



# bluebird bio

March 2024

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 our expectations for our programs and therapies, including but not limited to our manufacturing and commercialization plans, including the number of anticipated patient starts across our portfolio of therapies, our expectations for expansion of the QTC network, patient demand for our therapies, and our ability to establish commercial infrastructure to support timely, equitable access to our therapies; our ability to establish favorable coverage for our therapies, including our ability to successfully partner with payers; our anticipated cash runway; our expectations regarding our ability to access future tranches of our term loan facility, 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 annual Report on Form 10-K, 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.



# pursuing curative gene therapies ...

to give patients and their  
families more bluebird days



Only commercial gene therapy company with three FDA approved products



**lyfgenia**<sup>™</sup>  
(lovotibeglogene autotemcel)  
suspension for IV infusion

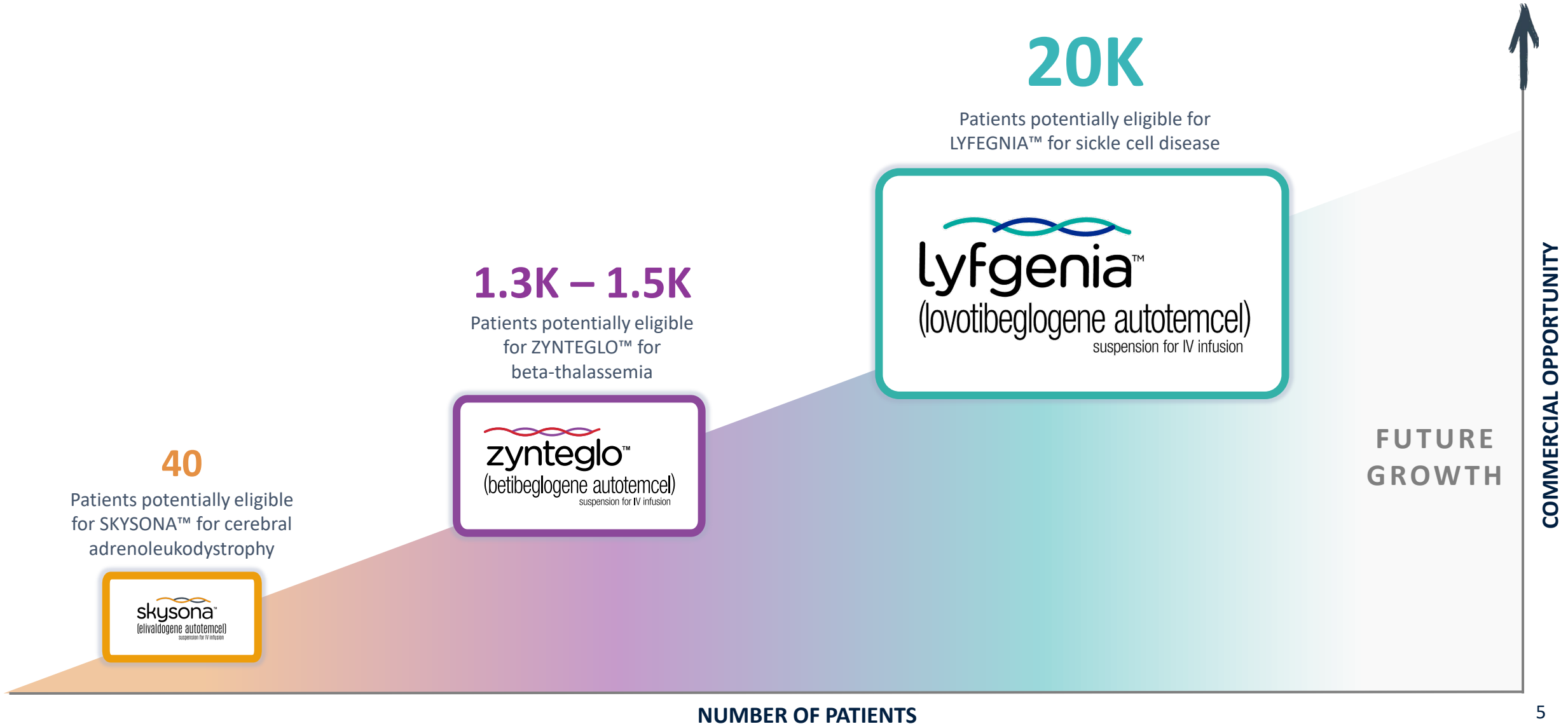


**zynteglo**<sup>™</sup>  
(betibeglogene autotemcel)  
suspension for IV infusion



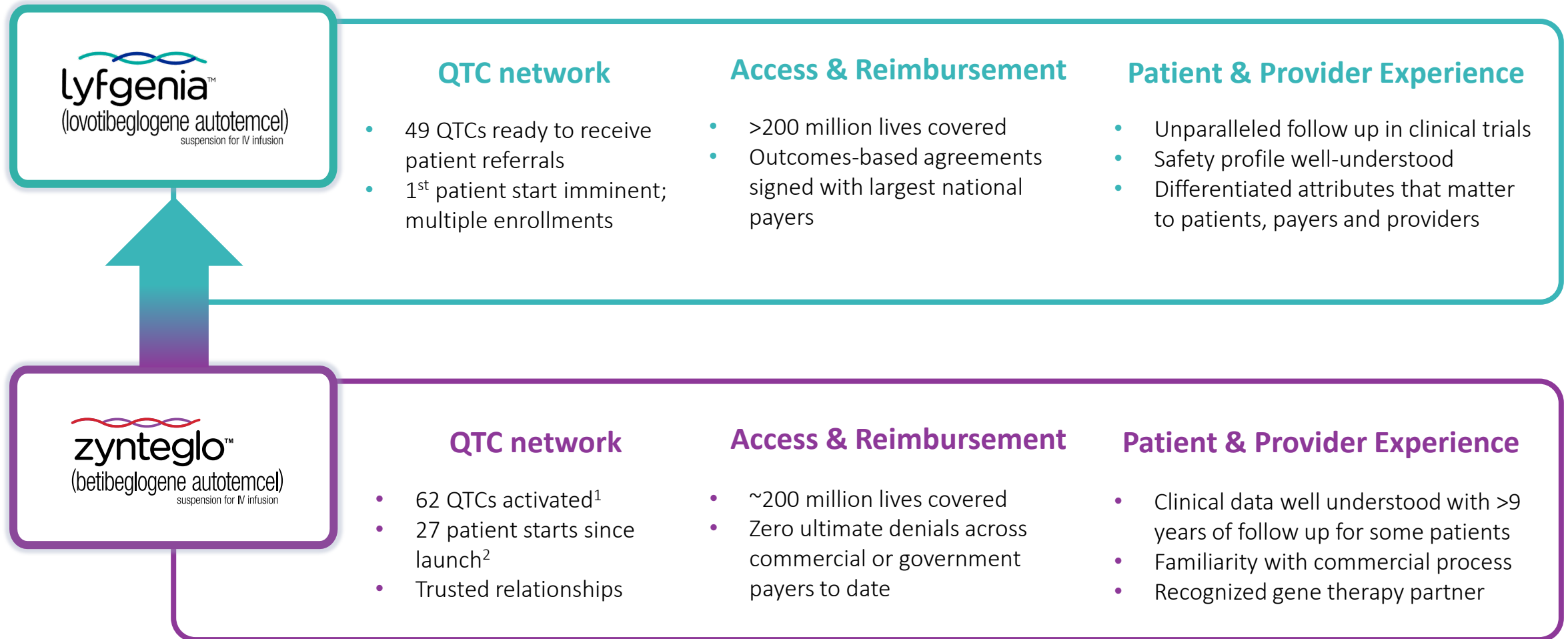
**skysona**<sup>™</sup>  
(elivaldogene autotemcel)  
suspension for IV infusion

# Momentum building with commercial launches; opportunity to deliver significant value for patients and shareholders





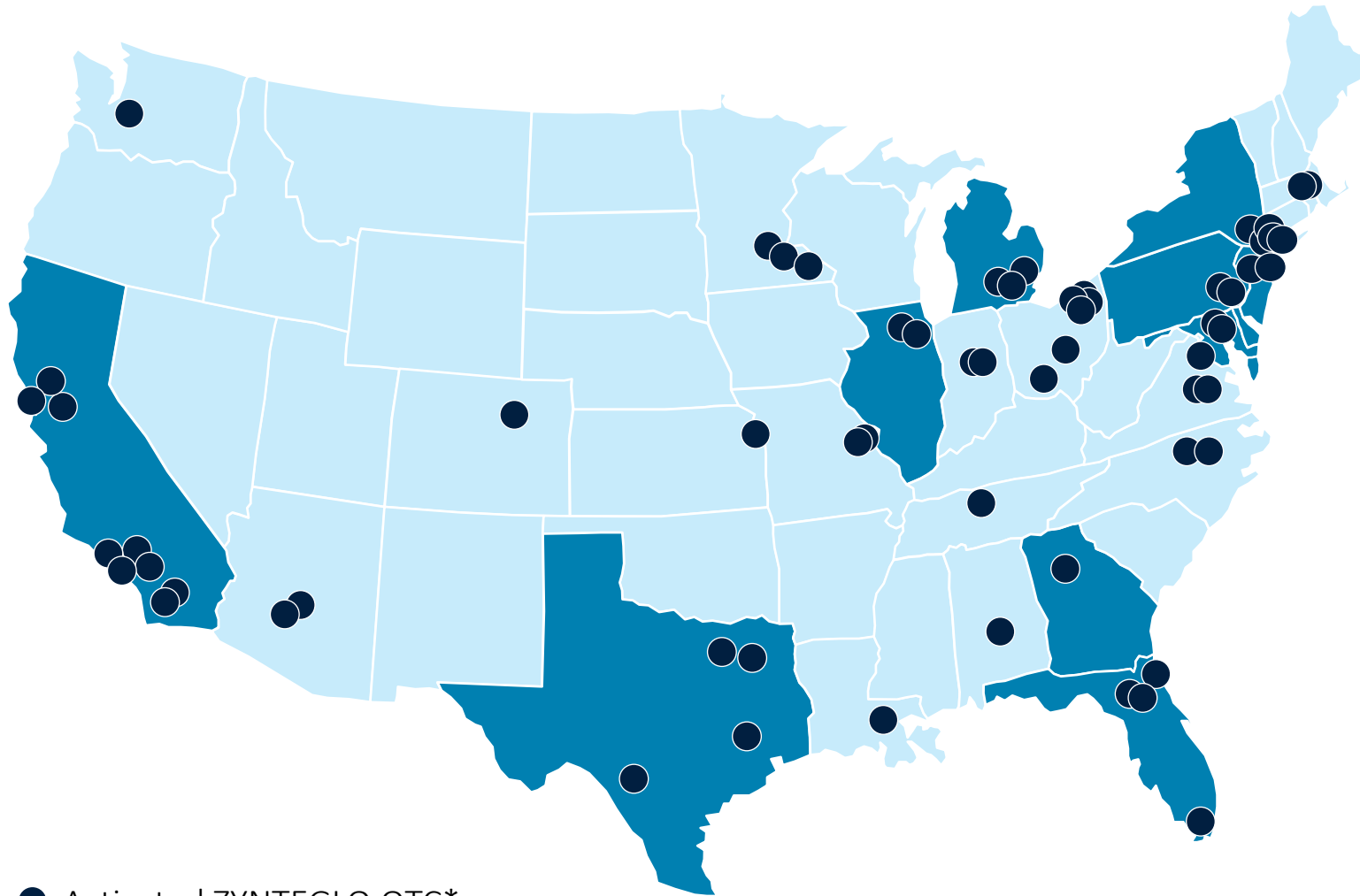
# Deploying a validated, commercial strategy for LYFGENIA – informed by our real-world experience with ZYNTGLO





# QTC Network

# 62 Qualified Treatment Centers (QTCs) activated for ZYNTEGLO and quickly onboarding for LYFGENIA



**100%** of ZYNTEGLO QTCs have initiated the activation process for LYFGENIA

**49 QTCs** already receiving referrals for LYFGENIA

Anticipate continued QTC network expansion across portfolio in 2024

● Activated ZYNTEGLO QTC\*

■ Shading indicates target SCD market

\*Activated QTC defined as signed MSA



# Optimized QTC network designed to reach individuals living with SCD

## STRONG DEMAND FOR GENE THERAPY

**>70%**

of SCD patients<sup>1</sup> would consider gene therapy if recommended by their doctor<sup>2</sup>

**80%**

of providers want both LYFEGNIA and its competitor available at their institution<sup>3</sup>

## POISED TO MEET PATIENTS **WHERE** THEY ARE

**95%**

of SCD patients<sup>2</sup> are within 200 miles of a planned QTC<sup>3</sup>

**88%**

of target SCD patients are actively being treated in the healthcare system<sup>4</sup>



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# Access & Reimbursement

Value of ZYNTEGLO is recognized

Patients with beta-thalassemia are **achieving access**

**~200M**

lives covered under  
contract or  
coverage policy

**~90%**

published coverage  
policies positive for  
ZYNTEGLO

**ZERO**

ultimate denials  
to date across  
commercial and  
government payers

# Validated access and reimbursement strategy designed to enable timely, equitable access to LYFGENIA for sickle cell disease



## \$3.1M price tied to value

Demonstrated robust and sustained clinical benefit (out **more than 5 years**)

Reflects **lifetime impact** of reducing or eliminating VOs

- Healthcare utilization
- Future earnings
- Life opportunities



## Outcomes-based agreement offerings

### Meaningful risk sharing

Tied to VOE related hospitalizations

Patients followed for **3 years**

Commercial payer and Medicaid options designed to offer **predictability** and **operational ease**



## Encouraging payer interactions

Signed outcomes-based agreements representing >200 million covered lives

Discussions ongoing with **>15** Medicaid agencies representing **~80%** of individuals with SCD in the US<sup>1</sup>

**Active engagement** with CMMI on innovative payment demonstration (anticipated 2025)

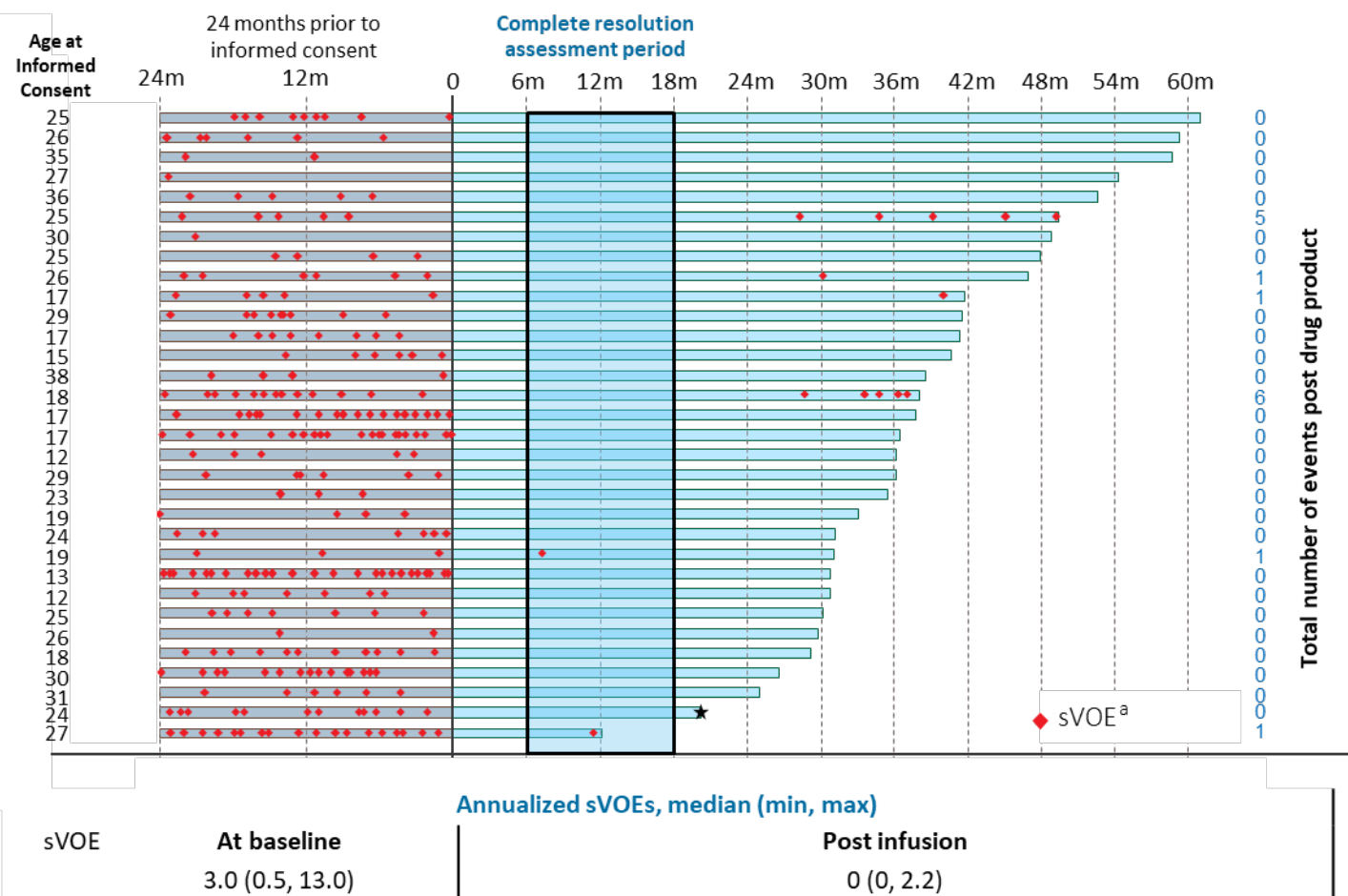




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# Treatment Experience

# LYFGENIA supported by the most robust and longest follow-up of any gene therapy program for SCD



## Clinical Data Supporting LYFGENIA

- Label based on efficacy data from 36 patients from HGB-206 Group C (median 38 months follow-up) and safety data from 54 patients\*
- February 13, 2023 data cut showing:
  - 32 patients evaluable for VOE endpoints including 8 adolescent patients
  - 94% complete resolution of severe VOEs in the 6-18 months post infusion
  - 88% resolution of VOEs in the 6-18 months post infusion
  - Maintenance of VOE resolution in majority of patients through long-term follow up + stable production of HbA<sup>T87Q</sup>
  - 100% of patients with history of stroke (n=5) remained free of recurrent stroke post-treatment with LYFGENIA
  - Majority of AEs attributed to underlying SCD or conditioning with busulfan
  - The label includes a Boxed Warning for hematologic malignancy

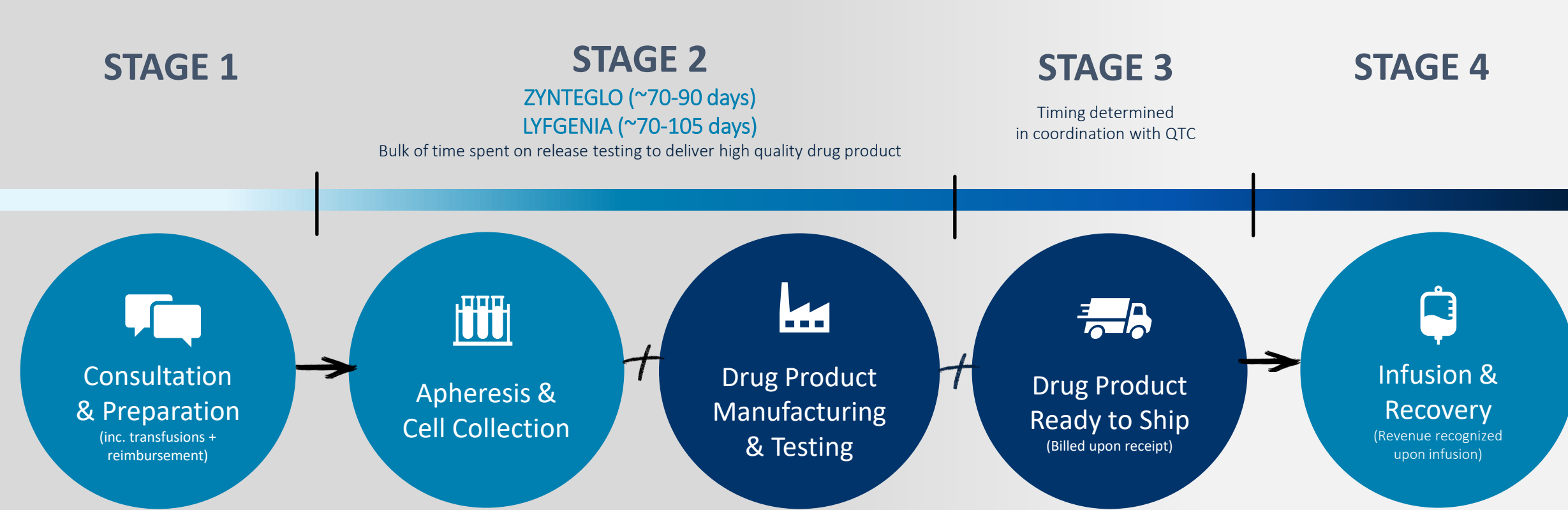
★ Death, due to significant baseline SCD-related cardiopulmonary disease; not considered related to lovo-cel.

Data as of Feb 13, 2023

\*54 patients initiated cell collection in HGB-206 Group A, Group B and Group C. <sup>a</sup> sVOE is defined as a VOE requiring a hospitalization or multiple visits to an emergency department/urgent care over 72 hours and receiving intravenous medications at each visit; all VOEs of priapism requiring any level of medical attention were also considered sVOEs

# Delivering a consistent manufacturing process is essential for patients, families and providers

ZYNTEGLO™ and LYFGENIA™ use a similar manufacturing process with distinct supply chain and release criteria



● Occurs at QTC ● Occurs at CMO

QTC: Qualified Treatment Center; CMO: Contract Manufacturing Organization. Stage 2 release time an estimate based on release criteria per product label

# Differentiated attributes of LYFGENIA that matter to patients, payers and providers



## Cell collections

In clinical trials, 85% required  $\leq 2$  cell collections for LYFGENIA<sup>1</sup>



## Drug product delivery

Process is designed to take between 70-105 days from cell collection to drug product delivery to the QTC



## Engraftment time

Median time to neutrophil engraftment 20 days, a key step to enabling patient discharge

Underpinned by clinical attributes – including >5 years of follow up, in-depth safety analyses, and data addressing SCD complications, including stroke



Experienced  
commercial gene  
therapy team well-  
positioned to lead in  
hemoglobinopathies

- 1 Potential multi-billion dollar opportunity**  
with ~22,000 addressable patients in the US;  
established commercial strategy and projected  
patient and physician demand
- 2 Significant commercial head start**  
leveraging ZYNTEGLO launch synergies – same  
treating physicians, same QTCs, same payers
- 3 Recognized gene therapy leader**  
with deep gene therapy expertise, manufacturing  
experience and commercial infrastructure



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SKYSONA™



# SKYSONA™ for cerebral adrenoleukodystrophy

  
**skysona™**  
(elivaldogene autotemcel)  
suspension for IV infusion

## Commercial

- SKYSONA is indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD)
- 8 patient starts since launch\*; 5 QTCs activated; zero ultimate denials across government and commercial payers

## Clinical

- 67 patients treated across all clinical trials
- Accelerated approval based on post-hoc analysis of 11 patients; estimated 72% likelihood of major functional disability free survival at 24 months
- Multiple boys treated with SKYSONA have developed hematologic malignancy and the label includes a boxed warning.\*\*

Patient starts is defined as a cell collection (apheresis); Activated QTC defined as Qualified Treatment Center with a signed MSA. \*Patient starts as of March 25, 2024

\*\*bluebird closely monitors potential and diagnosed cases of hematologic malignancy in patients treated with SKYSONA and additional cases are expected to arise over time. bluebird is communicating regularly with treating physicians and regulatory authorities.





Closing



# Established gene therapy leader poised to deliver shareholder value

## Established Clinical Leadership

- 10+ years of gene therapy research
- 200+ patients treated
- 8 clinical trials

## Demonstrated Regulatory Success

- Established track record for LVV platform
- 3 FDA-approved gene therapies

## Commercial Gene Therapy Leader

- Scaled for 3 commercial launches
- Synergistic transplant and cell therapy infrastructure
- Proven reimbursement

**85-105 patient starts<sup>1</sup> combined across LYFGENIA, ZYNTEGLO and SKYSONA anticipated in 2024**

## Current Financial Position

**\$275M**

unaudited cash, cash equivalents, restricted cash & marketable securities balance as of December 31, 2023<sup>2</sup>

**Up to \$175M**

secured in debt financing in Q1 2024

**Cash runway through Q1 2026<sup>3</sup>**

# bluebird occupies a unique strategic position as a standalone gene therapy company

- 10+ years of gene therapy research
  - 200 patients treated
  - 8 clinical trials



- Established track record for LVV platform
- 3 FDA approvals

- 3 commercial launches
- Transplant and cell therapy infrastructure
- Proven reimbursement

*Clinical & pre-clinical companies*

*Large cap pharma*

Thank you