

# The Power of Predictability

May 2026

NASDAQ: BEAM



# 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 SCD, AATD, GSDIa, PKU and beta-thalassemia; our plans, and anticipated timing, to advance our programs, including the clinical trial designs and expectations for 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](http://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.

# Our vision is to provide lifelong cures for patients suffering from serious diseases

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**GENE EDITING FOR**  
rare and common  
diseases



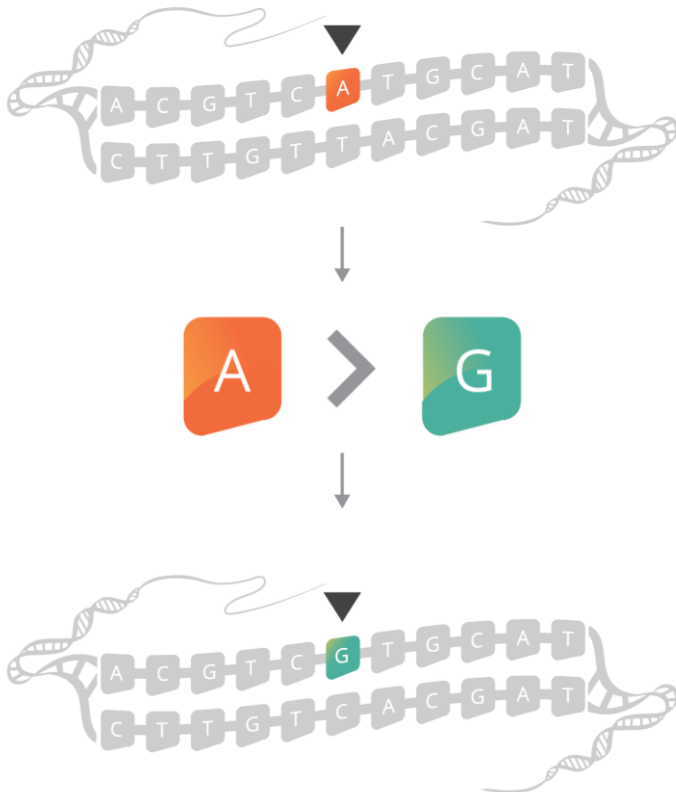
**POTENTIAL FOR**  
one-time, curative  
therapies



**PLATFORM FOR**  
rapidly programmable  
precision medicines

# Beam was founded based on a simple concept with profound implications

## BASE EDITING TECHNOLOGY



**CONSISTENT** gene  
sequence outcomes

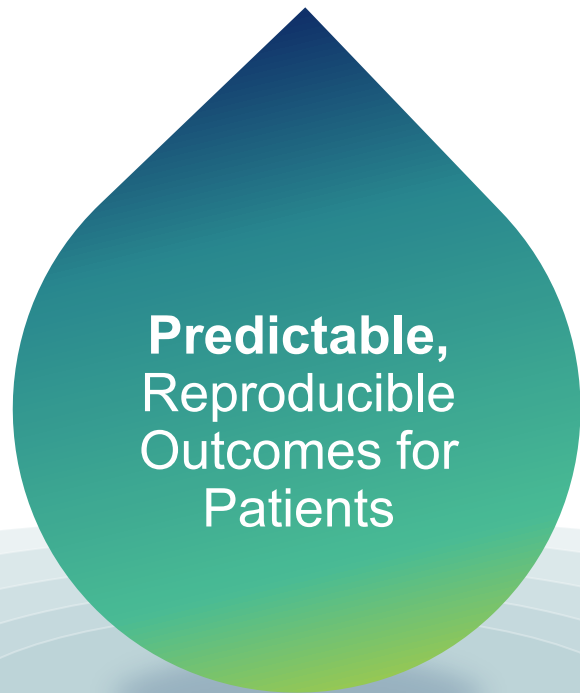
**DURABLE** correction  
for one-time cures

**LESS GENOTOXICITY** than  
traditional gene editing



**Predictable,  
Reproducible  
Outcomes for  
Patients**

# Predictability as a driver of progress: The potential to ripple through the broader healthcare ecosystem



## Streamlined R&D Cycles

- Reduced development risk

## Regulatory Acceleration

- Efficient regulatory pathways

## Physician Confidence

- Predictable safety and durability
- Clarity in treatment decisions

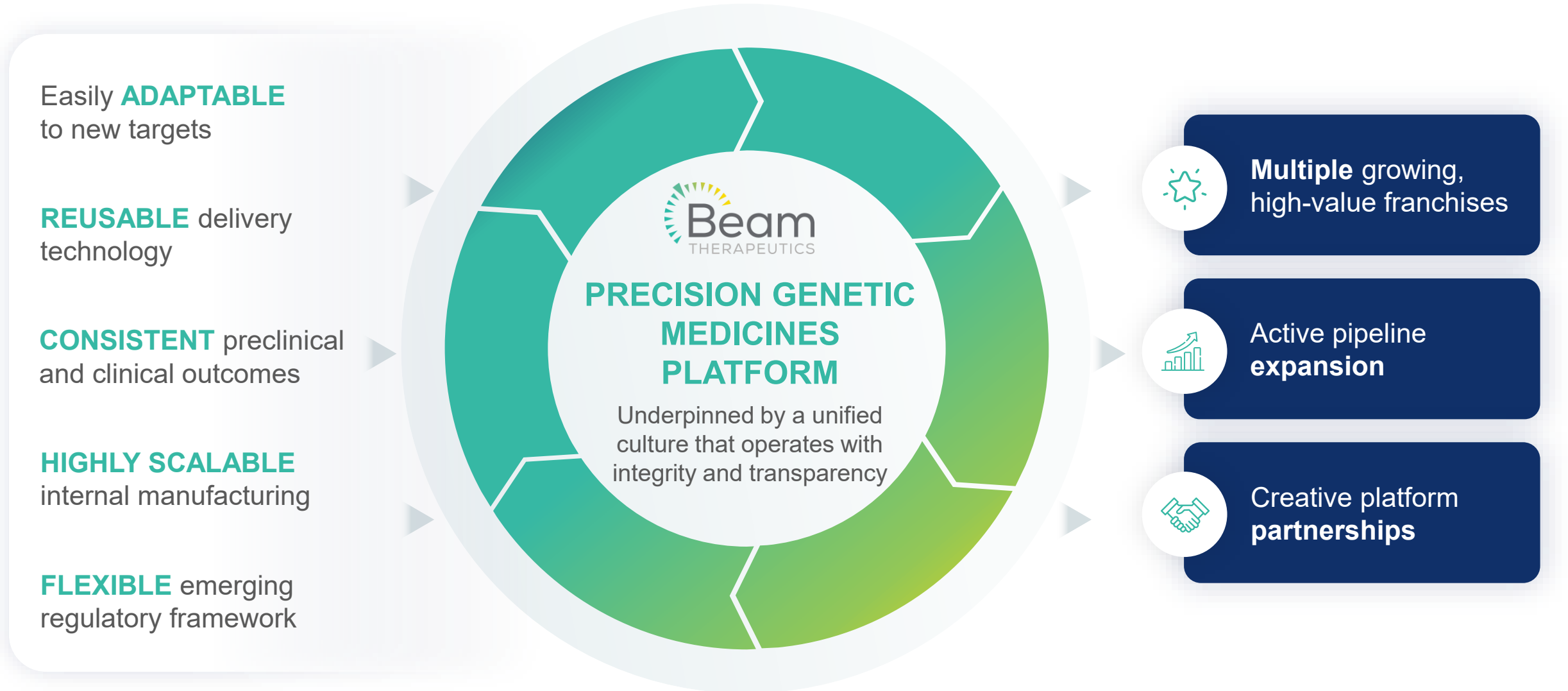
## Patient Experience Improvement

- Reliable therapeutic outcomes
- Enhanced quality of daily living

## Payer & System Impact

- Sustainable, outcomes-aligned payer models
- Reduced lifetime healthcare utilization

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



# Establishing a foundation of financial strength for sustainable growth

## Financial Strength

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- With \$1.2 billion in cash as of March 31, 2026\* and the anticipated \$200 million minimum drawdown from Sixth Street facility, expected operating runway now into mid-2029
- Anticipate funding through risto-cel launch, execution of BEAM-302 pivotal development plan, and clinical POC for BEAM-304 in PKU

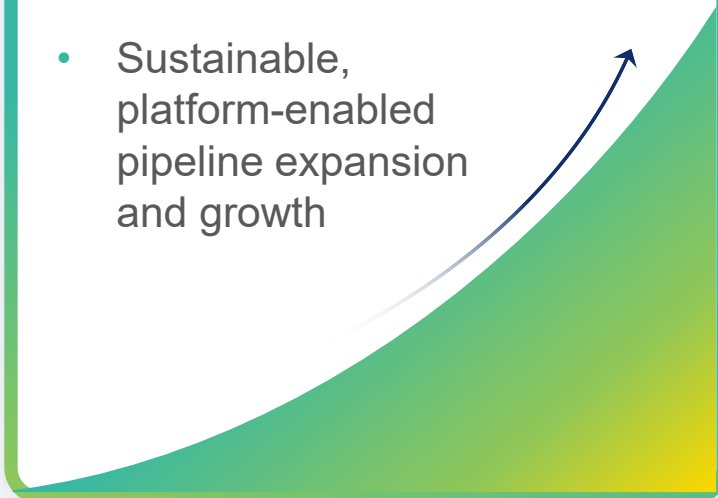
## Focused Investment

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- Optimized spend and expense management strategy in place
- Efficient commercial build for potential risto-cel launch
- Potential accelerated pathway for BEAM-302 development
- Efficient, novel development path for BEAM-304 in PKU

## Clear Path to Value and Sustainable Growth

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- Wholly owned pipeline
  - Significant addressable markets
  - Sustainable, platform-enabled pipeline expansion and growth
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# Liver-targeted Genetic Disease Franchise

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# Rapidly advancing and growing a portfolio of liver-targeted *in vivo* programs for genetic diseases



Potential **best-in-class** and **first-in-class** AATD program

Strategic **pipeline expansion** into PKU

**Industry-leading** LNP capabilities

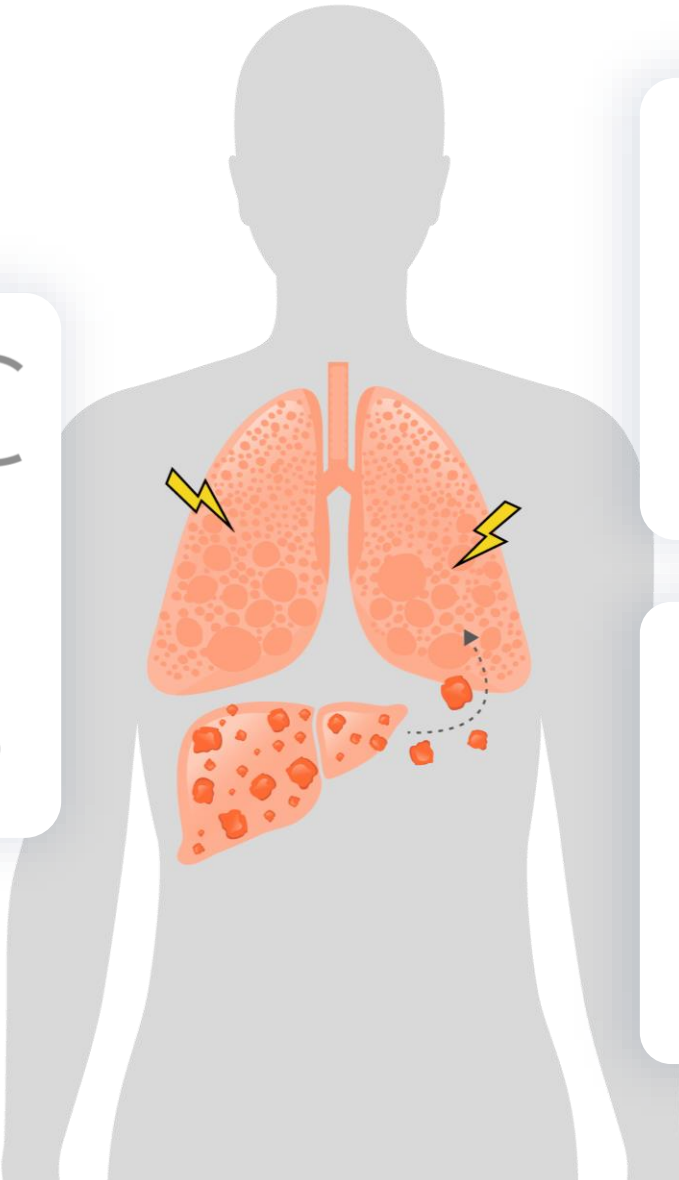
Platform synergies well positioned for **novel regulatory pathways**

PROGRAM	DISEASE	DELIVERY	EDITING APPROACH	LEAD			PIVOTAL
				RESEARCH	OPTIMIZATION	IND ENABLING	
<b>BEAM-302</b>	Alpha-1 antitrypsin deficiency (AATD)	<i>In vivo</i> LNP	Correction of E342K mutation	[Progress bar]			
<b>BEAM-301</b>	Glycogen storage disease type Ia (GSDIa)	<i>In vivo</i> LNP	Correction of R83C mutation	[Progress bar]			
<b>BEAM-304</b>	Phenylketonuria (PKU)	<i>In vivo</i> LNP	Correction of multiple mutations	[Progress bar]			

# Severe AATD (PiZZ genotype) impacts >100,000 individuals in the U.S. with limited treatment options



Single G to A point mutation in the SERPINA1 gene (PiZ or “Z” mutation)



## Progressive lung disease due to:

- Low and poorly functioning systemic Z-AAT levels
- Circulating Z-AAT aggregates, causes inflammation

- × Routine **COPD care**
- × IV augmentation therapy given **weekly is only approved option**

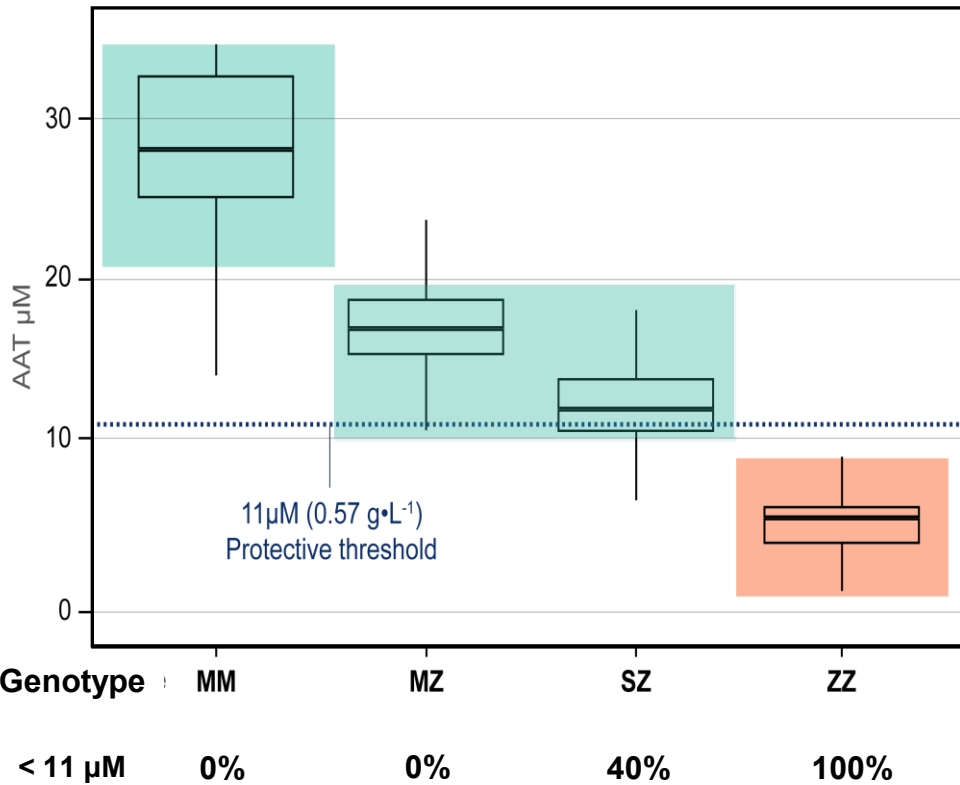
## Progressive liver disease with fibrosis and cirrhosis due to:

- Aggregation and accumulation of mutant Z-AAT

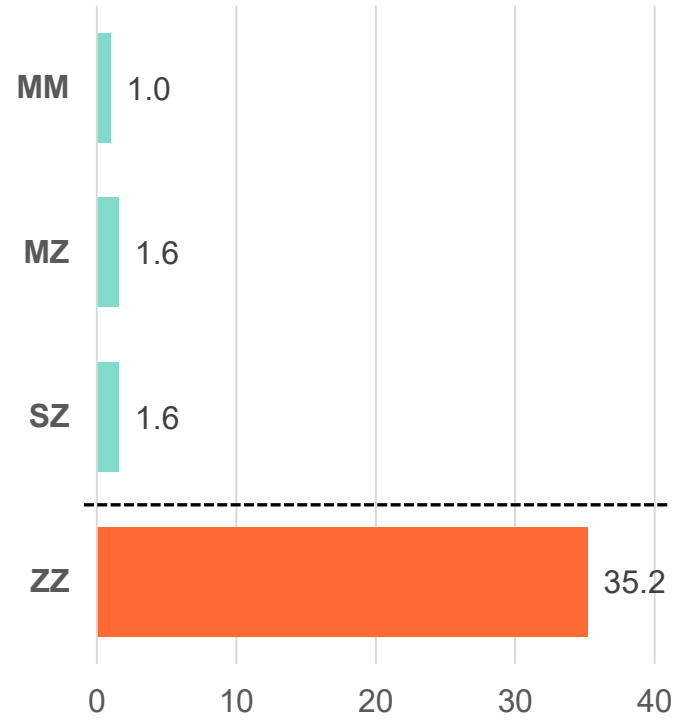
- × **Supportive care and liver transplant** for advanced disease
- × **No approved treatments** for liver disease

# AAT protective threshold >11 $\mu\text{M}$ informed by clinical genetics, translates into marked risk reduction for AATD lung and/or liver disease

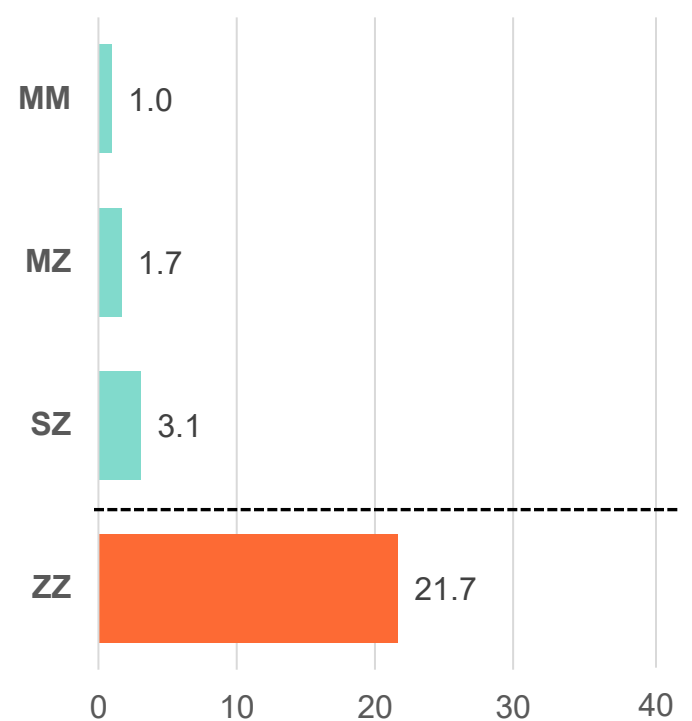
## AAT Levels Across Genotypes



## Emphysema Risk



## Liver Fibrosis / Cirrhosis Risk



Disease in MZ and SZ individuals requires presence of additional risk factor, e.g., smoking or obesity

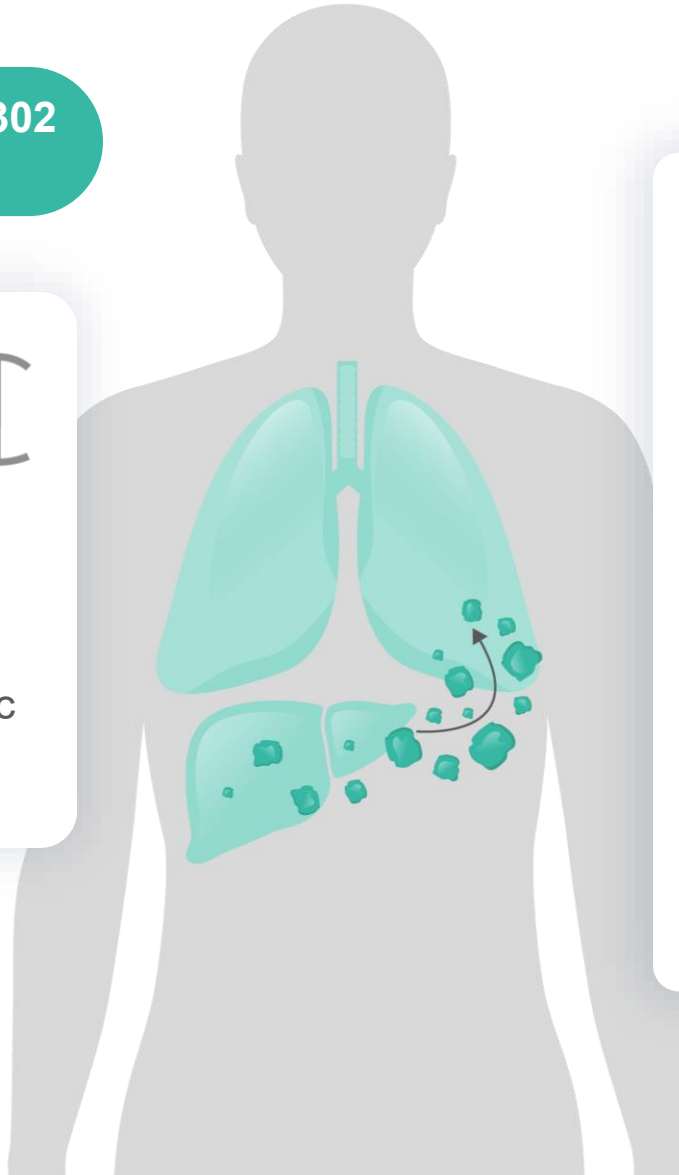
# BEAM-302 has the potential to be first, one-time treatment to address full spectrum of disease manifestations of AATD

## GOALS OF BEAM-302 TREATMENT



**Correction** at root cause of disease

**Restore** physiologic control of AAT



- **Liver produces M-AAT** for the first time
- Significantly **reduces Z-AAT**
- Total AAT **above 11  $\mu$ M protective threshold**
- Increased total **AAT is functional**
- AAT increases with **inflammatory response**

- ✓ **Durable, one-time** treatment
- ✓ **Address both lung and liver** manifestations

# Updated data from BEAM-302 Phase 1/2 trial support potential as best-in-class and first-in-class one-time treatment for AATD



Treatment with single dose 60 mg **BEAM-302** achieved durable levels of total AAT into the **MZ range and a more favorable M-AAT to Z-AAT ratio with follow-up out to 12 months**

- Steady-state mean **total AAT level was 16.1  $\mu\text{M}$ \***, with all patients consistently  $>11 \mu\text{M}$  protective threshold important for lung health
- **Mutant Z-AAT reduced 84%**, important for liver function and health
- Newly produced M-AAT was **functional and comprised 94% of total AAT**



Strong evidence of inducibility, with **a patient reaching  $\sim 30 \mu\text{M}$**  ( $\sim 95\%$  M-AAT) during respiratory infection



**Well-tolerated safety profile** with transient Grade 1 transaminase elevations in single-dose cohorts



Based on strength of safety and efficacy profile of BEAM-302 in single dose cohorts, **60 mg chosen as optimal biological dose for pivotal trial**

# Beam intends to pursue accelerated approval pathway for BEAM-302 based on FDA feedback to date

## Q1 2025

- ✓ Clinical proof of concept
- ✓ U.S. IND clearance

## Q2 2025

- ✓ RMAT Designation
- ✓ Orphan Drug Designation

## Q4 2025

### FDA alignment

- Alignment reached with FDA on a potential accelerated approval pathway for BEAM-302 in AATD
- Primary endpoint expected to be based on AAT biomarkers evaluated over 12 months
- Anticipate enrolling approximately 50 additional patients in expansion of open-label Phase 1/2 trial
- Ongoing dialogue with FDA on confirmatory trial design
- Accepted to FDA CMC Development and Readiness Pilot (CDRP) program

# Enrollment in 60 mg Part A and Part B cohorts to continue while operationalizing pivotal cohort for planned initiation in 2H 2026

## **PART A:** AATD-associated Lung Disease

### **DOSE ESCALATION & EXPANSION**

- Enrolling additional patients in 60 mg expansion
- Dosing started at U.S. sites

## **PART B:** AATD-associated Liver Disease with or without Lung Disease

### **DOSE ESCALATION**

- Continuing to enroll 60 mg cohort

## **PART C: PIVOTAL COHORT** AATD-associated Lung Disease with or without Liver Disease

- Plan to initiate pivotal cohort in second half of 2026
- Protocol amendment in process
- Plan to utilize existing extensive global site network: 12+ sites in 6 countries
- U.S. sites now activated

# Beam's long-term commitment to leading innovation for the AATD community

- Internal Beam lifecycle management through ongoing R&D efforts

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- Member of C-Path's Critical Path for AATD (CPA-1) consortium in collaboration with FDA to accelerate AATD research by identifying clinical efficacy endpoints

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- Collaboration with Alpha-1 Foundation and Alpha-1 Europe Alliance to educate about gene editing and obtain critical input on clinical trial design and patient experience



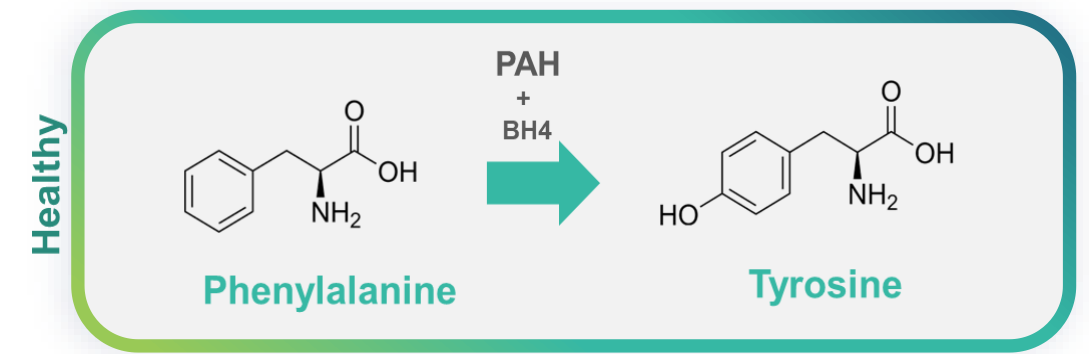
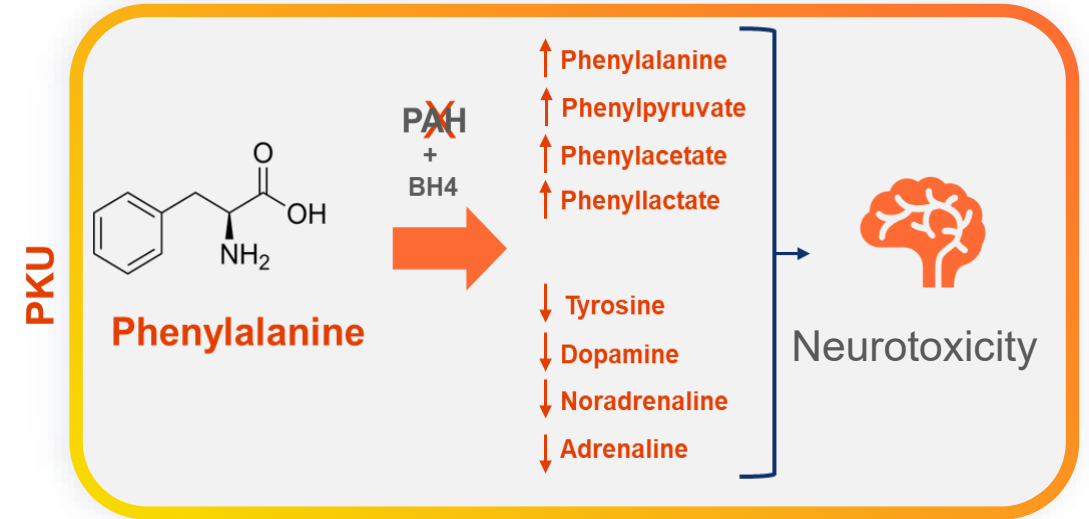
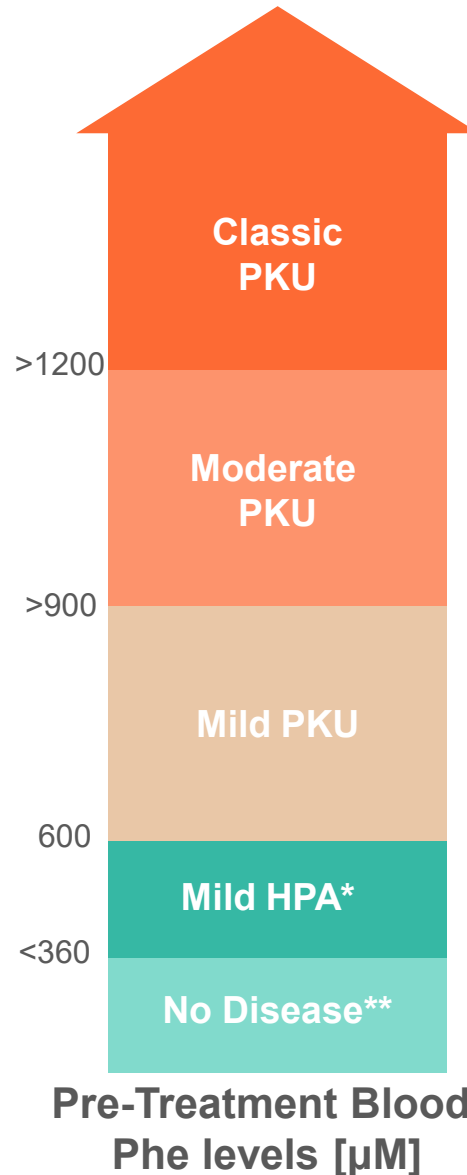
# PKU is a genetic metabolic disease in which failure to metabolize phenylalanine leads to neurotoxicity

PKU is caused by mutations in *PAH* gene, resulting in loss of PAH activity, failure to metabolize Phe and elevated Phe levels

U.S. PKU patients identified with widespread newborn screening

- Majority, but not all, patients are genotyped

Majority of patients have uncontrolled disease, above the recommended guidelines of blood Phe <360µM



\*HPA: hyperphenylalaninemia  
 \*\*Normal Phe <120; treatment target <360

# Target product profile for PKU gene therapy driven by established regulatory precedents, the literature and clinician feedback

- Blood Phe has been an accepted surrogate endpoint for patients with PKU for full approval in U.S. and EU
- Diet normalization can be demonstrated in clinical setting
- Phe reduction associated with positive trends in patient outcomes, including improvements in inattention and confusion, but has not been required for approval

## Target profile for successful clinical uptake of PKU gene therapy:



**Significant and sustained reduction in Phe levels** (<360 $\mu$ M)



**Well tolerated**



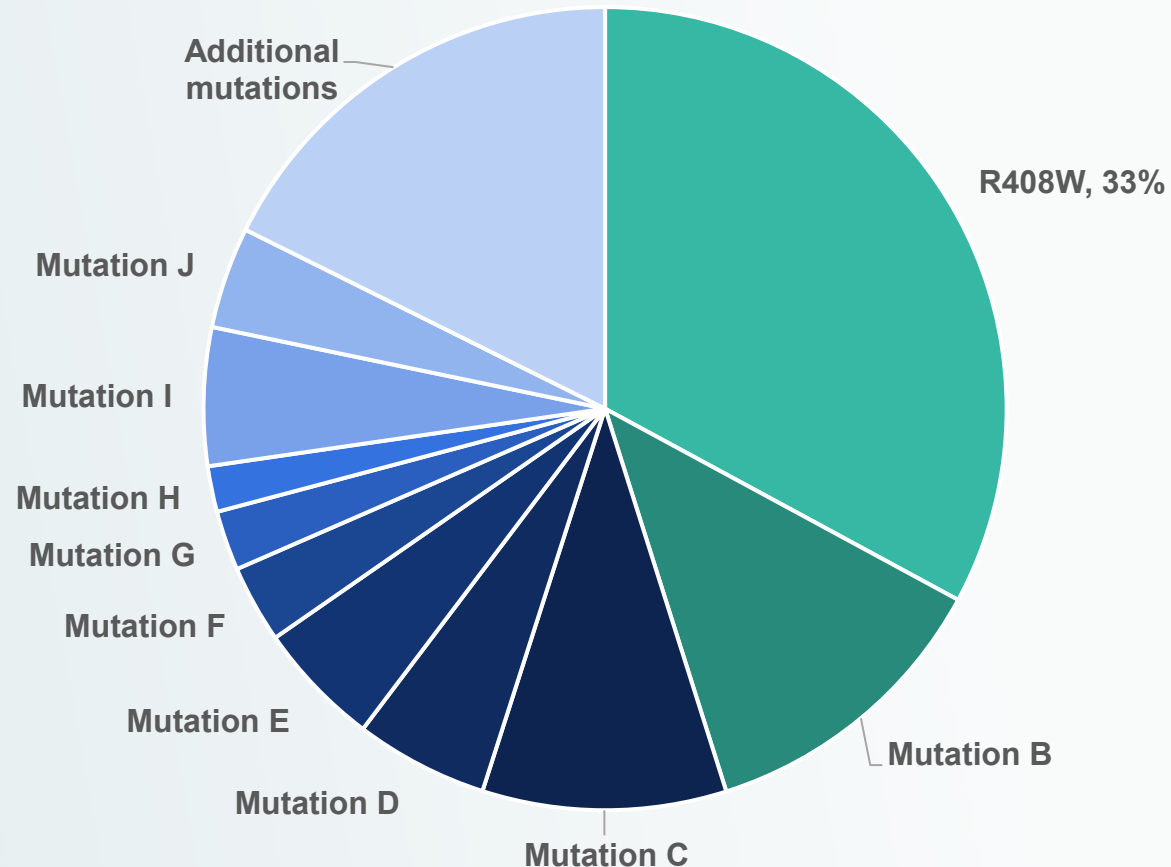
**Diet normalization**  
(no medical food or Phe restrictions)



**One-time therapy for lifelong Phe control**

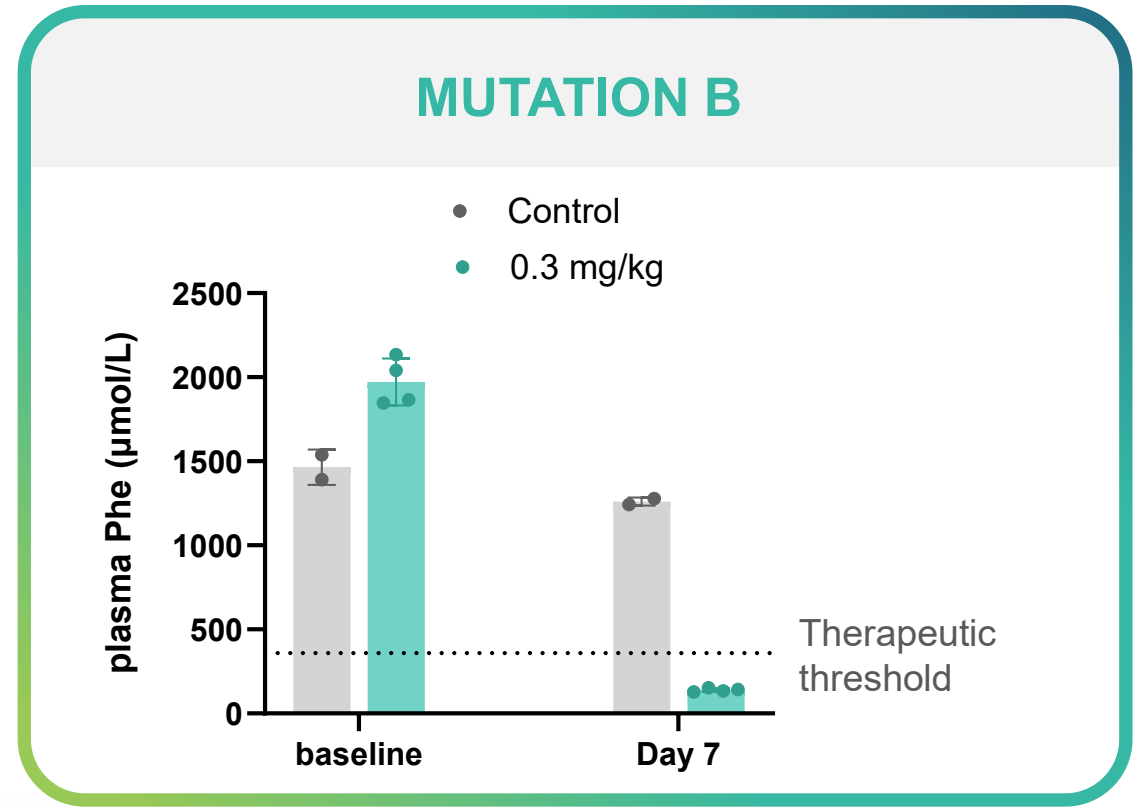
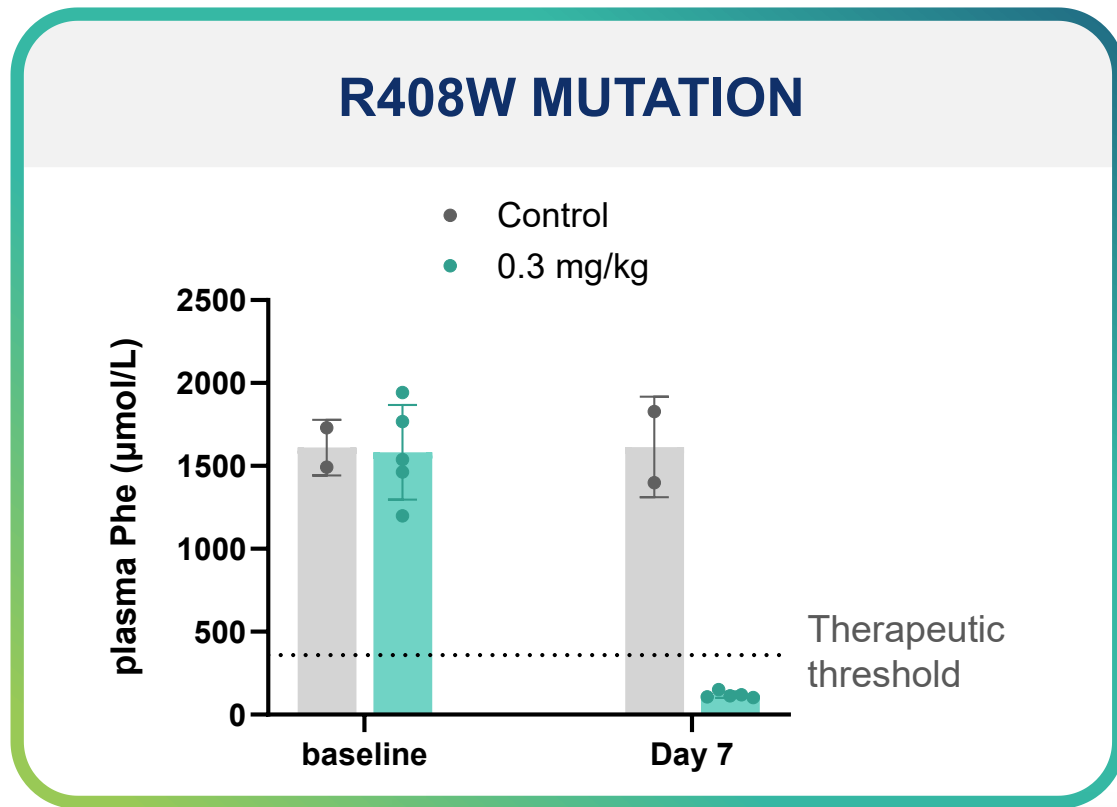
# Beam has potential to address vast majority of patients with PKU, leveraging an efficient development approach

~20,000 PKU Individuals in U.S.



- Two identified development candidates have potential to address nearly half of PKU patients
- Ongoing research efforts focused on correcting additional mutations
- Opportunity to leverage novel regulatory and clinical approach to efficiently address vast majority of PKU

# Preclinical data demonstrate the potential of BEAM-304 to correct underlying PKU mutations and rapidly normalize plasma Phe



**For both target mutations, BEAM-304 normalized plasma Phe in mice consuming standard protein-containing feed**

# BEAM-304 Phase 1/2 trial in PKU patients designed to achieve early clinical proof of concept



- Beam will initially focus development of BEAM-304 in U.S.
- Potential to rapidly expand to other mutations
- Productive pre-IND interaction with FDA

## PHASE 1/2

Open-label, single-ascending dose trial

**DOSE  
EXPLORATION**



**DOSE  
EXPANSION**

## KEY ENDPOINTS

- Safety and tolerability
- Reduction of blood Phe concentration

**Expect to file IND in 2026**

# BEAM-301 Phase 1/2 trial in GSD1a patients with R83C mutation designed to achieve early clinical proof of concept

## Glycogen Storage Disease Type 1a

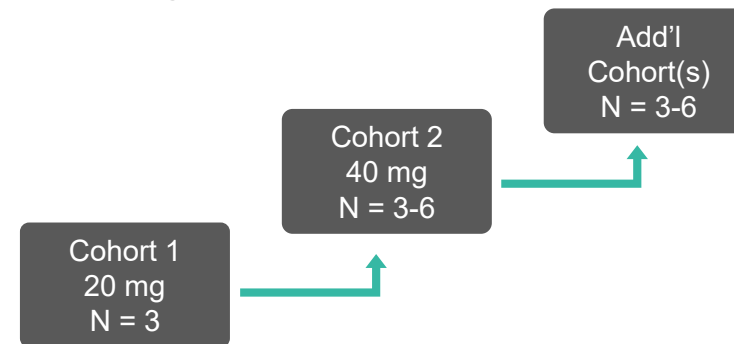
- Orphan disease
- Inability to convert glycogen back to glucose to sustain blood sugar while fasting
- Constant risk of hypoglycemia that can result in seizures, coma or death
- Standard of care is cornstarch taken every 2-4 hours, even overnight

## BEAM-301 Potential

- Correct liver G6PC mutation to restore enzyme activity and enable normal glucose metabolism
- Animal studies suggest ~11% enzyme activity sufficient for restoring metabolic profile
- Same LNP as BEAM-302

## 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

**Plan to report  
initial clinical  
data in 2026**

# Hematology Franchise

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# Multi-wave strategy in SCD focuses on near-term *ex vivo* commercial opportunity, followed by *in vivo* delivery for maximum scalability



Severe SCD gene therapy market (~10K patients) poised for growth with significant demand in U.S.

Clinical differentiation of *risto-cel* from base editing and manufacturing process

ESCAPE technology has potential broad utility

*In vivo* HSC editing progress, prioritized for next wave program

PROGRAM	DISEASE	DELIVERY	EDITING APPROACH	LEAD			PIVOTAL
				RESEARCH	OPTIMIZATION	IND ENABLING	
<b>Risto-cel</b>	Sickle cell disease (SCD)	<i>Ex vivo</i> HSC	Activation of fetal hemoglobin (HbF)	[Progress bar: Research, Optimization, Ind Enabling, Phase I/II]			
<b>BEAM-103</b>	SCD Beta-thalassemia	<i>Ex vivo</i> or <i>in vivo</i> HSC	CD117 edit-antibody (ESCAPE)	[Progress bar: Research, Optimization, Ind Enabling]			
<b><i>In vivo</i> HSC editing</b>	SCD Beta-thalassemia	<i>In vivo</i> LNP	Activation of HbF	[Progress bar: Research]			

# BEACON results provide evidence of potential differentiation and best-in-class profile of base editing and risto-cel for severe SCD

## Deeper Resolution of SCD



- HbF levels >60% and HbS levels <40%, comparable to sickle trait
- Resolution of anemia
- Markers of hemolysis and oxygen delivery normalized or improved

## Less Time in Hospital



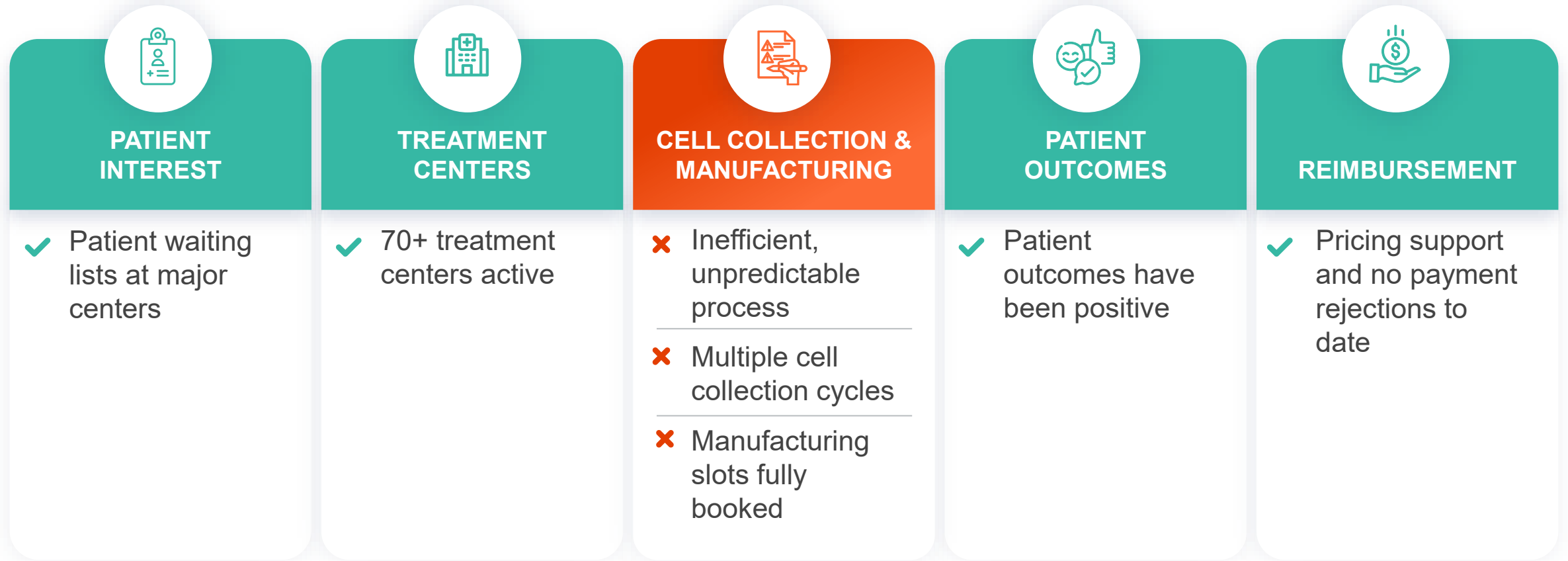
- Rapid neutrophil and platelet engraftment
- Low number of neutropenic days

## Predictable Manufacturing, Fast Patient Delivery



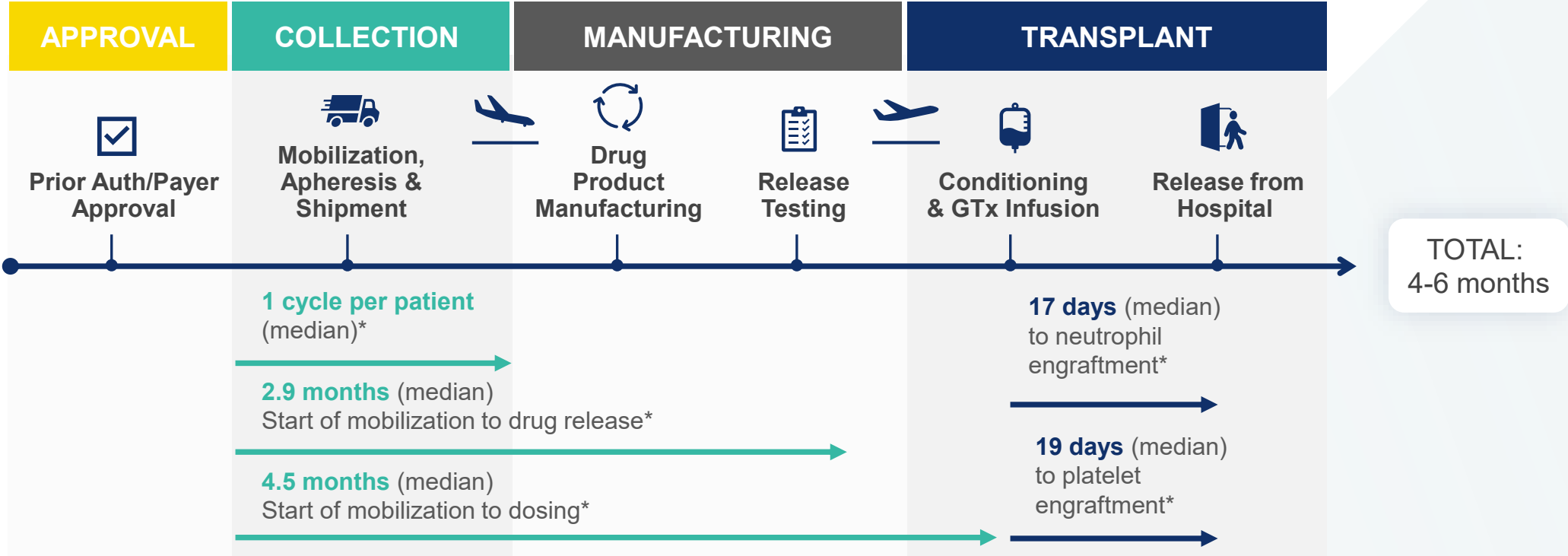
- Median of 1 cell collection cycle
- Consistently high yields and viability
- Median 2.9 months from start of mobilization to drug product release
- Beam NC facility allows flexible scheduling for patients and treatment centers

# Major bottlenecks for SCD gene therapy commercial adoption are limited manufacturing capacity and low process efficiency



“ We have patients knocking at the door every day. Current manufacturers cannot handle the volume of patients. ”  
– Southeast U.S. KOL

# End-to-end, efficient risto-cel process designed to reduce burden and optimizes experience for patients and treatment centers



“ With a more predictable and shorter process overall, we could treat more patients per year. ”

-Northeast KOL

- ✓ Internal manufacturing with optimized process and product release
- ✓ Predictable scheduling and consistent process outcomes
- ✓ Faster process can also expand market: higher throughput per center

\* BEACON trial data from ASH 2025

# Preparing for BLA submission and a potential best-in-class launch in a growing market

**Engaged with regulators in 2025**  
to provide clarity on BLA package

**Completed manufacturing of all doses**  
in BEACON trial

**Plan to submit risto-cel BLA package**  
as early as YE 2026

**Initiate efficient commercial build**  
to support potential risto-cel launch

# Expanding curative therapy for all patients with SCD will be enabled by improvements in delivery

## Potential Eligible SCD Patient Population



10%  
~10,000

Up to 100%  
~100,000

**Ex vivo HSC Transplant:**  
Potential best-in-class profile with risto-cel

### **In vivo HSC Editing:** Next wave in SCD

- Recent progress optimizing delivery to HSCs enabled by multiple breakthroughs in LNP technology
- Advancing *in vivo* approach to DC
- Potential to enhance *in vivo* editing with ESCAPE technology
  - Healthy volunteer study of BEAM-103, an anti-CD117 monoclonal antibody, ongoing
  - Maintain optionality to pursue *ex vivo* applications in SCD and other hematologic diseases

# Beam has assembled all capabilities needed to enable *in vivo* delivery of SCD gene editing

## LNP

- ✓ Potent, wholly-owned, highly biodegradable ionizable lipid and LNP process

## Extra-hepatic Targeting

- ✓ LNP modifications to de-target liver
- ✓ Targeting binders enable cell-specific delivery *in vivo*

## Payload

- ✓ Precise and efficient gene editing payloads

## ESCAPE

- ✓ Selectively suppresses diseased cells, enabling non-genotoxic therapy both *ex vivo* and *in vivo*



## *In vivo* LNP delivery to T cells

- ✓ NHP proof of concept
- ✓ Progressing to clinical studies
- ✓ Acquisition by BMS for \$1.5B



## *In vivo* LNP delivery to HSCs

- ✓ Multiple HSC-targeting LNPs identified
- ✓ Development scale-up ready
- ✓ Lead optimization ongoing

# Beam's innovative partnership strategy has consistently delivered value creation and therapeutic impact



Acquired  
by Lilly



Acquired  
by BMS



Opted-in to  
FcRn program



Opted-in to  
program



Partnerships have resulted in more than **\$900M non-dilutive funding to date** and more than **\$1B in potential milestones**

Gained rights to **innovative and complementary technologies**

Advanced Beam science toward patients in non-core areas

# Beam is well positioned to realize the power of predictability in 2026 through key anticipated milestones



## Pursue Path to Approval for Lead Programs

- Initiate pivotal cohort for BEAM-302 in 2H 2026
- Present detailed and updated BEAM-302 data at medical congress in 2026
- Plan to submit risto-cel BLA as early as YE 2026



## Advance and Expand Pipeline

- File IND for BEAM-304 in 2026
- Report initial BEAM-301 data by YE 2026
- Complete BEAM-103 healthy volunteer study in 1H 2026
- Advance *in vivo* HSC editing program



## Maintain Financial Strength

- \$1.2 billion in cash as of March 31, 2026\*
- Expected runway into mid-2029 through anticipated risto-cel launch, execution of BEAM-302 pivotal development plan and clinical proof of concept for BEAM-304\*\*

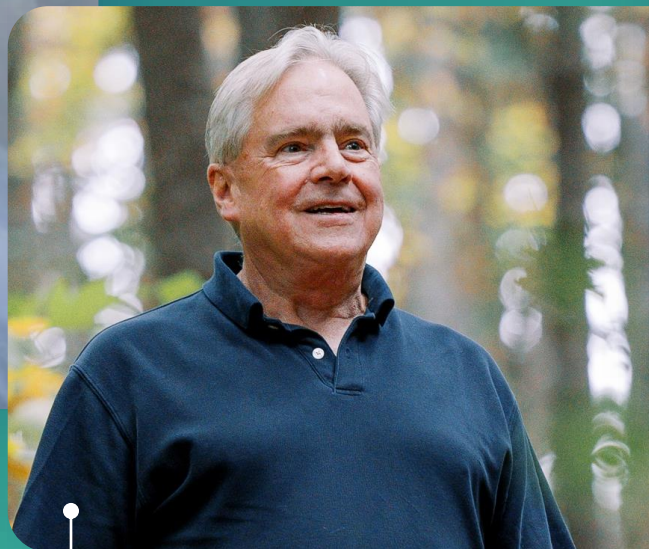
\*Inclusive of cash, cash equivalents, and marketable securities.

\*\*Inclusive of expected \$200 million minimum draw 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