### 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 9, 2023

### 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 teleph	none number, including area code: (8	857) 327-8775
	(Former Nam	e or Former Address, if Changed Since Last	Report)
	ck the appropriate box below if the Form 8-K filin provisions (see General Instruction A.2. below):	ng is intended to simultaneously satisfy	y the filing obligation of the registrant under any of the
	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
Com	mon Stock, \$0.01 par value per share	BEAM	Nasdaq Global Select Market
	cate by check mark whether the registrant is an ener) or Rule 12b-2 of the Securities Exchange Act of		Rule 405 of the Securities Act of 1933 (§230.405 of
Eme	erging growth company		
	n emerging growth company, indicate by check ma or revised financial accounting standards provided	e	ise the extended transition period for complying with large Act. $\square$

#### 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, 2022, Beam Therapeutics Inc. (the "Company") announced in a press release on January 9, 2023 that it estimates that it had cash, cash equivalents and marketable securities of approximately \$1.0 billion as of December 31, 2022.

The information contained in Item 2.02 of this Form 8-K regarding the Company's estimated cash balance as of December 31, 2022 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, 2022 and its results of operations for the three months and year ended December 31, 2022. 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 9, 2023, 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 41st Annual J.P. Morgan Healthcare Conference on January 9, 2023. 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 filling.

#### Item 8.01. Other Events.

On January 9, 2023, 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 41st Annual J.P. Morgan Healthcare Conference; the Company's plans, and the anticipated timing, to advance its programs; 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 clinical trial design for BEAM-201; the Company's estimated cash, cash equivalents and marketable securities as of December 31, 2022 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." "blan."

"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 the Company's 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 the Company's product candidates will receive regulatory approval necessary to initiate human clinical studies; that preclinical testing of 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 enrollment and initiation of 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 relate 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, 2022; and other factors discussed in the "Risk Factors" and "Risk Factors Summary" sections of the Company's Annual Report on Form 10-K for the year ended December 31, 2021, filed with the SEC on Fovember 7, 2022, and in other filings that the Company makes with the SEC in the future. These forward-looking statements speak only as of the date of this C

#### 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 9, 2023
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

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

#### BEAM THERAPEUTICS INC.

Date: January 9, 2023

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



# Beam Therapeutics PRECISION GENETIC MEDICINES THROUGH BASE EDITING

### 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-lost statements include statements regarding: the initiation, timing, progress and results of preclinical studies and research and development program the initiation and progress of clinical trials, including our BEACON trial and our BEAM-201 trial; the advancement of our pipeline, including the ad of BEAM-101, BEAM-301, BEAM-301, BEAM-302, additional CAR-T and liver programs, and Stargardt disease program in multiple preclinical sticurrent expectations and anticipated results of operations, including our expected use of capital; the potential activities and benefits under license collaboration agreements and the formation of new collaborations; and the therapeutic applications and potential of our technology, including our develop life-long, curative, precision genetic medicines for patients through base editing, including potential safety advantages, all of which are st known and unknown important risks, uncertainties and other factors that may cause our actual results, performance or achievements, market tree industry results to differ materially from those expressed or implied by such forward-looking statements. Therefore, any statements contained her not statements of historical fact may be forward-looking statements and should be evaluated as such. Without limiting the foregoing, the words "a "expect," "suggest," "plan," "vision," "believe," "intend," "project," "forecast," "estimates," "targets," "projections," "potential," "should," "could," "wou "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 expre 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 our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the potential impact of the ( pandemic; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be p 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 p candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncert identified under the headings "Risk Factors Summary" and "Risk Factors" and elsewhere in our annual report on Form 10-K for the year ended D 2021, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2022, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, and in any subsequent filings with the Securities and Exchange Com "SEC") which are available on the SEC's website at www.sec.gov. Additional information will be made available by our annual and quarterly repor 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 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 any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicab



# Our vision is to provide life-long cures patients suffering from serious diseas

- Potential for one-time, curative therapies
- Gene editing for rare and common diseases
- ► Platform for rapidly-programmable precision medicin

### Base editing is a differentiated, potentially best-inclass gene editing technology



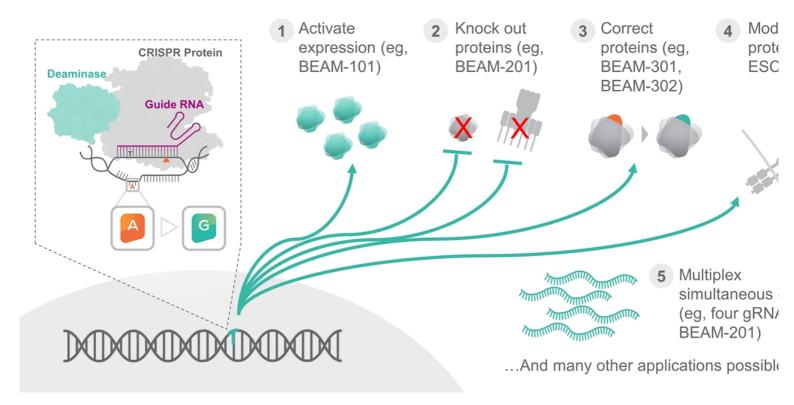




Precise targeting?	Yes (guide RNA or ZF/TALE)	Yes (guide RNA)
Durability of edit?	Permanent	Permanent
Double strand breaks?	Yes	No
Applications?	Primarily knockout	Correct, modify, activate, mul
Editing predictability	Random insertions and deletions 100s of uncharacterized edits	Single base edits All edits fully characterized
Efficiency of precise edit?	Low – dividing cells only	High – any cell type

# A precise gene editing technology with highly versatile applications





# We are establishing a leading platform for precision genetic medicine



### Suite of gene editing technologies

- Base editing
  - o ABE: A-to-G (or T-to-C) editors
  - o CBE: C-to-T (or G-to-A) editors
  - Additional kinds of base editors
- Nuclease editing
- RNA editing
- Prime editing



### Suite of delivery technologies

- Autologous cell thera
- ► Allogeneic cell thera
- ► mRNA
- LNP vectors
- Viral vectors

### Internal manufacturing capability

▶ 100,000 square foot cGMP clinical/commercial facility in NC, phased build, anticipated to be operational in 2023

### Advancing a diversified pipeline into the clinic

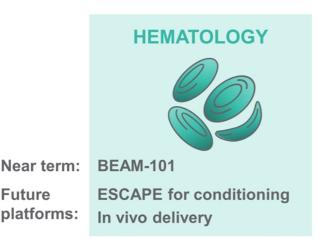


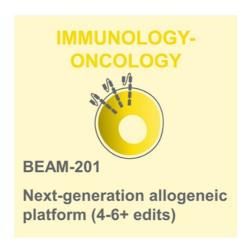
DELIVERY	PROC	GRAM / DISEASE	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II
Ex vivo HSCs	BEAM-101	Sickle Cell Disease Beta Thalassemia	Activation of fetal hemoglobin				
	BEAM-102	Sickle Cell Disease	Correction of HbS sickle mutation	Refocusing o	n ESCAPE or i	<i>in vivo</i> delivery	
	ESCAPE	Sickle Cell Disease Beta Thalassemia	Multiplex CD117 edit-antibody pair				
Ex vivo T cells	BEAM-201	T-ALL / T-LL CD7+ AML	Multiplex silenced CD7 CAR-T				
	BEAM-301	Glycogen Storage Disease la	Correction of R83C mutation				
	BEAM-302	Alpha-1 Antitrypsin Deficiency	Correction of E342K mutation				
	Glycogen Sto	rage Disease Ia	Correction of Q347X mutation				
	Hepatitis B Vi	rus	Multiplex silencing				
	Complement	Pathway (Apellis)	Undisclosed				
	3 undisclosed	targets (Pfizer)	Undisclosed				
AAV	Stargardt Disease		Correction of G1961E mutation				

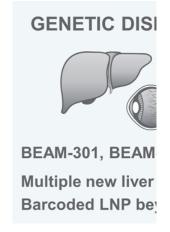
 $LNP = Lipid \ Nanoparticle; AAV = Adeno \ Associated \ Virus; \ HSC = Hematopoietic \ Stem \ Cell; \ T-ALL \ / \ T-LL = T-Cell \ Acute \ Lymphoblastic \ Leukemia \ / \ T-Cell \ Lymphoblastic \ Lymphoma; \ AML = Acute \ Myeloid \ Leukemia; \ ESCAPE: \ Engineered \ Stem \ Cell \ Antibody \ Paired \ Evasion \ Paired \ Nanoparticles \ Antibody \ Paired \ Nanoparticles \ Nanopartic$ 

### Beam is developing medicines across three franchises, each with near- and long-term potential









- Lead Programs: Potentially de-risk technology (higher probability of technical succe faster path), generate revenue, and benefit patients with high unmet need
- Future platforms: Expand addressable patient populations to create highly valuable differentiated franchises through further innovation in editing and delivery

ESCAPE: Engineered Stem Cell Antibody Paired Evasion

**Future** 

### Key progress and anticipated milestones

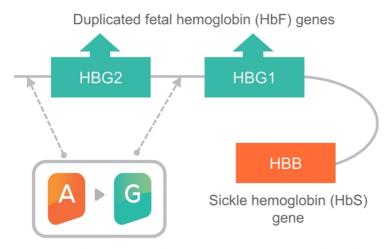


	2022 Achievements	opcoming winestones
Hematology	First subject enrolled for <b>BEAM-101</b> Refocused on new technology: ESCAPE & LNP	Complete sentinel cohort enrollment ar expansion cohort of <b>BEACON</b> in 2023  Data presentation on multiple patients <b>BEACON</b> in 2024
Immunology - Oncology	Submit IND for <b>BEAM-201</b> and respond to hold  Refocused on next gen allogeneic strategies	Dose first <b>BEAM-201</b> patient by mid 20
Genetic disease	Initiate IND-enabling studies for <b>BEAM-301</b> Nominated <b>BEAM-302</b> development candidate	Regulatory filing for <b>BEAM-301</b> by late early 2024 Regulatory filing for <b>BEAM-302</b> in early
Platform	Strategic platform partnerships (Pfizer, Orbital)	

### BEAM-101: Designed to treat sickle cell disease with a potentially one-time, direct, non-cutting activation of HbF



Sickle Cell Disease: 100,000 patients in the US; severe pain crises, multi-organ damage, early mort



A single base editor + gRNA edits regulatory element of both fetal hemoglobin genes, without cutting DNA

HPFH = Hereditary Persistence of Fetal Hemoglobin

### Designed for best-in-class profile:

- One-time therapy with potential for fetal hemoglobin (HbF) induction
- Direct editing of HbF genes to turn th
- Potential for greatest reduction of d causing HbS due to hemoglobin swite
- Non-viral: No detectable random inse
- Non-cutting: Lower risk for genotoxic and chromosomal abnormalities

### Investment in patient delivery to differe

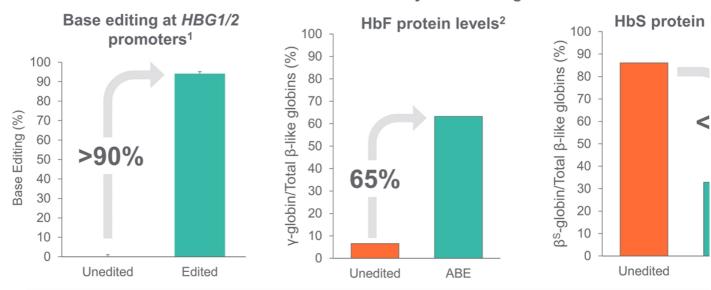
- Wholly owned manufacturing: contra quality and connection to patient service
- Investment in patient services: optin patient experience



### Potentially best-in-class attributes of BEAM-101 product



### Edited human CD34+ cells followed by 16 week engraftment in mice

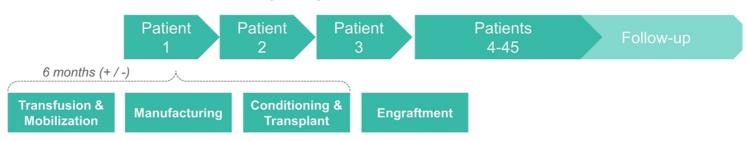


- Potential for highest HbF induction and lowest residual HbS levels versus other approaches in
- ▶ Building capabilities for potential best-in-class patient delivery including internal manufacturing

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

# BEAM-101 is the first clinical base editing program in the U.S., accelerating path to patients and the market

BEACON-101 Phase 1/2 Study Design



### Select inclusion criteria

- Patients with sickle cell disease (SCD) with severe veno-occlusive crises despite hydroxyurea or other supportive measures
- Age ≥18 to ≤35 years for initial cohort

### Select safety endpoints

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

### Select efficacy endpoi

- Severe vaso-occlusive
- ▶ Transfusion requireme
- Hemoglobin F levels
- Quality of life and abilit
- Markers of red blood c and organ damage

# Well positioned to deliver potentially best-in-class regimens for SCD patients, now and in the future



### Wave 1

Base Editing + HSC Transplant

Precise gene editing (non-cutting, non-viral) Busulfan conditioning

BEAM-101 (HbF)



### Wave 2

Improved Conditioning

Less toxic conditioning selects for edited cells – potential to expand to younger and broader patient population

ESCAPE-1 (HbF+CD117) ESCAPE-2 (Makassar+CD117)



### Wave 3

*In vivo* Delivery

In vivo editing after infusi HSC-targeted LNPs (no transplant)

HbF + Makassar availa

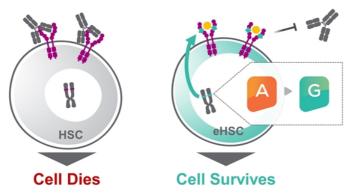


<sup>\*</sup> **ESCAPE:**  $\underline{\underline{E}}$ ngineered  $\underline{\underline{S}}$ tem  $\underline{\underline{C}}$ ell  $\underline{\underline{A}}$ ntibody  $\underline{\underline{P}}$ aired  $\underline{\underline{E}}$ vasion

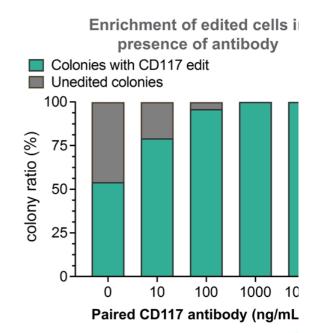
# ESCAPE\* designed for selective depletion of diseased cells, which may enable non-genotoxic conditioning



- ▶ Stem cell factor (SCF) signaling via CD117 is required for HSC survival and proliferation
- ▶ A single base edit changes an epitope on the CD117 receptor and is designed not to impact HSC biology
- Customized conditioning antibody depletes diseased unedited cells, but enables CD117-edited cells to "ESCAPE" and grow normally



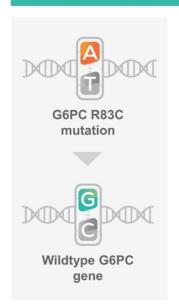


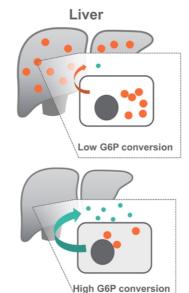


# BEAM-301 program aims to restore impaired glycogen metabolism which otherwise causes significant morbidity



Glycogen Storage Disease la: 900 US R83C patients; severe hypoglycemia, liver & kidney dysfunc





### **GSD1a unmet need:**

- ▶ Low G6PC activity can result in severe drop in levels within 1-3 hrs
- ▶ Hypoglycemia may result in seizures or can be
- ► Multiple organ dysfunction (e.g. renal and liver)

### **BEAM-301** potential:

- ► Near-normal serum metabolites, G6PC activity, morphology, increased survival in mice
- ► Animal studies suggest 11% editing sufficient for fasting glucose¹

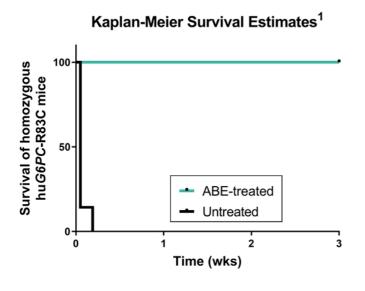
### **Key points:**

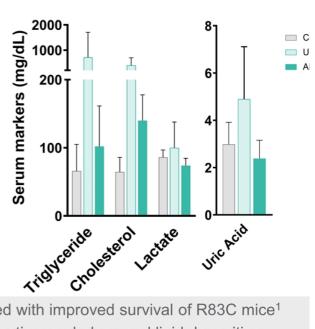
- ▶ Beam's first in vivo DC
- ▶ First DC in industry with *in vivo* direct correction
- ▶ Regulatory filing expected by late 2023 / early 2

- 1. Chou & Mansfield. 2007. Curr. Gen. Ther.
- 2. Based on publicly announced development candidates

# BEAM-301 program aims to restore impaired glycogen metabolism which otherwise causes significant morbidity







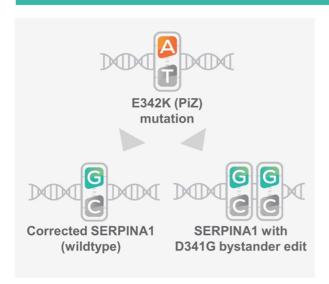
- ▶ ABE correction of GSDIa R83C mutation associated with improved survival of R83C mice¹
- ▶ Near-normal serum metabolites, G6PC activity, hepatic morphology and lipid deposition

Preclinical data presented at ESGCT 2021; 1. Homozygous huG6PC-R83C mice untreated or treated with LNP via temporal vein shortly after birth, and untreated mice survived less than 3 days with glucose therapy

# BEAM-302: Development candidate nominated for potential one-time treatment of AATD



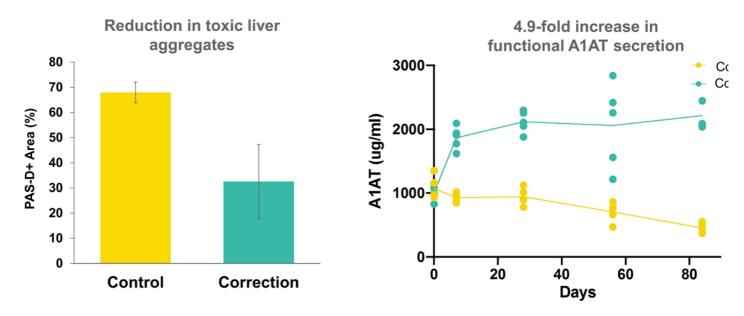
Alpha-1 Anti-trypsin Deficiency (AATD): 60,000 ZZ patients in US; severe progressive lung & liver



- Potential one time treatment to create permanent con E342K and enable normal A1 secretion and gene reg
- Designed to address disease pathology in both the lively lung
- In preclinical studies, lead candidate delivered up to correction editing, that resulted in >3X increase in protein (> 11 uM protective threshold) at clinically dose of 0.75mpk
- In a minority of cells, correction resulted in wildtype a D341G allele (bystander) that was observed to function normally
- ► BEAM-302 nominated for development; regulatory expected in early 2024

# BEAM-302 program has the potential to address both lung and liver pathology of AATD in one course treatment





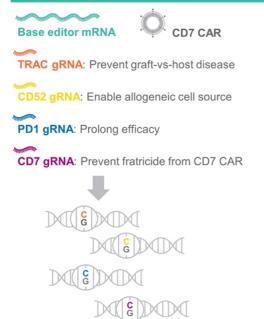
Representative in vivo studies of PiZZ mouse with precursor base editors

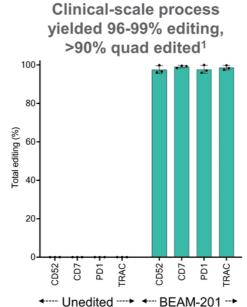
Preclinical data presented at ASGCT 2020; Editing in NSG-PiZ mice with either control (PCSK9) or correction (E342K) provided above results

# BEAM-201: Base edited allogeneic cell therapy candidate with an opportunity to treat aggressive CD7+ leukemias



T-Cell Acute Leukemia: 15% of ALL, not treated by B-cell CARTs, few options for relapsed/refractory



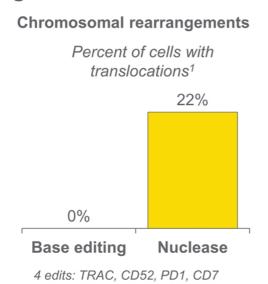


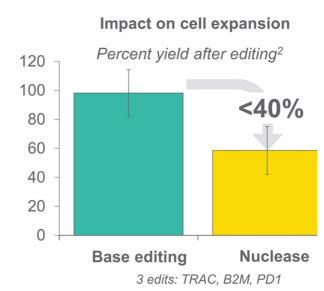
- Multiplex base editing: nuclease editors, no dete chromosomal rearranger normal cell expansion, ai detected DNA damage re in preclinical studies
- ► Clinical-scale process: editing, >90% quad edite
- ▶ BEAM-201 US IND clear patient dosing expecter mid-2023

Preclinical data presented at SITC 2020; 1. Simultaneous base editing at four target loci using clinical-scale process as measured by NGS.

# BEAM-201: Significant advantages of multiplex base editing without double strand breaks







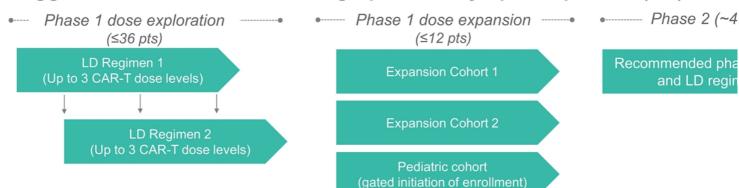
- Multiplex editing more efficient with base editing which translates to better cell product
- ▶ Optimization of platform ongoing with focus on generating next generation "true allogeneic" pro

Preclinical data presented at SITC 2020; 1. Base editing versus nuclease editing with the same four guide RNAs measured via G-banded karyotypes from 100 cells; upda analysis shows <0.1% translocations using first generation CBE (data unpublished) 2. Extensive guide screen across three targets, with BE4 and spCas9 sgRNAs selecte editing efficiency and expansion in single-plex test, final cell yields compared between 3 edits, normalized to electroporation only control

### IMMUNOLOGY-ONCOLOGY

### BTX-ALO-001: Multiplex edited BEAM-201 enables evaluation in aggressive T-cell cancers using optimized lymphodepletion (LD)





### Select inclusion criteria

- ▶ ≥18 to ≤50 yrs for dose exploration
- ▶ ≥1 yrs for peds after FDA review
- ▶ T-ALL or T-LL with one of following:
  - Relapsed after 2nd CR
  - Relapse after HSCT
  - Primary refractory or R/R
- ▶ Eligible for allo HSCT (donor available)

### Select safety endpoints

Incidence and severity of treatment emergent adverse events (TEAE) and treatment-related AEs, including serious AEs (SAEs) and DLTs

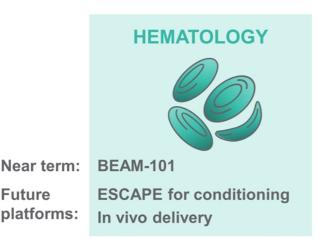
### Select efficacy endpo

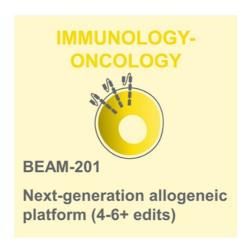
- Proportion of T-ALL pts CRi or T-LL pts with CF time post BEAM-201
- Proportion of pts eligibl based on response to E
- Proportion achieving M status
- ▶ Duration of response, (

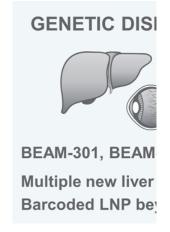
T-ALL = T cell acute lymphoblastic leukemia; T-LL = T cell lymphoblastic lymphoma; CR = Complete response; CRi = CR with incomplete count recovery; PR = Partial res OS = Overall survival; HSCT = Hematopoietic stem cell transplant; DLT = Dose limiting toxicity; MRD = Minimal residual disease

### Beam is developing medicines across three franchises, each with near- and long-term potential









- Lead Programs: Potentially de-risk technology (higher probability of technical succe faster path), generate revenue, and benefit patients with high unmet need
- Future platforms: Expand addressable patient populations to create highly valuable differentiated franchises through further innovation in editing and delivery

ESCAPE: Engineered Stem Cell Antibody Paired Evasion

**Future** 

### Additional strategic and innovator deals potentially unlock base editing value and broaden therapeutic impact



Strategic deals

Innovator deals



- ▶ \$300M upfront, \$1B+ in potential milestones
- 3 gene targets using Beam's editing and delivery to target liver, muscle, CNS
- ▶ Beam option at end of P1/2 for 35% WW cost/net profit split on one program



- ▶ \$75M in upfront payments for base editing for complement mediated diseases
- ▶ Beam opt-in to 50% of US rights after Phase 1 on one program



- ▶ \$50M upfront for license to Cas12b nuclease for certain engineered cell therapies
- ► Non-exclusive license Beam retains ability to use or repartner Cas12b



- License to Beam's base editing technology for the prevention of cardiovascular disease
- ▶ 3 targets: VERVE-101 (PCSK9), VERVE-102 (ANGPTL3), Undisclosed #3
- ▶ Beam opt-in after P1: 50% US (VERVE-101, VERVE-102) or 35% of WW (Target 3)



- ▶ Prime editing (PE) is a novel gene editing technology, complementary to base editing
- Beam provides delivery and CRISPR technology/know-how
- ▶ Beam has exclusive rights to PE: SCD transversion edit, any transitions (30% of





- ORBITAL ▶ Beam has meaningful equity stake in Orbital
  - ▶ Beam access to Orbital IP for gene editing (exclusive) and certain fields (non-exc

### **Meet the Beam Team**























President, Chief Scientific Officer











FORMA

























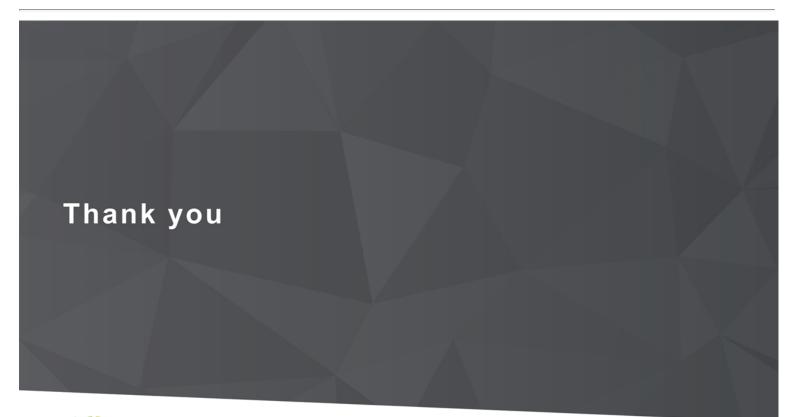








Significant team track record in discovery, development, approval of first-in-class medicines







#### Beam Therapeutics Reports Progress Across Base Editing Portfolio and Outlines Key Anticipated Milestones

BEACON Trial of BEAM-101 in Sickle Cell Disease Ongoing with Data from Multiple Patients Expected in 2024; Expansion Phase Initiation Expected in 2023

First Patient Dosing in BEAM-201 Trial in Patients with T-ALL/T-LL Expected by Mid-2023

Regulatory Submissions Planned for BEAM-301 by Late 2023/Early 2024 and BEAM-302 in Early 2024

North Carolina Manufacturing Facility Expected to Initiate GMP Operations in Late 2023

Approximately \$1 Billion Estimated Cash, Cash Equivalents and Marketable Securities at Year-End 2022; Cash Runway Expected into 2025, through Anticipated Key Milestones for Lead Programs and Long-Term Platform Opportunities

CAMBRIDGE, Mass., January 9, 2023 – Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported progress across the company's hematology, immunology-oncology and genetic disease portfolios and provided updates on anticipated upcoming milestones.

"Beam enters 2023 with significant momentum across all of our core pipeline areas and an expanding leadership position in the next generation of gene editing," said John Evans, chief executive officer of Beam. "We have multiple clinical-stage candidates with BEAM-101 and BEAM-201, another two candidates — BEAM-301 and BEAM-302 — moving toward clinical trials, and an integrated platform of editing technologies, scalable manufacturing capabilities and diverse delivery modalities. We are also making key investments in long-term platform opportunities that may dramatically expand the reach and impact of base editing and create a sustainable pipeline of highly differentiated programs, including improved conditioning for transplant, allogeneic cell therapies and in vivo delivery. I'm so proud of the accomplishments this organization has achieved in our first five years of operations. We believe we now have a unique opportunity to deliver on the potential of our science and achieve our mission of bringing life-changing treatments to patients suffering from serious diseases."

#### Hematology Portfolio

• BEAM-101: In November 2022, Beam enrolled the first patient in its BEACON clinical trial evaluating BEAM-101 as a treatment for sickle cell disease (SCD). Beam expects to complete enrollment in the sentinel cohort and initiate enrollment in the expansion cohort of BEACON in 2023, with plans to report data from multiple patients from one or both cohorts in 2024. BEAM-101 is a patient-specific, autologous hematopoietic stem cell (HSC) investigational therapy designed to offer a potentially best-in-class profile, incorporating base edits that are intended to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin. BEAM-101 aims to potentially alleviate the effects of SCD or beta-thalassemia by leading to increases in fetal hemoglobin, which is expected to restore the formation of a functional hemoglobin tetramer and, in the case of SCD, inhibit hemoglobin S polymerization.



Platform Opportunity: Beam is advancing its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning strategy in an effort to bring base editing treatments to more patients. ESCAPE aims to avoid toxicity challenges associated with currently available conditioning regimens for patients with SCD and beta-thalassemia ahead of autologous transplant. ESCAPE may also have applications in other diseases of the blood and immune system where transplant could deliver potential benefits but has been limited by toxicities associated with standard conditioning regimens. In December 2022, Beam presented in vivo proof-of-concept data at the American Society of Hematology Annual Meeting and Exposition (ASH) highlighting its potential. Beam has made significant investments in its ESCAPE platform and plans to continue its advancement in 2023.

#### Immunology-oncology Portfolio

- BEAM-201: In December 2022, Beam received clearance from the FDA for its Investigational New Drug (IND) application for BEAM-201. The company has initiated a first-in-human Phase 1/2 clinical trial to evaluate the safety and efficacy of BEAM-201 in patients with relapsed/refractory T-cell acute lymphoblastic leukemia (T-ALL)/T-cell lymphoblastic lymphoma (T-LL) and expects to dose the first patient by mid-2023. The Phase 1 portion of the trial is expected to include up to 48 patients between the ages of 18 and 50, followed by a Phase 2 portion with approximately 48 patients. Key safety endpoints for the trial include treatment-emergent and treatment-related adverse events, and key efficacy endpoints include proportion of patients with complete or partial responses, proportion eligible for HSC transplant, and proportion achieving minimal residual disease negative status. Beam believes that BEAM-201 is the first quadruple-edited, allogeneic CAR-T cell investigational therapy in clinical-stage development. BEAM-201 is designed to target CD7 to treat relapsed/refractory T-ALL/T-LL, a severe disease affecting children and adults.
- Platform Opportunity: Beyond BEAM-201, Beam continues to research potential next-generation allogeneic strategies that could
  dramatically expand the utility and accessibility of cell therapies in cancer and other diseases. Beam anticipates that multiple edits will be
  required to enable allogeneic cells to successfully avoid immune rejection and provide the cells with other desirable properties. Beam
  believes that multiplex base editing, with its high potency, efficiency in editing and lack of double-strand breaks, is well suited for making
  such highly engineered cells, and anticipates providing additional updates on this research in 2023.

#### Genetic Disease Portfolio

• BEAM-301: IND-enabling studies for BEAM-301 continue, and by late 2023 or early 2024, the company plans to submit a regulatory application for authorization to initiate clinical trials for the program. BEAM-301 is a liver-targeting lipid nanoparticle (LNP) formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation which results in the most severe form of glycogen storage disease 1a (GSDIa). GSDIa is an autosomal recessive disorder caused by mutations in the G6PC gene that disrupt a key enzyme, glucose-6-phosphatase, critical for maintaining glucose homeostasis. Patients with this mutation typically require ongoing corn starch administration, without which, they may enter into hypoglycemic shock within one to three hours.



- BEAM-302: Beam also continues to advance its second liver-targeted in vivo program, BEAM-302, and in early 2024, plans to submit a
  regulatory application for authorization to initiate clinical trials for the program. BEAM-302 is designed to offer a one-time treatment to
  genetically correct the E342K point mutation (PiZZ genotype), which is most commonly responsible for severe alpha-1 antitrypsin
  deficiency (AATD). AATD is an inherited genetic disorder that can cause early onset emphysema and liver disease.
- Platform Opportunity: Beam continues to advance its LNP delivery technologies using its barcode screening technology, which is
  designed to enable delivery of base editing treatments to the liver and tissues beyond, potentially expanding the number of diseases and
  patients that could benefit from base editing medicines.

#### **Manufacturing Facility**

 Beam continues to expect operations at its North Carolina manufacturing facility to commence in the first quarter of 2023 and expects to initiate current good manufacturing practice compliant operations in late 2023.

#### Cash Position and Runway

- Cash Position: Beam estimates that it had cash, cash equivalents and marketable securities of approximately \$1.0 billion as of
  December 31, 2022. 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, 2022,
  and its results of operations for the three months and year ended December 31, 2022. Accordingly, undue reliance should not be placed on
  this preliminary estimate.
- Cash Runway: Beam expects that its cash, cash equivalents and marketable securities as of December 31, 2022, will enable the company
  to fund its anticipated operating expenses and capital expenditure requirements at least into 2025. This expectation includes funding
  directed toward reaching each of the key 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 41st Annual J.P. Morgan Healthcare Conference today, Monday, January 9, 2023, at 11:15 a.m. PT. A live webcast will be available in the investor section of the company's website at <a href="https://www.beamtx.com">www.beamtx.com</a> 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 rans pies rectace contains to water-tooking statements within the including, but not limited to, statements related to: our upcoming presentations at the 41st 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, GSDIa, T-ALL/TLL, AATD and our conditioning regimens; the clinical trial design for BEAM-201; our plans, and anticipated timing, to advance our programs; our estimated cash, cash equivalents and marketable securities as of December 31, 2022 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 potential impact of the COVID-19 pandemic, including its 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 enrollment and initiation 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; whether our actual audited results will be consistent with our estimated cash, cash equivalents and marketable securities as of December 31, 2022; 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, 2021, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, 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.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference in this press release.



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