

# Accelerating access to gene therapy: Lessons from commercial implementation in sickle cell disease and transfusion- dependent thalassemia

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# Real-World Analysis of Patients Prescribed Zynteglo & Lyfgenia

## Zynteglo

Betibeglogene Autotemcel (beti-cel)

### Indication

ZYNTGLO is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of **adult & pediatric patients with  $\beta$ -thalassemia** who require regular red blood cell (RBC) transfusions

FDA approved August 2022

### Mechanism

*ex vivo* Gene Addition:  
Adds functional  $\beta^{A-T87Q}$ -globin gene designed to **correct the  $\beta/\alpha$ -globin imbalance in erythroid cells** and increase adult Hb

### Dosing

One-time autologous stem cell transplant

## Lyfgenia

Lovotibeglogene Autotemcel (lovo-cel)

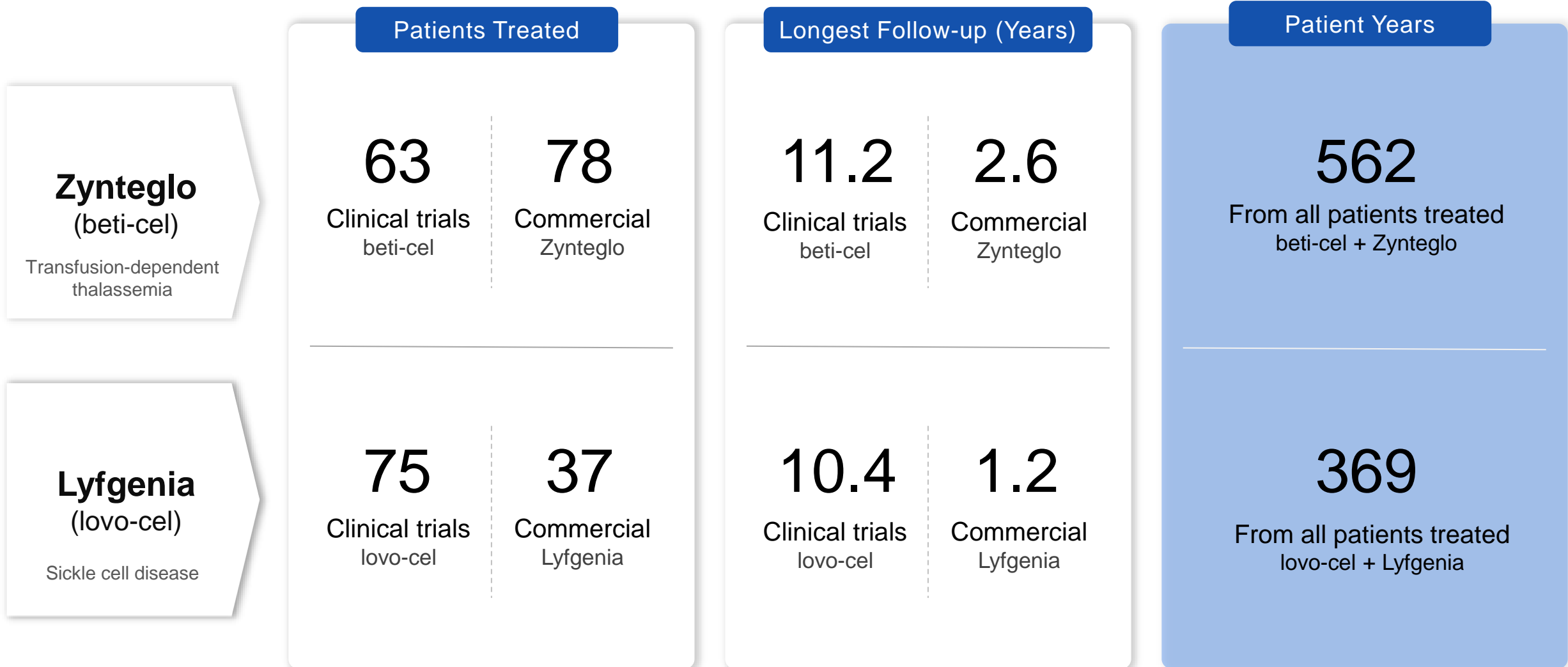
LYFGENIA is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of **patients 12 years of age or older with sickle cell disease** and a history of vaso-occlusive events

FDA approved December 2023

*ex vivo* Gene Addition:  
Adds anti-sickling  $\beta^{A-T87Q}$ -globin gene designed to **inhibit polymerization of HbS** thereby limiting the sickling of red blood cells

One-time autologous stem cell transplant

# The most robust long-term dataset in *ex vivo* gene therapy for hemoglobinopathies



# The treatment journey follows five key steps



## ENROLLMENT

BEGIN PREPARATION FOR TREATMENT

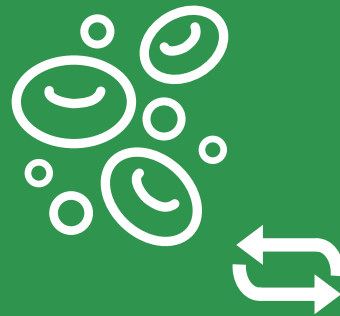
*QTCs ready patients for therapy medically and financially and submit enrollment to Genetix*



## SCHEDULING

SCHEDULE COLLECTION & PRODUCT MANUFACTURING

*QTCs schedule patient's cell collection and complete medical preparation of patient*



## COLLECTION

MOBILIZE & COLLECT CD34+ HSPCs

*Mobilization of HSPCs  
Collection via apheresis  
Shipment to manufacturer*



## MFG & TESTING

VECTOR TRANSDUCTION & QUALITY RELEASE TESTING

*Drug product is manufactured, meets dose and passes quality release testing before shipment to treatment center*

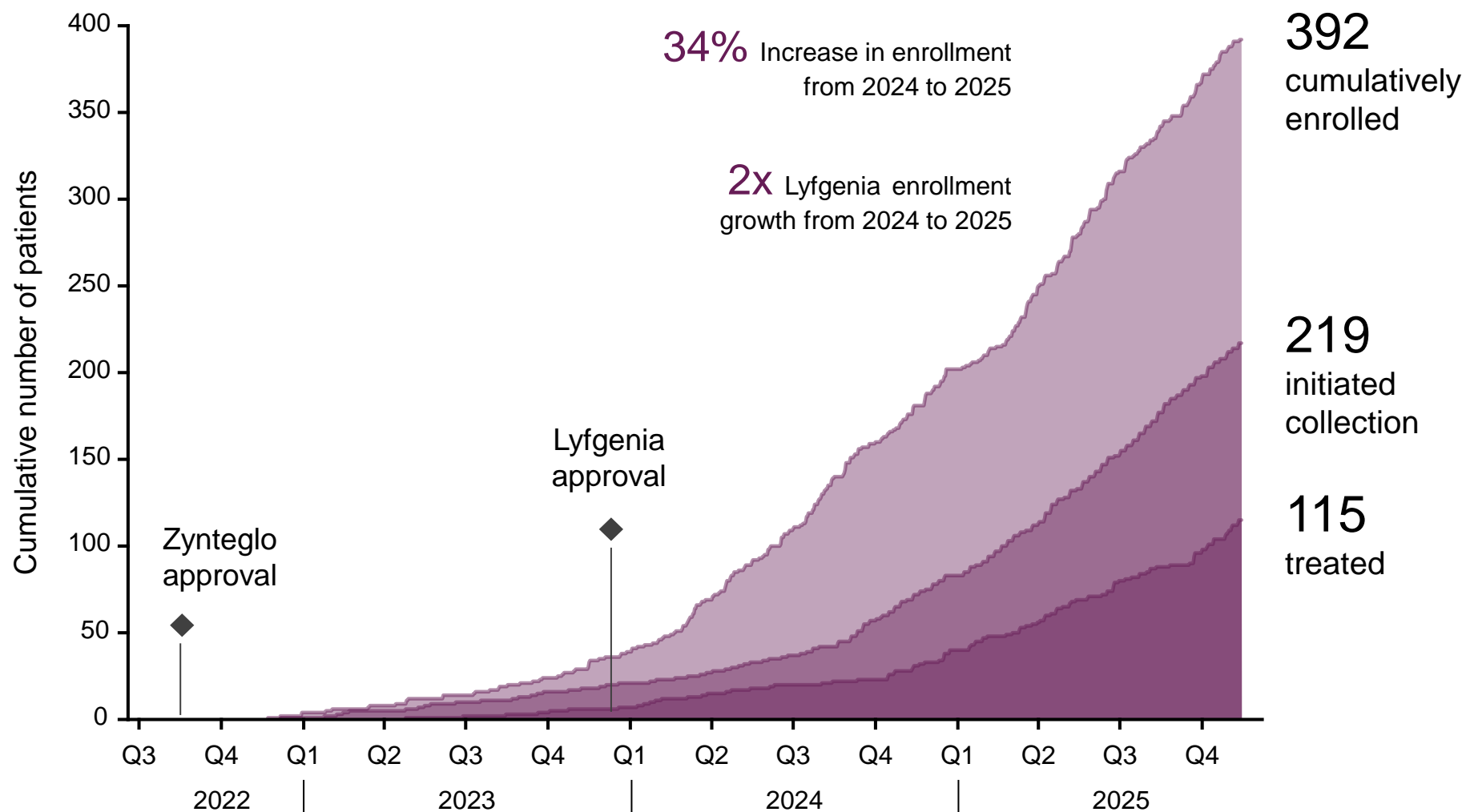


## TREATMENT

CONDITION, INFUSE, SUPPORT

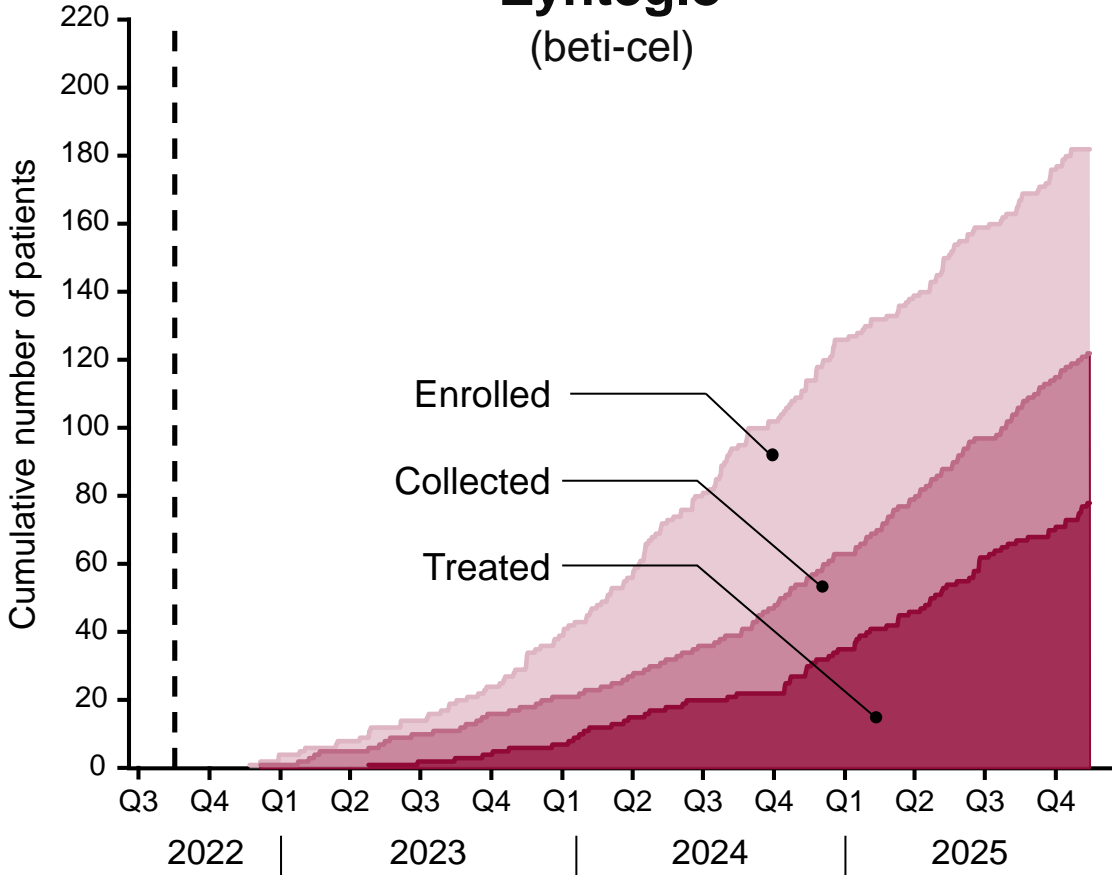
*Patient is admitted for conditioning and infusion of the drug product and is monitored until engraftment*

# Consistent Growth in Patients Advancing Through Treatment Pathway

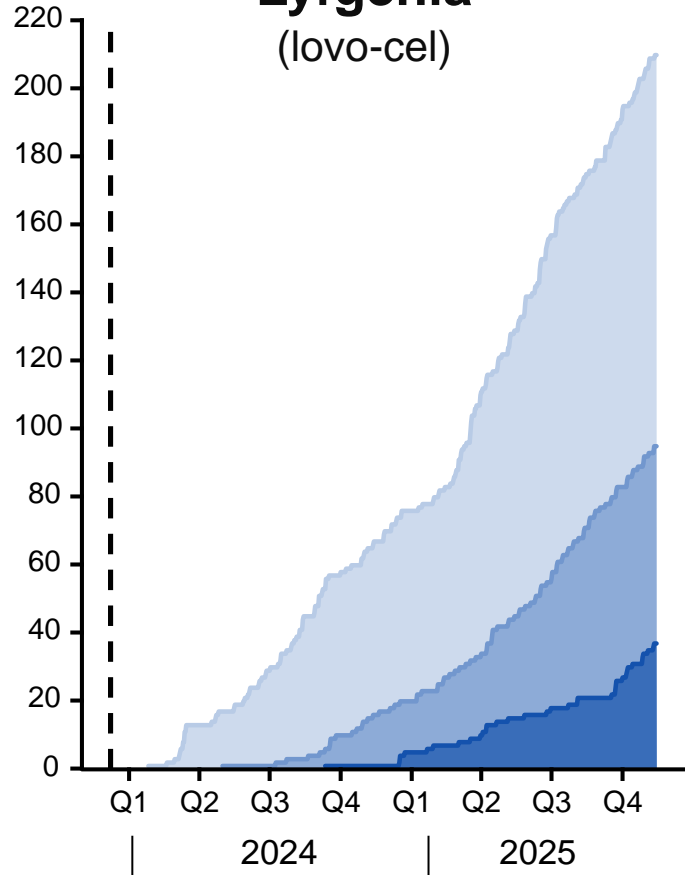


# Experience from Zynteglo launch supported rapid uptake of Lyfgenia

## Zynteglo (beti-cel)



## Lyfgenia (lovo-cel)



Compared to Zynteglo:

**2x** faster from approval to first patient enrollment

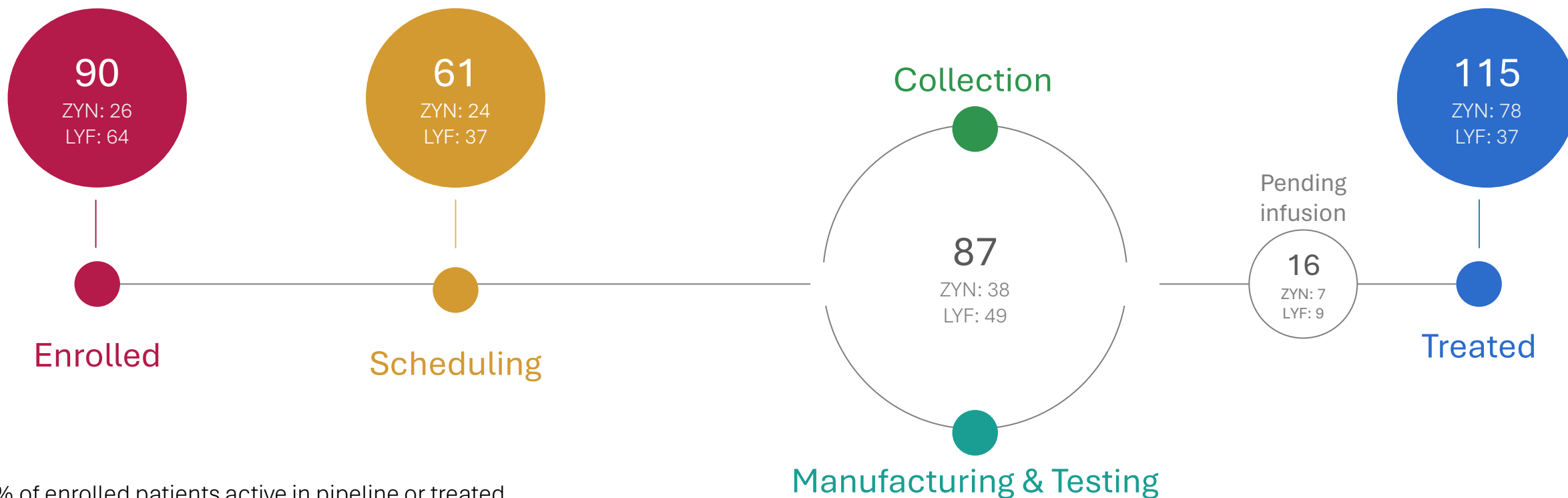
**167%** higher first quarter enrollment rates

**6x** more QTC activations first quarter post launch

# Patients Are Progressing Across All Steps of the Treatment Pathway



## Pipeline snapshot: November 2025



94.1% of enrolled patients active in pipeline or treated

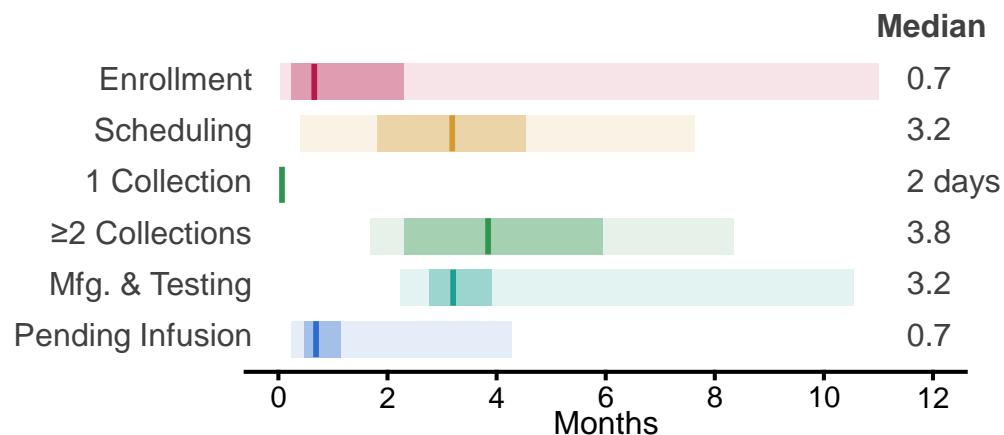
# Real-World Analysis of the Patient Journey identifies Key Drivers

## Enrollment-to-Treatment Lead Time

### Zynteglo (beti-cel)

**9.9 months (median)**

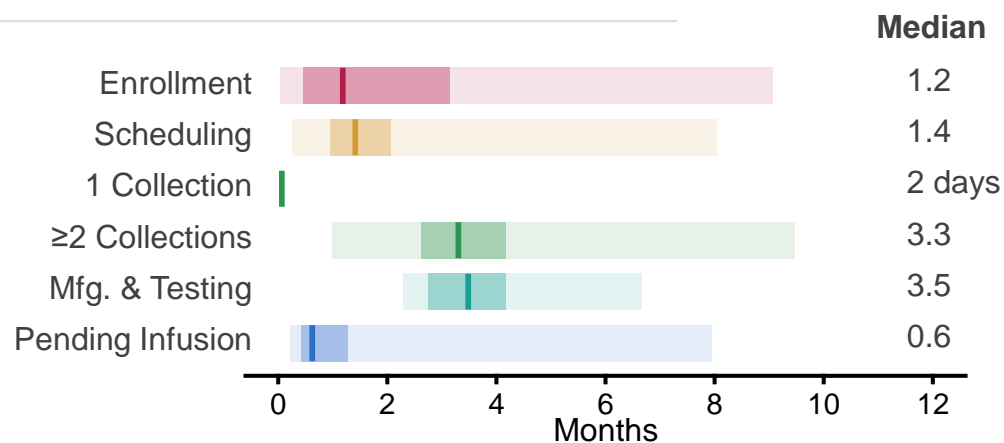
72% of patients treated within 12-mos of enrollment



### Lyfgenia (lovo-cel)

**8.0 months (median)**

76% of patients treated within 12-mos of enrollment



## Drivers of Timeline

### ▶ Enrollment

Timing of enrollment may be before or after payer approvals

### ▶ Scheduling

Scheduling lead times are dependent on manufacturing capacity and increasing demand

### ▶ Cell Collection

If additional cell collection is needed, the patient journey timeline is extended by ~82-days

### ▶ Manufacturing & testing

The largest share of the overall timeline includes comprehensive quality testing

# Opportunities to Streamline the Patient and Provider Experience



## **Enrollment**

Work alongside treatment centers, patients and payers to accelerate access and confirmation of coverage/reimbursement



## **Scheduling**

Expand manufacturing capacity to support increased demand & ensure slots are available when patients are ready



## **Collection**

Enhance mobilization and collection efforts while expanding training and education to improve success rates

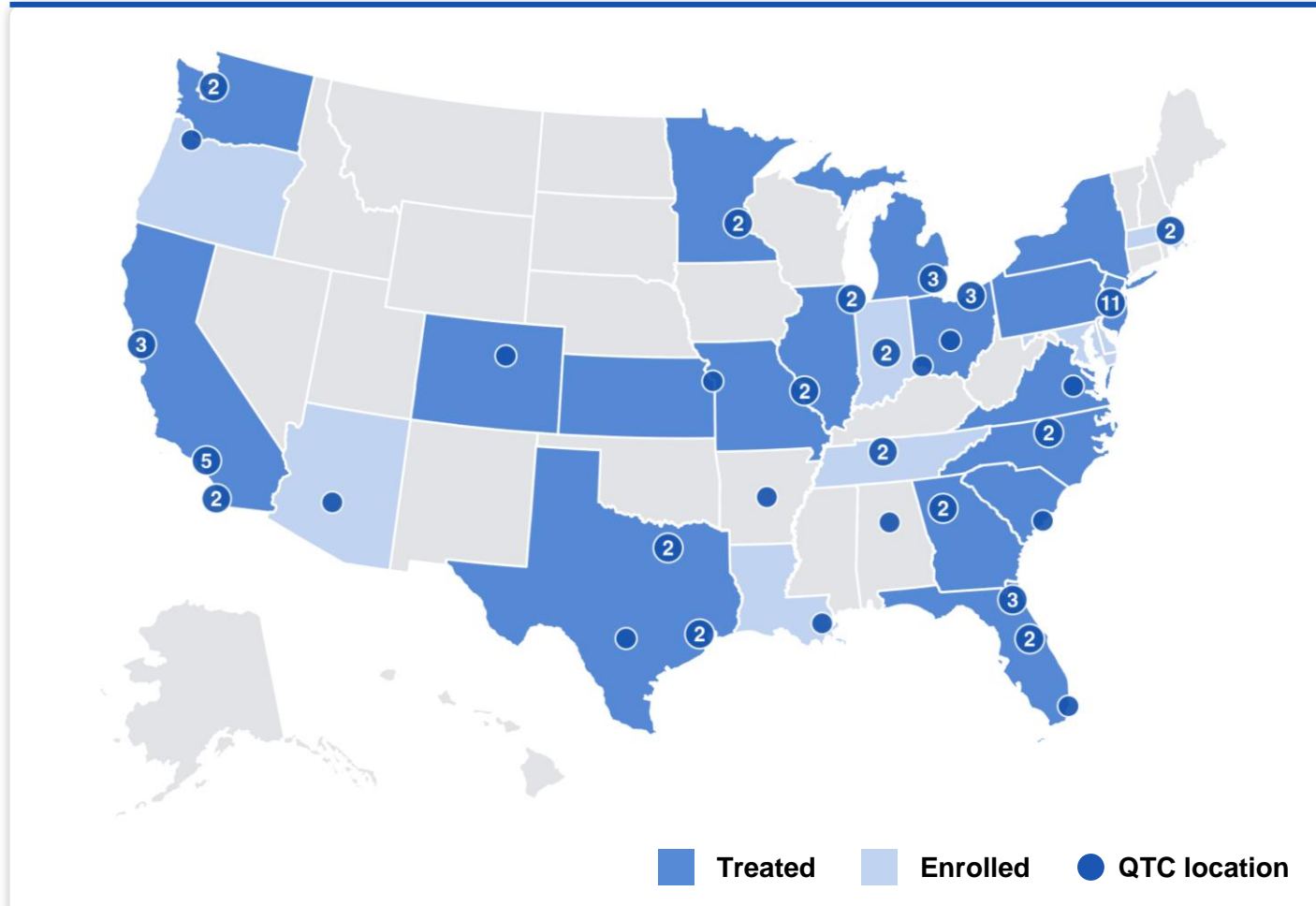


## **Manufacturing**

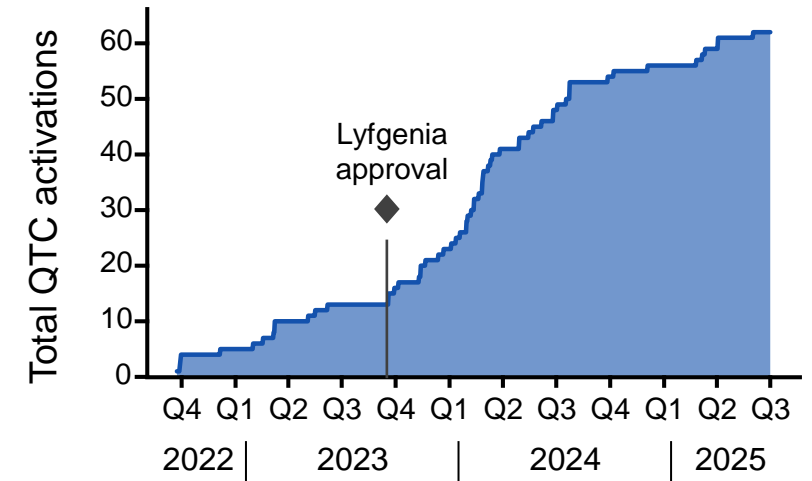
Optimize the manufacturing process and streamlining quality testing

# Zynteglo & Lyfgenia have the largest network of gene therapy treatment centers for hemoglobinopathies across the U.S.

~70 activated Qualified Treatment Centers (QTCs) in the U.S.



Rising QTC Utilization Nationwide



**115 Patients Treated Nationwide**

- ▶ 39 patients treated in the Northeast
- ▶ 27 patients treated in Western U.S.
- ▶ 27 patients treated in Southern U.S.
- ▶ 22 patients treated in the Midwest

# Cell & Gene Therapy (CGT) Access Model Enables Faster Medicaid Treatment Pathways

## CMS/CMMI CGT access model

Establishes one national framework for Medicaid gene therapy coverage and payment

## States opt in

Determines where standardized coverage and reimbursement/payment rules apply

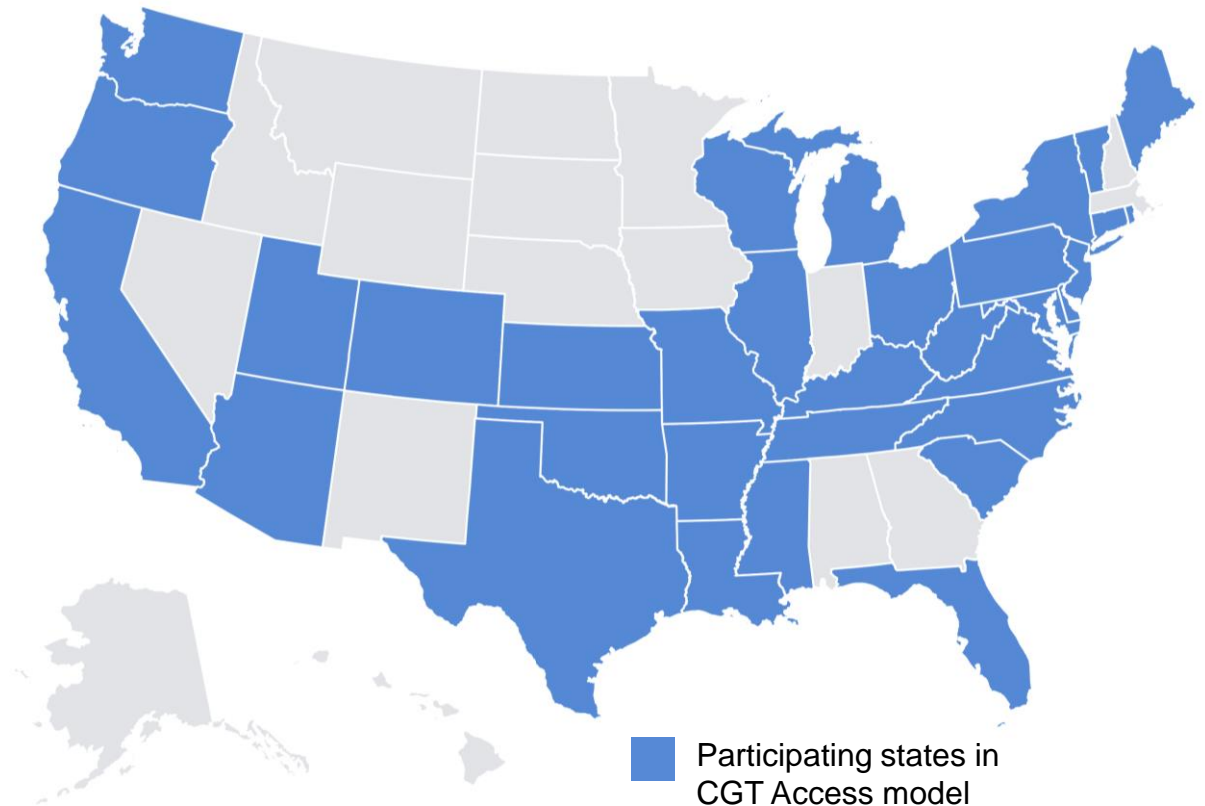
## Standard coverage + prior authorization rules

Reduces variability + lowers payer-level uncertainty

- ▶ Centers gain predictable Medicaid coverage and payment landscape
- ▶ Faster and cleaner financial clearance
- ▶ Patients reach treatment steps earlier

**84%** of U.S. Medicaid SCD beneficiaries covered

**88%** of active QTCs operate in CGT Access Model states

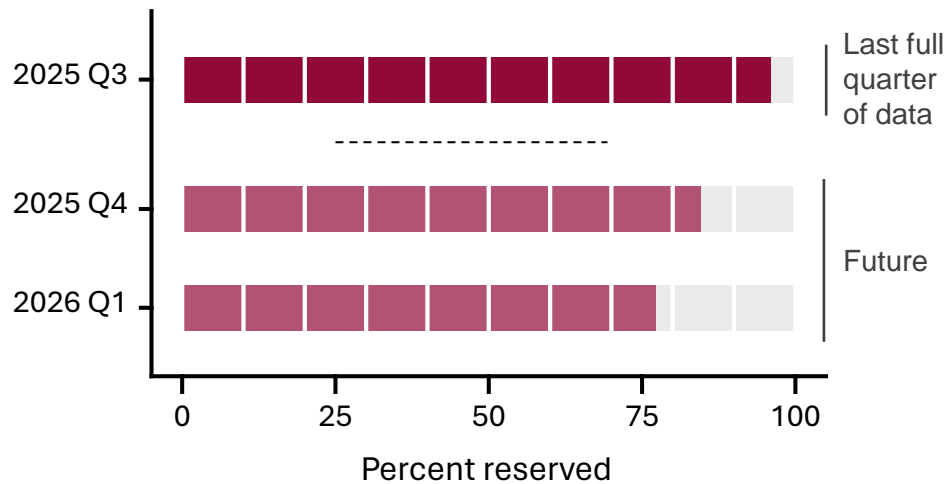


■ Participating states in CGT Access model

# Expanded Manufacturing Capacity Enables Faster Scheduling and Collection Initiation

## Zynteglo (beti-cel)

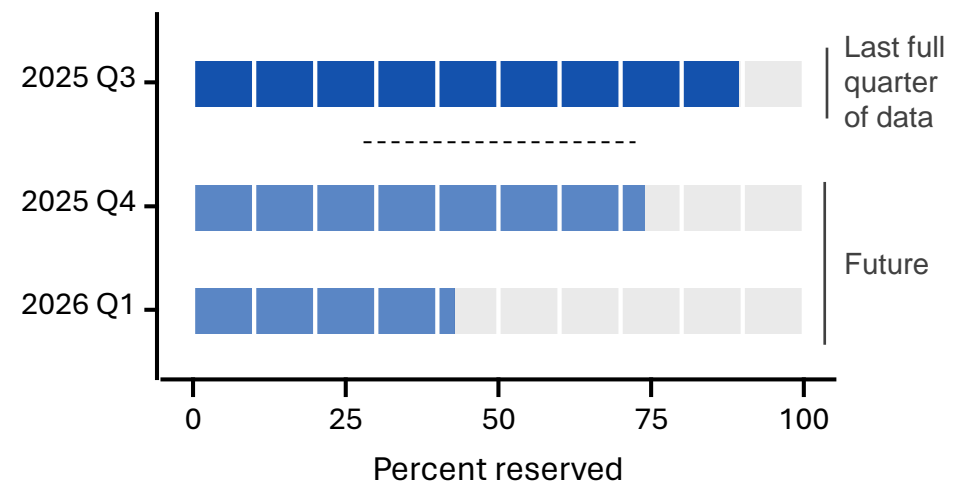
### Manufacturing Capacity Utilization



**96%** Manufacturing capacity utilization in Q3 2025 with slots booked out through Q1 2026<sup>1</sup>

## Lyfgenia (lovo-cel)

### Manufacturing Capacity Utilization

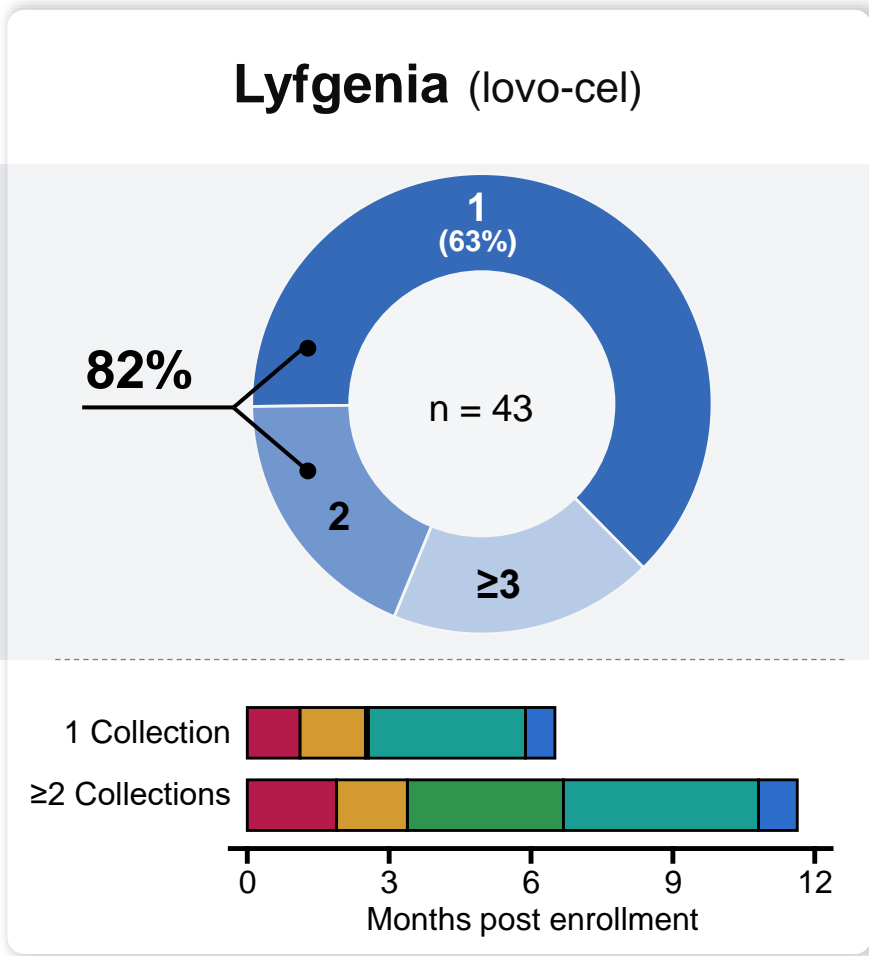
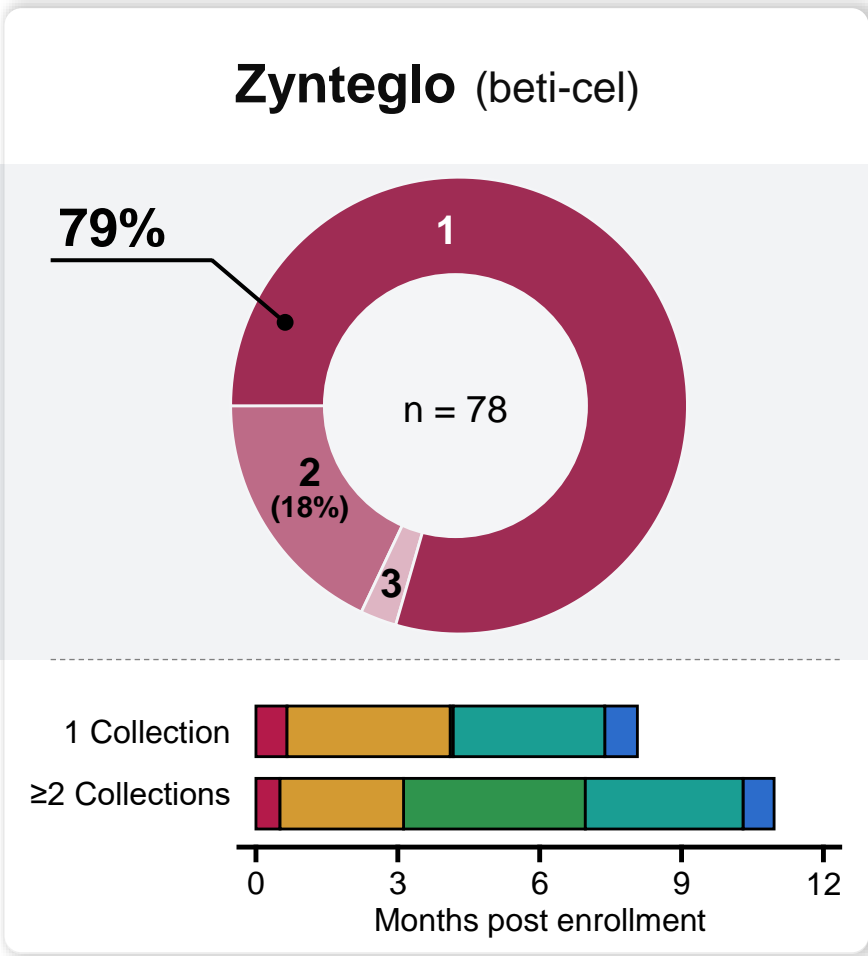


**89%** Manufacturing capacity utilization in Q3 2025 with slots booked out through Q1 2026

# Collection Success Enables Timely Progression to Treatment

The **majority** of commercially treated patients require **1 cell collection**

# of cell collection cycles is a key variable for overall **patient journey timeline**



■ Enrollment   
 ■ Scheduling   
 ■ Repeat Collection   
 ■ Manufacturing & testing   
 ■ Pending Infusion

Data as of November 14, 2025; Collection defined as 2 subsequent days of mobilization and apheresis; collection statistics for Lyfgenia patients include all patients who are ≥6 months after their first collection as of data cut (37 treated); bottom graphs n = 115 treated patients

# Real-world experience demonstrates the value of durable, one-time gene therapy

- Recent data collected from patients prescribed beti-cel & lovo-cel is the first to analyze commercial stem cell gene therapy implementation in the U.S.
- Data demonstrates the growing demand for and scalability of one-time gene therapies for sickle cell disease and thalassemia with increasing accessibility
- Experiences learned from the beti-cel launch and the commercial infrastructure Genetix has built has enabled rapid access to lovo-cel treatment for people with sickle cell disease nationwide
- Our commercial implementation sets a benchmark for delivery and reinforces the value of durable, one-time gene therapy to patients, providers, & payers