



bluebirdbio®

PATH TO PATIENTS

**36th Annual J.P. Morgan
HEALTHCARE CONFERENCE**

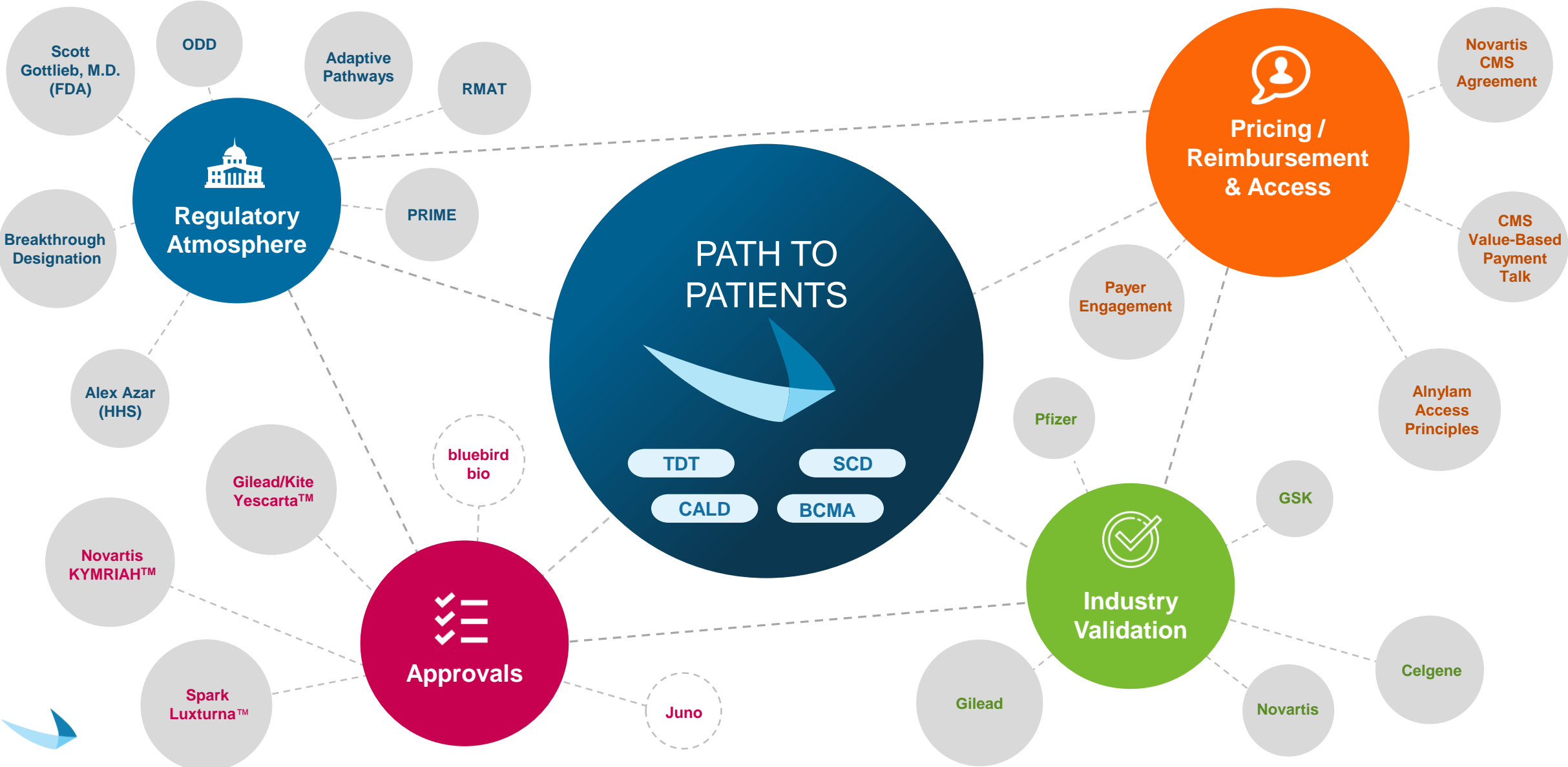
January 9, 2018

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

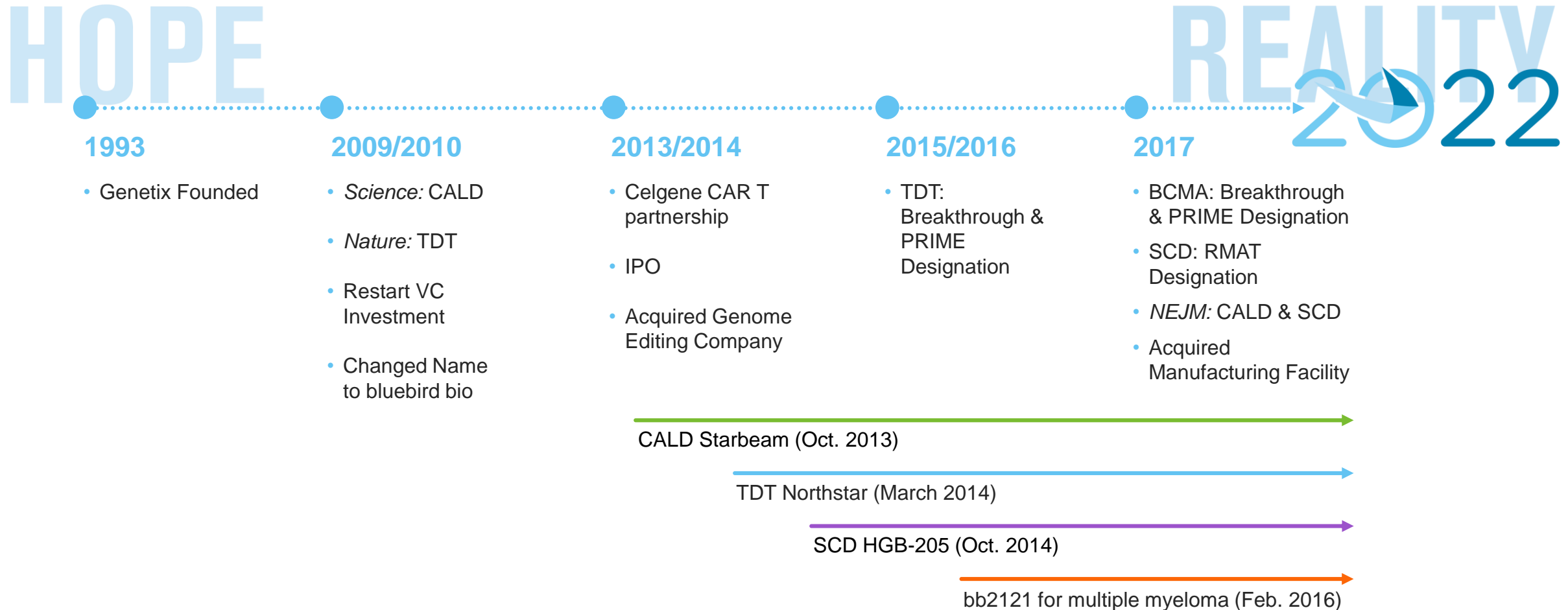
Lead The Way

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

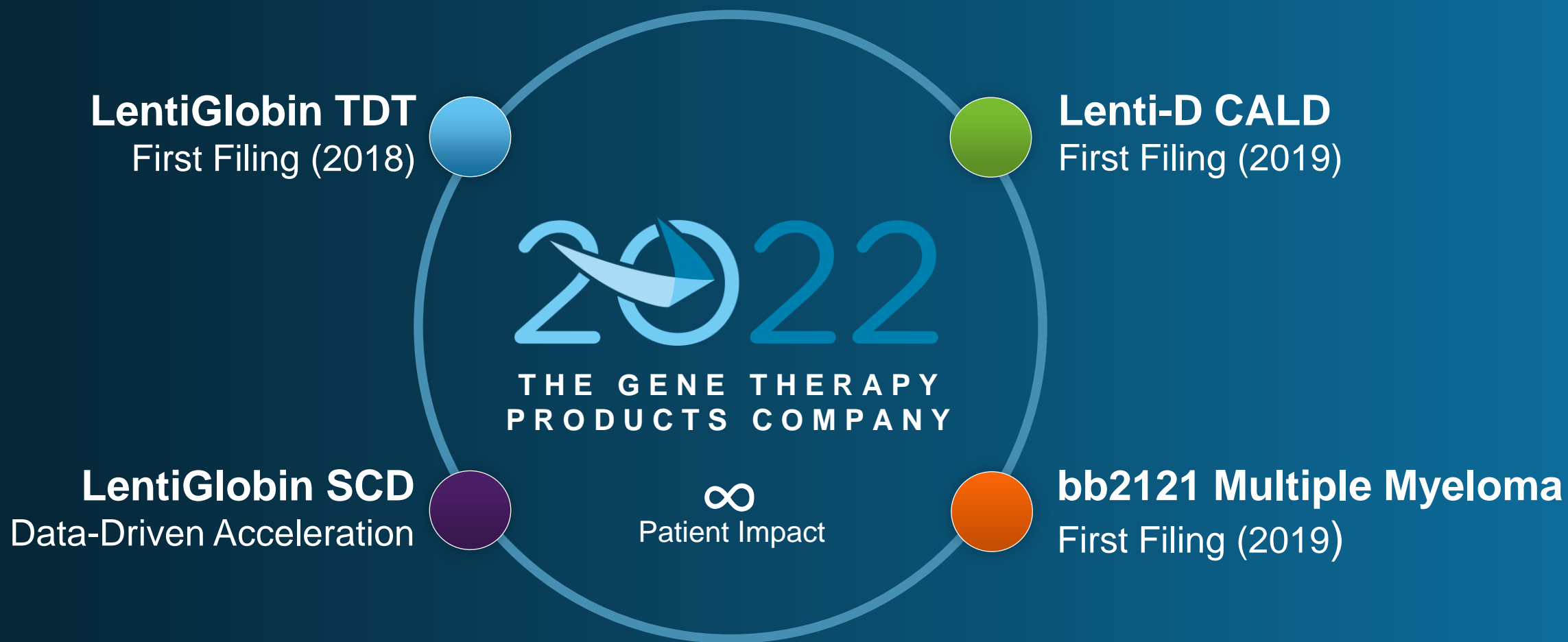
Stay BLUE

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

Hopes & Dreams Becoming a Reality



Three Regulatory Filings Anticipated by End of 2019



2+ Products
on the Market

2+ Programs Nearing
Commercialization

4+ Additional Programs
in the Clinic

2018 Milestones

BY MID YEAR**

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

BY END OF YEAR**

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

**\$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.

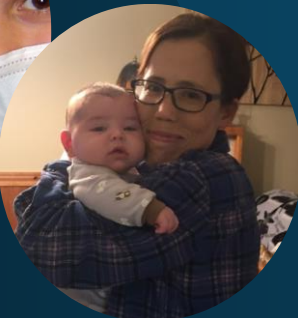


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Making Hope
A Reality

bluemojo





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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

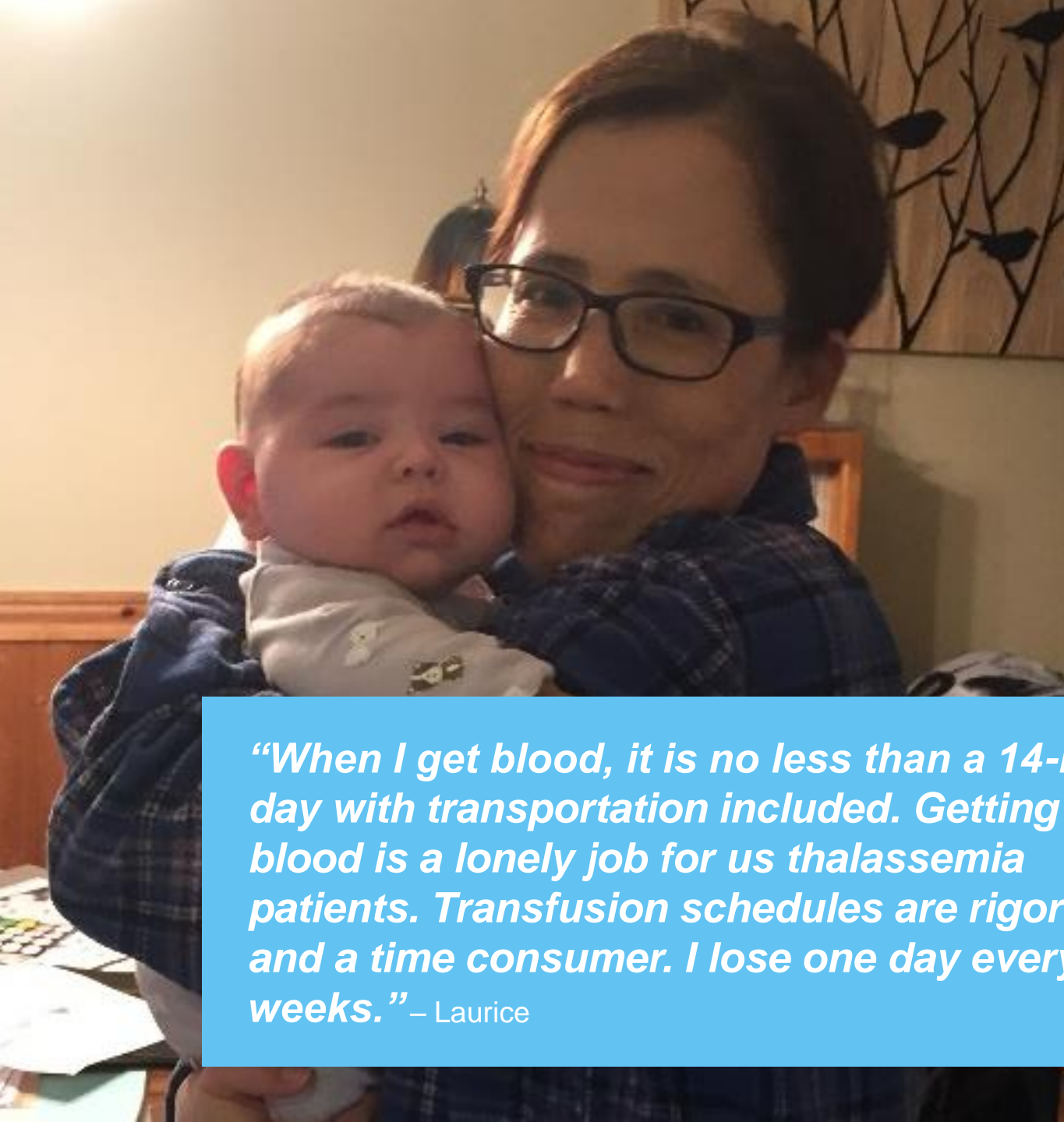
STATUS

- 15/17 patients hit the primary endpoint so far
- Newborn screening active in 5 states¹

NEXT STEPS

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

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



“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 β -thalassemia

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

STATUS

- Majority of patients with non- β^0/β^0 genotype are free of transfusions
- Refined manufacturing leading to robust increase in HbA^{T87Q}
- 3+ years durability of effect in early studies

NEXT STEPS

- Anticipated first regulatory filing in EU in patients with non- β^0/β^0 genotypes in 2018



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

“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



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

“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

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OUR PEOPLE

Driving the Product Platform to Reality for Patients

Make & Scale It

Relentlessly Learn & Innovate

Deliver It

Relentlessly Learn & Innovate

Value It

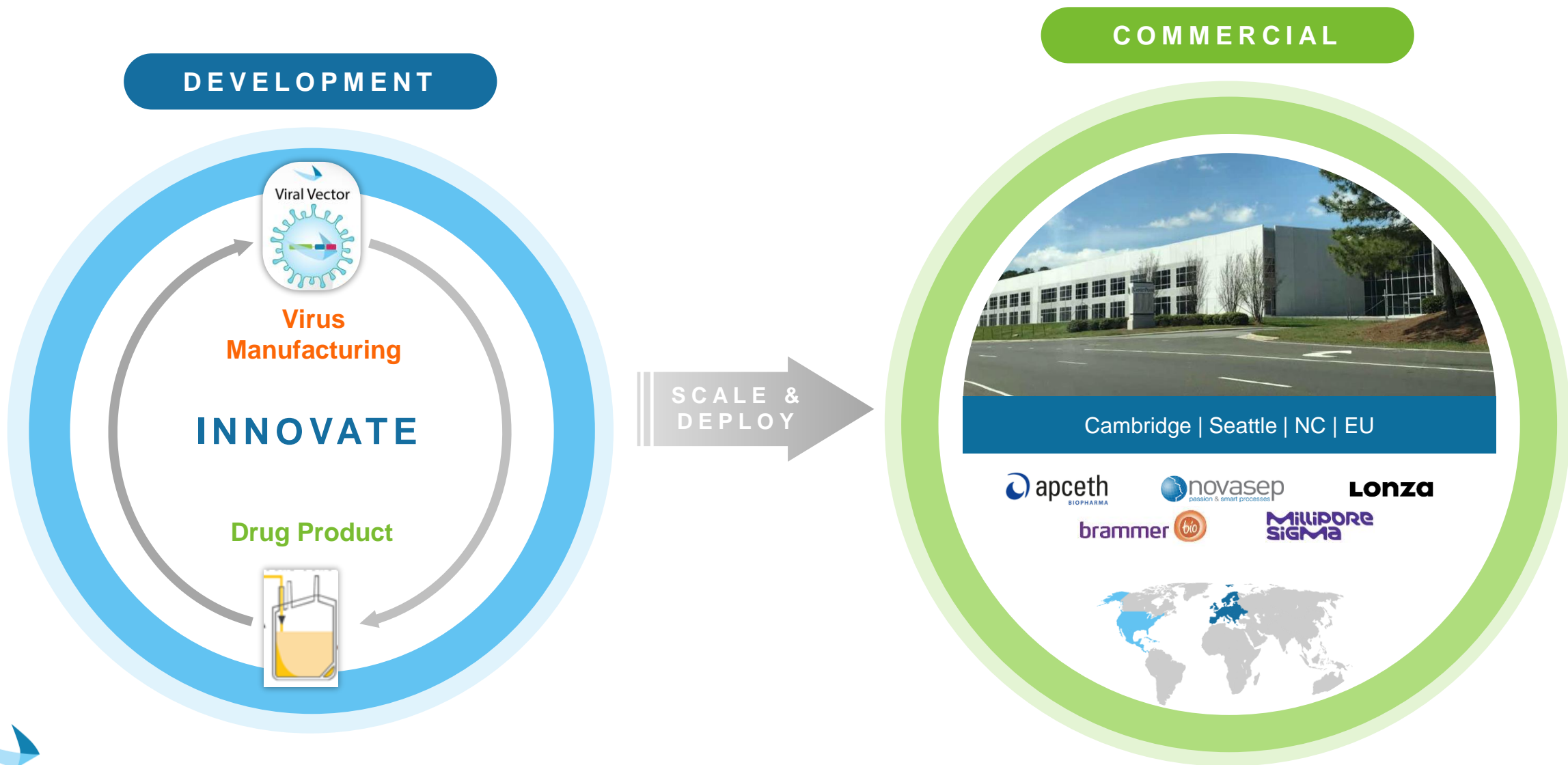
Relentlessly Learn & Innovate

Lever It

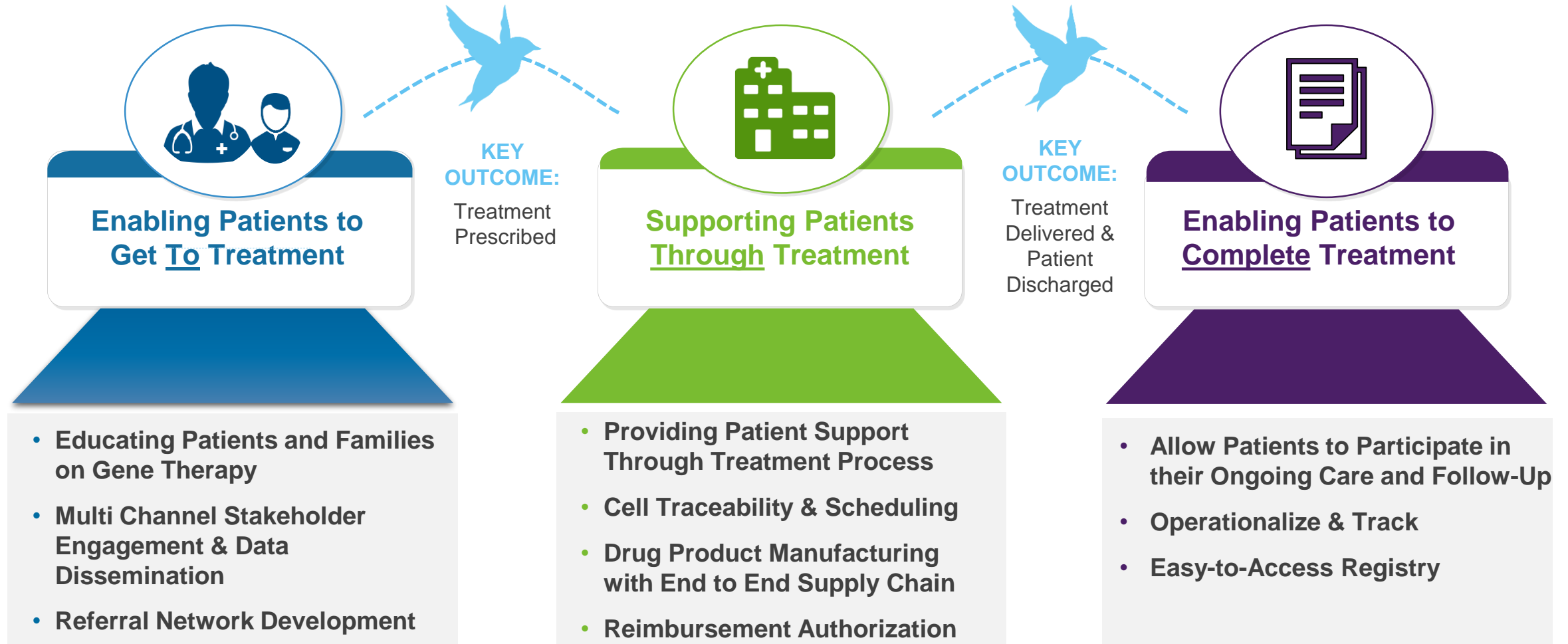
Relentlessly Learn & Innovate



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



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



Patient Case Management, Navigation, & Services

Value It: Time to Get It Right



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

Lever It: Experience, Capabilities and Partnerships Driving Pipeline Expansion

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 |
|--------------------------------------|-------------------------------------|-------------|-----------|-----------|----------------|
| Severe Genetic Diseases | | | | | |
| 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 |
| Cancer | | | | | |
| bb2121 | Multiple Myeloma | | | | Celgene |
| bb21217 | Multiple Myeloma | | | | Celgene |
| Undisclosed Targets | Various Indications | | | | Worldwide |
| Early Research | | | | | |
| Early Pipeline | Undisclosed + Gene Editing | | | | Worldwide |

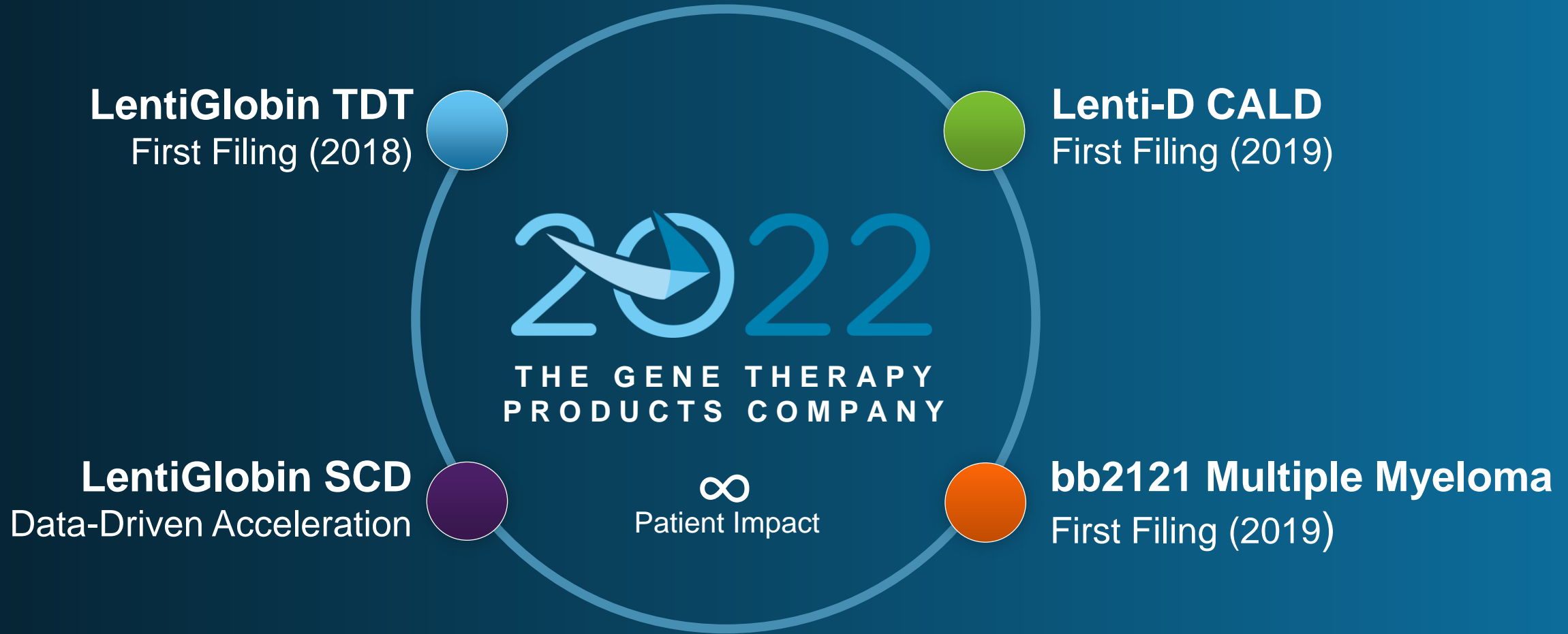
*Development is led by Boston Children's Hospital

COLLABORATORS



Path to Patients

Three Regulatory Filings Anticipated by End of 2019



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on the Market

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Commercialization

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in the Clinic

Go TRUE BLUE

*We Must
Make Hope a
Reality*

