



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

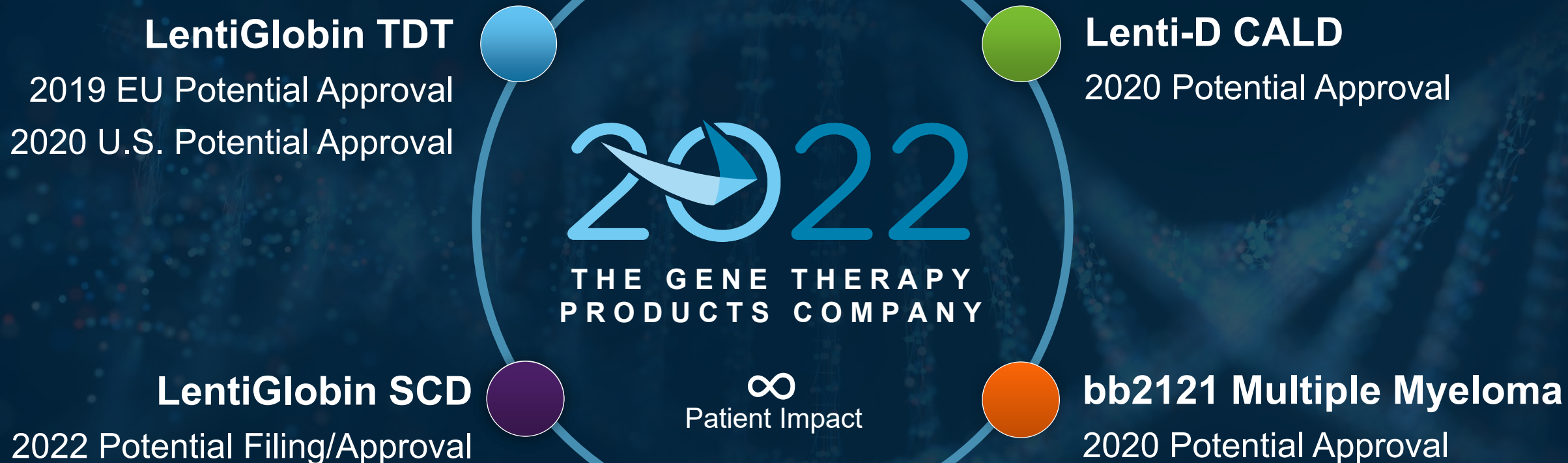
We're exploring new frontiers for
the sake of patients.

We LIVE By Our Non-negotiables

true blue | b colorful • b cooperative • b yourself



Our 2022 Vision -- Just Got BOLDER



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

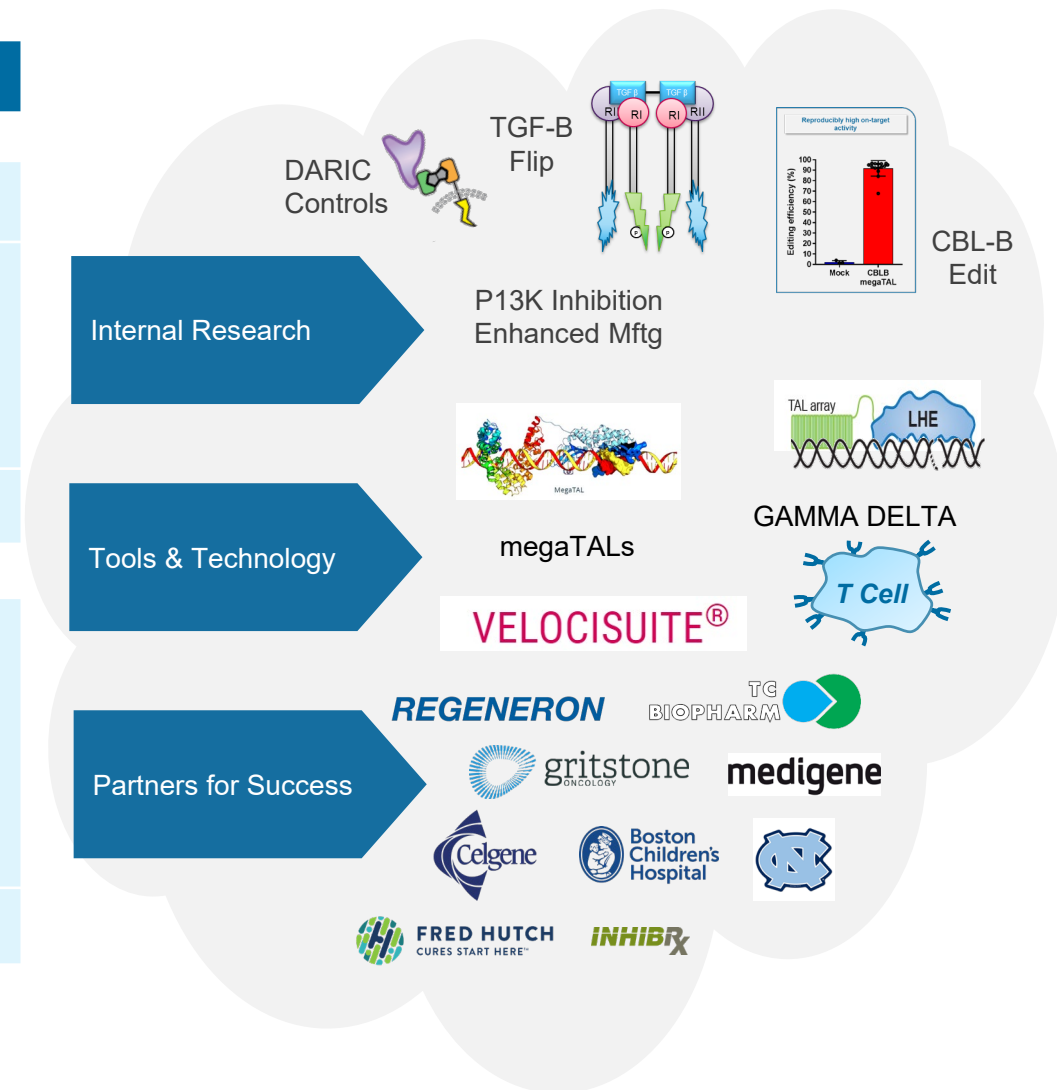
WHAT YOU SEE

| PRODUCT CANDIDATES | PROGRAM AREA | PRECLINICAL | PHASE 1/2 | PHASE 2/3 | RIGHTS/PARTNER |
|--------------------------------|--|-------------|-----------|-----------|----------------|
| Severe Genetic Diseases | | | | | |
| Lenti-D™ Drug Product | Cerebral Adrenoleukodystrophy | | | | Worldwide |
| LentiGlobin™ Drug Product | Transfusion-Dependent β-Thalassemia Non-β ⁰ /β ⁰ | | | | Worldwide |
| | Transfusion-Dependent β-Thalassemia β ⁰ /β ⁰ | | | | |
| | Sickle Cell Disease | | ** | | |
| BCL11a shRNA (miR)* | Sickle Cell Disease | | | | Worldwide |
| Cancer | | | | | |
| 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

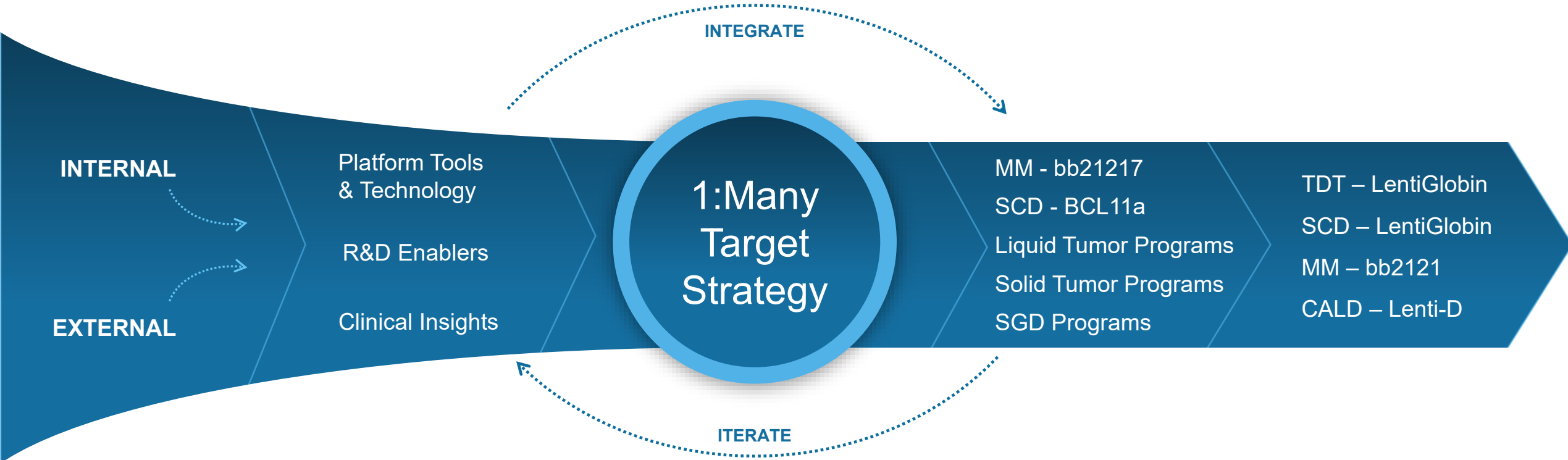
**Planned studies

WHAT YOU DON'T SEE



Anti-Pure Play Principles - What Do We Mean?

RECODING TRADITIONAL R&D



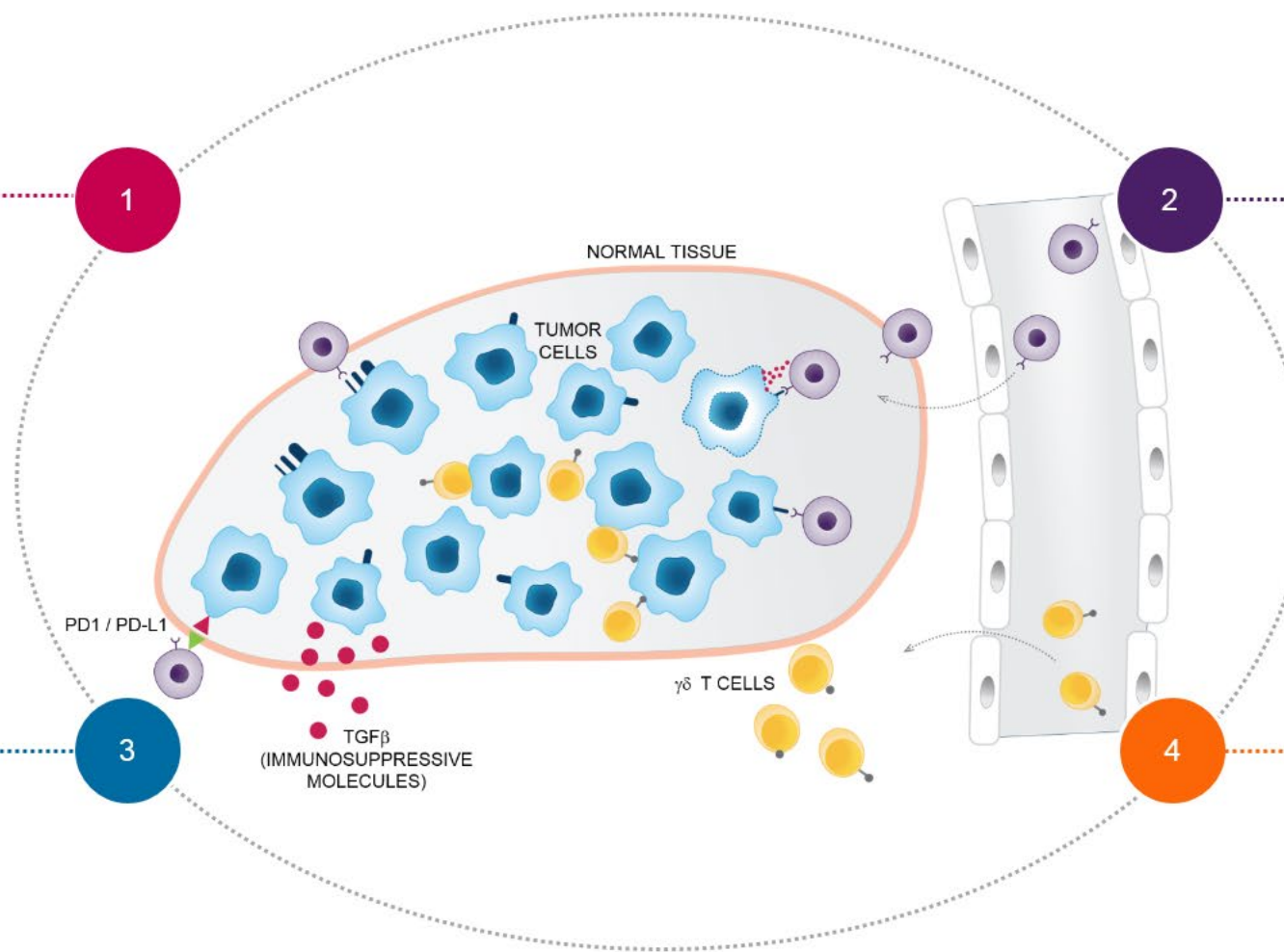
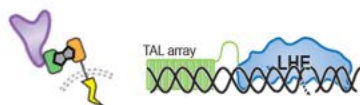
Our Philosophy Applied in a Tumor Microenvironment

TUMOR TARGET

REGENERON



POTENCY ENHANCEMENT



BINDER TECHNOLOGY

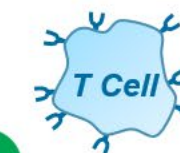
REGENERON

medigene

INHIBRx

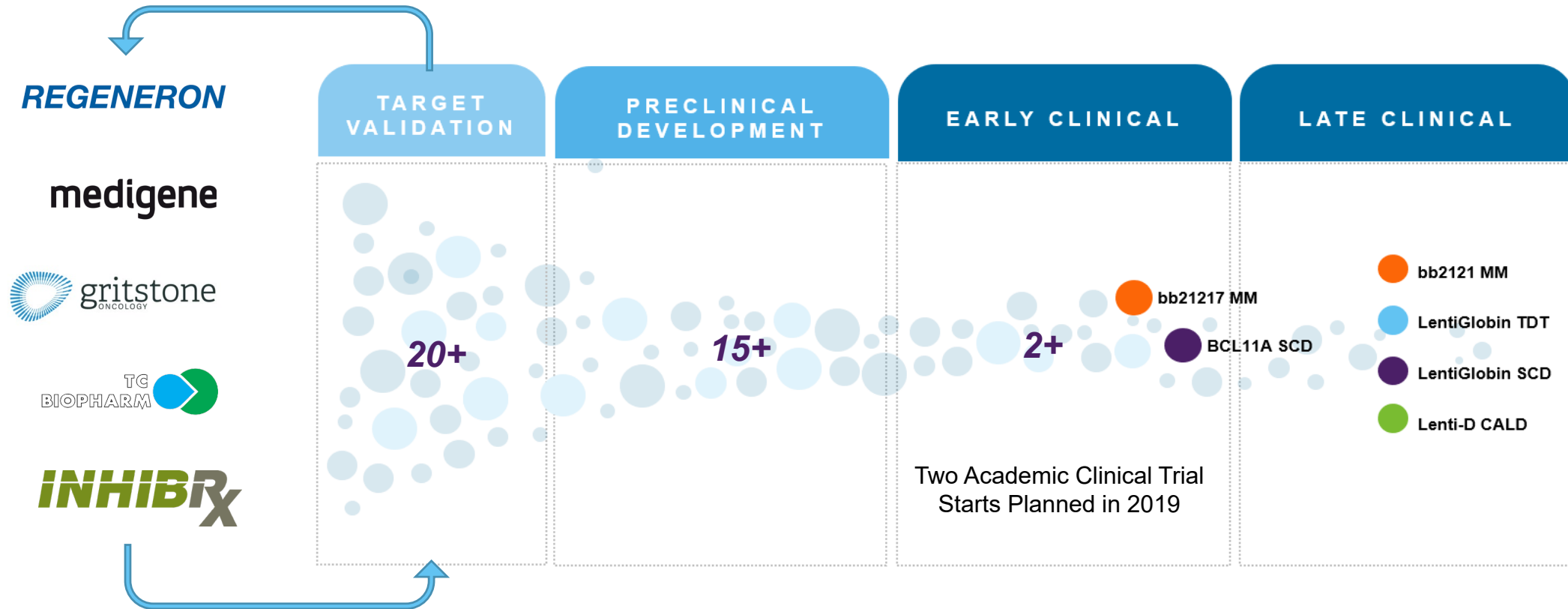


T CELL TYPES / ALLO

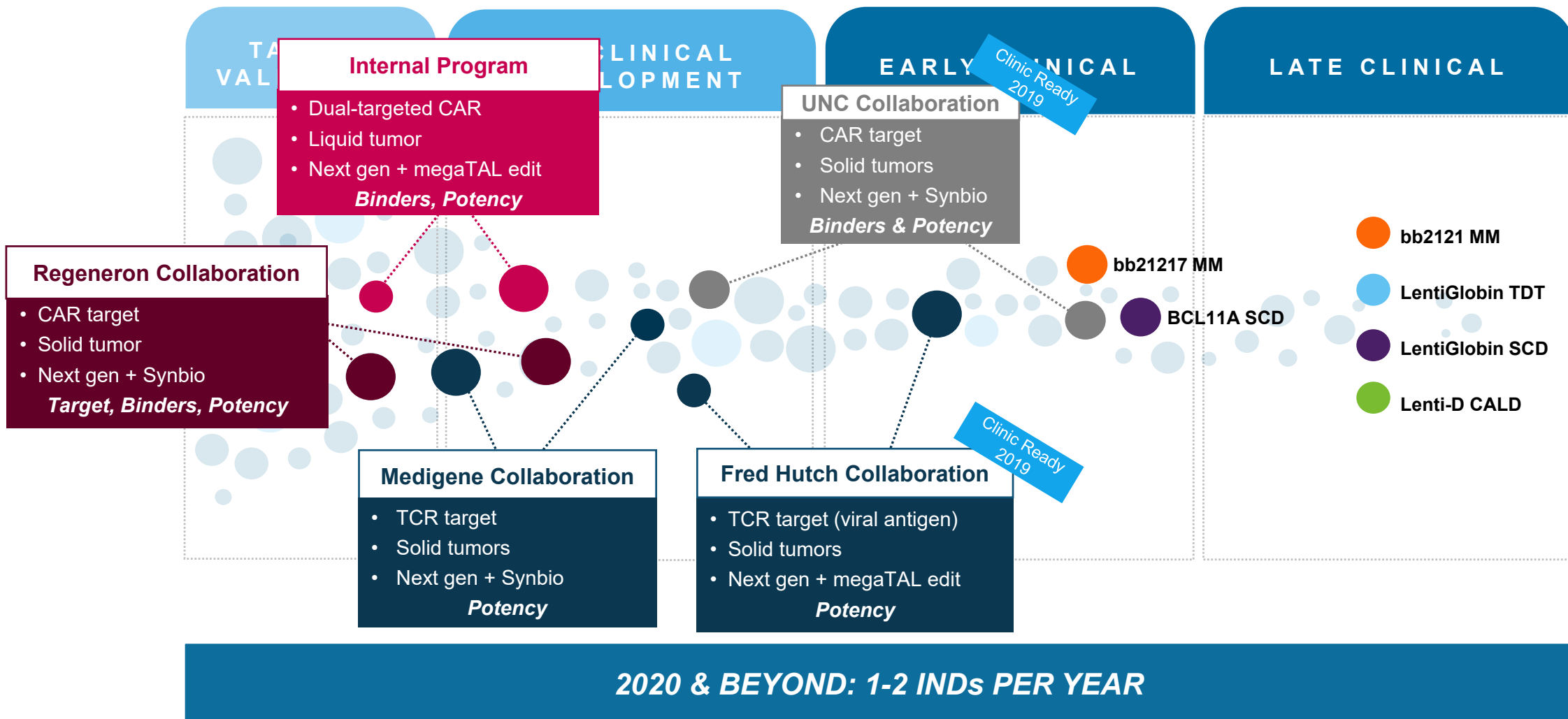


P13K Inhibition
Enhanced Mftg

Oncology Pipeline Enabled by Our Partners and Our Core Technologies



Research Strategy Yielding Emerging Oncology Pipeline



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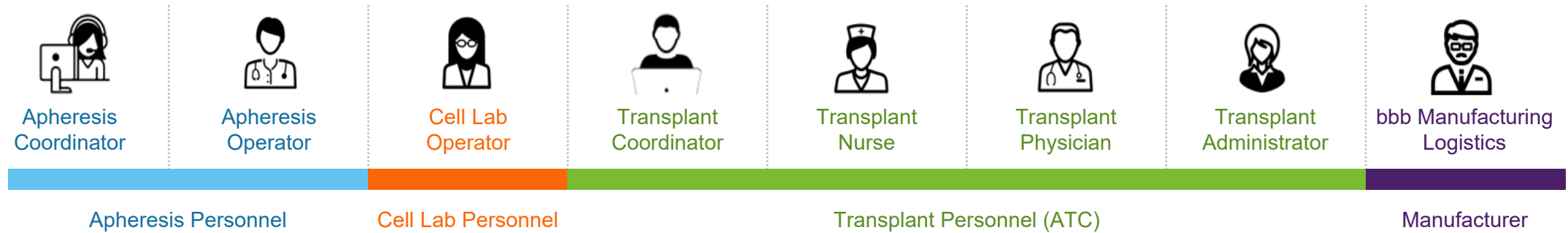
RECODE THE SYSTEMS: ANYTHING BUT TRADITIONAL



Platform Is Gearing Up for Launch

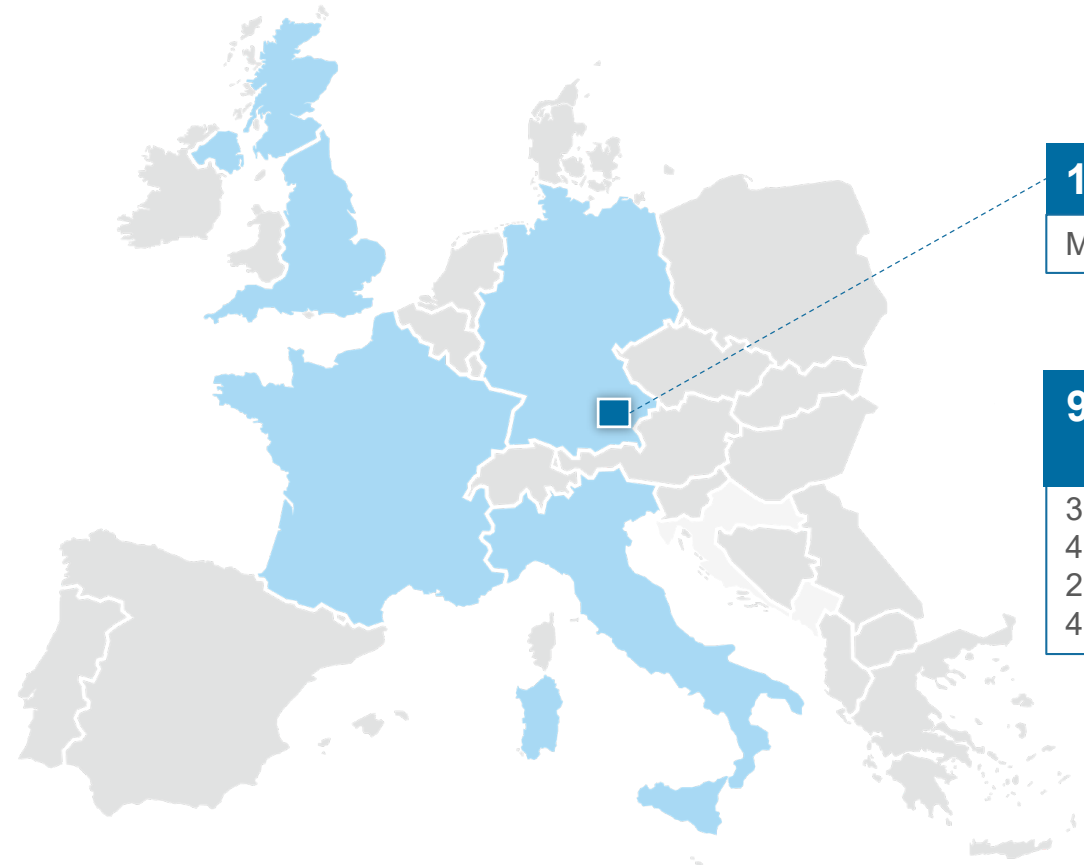


Preparing to Serve Patients in Europe in 2019



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)*

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Business of Cures – Complex with Lots of Questions, Perspectives, etc.

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”

BioPharmaDIVE

“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”**

Access and Reimbursement Model – A Major Opportunity Across Stakeholders

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

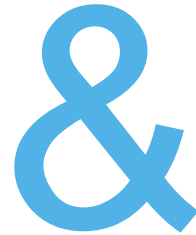
BLUEBIRD OPPORTUNITY

- **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

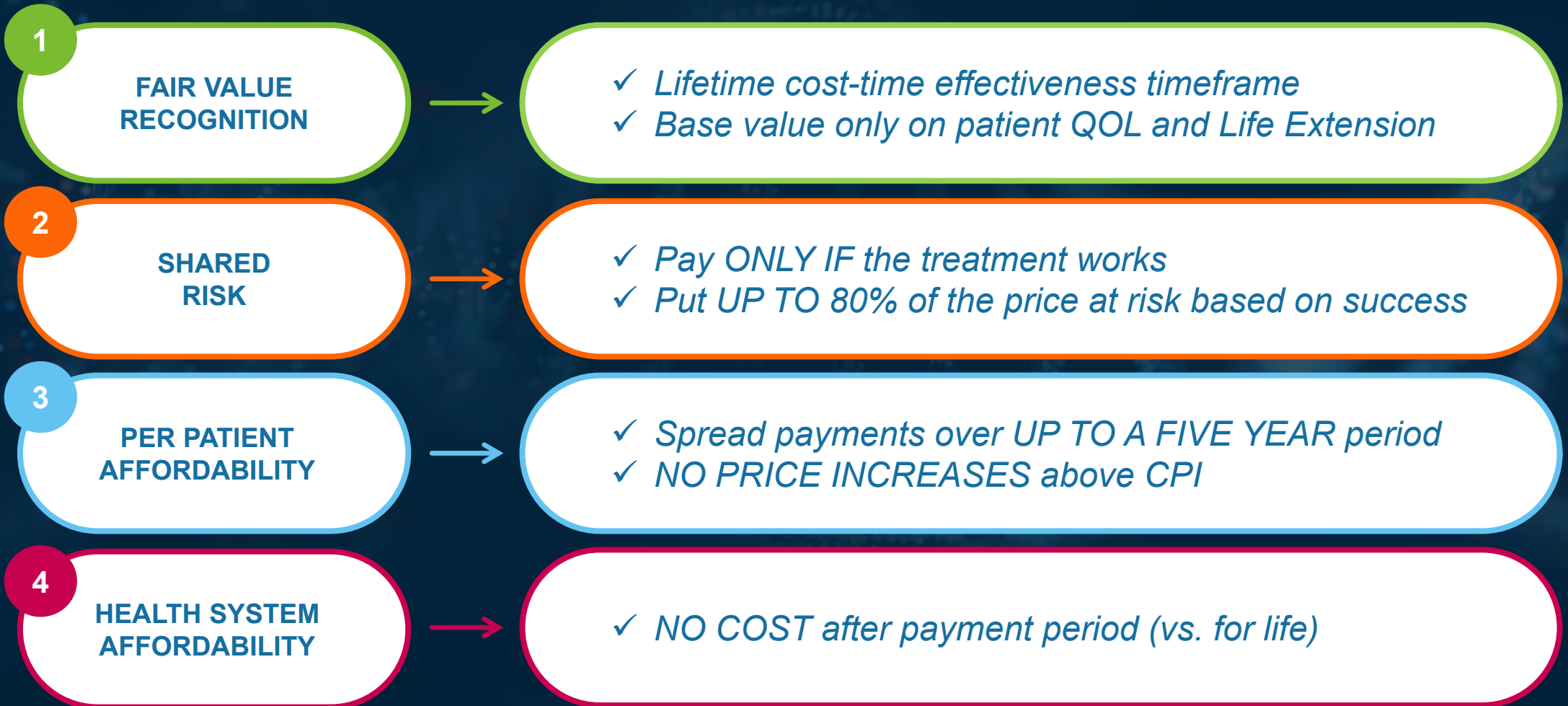
3 **BLUE** wants to make its treatments **AFFORDABLE** so **PATIENTS (and SYSTEM)** can actually
4 **REALIZE** the **VALUE** of its therapies

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

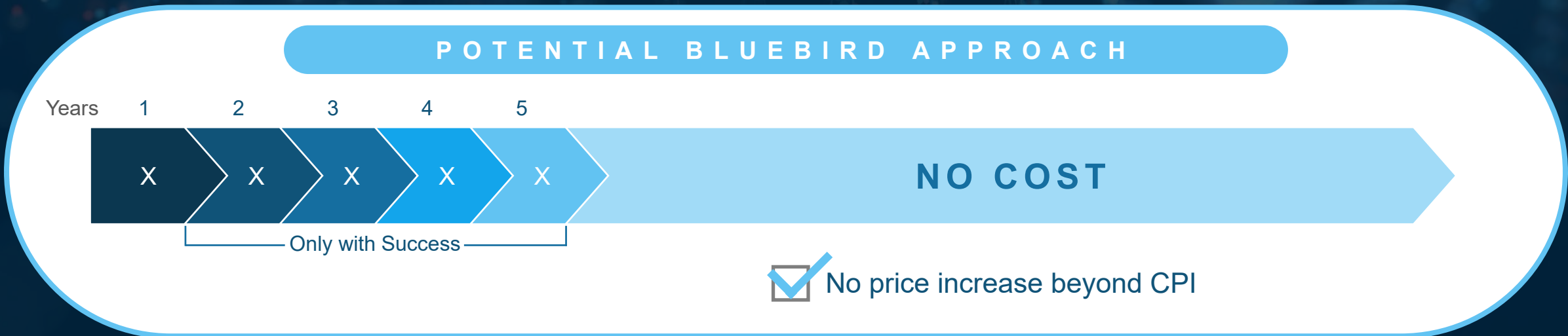
Approach – VALUE-BASED PAYMENT Over Time Based on OUTCOME

OBJECTIVE

STRATEGIC APPROACH

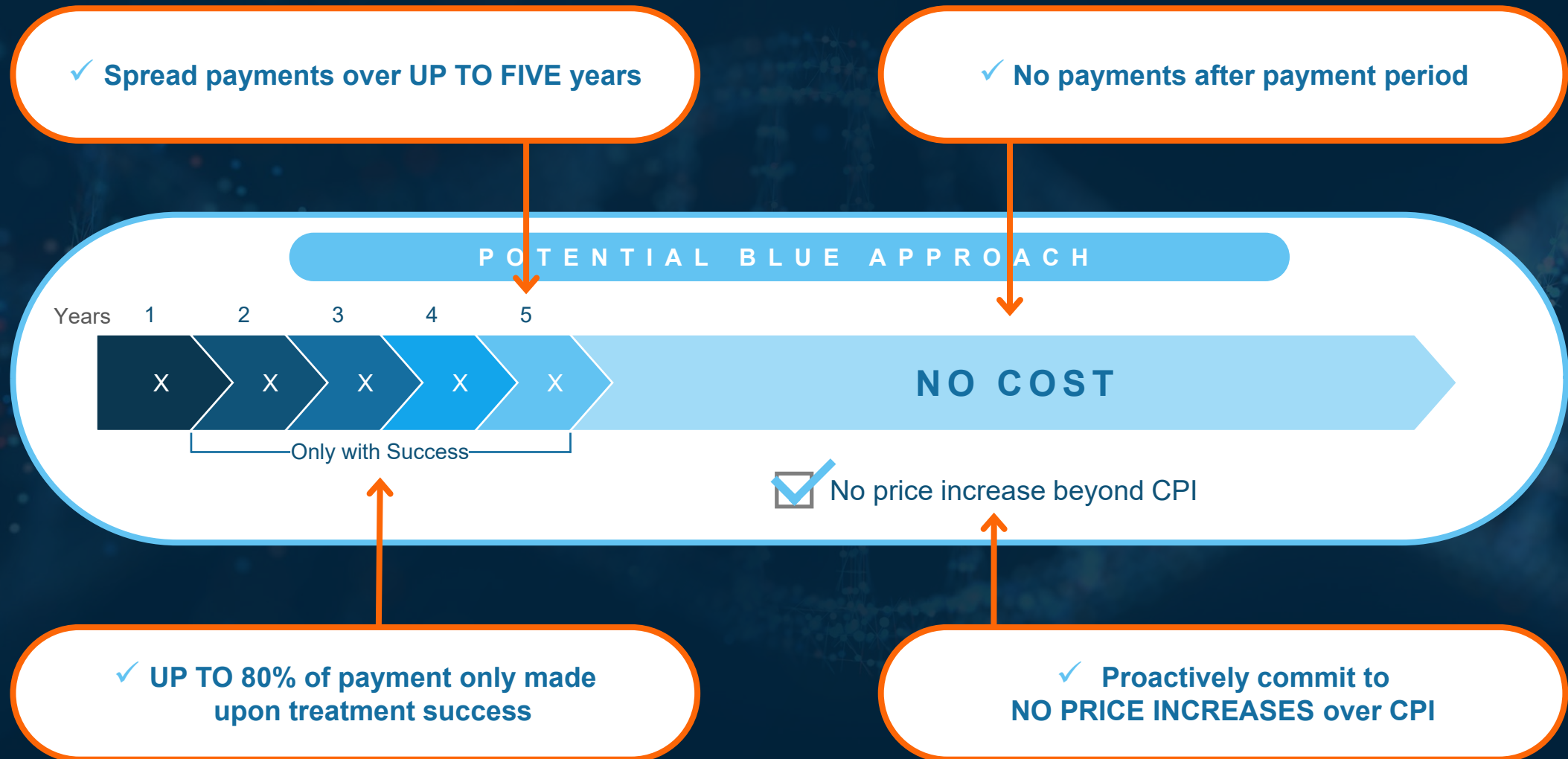


Payment Model – Patient and System Friendly

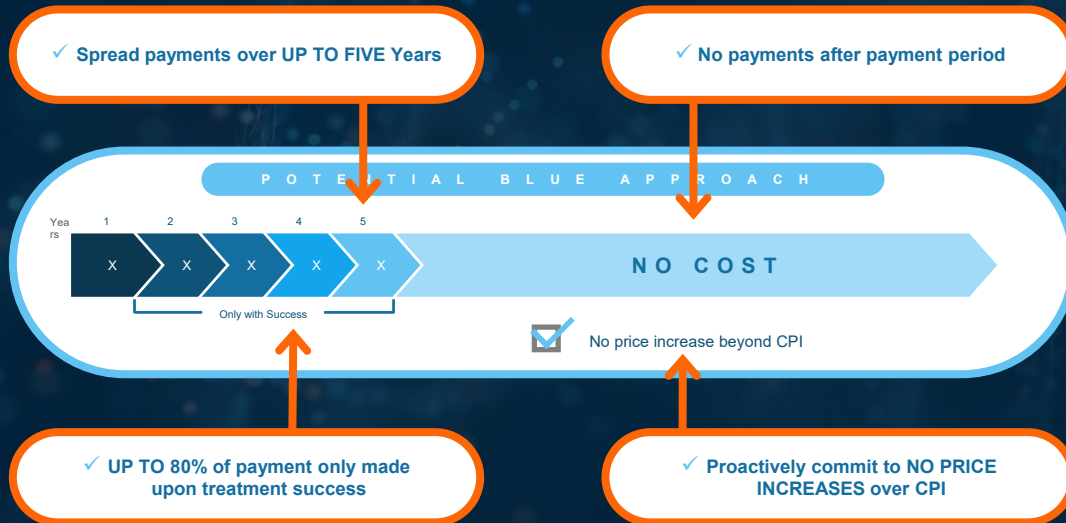


*Ranges based on 2018 U.S. list prices from a sampling of seven chronic rare disease therapies.

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

1

FAIR VALUE
RECOGNITION



STRATEGIC APPROACH

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

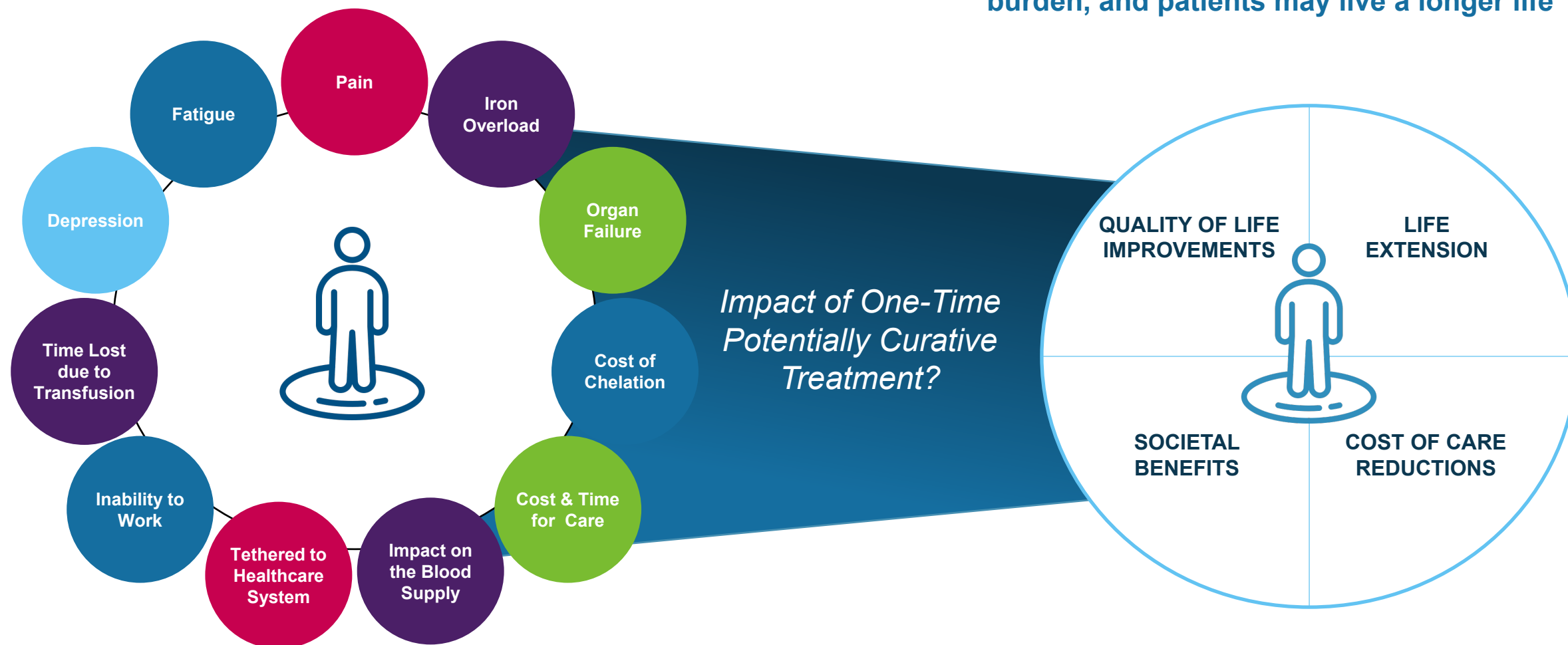
***What is the value that LentiGlobin can bring to
TDT patients, payers, system and society?***

AND how can it be fairly measured?

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

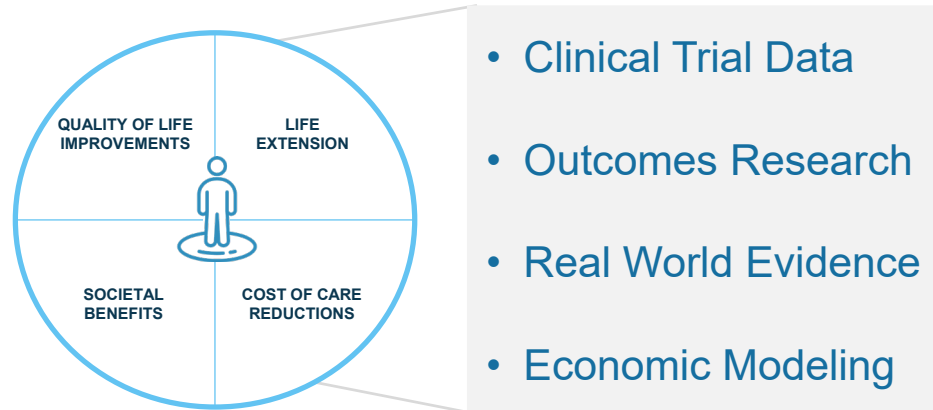
Disease and treatment-related burden builds up over time and can even lead to early death

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

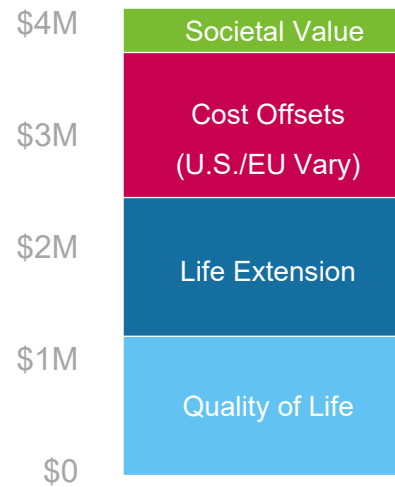


Cost Effective Analysis Focused on Actual Patient Value: QOL and Life Extension

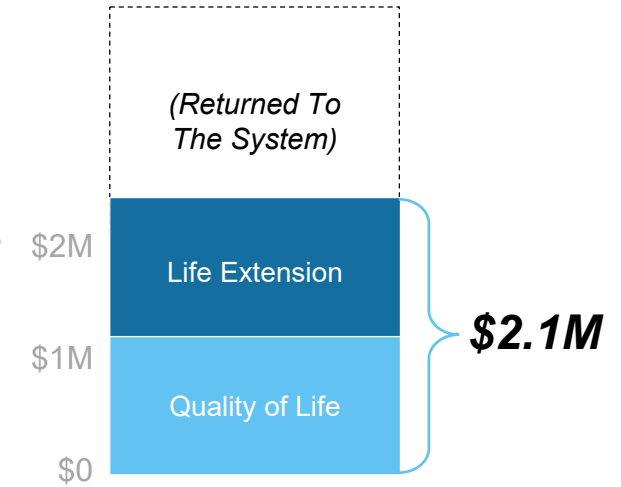
THE VALUE AT WHICH TREATMENT IS COST EFFECTIVE* (**NOT PRICE**)



Traditional
All Inclusive Calculation



LentiGlobin
Intrinsic Value



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

Catalyst for Change - Recode the Status Quo for the Better



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**

5

CATALYST FOR
CHANGE

WE RECODE FOR LIFE

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

