



bluebird bio

January 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 regarding our programs and therapies, including but not limited to the timing or likelihood of regulatory filings and approvals; our manufacturing and commercialization plans, including without limitation, patient demand for our therapies, our ability to establish commercial infrastructure to support timely, equitable access to our therapies, our ability to successfully partner with payers and CMMI, our expectations on timing for activating QTCs, and our expectations on the timing and size of our QTC network and the timing of our therapies’ availability at our QTCs; addressable market for our therapies; our preliminary unaudited cash position as of December 31, 2023; and our cash runway 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.



pursuing curative gene therapies ...

to give patients and their
families more bluebird days



Only commercial gene therapy company with three FDA approved products



lyfgenia[™]
(lovotibeglogene autotemcel)
suspension for IV infusion

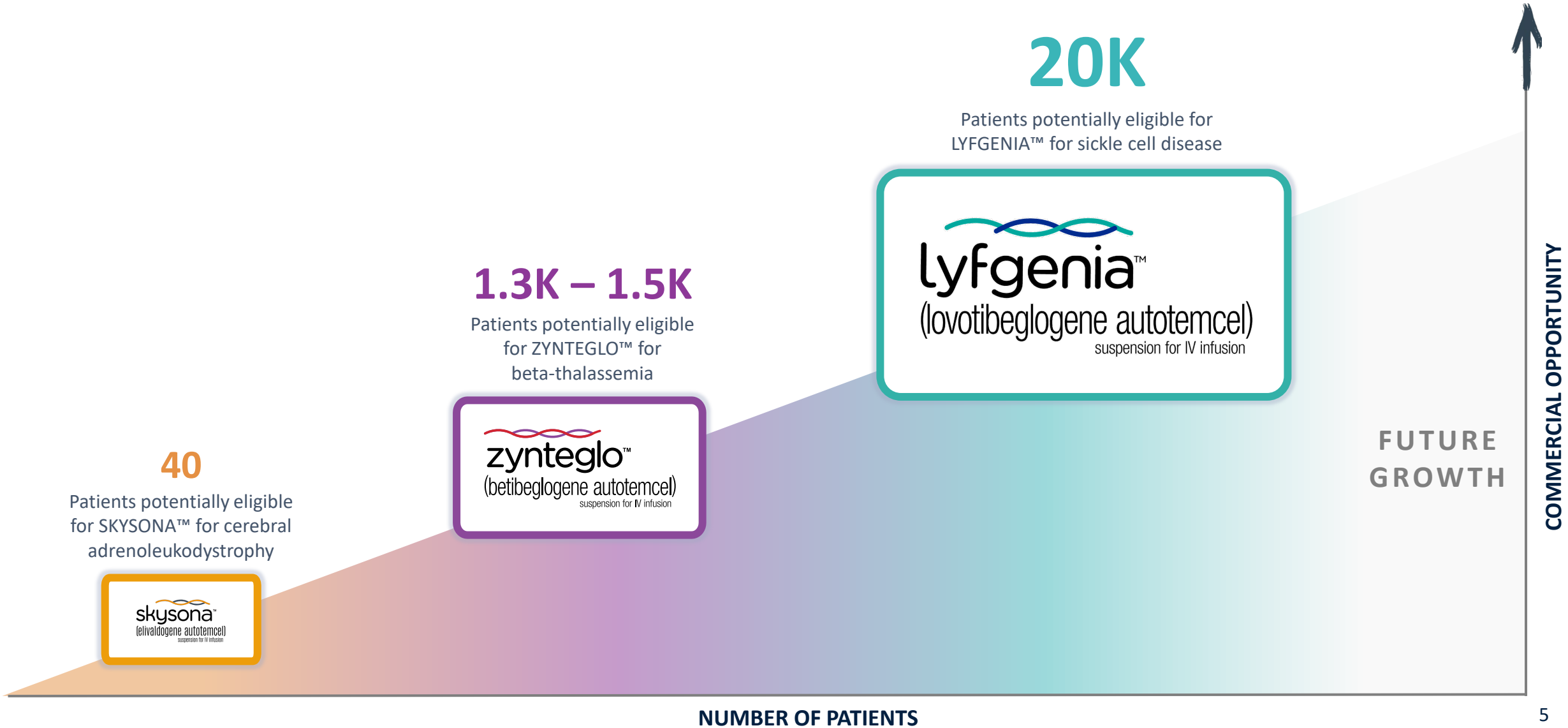


zynteglo[™]
(betibeglogene autotemcel)
suspension for IV infusion

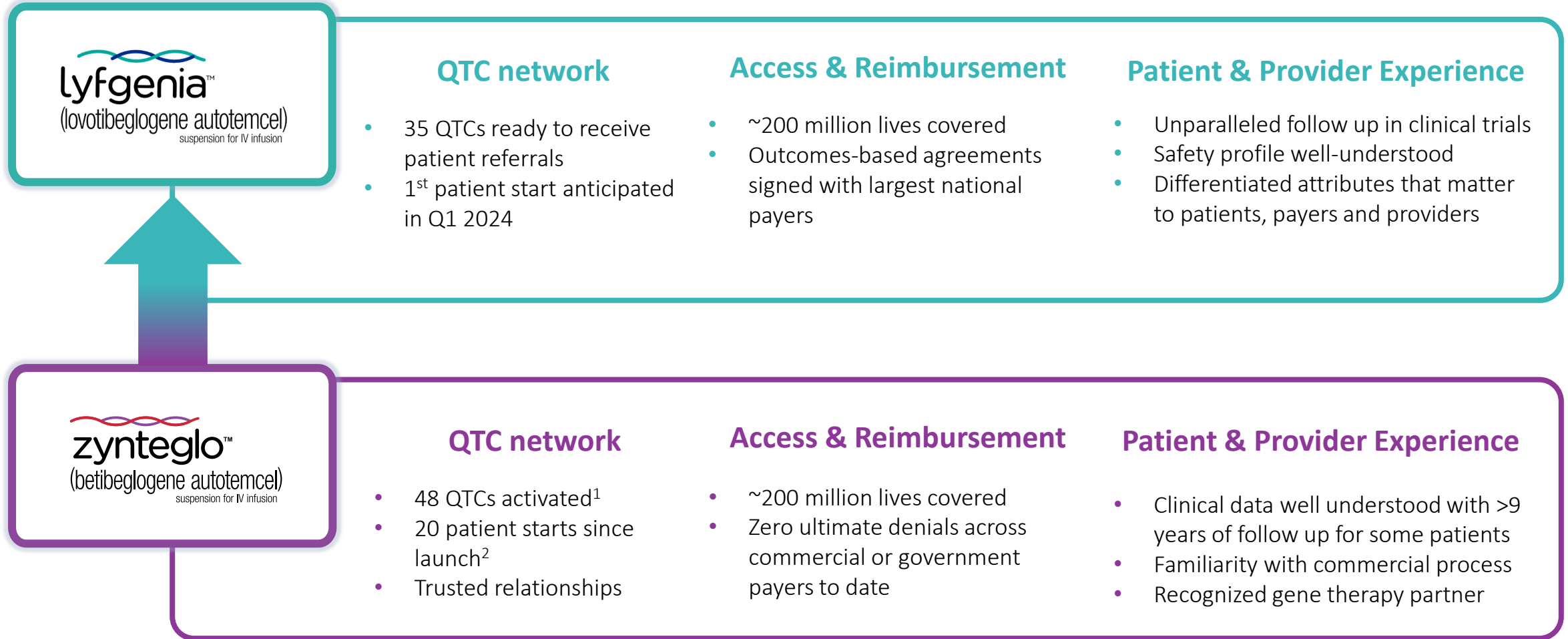


skysona[™]
(elivaldogene autotemcel)
suspension for IV infusion

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

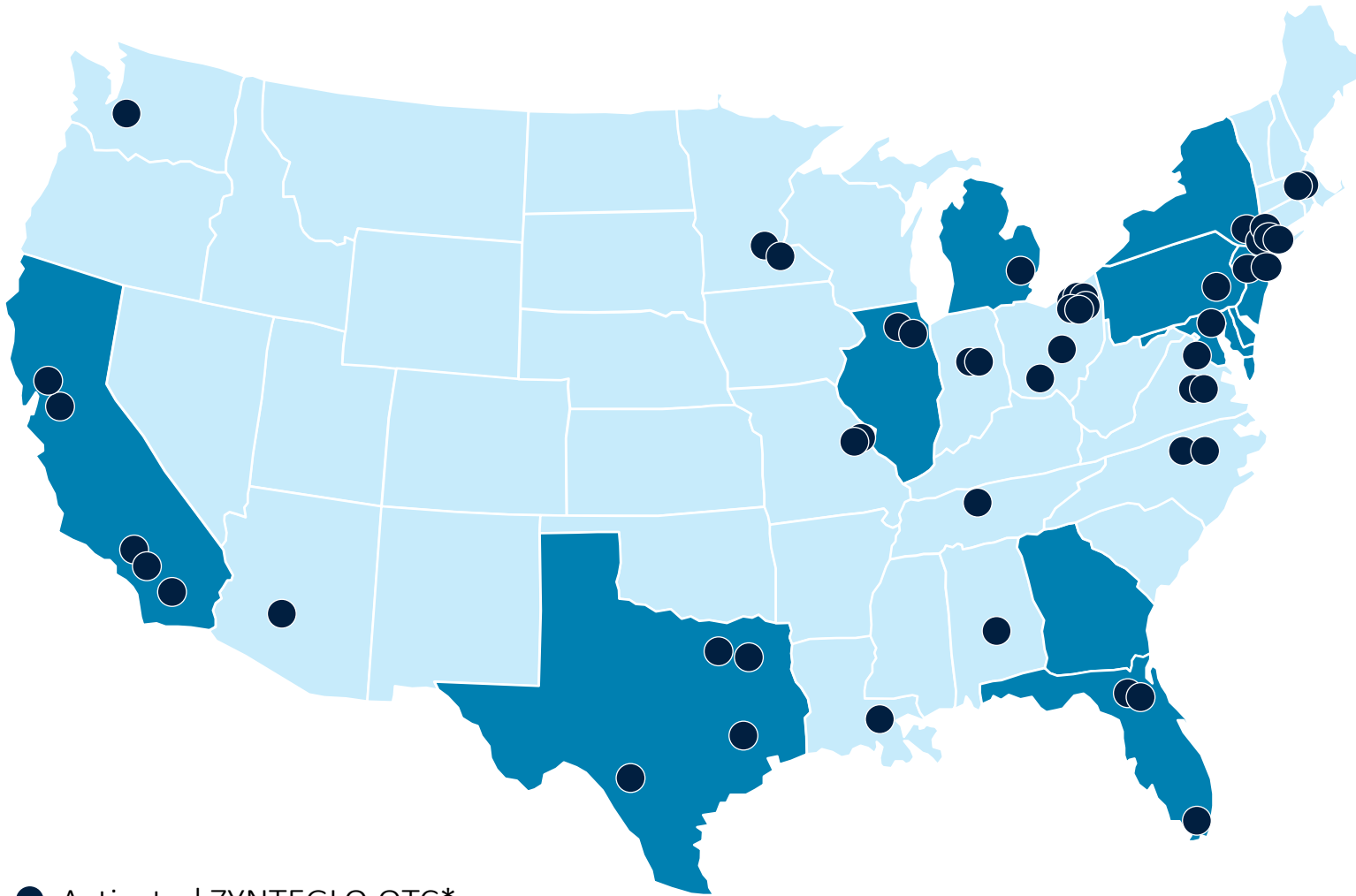


1. Activated QTC defined as signed MSA 2. Patient starts as of December 31, 2023



QTC Network

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



● Activated ZYNTEGLO QTC*

■ Shading indicates target SCD market

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

35 QTCs are ready to receive SCD patient referrals now

Anticipate ZYNTEGLO network will be fully activated for LYFGENIA by **end of Q1 2024**

Planned **QTC network expansion** in 2024

*Activated QTC defined as signed MSA

Optimized QTC network designed to reach individuals living with SCD

STRONG DEMAND FOR GENE THERAPY

>70%

of SCD patients¹ would consider gene therapy if recommended by their doctor²

80%

of providers want both LYFGENIA and its competitor available at their institution³

POISED TO MEET PATIENTS **WHERE** THEY ARE

95%

of SCD patients² are within 200 miles of a planned QTC³

88%

of target SCD patients are actively being treated in the healthcare system⁴



bluebirdbio®

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

Advanced discussions with **>15** Medicaid agencies representing **~80%** of individuals with SCD in the US¹

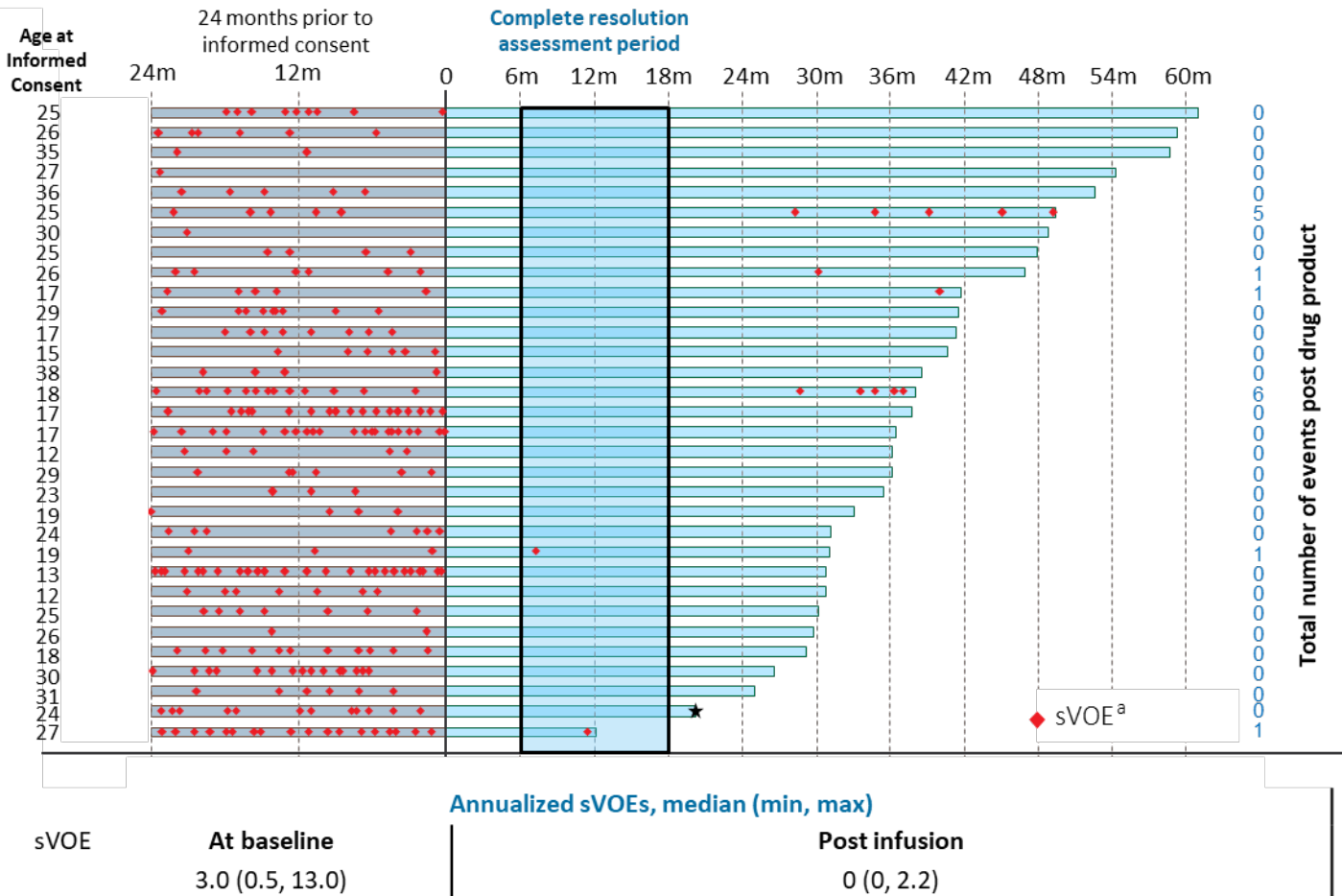
Active engagement with CMMI on innovative payment demonstration (anticipated 2025)



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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^{T87Q}
 - 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

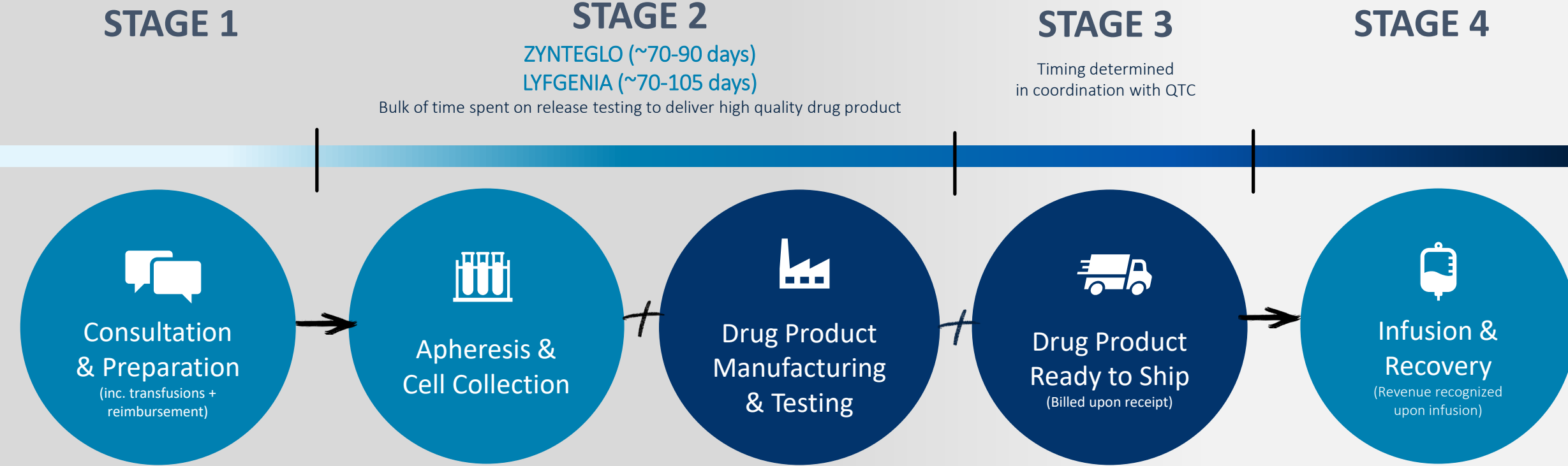
Data as of Feb 13, 2023

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

*54 patients initiated cell collection in HGB-206 Group A, Group B and Group C. ^a 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 ≤ 2 cell collections for LYFGENIA¹



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 18-month 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



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)
- 6 patient starts since launch*; 4 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
- Five boys treated in clinical trials developed myelodysplastic syndrome; label includes boxed warning for hematologic malignancy**



Closing

Established gene therapy leader poised to deliver shareholder value

Current Financial Position

\$275M unaudited cash, cash equivalents, restricted cash & marketable securities balance as of December 31, 2023¹

Zero debt on balance sheet today

Cash runway into Q1 2025²

Evaluating additional non-dilutive funding to bridge to near term profitability

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 combined across LYFGENIA, ZYNTEGLO and SKYSONA anticipated in 2024

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

Thank you