

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 13, 2025

Beam Therapeutics Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-39208
(Commission File Number)

81-5238376
(IRS Employer
Identification No.)

238 Main Street
Cambridge, Massachusetts
(Address of Principal Executive Offices)

02142
(Zip Code)

Registrant's Telephone Number, Including Area Code: 857 327-8775

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.01 per share	BEAM	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 2.02 Results of Operations and Financial Condition.

Although it has not finalized its full financial results for the fourth quarter and fiscal year ended December 31, 2024, Beam Therapeutics Inc. (the “Company”) announced in a press release on January 13, 2025 that it estimates that it had cash, cash equivalents and marketable securities of approximately \$850.7 million as of December 31, 2024.

The information contained in this Item 2.02 regarding the Company’s estimated cash balance as of December 31, 2024 is preliminary, unaudited and is subject to completion of the Company’s financial statement closing procedures. This estimate also does not present all information necessary for an understanding of the Company’s financial condition as of December 31, 2024 and its results of operations for the three months and year ended December 31, 2024. Accordingly, undue reliance should not be placed on this preliminary estimate.

The information in this Item 2.02 is being furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended (the “Securities Act”) or the Exchange Act, except as expressly set forth by specific reference in such a filing.

Item 7.01 Regulation FD Disclosure.

On January 13, 2025, the Company updated its corporate presentation that it intends to use in connection with presentations at conferences and meetings, including an investor presentation at the 43rd Annual J.P. Morgan Healthcare Conference on January 13, 2025. The slides from the Company’s corporate presentation are furnished as Exhibit 99.1 to this Current Report on Form 8-K and are incorporated herein by reference.

The information in this Item 7.01 (including Exhibit 99.1 attached hereto) is being furnished and shall not be deemed “filed” for purposes of Section 18 of the Exchange Act or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

On January 13, 2025, the Company issued a press release announcing progress across its base editing portfolio and outlining key anticipated milestones. The full text of the press release is filed as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. The information contained on the website referenced in the press release is not incorporated herein.

Cautionary Note Regarding Forward-Looking Statements

Statements in this Current Report on Form 8-K about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute “forward-looking statements” within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements relating to: the Company’s upcoming presentations at the 43rd Annual J.P. Morgan Healthcare Conference; the therapeutic applications and potential of the Company’s technology, including with respect to SCD, AATD, GSD1a and beta thalassemia; the Company’s plans, and anticipated timing, to advance its programs; the clinical trial designs and expectations for BEAM-101, BEAM-103, BEAM-301 and BEAM-302; the Company’s estimated cash, cash equivalents and marketable securities as of December 31, 2024 and its expectations related thereto; the sufficiency of the Company’s capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; and the Company’s ability to develop life-long, curative, precision genetic medicines for patients through base editing. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. 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: the Company’s ability to develop, obtain regulatory approval for, and commercialize its product candidates, which may take longer or cost more than planned; the Company’s ability to raise additional funding, which may not be available; the Company’s ability to obtain, maintain and enforce patent and other intellectual property protection for its product candidates; the uncertainty that the Company’s product candidates will receive regulatory approval necessary to initiate or continue human clinical trials; that preclinical testing of the Company’s 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, and anticipated timing to advance, the Company’s clinical trials may take longer than expected; that the Company’s product candidates, including the delivery modalities it relies on to administer them, may cause serious adverse events; that the Company’s product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; whether the Company’s actual audited results will be consistent with its estimated cash, cash equivalents and marketable securities as of December 31, 2024; and the other risks and uncertainties identified under the headings “Risk Factors Summary” and “Risk Factors” in the Company’s Annual Report on Form 10-K for the year ended December 31, 2023,

the Company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this Current Report on Form 8-K. Factors or events that could cause the Company's actual results to differ may emerge from time to time, and it is not possible for the Company to predict all of them. The Company undertakes 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.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

<u>Exhibit</u>	<u>Description</u>
99.1	Beam Therapeutics Inc. Corporate Presentation
99.2	Press Release dated January 13, 2025
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

BEAM THERAPEUTICS INC.

Date: January 13, 2025

By: /s/ John Evans
John Evans
Chief Executive Officer



PRECISION GENETIC MEDICINES THROUGH BASE EDITING

NASDAQ: BEAM

January 2025

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.

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; whether our actual audited results will be consistent with our estimated cash, cash equivalents and marketable securities as of December 31, 2024; 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, 2023, 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 LIFE-LONG CURES for patients suffering from serious diseases



POTENTIAL FOR
one-time, curative
therapies



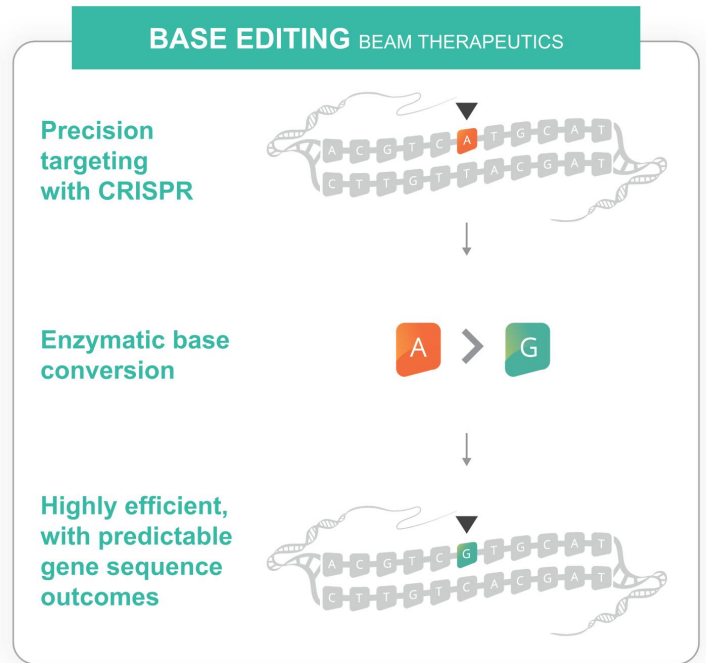
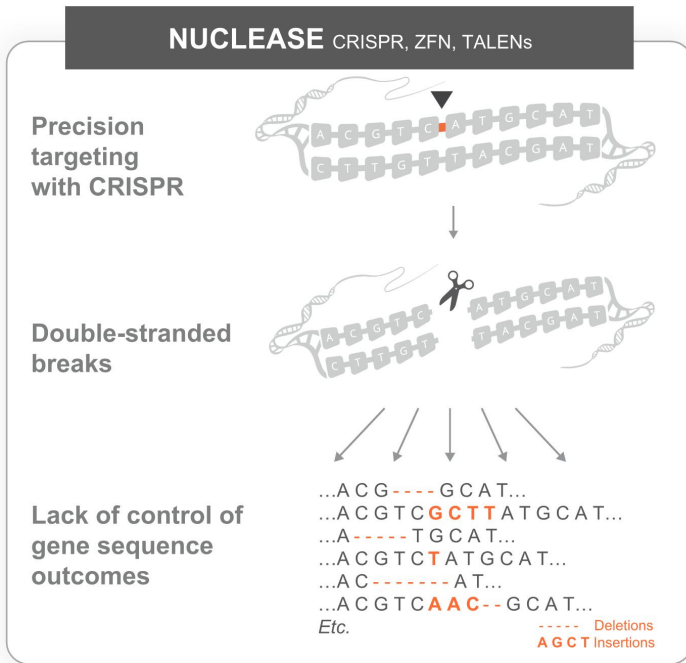
GENE EDITING FOR
rare and common
diseases



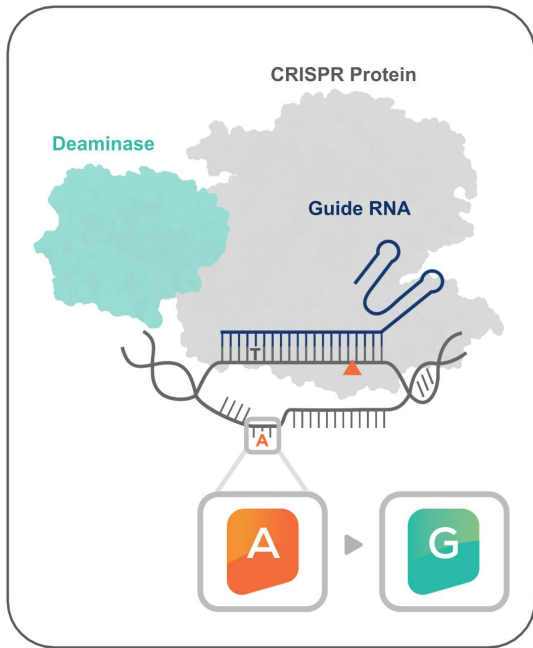
PLATFORM FOR
rapidly programmable
precision medicines



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



Base editing technology has multiple, highly versatile applications



		PROGRAMS
Correct mutations	Repairs the most common type of gene mutation, single base changes	BEAM-302, BEAM-301
Silence proteins	Turns off gene with disease-causing activity	Multiple at Beam and partners
Activate expression	Turns on genes to restore or increase function	BEAM-101
Modify proteins	Changes how proteins bind or signal without disrupting their function	ESCAPE
Multiplex edits	Targets multiple pathways simultaneously with high efficiency	BEAM-201, ESCAPE

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.



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



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

A graphic with a blue-to-green gradient background. At the top, there are two yellow right-pointing chevrons. Below them, the text "RAPID EXECUTION Across Clinical Programs" is written in white, with "RAPID EXECUTION" in a larger, bold font and "Across Clinical Programs" in a smaller font below it.

**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



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

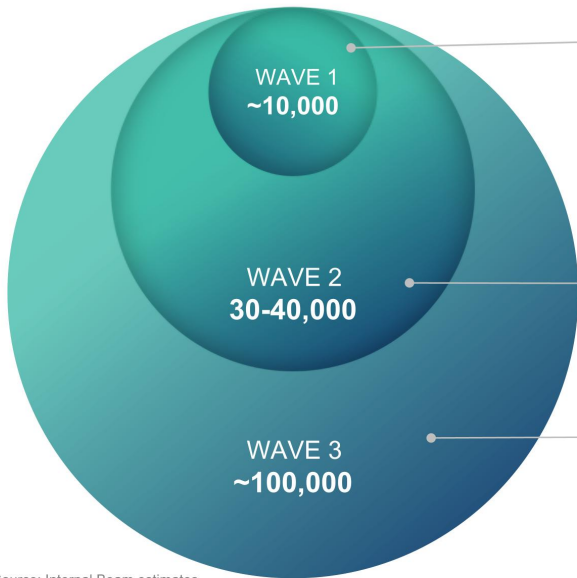
What if we could develop better one-time therapies for patients with SCD?

SICKLE CELL DISEASE



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

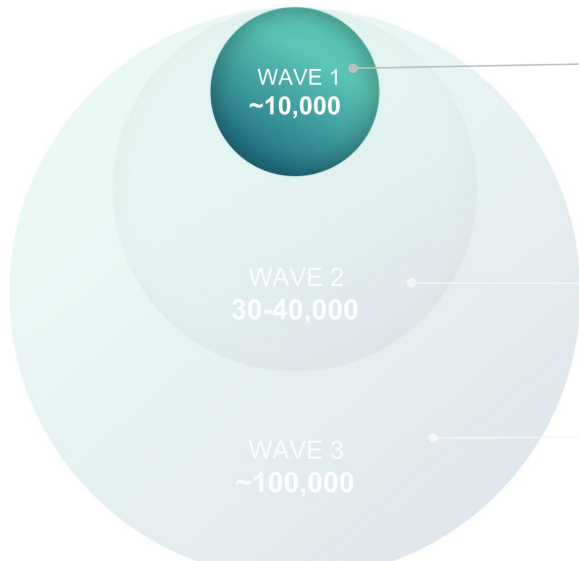
Designed to eliminate chemotherapy from *ex vivo* gene therapy and expand patient population

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

Potential Eligible SCD Patient Population (U.S.)



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

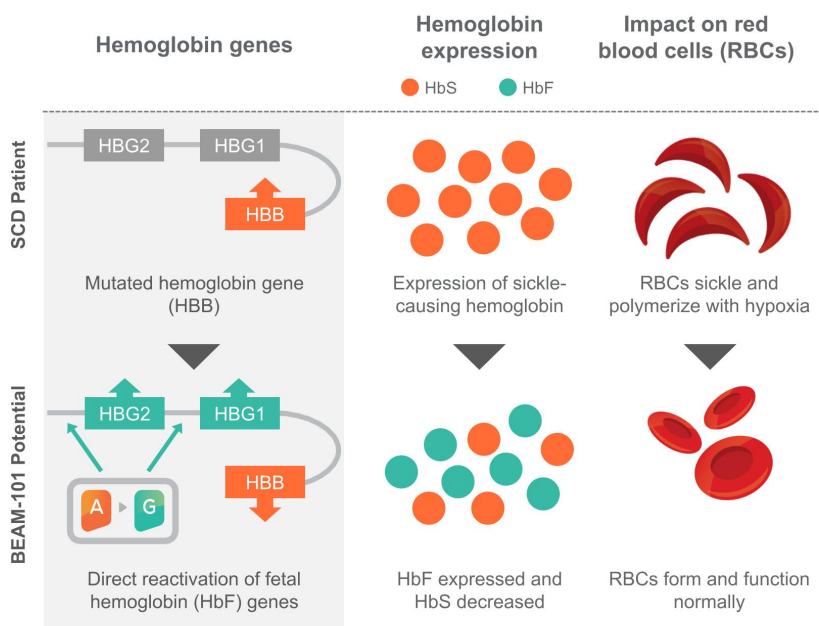
- Current demand for matched allo transplants in SCD implies at least an annual \$1.5B gene therapy market* in U.S.
- Market research suggests peak potential of 1,000-1,200 patients/year
- Market infrastructure for SCD gene therapy is building momentum

“ The demand for gene therapy in SCD will be dictated by the industry’s ability to supply. ”

– High-volume U.S. transplant 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



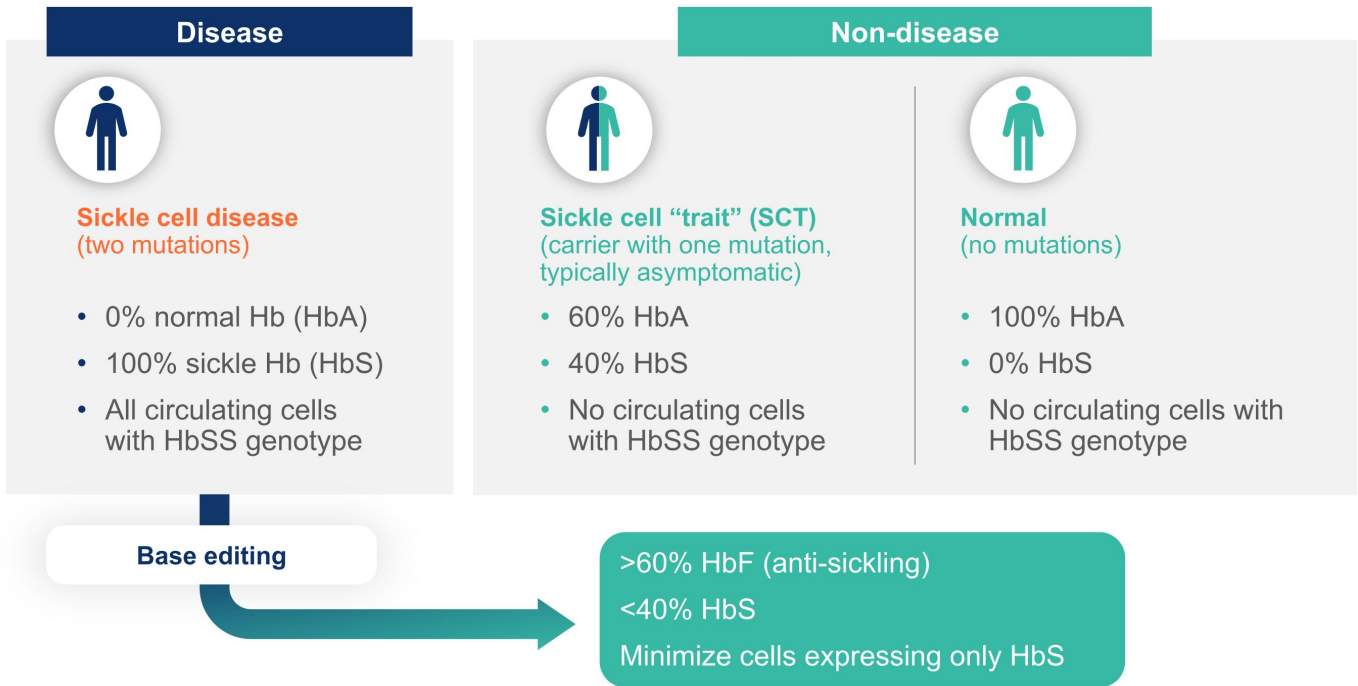
SCD Unmet Need

- 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

What would an ideal outcome for BEAM-101 look like?



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

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

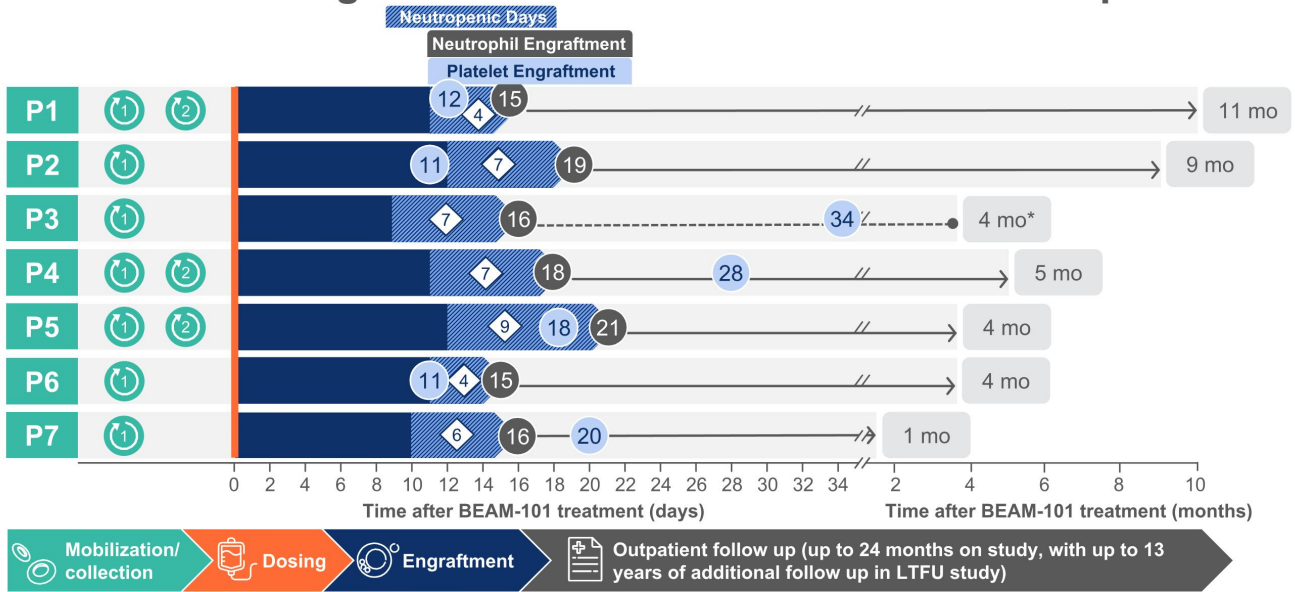
→ Initial potent HbF induction and HbS reduction in all patients: >60% HbF, <40% HbS

- 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

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

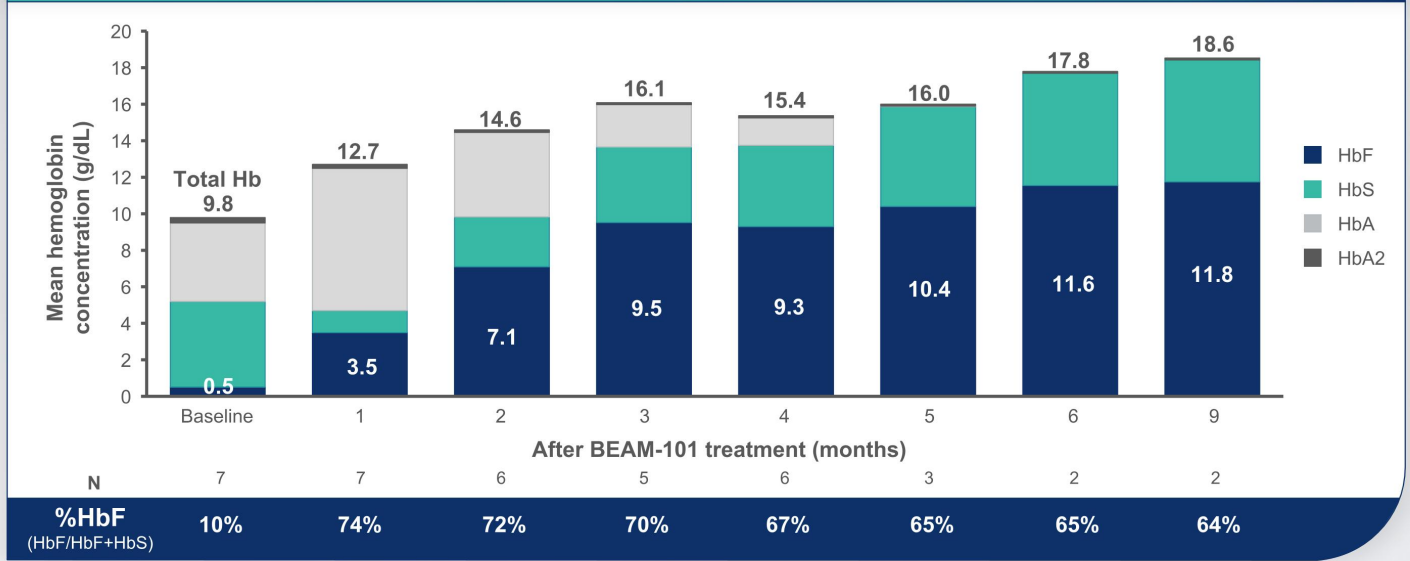
Patients remain as inpatients until neutrophil engraftment has occurred, and the patient is deemed clinically stable for discharge and outpatient management. Patients assessed at daily intervals to evaluate engraftment success status. Patients will be discharged home after neutrophil engraftment. Platelet 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



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

BEAM-101 initial safety data are consistent with busulfan conditioning and autologous HSCT



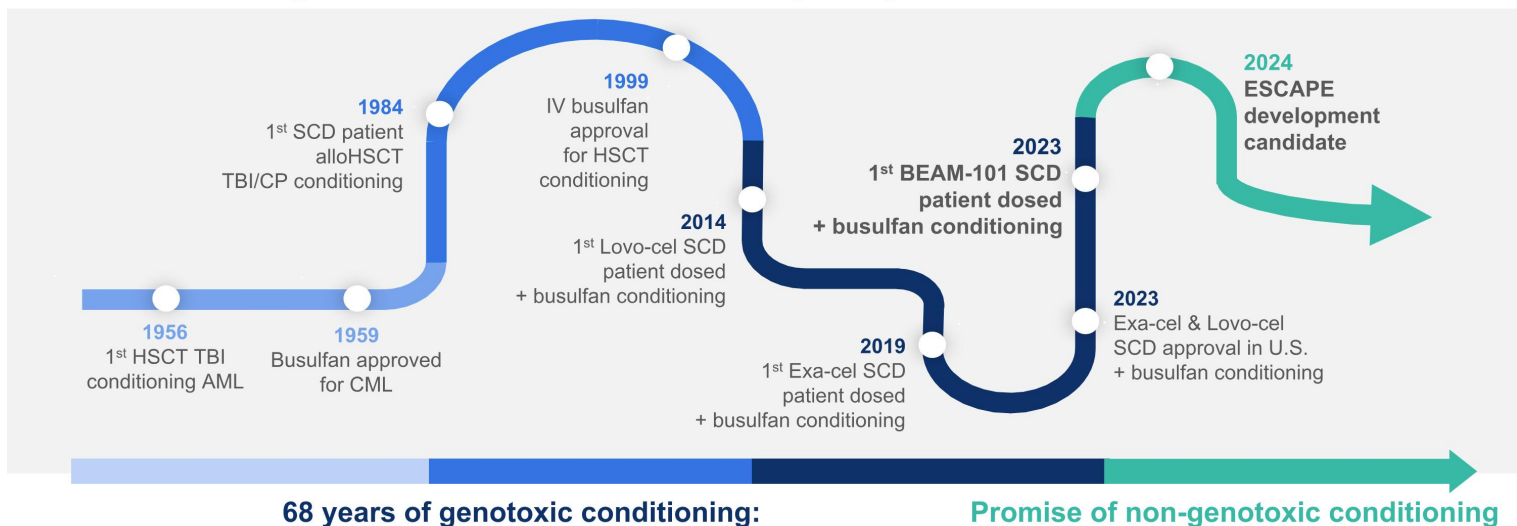
Patients with, n (%)	N=7
Any TEAEs	7 (100)
Related to BEAM-101	1 (14.3)
Any TEAEs ≥Grade 3	7 (100)
Related to BEAM-101	0
AEs leading to discontinuation	0
Serious TEAEs	4 (57.0)
Related to BEAM-101	0
Death	1
Related to BEAM-101	0

- ▶ Most common TEAEs (≥3 patients) included febrile neutropenia*, stomatitis*, skin hyperpigmentation, pharyngeal inflammation, anemia*, edema peripheral, decreased appetite*, headache, hypervolemia, hypokalemia
- ▶ All but 1 non-serious TEAE (Grade 1 dizziness) were assessed as not related to BEAM-101
- ▶ No serious TEAEs occurred in >1 patient

No patients have experienced any VOCs post-engraftment

Data cutoff Oct 28, 2024; Presented at ASH 2024
Related events include events where investigator has assessed relationship as possibly or definitely related to BEAM-101
*Includes events that were ≥Grade 3 in at least 3 patients
AE, adverse event; HSCT, hematopoietic stem-cell transplantation; TEAE, treatment-emergent adverse event

ESCAPE technology designed to bring a paradigm shift to conditioning for the first time in nearly 70 years



68 years of genotoxic conditioning:

- Infertility
- Secondary malignancy
- Organ toxicities
- Infection complications
- Inpatient

Promise of non-genotoxic conditioning

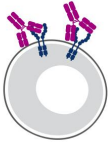
- Prevent acute and chronic toxicities
- Preserve fertility
- Potential to be outpatient

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

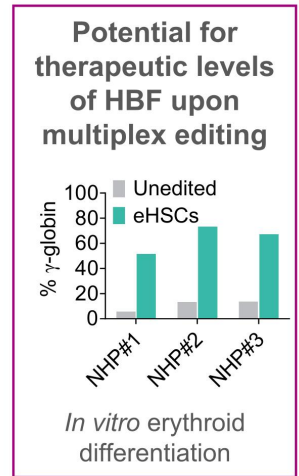
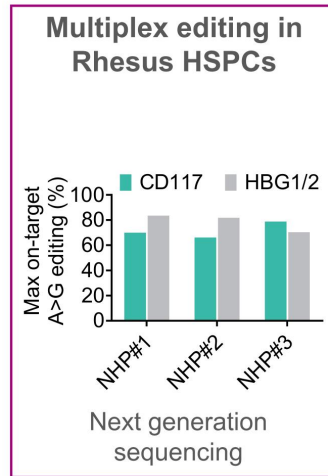
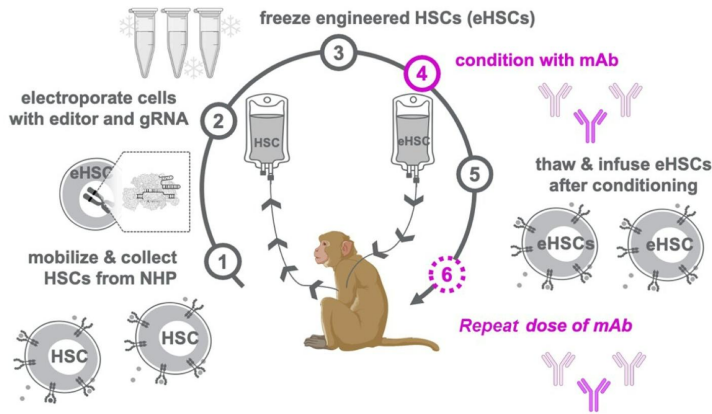
Engineered CD34 cell product with a therapeutic edit (same as in BEAM-101) plus an edit to CD117 to prevent binding of BEAM-103



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

NHP autologous transplant model for our ESCAPE conditioning approach

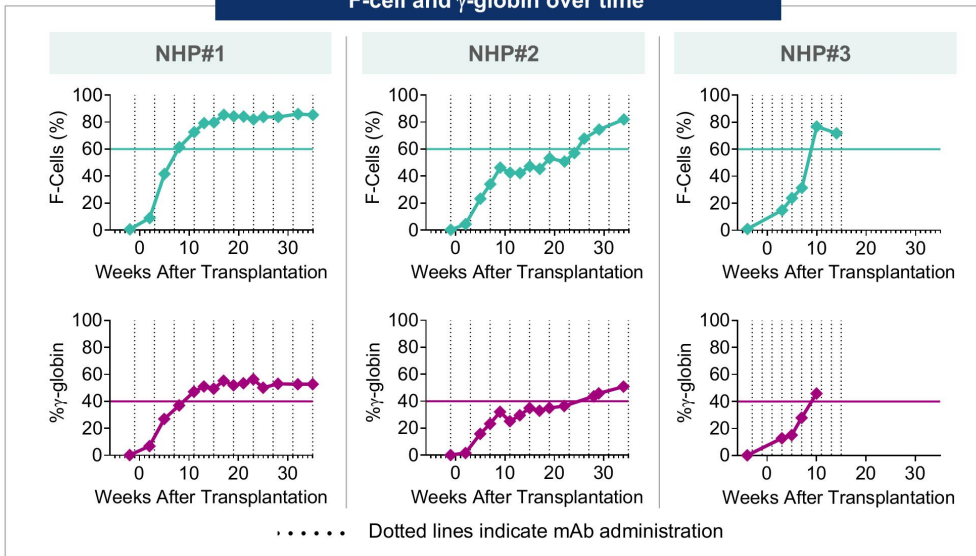
Multiplex base-editing and erythroid differentiation of Rhesus CD34+ cells



Infusion product was manufactured with priority for maximizing total CD34+ cell dose for transplant

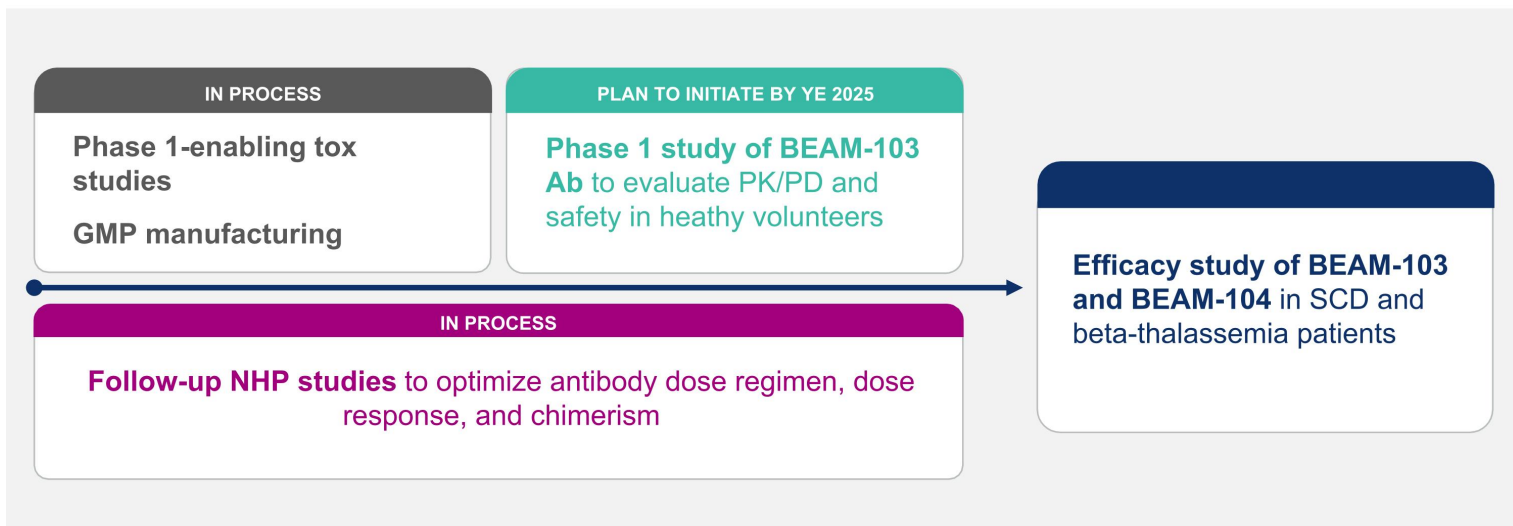
Preclinical proof-of-concept for ESCAPE antibody conditioning and engraftment in NHPs without chemotherapy

F-cell and γ -globin over time

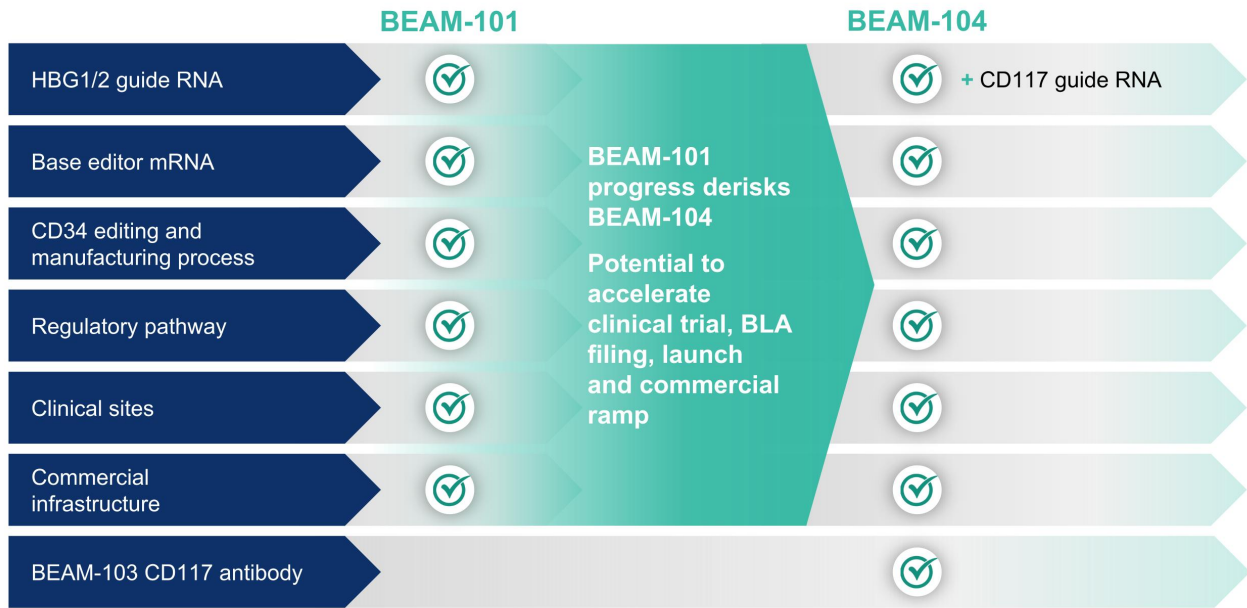


- Rapid and complete replacement of erythroid cells by edited cells
- F-cell levels reached ~70-80% post-transplant
- γ -globin (proxy for HbF) reached ~50% in all animals
- mAb dosing was well tolerated with no use of transfusions/antibiotic support

Anticipated next steps and pathway to patients for ESCAPE



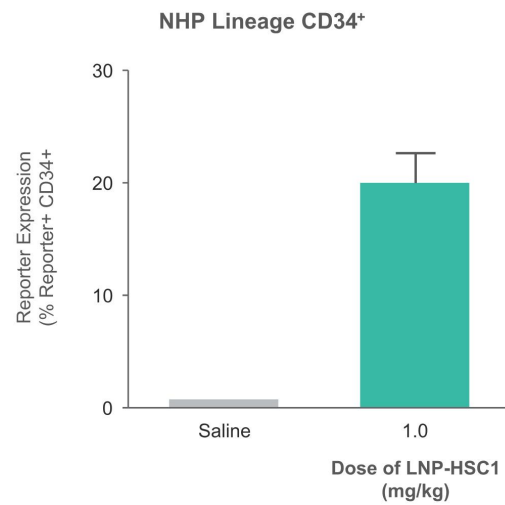
Synergy between BEAM-101 and ESCAPE technology (BEAM-104 and BEAM-103) support efficient development in SCD



Wave 3 *in vivo*: Developing LNPs for delivery of base editors to blood stem cells

- In preclinical studies, Beam LNP technology allowed targeting of blood stem cells for delivery of mRNA payloads at clinically relevant doses
- Research to adapt system to base editing payloads is ongoing
- **Ultimate goal:** deliver curative base editing machinery directly to HSCs with an intravenous transfusion

Expression of mRNA payload in NHP HSCs at clinically relevant doses

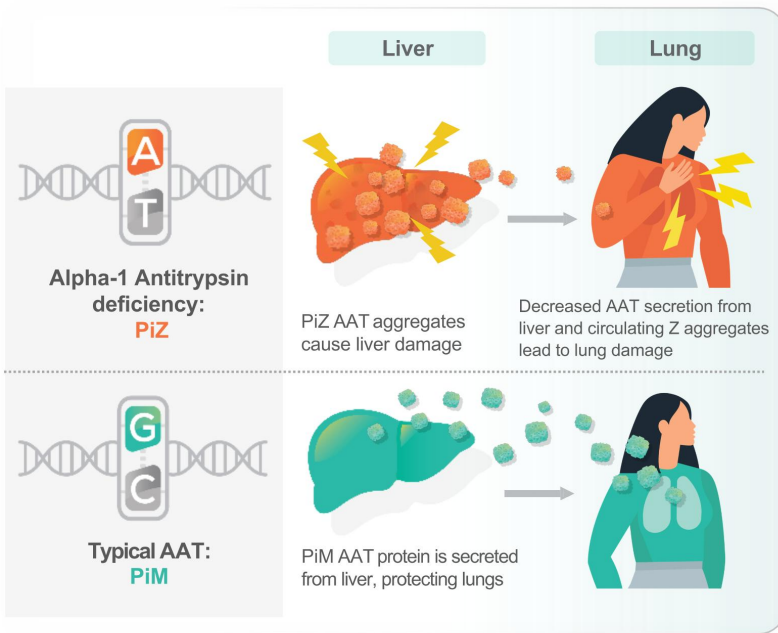


What if we could use base editing to correct disease-causing mutations *in vivo*?

GENETIC DISEASES



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



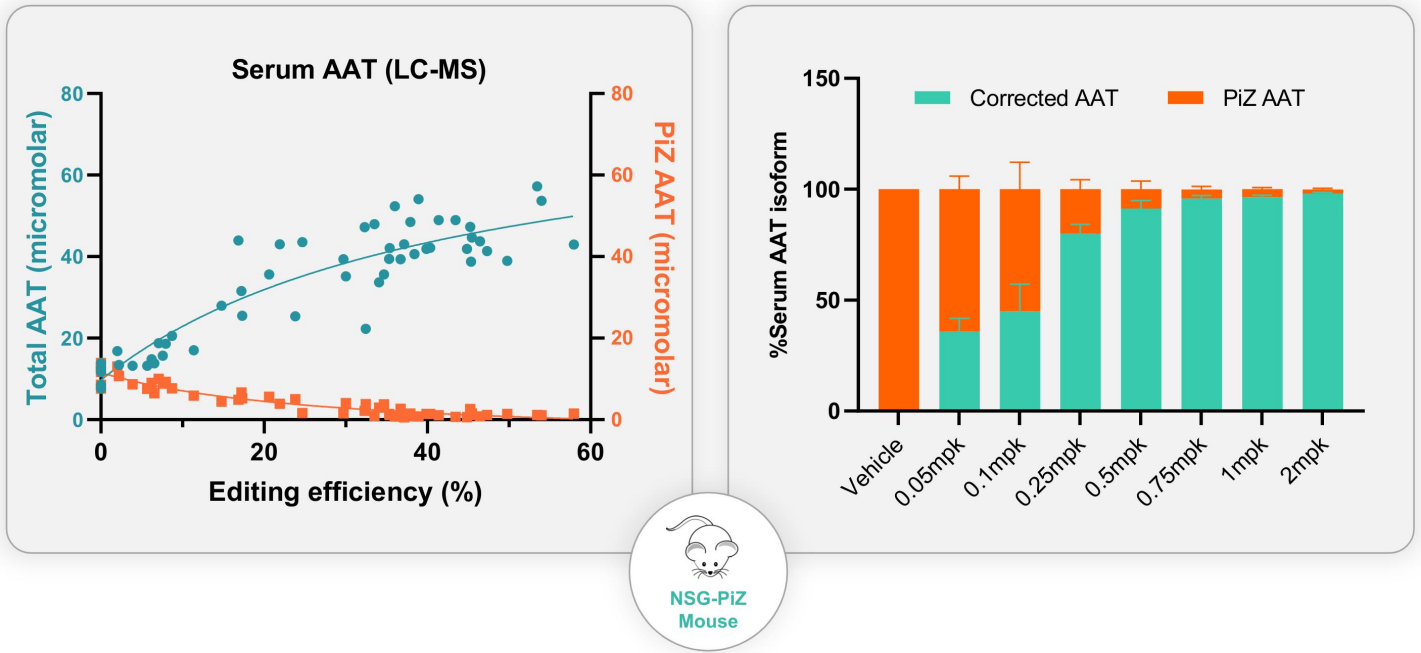
Alpha-1 Antitrypsin Deficiency (AATD)

- 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

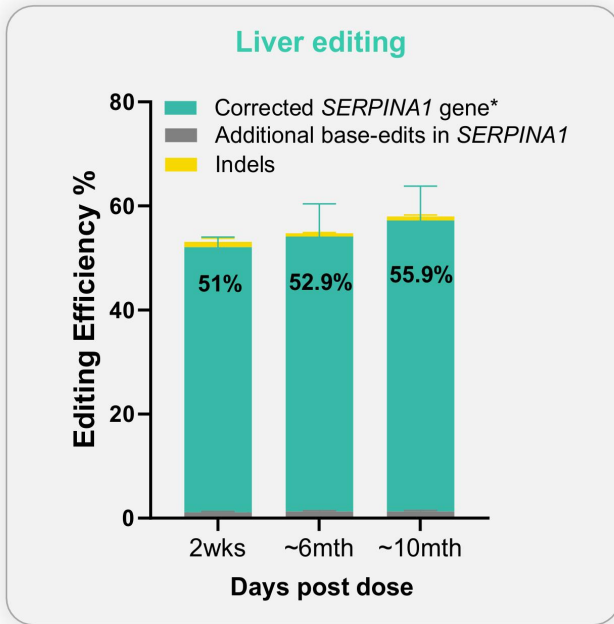
BEAM-302 Potential

- 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

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



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

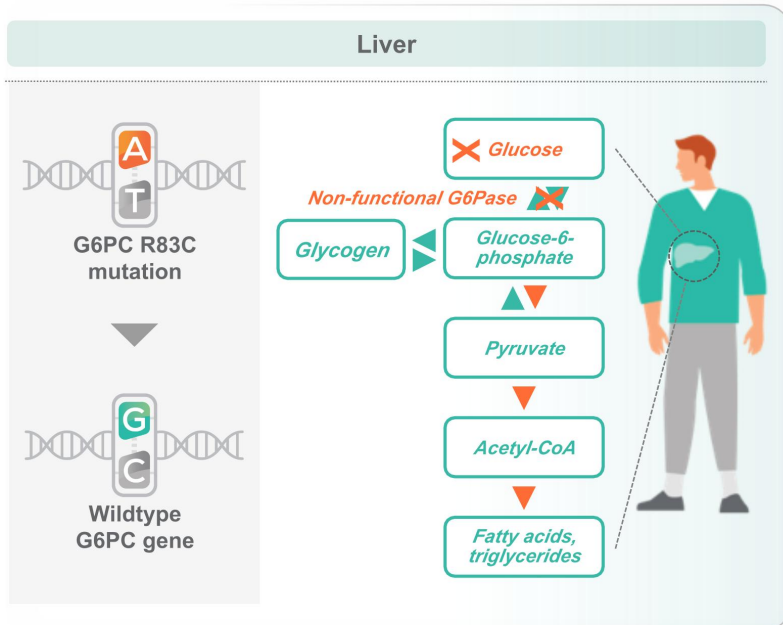
- Up to 4 dose cohorts
- Patients included with mild to moderate liver disease

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



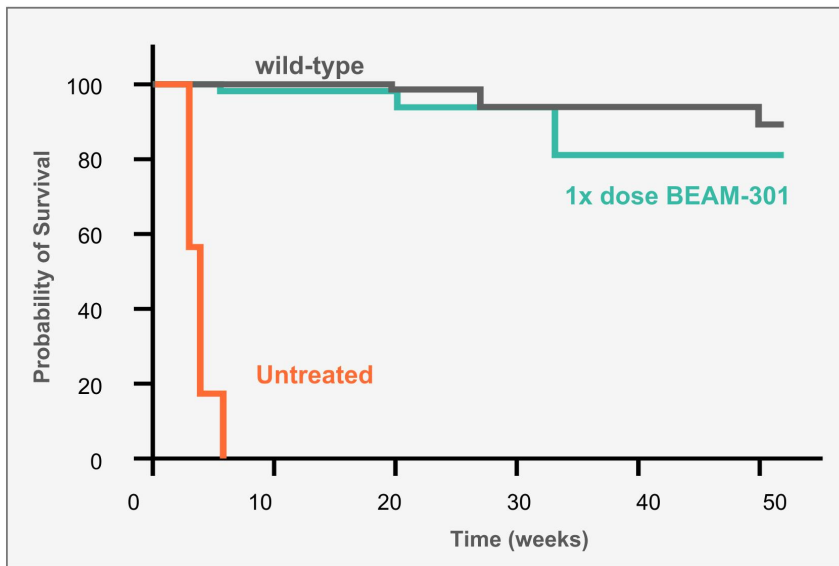
GSD1a in Patients with R83C Mutations:

- 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

BEAM-301: Treatment with a single dose significantly improved long-term survival in GSD1a mouse model

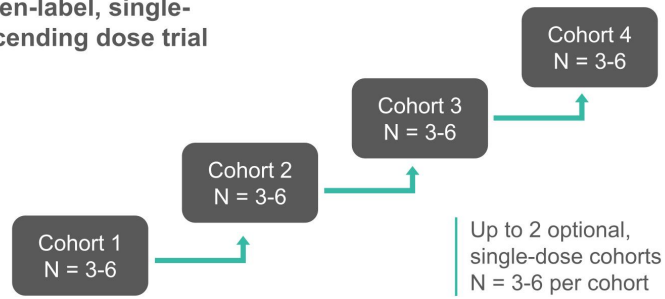


- Preclinical studies of BEAM-301 demonstrated a single dose **significantly improved long-term survival out to a year** in humanized R83C homozygous mice
- Untreated homozygous R83C mice die within weeks of birth

BEAM-301 Phase 1/2 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

Creative pipeline and platform partnerships unlock additional value and broaden therapeutic impact

Strategic Deals

resulting in \$675M upfront and more than \$1B in potential milestones



- \$300M upfront for 3 base editing targets
- Beam option at end of Phase 1/2 for 35% WW cost/profit split on 1 program



- \$250M in upfront/equity plus up to \$350M in potential development-stage payments to acquire Beam's cost/profit split options in 3 Verve cardiovascular programs



- \$75M upfront for base editing for complement-mediated diseases
- Beam option at end of Phase 1 for 50% of U.S. rights on one program



- \$50M upfront for non-exclusive license to Cas12b nuclease for certain engineered cell therapies

Innovator Deals

gaining rights to innovative and complementary technologies



- Prime editing (PE) technology is complementary to base editing
- Beam exclusive PE rights for all A-G and C-T edits plus any edit for SCD



- Next-gen RNA and delivery technologies
- Beam equity stake in Orbital plus IP access in gene editing and other fields

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*

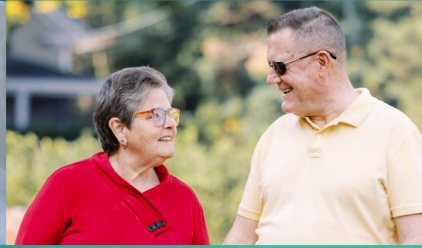
expected to fund operations, including anticipated commercial readiness activities for BEAM-101, into 2027

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

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



Beam Therapeutics Announces Progress in Hematology and Genetic Disease Franchises and Outlines Key 2025 Anticipated Catalysts

More Than 40 Adult Sickle Cell Disease Patients Now Enrolled in BEACON Trial of BEAM-101; Beam Expects to Dose 30 Patients and Present Updated Data by Mid-2025

Initial Data from Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency Expected in First Half 2025

Dosing Anticipated to Commence in Phase 1/2 Trial of BEAM-301 in Glycogen Storage Disease Type 1a in Early 2025

IND-enabling Studies of ESCAPE Nongenotoxic Conditioning Approach Underway, with Healthy Volunteer Study of BEAM-103 Antibody Expected to Initiate by Year-end

Cash Runway Expected to Support Operating Plans into 2027, Now Inclusive of Commercial Readiness Activities for BEAM-101

Cambridge, Mass., January 13, 2025 – Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced progress across the company’s hematology and genetic disease franchises and provided updates on anticipated upcoming milestones.

“We’re entering 2025 at an important inflection point in the evolution of Beam, having advanced four programs into the clinic, established clinical differentiation for base editing with our lead sickle cell disease program, and prioritized two high-value core franchises with best-in-class potential – all of the key attributes needed to create a long-term leading company in gene editing,” said John Evans, chief executive officer of Beam.

“Importantly, we remain in a strong financial position, with our core manufacturing, regulatory and clinical capabilities now in place. This year, we are poised to deliver critical data and achieve key milestones across our pipeline, which we expect will bring us closer to our mission of offering life-long cures for patients in need.”

Pipeline Updates and 2025 Anticipated Milestones

Hematology Franchise

Beam is pursuing a long-term, staged development strategy for sickle cell disease (SCD) that includes three “waves” of innovation intended to progressively expand the reach of the company’s base editing approach to broader subsets of patients.

BEAM-101: Wave 1 gene editing treatments aim to deliver a genetically modified cell product through stem cell transplant, enabled by chemotherapy conditioning, for the most severe SCD patients. Beam’s wave 1 approach is BEAM-101, an autologous investigational cell therapy designed to efficiently and uniformly increase fetal hemoglobin (HbF) in red blood cells without

relying on double-stranded breaks, offering a potentially best-in-class profile. BEAM-101 is being evaluated in the BEACON Phase 1/2 clinical trial, and initial results were presented at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2024.

- To date, more than 40 adult patients with SCD have cleared screening and enrolled in BEACON, and, of these, 13 have been dosed. Beam expects to dose 30 patients by mid-2025.
- The U.S. Food and Drug Administration (FDA) and the BEACON data monitoring committee approved enrollment of adolescent SCD patients ages 12-17 years in the study, and screening has commenced.
- Beam expects to present updated data from the BEACON trial in mid-2025.

ESCAPE: Beam's wave 2 approach is its Engineered Stem Cell Antibody Evasion (ESCAPE) platform, which aims to provide the same *ex vivo*-manufactured cell product deployed in wave 1, but now using a non-genotoxic alternative to traditional transplant myeloablative conditioning. Proof-of-concept data in non-human primates (NHPs) demonstrating engraftment of base-edited cells using antibody conditioning were presented at ASH. Beam plans to develop the ESCAPE technology initially in SCD and beta-thalassemia as well as potential future hematology indications.

- In December, Beam initiated Phase 1-enabling preclinical toxicology studies for ESCAPE.
- The company expects to initiate a Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody (mAb) designed to suppress hematopoietic stem and progenitor cells that express CD117, by the end of 2025.

In vivo: In wave 3, Beam is exploring the potential for *in vivo* base editing programs for SCD, in which base editors would be delivered to the patient through intravenous infusion of lipid nanoparticles (LNPs) targeted to hematopoietic stem cells, eliminating the need for transplantation altogether.

Genetic Disease Franchise

Beam's second core area of focus seeks to create single-course gene editing therapies for genetic diseases by delivering base editors through intravenous infusion of LNPs, which are a clinically validated technology for delivery of nucleic acid payloads to the liver.

BEAM-302: Beam's lead genetic disease program is BEAM-302, a potentially best-in-class liver-targeting LNP formulation of base editing reagents designed to correct the PiZ allele, the most common gene variant associated with severe alpha-1 antitrypsin deficiency (AATD). BEAM-302 has the potential to simultaneously reduce the aggregation of mutant, misfolded AAT protein that causes toxicity to the liver and increase circulating levels of corrected and functional AAT protein, thus addressing the underlying pathophysiology of both the liver and lung disease. BEAM-302 is being evaluated in a Phase 1/2 dose-escalation clinical trial.

- The company continues to advance global regulatory and site activation activities with sites now open in the United Kingdom, New Zealand, Australia and Netherlands.
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- Beam expects to report initial data from multiple cohorts from the Phase 1/2 study in the first half of 2025.

BEAM-301: BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation that results in the most severe form of glycogen storage disease type 1a (GSD1a). GSD1a is an autosomal recessive disorder caused by mutations involved in maintaining glucose homeostasis and is associated with life-threatening fasting hypoglycemia as well as long-term complications impacting the liver and kidney. BEAM-301 has the potential to normalize blood glucose without continuous supplementation and improve metabolic parameters. BEAM-301 is being evaluated in a Phase 1/2 dose-escalation clinical trial.

- The first clinical trial site for the Phase 1/2 clinical trial of BEAM-301 is now active, with patient dosing expected to commence in early 2025.

Partnered Programs: Beam continues to progress its research collaborations with Pfizer and Apellis. Under the Apellis collaboration, which is focused on multiple base editing programs that target specific genes within the complement system, the companies are advancing preclinical studies for a one-time treatment targeting the neonatal Fc receptor (FcRn) using gene editing technology from Beam.

Cash Position and Updated Operating Runway

As of December 31, 2024, Beam estimates that it had \$850.7 million in cash, cash equivalents and marketable securities. This estimate is preliminary, unaudited and is subject to completion of Beam's financial statement closing procedures. This estimate also does not present all information necessary for an understanding of Beam's financial condition as of December 31, 2024, and its results of operations for the three months and year ended December 31, 2024. Accordingly, undue reliance should not be placed on this preliminary estimate.

Beam now expects that its estimated cash, cash equivalents and marketable securities as of December 31, 2024, will enable the company to fund its anticipated operating expenses and capital expenditure requirements into 2027, inclusive of commercial spend related to the potential launch of BEAM-101.

J.P. Morgan Healthcare Conference

Beam management will present and discuss Beam's pipeline and business updates during a presentation at the 43rd Annual J.P. Morgan Healthcare Conference today, Monday, January 13, 2025, at 1:30 p.m. PT. A live webcast will be available in the investor section of the company's website at www.beamtx.com and will be archived for 60 days following the presentation.

About Beam Therapeutics

Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. To achieve this vision, Beam has assembled a platform with integrated gene editing, delivery and internal manufacturing capabilities. Beam's suite of gene editing technologies is anchored by base editing, a proprietary technology that is designed to enable precise, predictable and efficient single base changes, at

targeted genomic sequences, without making double-stranded breaks in the DNA. This has the potential to enable a wide range of potential therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is a values-driven organization committed to its people, cutting-edge science, and a vision of providing life-long cures to patients suffering from serious diseases.

Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Investors are cautioned not to place undue reliance on these forward-looking statements, including, but not limited to, statements related to: our upcoming presentations at the 43rd Annual J.P. Morgan Healthcare Conference; the therapeutic applications and potential of our technology, including with respect to SCD, AATD, GSD1a and beta thalassemia; our plans, and anticipated timing, to advance our programs; the clinical trial designs and expectations for BEAM-101, BEAM-103, BEAM-301 and BEAM-302; 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 our ability to develop life-long, curative, precision genetic medicines for patients through base editing. 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; the uncertainty that our product candidates will receive regulatory approval necessary to initiate or continue human clinical trials; 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, and anticipated timing to advance, our clinical trials may take longer than expected; that our product candidates, including the delivery modalities we rely on to administer them, may cause serious adverse events; that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; whether our actual audited results will be consistent with our estimated cash, cash equivalents and marketable securities as of December 31, 2024; and the other risks and uncertainties identified under the headings “Risk Factors Summary” and “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2023, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release. 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.

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