



# Taking flight – ZYNTEGLO<sup>®</sup> FDA approved

August 18, 2022

# 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 our expectations regarding our programs and therapies, including but not limited to the timing or likelihood of regulatory filings and approvals, our commercialization plans, and addressable market 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.

# agenda

Welcome and opening remarks

*Andrew Obenshain, chief executive officer*

Ready to launch ZYNTEGLO®

*Tom Klima, chief commercial and operating officer*

Strengthened path to financial sustainability

*Jason Cole, chief strategy and financial officer*

Entering a catalyst-rich period as a commercial company

*Andrew Obenshain, chief executive officer*

Q&A

pursuing curative gene therapies ...



to give patients and their families  
more bluebird days



## Olivia's Story

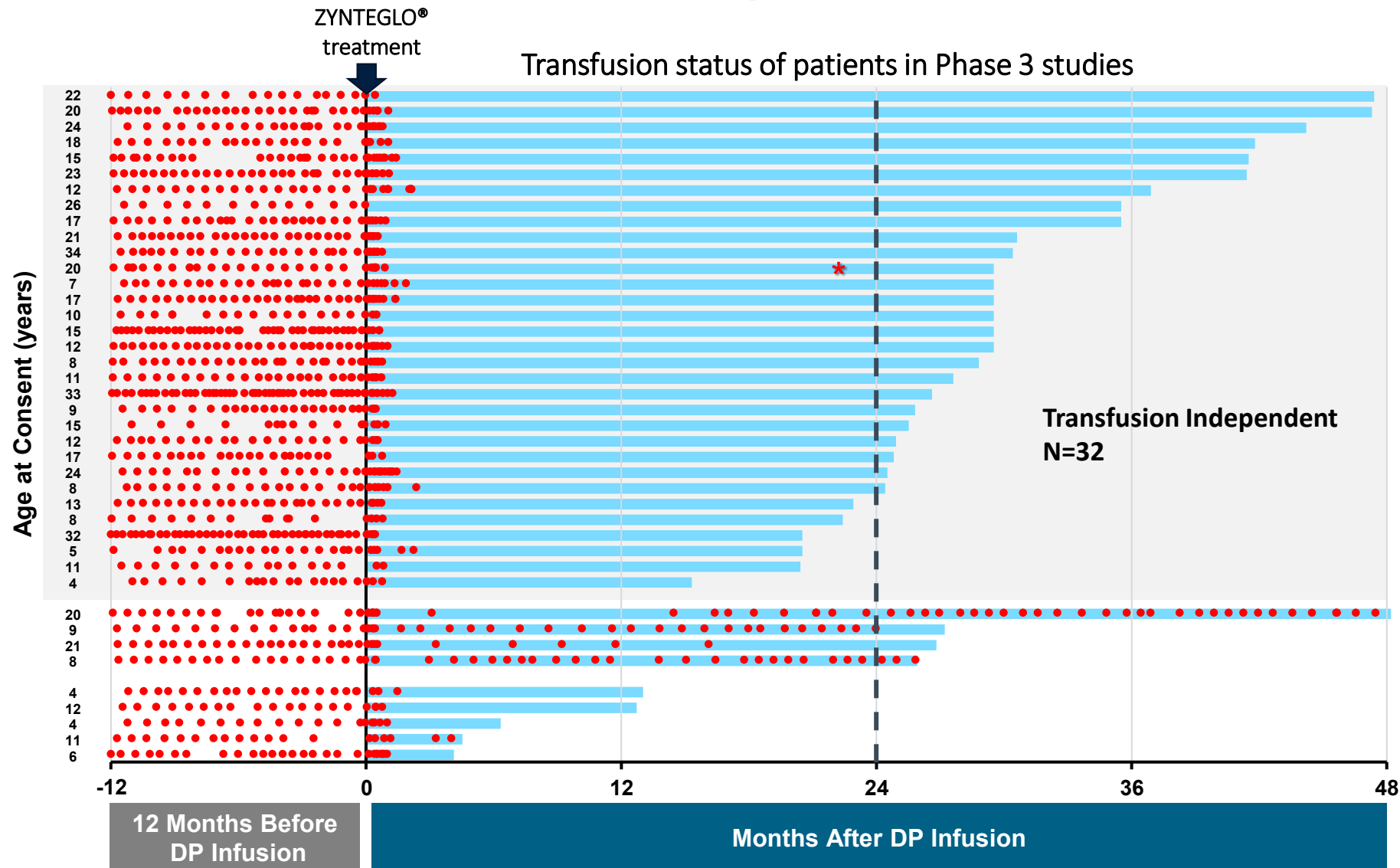


# ZYNTEGLO: Now FDA Approved



  
**zynteglo<sup>®</sup>**  
(betibeglogene autotemcel)  
suspension for IV infusion

# ZYNTEGLO® approval is underscored by impressive clinical study data



## In Phase 3 studies:

- 89% of patients achieved **transfusion independence (TI)** and normal or near-normal hemoglobin levels
- All patients who achieved TI have **remained transfusion free**
- **Durable results** with longest follow-up out to **4 years**
- Majority of AEs and SAEs **were consistent with myeloablative conditioning**

# Ready to launch ZYNTEGLO®

Tom Klima, Chief Commercial and Operating Officer



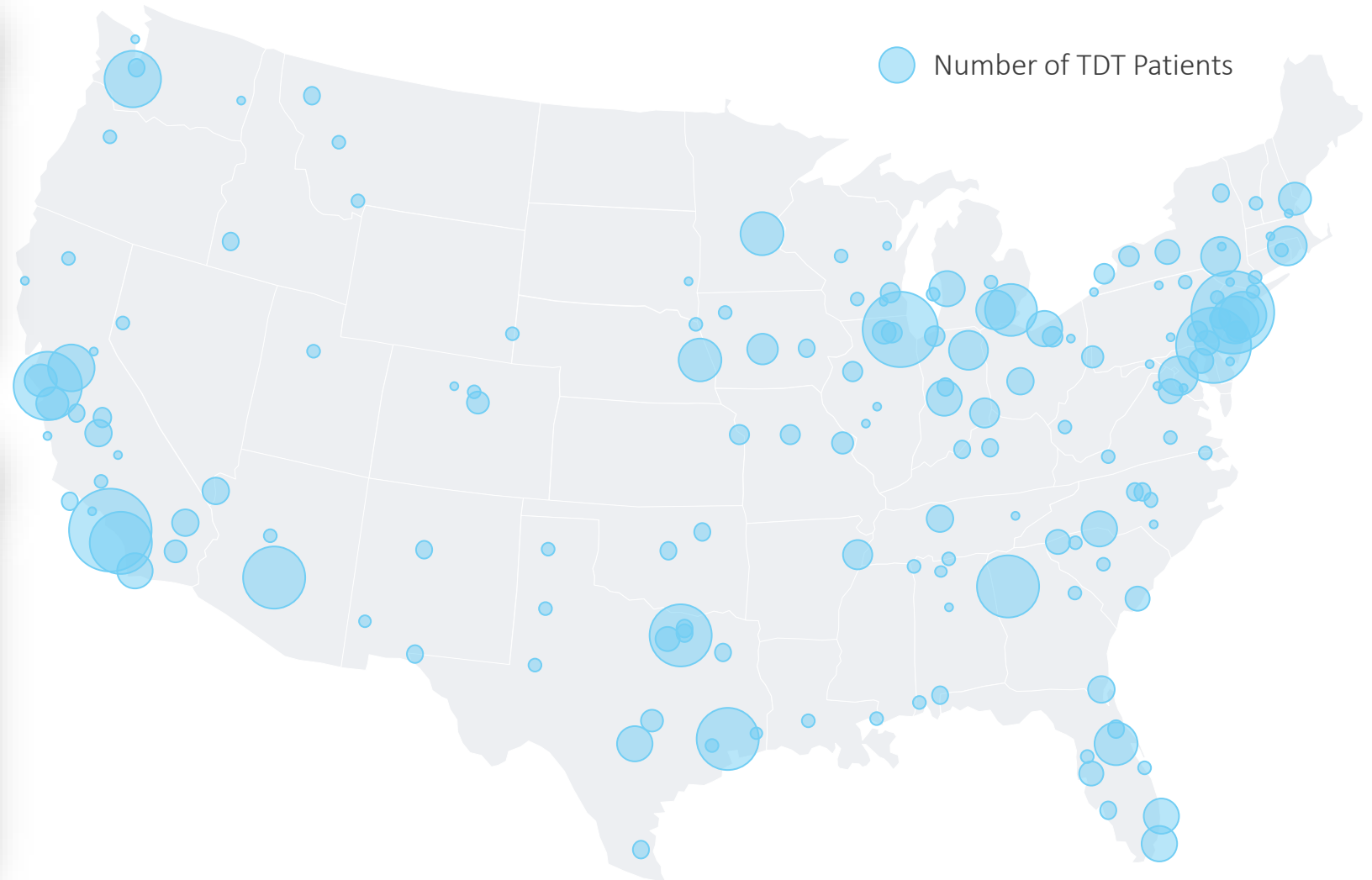
# Fit-for-purpose Qualified Treatment Center (QTC) network expected to activate in waves

## Targeted QTC selection

- Focused on high prevalence states
- Centers actively treating beta-thalassemia today
- Deep experience with commercial cell and gene therapies

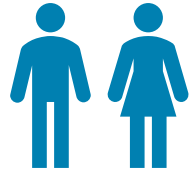
## QTC growth aligned with demand

- Wave 1 QTCs to be fully activated by end of September
- Anticipate 1<sup>st</sup> apheresis in Q4 2022
- Expect to more than double launch network by year end 2022
- Network expands substantially with SCD launch

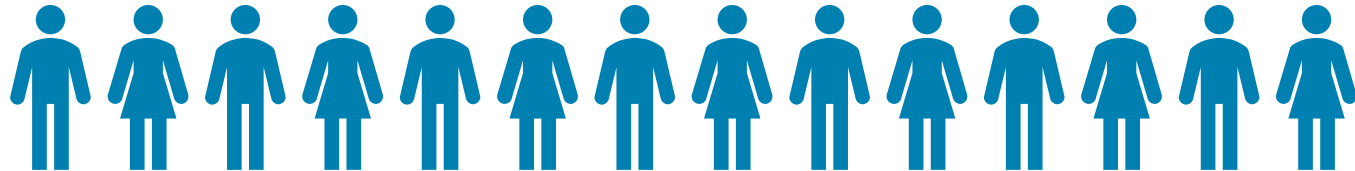


# Planned QTC network supports significant U.S. patient opportunity

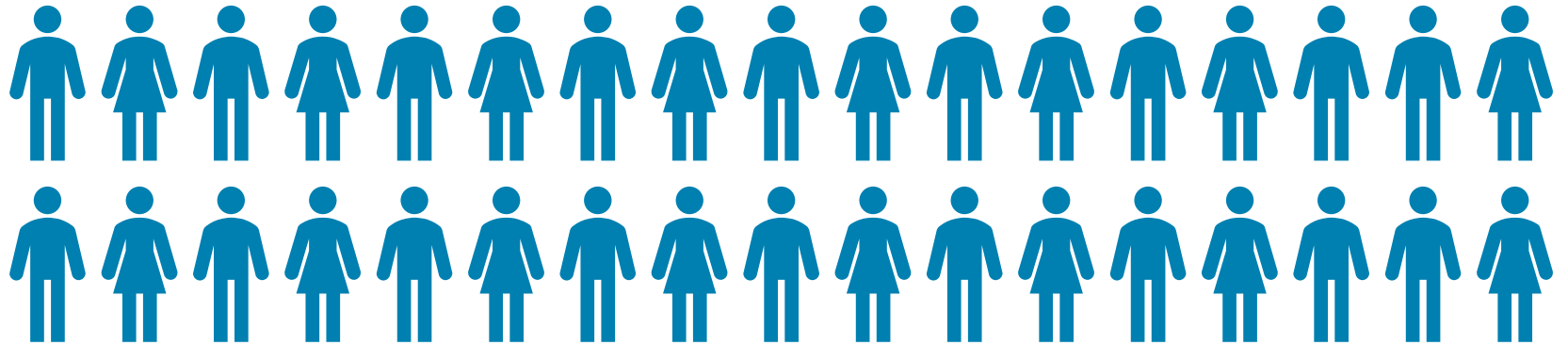
**~50** potentially eligible patients currently seen at Wave 1 QTCs



**~350** patients eligible with QTC expansion



**More than 850** patients likely eligible for ZYNTEGLO®



**55 – 60%** of the ~1,500 patients with transfusion dependent beta-thalassemia in the US may be eligible for gene therapy

# Confident in timely, quality access and reimbursement with upfront payment at \$2.8M price

## Price tied to recognized value

- Beta-thalassemia requiring regular RBC transfusions is associated with:
  - \$6.4 million average lifetime medical care cost per patient<sup>1</sup>
  - 23X higher average total health care cost per patient per year vs. general population<sup>2</sup>
  - Blood transfusions every 2-5 weeks for life<sup>3</sup>

## Simple and innovative payment strategy

- bluebird is offering payers:
  - One-time upfront payment
  - Outcomes-based agreement with 80% rebate if patient does not reach transfusion independence within 2 years
  - Clinically-relevant outcome, easily tracked in claims data

## Encouraging payer interactions

- All target payers have responded favorably to approach:
  - 70-75% of patients with beta-thalassemia have commercial insurance
  - Engaging with state Medicaid agencies representing ~80% of publicly-insured beta-thalassemia patients

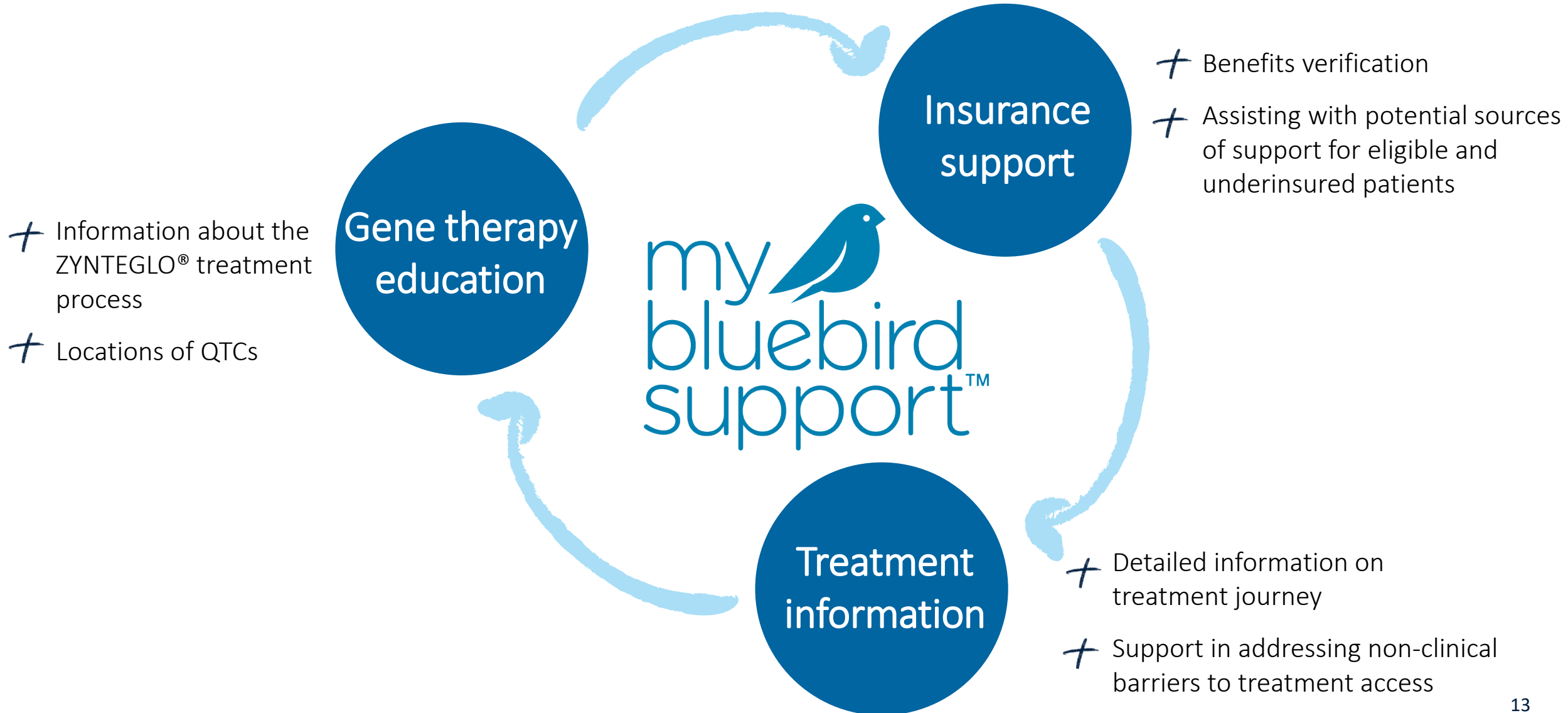
# ZYNTEGLO® manufacturing allows for flexible scheduling and is designed to ensure high quality drug product



*Bulk of time spent on release testing to ensure high quality drug product*



# *my bluebird support* helps patients navigate every step of the treatment journey



# Strengthened path to financial sustainability

Jason Cole, Chief Strategy & Financial Officer

# ZYNTEGLO® approval strengthens financial position

## Current cash runway into 1H23

Cash on hand of **\$218 million\*** as of 6/30/22

Targeting **\$60 million** per quarter net cash burn by year end 2022 and carry into 2023

## Near-term financing plans bring cash runway into 1H24

**\$24.7 million in gross** ATM proceeds to date

ZYNTEGLO® **PRV in hand** – plan to monetize promptly and maximize value

**Additional PRV** may be issued upon potential eli-cel approval

*Non-dilutive capital*

## Additional resources may extend cash runway further

Evaluating public and private **equity financings**

**Product revenue expected** beginning in Q1 2023

\*Cash balance includes restricted cash of ~\$45 million; PRV: Priority Review Voucher; ATM: At-the-Market equity offering

# Entering a catalyst-rich period as a commercial company

Andrew Obenshain, Chief Executive Officer



# ZYNTEGLO® approval is the first of several exciting milestones on the horizon



**ZYNTEGLO® now FDA approved for patients with beta-thalassemia who require regular RBC transfusions**

**eli-cel for CALD  
PDUFA date  
Sept. 16, 2022**

**lovo-cel for SCD  
BLA submission  
anticipated in  
Q1 2023**

- *Proving our commercial model*
- *Building our infrastructure today*
- *Delivering significant value for patients and shareholders*

pursuing curative gene therapies ...



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Q + A