A New Era of Medicine

October 27, 2026

NASDAQ: BEAM

Beam THERAPEUTICS

Today's agenda

TOPIC	PARTICIPANT
Introduction	**Holly Manning** Vice President, Investor Relations & External Communications
Beam's Vision for Leading a New Era of Medicine	**John Evans** Chief Executive Officer
GSDIa: BEAM-301 Clinical Data at ESGCT	**Amy Simon, M.D.** Chief Medical Officer
PKU: Beam's Platform Approach in Action	**Giuseppe Ciaramella, Ph.D.** President
Q&A	**Mr. Evans, Dr. Simon & Dr. Ciaramella**

Cautionary note regarding forward-looking statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding: the therapeutic applications and potential of our technology, including with respect to GSDIa and PKU; our plans, and anticipated timing, to advance our programs, including risto-cel, BEAM-103, BEAM-301, BEAM-304 and BEAM-302; our plans and anticipated timing to present data from ongoing clinical trials; our anticipated regulatory interactions and filings; our current expectations and anticipated results of operations, including our expected use of capital; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; and the therapeutic applications and potential of our technology, including our potential to develop lifelong, curative, precision genetic medicines for patients through base editing, including potential safety advantages, all of which are subject to known and unknown important risks, uncertainties and other factors that may cause our actual results, performance or achievements, market trends, or industry results to differ materially from those expressed or implied by such forward-looking statements. Therefore, any statements contained herein that are not statements of historical fact may be forward-looking statements and should be evaluated as such. Without limiting the foregoing, the words "anticipate," "expect," "suggest," "plan," "vision," “strategy,” “possibility,” “promise,” "believe," "intend," "project," "forecast," "estimates," "targets," "projections," "potential," "should," "could," "would," "may," "might," "will," and the negative thereof and similar words and expressions are intended to identify forward-looking statements. Each forward-looking statement is subject to important risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval for, and commercialize our product candidates, which may take longer or cost more than planned; our ability to raise additional funding, which may not be available; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that initiation and enrollment of our clinical trials may take longer than expected; that our product candidates or the delivery modalities we rely on to administer them may cause serious adverse events; the uncertainty that our product candidates will receive regulatory approval necessary to initiate or continue human clinical trials, that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the headings "Risk Factors Summary" and "Risk Factors" and elsewhere in our annual report on Form 10-K for the year ended December 31, 2025, our quarterly reports on Form 10-Q, and in any subsequent filings with the Securities and Exchange Commission (the "SEC") which are available on the SEC's website at www.sec.gov. Additional information will be made available by our annual and quarterly reports and other filings that we make from time to time with the SEC. These forward-looking statements speak only as of the date of this presentation. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

Leading a new era of medicine

At Beam, we aim to:

Address the root cause of serious genetic diseases

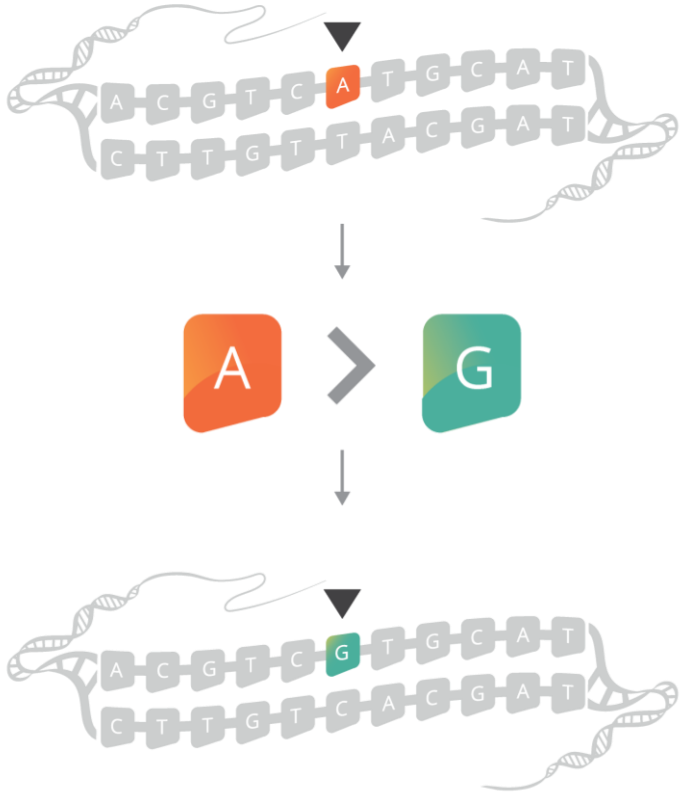
With one-time therapies that prevent a lifetime of disease progression

Ending chronic treatment for patients

Lowering lifetime costs for the healthcare system

Beam aims to correct the genetic root cause of severe diseases with base editing

BASE EDITING TECHNOLOGY



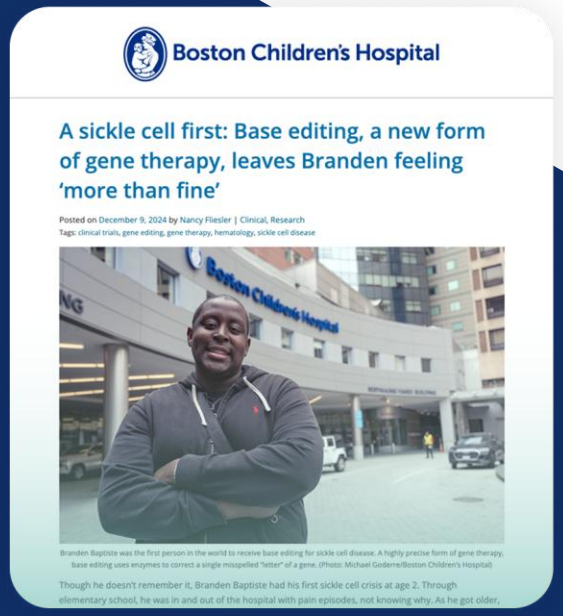
DESIGNED FOR:

- **CONSISTENT gene sequence outcomes**
- **DURABLE correction for one-time cures**
- **LESS GENOTOXICITY than traditional gene editing**

Predictable, Transformative Outcomes for Patients

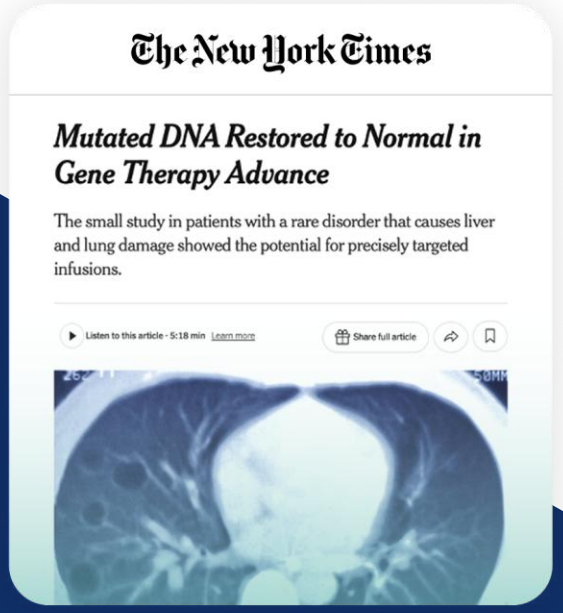
Our vision for base editing is becoming a reality for patients

2024



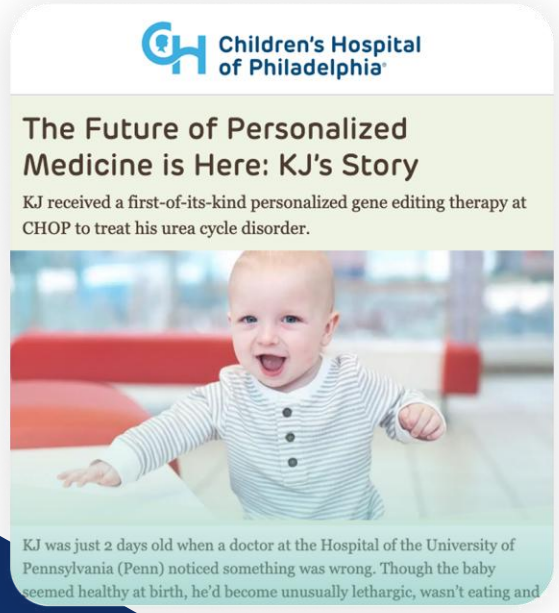
Sickle cell disease (SCD)

2025



Alpha-1 antitrypsin deficiency (AATD)

2025



Urea cycle disorder

2026

Glycogen storage disease Ia (GSDIa)

https://answers.childrenshospital.org/sickle-cell-base-editing/

https://www.nytimes.com/2025/03/10/health/gene-editing-beam-mutation-dna.html

https://www.chop.edu/centers-programs/genetherapy4inheritedmetabolicdisorders/future-personalized-medicine-here-kjs

Genetic correction could transform daily life for patients with GSDIa

Beam THERAPEUTICS

Life today with GSDIa
Constant vigilance, little room for error

Cornstarch doses every day, for life + emergency rescue kit

Missing a dose can lead to severe hypoglycemia, coma or death

Constant spikes and plunges in glucose

Wake multiple times each night for scheduled cornstarch doses

Vision after correcting root cause
A life no longer organized around GSDIa

No more cornstarch doses

Freedom from around-the-clock dietary dosing

Normal, stable glucose levels

Sleep through the night

Uninterrupted rest for patients and families

The power of predictability:
Beam is building a reliable model for advancing genetic medicine

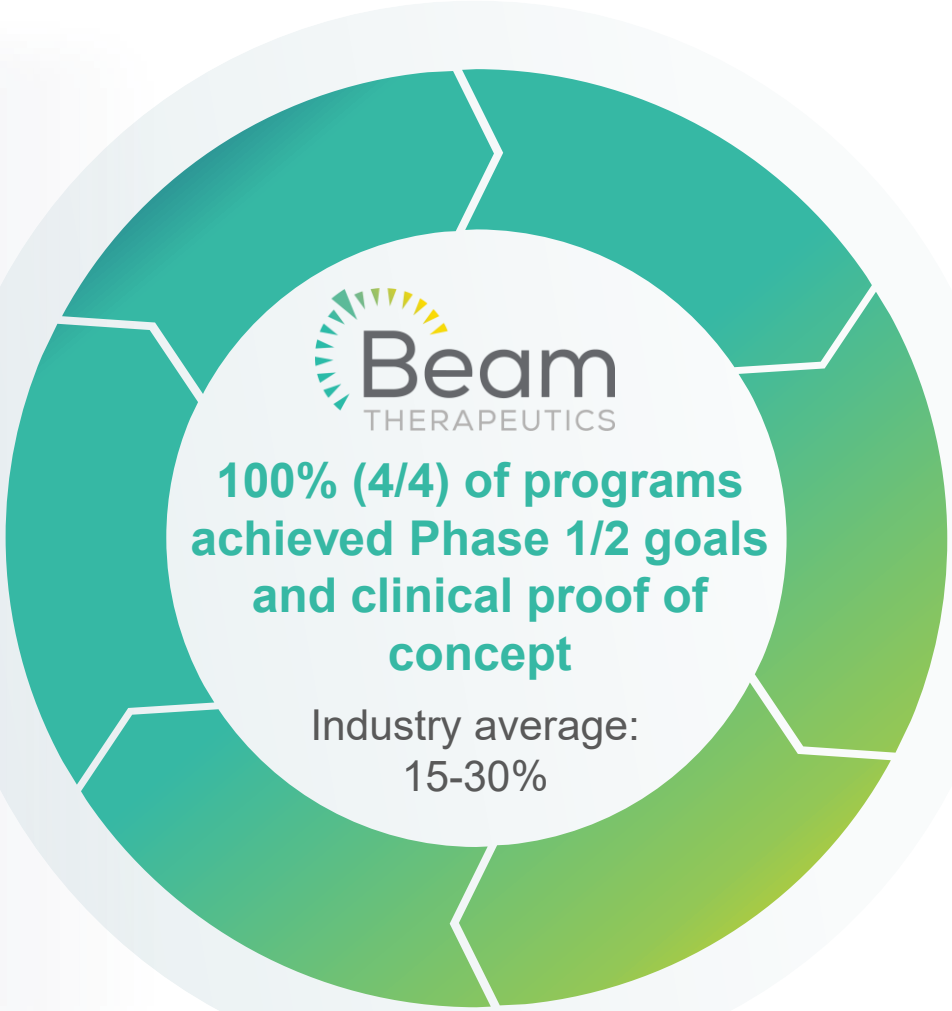
Easily **ADAPTABLE** to new targets

REUSABLE delivery technology

CONSISTENT preclinical and clinical outcomes

HIGHLY SCALABLE internal manufacturing

FLEXIBLE emerging regulatory framework



Multiple growing, high-value franchises

Active pipeline **expansion**

Creative platform **partnerships**

4/4 includes risto-cel, BEAM-302, BEAM-301 and BEAM-201.
Industry average is per program from start of Phase 1 to end of Phase 2. Source: BIO report; Clinical Development Success Rates and Contributing Factors 2011–2020

Building the capabilities and infrastructure to advance genetic medicines at scale

Decision in 2020 to establish in-house "full stack" production of CRISPR medicines

Beam North Carolina: 100,000+ sq. ft. for manufacturing and quality release

Beam Cambridge: Editing platform, automation and computational biology

200 FTEs for design and GMP production of customized gene correction doses

- Vertically integrated
- On-demand multi-scale production
- AI-enabled editor optimization

>100 patients treated in clinical studies to date

- 8 IND/CTA approvals
- 3 FDA priority designations
- 100s of validated assays and procedures for cross-referencing to new programs

Our most advanced, pivotal-stage programs target large markets and provide opportunity for significant value creation

Sickle Cell Disease (SCD)

RISTO-CEL

~10K-100K

U.S. patients eligible for *ex vivo* treatment and *in vivo* treatment, respectively

- Differentiated clinical profile for risto-cel
- Internal manufacturing
- Poised to enter an established market with high patient demand
- Next-wave *in vivo* approach making rapid progress

Alpha-1 Antitrypsin Deficiency (AATD)

BEAM-302

~100K

U.S. patients with severe (PiZZ) AATD

- Potential first-in-class, one-time *in vivo* therapy addressing liver and lung manifestations
- Only genetic medicine in pivotal trial for AATD
- Designed to convert patients' disease risk to carrier-like status
- Long-term leadership and commitment to AATD patients

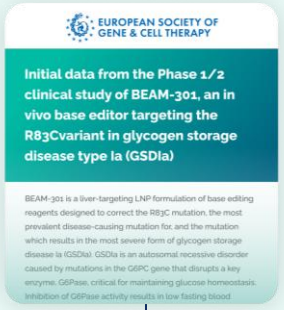
Beam has a long-term commitment to provide **best-in-class therapies for SCD and AATD**

U.S. addressable population estimates per Beam internal data and market research.

FDA is embracing new regulatory paradigms for advanced therapies, potentially enhancing our ability to help more patients

Invention of base editing for precise CRISPR gene editing

Beam industrializes base editing and builds internal manufacturing



Base Editing and LNP Platform Technology

2016-2017	2018-2023	2024	2025	2025	2026

FDA Platform Regulations Applicable to Base Editing

"Toward a new framework for… individualized therapies"

Peter Marks, Celia Witten Nature

FDA Guidance:

"Platform Technology Designation Program"*

"FDA's New Plausible Mechanism Pathway"

Marty Makary, Vinay Prasad NEJM

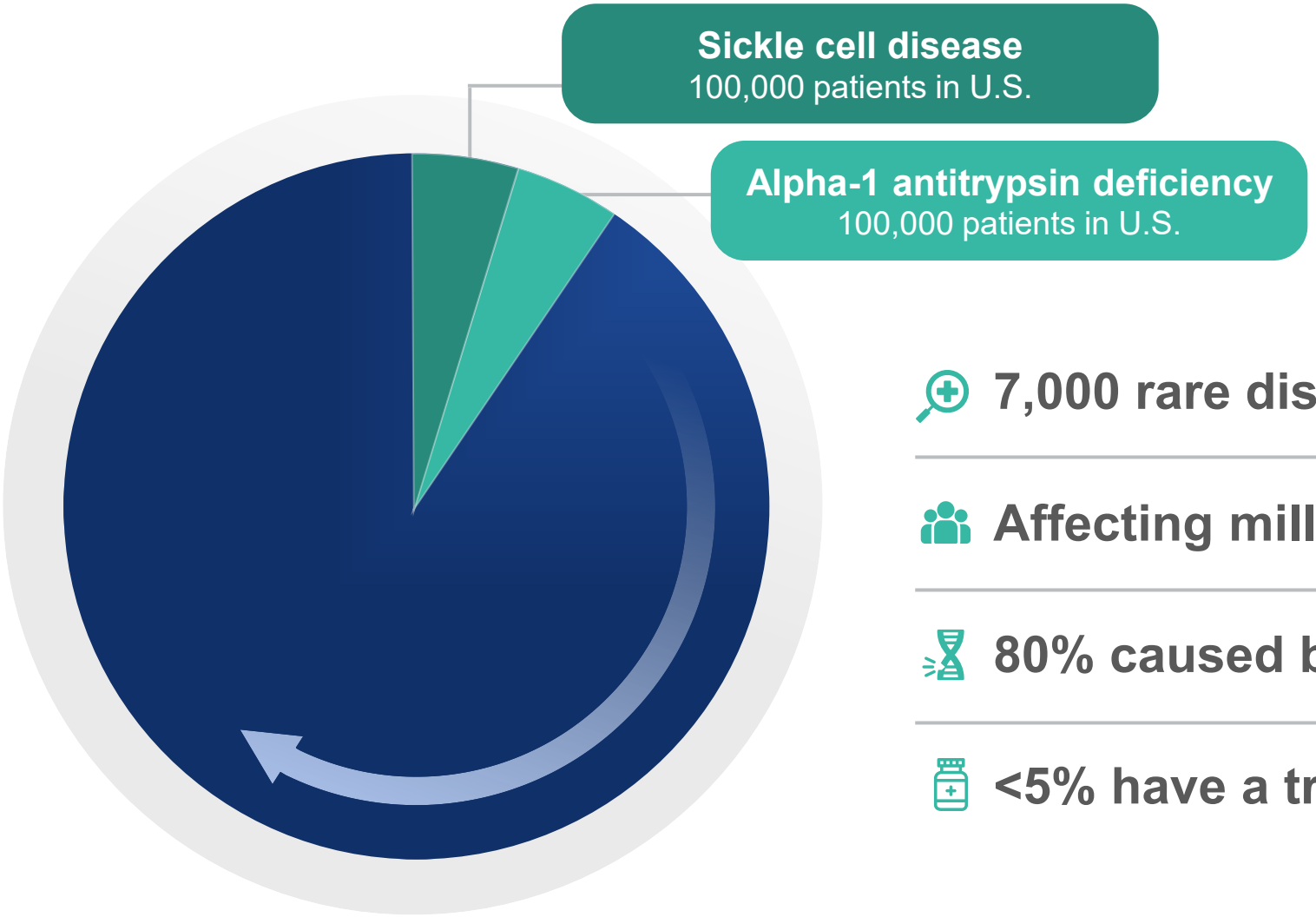
Pediatric Rare Disease Priority Review Voucher program renewed

FDA Guidance:

1. "Use of the Plausible Mechanism Framework to Develop Individualized Therapies"*
2. "CMC Flexibilities for Gene Therapy Products"
3. "Leveraging Prior Knowledge in Gene Editing Products"*

*Draft guidance as of October 2026

We believe we have the potential to significantly expand the addressable patient population for gene correction



- **7,000 rare diseases**
- **Affecting millions of patients**
- **80% caused by a single genetic defect**
- **<5% have a treatment option**

Cabré-Romans J-J and Cuella-Martin R (2025) CRISPR-dependent base editing as a therapeutic strategy for rare monogenic disorders. Front. Genome Ed. 7:1553590.

GSDIa and PKU serve as examples of efficient, platform-based development of precision genetic medicines

GSDIa: Efficient Development for Rare Indication

- Initial clinical data suggest BEAM-301 restored endogenous glucose production following a single *in vivo* treatment
- Two patients at 40 mg reached 15-hour fasting maximum and 100% decrease in daily starch intake
- Data further validate Beam's *in vivo* LNP capabilities and reusable delivery technology
- 100% (4/4) of Beam programs have delivered clinical proof of concept in line with preclinical studies

PKU: Platform Approach to Reach Multiple Variants

- Pursuing a multi-mutation approach using novel regulatory pathways
- Beam selected third base editor for new PKU variant
- BEAM-304 program now has potential to address up to 50% of U.S. patients with PKU

GSDIa: Clinical Proof of Concept for BEAM-301

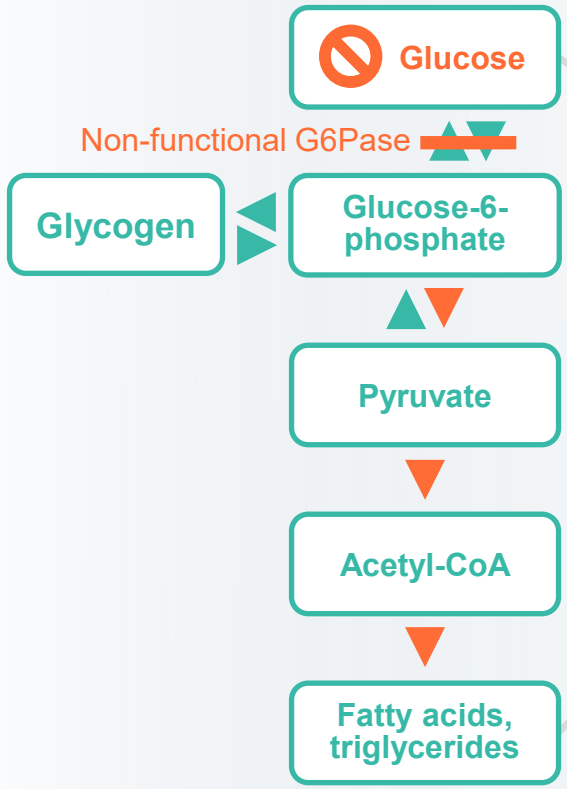
Amy Simon, M.D.
Chief Medical Officer

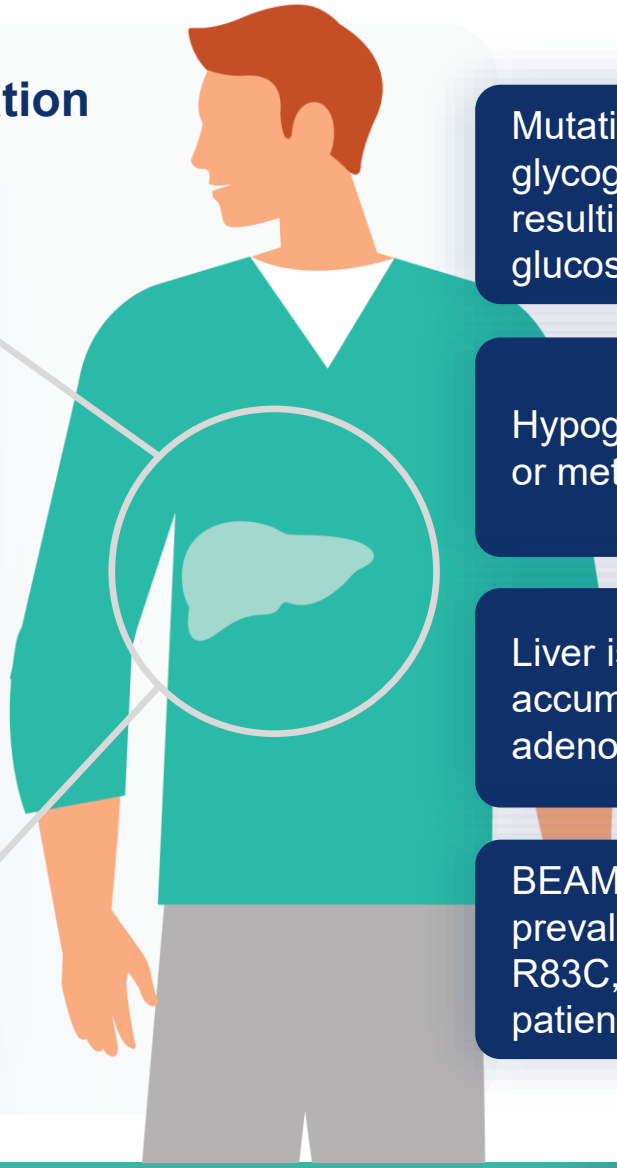
Glycogen storage disease Ia (GSDIa) patients are at risk of severe hypoglycemia and excessive accumulation of other metabolites

GSDIa can be caused by a single point mutation

G6PC R83C mutation

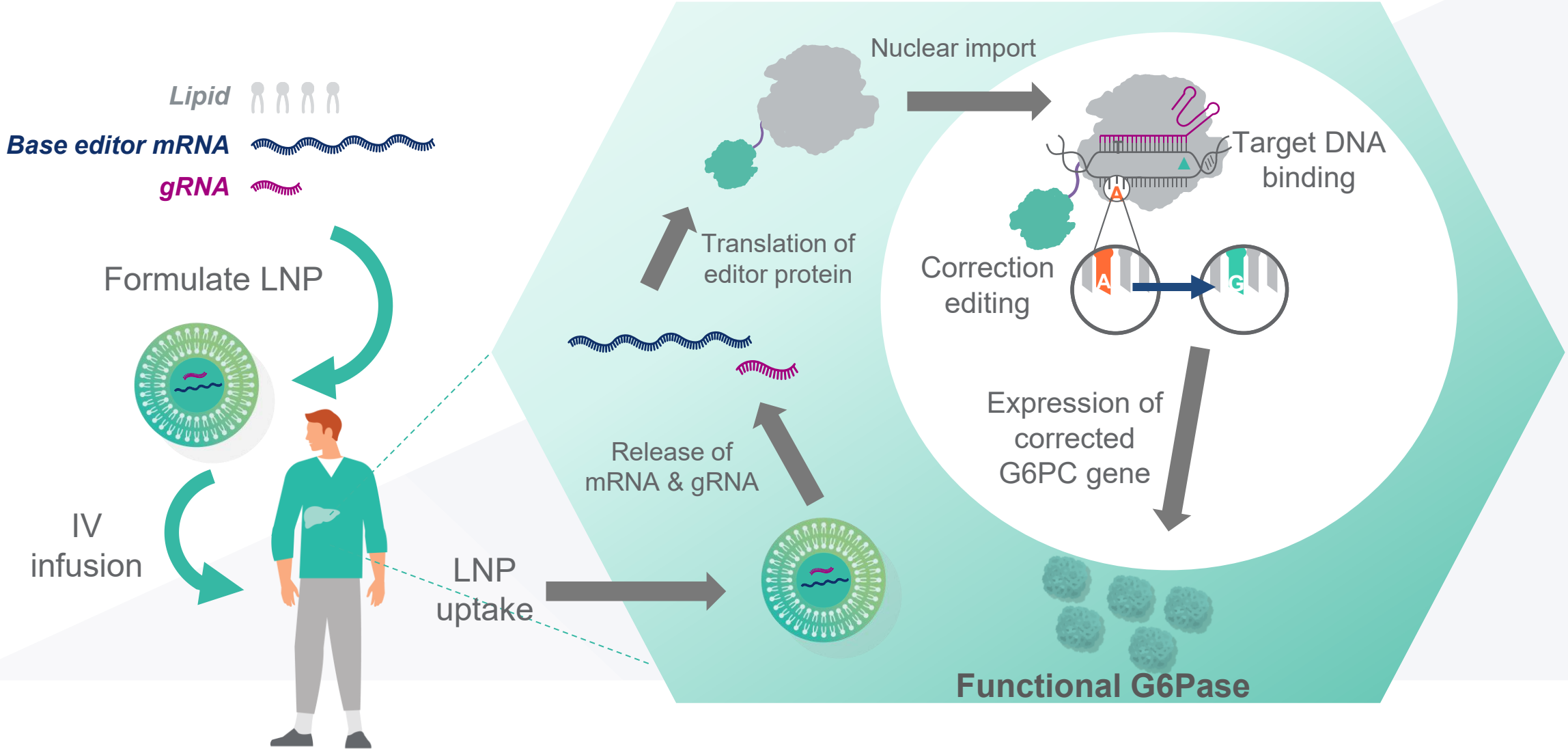
Wildtype G6PC gene



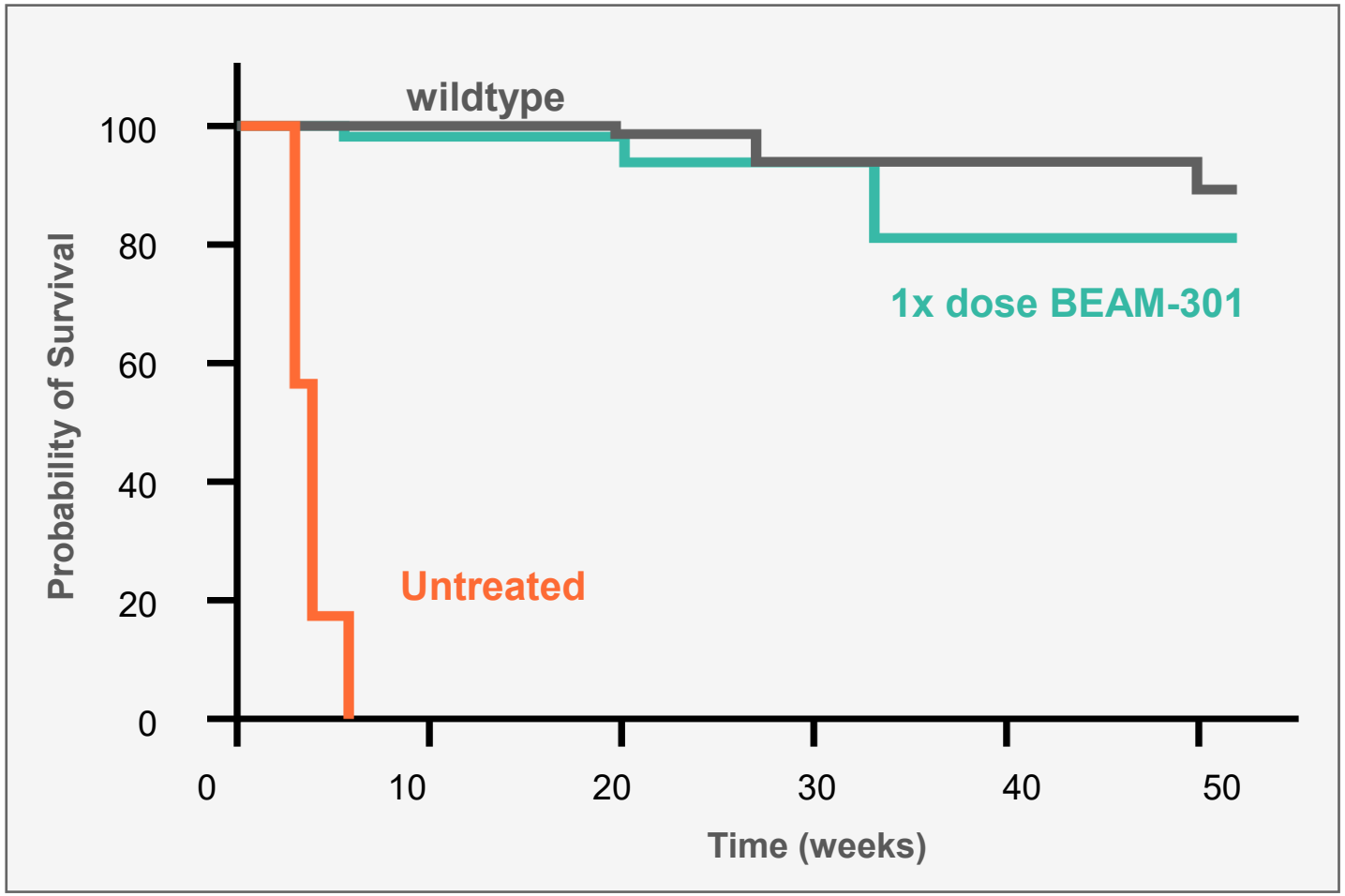


- Mutation of G6Pase prevents glycogen conversion to glucose, resulting in severe drop in blood glucose levels within 1–4 hrs with fast
- Hypoglycemia may result in seizures or metabolic crisis that can be lethal
- Liver is often enlarged due to accumulated glycogen and lipid and adenomas are common
- BEAM-301 targets one of the most prevalent and severe mutations, R83C, estimated to affect 300 patients in U.S.

BEAM-301 is designed to correct the R83C mutation causing GSDIa, utilizing non-viral, lipid nanoparticle (LNP) delivery to target the liver



In preclinical studies, treatment with a single dose of BEAM-301 significantly improved long-term survival in GSDIa mouse model

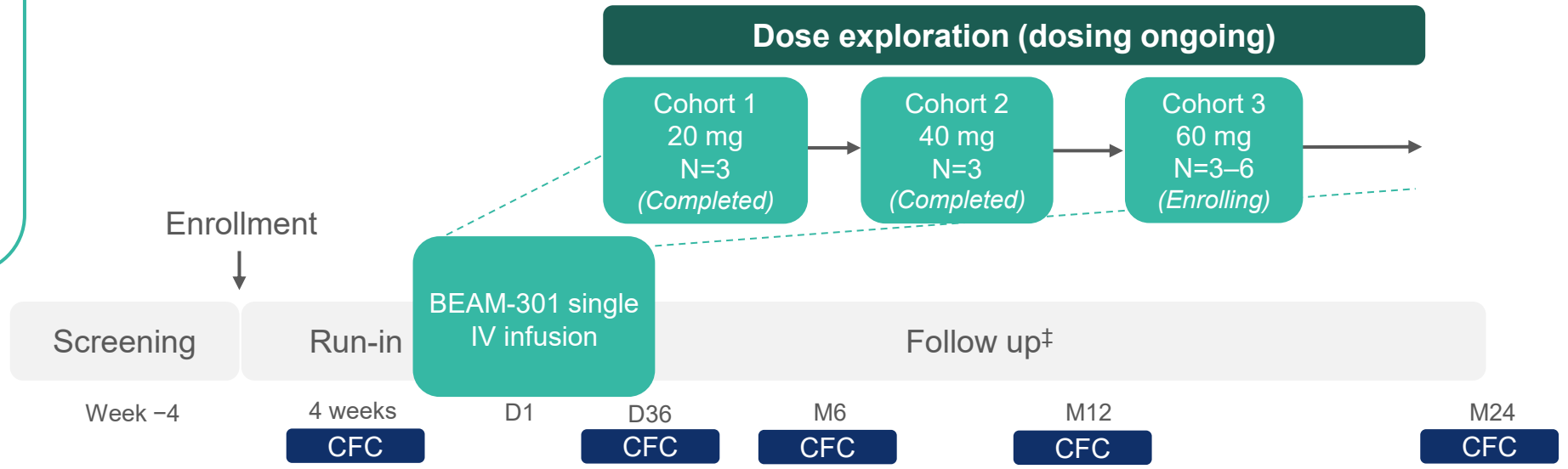


- Preclinical studies of BEAM-301 demonstrated a single dose **significantly improved long-term survival out to a year** in humanized R83C homozygous mice
- Untreated homozygous R83C mice die within weeks of birth

BEAM-301 Phase 1/2 study design

Patient population

Key inclusion criteria*
- Age ≥18 years
- GSDIa diagnosis (one or two R83C copies)
- ≥1 hypoglycemia episode <60 mg/dL† ≤2 years prior



Controlled fasting challenge (CFC) to establish time to fasting hypoglycemia (TTH):
- Stopping criteria include glucose <60 mg/dL†§
- Maximum allowable duration is 15 hours

Key endpoints*

Primary:
TEAEs, serious TEAEs, and DLTs

Secondary:
Change from BL in TTH; metabolic parameters over time; change from BL in starch supplementation‖

Exploratory:
Change from BL in % of time spent in euglycemia, hypoglycemia, and hyperglycemia (CGM); change from BL in glucose and metabolic parameters during CFCs

*List is not exhaustive; †3.3 mmol/L; ‡only follow-up visits during which patients are hospitalized for CFC are shown; §other criteria include lactate >10 mmol/L, pH <7.25, HCO_3 <15 mmol/L, altered mental status, vomiting, loss of consciousness, OR patient requests to stop; ‖total amount and number of doses per day

BL, baseline; CGM, continuous glucose monitoring; D, Day; DLT, dose-limiting toxicity; GSDIa, glycogen storage disease type Ia; IV, intravenous; M, Month; TEAE, treatment-emergent adverse event; TTH, time to fasting hypoglycemia

Baseline demographics and clinical characteristics

- As of data cutoff (August 17, 2026), six patients have received BEAM-301 with a median (range) follow-up of 7.6 (1.9–16.6) months

	Cohort 1: 20 mg (N=3)	Cohort 2: 40 mg (N=3)	Total (N=6)
Age (years), median (range)	26.0 (26.0–43.0)	42.0 (26.0–50.0)	34.0 (26.0–50.0)
Sex, n (%)			
Male	2 (66.7)	1 (33.3)	3 (50.0)
Female	1 (33.3)	2 (66.7)	3 (50.0)
BMI (kg/m^2), median (range)	28.1 (24.3–43.8)	24.6 (22.5–53.4)	26.4 (22.5–53.4)
Genotype, n (%)			
R83C homozygous	2 (66.7)	2 (66.7)	4 (66.7)
R83C compound heterozygous*	1 (33.3)	1 (33.3)	2 (33.3)
TTH (hours), median (range)	2.9 (1.1–3.0)	2.5 (2.3–3.1)	2.7 (1.1–3.1)
Daily starch intake, median (range)			
Total dose (g/day)	315 (248.0–330.0)	255 (236.0–275.0)	265 (236.0–330.0)
Number of doses	6 (6–7)	5 (5–6)	6 (5–7)
Triglyceride (mg/dL), median (range)	260 (130.7–289.7)	237 (202.7–296.0)	248.5 (130.7–296.0)

*All patients had the p.R83C and p.G270V mutations
BMI, body mass index; TTH, time to fasting hypoglycemia

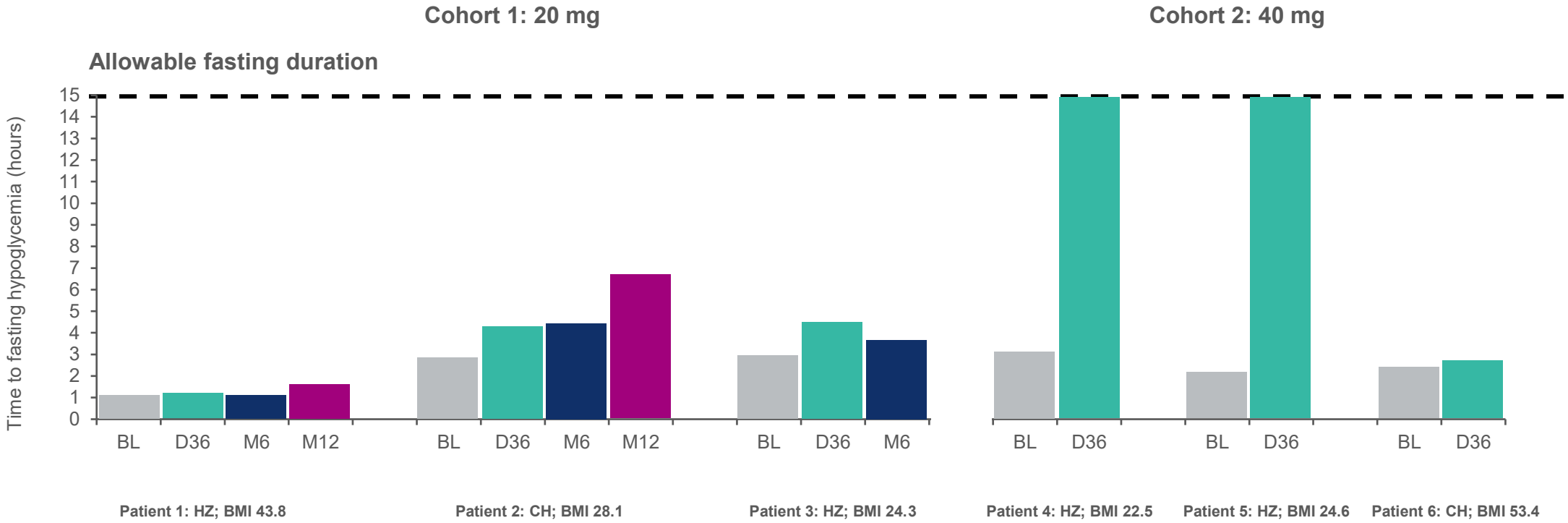
Safety profile of BEAM-301 consistent with LNP therapies

Number of patients with events (%)	Cohort 1: 20 mg (N=3)	Cohort 2: 40 mg (N=3)	Total (N=6)
Any TEAE	3 (100)	3 (100)	6 (100)
Related to BEAM-301	3 (100)	3 (100)	6 (100)
Any TEAE ≥Grade 3*	2 (66.7)	1 (33.3)	3 (50.0)
Related to BEAM-301	2 (66.7)	1 (33.3)	3 (50.0)
Any serious TEAE†	2 (66.7)	2 (66.7)	4 (66.7)
IRR	2 (66.7)	1 (33.3)	3 (50.0)
Diarrhea	0	1 (33.3)	1 (33.3)
Rhabdomyolysis‡	0	1 (33.3)	1 (33.3)
Serious TEAE related to BEAM-301	2 (66.7)	2 (66.7)	4 (66.7)
Any DLT	0	0	0
Any TEAE leading to death	0	0	0
Any TEAE leading to dose withdrawal	0	0	0
IRR Grade 1 / 2 / 3	1 (33.3) / 1 (33.3) / 2 (66.7)	0 / 1 (33.3) / 1 (33.3)	1 (16.7) / 2 (33.3) / 3 (50.0)
ALT elevation Grade 1 / 2 / 3	2 (66.7) / 0 / 0	2 (66.7) / 0 / 0	4 (66.7) / 0 / 0
AST elevation Grade 1 / 2 / 3	2 (66.7) / 0 / 0	1 (33.3) / 1 (33.3) / 0	3 (50.0) / 1 (16.7) / 0

- BEAM-301 20 mg and 40 mg showed an **acceptable safety profile**, with **no dose-limiting toxicities**, no TEAE-related discontinuations, and **no Grade 4/5 TEAEs**
- IRRs and transient transaminase elevations were the most frequent treatment-related TEAEs, consistent with the known safety profile of IV LNPs
  - IRRs occurred in 5/6 patients, comprising three serious Grade 3 events and three that were nonserious (two Grade 2, one Grade 1); all events resolved with or without treatment
  - Use of shorter-acting, lower-potency corticosteroids may have contributed to IRR severity; premedication and infusion rate regimens have been modified
  - Transaminase elevations were primarily Grade 1, transient, and resolved without intervention; patients remained asymptomatic and total bilirubin remained within normal limits

*No Grade 4 or 5 TEAEs were reported; †all serious TEAEs have resolved; ‡occurred in a patient who also had an IRR, with bilateral calf involvement, peak creatine kinase <1,000 U/L, and no renal involvement. ALT, alanine aminotransferase; AST, aspartate aminotransferase; DLT, dose-limiting toxicity; IRR, infusion-related reaction; IV, intravenous; TEAE, treatment-emergent adverse event

In the 40 mg cohort, 2 out of 3 patients demonstrated restoration of glucose production during the controlled fasting challenge (CFC)



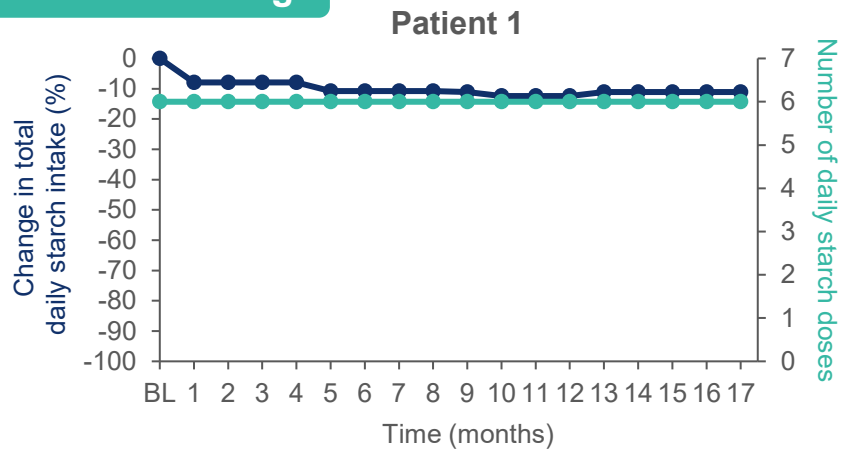
- Median (range) time to hypoglycemia (TTH) increased from 2.9 (1.1–3.0) hours at baseline to 4.3 (1.3–4.6) hours at Day 36 in the 20 mg cohort, and from 2.5 (2.3–3.1) hours to 15.0 (2.8–15.0) hours in the 40 mg cohort
- In the 40 mg cohort, **two of three patients achieved the protocol-defined maximum CFC duration (TTH) of 15 hours**, consistent with endogenous glucose production

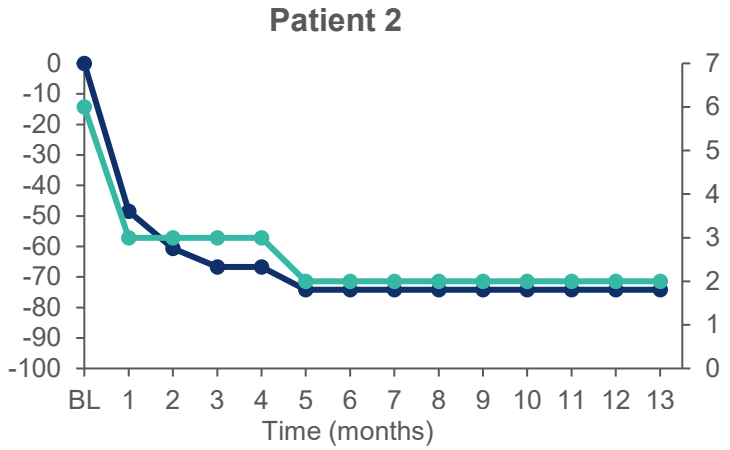
BMI, body mass index (kg/m^2); CFC, controlled fasting challenge; CH, compound heterozygous; D, day; HZ, homozygous; M, month; TTH, time to hypoglycemia

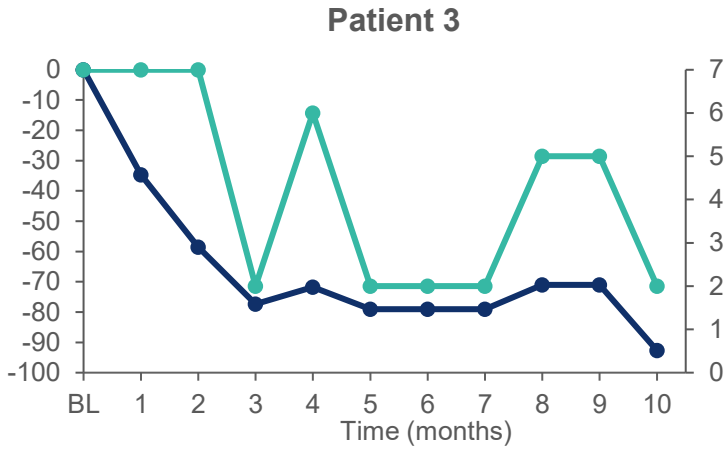
Daily starch requirements post-BEAM-301 generally decreased and were eliminated in two patients

Change in total daily starch intake (%)
Number of daily starch doses

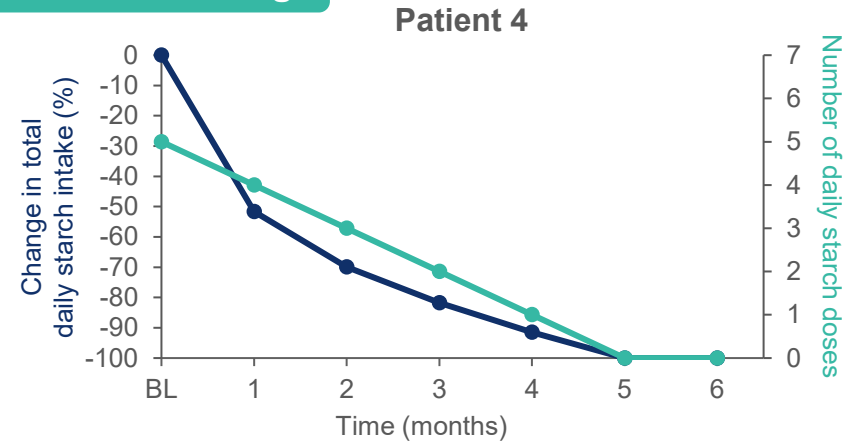
Cohort 1: 20 mg

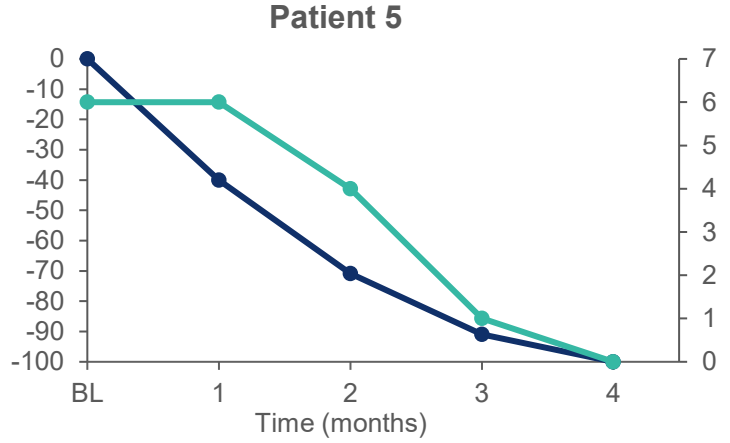


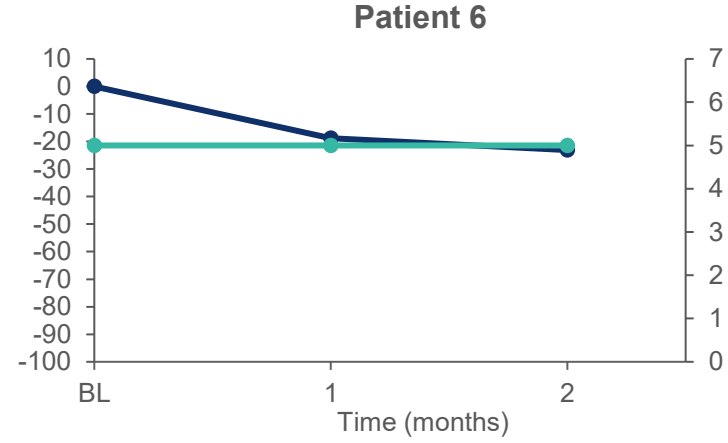




Cohort 2: 40 mg







- Two of three patients in the 40 mg cohort **no longer required any starch supplementation** and **maintained glucose within the normal range**

BL, baseline

Conclusions

Initial data from the BEAM-301 study demonstrate an acceptable safety profile and suggest induction of G6Pase activity in patients with GSDIa:

- Safety profile across 20 mg and 40 mg doses that is consistent with known risks associated with IV LNP administration
- Evidence of significant endogenous glucose production in GSDIa after single-dose administration
- Two of three patients in the 40 mg cohort reached the 15-hour fasting maximum, accompanied by a 100% reduction in daily starch intake (i.e., freedom from any starch supplementation)
- No overall increase in time spent in hypoglycemia despite significantly reduced starch intake

Based on these results, patients are now being recruited into the 60 mg dose cohort

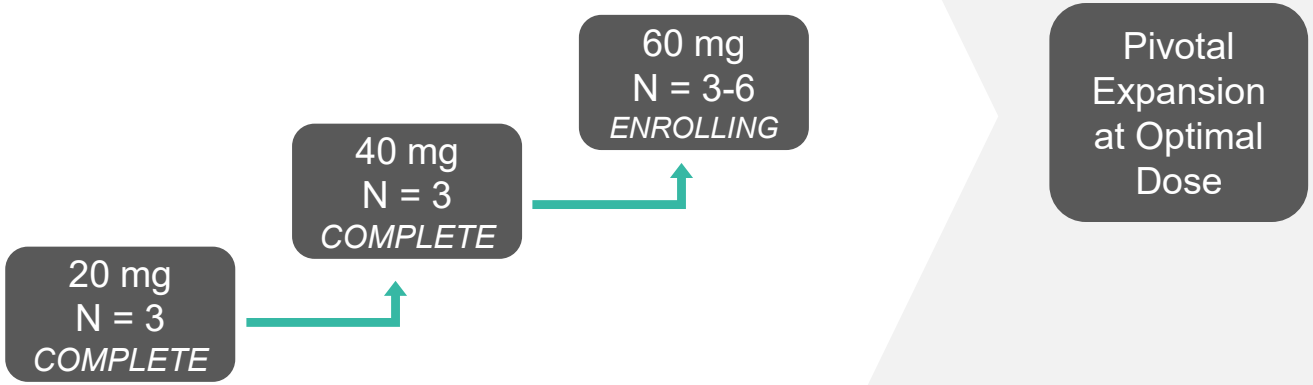
GSDIa, glycogen storage disease type Ia; IV, intravenous; LNP, lipid nanoparticle

BEAM-301 Phase 1/2 trial in GSDIa patients with R83C mutation continues to enroll and supports FDA dialogue for pivotal expansion

- Enrollment in 60 mg cohort underway
- Align with FDA on pathway to potential approval for BEAM-301

PHASE 1/2

Open-label, single-ascending dose trial



KEY ENDPOINTS

- Safety and tolerability
- Time to hypoglycemia during fasting
- Changes from baseline in starch supplementation

Beam's growing metabolic disease expertise creates opportunities for unique synergies in treating rare metabolic liver diseases

Translation

- Shared biological pathways across some indications
- Experience translating editing to enzymatic activity in humans based on animal model and *in vitro* data
- Low threshold for clinical benefit
- Readily available biomarkers

Clinic

- Multi-center registries, patient advocacy groups and metabolic treatment centers for patient education and referrals
- Efficiently test new products targeting different mutations under the same master clinical protocol and IND
- Experience in dietary management strategies that are fit-for-purpose

Commercial

- Strong connections in the metabolic disease community
- Focused commercial effort can support multiple metabolic indications
- Specialized metabolic disease treatment centers
- Leverage market access infrastructure in gene therapy

PKU: Beam's Platform Approach in Action

Giuseppe Ciaramella, Ph.D.
President

The power of predictability:
Beam is building a reliable model for advancing genetic medicine

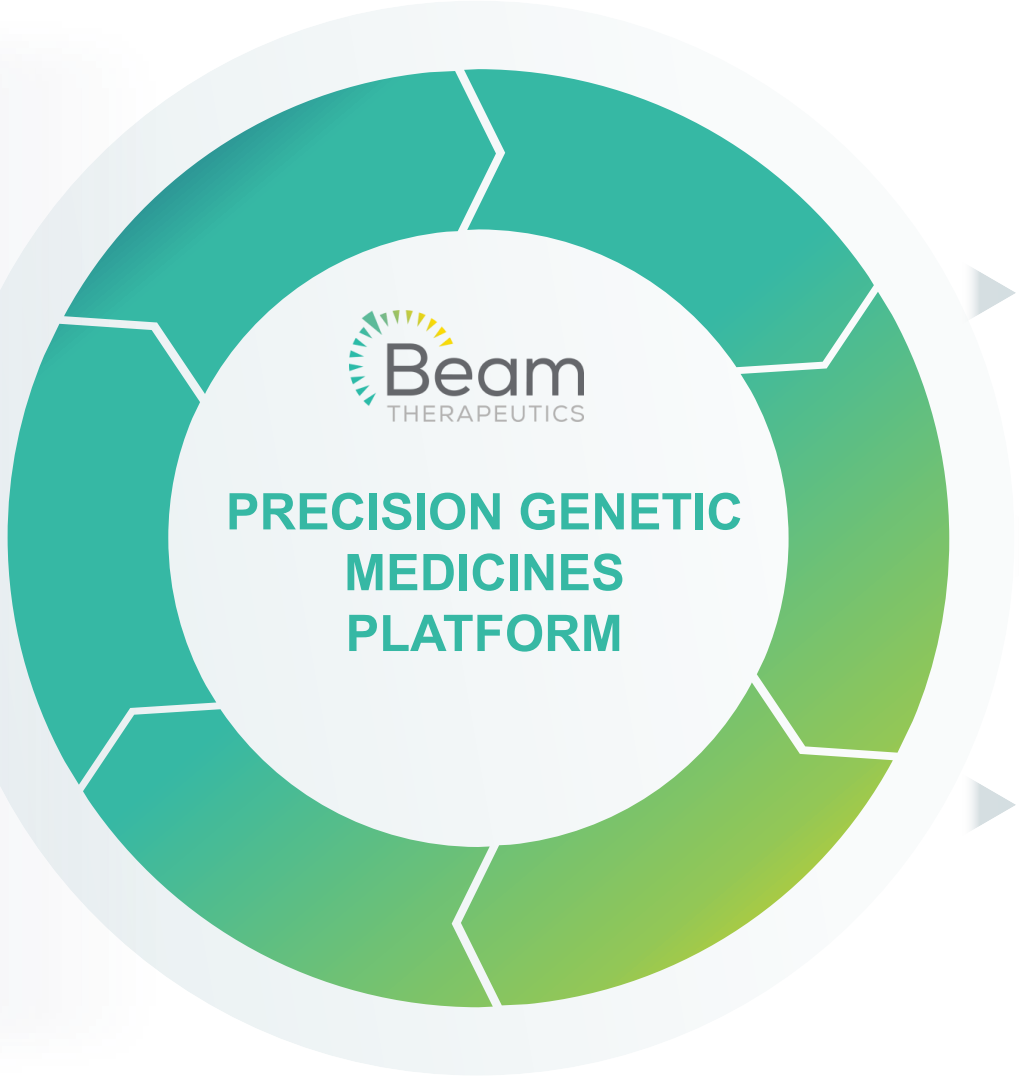
Easily **ADAPTABLE** to new targets

REUSABLE delivery technology

CONSISTENT preclinical and clinical outcomes

HIGHLY SCALABLE internal manufacturing

FLEXIBLE emerging regulatory framework



Multiple growing, high-value franchises

Active pipeline **expansion**

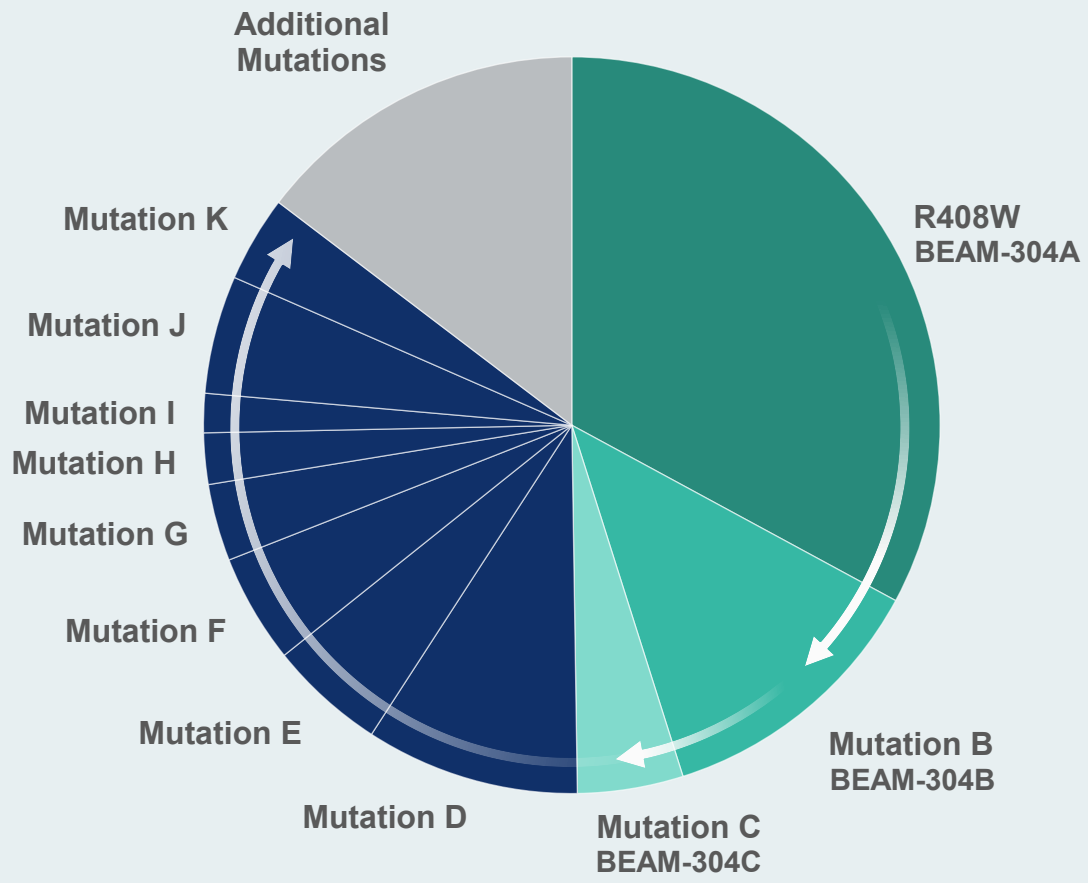
Creative platform **partnerships**

PKU is an example of the platform in action

Easily **ADAPTABLE** to new targets	**Targeting multiple mutations with same underlying platform**
REUSABLE delivery technology	**Builds on LNP capabilities validated by BEAM-302 and BEAM-301 clinical programs**
CONSISTENT preclinical and clinical outcomes	**Consistent preclinical outcomes across three PKU variants**
HIGHLY SCALABLE internal manufacturing	**Shared components between BEAM-302 and BEAM-304 manufacturing accelerate development**
FLEXIBLE emerging regulatory framework	**One IND for all PKU variants and one master clinical protocol for development**

BEAM-304 has potential to address vast majority of patients with PKU, leveraging an efficient development approach

~20,000 Individuals with PKU in U.S.



- Hundreds of mutations in phenylalanine hydroxylase (PAH) cause PKU
- Beam has potential to develop a first-in-class, one-time *in vivo* treatment for the vast majority of patients with PKU
- Multi-mutation approach has potential to utilize novel regulatory pathways
- BEAM-304 program advancing three base editors for distinct mutations to the clinic with potential to address up to 50% of U.S. patients with PKU
- Limited competition

Source: Beam internal data; Hillert, Alicia et al. The American Journal of Human Genetics, Volume 107, Issue 2, 234 – 250; Stone WL, Los E. Phenylketonuria (PKU). StatPearls Publishing; 2025 Jan

A platform approach allows us to leverage nonclinical and clinical data across PKU product variants

PKU Platform Product

PKU patient with mutation A

Base Editor mRNA + Guide RNA

BEAM-304A

PKU patient with mutation B

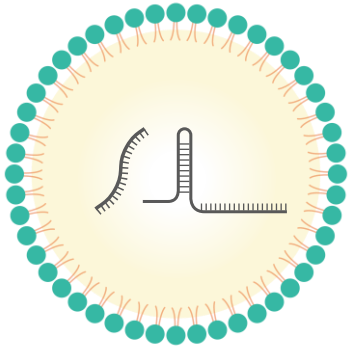
Base Editor mRNA + Guide RNA

BEAM-304B

PKU patient with mutation C

Base Editor mRNA + Guide RNA

BEAM-304C



Same LNP delivery

Patient-specific payloads
(A, B, C)

Same physicochemical characteristics

Same PK and biodistribution

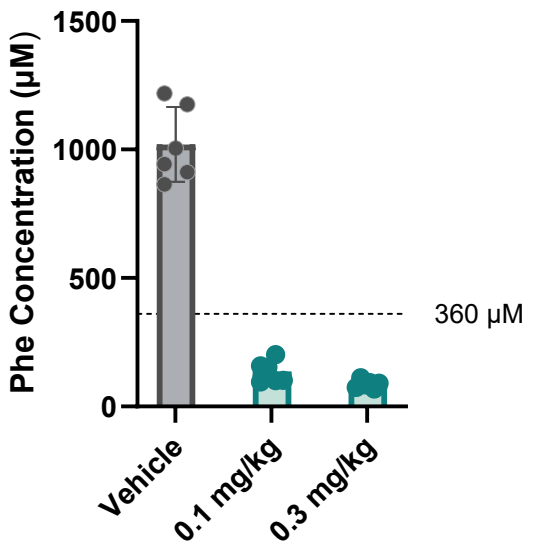
Same toxicity

Expectation of same safety and clinical efficacy

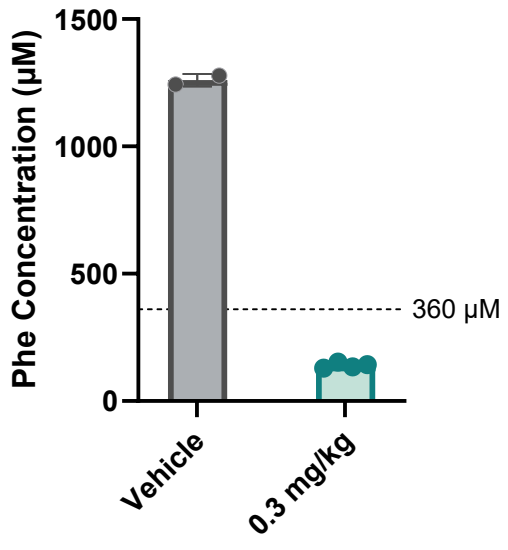
Plan to add each subsequent mutation to the same IND, clinical trial protocol, BLA

Potent correction of three different PAH mutations consistently normalized blood Phe in mouse models of PKU

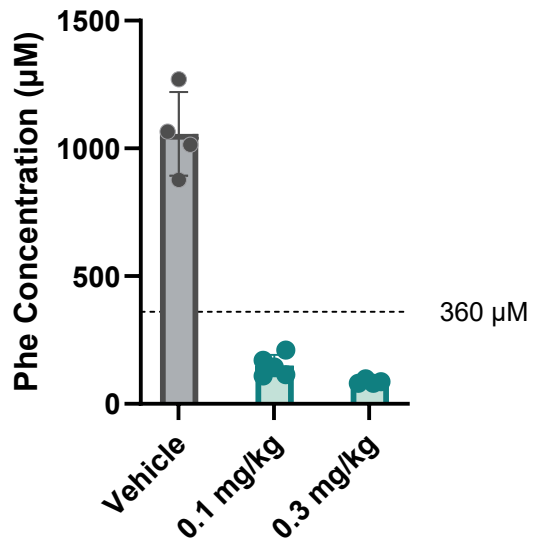
Mutation A



Mutation B



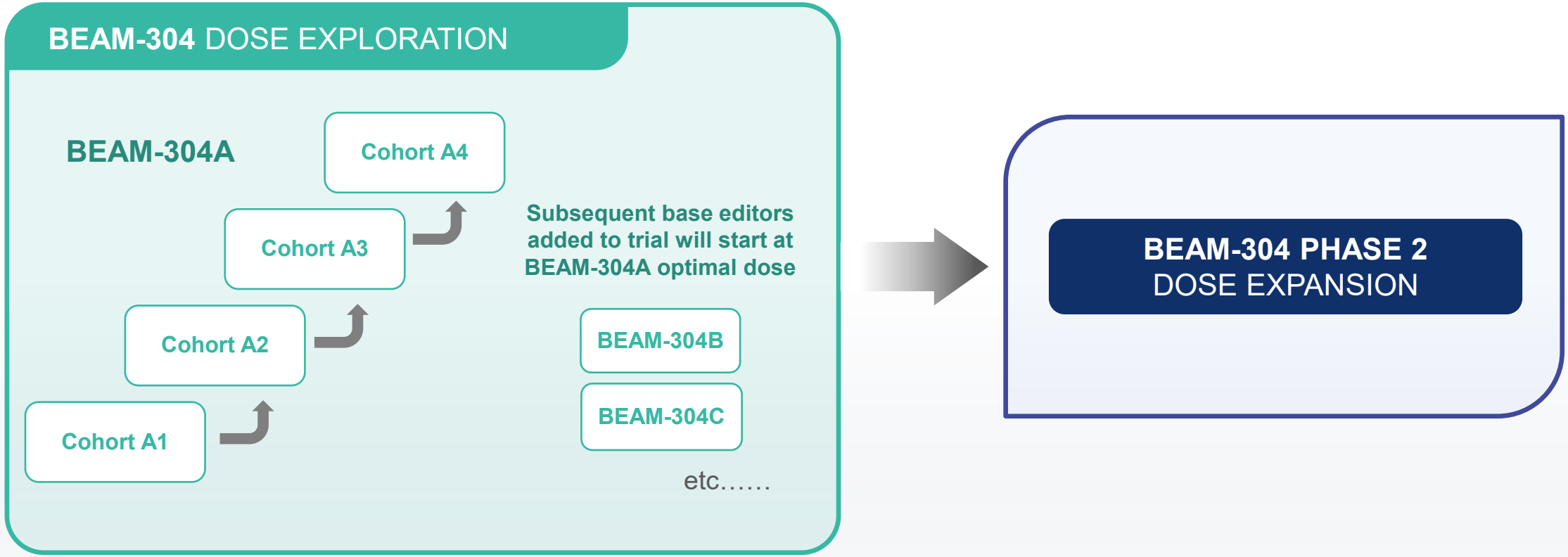
Mutation C



- BEAM-304 platform approach yielded predictable and reproducible results in preclinical models
- Preclinical results informed the selection of three base editors: BEAM-304A, BEAM-304B, and most recently, BEAM-304C, targeting a mutation affecting ~4% of individuals with PKU

Data shown are plasma Phe in respective mouse models 7 days after LNP administration

Phase 1/2 study designed to evaluate the safety and efficacy of all BEAM-304 editors under one IND and one master protocol



Key Inclusion Criteria:

1. Adults or adolescents **12 through 65 years old**
2. PAH gene with target **PKU-causing variant**
3. At least one historical Phe **≥600 µmol/L**
4. Screening Phe **≥360 µmol/L**

Key endpoints

- **Safety and tolerability**
- **Blood Phe reduction**
- **Diet liberalization**

Beam has the platform, programs and capital to become a leading genetic medicines company that ushers in a new era of medicine

Multiple Near-term Value Drivers

- Advance risto-cel toward BLA submission
- Execute BEAM-302 pivotal trial
- Generate proof of concept for additional *in vivo* programs

A Platform That Learns and Repeats

- Apply shared editing, delivery and manufacturing capabilities to new and existing clinical programs
- Derisked editing and delivery across >100 patients and 4 clinical programs
- Expand into new targets and delivery contexts

Financial Capacity to Execute

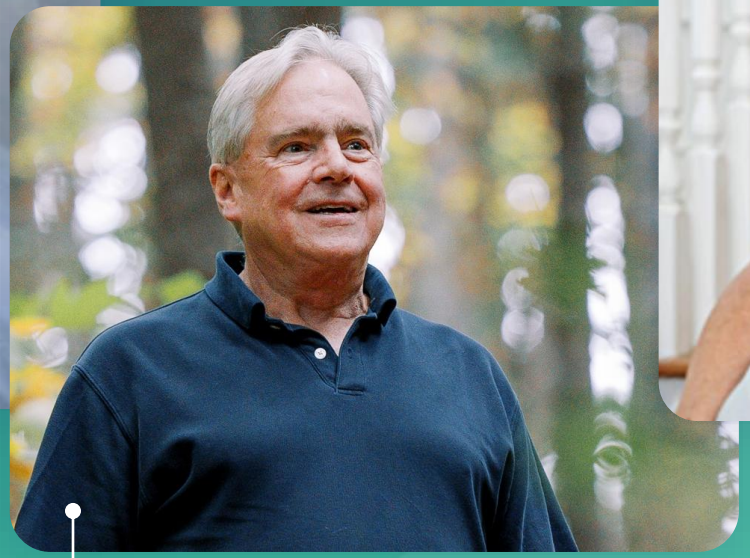
- $1.2B in cash as of June 30, 2026*
- Sixth Street facility structured to fund potential risto-cel launch
- Expected runway into mid-2029 under current assumptions**

BEAM IS BUILDING THE LEADING GENETIC MEDICINES COMPANY

*Inclusive of cash, cash equivalents, and marketable securities.
**Inclusive of potential additional $200 million expected to be drawn from Sixth Street facility.

THANK YOU

Kyle
LIVING WITH
SICKLE CELL DISEASE



Dan
LIVING WITH
ALPHA-1 ANTITRYPSIN DEFICIENCY

Alyssa and Gayle
LIVING WITH
GLYCOGEN STORAGE DISEASE TYPE IA