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Recoding in Action

JP Morgan 2020

LET'S RECODE THE STORY

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,” “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 initiate or complete, clinical studies, the timing or likelihood of regulatory filings and approvals or the requirements that may be imposed, and the timing and likelihood of entering into contracts with payors for value-based payments over time or reimbursement approvals, and our commercialization plans for approved products 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.

Must Beat the Odds.

Period.

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Antifragility is beyond resilience or robustness.
The resilient shocks and stays the same;
the **antifragile gets better.**

- Nassim Nicholas Taleb, author, *Antifragile: Things That Gain from Disorder*

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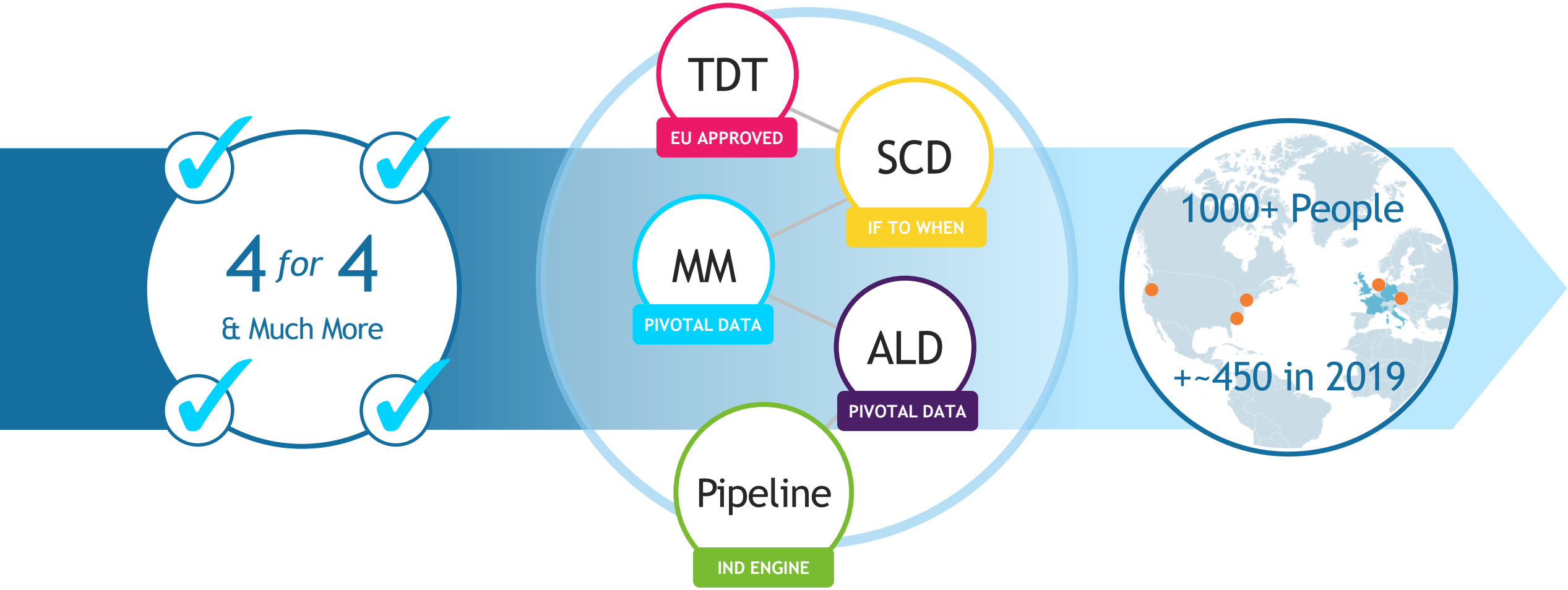
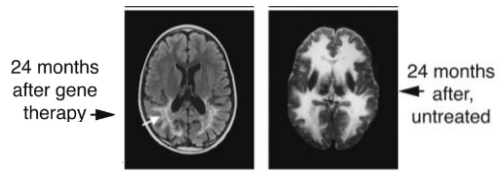
2019 - A Foundational Year



Cerebral Adrenoleukodystrophy - From Tragedy to Hope

2009

Science
AAAS



RECODE

Enhanced Construct
&
Manufacturing

EPNS: 2019

- ✓ 15/17 patients alive and MFD-free at 24 months follow up and continue to be MFD-free with up to 5 years of follow-up
- ✓ 32 total patients treated

Data as of April 25, 2019

2020

- **2H 2020 anticipated regulatory submission**
- **Newborn screening active in 14 US states; several pilot programs in EU**

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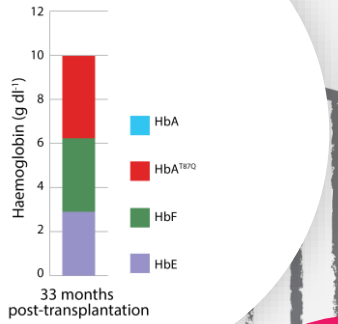
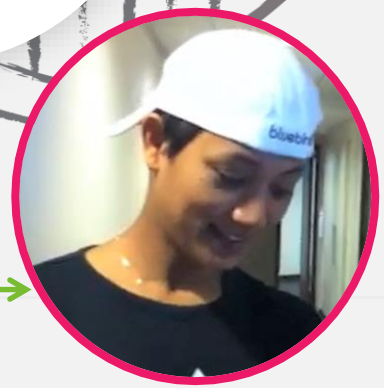
Science 2009

Transfusion-Dependent ß-Thalassemia - Reimagined Future

2010

nature





RECODE

Vector Potency &

Manufacturing

Enhancement

ASH 2019

- 90% of evaluable patients with a non-ß0/ß0 genotype achieved TI, with median average total Hb levels of 12.2 g/dL in Phase 3 Northstar-2 (HGB-207) study

Data as of June 12, 2019

zynteglo®

(autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene)

✓ **EU Approved 2019**

✓ **US rolling BLA initiated 2019**

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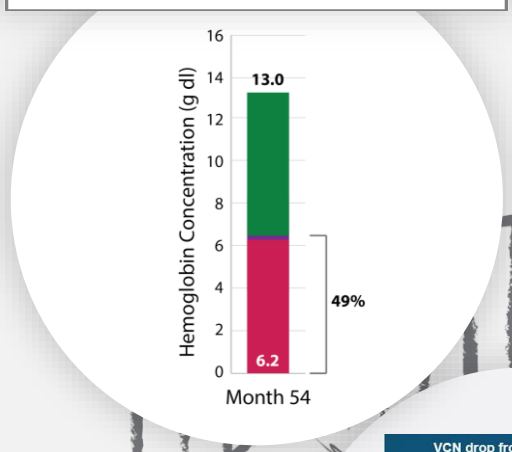
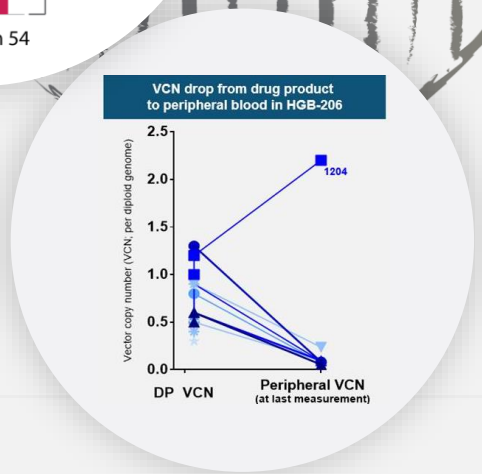
Nature 2010

Sickle Cell Disease – Daring to Dream

2017

The NEW ENGLAND JOURNAL of MEDICINE





RECODE

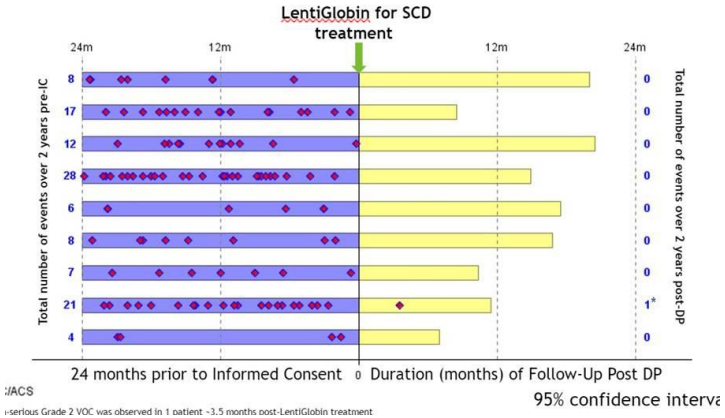
Pre-Tx Transfusions

More Thorough Conditioning

Higher Cell Dose

Higher VCN

ASH 2019



99% reduction in annualized rate of VOC + ACS*

- **Accelerated development underway**

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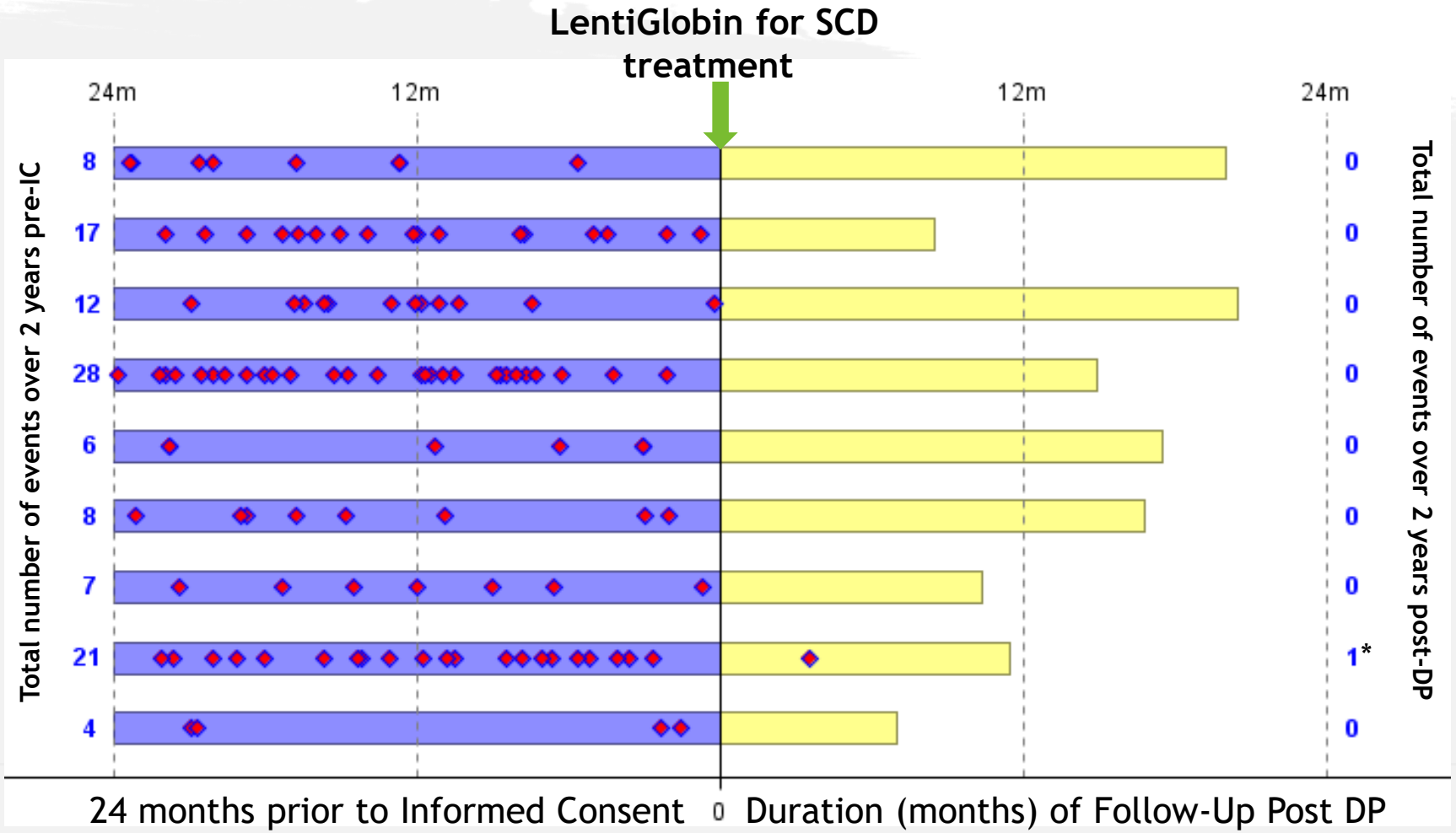
New England Journal of Medicine 2017

*HGB-206 Group C patients with history of VOCs and ACS who had ≥ 6 months of follow-up; data as of August 26, 2019

Sickle Cell Data – Take a Closer Look



*As previously reported, 1 non-serious Grade 2 VOC was observed in 1 patient ~3.5 months post-LentiGlobin treatment
Investigator-reported adverse events of VOC or ACS are shown; *Patients with ≥ 4 VOC/ACS at baseline before Informed Consent and with ~ ≥ 6 months of follow-up post-DP infusion
ACS, acute chest syndrome; VOCs, vaso-occlusive crises; DP, drug product

Data as of 26 August 2019

Multiple Myeloma – Changing What's Possible

Standard of Care*

- ~4 months PFS
- ~30% ORR
- ~3% CR

RECODE

BCMA Target &

Next-Gen CAR

2019 - KarMMa topline

- ✓ Positive Pivotal Data
- ✓ Met primary and secondary endpoints
- ✓ Deep and durable responses across dose levels

2020

- 1H 2020 anticipated U.S. BLA submission
- Ongoing studies in 3L, 2L and 1L (Newly Diagnosed)

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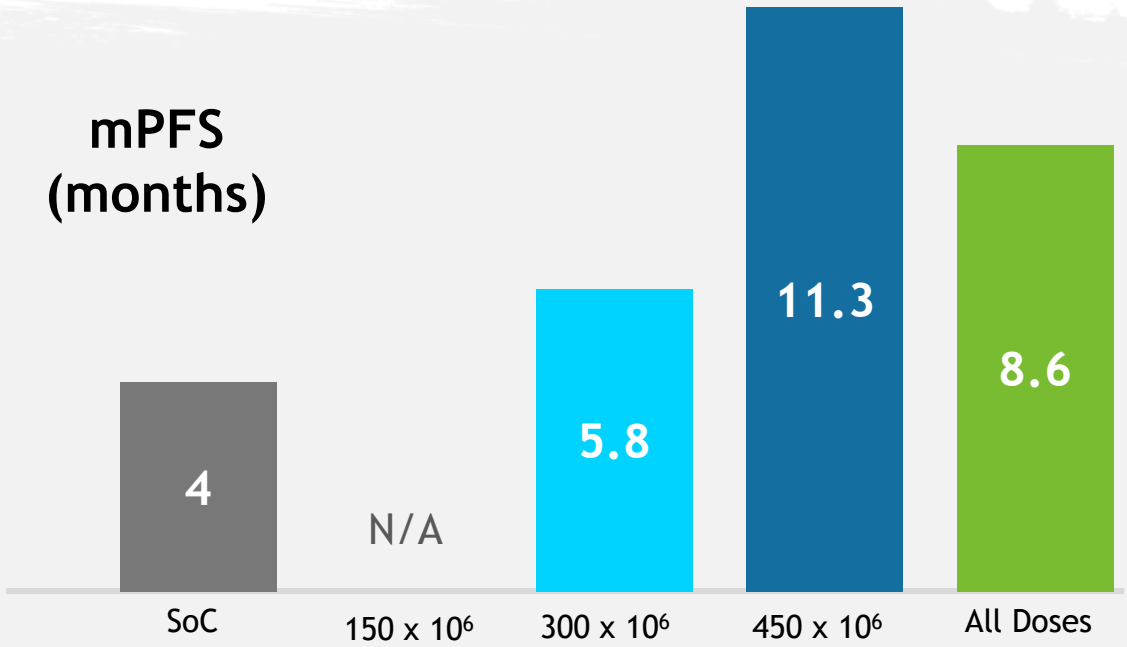
*Lonial et al, Lancet 2016 (Dara); Siegel et al, Blood 2012 (Kyprolis); Hajek et al, Leukemia 2017 (Kyprolis); Chari et al, NEJM 2019 (Selinexor); Richardson et al, Blood 2014 (PomDex)

ide-cel (bb2121) - Positive Pivotal Data



	150 x 10^6 CAR+ T cells (N=4)	300 x 10^6 CAR+ T cells (N=70)	450 x 10^6 CAR+ T cells (N=54)	All Doses (N=128)
ORR, n (%)	2 (50.0)	48 (68.6)	44 (81.5)	94 (73.4)
CR/sCR, n (%)	1 (25.0)	20 (28.6)	19 (35.2)	40 (31.3)
Median DoR, mo	---	9.9	11.3	10.6

Heavily pretreated population

- 94% refractory to anti-CD38, 84% triple refractory
- All patients were refractory to their last treatment (progression during or within 60 days of last therapy)

Deep and durable responses across dose levels

- mPFS of >11mo at the 450 x 10^6 dose
- Durability is consistent across doses

Safety consistent with the Ph1 data

- Gr ≥ 3 CRS and iiNT were reported in <6% of subjects at each target dose
- CRS and iiNT of any grade occurred in 83.6% and 18% of patients, respectively

iiNT: investigator identified neurotoxicity
Ide-cel is being developed in collaboration with Bristol-Myers Squibb

Intense & Steep Innovation Curve – R&D With a Soul

RESEARCH INNOVATION ENGINE

- PLATFORM TOOLS & TECH
- ACADEMIC COLLABORATORS
- INDUSTRY LEADING PARTNERS

→ NEXT GEN PRODUCT CANDIDATES

CLINICAL EXPERIENCE

- MM – bb21217
- SCD – BCL11a
- MCC – MCC1 TCR

- ZYNTEGLO™ for β-thalassemia
- ide-cel for MM
- LentiGlobin for SCD
- Lenti-D for CALD

ASK & ANSWER ENGINE

apceth BIOPHARMA · Boston Children's Hospital Until every child is well · Bristol-Myers Squibb · gritstone ONCOLOGY · INHIBRX · FortySeven · FRED HUTCH CURES START HERE · Hitachi Chemical Working On Wonders · Lonza · medigene · University of Minnesota · novo nordisk · REGENERON · Seattle Children's HOSPITAL · RESEARCH · FOUNDATION · TC BIOPHARM

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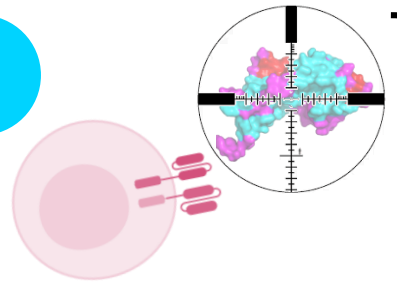
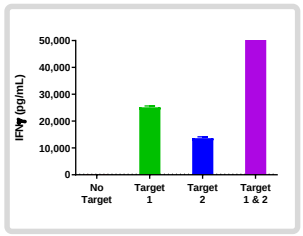
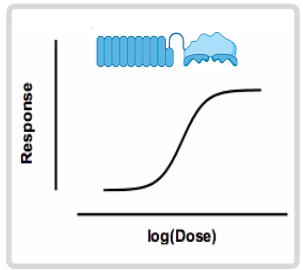
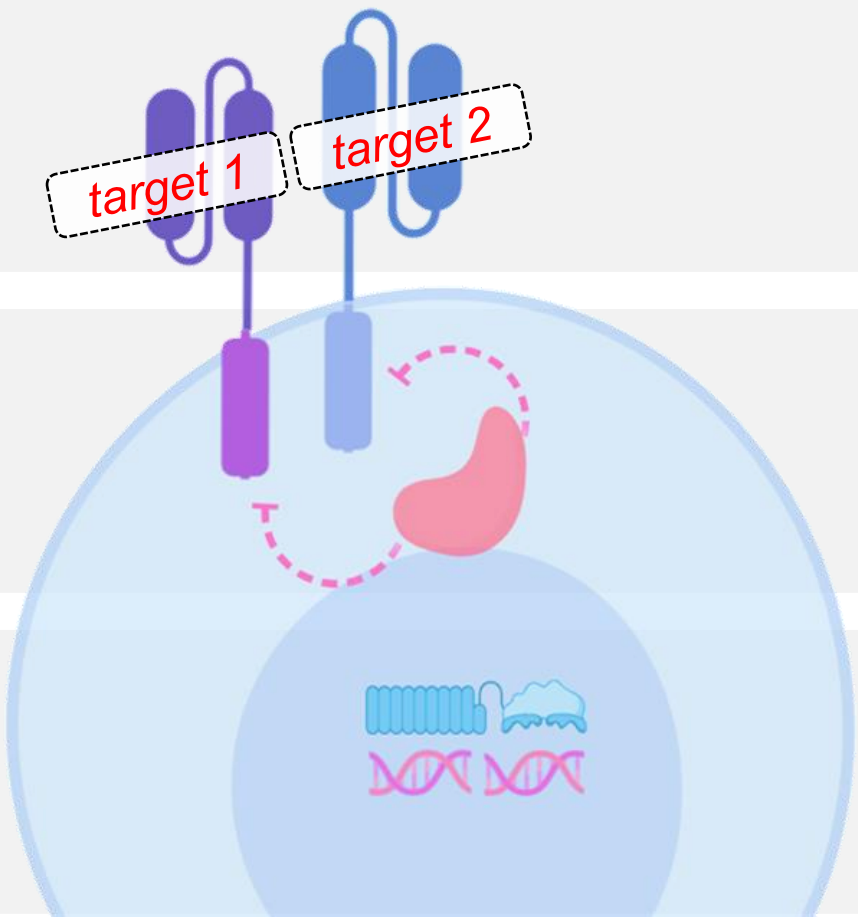
Diffuse Large B-Cell Lymphoma -Triple Threat Approach

	LAYER	PURPOSE
1	*dual-CAR targeting*	prevent escape
2	*signal extension*	enhance T cell activation
3	*signal amplification*	improve T cell persistence









EACH LAYER INFORMS 1:MANY PLATFORM

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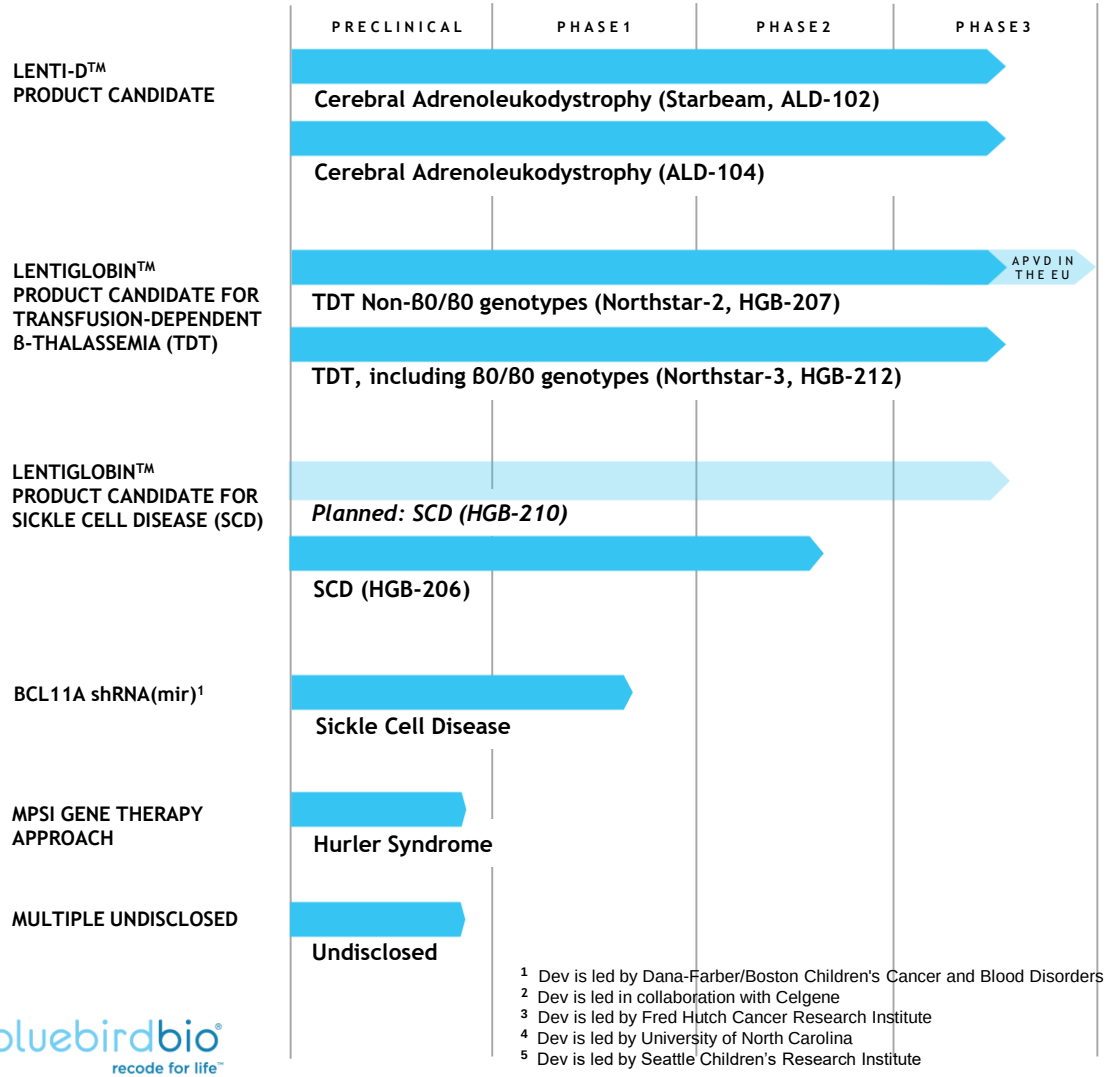
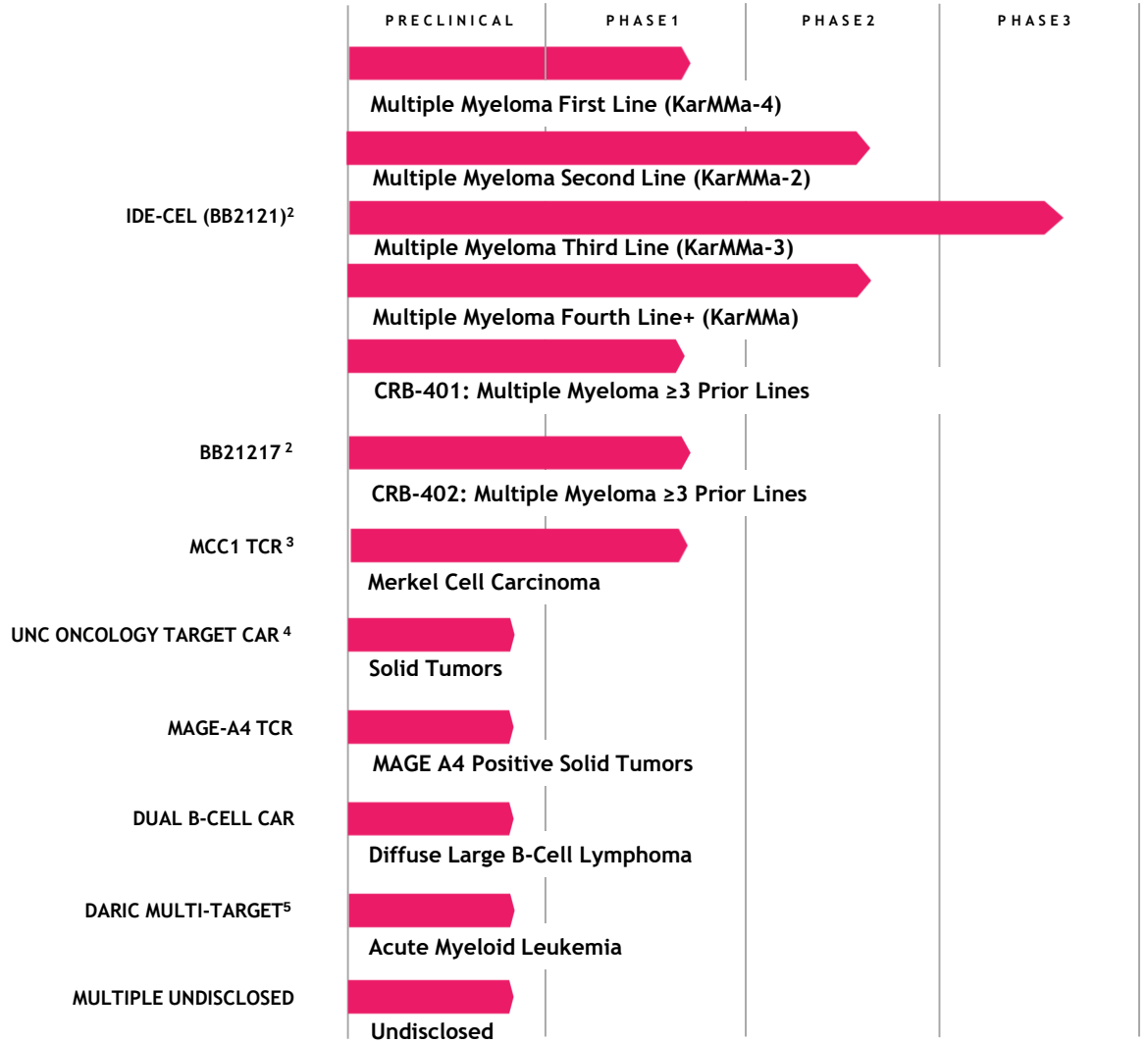
Engine Starting to Deliver

Severe Genetic Diseases



Oncology



[1] Dev is led by Dana-Farber/Boston Children's Cancer and Blood Disorders Center
[2] Dev is led in collaboration with Celgene
[3] Dev is led by Fred Hutch Cancer Research Institute
[4] Dev is led by University of North Carolina
[5] Dev is led by Seattle Children's Research Institute

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Path to Patients - Underappreciated Opportunity

INTEGRATE

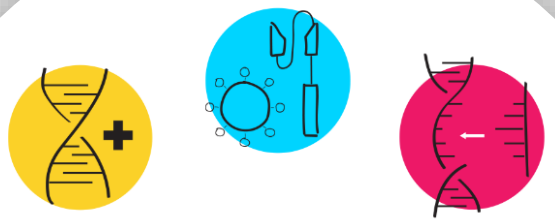
TRANSLATIONAL ENGINE DELIVERING

ITERATE

Make and Scale

Access and Value

Show Up bluestyle



FULLY INTEGRATED CELL AND GENE THERAPY PRODUCTS COMPANY

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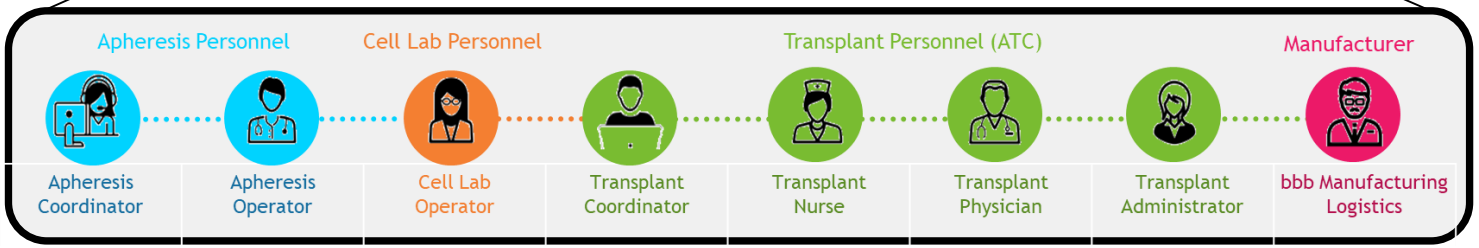
Path To Patients - Making It Happen In The Real World

SUSPENSION

Treating Patients At Scale

COMMERCIAL QUALIFIED TREATMENT CENTERS (QTCs) & DRUG PRODUCT

Delivering For Commercial



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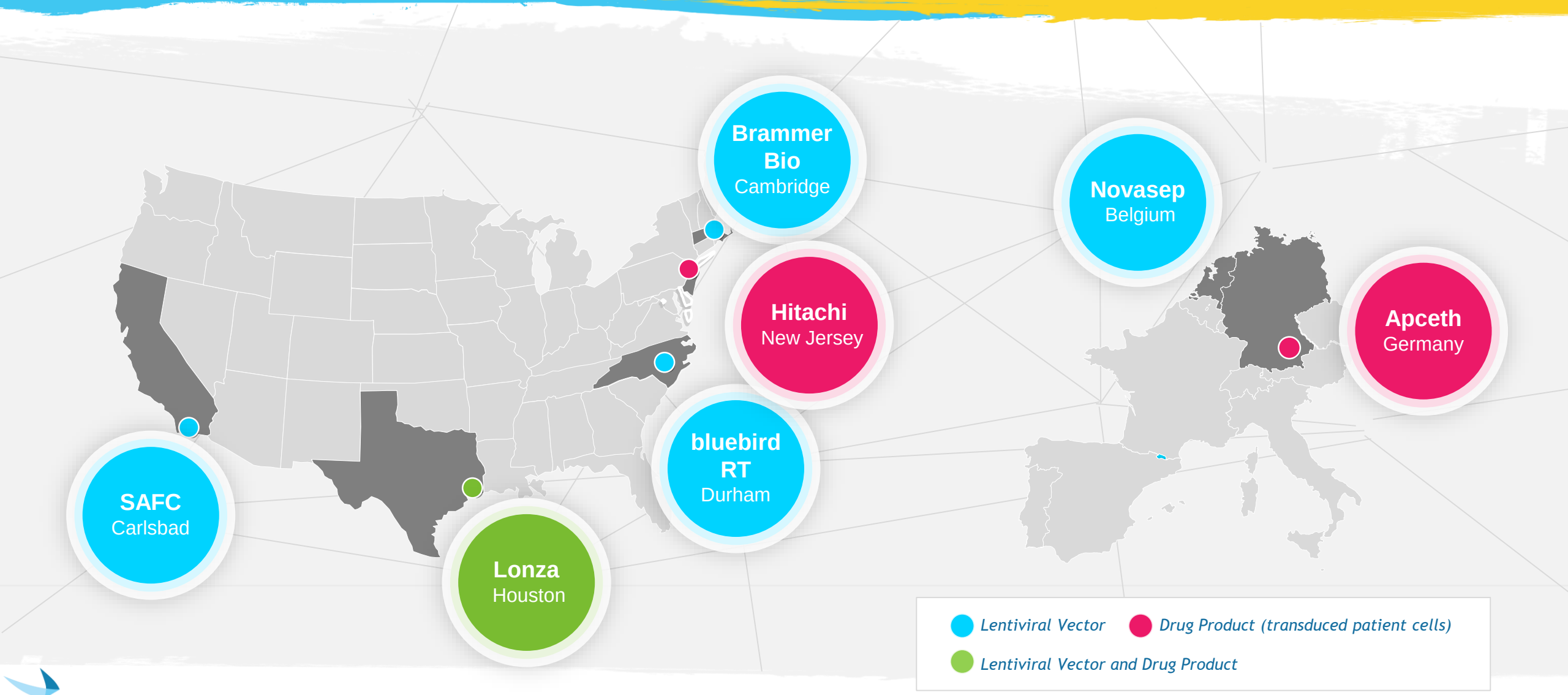
Manufacturing Network Strategy - Product Supply Through Both Internal Capacity & Contract Partners



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Access & Value – As An Ecosystem We Can ALL Do Better

Poor Behaviors Persist

- EPS & Q2Q Driven Mindset
  - Because-You-Can Pricing
  - Unexplainable Price Increases
  - Excessive Innovator Rewards - Top 10 Medicines Anticipated ~$1.4 TRILLION in Revenue Through 2024*
- Defending the Status Quo
  - Biosimilar Resistance and Patent Extensions

Good Behaviors Gaining

- Transparent Engagement Across Stakeholders
- Limiting/Eliminating Price Increases
- Resolving Patient Co-Pay and Access
- New Ideas Taking Hold (And Expected)
  - Prevalence Based Revenue Capping
  - At-Risk / Outcomes Based Payments
  - EQRx Newco – "Me Too @ Lower Cost"

Source: EvaluatePharma

bluebird Value Principles and Proposed Payment Model

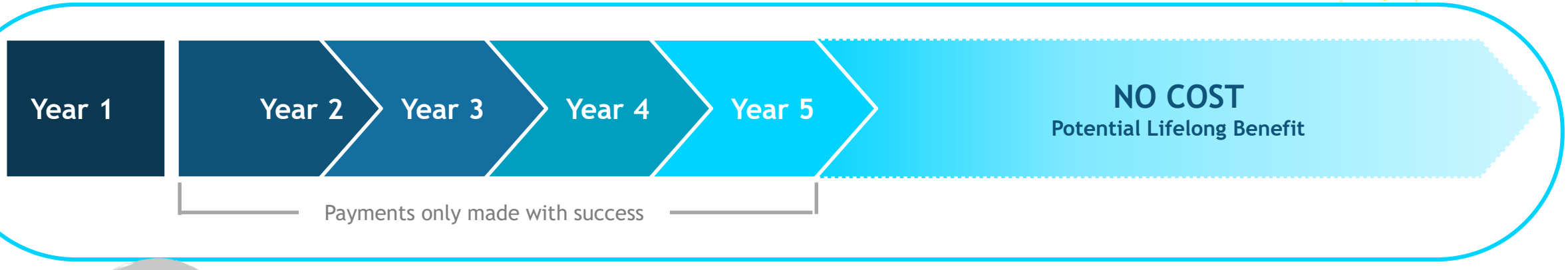
Fair Value Recognition
QoL & Life Extension

Share Risk
Up to 80% at Risk

Consider Patient Affordability
Payment over ~5 years

System Affordability
Capped Lifetime Cost

BE RESPONSIBLE, FUND INNOVATION AND DON'T DO STUPID STUFF



zynteglo®

- ✓ Estimated cost effective value $3-4 million, ~$2M based on life extension and quality of life (excluding cost offsets)
- ✓ Lifetime cost if successful: $1.76* million (€315K/year, capped at 5 years)

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Based on exchange rate of 1 Euro = $1.11 USD on January 7, 2020

Establishing Promising Access & Value Foundation

EU Launch Readiness

- ✓ First ever at-risk value-based agreement signed with multiple Sick Funds in Germany (~50-70% of patients in Germany covered)
- ✓ Team in place in Zug, UK, France, Italy, Germany, and Nordic Markets
- ✓ Qualified Treatment Centers and manufacturing ready in Germany

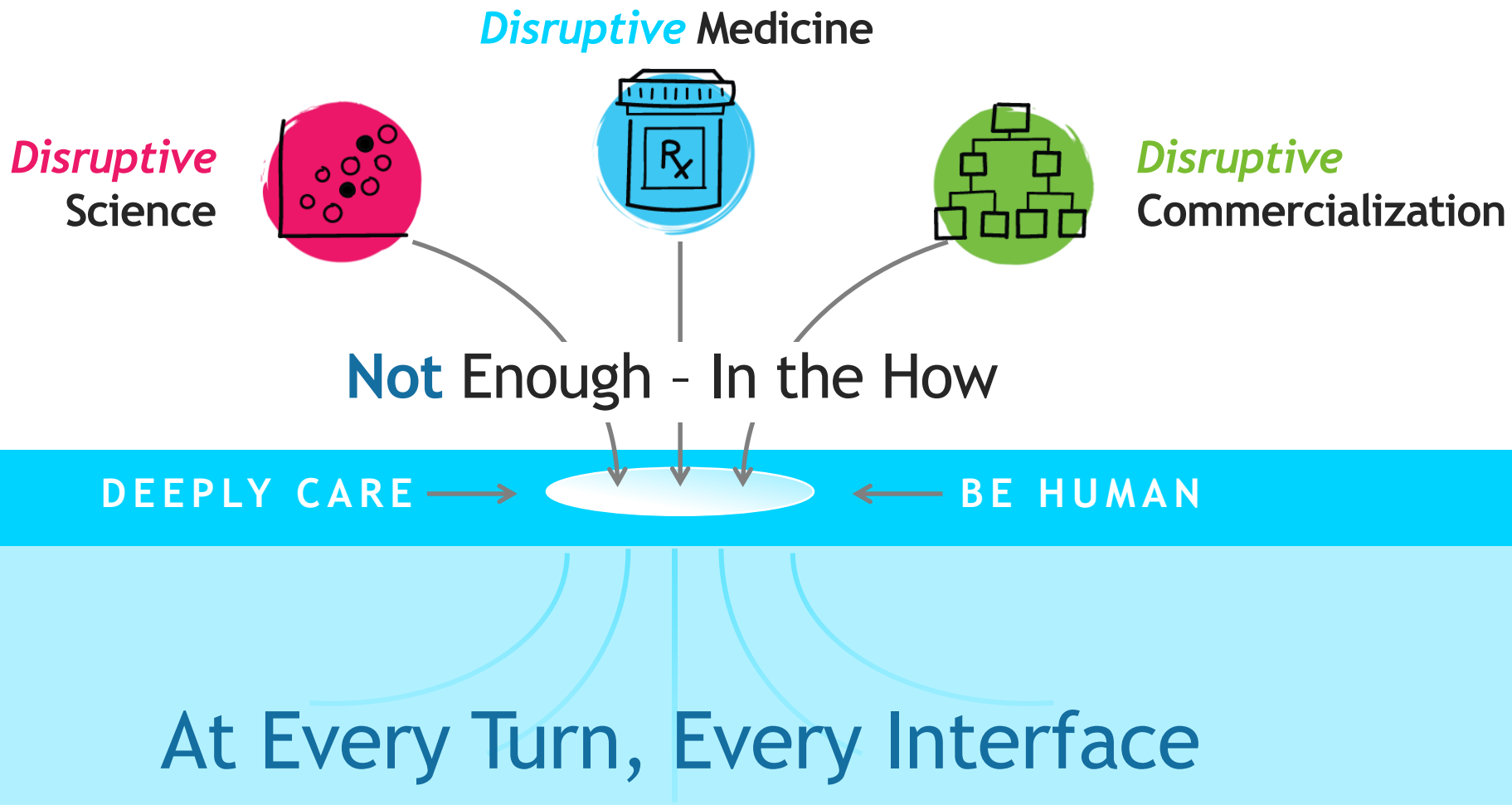
U.S. Launch Readiness

- ✓ Team in place for U.S. commercialization
- ○ Payers (Commercial) – Actively engaging to enable access & value-based payment over time at launch
- ○ Policy (State & Federal) - Focused on enabling value-based payment over time in commercial and for Medicaid markets to drive access
- ○ Distribution – Establishing customized distribution model to serve QTC & payer needs

Market and Patient Engagement

- ✓ Disease Education and outreach in place
- ✓ Patient Advocacy education and initiative support

Path to Patients - Show up bluestyle



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Just Different - Our Biggest Compliment

Apheresis Centers & Staff

Institutions/Doctor's Office

Families & Patients

bluestyle

- Truly put patients first – care deeply and be human
- Focus on enabling best decision for patient/family
- Seamless gene therapy delivery for optimized patient experience
- Relationships = Trust = Better Care
- Listen and Learn
- Improve every day

What if this were you, your family, your child?

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Planned 2020 Milestones – Distilling to Practice

	FIRST HALF 2020	SECOND HALF 2020
Regulatory Submissions	• Ide-cel (bb2121) MM U.S. BLA submission • ZYNTEGLO completion of U.S. BLA submission	• Lenti-D CALD EU MAA and U.S. BLA Submissions
Clinical Updates	• Ide-cel (bb2121) KarMMa and CRB-401 data* • LentiGlobin SCD Phase 3 HGB-210 study start	• Lenti-D ALD-102 data update • Zynteglo Phase 3 (HGB-207 and HGB-212) data • LentiGlobin SCD HGB-206 data and regulatory update
Commercial & Foundation Building	• ZYNTEGLO first commercial patients treated • ZYNTEGLO QTC and Sick Fund contracts in place	• ZYNTEGLO Access and Reimbursement in additional EU countries established • ZYNTEGLO and ide-cel U.S. launch ready • 1-2 New INDs

CASH RUNWAY INTO SECOND HALF 2021

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*submission for data presentation planned in H1:20

A Bold Vision in 2019 - Becoming a Reality in 2020

1: Many translational engine delivering

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everyone deserves a bluebird day!




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