

**Live-cel™ Therapy**  
**Clinically Demonstrated**  
**lasts years, not weeks**

**Glafabra**  
Therapeutics

**Dr. Chris Hopkins, PhD, MBA**  
**chris@glafabra.com**  
**DM: 801 631 9114**

# Fabry Disease

## Standard of Care ERT

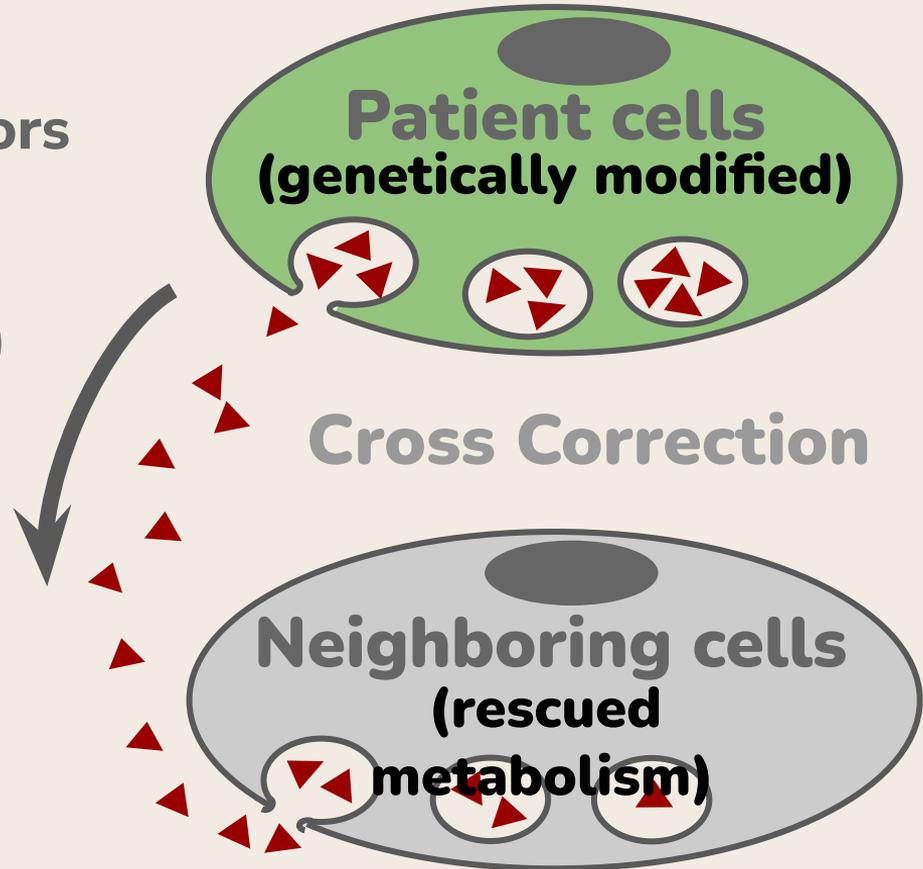
# Live-cel Therapy: GT-GLA-S03

One dose - every 5 years



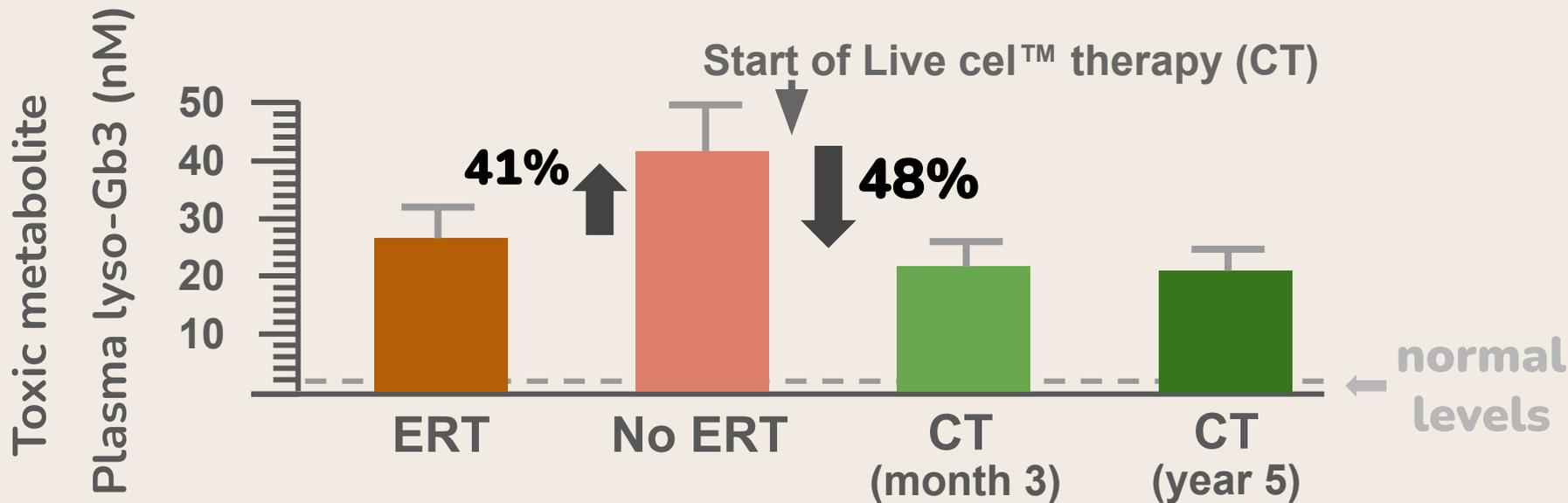
# Understanding GT-GLA-S03 Live-cel Therapy

- 1 Harvest Cells**  
White blood cell precursors
- 2 Modify Cells**  
Make missing enzyme (▲)
- 3 Return Cells**  
One-time cell infusion
- 4 Stop Toxic Metabolites**  
Rescue neighboring cells



# Live-cel GT-GLA-S03 in Patients

## Canadian Clinical Trial for Fabry on 5 Patients



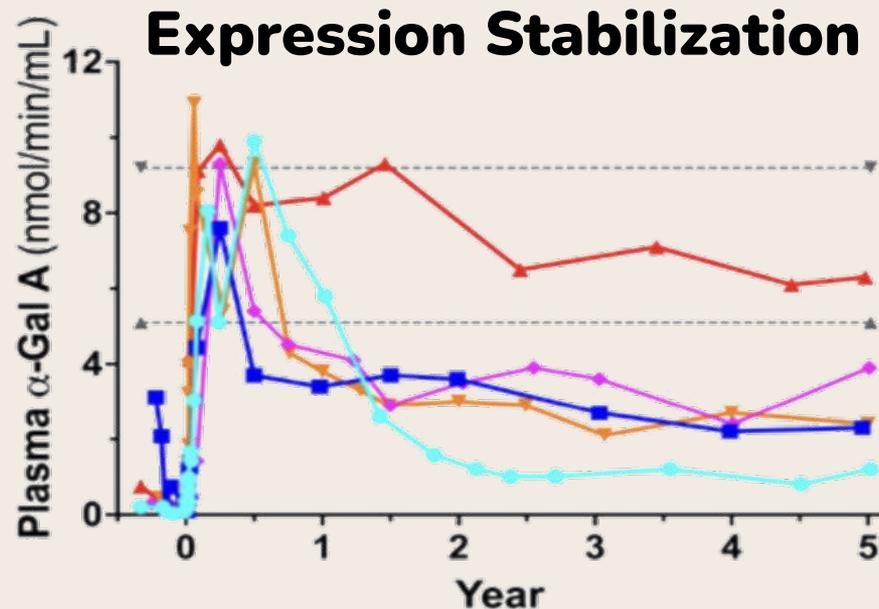
Joe - ***"The benefits were many!"***

***I have felt the BEST I've felt in my life"***

# Live-cel GT-GLA-S03: Safe and Stable

## Adverse Events

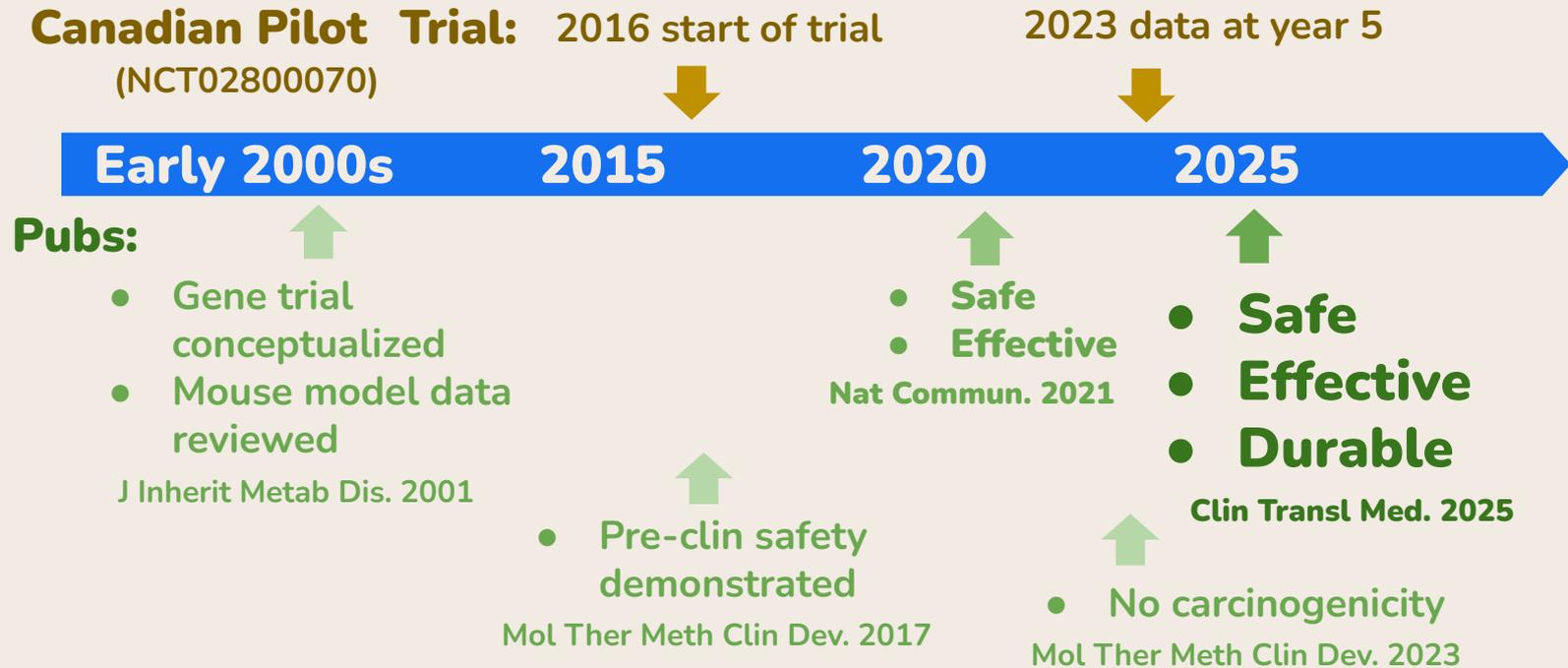
- AE: 228
- SAE: 2 (resolved)
- No Cardiac
- No Renal



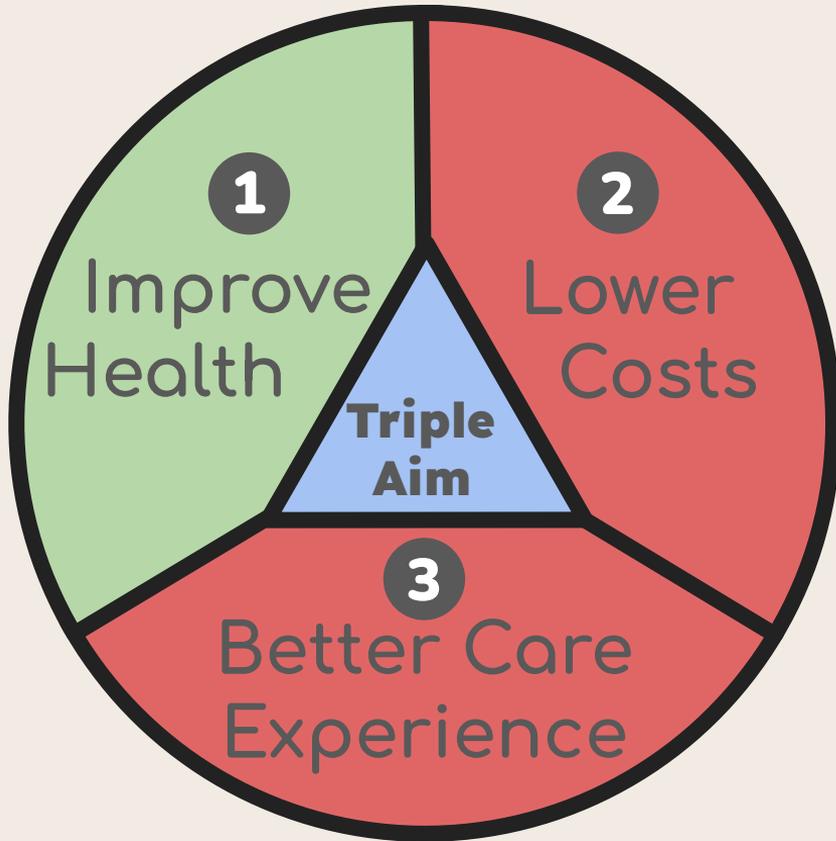
- No major adverse events
- Durable out to year 5 - redosable

# Glafabra Commercializing Live-cel: GT-GLA-S03

**Founders:** Drs. Chris Hopkins, Jeffrey Medin and Ronan Foley



# Prior Company Misstep



## Other company



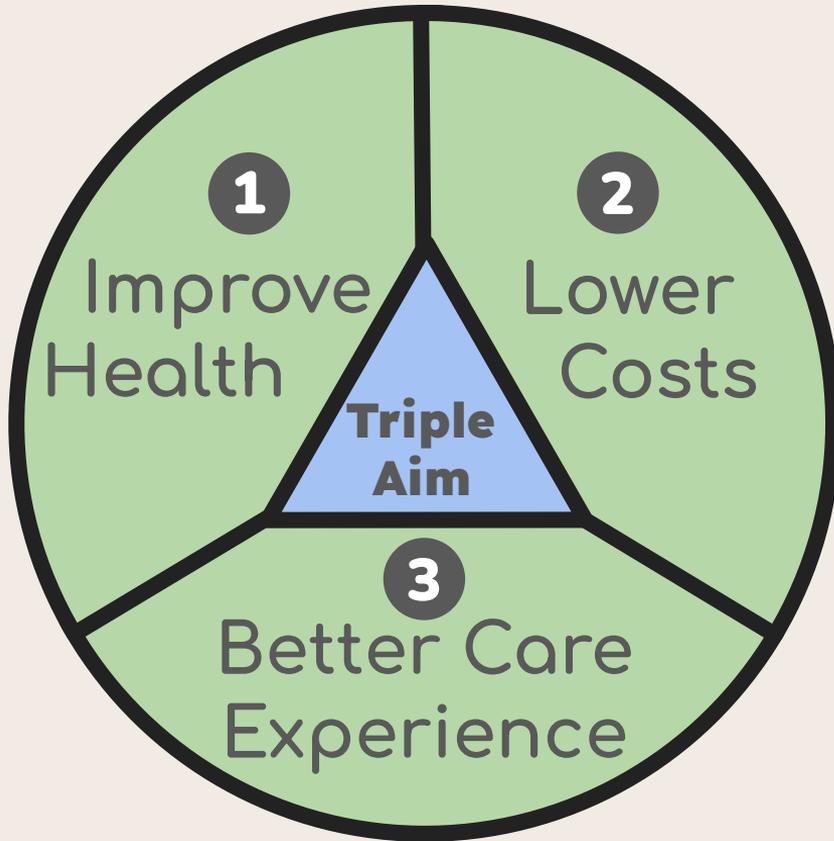
1. Decades long durability
2. High cost procedure
3. High patient burden

inpatient



**Bad market fit,  
Shelved asset**

# Superior Approach to Drug Development



**GT-GLA-S03**

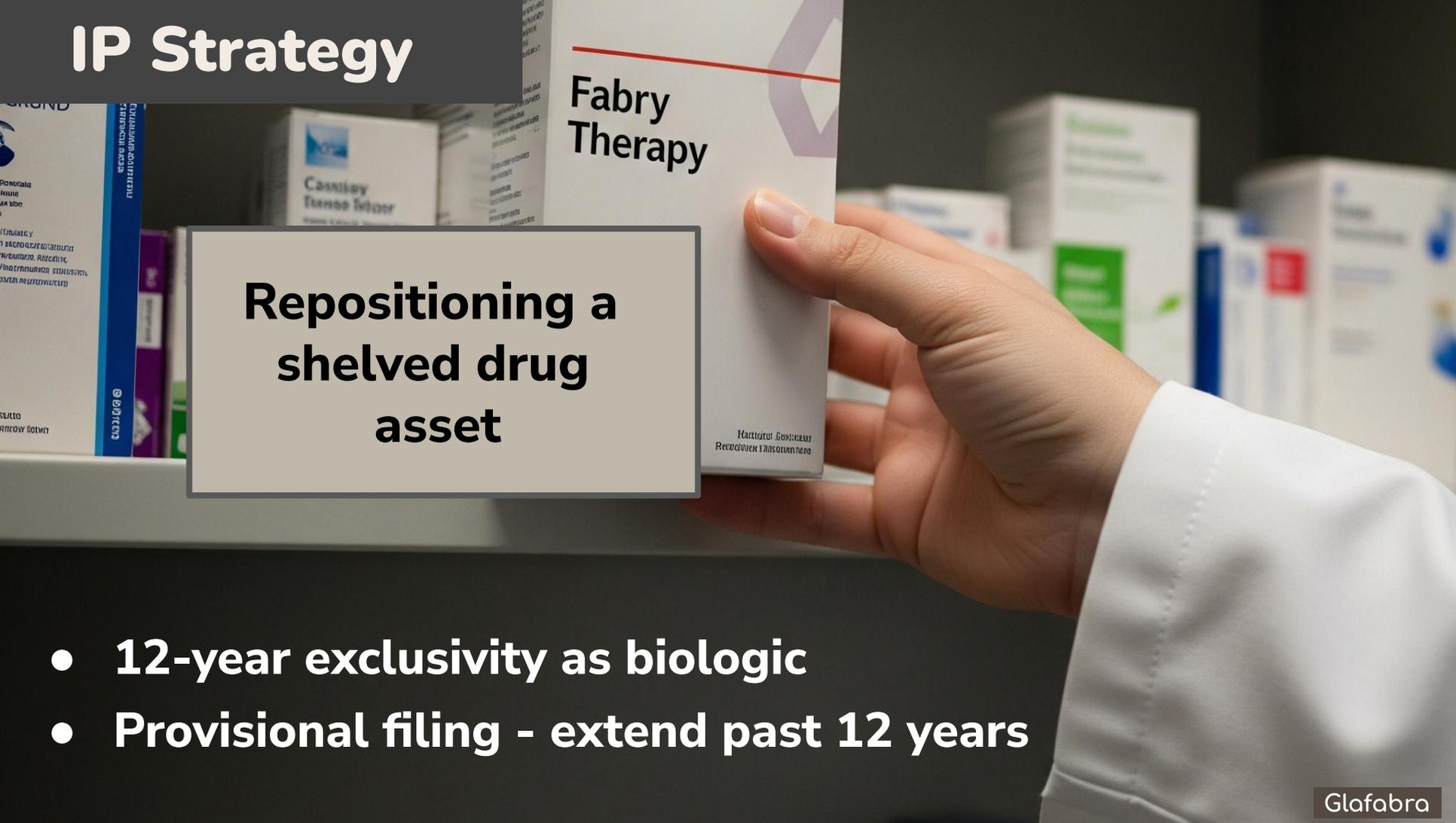


1. Long duration therapy
2. Outpatient and repeatable
3. Lower compliance burden



**Product-to-Market  
fit**

# IP Strategy

A hand in a white lab coat is holding a white box labeled "Fabry Therapy". The box features a red horizontal line and a purple geometric logo. The background shows a pharmacy shelf with various other medicine boxes, some with Cyrillic text. A semi-transparent white box with a dark border is overlaid on the image, containing the text "Repositioning a shelved drug asset".

## Repositioning a shelved drug asset

- **12-year exclusivity as biologic**
- **Provisional filing - extend past 12 years**

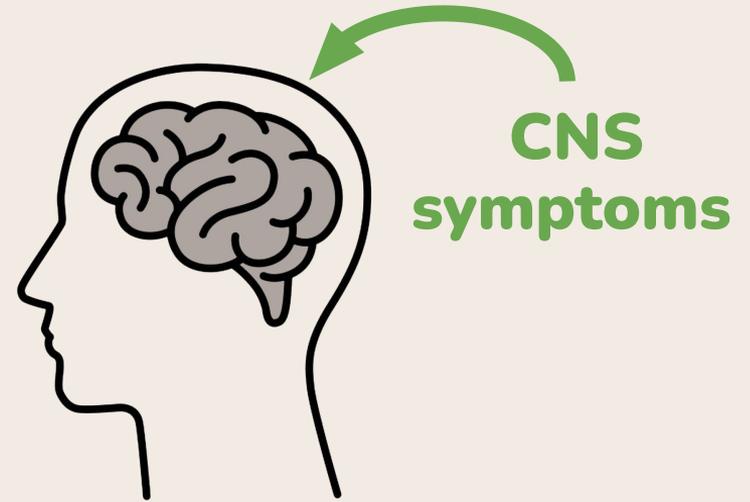
# Comps: Live-cel Therapy - Lifestyle Freedom

**Therapy:** GT-GLA-S03

**Long Duration:** ✓

**Large Effect:** ✓

**All Tissues:** ✓



- Long duration and dramatic effect
- Crosses blood brain barrier to treat CNS symptoms

# Comps: Live-cel Therapy - Lifestyle Freedom

Therapy: GT-GLA-S03 ERT

Long Duration:



Large Effect:



ERT spikes



Interrupted supply

GT-GLA-S03



Continuous supply

- Shorter duration and weaker effect
- Delivers dose in spikes instead of steady continuous supply

# Comps: Live-cel Therapy - Lifestyle Freedom

Therapy: **GT-GLA-S03**    **ERT**

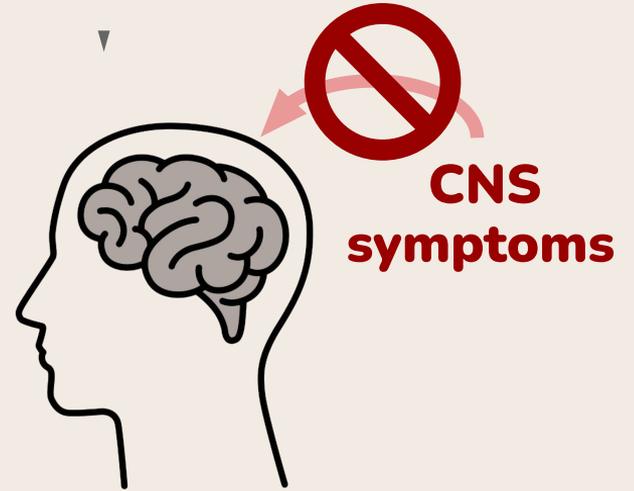
**Long Duration:**



**Large Effect:**



**All Tissues:**



- **Cannot easily cross blood brain barrier - CNS symptoms unresolved**

# Glafabra's Live-cel™ Disease Markets

## Fabry Disease

GT-GLA-S03



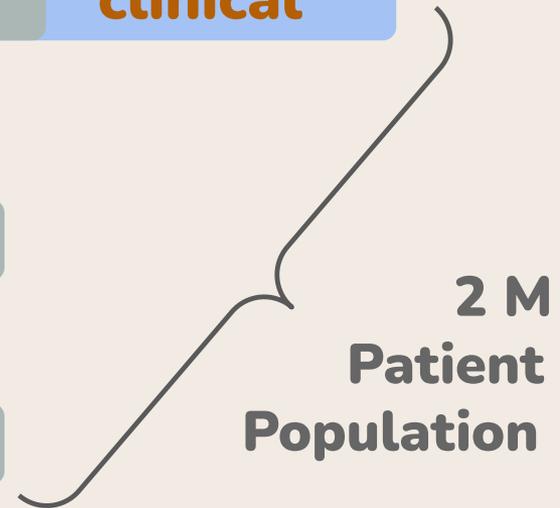
## Pompe Disease

GT-GAA-T03



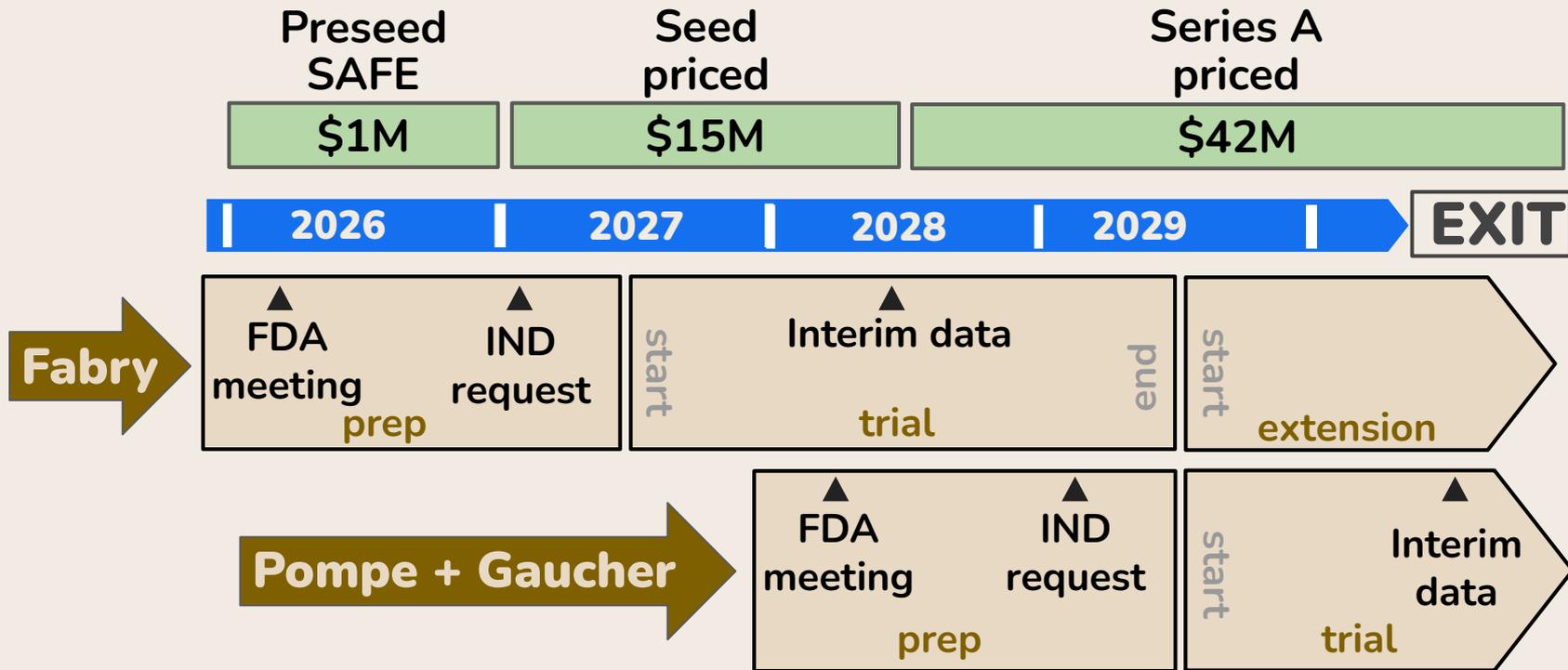
## Gaucher Disease

GT-GLB1-T04



- Sanofi - \$2.9 B/y from these disease categories with ERT

# Regulatory and Funding Map



- Phase II efficacy data - multi \$B exit

# Experienced Team

## Management



**CEO:**  
Dr. Chris Hopkins  
PhD, MBA



**COO:**  
Elizabeth Wagner  
MS, MBA



**Reg. Science:**  
Dr. Krista Casazza  
PhD



## Co-founders



**CSO:**  
Dr. Jeffrey Medin  
PhD



**CMO:**  
Dr. Ronan Foley  
MD

### Business Advisors:

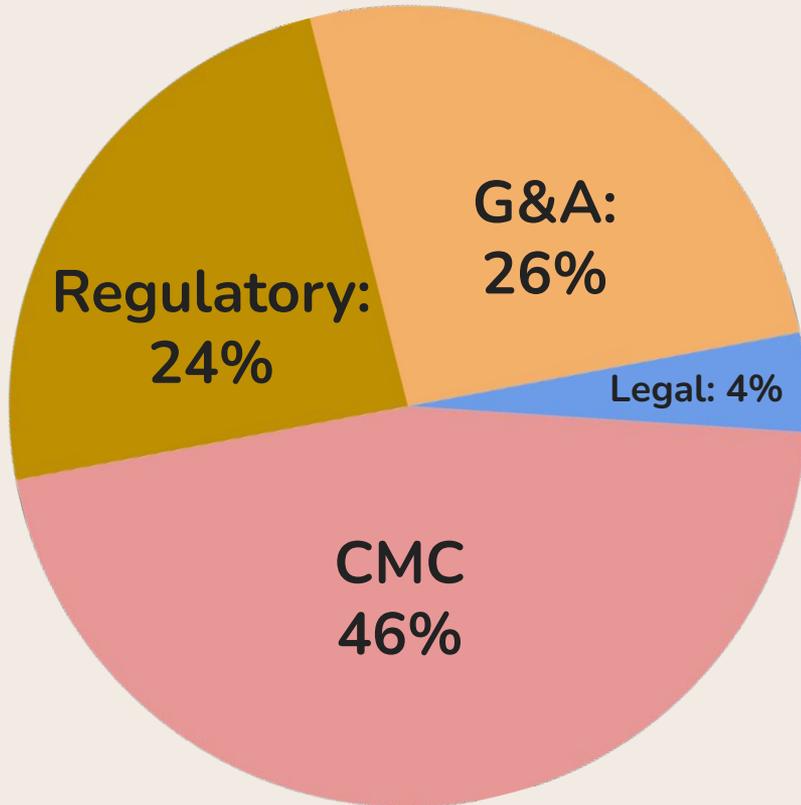
Katie Hewitt      Dwayne Barber  
Laura Viaches      Lara Silverman

### Science Advisors:

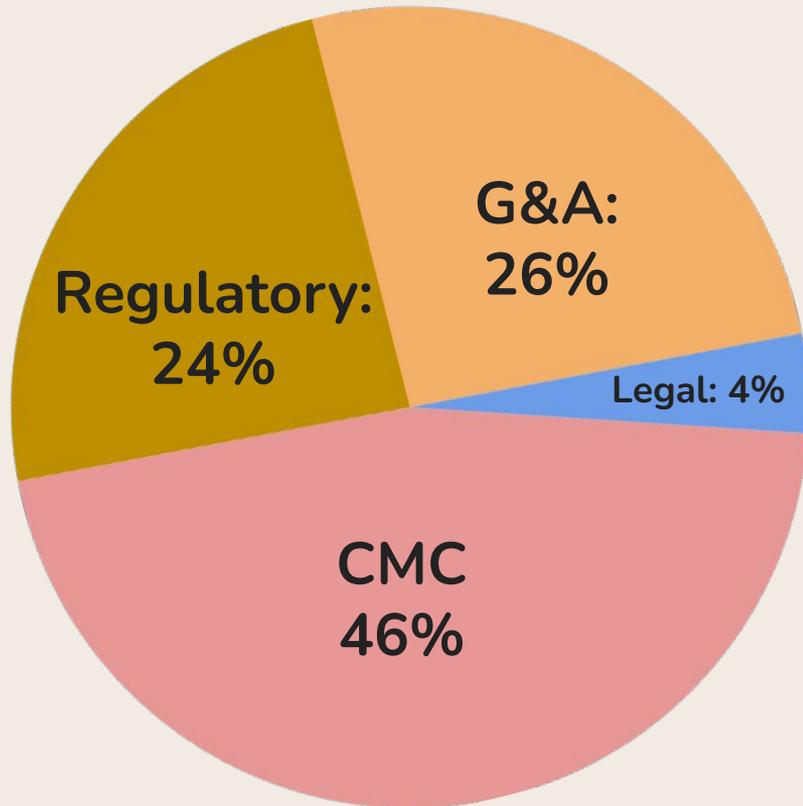
Dr. Armand Keating, MD      Dr. Aneal Khan, MD  
Dr. Michael West, MD,      Dr Daniel Fowler, MD

# Use of Funds

Pre-seed  
**\$1,000,000**



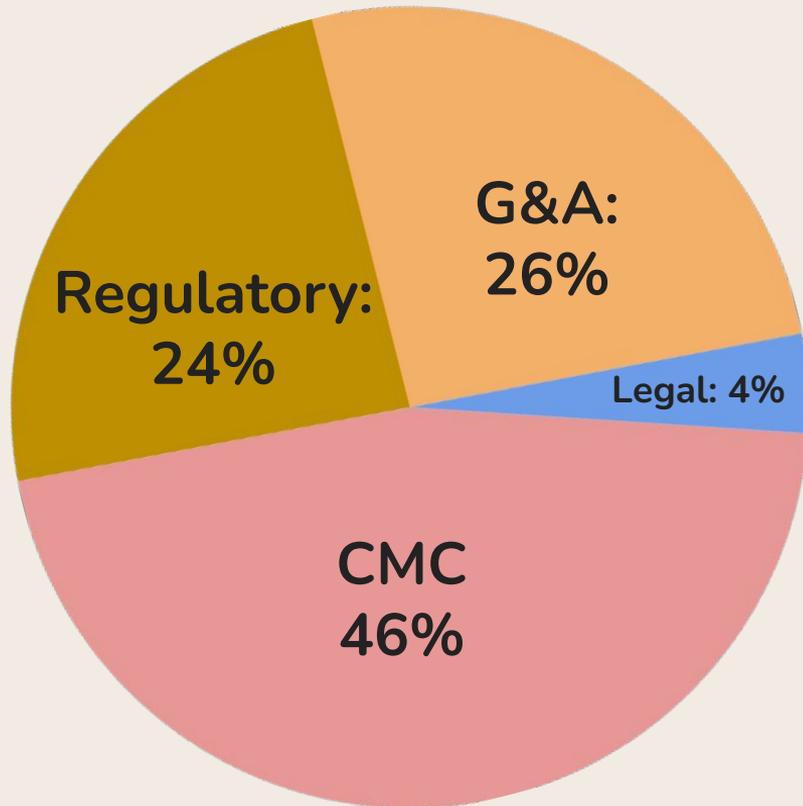
# Use of Funds



**Pre-seed**  
**\$1,000,000**

- Secure orphan drug designation (ODD)

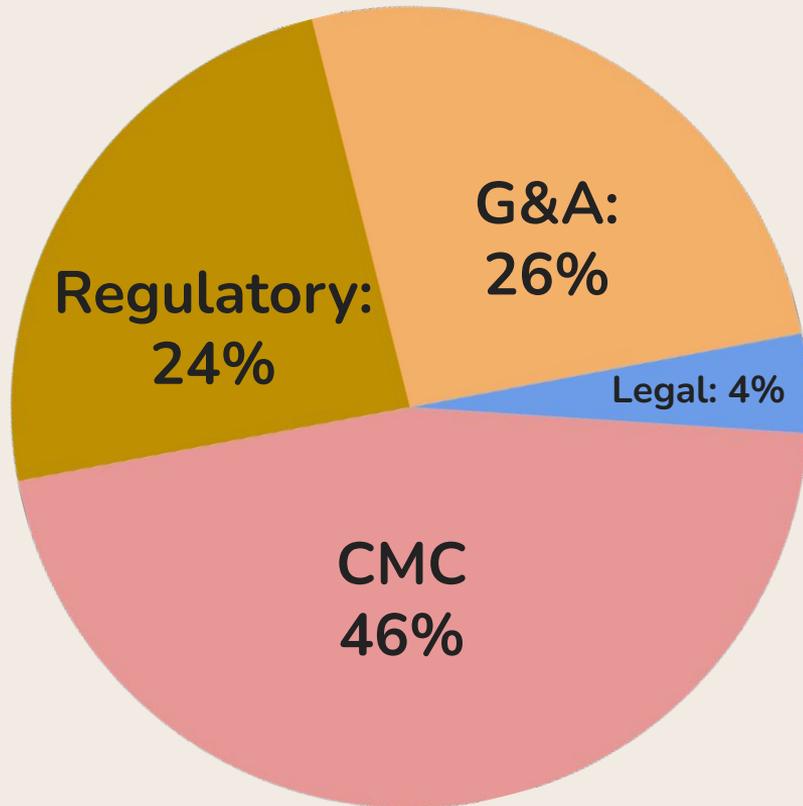
# Use of Funds



**Pre-seed**  
**\$1,000,000**

- Secure orphan drug designation (ODD)
- Hold first meeting with FDA (INTERACT)

# Use of Funds



**Pre-seed**  
**\$1,000,000**

- Secure orphan drug designation (ODD)
- Hold first meeting with FDA (INTERACT)
- Test CMC pathway and find best manufacturing approach

- **Live-cel™ Technology**
- **Clinical Fabry data**

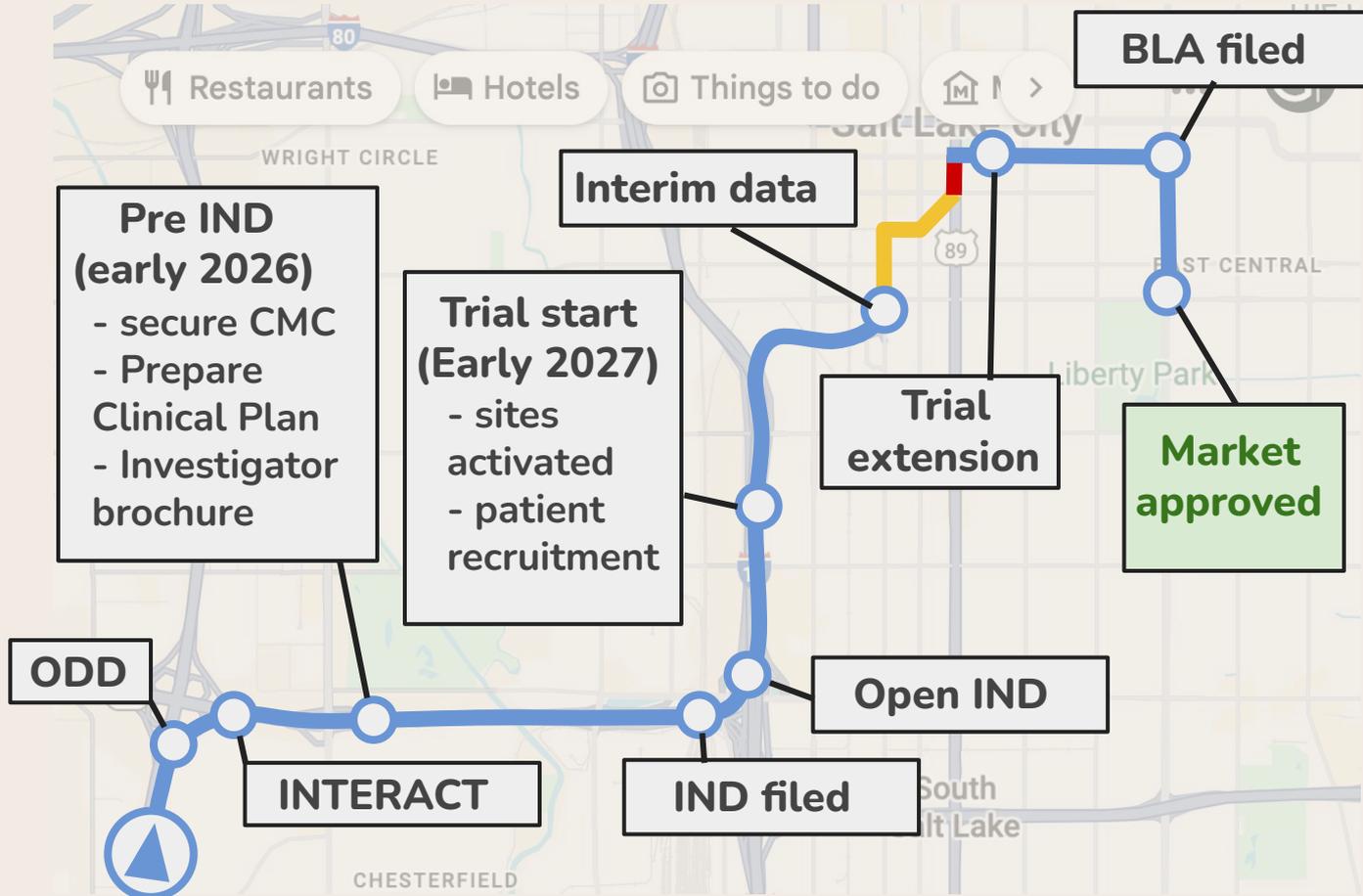
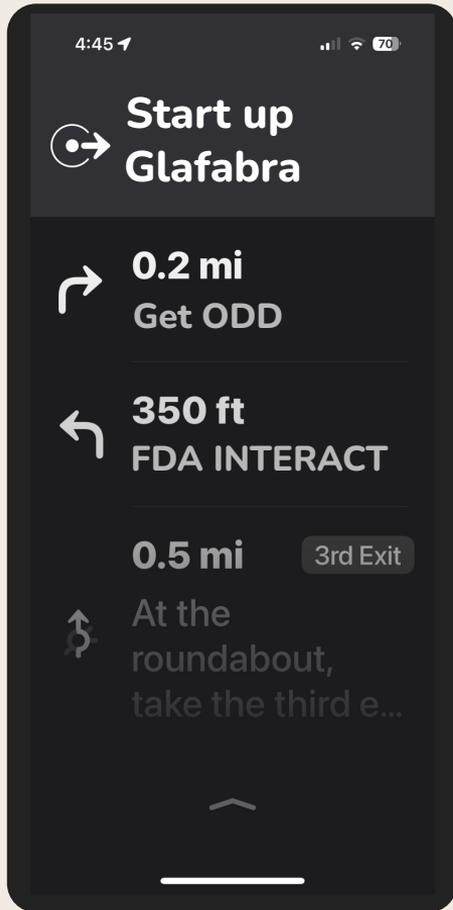
**Safe - Effective - Durable**

**Glafabra**  
Therapeutics

**Joe - “At one point, my wife  
and I realized we were  
forgetting I had Fabry at all”**

**END**

# Roadmap to Market Access



# Clinical Trial Plans

## Obtain IND



### Steps:

- Orphan Drug Designation
- INTERACT Meeting
- Pre IND meeting
- Submit IND package
- FDA clearance (Open IND)



## Clinical Site (main)



**HEALTH**  
UNIVERSITY OF UTAH



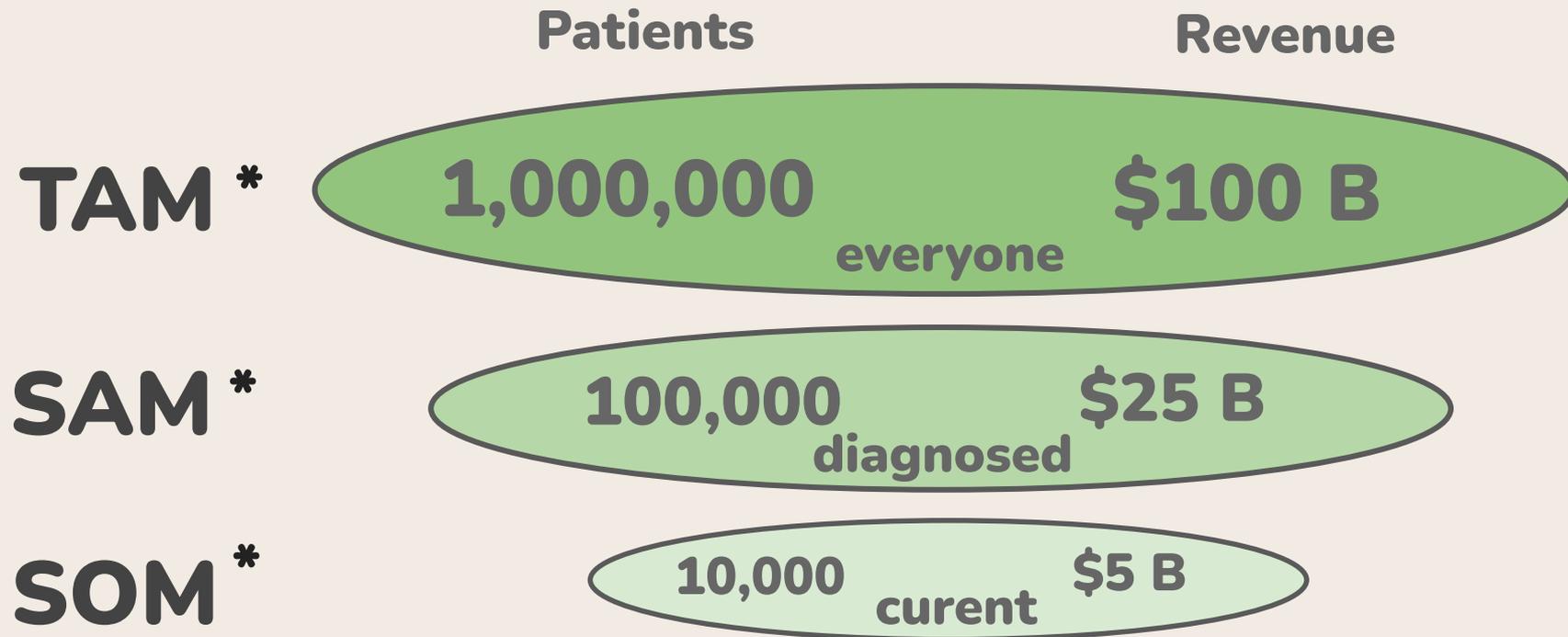
### Clinical lead:

- Dr. Brian Shayota,  
MD, MPH

### Specialty:

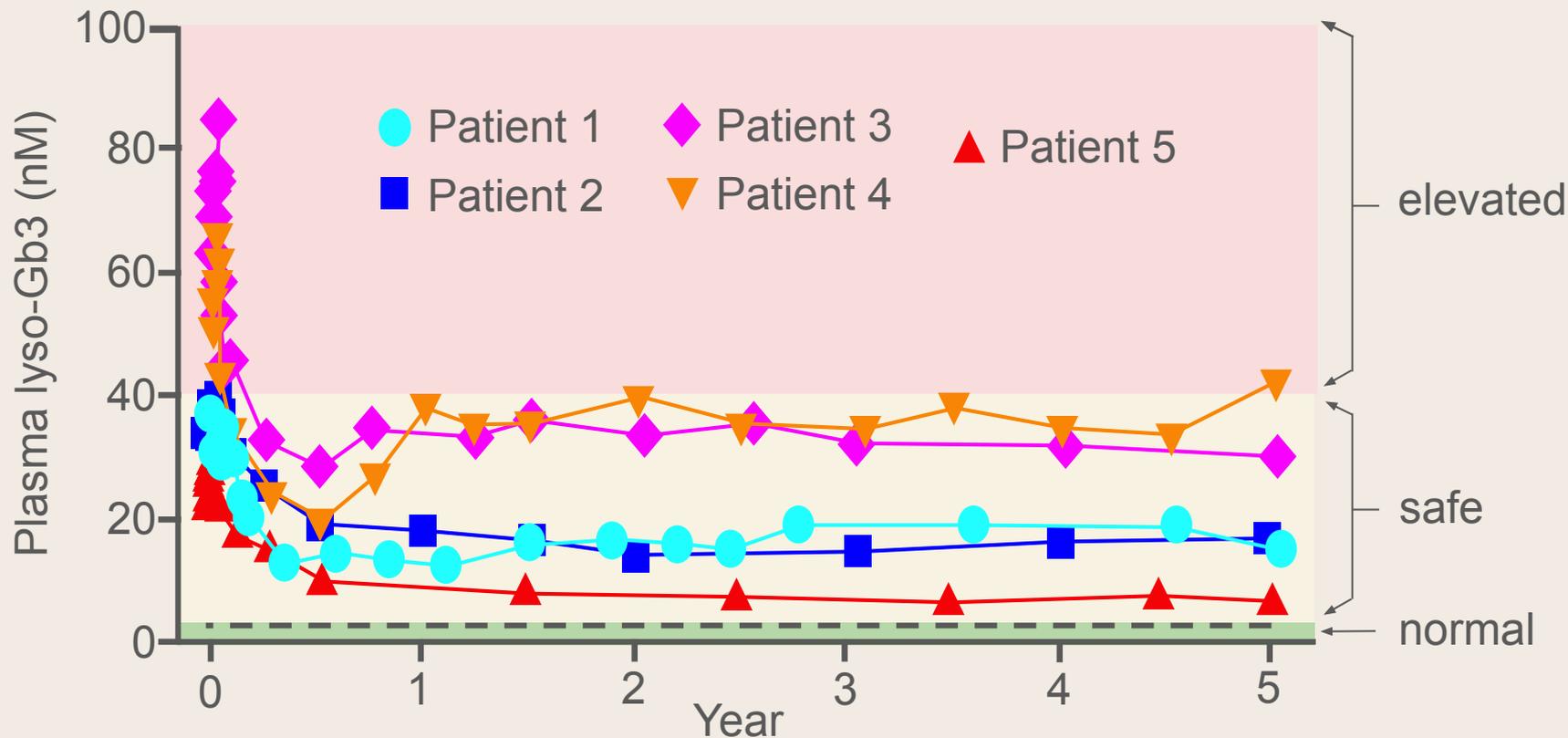
- Medical Genetics

# Glafabra's Live-cel™ Market Opportunity



\* 50% market share

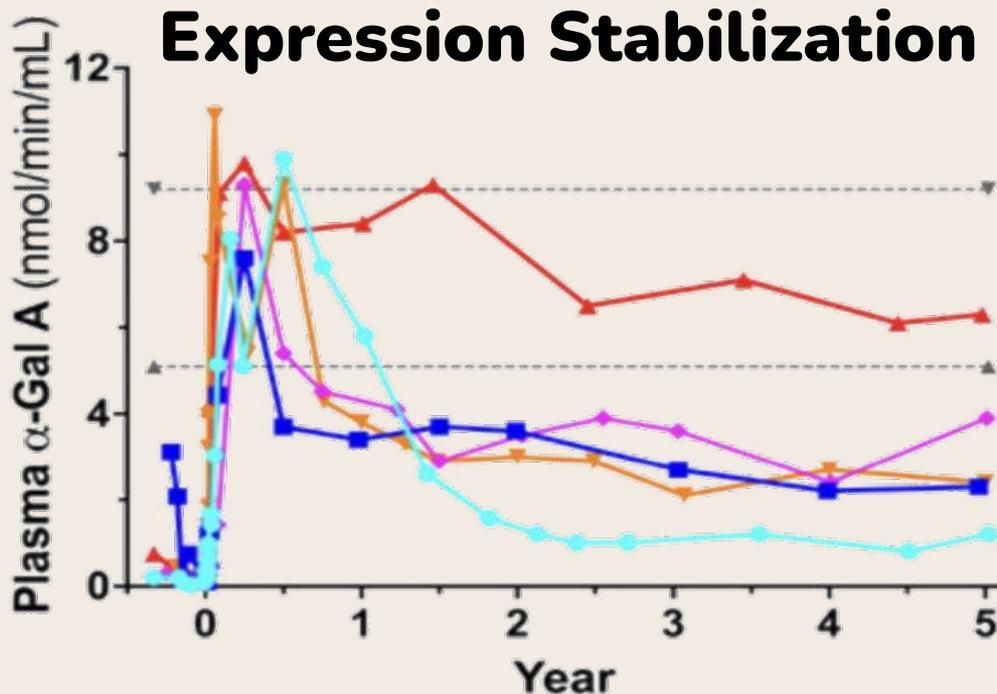
# Live-cel GT-GLA-S03: 5 Patients



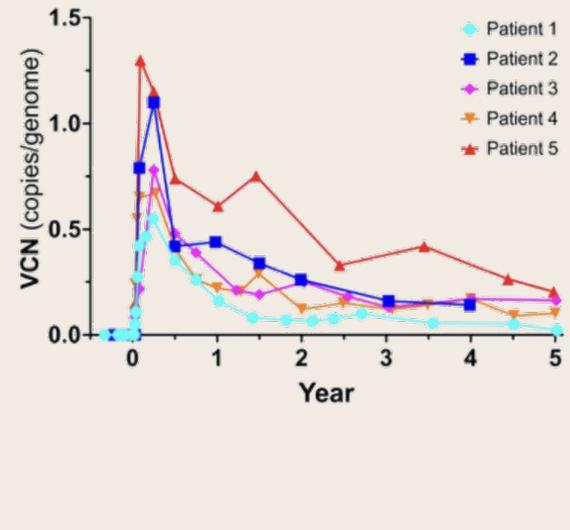
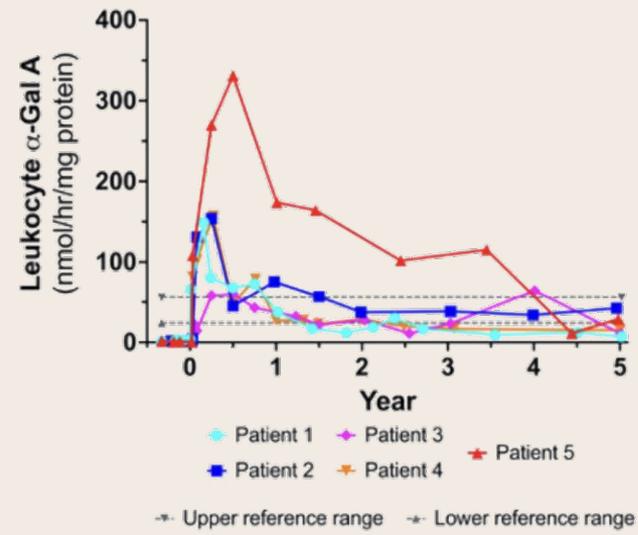
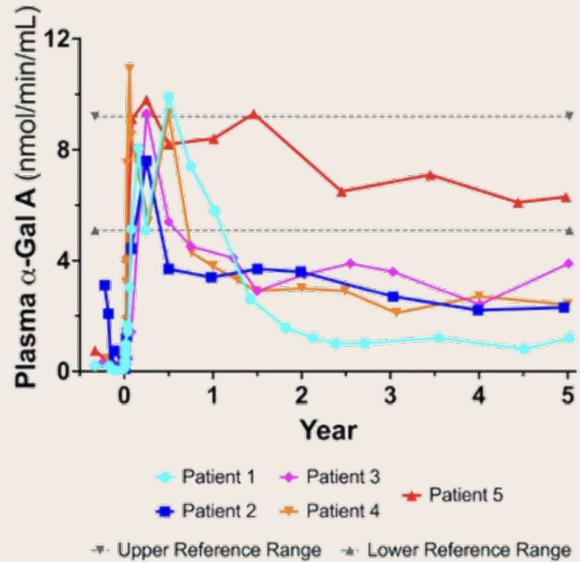
# Live-cel GT-GLA-S03: Safe and Stable

## Adverse Events

- AE: 228
- SAE: 2 (resolved)
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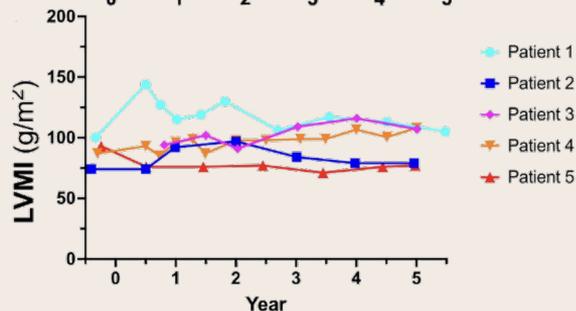
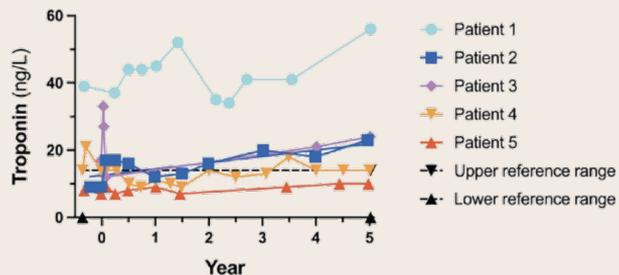
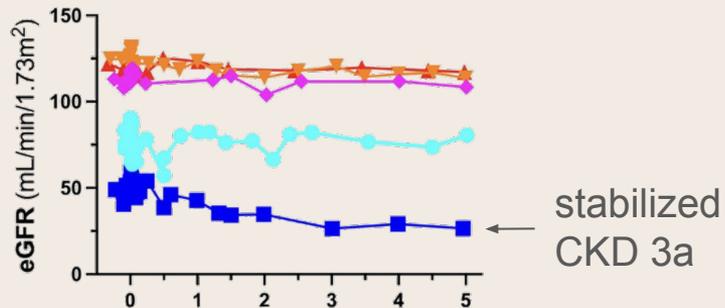
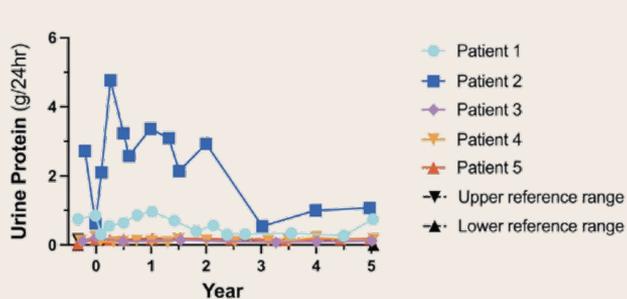
# GT-GLA-S03: Durability in expression and copy number (Pilot clinical trial for Fabry in Canada)



- **Monitored: Plasma  $\alpha$ -Gal A, Leukocyte  $\alpha$ -Gal A, and vector copy number**

# GT-GLA-S03: Renal and Cardiac Stability

## (Pilot clinical trial for Fabry in Canada)



- AEs: 228
- AEs G3-4: 20
- pSAEs: 2

- **Kidney: Proteinuria and eGFR Rates**
- **Heart: Troponin and LVMI**
- **Adverse Events: AEs and SAEs**

# Comps: Live-cel Therapy - Lifestyle Freedom

Therapy:

GT-GLA-S03

ERT

chaperone \*

AAV

Long Duration:



Large Effect:



Repeatable:



All Tissues:



5 year cost:

\$0.5 M

\$1.5 M

\$1.5 M

??

- 3x lower cost, 5x margin.