

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 08, 2024

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, 2023, Beam Therapeutics Inc. (the “Company”) announced in a press release on January 8, 2024 that it estimates that it had cash, cash equivalents and marketable securities of approximately \$1.2 billion as of December 31, 2023.

The information contained in this Item 2.02 regarding the Company’s estimated cash balance as of December 31, 2023 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, 2023 and its results of operations for the three months and year ended December 31, 2023. 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 8, 2024, 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 42nd Annual J.P. Morgan Healthcare Conference on January 8, 2024. 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 8, 2024, 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 42nd Annual J.P. Morgan Healthcare Conference; the Company’s expectations for transitioning to a multi-program clinical stage company; the therapeutic applications and potential of the Company’s technology, including with respect to sickle cell disease, glycogen storage disease 1a, relapsed/refractory T-cell acute lymphoblastic leukemia/T-cell lymphoblastic lymphoma, alpha-1 antitrypsin deficiency, and the Company’s conditioning regimens; the Company’s plans, and anticipated timing, to advance its programs, the clinical trial designs and expectations for BEAM-101, BEAM-201, BEAM-301 and BEAM-302; the Company’s estimated cash, cash equivalents and marketable securities as of December 31, 2023 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 successfully achieve the benefits of its portfolio prioritization and strategic restructuring; 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 potential impact of pandemics and other health emergencies, including their impact on the global supply chain; the uncertainty that the Company’s product candidates will receive regulatory approval necessary to initiate human clinical studies; 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 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, 2023; and the other risks and uncertainties identified under the headings "Risk Factors" and "Risk Factors Summary" in the Company's Annual Report on Form 10-K for the year ended December 31, 2022, its Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, its Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, its Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, 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

Exhibit No.	Description
99.1	Beam Therapeutics Inc. Corporate Presentation
99.2	Press Release dated January 8, 2024
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 8, 2024

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



PRECISION GENETIC MEDICINES THROUGH BASE EDITING

JANUARY 2024 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 and progress of clinical trials, including our BEACON trial and our BEAM-201 trial; the advancement of our pipeline, including the advancement of BEAM-101, BEAM-201, BEAM-301, BEAM-302, and additional liver programs in multiple preclinical studies; our current expectations and anticipated results of operations, including our expected use of capital; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; the potential activities and benefits under license and collaboration agreements and the formation of new collaborations; 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," "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; the potential impact of pandemics and other health emergencies, including their impact on the global supply chain; 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 may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the headings "Risk Factors Summary" and "Risk Factors" and elsewhere in our annual report on Form 10-K for the year ended December 31, 2022, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, our Quarterly Report on Form 10-Q for the quarter ended June 30, 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



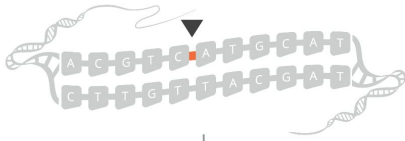
PLATFORM FOR
rapidly-programmable
precision medicines



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

NUCLEASE CRISPR, ZFN, TALENs

Precision targeting with CRISPR



Double-stranded breaks

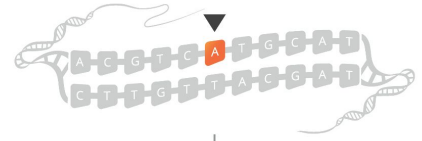


Lack of control of gene sequence outcomes



BASE EDITING BEAM THERAPEUTICS

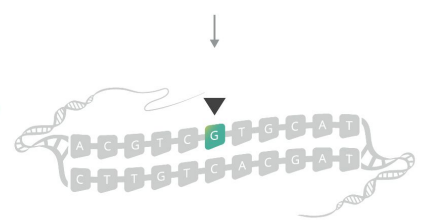
Precision targeting with CRISPR



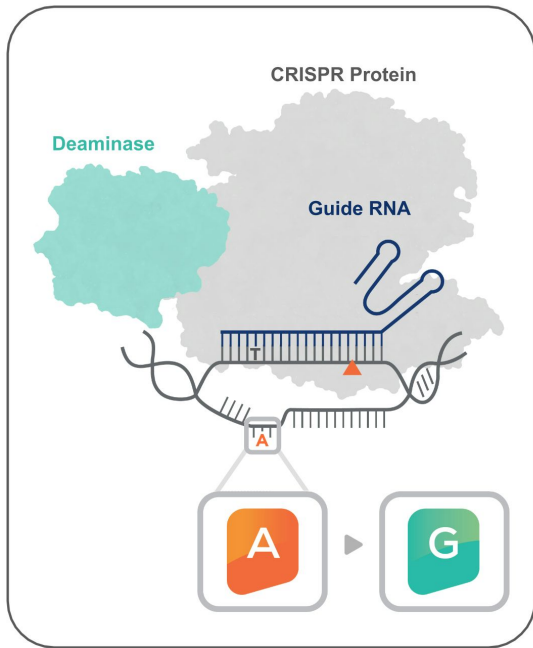
Enzymatic base conversion



Highly efficient with predictable gene sequence outcomes

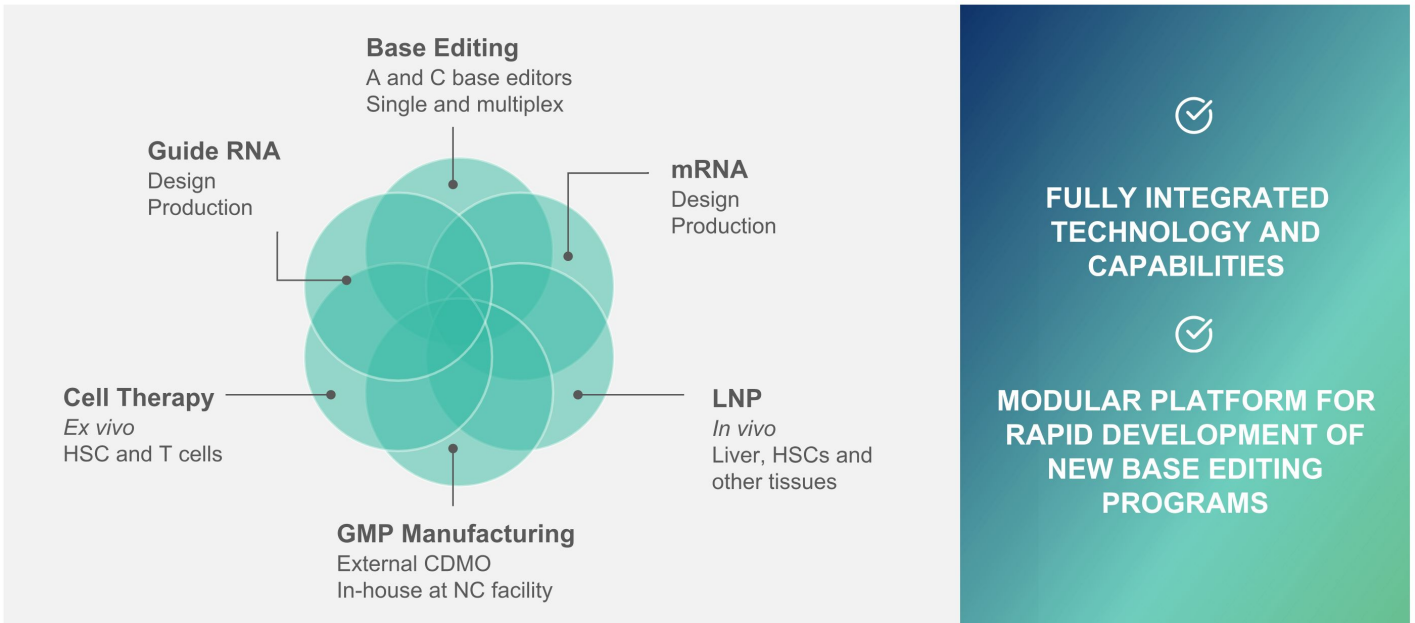


Base editing technology has multiple, highly versatile applications



		PROGRAMS
Correct mutations	Repairs the most common type of gene mutation, single base changes	BEAM-302, BEAM-301
Silence proteins	Turns off any 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

We have built a comprehensive, fully-integrated platform for precision genetic medicines



Advancing a diversified pipeline into the clinic



PROGRAM / DISEASE	DELIVERY	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
BEAM-101	Sickle Cell Disease (SCD)	<i>Ex vivo</i> HSC	Activation of fetal hemoglobin (HbF)				
ESCAPE	Sickle Cell Disease Beta Thalassemia	<i>Ex vivo</i> HSC	Multiplex HbF edit + CD117 edit-antibody pair				
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				
BEAM-201	T-cell Leukemia/Lymphoma (T-ALL / T-LL) and CD7+ AML	<i>Ex vivo</i> T cells	Multiplex silenced CD7 CAR-T				
Pfizer collaboration target	<i>In vivo</i> LNP	Undisclosed					
Apellis collaboration target	<i>In vivo</i> LNP	Undisclosed					

LNP = Lipid Nanoparticle; HSC = Hematopoietic Stem Cell; T-ALL / TLL = T-Cell Acute Lymphoblastic Leukemia / T-Cell Lymphoblastic Lymphoma; AML = Acute Myeloid Leukemia; ESCAPE: Engineered Stem Cell Antibody Paired Evasion

Sickle Cell Disease

HEMATOLOGY

- Best-in-class potential for BEAM-101

- Increased probability of technical success for *ex vivo* gene editing and HbF upregulation

- Validated FDA regulatory pathway

- ESCAPE has potential to eliminate chemotherapy from transplant, expanding reach of base editing to more patients

- Platform for future hematology pipeline

Alpha-1 Antitrypsin Deficiency

LIVER GENETIC DISEASE

- Best-in-class potential for BEAM-302

- Increased probability of technical success for *in vivo* LNP gene editing in liver

- Potential for rapid clinical proof of concept (change in functional AAT and PiZ AAT levels)

- Clinical-stage AATD program with potential to be a one-time treatment that benefits both lung and liver disease

- Platform for future liver pipeline

2023 was a transformative year for CRISPR gene editing, for base editing, and for Beam



2023 Highlights

GENE EDITING

First *in vivo* gene editing INDs cleared by FDA

First *in vivo* liver base editing clinical data

First CRISPR-based product approved for SCD

BEAM

First patients dosed with base edited therapies in U.S. in multiple trials

- ✓ BEAM-201 dosed Q3
- ✓ BEAM-101 dosed and engrafted Q4

Lilly acquisition of Beam's rights to Verve programs

Prioritized portfolio to focus on core value drivers in SCD and AATD

Expected cash runway into 2027

2024 Anticipated Catalysts

BEAM-101 SCD

Complete sentinel dosing and initiate expansion dosing in first half of 2024

Present clinical data on multiple patients in second half of 2024

ESCAPE SCD

Initiate Phase 1-enabling preclinical studies in 2024

BEAM-302 AATD

File BEAM-302 CTA ex-U.S. 

Initiate clinical trial for BEAM-302 ex-U.S. in first half of 2024*

BEAM-301 GSD1a

Submit U.S. IND application in first half of 2024

BEAM-201 T-ALL / T-LL

Present clinical data in second half of 2024

*assuming CTA acceptance

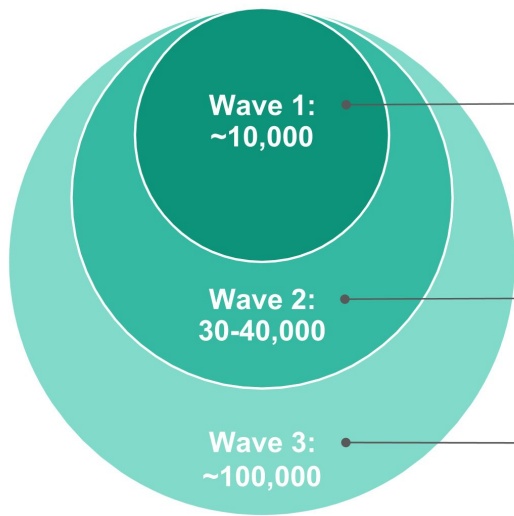
What if we could develop better one-time therapies for patients with SCD?

SICKLE CELL DISEASE



Beam's multi-wave strategy is focused on developing safer, more effective, and more accessible treatments for patients with SCD

Eligible SCD Patient Population (U.S.)



Wave 1 BEAM-101: Precise HbF upregulation

Potentially best-in-class gene editing

Non-cutting, non-viral therapy with busulfan conditioning to address severe SCD with high vaso-occlusive crisis (VOC) burden

Wave 2 ESCAPE: Multiplex HbF edit + CD117 selection edit

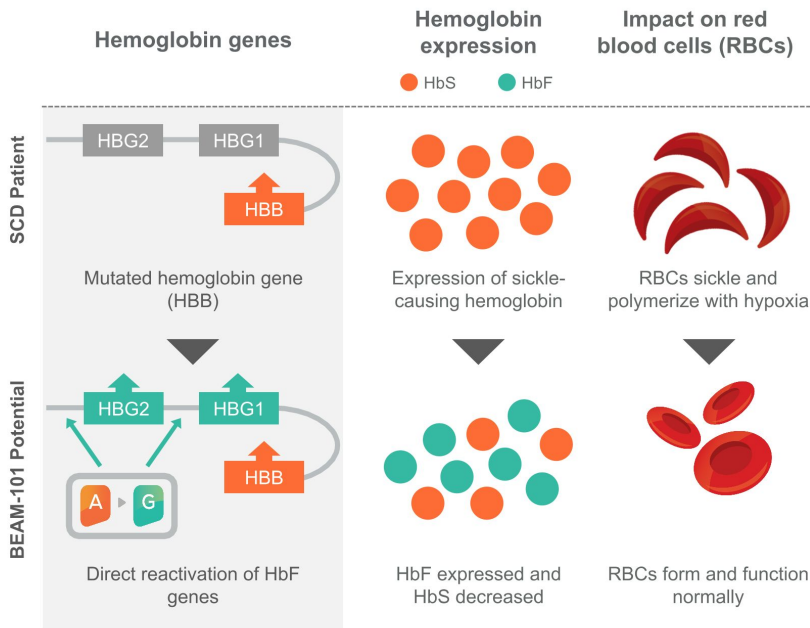
Non-genotoxic conditioning eliminates chemotherapy and broadens patient population for *ex vivo* gene therapy

- Broader range of disease severity
- Increased willingness-to-treat
- Wider age range

Wave 3 *In vivo*: Base editing with HSC-targeted LNPs

***In vivo* delivery** would overcome need for transplantation, lower infrastructure requirements and unlock wider patient access and geographies

BEAM-101: Designed to be best-in-class genetic medicine for SCD



SCD Unmet Need

- Sickle cell 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

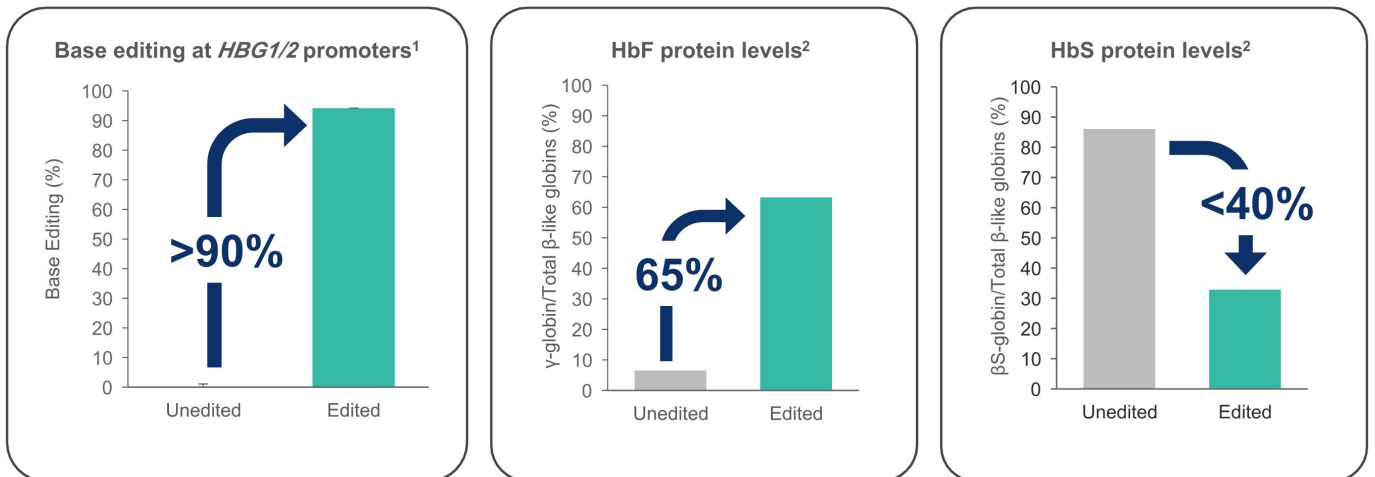
Current Available Treatments

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

BEAM-101 Potential

- Precision editing without requirement of double-stranded DNA breaks or viral insertion
- More efficient editing leading to greater and more uniform induction of HbF and reduction of HbS and normalization of hemoglobin
- Investment in wholly owned manufacturing and improved process and patient experience

BEAM-101: Potential for highest HbF induction and lowest residual HbS levels versus other approaches in the field



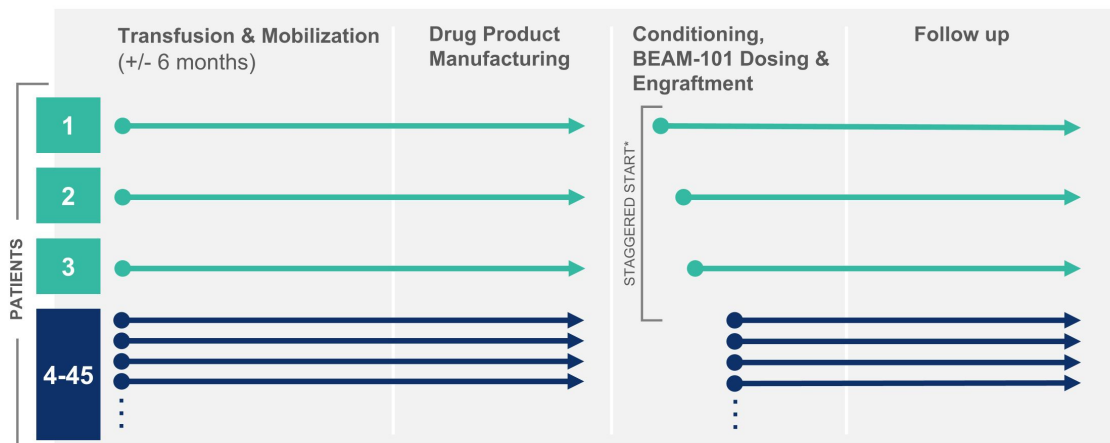
Preclinical data presented at ASGCT 2020; Edited human HSPCs analyzed 16 weeks after infusion in NBSGW mice (Mean \pm SEM, n=4-6); 1. Sorted human Lineage-CD34+ bulk bone marrow; 2. Sorted erythroid cells (GlyA+)

Precise, single base editing without need for double-stranded breaks or viral insertion results in highest editing efficiency in pre-clinical models

BEAM-101: First clinical base editing program in the U.S., accelerating path to SCD patients and the market



BEACON Phase 1/2 Study Design



Select safety endpoints

- Proportion of patients with successful neutrophil engraftment by day 42
- Safety and tolerability assessments

Select efficacy endpoints

- Severe VOCs
- Total Hb and hemolysis
- HbF levels
- Patient reported outcomes
- RBC function and organ damage
- Time to engraftment

*Engraftment of each sentinel patient required before conditioning next patient

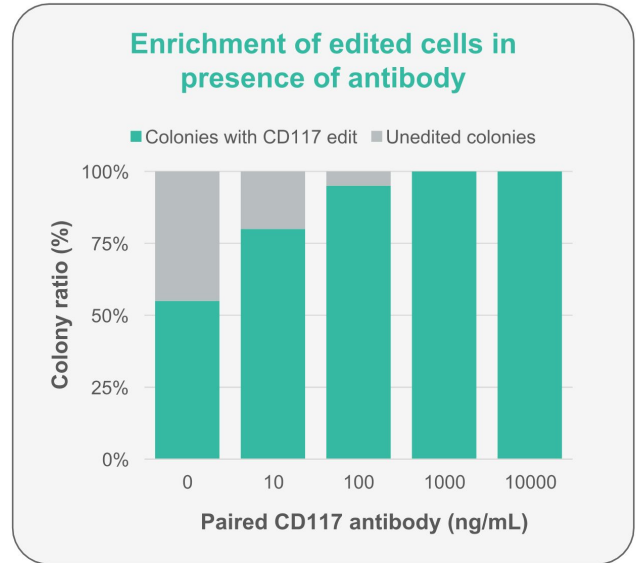
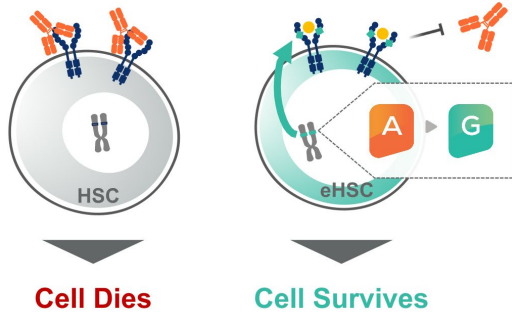
- Sentinel cohort
- Expansion cohort

- First patient dosed and successfully engrafted in Q4
- Completed manufacturing of BEAM-101 for multiple patients
- Multiple patients consented for sentinel and expansion cohorts; expansion dosing expected to initiate in 1H 2024

Anticipate presenting clinical data on multiple patients in the second half of 2024

Wave 2 ESCAPE: Designed for selective depletion of diseased cells to enable non-genotoxic conditioning for SCD

- Stem cell factor (SCF) signaling via CD117 required for HSC survival and proliferation
- Single base edit changes the epitope on CD117 receptor without observed impact on HSC biology
- Customized conditioning antibody depletes diseased unedited cells, but enables CD117-edited, non-diseased cells to “ESCAPE” and grow normally

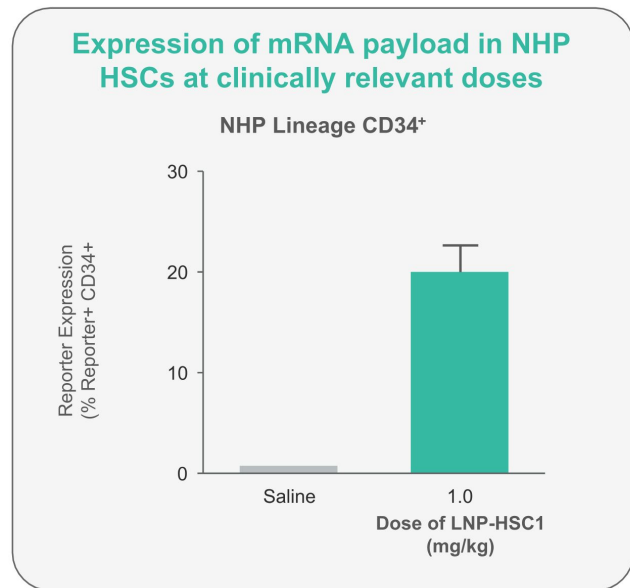


ESCAPE: Engineered Stem Cell Antibody Paired Evasion

Plan to initiate Phase 1-enabling studies in 2024

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

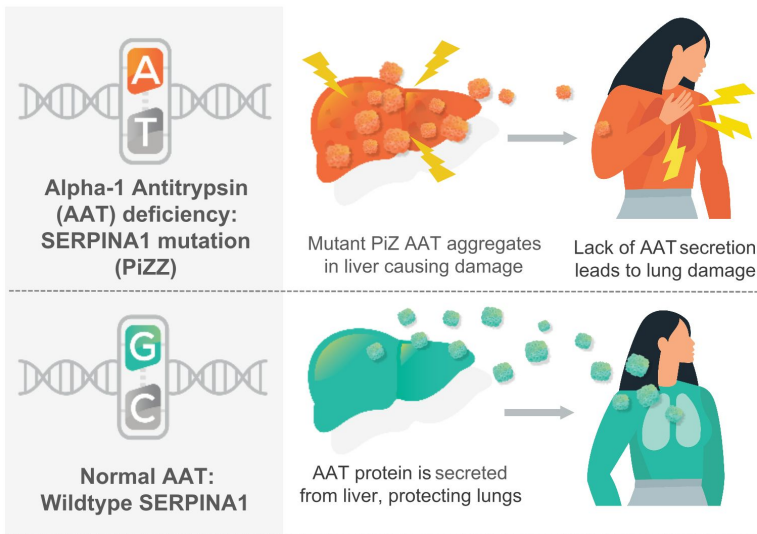


What if we could use base editing to correct disease-causing mutations *in vivo*?

GENETIC DISEASES



BEAM-302: Aims to restore expression of functional AAT to address AATD-related lung and liver disease



AATD Unmet Need

- PiZZ genotype is >95% of severe AATD population that typically develop progressive lung and/or liver disease
- 100,000 PiZZ individuals in the U.S.; ~10% diagnosed

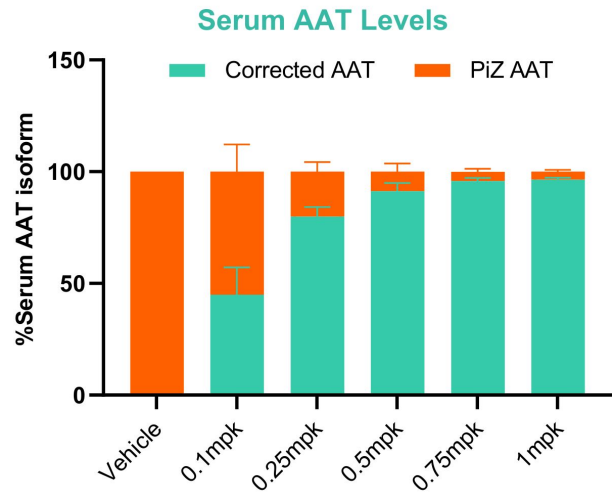
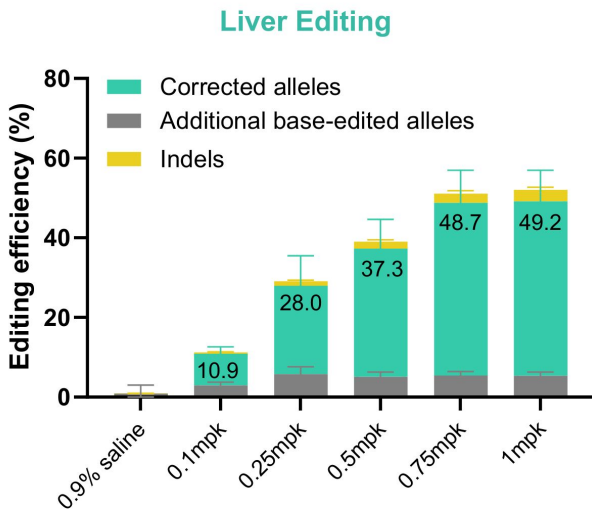
Current Available Treatments

- Lung disease: Medications for emphysema and possible weekly IV plasma-derived AAT (augmentation); lung transplant considered for severely affected patients
- Liver disease: Supportive care; liver transplant considered for end-stage disease

BEAM-302 Potential

- One-time therapy that addresses both lung and liver disease, with corrected gene under normal regulation
- Reduction of mutant PiZ AAT in liver and restored circulating functional AAT

BEAM-302: Corrected the PiZ mutation and restored functional AAT with a single dose in AATD mouse model



Liver editing correlated with increased corrected serum AAT and decreased mutant PiZ AAT, at or below 0.75mpk

BEAM-302: Phase 1/2 trial 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

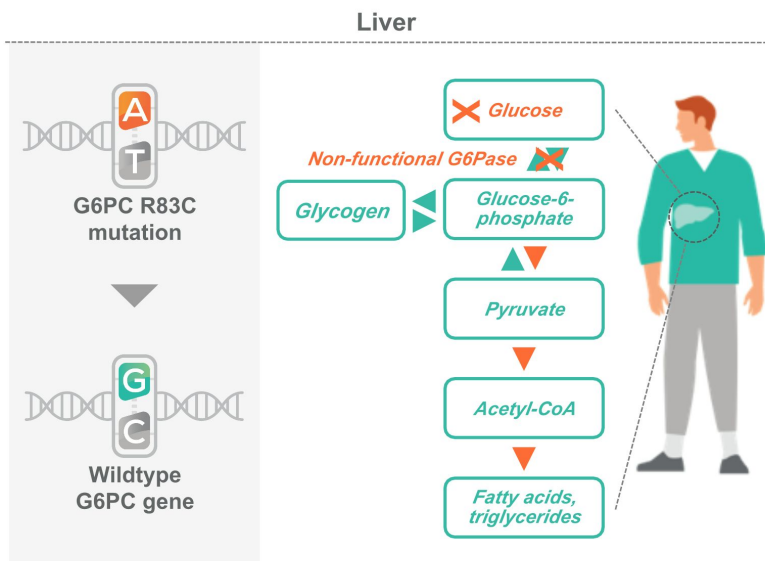
- Up to 4 dose cohorts
- Patients included with mild to moderate liver disease

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
- CTA submitted to UK; additional filings to follow

Initiate Phase 1/2 trial ex-U.S. in first half of 2024

BEAM-301: Aims to normalize glycogen metabolism in patients with GSD1a to prevent hypoglycemia and other disease manifestations



Unmet Need in GSD1a Patients with Severe R83C Mutation:

- 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
- Estimated ~300 R83C patients in U.S. based on updated epidemiology

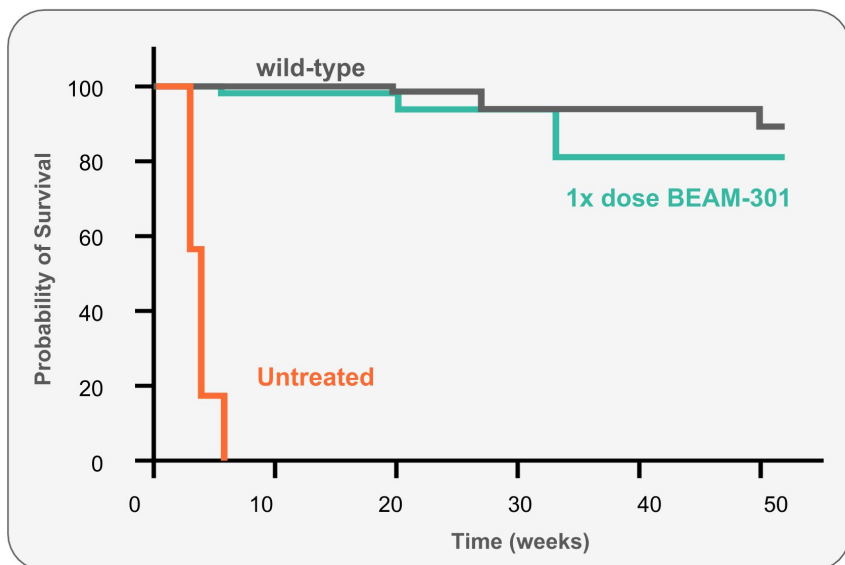
Current Standard of Care:

- Liquid cornstarch supplementation every 2-4 hours, even throughout the night

BEAM-301 Potential:

- Correct liver G6PC mutation to restore enzyme activity and enable normal glucose homeostasis, as well as eliminate chronic cornstarch supplementation
- Animal studies suggest ~11% editing sufficient for restoring fasting glucose and 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
- Given its rare nature and geographic distribution of patients, Beam will initially focus development of BEAM-301 in the U.S.

U.S. IND filing expected in first half 2024

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

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



- \$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



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

2024 Anticipated Catalysts

BEAM-101 SCD

Complete sentinel dosing and initiate expansion dosing in first half of 2024

Present clinical data on multiple patients in second half of 2024

ESCAPE SCD

Initiate Phase 1-enabling preclinical studies in 2024

BEAM-302 AATD

File BEAM-302 CTA ex-U.S. 

Initiate clinical trial for BEAM-302 ex-U.S. in first half of 2024*

BEAM-301 GSD1a

Submit U.S. IND application in first half of 2024

BEAM-201 T-ALL / T-LL

Present clinical data in second half of 2024

*assuming CTA acceptance



THANK YOU

J.P. MORGAN HEALTHCARE CONFERENCE JANUARY 2024

Beam Therapeutics Highlights Progress Across Base Editing Portfolio and Outlines 2024 Anticipated Milestones

First Patient Dosed and Successfully Engrafted in BEACON Phase 1/2 Trial of BEAM-101 in Patients with Severe Sickle Cell Disease; Significant Enrollment Progress Supports First Expected Clinical Data Readout in Second Half of 2024

European Clinical Trial Application (CTA) Submitted for BEAM-302; Trial Initiation in Alpha-1 Antitrypsin Deficiency Planned for First Half of 2024

Investigational New Drug (IND) Application for BEAM-301 On-track for First Half of 2024

Cash Runway Expected to Support Operating Plans into 2027

CAMBRIDGE, Mass., Jan. 8, 2024 - Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported progress across the company's hematology and genetic disease portfolios and provided updates on anticipated upcoming milestones.

"Our vision is to establish Beam as a sustainable, fully integrated company pioneering a new class of genetic medicines with base editing. We made tremendous progress toward this goal in 2023, including opening our own GMP manufacturing facility, dosing the first patients in multiple *ex vivo* clinical programs, including BEAM-101 for sickle cell disease (SCD), and accelerating our *in vivo* program for alpha-1 antitrypsin deficiency (AATD), as exemplified by the filing of our CTA for BEAM-302," said John Evans, chief executive officer of Beam Therapeutics. "Building on this momentum and benefiting from the significant clinical validation, regulatory clarity, and scientific breakthroughs occurring in the broader gene editing field, we expect 2024 to be a year of significant catalysts for Beam. Our highly differentiated SCD and AATD programs have the potential to provide best-in-class therapies for significant patient populations with high unmet need, while also establishing a platform for sustainable long-term growth across multiple therapeutic areas."

Pipeline Updates and 2024 Anticipated Milestones

Sickle Cell Disease (SCD) Franchise

Beam is pursuing a long-term, staged development strategy for SCD that has three Waves of innovation intended to progressively expand the reach of our base editing approach to broader subsets of patients.

- **Wave 1:** BEAM-101 is 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. Preclinical models
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suggest base editing could lead to improved HbF induction and lower residual disease-causing hemoglobin S compared to existing gene therapy options.

- o The first patient was dosed in the fourth quarter of 2023 and successfully achieved engraftment in the BEACON Phase 1/2 clinical trial, an open-label, single-arm, multicenter study evaluating the safety and efficacy of BEAM-101 in adult patients with severe SCD. Treatment with BEAM-101, in which the edited cell product is delivered in an autologous bone marrow transplant, will occur on a sequential basis for the first three patients treated in the trial, and then will be given in parallel for all subsequent patients.
- o Patients have continued to be consented in the BEACON trial, and Beam anticipates dosing the remaining two patients in the sentinel cohort and initiating dosing in patients in the expansion cohort in the first half of 2024.
- o The company is on-track to report initial data on multiple patients from the BEACON trial in the second half of 2024.
- **Wave 2:** Beam continues to advance and invest in its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning platform and anticipates initiating Phase 1-enabling preclinical studies for the program in 2024. ESCAPE aims to avoid the toxicities associated with currently available conditioning regimens for patients with SCD required prior to autologous transplant.
- **Wave 3:** The company is also 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 seeks to treat genetic diseases using single course gene editing therapies delivered through intravenous infusion of LNPs, which are a clinically validated technology for delivery of nucleic acid payloads to the liver.

- BEAM-302, the company's priority genetic disease program, is a potential treatment for AATD, which is characterized by early onset emphysema, liver disease, and increased all-cause mortality compared to the general population. There is a large unmet need for novel therapies that can treat patients with AATD-associated lung and liver disease.
 - o BEAM-302 is 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 AATD. Approximately 100,000 patients in the U.S. are estimated to carry the PiZZ genotype.
 - o Preclinical data to date demonstrated that treatment with BEAM-302 led to significantly increased levels of corrected and functional alpha-1 antitrypsin (AAT) and reduced mutant PiZ AAT in multiple *in vivo* rodent disease models at clinically relevant doses. These findings support the potential of BEAM-302 to
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efficiently correct the disease-causal PiZ mutation after a single dose and potentially address both the liver and lung disease associated with AATD.

- o Beam has filed a CTA for BEAM-302, and, assuming CTA acceptance, plans to initiate a clinical trial for BEAM-302 in the first half of 2024.
- In addition, Beam is also advancing BEAM-301 for the potential treatment of glycogen storage disease type 1a (GSD1a), an autosomal recessive disorder caused by mutations in the G6PC gene that disrupt a key enzyme, glucose-6-phosphatase, involved in maintaining glucose homeostasis.
 - o 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 GSD1a.
 - o Preclinical data have shown that a single administration of BEAM-301 directly and durably corrected the R83C mutation *in vivo*, with an ongoing significant survival benefit one year after the initial dosing.
 - o Beam is focusing initial development of BEAM-301 in the U.S. and expects to submit an investigational new drug (IND) application in the first half of 2024.
- Beam is also advancing lead liver-targeted programs from its collaborations with Pfizer and Apellis.

Sustainable Research Portfolio

- Beam's near-term research and platform investments are focused on specific applications leveraging Beam's *in vivo* editing capabilities in the liver targeting both rare and common genetic disorders, as well as opportunities in hematology and immunology/oncology.
- Enrollment in the company's Phase 1/2 clinical trial of BEAM-201, a multiplex-edited allogeneic CAR-T product candidate, is ongoing for the treatment of relapsed/refractory T-cell acute lymphoblastic leukemia (T-ALL)/T-cell lymphoblastic lymphoma (T-LL). Beam expects to report an initial clinical dataset for BEAM-201 in the second half of 2024.

Cash Position and Updated Operating Runway

As of December 31, 2023, Beam estimates that it had \$1.2 billion 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, 2023, and its results of operations for the three months and year ended December 31, 2023. 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, 2023 will enable the company to fund its anticipated operating expenses and capital expenditure requirements into 2027. This expectation assumes anticipated cost savings



related to the company's previously announced portfolio prioritization and streamlining of operations and includes funding directed toward reaching each of the key anticipated milestones for BEAM-101, BEAM-201, BEAM-301 and BEAM-302 described above, as well as continued investments in platform advancements and manufacturing capabilities.

J.P. Morgan Healthcare Conference

Beam management will present and discuss Beam's pipeline and business updates during a presentation at the 42nd Annual J.P. Morgan Healthcare Conference today, Monday, January 8, 2024, at 11:15 a.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 that includes a suite of gene editing and delivery technologies and is in the process of building 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 42nd Annual J.P. Morgan Healthcare Conference; our expectations for transitioning to a multi-program clinical stage company; the therapeutic applications and potential of our technology, including with respect to SCD, AATD, GSD1a, T-ALL/TLL, and our conditioning regimens; our plans, and anticipated timing, to advance our programs, the clinical trial designs and expectations for BEAM-101, BEAM-201, BEAM-301 and BEAM-302; our estimated cash, cash equivalents and marketable securities as of December 31, 2023 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 successfully achieve the benefits of our portfolio prioritization and strategic restructuring; 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 potential impact of pandemics and other health emergencies, including their impact on the global supply chain; the uncertainty that our product candidates will receive regulatory approval necessary to initiate human clinical studies; 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 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, 2023; 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, 2022, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, 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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