Beam THERAPEUTICS

PRECISION GENETIC MEDICINES THROUGH BASE EDITING

2025 J.P. Morgan Healthcare Conference

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 initiation, timing, progress and results of preclinical studies and research and development programs, including the initiation, design and progress of clinical trials, including trials for BEAM-101, BEAM-103, BEAM-301 and BEAM-302; the advancement of our pipeline and additional liver programs in multiple preclinical studies; our current expectations and anticipated results of operations, including our expected use of capital; our estimated cash, cash equivalents and marketable securities as of December 31, 2024 and our expectations related thereto; 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 life-long, 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.

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OUR VISION IS TO PROVIDE LIFE-LONG CURES for patients suffering from serious diseases

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

GENE EDITING FOR **rare and common diseases**

PLATFORM FOR **rapidly programmable precision medicines**

Beam THERAPEUTICS

People like Branden, living with sickle cell disease, are at the heart of our vision

> “From age 12, things skyrocketed. I was in the hospital every other month with crises.”

In sixth grade, Branden had his left hip replaced because of damage to his hip joint.

His right hip was replaced not long after.

At age 17, he suffered a life-threatening acute chest syndrome (ACS), with sickled cells blocking blood vessels in his lungs.

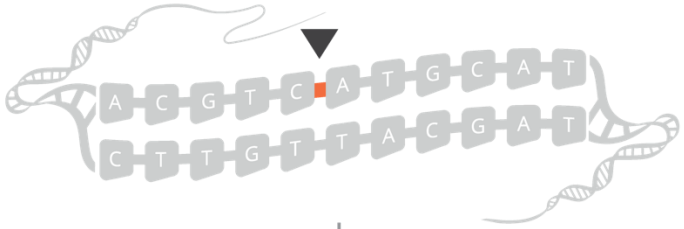
Four more ACS episodes that year caused Branden to miss his entire junior year of high school.

From BOSTON CHILDREN’S HOSPITAL ANSWERS BLOG
‘A sickle cell first’
Posted December 9, 2024

Base editing is an efficient, predictable and potentially best-in-class gene editing technology

NUCLEASE CRISPR, ZFN, TALENs

Precision targeting with CRISPR



Double-stranded breaks

Lack of control of gene sequence outcomes

…A C G - - - - G C A T…
…A C G T C **G C T T** A T G C A T…
…A - - - - - T G C A T…
…A C G T C **T** A T G C A T…
…A C - - - - - - - A T…
…A C G T C **A A C** - - G C A T…
Etc.

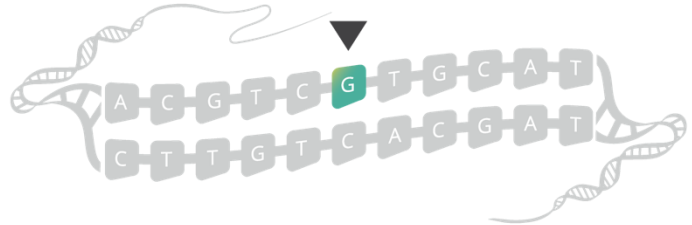
- - - - - Deletions
A G C T Insertions

BASE EDITING BEAM THERAPEUTICS

Precision targeting with CRISPR

Enzymatic base conversion

Highly efficient, with predictable gene sequence outcomes



Key elements of Beam's leadership position in gene editing

BASE EDITING PLATFORM
Clinical Validation, Fully Integrated Manufacturing

HIGH VALUE FRANCHISES
with Best-in-Class Potential

RAPID EXECUTION
for Clinical Programs

MULTIPLE CATALYSTS
Expected in 2025

$850.7M in cash estimated as of December 31, 2024*
expected to fund operations, including anticipated commercial readiness activities for BEAM-101, into 2027

*This estimate is preliminary, unaudited and is subject to completion of Beam's financial statement closing procedures.

A comprehensive, fully integrated base editing platform

BASE EDITING PLATFORM
Clinical Validation, Fully Integrated Manufacturing

GENE EDITING AND DELIVERY TECHNOLOGIES

- Base editing — ✓ *Clinical proof of concept*
- *ex vivo* cell therapy — ✓ *Clinical proof of concept*
- *in vivo* LNP

FULLY INTEGRATED CAPABILITIES

GMP manufacturing at NC facility

>100 GMP batches/ isolations

Global regulatory filings

7 *IND/CTA approvals*
5 *countries*

Global clinical operations

30+ *clinical sites*
>20 *patients treated*

INTERNAL GMP MANUFACTURING FACILITY

Research Triangle, NC

Strategic focus areas with potential for significant value creation

HIGH VALUE FRANCHISES
with Best-in-Class Potential

Hematology

- Best-in-class potential for BEAM-101 in sickle cell disease (SCD)
  → *ASH 2024 data*
- Well-established FDA path to BLA
- Lifecycle strategy with ESCAPE and *in vivo* editing
- Platform for future hematology pipeline

Liver Genetic Diseases

- Best-in-class potential for BEAM-302 in alpha-1 antitrypsin deficiency (AATD)
- Potential one-time treatment for both lung and liver, under normal gene regulation
- Platform for future liver-targeted pipeline

Proven ability to execute on key R&D priorities

RAPID EXECUTION
Across Clinical Programs

- 40+ patients enrolled, 20+ doses manufactured and 13 patients dosed* with BEAM-101 in 1 year
- Opening of BEACON adolescent cohort approved by FDA
- First *in vivo* CTA clearances achieved (BEAM-302) with enrollment on track and sites active in UK, NZ, Australia, and Netherlands
- First *in vivo* IND clearance achieved (BEAM-301) with first site open in US
- ESCAPE programs named (BEAM-103, BEAM-104) with Phase 1-enabling tox studies initiated in December 2024

*As of January 9, 2025

Significant potential catalysts on the horizon

MULTIPLE CATALYSTS
Expected in 2025

BEAM-101
SCD (BEACON TRIAL)

- Dose 30 patients by mid-2025
- Enroll and dose adolescent patients
- Present updated data in mid-2025

BEAM-302
AATD

- Present initial data on multiple cohorts in 1H 2025

ESCAPE
SCD & BETA-THALASSEMIA

- Initiate Phase 1 healthy volunteer study of BEAM-103 antibody by YE

BEAM-301
GSD1a

- Dose first patient in Phase 1/2 study in early 2025

Advancing a diversified pipeline

PROGRAM / DISEASE or TARGET		DELIVERY	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
BEAM-101	Sickle cell disease (SCD)	*Ex vivo* HSC	Activation of fetal hemoglobin (HbF)					
ESCAPE (BEAM-103 & BEAM-104)	SCD Beta-thalassemia	*Ex vivo* HSC	Multiplex HbF edit + CD117 edit-antibody pair					
***In vivo* HSC editing**	SCD Beta-thalassemia	*In vivo* LNP	Activation of HbF					
BEAM-302	Alpha-1 antitrypsin deficiency (AATD)	*In vivo* LNP	Correction of E342K mutation					
BEAM-301	Glycogen storage disease 1a (GSD1a)	*In vivo* LNP	Correction of R83C mutation					
Apellis collaboration	Autoimmune disorders and other undisclosed	*In vivo* LNP	Modification of FcRN and other undisclosed					
Pfizer collaboration	Undisclosed	*In vivo* LNP	Undisclosed					

LNP = Lipid Nanoparticle; HSC = Hematopoietic Stem Cell; ESCAPE: Engineered Stem Cell Antibody Paired Evasion; FcRN = neonatal Fc receptor
Pipeline does not include assets designated for partnering

What if we could develop better one-time therapies for patients with SCD?

SICKLE CELL DISEASE

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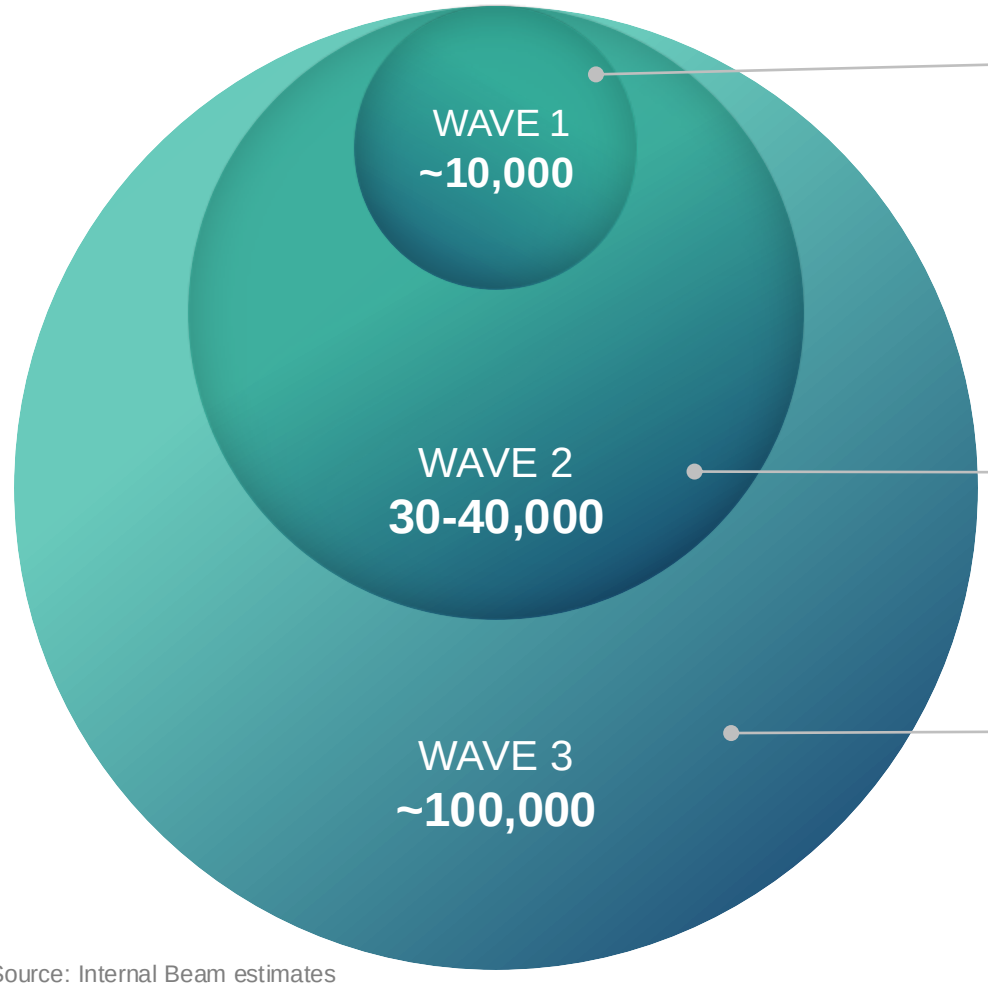
Multi-wave strategy is focused on developing safer, more effective and more accessible treatments for patients with SCD

Potential Eligible SCD Patient Population (U.S.)



BEAM-101:
Precise HbF upregulation

Potentially best-in-class clinical profile
Non-cutting, non-viral therapy with busulfan conditioning to address most severe SCD patients

ESCAPE:
Multiple edits for non-genotoxic conditioning

Designed to eliminate chemotherapy from *ex vivo* gene therapy and expand patient population

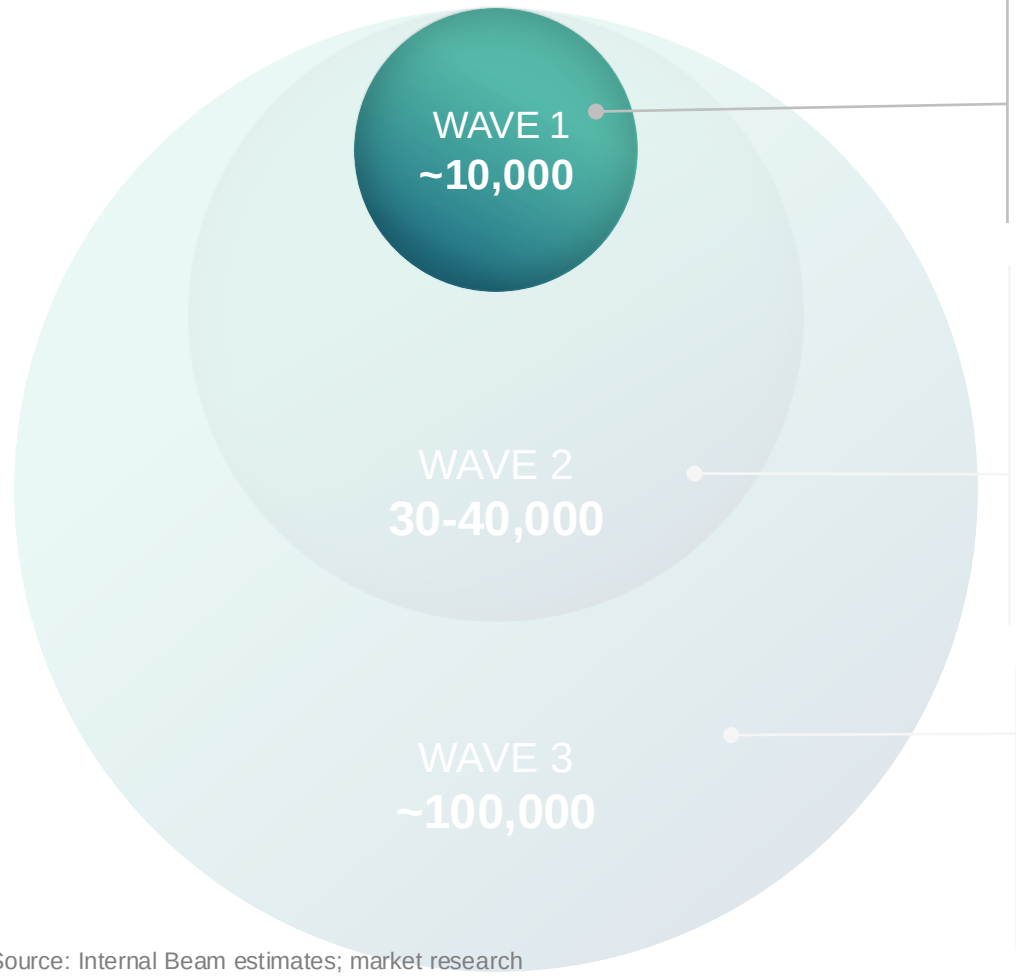
In vivo:
Direct base editing of stem cells with LNPs

***In vivo* delivery is maximally scalable**, lowering infrastructure needs and unlocking wider patient access

Source: Internal Beam estimates

Significant market potential for SCD gene therapy

Potential Eligible SCD Patient Population (U.S.)



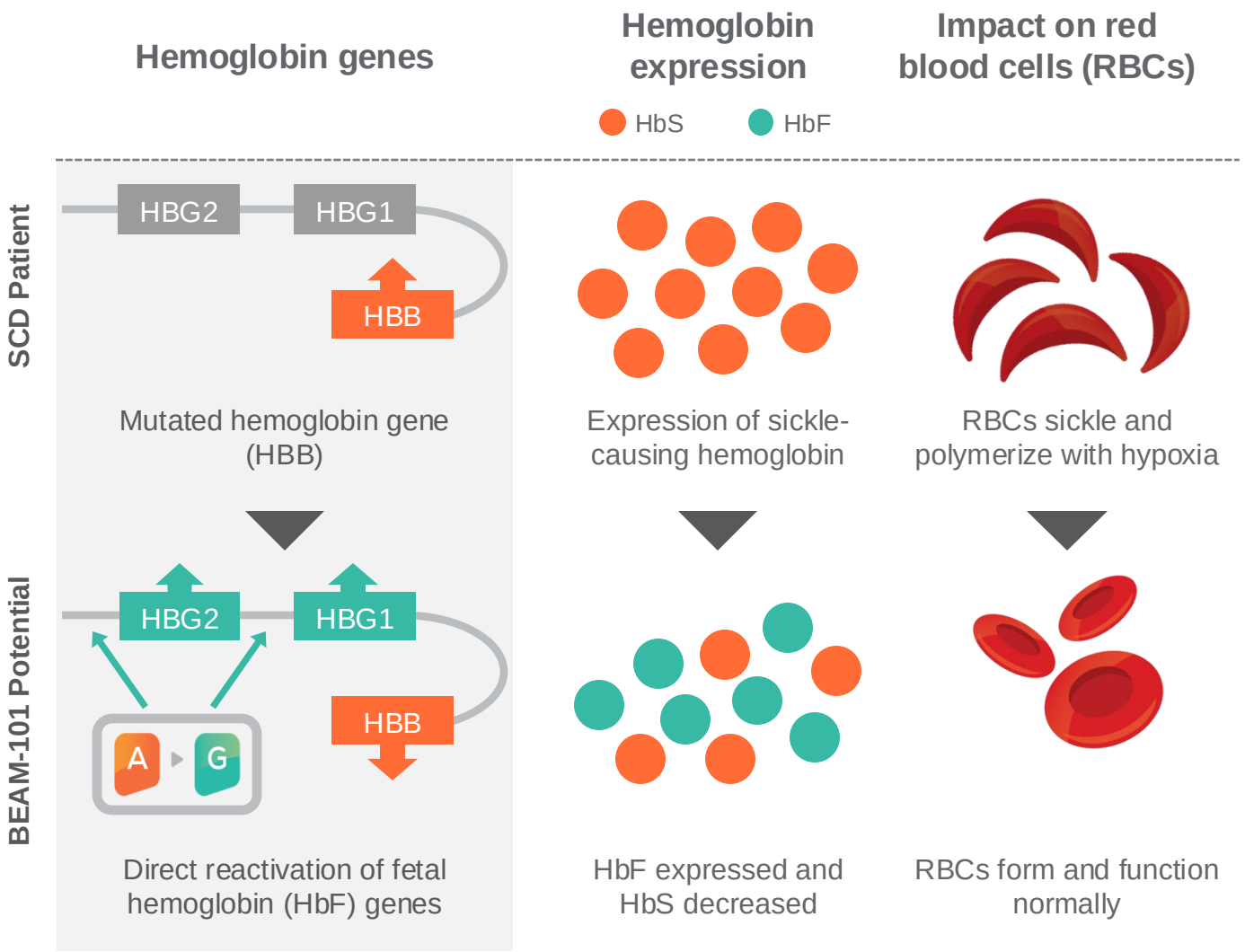
Highlights on potential market for gene therapy with busulfan conditioning in SCD

- Current demand for matched allo transplants in SCD implies at least an annual $1.5B gene therapy market* in U.S.
- Market research suggests peak potential of 1,000-1,200 patients/year
- Market infrastructure for SCD gene therapy is building momentum

> *"The demand for gene therapy in SCD will be dictated by the industry's ability to supply."*
>
> – High-volume U.S. transplanter and KOL

Source: Internal Beam estimates; market research
* ~200 matched transplants / year with ~33% match rate; $2-3M price / patient for gene therapy

BEAM-101 designed to be best-in-class genetic medicine for sickle cell disease



SCD Unmet Need

- Sickle hemoglobin (HbS) polymerization is root cause of sickle cell pathophysiology
- Affects millions of people worldwide and ~100K in U.S.
- Median survival in the U.S. is ≥ 20 years shorter

Current Available Treatments

- Disease-modifying therapies do not prevent organ dysfunction and require ongoing treatment
- Recently approved gene therapies reduce VOCs but residual HbS >50% suggests room for improvement

CDC Data & Statistics; Lancet Haematol 2023; 10: e585–99; DeBaun et al. Blood. 2019 Feb 7; 133(6):615-617

BEACON Phase 1/2 study rapidly advancing

SENTINEL COHORT (N=3)

- ✓ Staggered start with SRC review in between
- ✓ Enrollment complete
- ✓ Dosing complete

DMC review →

EXPANSION COHORT

- ✓ 40+ patients cleared screening and enrolled
- ✓ 13 patients dosed as of Jan. 9; 30 expected to be dosed by mid-2025
- ✓ Screening initiated for adolescent patients

KEY ELIGIBILITY CRITERIA

- Age ≥18 to ≤35 years
- SCD with β^S/β^S, β^S/β^0, or β^S/β^+ genotypes
- ≥4 sVOCs in 24 months pre-screening
- No available matched sibling donor
- No history of overt stroke

KEY SAFETY ENDPOINTS

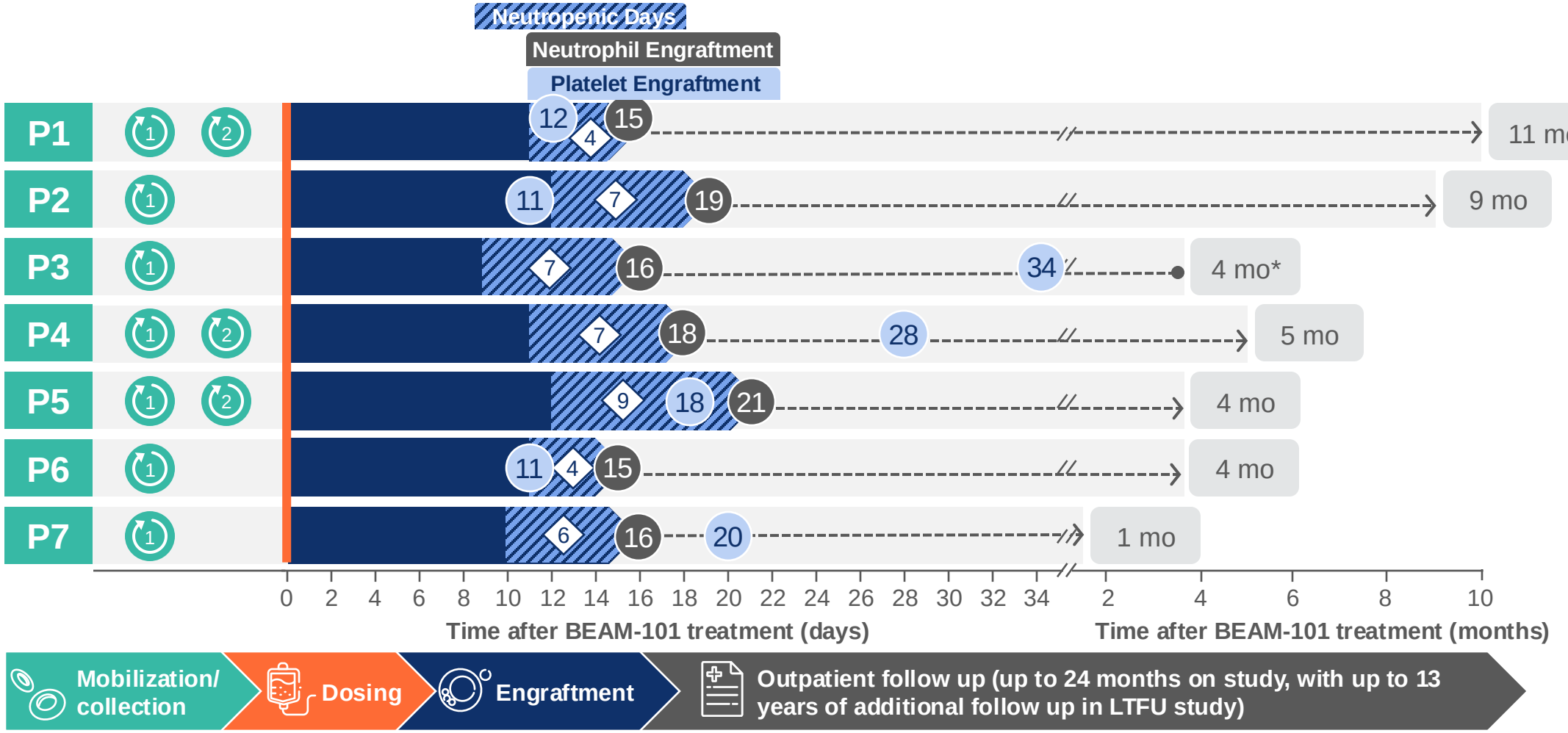
- Proportion of patients with successful neutrophil engraftment
- Time to neutrophil engraftment
- Time to platelet engraftment

KEY EFFICACY ENDPOINTS

- Proportion of patients sVOC-free for 12 consecutive months
- Total Hb levels
- HbF and HbS levels
- Hemolysis parameters
- Patient-reported outcomes
- RBC function and organ damage

Phase 1/2, non-randomized, open-label, single-arm, multicenter, safety and efficacy study of the administration of BEAM-101 to patients with SCD (NCT05456880)
DMC, Data Monitoring Committee; HbF, fetal hemoglobin; HbS, sickle hemoglobin; RBC, red blood cell; SCD, sickle cell disease; SRC, Safety Review Committee; sVOC, severe vaso-occlusive crisis

BEAM-101 and its treatment process aim to minimize mobilization and engraftment burden to reduce time in hospital



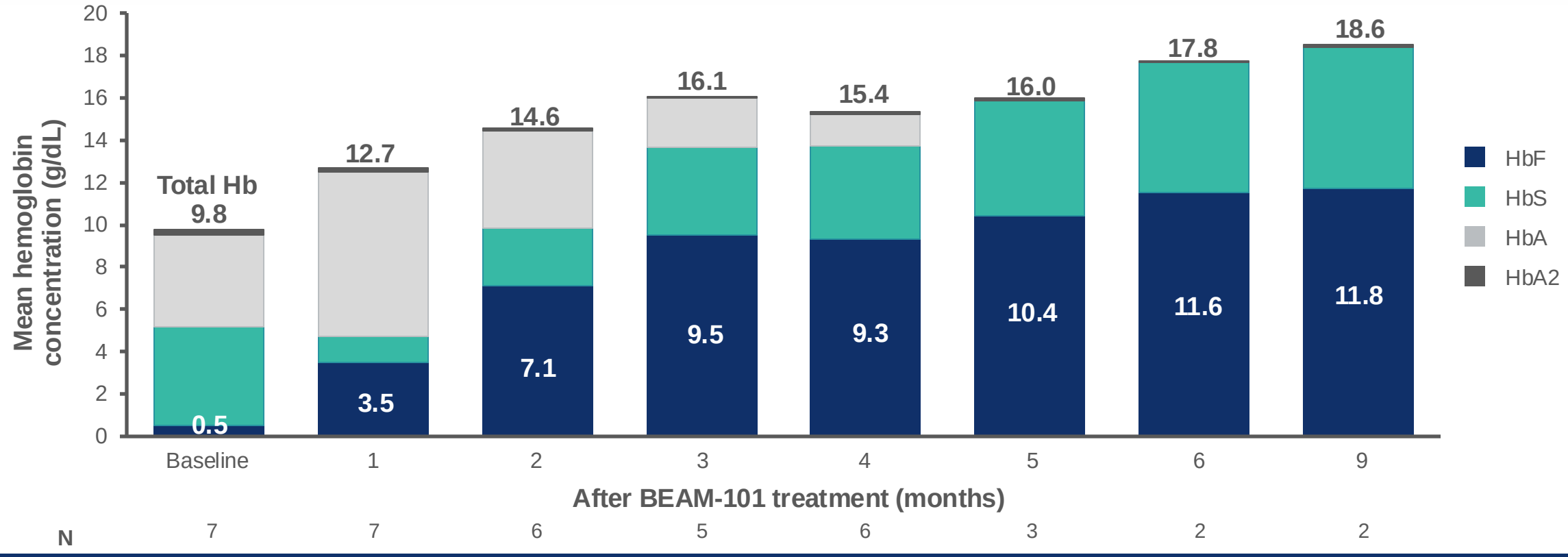
Data cutoff Oct 28, 2024; Presented at ASH 2024

Patients remain as inpatients until neutrophil engraftment has occurred, and the patient is deemed clinically stable for discharge and outpatient management. Patients assessed at daily intervals to evaluate engraftment success status. Patients will be discharged home after neutrophil engraftment. Platelet engraftment may be monitored in the outpatient setting on a weekly basis.
*P3 died due to refractory respiratory failure 4 months after infusion, assessed as due to busulfan and unrelated to BEAM-101.
LTFU, long-term follow up; mo, month; ND, neutropenic days; NE, neutrophil engraftment; P, patient; PE, platelet engraftment

Day of neutrophil engraftment
Day of platelet engraftment
Mobilization cycle
Daily monitoring
Neutropenic days
Follow up

Patients treated with BEAM-101 achieved rapid and robust HbF induction, HbS reduction, and resolution of anemia

All patients achieved endogenous HbF >60% and HbS <40% by 1 month after BEAM-101 treatment



N	7	7	6	5	6	3	2	2
%HbF (HbF/HbF+HbS)	10%	74%	72%	70%	67%	65%	65%	64%

Data cutoff Oct 28, 2024; Presented at ASH 2024

Female total Hb LLN-ULN: 11.5-15 g/dL; Male LLN-ULN: 13-17 g/dL. Hb, hemoglobin; HbA, adult hemoglobin; HbF, fetal hemoglobin; HbS, sickle hemoglobin; LLN, lower limit of normal; ULN, upper limit of normal

Emerging BEACON data show potential for significant differentiation of base editing and BEAM-101 for SCD

Efficient cell collection resulting in 1-2 mobilization cycles

- Assisted by automated, internal manufacturing process
- Potential for fewer hospital days

Initial safety profile consistent with myeloablative conditioning with busulfan and autologous transplant

- No SAEs related to BEAM-101

Neutrophil engraftment following busulfan occurred <20 days

- Potential for fewer hospital days

Initial potent HbF induction and HbS reduction in all patients: >60% HbF, <40% HbS

- Similar to sickle cell trait profile

Improved red blood cell health and function after BEAM-101

- Sickling parameters decreased to levels comparable to sickle cell trait
- Markers of hemolysis improved or normalized

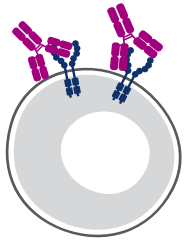
N=7; Data cutoff Oct 28, 2024 / Presented at ASH 2024

ESCAPE technology designed for selective suppression of diseased cells to enable non-genotoxic conditioning

ESCAPE technology for hemoglobinopathies is comprised of two components:

BEAM-103

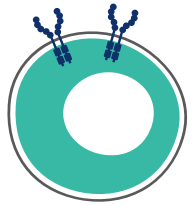
Anti-CD117 conditioning monoclonal antibody (mAb), which binds to the specific epitope edited by ESCAPE



Old diseased cells are targeted and suppressed by BEAM-103, creating space for the graft

BEAM-104

Engineered CD34 cell product with a therapeutic edit (same as in BEAM-101) plus an edit to CD117 to prevent binding of BEAM-103

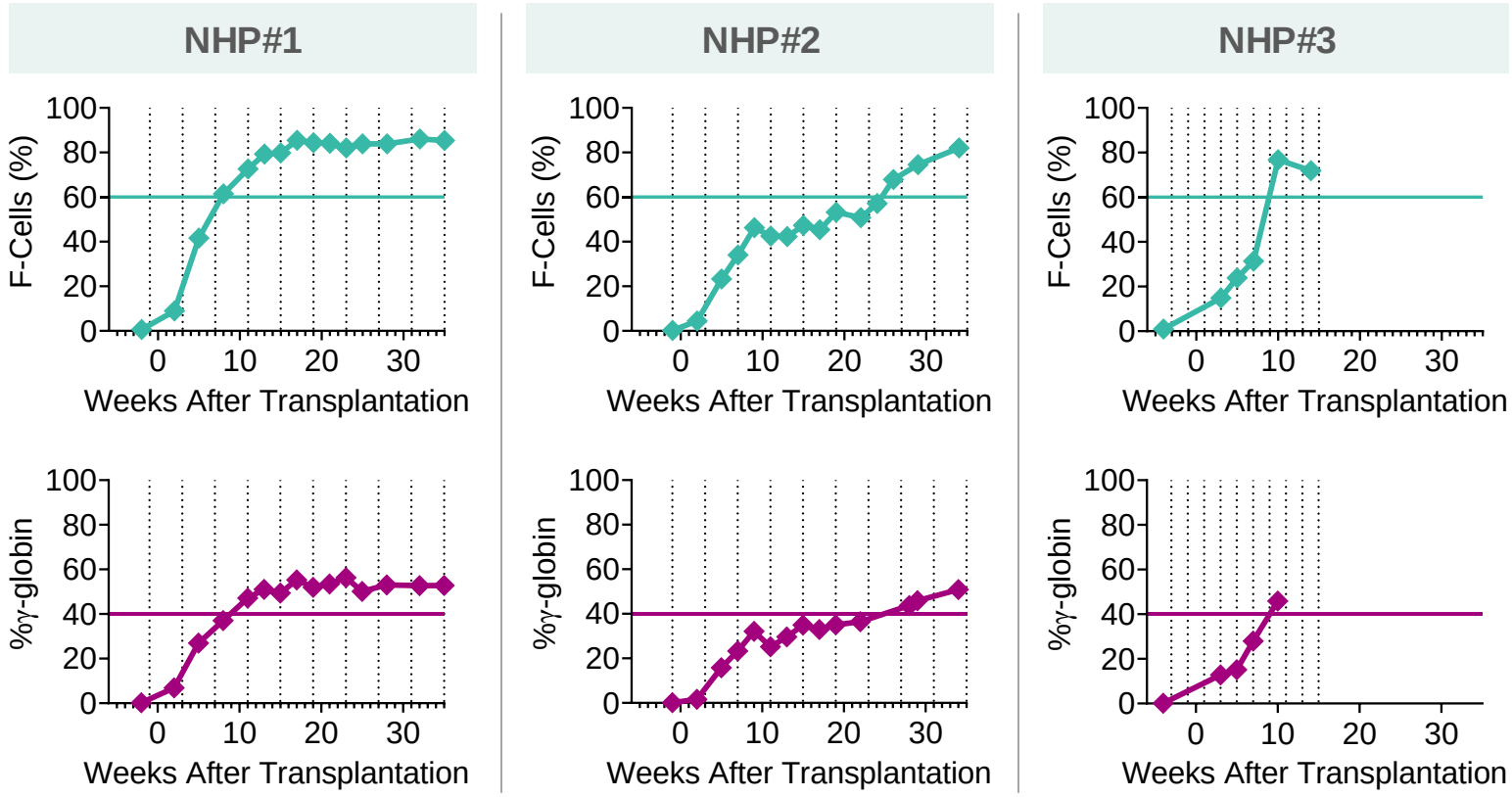


New edited cells “escape” binding by BEAM-103 and expand, leading to engraftment

Synergy between BEAM-101 and ESCAPE technology supports efficient development with potential to accelerate clinical trial, BLA filing, launch and commercial ramp

Preclinical proof-of-concept for ESCAPE antibody conditioning and engraftment in NHPs without chemotherapy

F-cell and γ-globin over time



- Rapid and complete replacement of erythroid cells by edited cells
- F-cell levels reached ~70-80% post-transplant
- γ-globin (proxy for HbF) reached ~50% in all animals
- mAb dosing was well tolerated with no use of transfusions/antibiotic support

Anticipated next steps and pathway to patients for ESCAPE

IN PROCESS

Phase 1-enabling tox studies

GMP manufacturing

PLAN TO INITIATE BY YE 2025

Phase 1 study of BEAM-103 Ab to evaluate PK/PD and safety in heathy volunteers

IN PROCESS

Follow-up NHP studies to optimize antibody dose regimen, dose response, and chimerism

Efficacy study of BEAM-103 and BEAM-104 in SCD and beta-thalassemia patients

What if we could use base editing to correct disease-causing mutations *in vivo?*

GENETIC DISEASES

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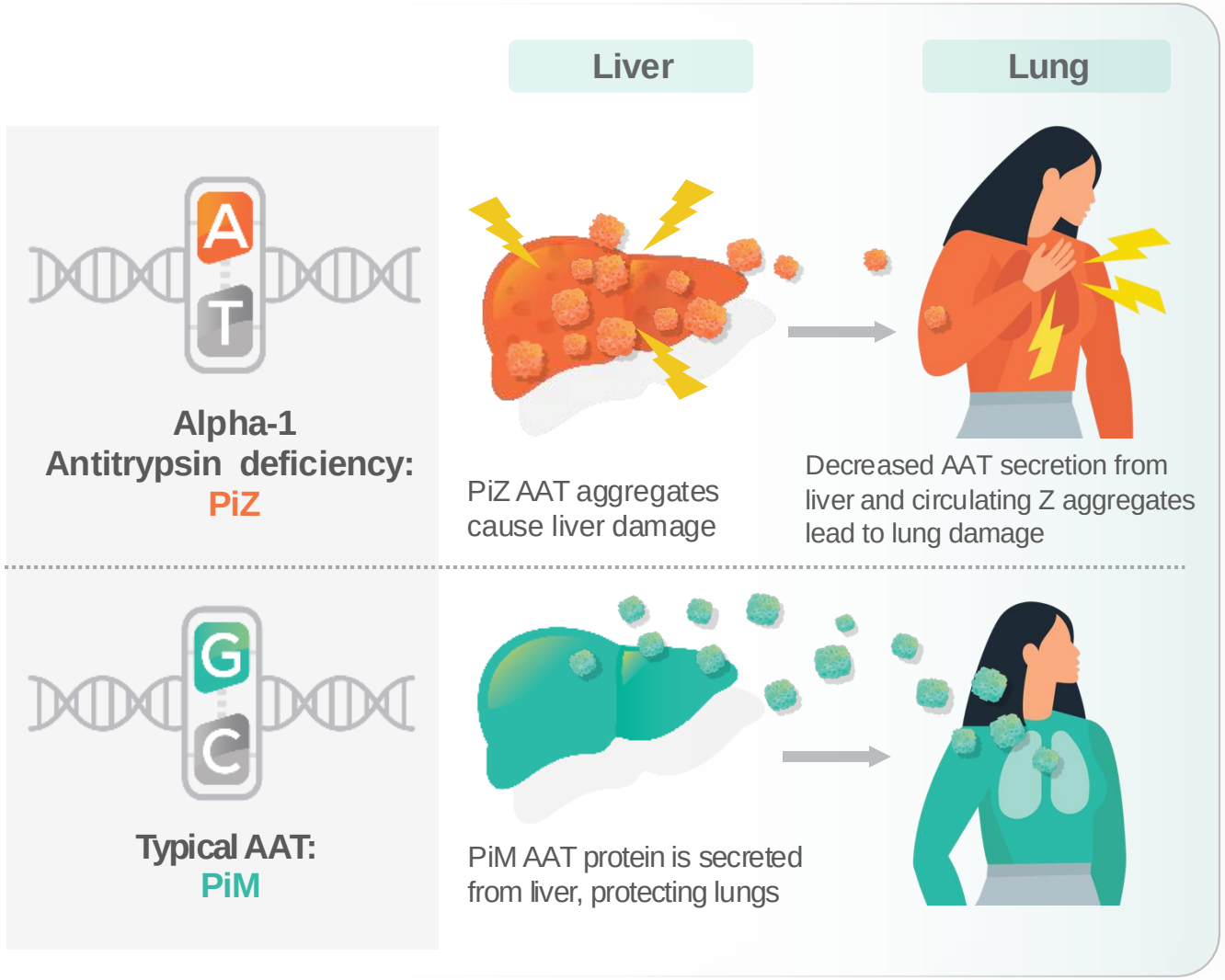
BEAM-302 aims to restore expression of functional AAT to address alpha-1-related lung and liver disease



Alpha-1 Antitrypsin Defiency (AATD)

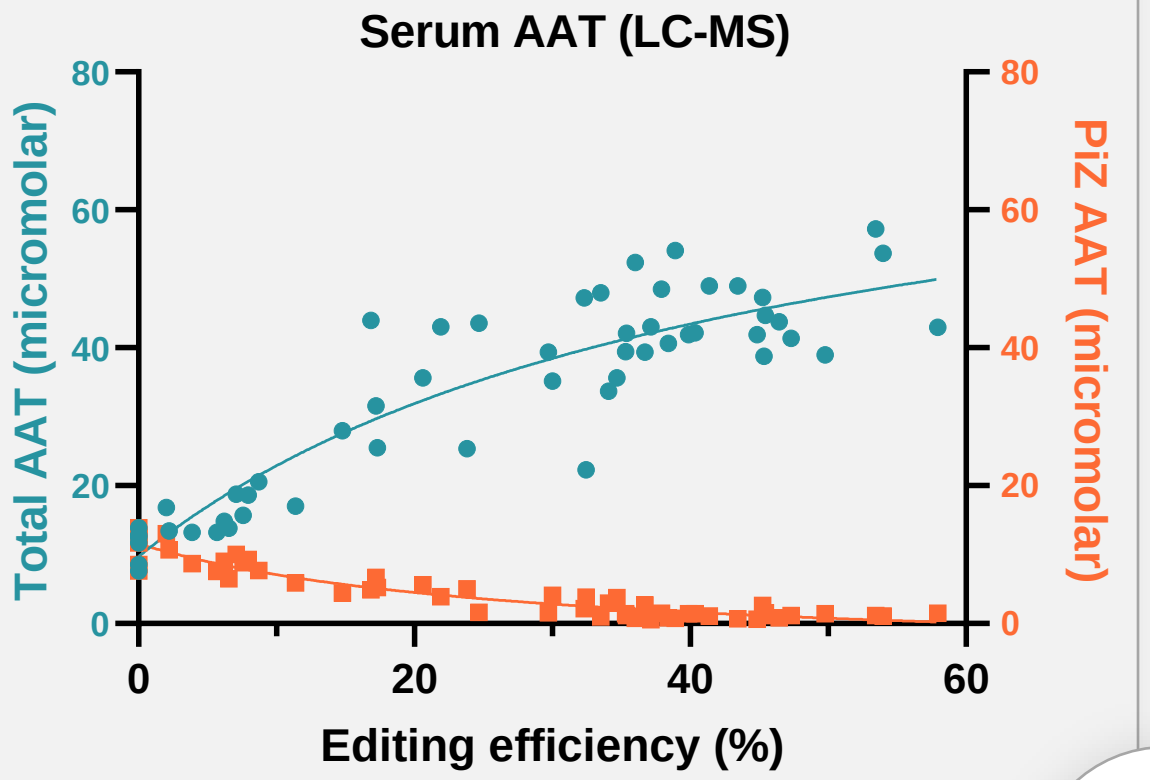
- PiZ is caused by a single G > A point mutation in the *SERPINA1* gene
- PiZ AAT is poorly secreted and less effective than normal PiM protein
- PiZZ genotype is >95% of severe AATD population, typically resulting in progressive lung and/or liver disease
- 100,000 PiZZ individuals in U.S.; ~10% diagnosed

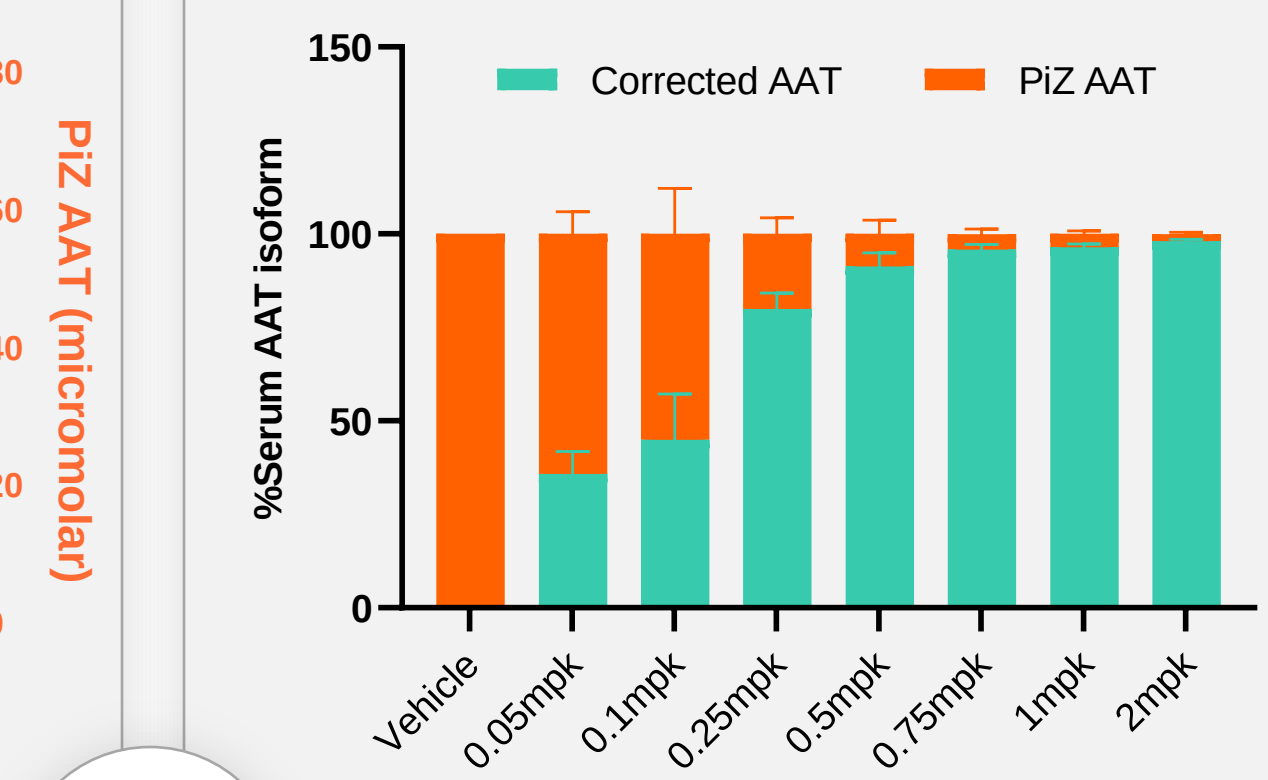
BEAM-302 Potential

- One-time therapy to address lung and liver disease, with gene under normal regulation
- Therapeutic levels of circulating PiM AAT
- Reduction of PiZ AAT in liver and blood

American Thoracic Society/European Respiratory Society statement: standards for the diagnosis and management of individuals with alpha-1 antitrypsin deficiency Am J Respir Crit Care Med, 2003

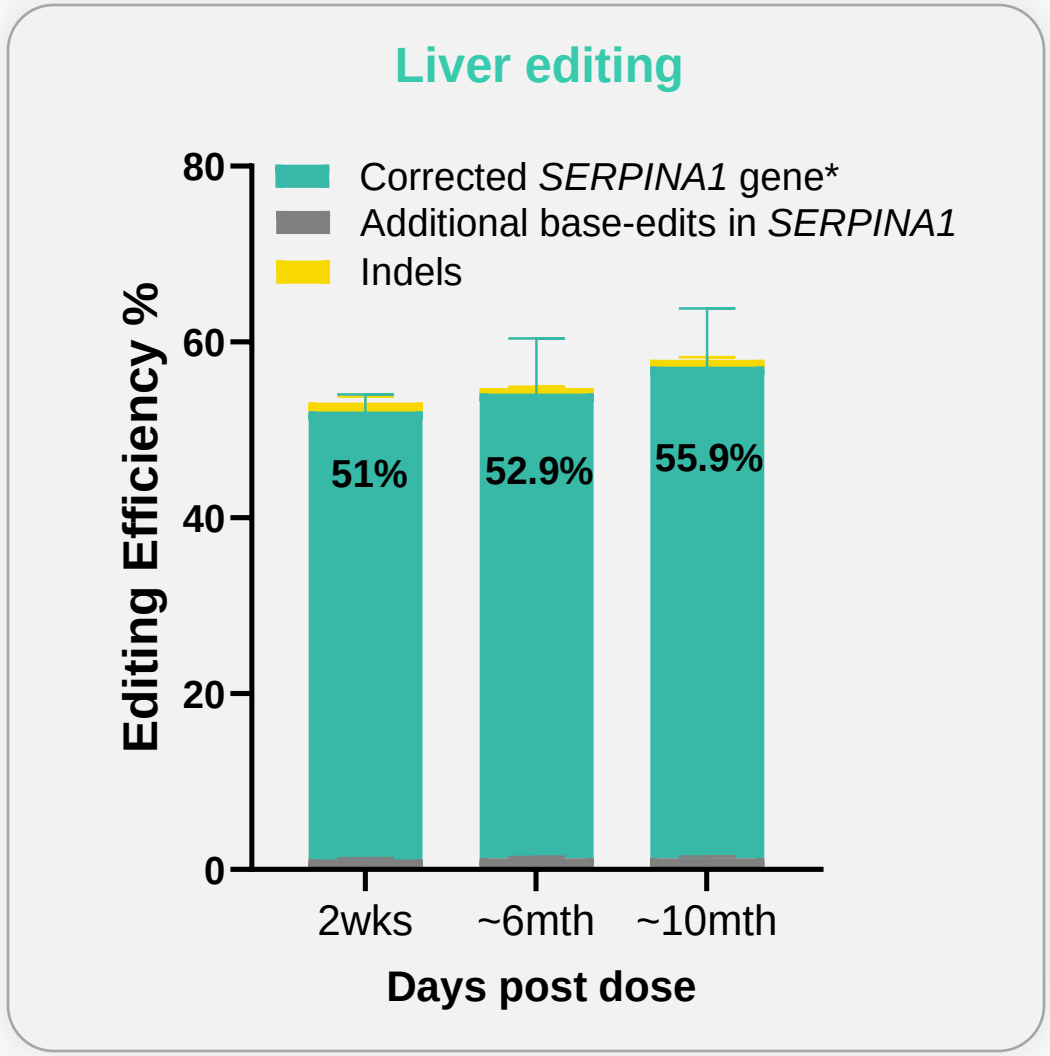
BEAM-302 resulted in both increased serum total & corrected AAT and decreased serum PiZ AAT *in vivo*





NSG-PiZ Mouse

Single dose of BEAM-302 led to durable correction of the PiZ variant in AATD mouse model



***SERPINA1* gene editing and AAT levels were either stable or increasing after a single dose**

- **Gene editing and AAT levels remained stable** in mice and rats over 8-10 months
- **Gene editing increased** in mice as of 3 months, suggesting potential improved survival of corrected liver cells

Long term studies were performed with precursor research grade reagents (1.5mpk)
Presented at Alpha-1 National Conference 2024

Phase 1/2 trial of BEAM-302 designed to achieve clinical proof-of-concept in patients across the spectrum of AATD

Part A: AATD-associated Lung Disease

Dose Exploration | Dose Expansion

- Up to 4 dose cohorts
- Patients excluded with liver disease

Part B: AATD-associated Lung and/or Liver Disease

Dose Exploration | Dose Expansion

- Up to 4 dose cohorts
- Patients included with mild to moderate liver disease

Assess early safety and efficacy and identify optimal dose for pivotal study

- Opportunity to achieve first ever clinical proof-of-concept of *in vivo* base editing leading to correction of a disease-causal mutation
- Global site activation and enrollment ongoing

Anticipate reporting initial clinical data for multiple cohorts from Phase 1/2 trial in 1H2025

BEAM-301 aims to normalize glycogen metabolism in GSD1a to prevent hypoglycemia and other disease manifestations

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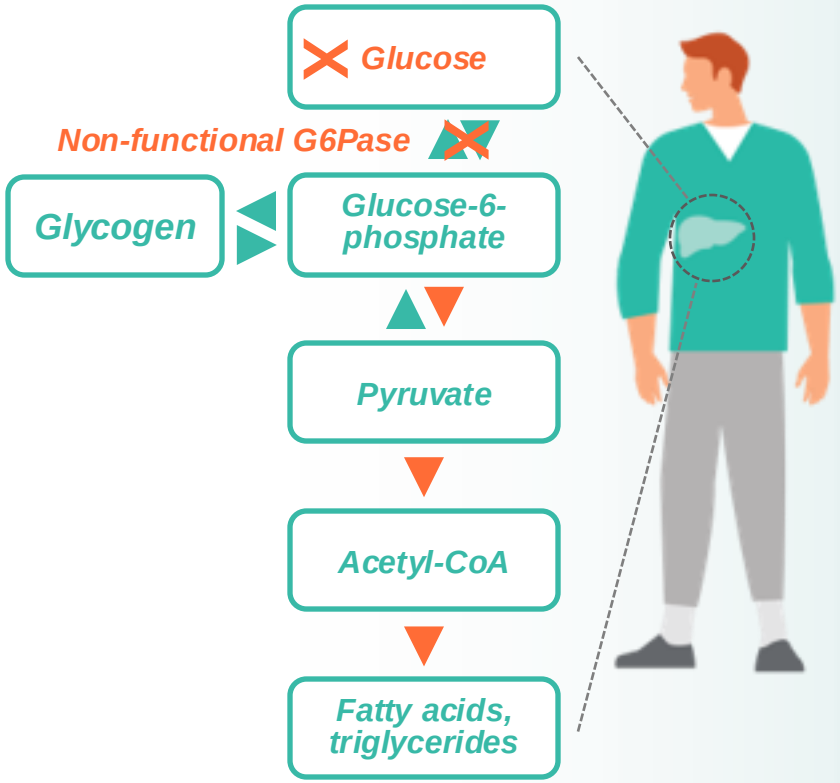
Liver

G6PC R83C mutation

Wildtype G6PC gene



GSD1a in Patients with R83C Mutations:

- Inability to convert glycogen back to glucose to sustain blood sugar while fasting
- Patients at constant risk of hypoglycemia that can result in seizures, coma or death
- Standard of care is cornstarch taken every 2-4 hours, even overnight
- Estimated ~300 R83C patients in U.S.

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

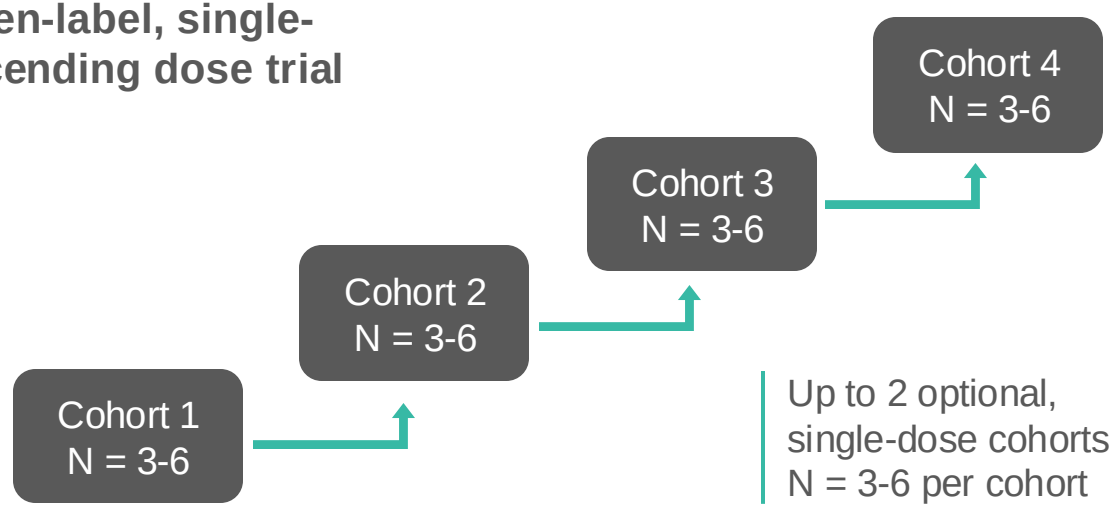
Chou & Mansfield. 2007. Curr. Gen. Ther.; Internal Beam estimates

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

- Beam will initially focus development of BEAM-301 in the U.S.
- U.S. IND application cleared in July 2024
- First clinical trial site activated

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

Patient dosing in the Phase 1/2 study expected to commence in early 2025

Key elements of Beam's leadership position in gene editing

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Patients are at the heart of our vision

Kyle
SICKLE CELL DISEASE

Dan and Kathi
ALPHA-1 ANTITRYPSIN DEFICIENCY

Alyssa and Gayle
GLYCOGEN STORAGE DISEASE 1A

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