The **Power** of Predictability

August 2026

NASDAQ: BEAM

Beam THERAPEUTICS

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; our expectations regarding the anticipated launch of risto-cel; the potential for an accelerated approval path for BEAM-302; our expectations regarding the funds that will be available to draw under our credit facility; 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; that we may not achieve the funding milestones under our credit facility; 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.

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

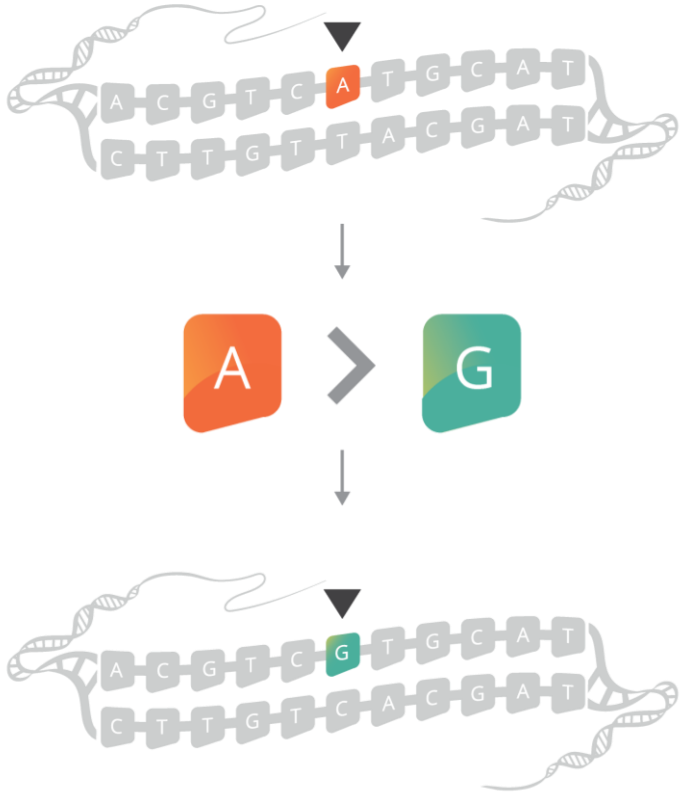
GENE EDITING FOR
rare and common diseases

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



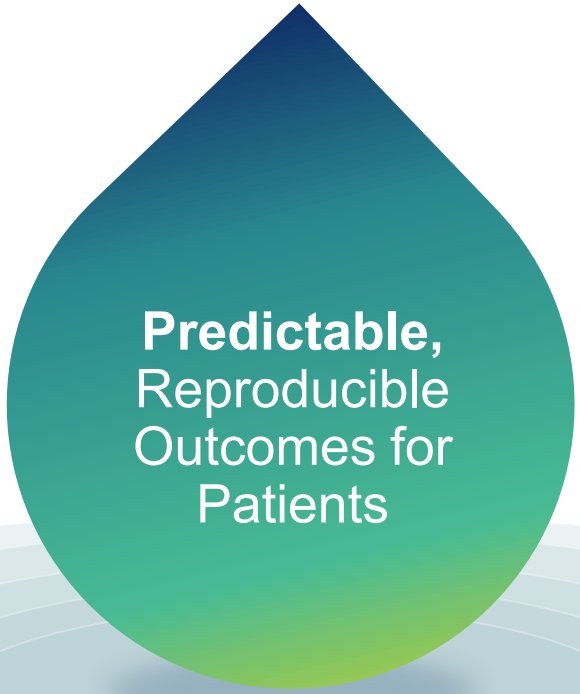
CONSISTENT gene sequence outcomes

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

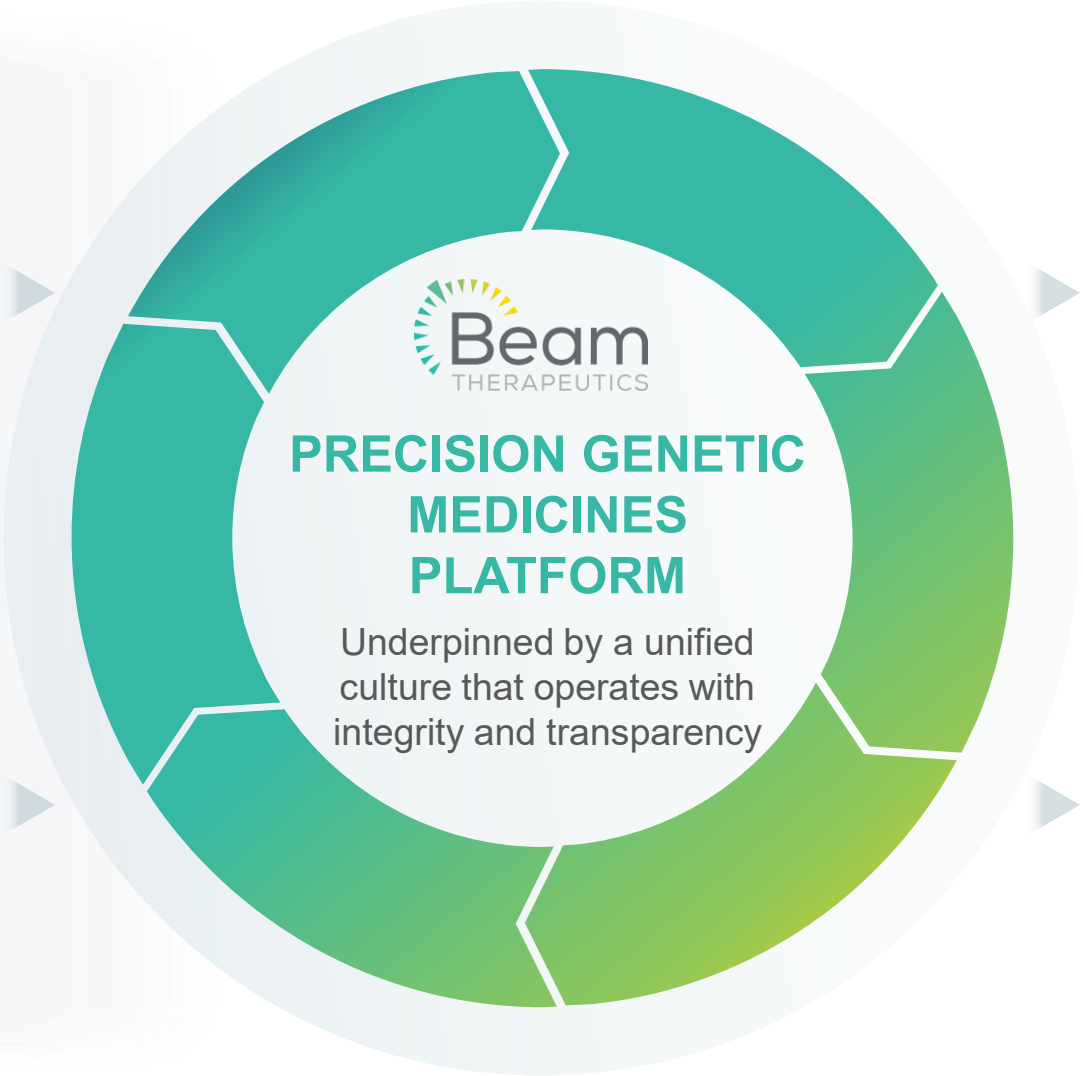
Easily **ADAPTABLE** to new targets

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**

Establishing a foundation of financial strength for sustainable growth

Financial Strength

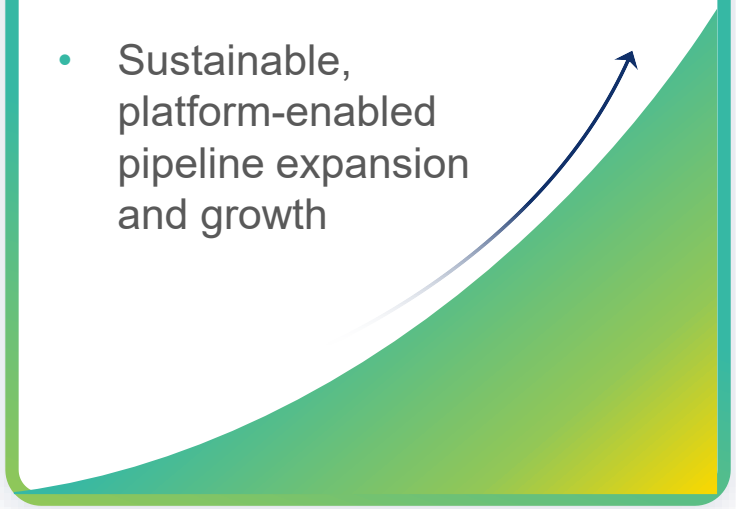
- With $1.2 billion in cash as of June 30, 2026*, together with a potential additional $200 million from the Sixth Street facility, expected to fund anticipated operating expenses and capital expenditures into mid-2029
- Anticipate funding through risto-cel launch, execution of BEAM-302 pivotal development plan, and clinical POC for BEAM-304 in PKU

Focused Investment

- 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

- Wholly owned pipeline
- Significant addressable markets
- Sustainable, platform-enabled pipeline expansion and growth



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

Liver-targeted Genetic Disease Franchise

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

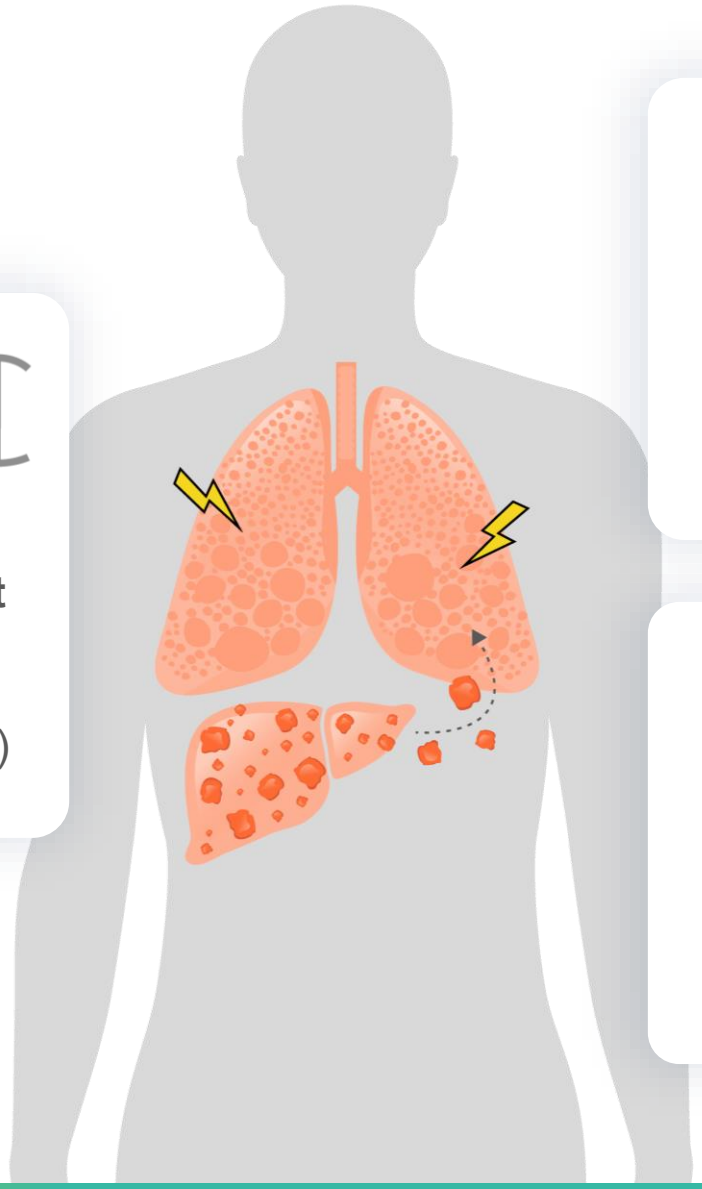
Platform synergies potentially enable use of novel regulatory pathways and established clinical footprint

PROGRAM	DISEASE	DELIVERY	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
BEAM-302	Alpha-1 antitrypsin deficiency (AATD)	*In vivo* LNP	Correction of E342K mutation					
BEAM-301	Glycogen storage disease type Ia (GSDIa)	*In vivo* LNP	Correction of R83C mutation					
BEAM-304	Phenylketonuria (PKU)	*In vivo* LNP	Correction of R408W and other mutations					

LNP = lipid nanoparticle

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:

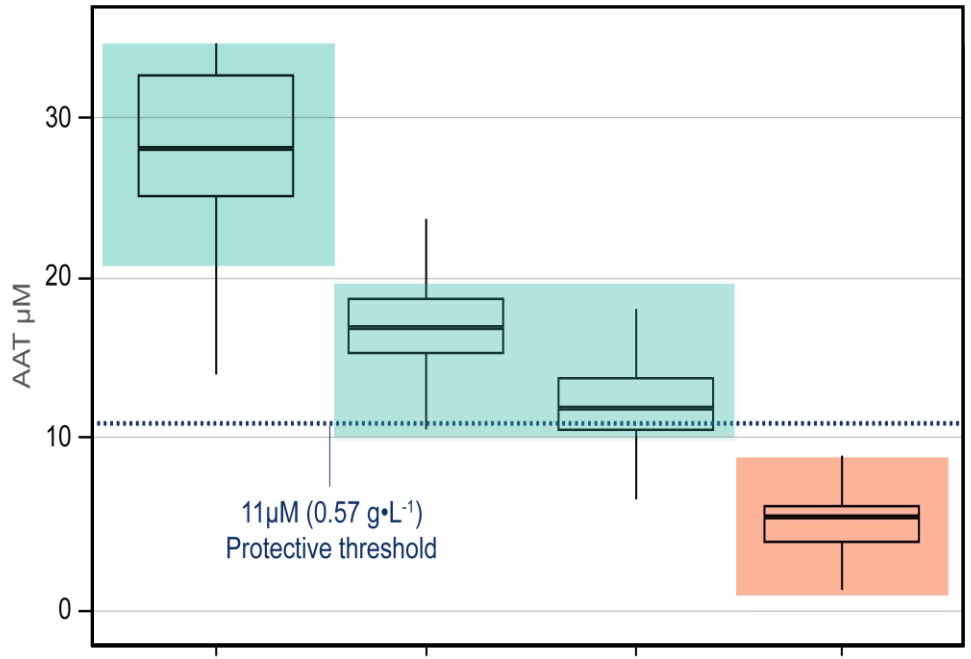
- Aggregation and accumulation of mutant Z-AAT

× **Supportive care and liver transplant** for advanced disease

× **No approved treatments** for liver disease

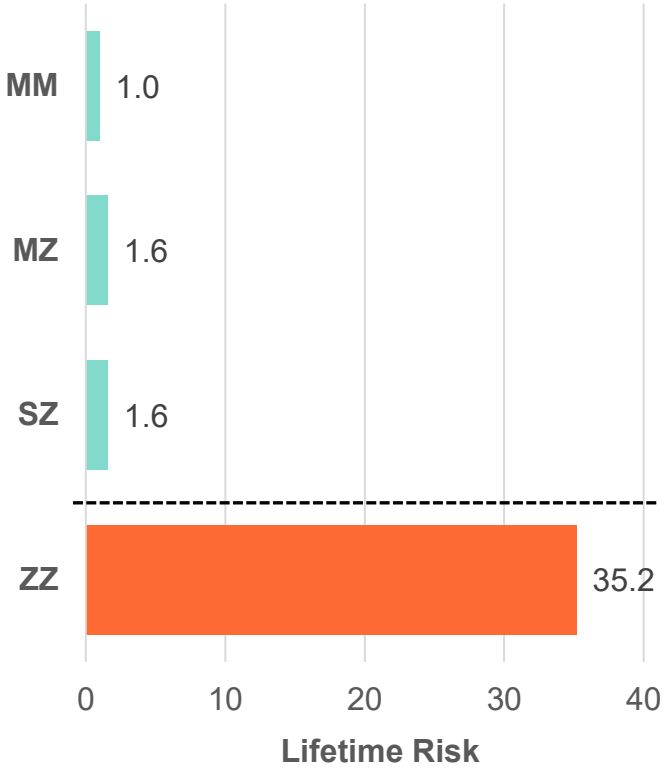
AAT protective threshold >11 µM informed by clinical genetics, translates into marked risk reduction for AATD lung and/or liver disease

AAT Levels Across Genotypes

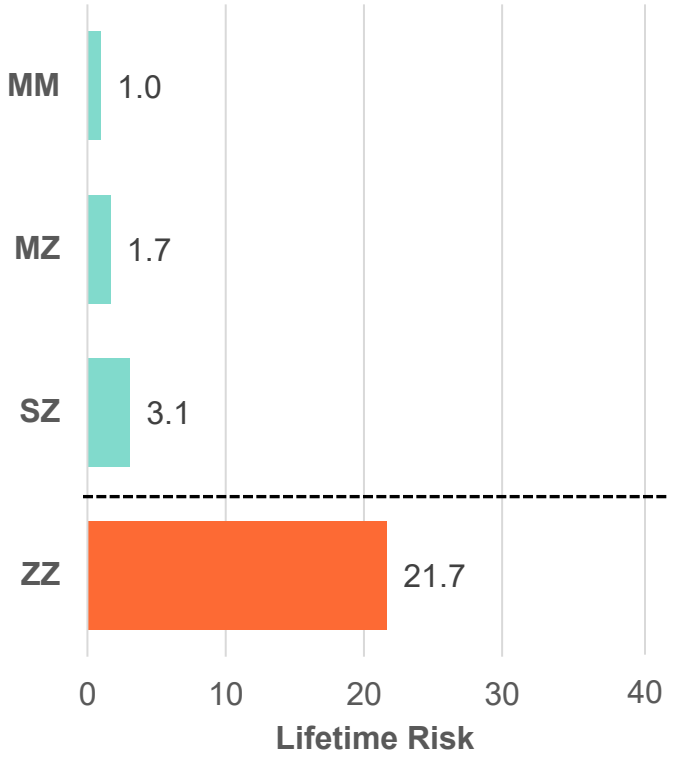


Genotype	MM	MZ	SZ	ZZ
< 11 µM	0%	0%	40%	100%

Emphysema Risk



Liver Fibrosis / Cirrhosis Risk



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

Micromolar (µM)

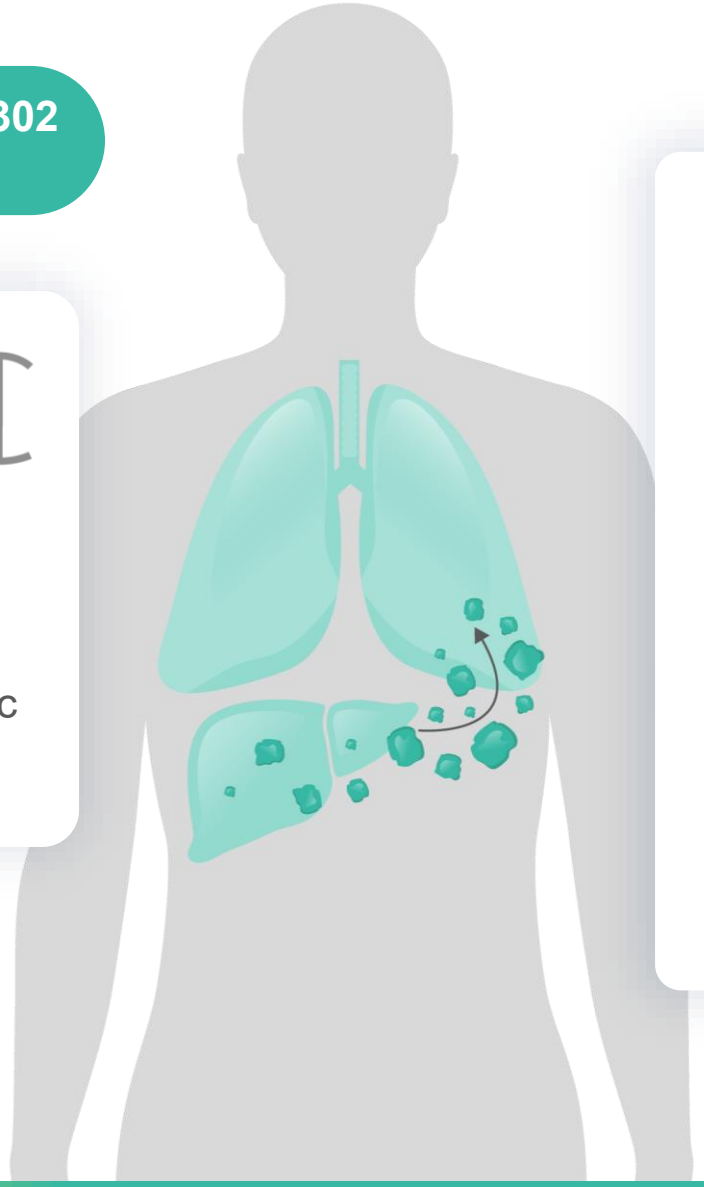
Adapted from Franciosi et al, ERJ 2022 and Vidal et al, Arch Bronconeumol 2006; Brode et al, CMAJ 2012; Fromme et al. 2022

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 µM 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 µM***, with all patients consistently >11 µM 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 ~30 µM** (~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 global pivotal trial**

Updated clinical data accepted for late-breaking oral presentation at the European Respiratory Society (ERS) Congress on Sept. 8, 2026

Data cutoff February 10, 2026

*Values shown are the mean of subject-level steady-state mean values, which is defined as the period beginning on a patient's Day 28 visit and lasting until that patient's Month 12 visit (or until that patient's last visit, if earlier than twelve months).

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 global, open-label Phase 1/2 trial
- Ongoing dialogue with FDA on confirmatory trial design
- Accepted to FDA CMC Development and Readiness Pilot (CDRP) program

IND = investigational new drug; RMAT Designation = Regenerative Medicine Advanced Therapy Designation

Dosing initiated in pivotal cohort, an expansion of the ongoing Phase 1/2 trial of BEAM-302

PART A:
AATD-associated Lung Disease

DOSE ESCALATION & EXPANSION

- Additional patients treated in 60 mg expansion, including at U.S. sites

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

DOSE ESCALATION

- Additional patients treated in 60 mg cohort

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

- Dosing initiated in global pivotal cohort in July 2026
- 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

- 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

- 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

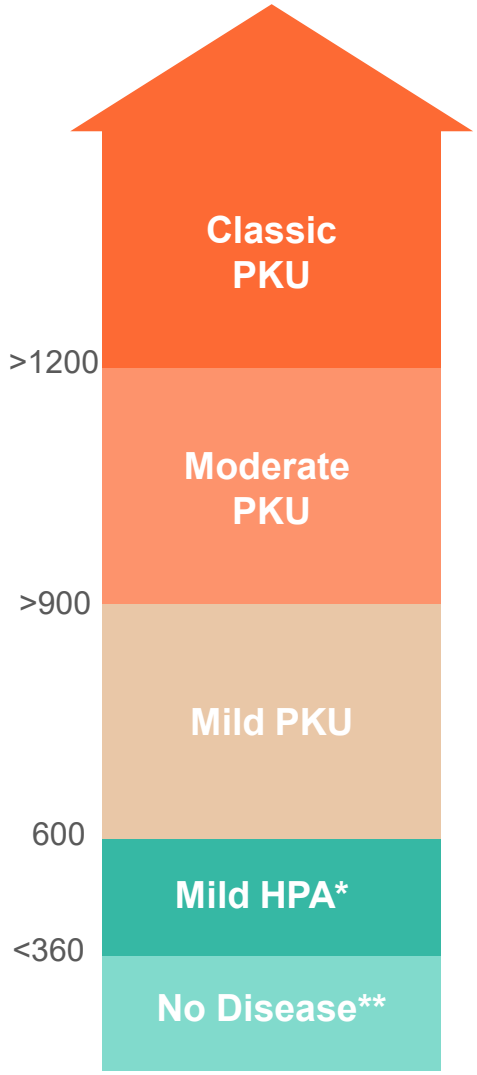
PKU is a genetic metabolic disease in which failure to metabolize phenylalanine leads to neurotoxicity

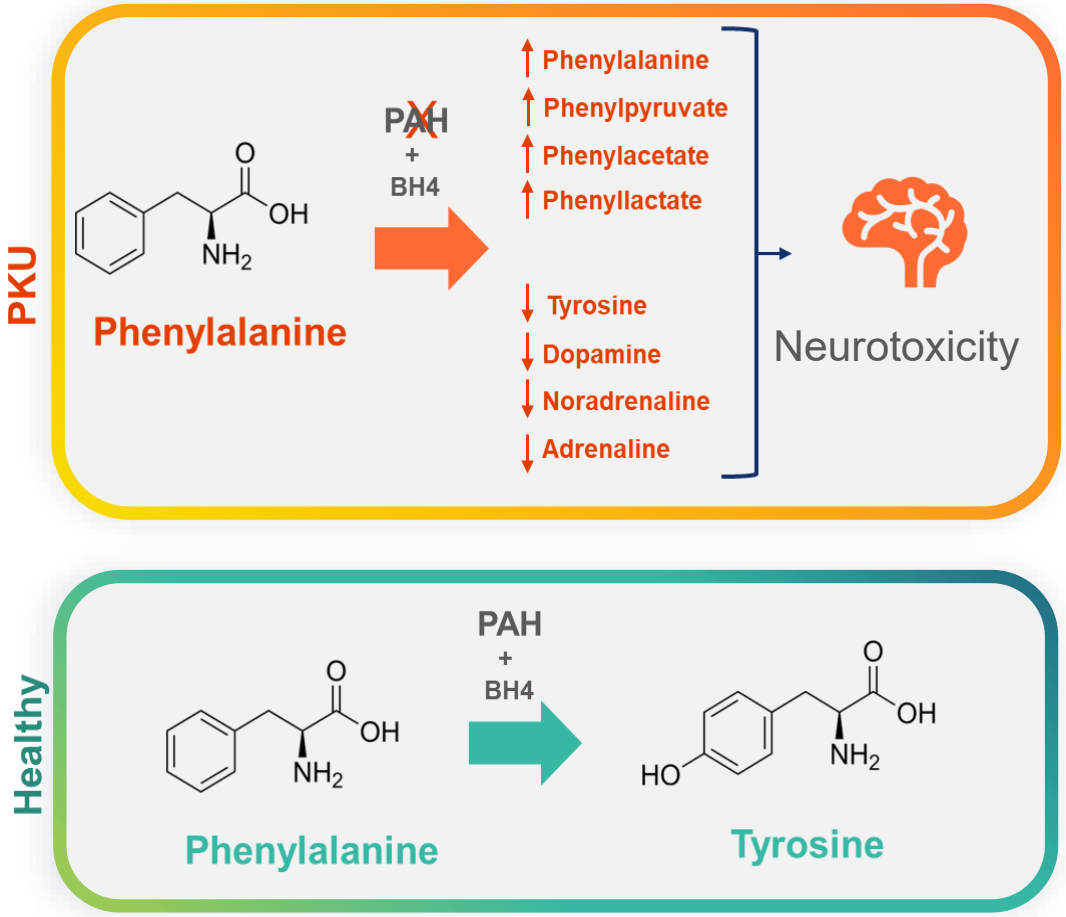
PKU is caused by mutations in *PAH* gene, resulting in loss of PAH activity, failure to metabolize Phe and elevated Phe levels

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

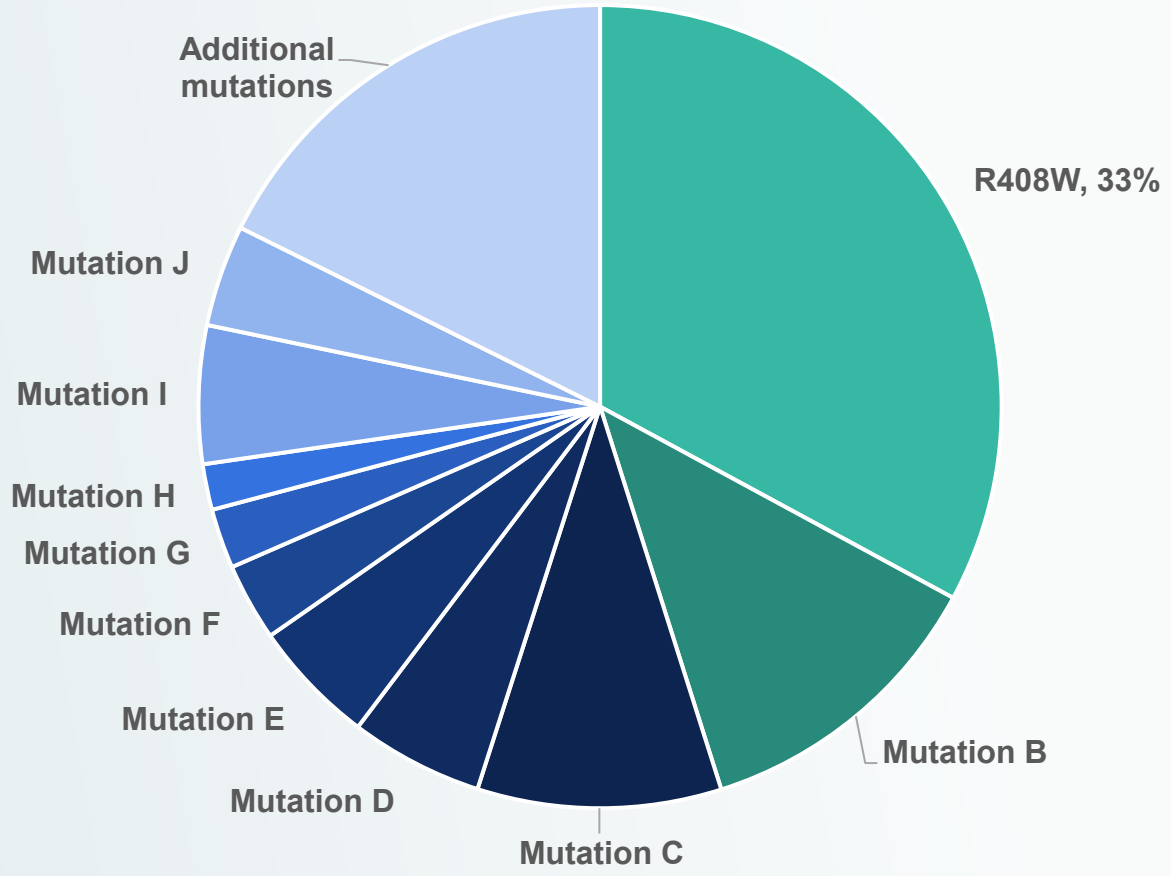
Target profile for successful clinical uptake of PKU gene therapy:

- **Significant and sustained reduction in Phe levels** (<360µM)
- **Well tolerated**
- **Diet normalization** (no medical food or Phe restrictions)
- **One-time therapy for lifelong Phe control**

Sources: Kuvan prescribing information; Palynziq prescribing information; EMA CHMP assessment report for Palynziq 2019; ACMG Board of Directors et al. Genetics in Medicine, Volume 27, Issue 1, 101289

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

~20,000 PKU Individuals in U.S.

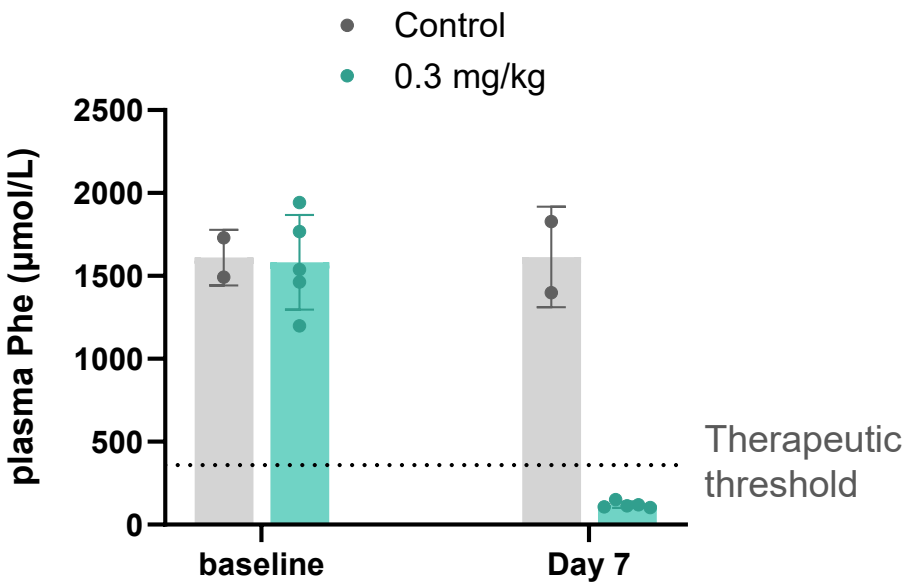


- Two identified development candidates have potential to address nearly half of PKU patients
- Ongoing research efforts focused on correcting additional mutations
- Opportunity to leverage novel regulatory and clinical approach to efficiently address vast majority of PKU

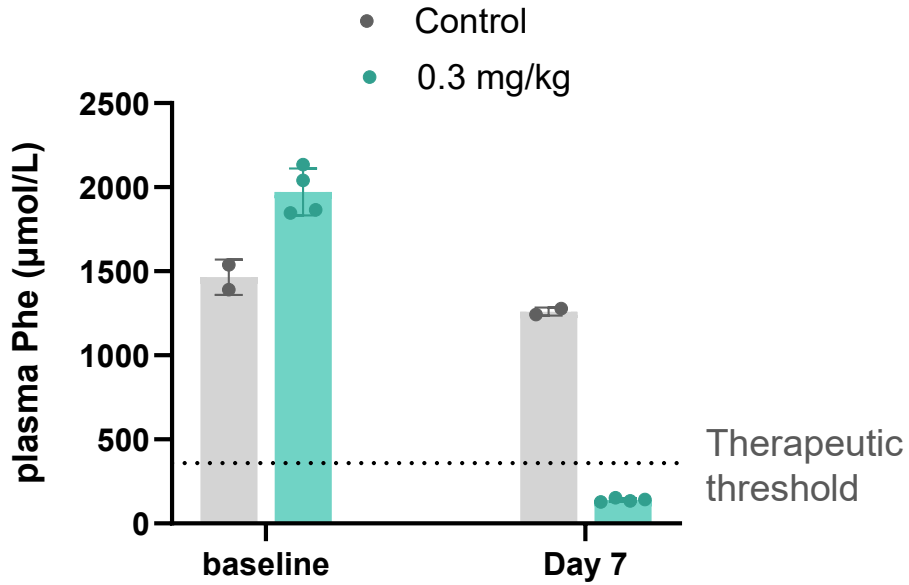
Source: Beam internal data; Hillert, Alicia et al. The American Journal of Human Genetics, Volume 107, Issue 2, 234 – 250; Stone WL, Los E. Phenylketonuria (PKU). StatPearls Publishing; 2025 Jan

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

R408W MUTATION



MUTATION B



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

Source: Beam internal data

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
- Study start-up activities ongoing

PHASE 1/2

Open-label, single-ascending dose trial in PKU patients 12-65 years old

DOSE EXPLORATION ▶ **DOSE EXPANSION**

KEY ENDPOINTS

- Safety and tolerability
- Reduction of blood Phe concentration

FDA IND cleared in June 2026

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

Glycogen Storage Disease Type Ia

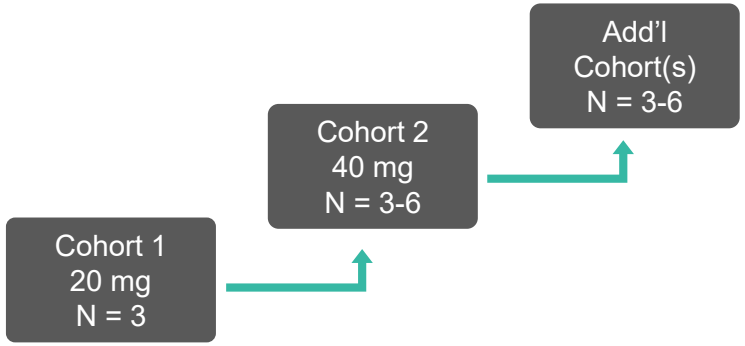
- Orphan disease
- Inability to produce glucose from endogenous sources to sustain blood sugar while fasting
- Constant risk of hypoglycemia that can result in seizures, coma or death
- Standard of care is cornstarch taken every 2-4 hours, even overnight

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

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	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
Risto-cel	Sickle cell disease (SCD)	*Ex vivo* HSC	Activation of fetal hemoglobin (HbF)					
BEAM-103	SCD Beta-thalassemia	*Ex vivo or in vivo* HSC	CD117 edit-antibody (ESCAPE)					
***In vivo* HSC editing**	SCD Beta-thalassemia	*In vivo* LNP	Activation of HbF					

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

Data from BEACON trial presented at ASH 2025

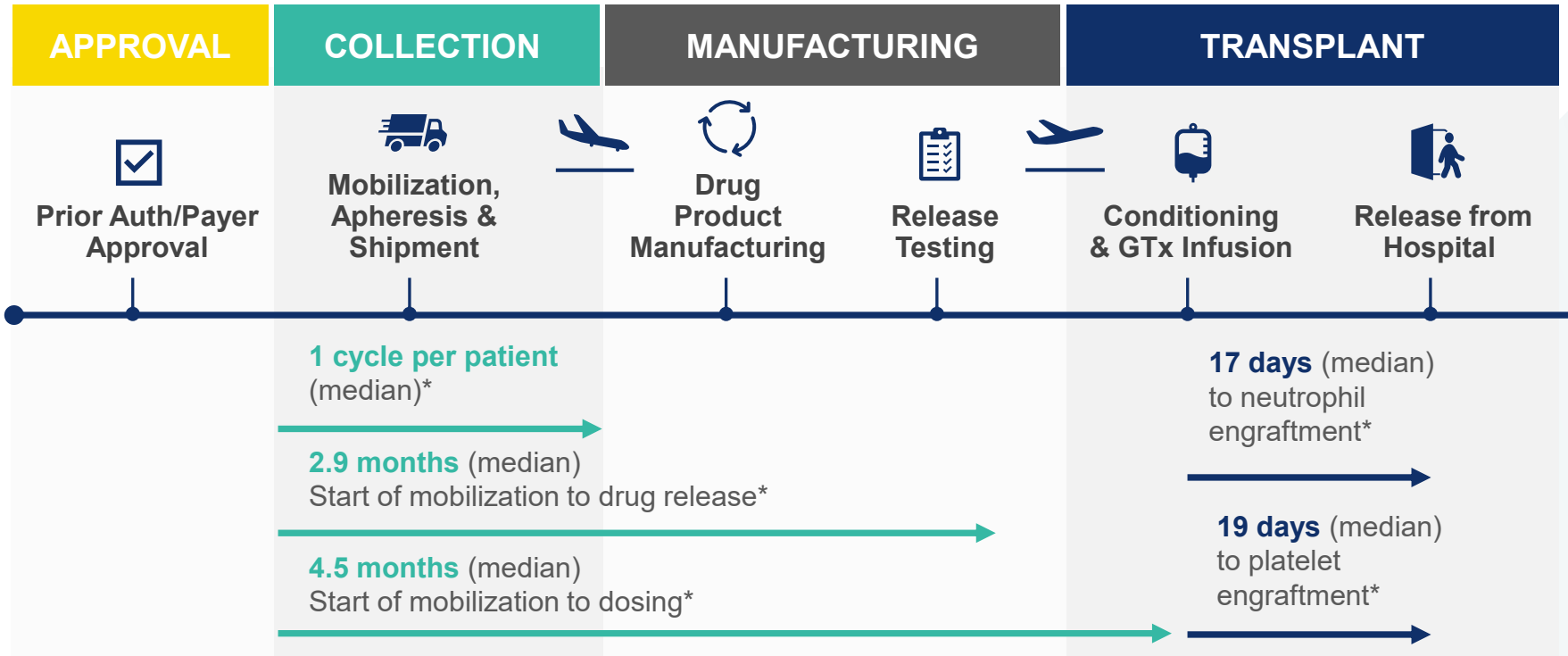
Major bottlenecks for SCD gene therapy commercial adoption are limited manufacturing capacity and low process efficiency

PATIENT INTEREST	TREATMENT CENTERS	CELL COLLECTION & MANUFACTURING	PATIENT OUTCOMES	REIMBURSEMENT
✓ Patient waiting lists at major centers	✓ 70+ treatment centers active	✗ Inefficient, unpredictable process ✗ Multiple cell collection cycles ✗ Manufacturing slots fully booked	✓ Patient outcomes have been positive	✓ Pricing support and no payment rejections to date

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

Source: Beam market research, competitive intelligence, sell side research and ASH 2025 data

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

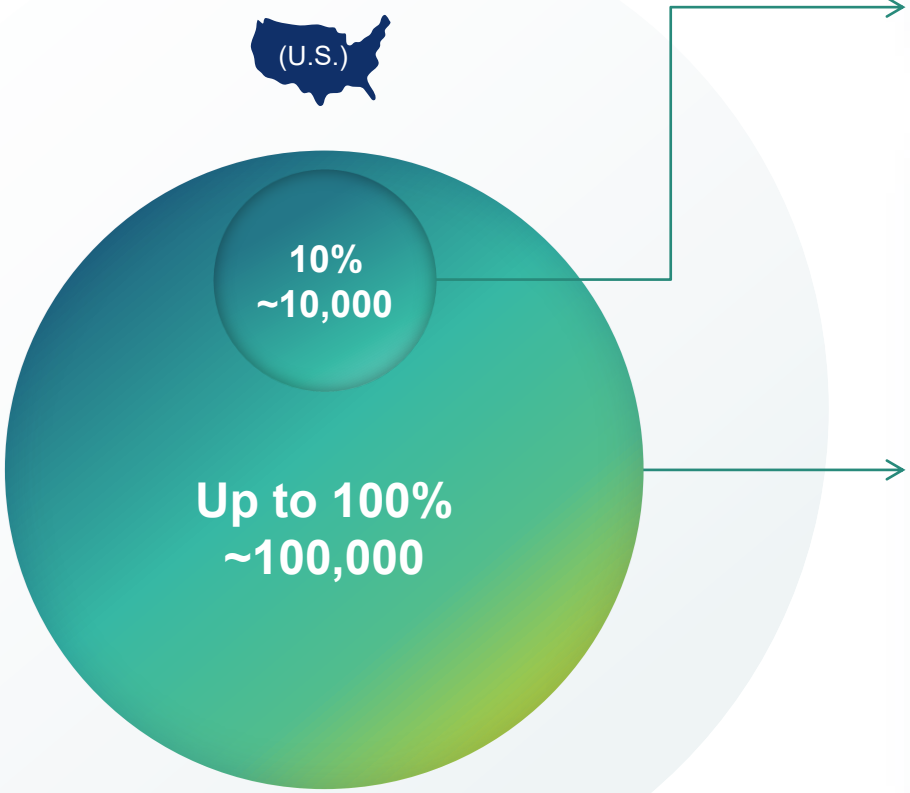
Dosing complete in all adult and adolescent patients
in BEACON trial

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



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
  - Dosing complete in healthy volunteer study of BEAM-103, an anti-CD117 monoclonal antibody; all doses tested were well tolerated
  - Maintain optionality to pursue *ex vivo* applications in SCD and other hematologic diseases

Source: Market research, internal Beam estimates

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

Beam THERAPEUTICS

verve THERAPEUTICS — Acquired by Lilly (Lilly)	ORBITAL THERAPEUTICS — Acquired by BMS (Bristol Myers Squibb™)
Apellis — Opted-in to FcRn program	Pfizer — Opted-in to program
Sana Biotechnology®	prime medicine

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

- Enrolling and dosing for BEAM-302 pivotal cohort ongoing
- Present detailed and updated BEAM-302 data at ERS in Sept. 2026
- Plan to submit risto-cel BLA as early as YE 2026

Advance and Expand Pipeline

- Study start-up activities ongoing for BEAM-304 Phase 1/2 clinical trial
- Report initial BEAM-301 data by YE 2026
- Advance *in vivo* HSC editing program

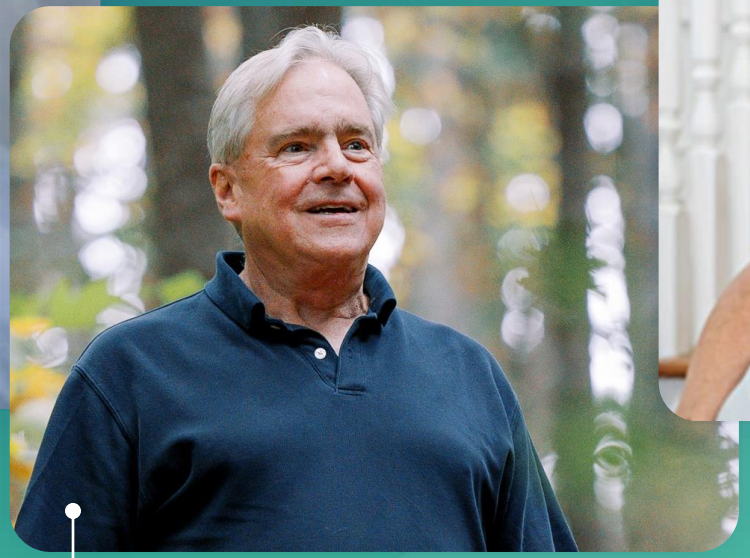
Maintain Financial Strength

- $1.2 billion in cash as of June 30, 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 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