extension

THE VALUE AT WHICH TREATMENT IS COST EFFECTIVE\* (**NOT PRICE**)



**The actual LentiGlobin price is TBD, but will not exceed the intrinsic value (total value minus cost offsets).**

- ✓ Value-based payment over time tied to outcomes
- ✓ Base value only on **actual patient value** (no offsets)
- ✓ Payments **over ~5 years** with **up to 80% at risk**
- ✓ **No price increases** beyond CPI
- ✓ Actual price TBD, but will **not exceed intrinsic value**



# WE RECODE FOR LIFE

Our ambition is to recode science,  
systems and the status quo,  
so **lives can be lived fully.**



