

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 09, 2022

**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

26 Landsdowne Street  
Cambridge, Massachusetts 02139  
(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.**

On January 10, 2022, in connection with its announcement of the Collaboration (as defined below) with Pfizer Inc. ("Pfizer"), Beam Therapeutics Inc. ("Beam") announced certain financial information for the year ended December 31, 2021. Beam estimates that, on a preliminary and unaudited pro forma basis, which includes the \$300 million upfront payment received in January 2022 from Pfizer in connection with entry into the Collaboration, it had cash, cash equivalents and marketable securities of approximately \$1.2 billion as of December 31, 2021. This estimate is based on Beam's expectation that it will report actual cash, cash equivalents and marketable securities of approximately \$0.9 billion as of December 31, 2021. This estimate of Beam's cash, cash equivalents and marketable securities and pro forma cash, cash equivalents and marketable securities as of December 31, 2021 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, 2021, and its results of operations for the three months and year ended December 31, 2021. 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.**

Beam will be conducting meetings with participants attending the 40<sup>th</sup> Annual J.P. Morgan Healthcare Conference. The slides to be presented by Beam 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.**

### ***Press Release***

On January 9, 2022, Beam issued a press release announcing future research and development goals for its clinical and preclinical programs. The full text of this 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 websites referenced in the press release is not incorporated herein.

### ***Collaboration with Pfizer Inc.***

On January 10, 2022, Beam announced that it has entered into an exclusive four-year research collaboration (the "Collaboration") with Pfizer focused on *in vivo* base editing programs for three targets for rare genetic diseases of the liver, muscle and central nervous system. The base editing programs to be evaluated as part of the Collaboration will leverage Beam's proprietary *in vivo* delivery technologies to deliver base editors to target organs. Under the terms of the Collaboration agreement, Beam will conduct all research activities through development candidate selection for three undisclosed targets, which are not included in Beam's existing programs. Pfizer may opt in to exclusive, worldwide licenses to each development candidate, after which it will be responsible for all development activities, as well as potential regulatory approvals and commercialization, for each such candidate. Beam has a right to opt in, at the end of Phase 1/2 studies, upon the payment of an option exercise fee, to a global co-development and co-commercialization agreement with respect to one program licensed under the Collaboration pursuant to which Pfizer and Beam would share net profits as well as development and commercialization costs in a 65%/35% ratio (Pfizer/Beam). Beam received an upfront payment of \$300 million and, assuming Pfizer exercises its opt-in license rights for all three targets, is eligible for development, regulatory and commercial milestone payments for potential total deal consideration of up to \$1.35 billion. Beam is also eligible to receive royalties on global net sales for each licensed program. The Collaboration has an initial term of four years and may be extended up to one additional year.

### ***Cautionary Note Regarding Forward-Looking Statements***

Statements in this Current Report on Form 8-K and in the exhibits incorporated herein by reference 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 concerning activities under Beam's research collaboration with Pfizer, future payments Beam may receive under such collaboration, Beam's participation in the J.P. Morgan Healthcare Conference; Beam's estimated cash, cash equivalents and marketable securities as of December 31, 2021 (actual and pro forma) and its expectations related thereto; the initiation, timing, progress and results of preclinical studies and research and development programs, including the initiation and progress of clinical trials, including Beam's anticipated Phase 1/2 trial designed to assess the safety and efficacy of BEAM-101 for the treatment of sickle

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cell disease; the advancement of Beam's pipeline, including the submission of INDs for BEAM-102 and BEAM-201, and the advancement of BEAM-102, BEAM-201, BEAM-301, additional CAR-T and liver programs, and Stargardt disease program in multiple preclinical studies; the potential activities under license and collaboration agreements and the formation of new collaborations; and the therapeutic applications and potential of Beam's technology, including Beam's potential to develop life-long, curative, precision genetic medicines for patients through base editing, including potential safety advantages. 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. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: Beam's ability to develop, obtain regulatory approval for, and commercialize its product candidates, which may take longer or cost more than planned; Beam's ability to raise additional funding, which may not be available; Beam's ability to obtain, maintain and enforce patent and other intellectual property protection for its product candidates; the potential impact of the COVID-19 pandemic; that preclinical testing of Beam'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 Beam's clinical trials may take longer than expected; that Beam's product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; whether the research collaboration with Pfizer will result in programs that are licensed from Beam; whether any product candidates that arise from these programs or are otherwise developed by Beam will advance into clinical trials or through the clinical trial process on a timely basis or at all; whether the results of clinical trials of product candidates will warrant submissions for regulatory approval or regulatory approval; whether any products that receive regulatory approval will be successfully distributed and marketed; whether Beam's actual audited results will be consistent with its estimated cash, cash equivalents and marketable securities as of December 31, 2021; and other factors discussed in the "Risk Factors" section of Beam's Annual Report on Form 10-K for the year ended December 31, 2020, Beam's Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, Beam's Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, Beam's Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, and in any subsequent filings with the Securities and Exchange Commission. Any forward-looking statements contained in this Current Report on Form 8-K and in the exhibits incorporated herein by reference speak only as of the date hereof, and Beam specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

#### Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

<b>Exhibit No.</b>	<b>Description</b>
99.1	<a href="#">Beam Therapeutics Inc. Presentation at the 40<sup>th</sup> Annual J.P. Morgan Healthcare Conference</a>
99.2	<a href="#">Press Release Issued by Beam Therapeutics Inc. on January 9, 2022</a>
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 thereunto duly authorized.

**BEAM THERAPEUTICS INC.**

Date: January 10, 2022

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

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# Beam Therapeutics

PRECISION GENETIC MEDICINES THROUGH BASE EDITING

NASDAQ: BEAM

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## 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 anticipated Phase 1/2 trial designed to assess the safety and efficacy of BEAM-101 for the treatment of sickle cell disease, which we refer to as our BEACON-101 trial; the advancement of our pipeline, including the submission of INDs for BEAM-102 and BEAM-201, and the advancement of BEAM-102, BEAM-201, BEAM-301, additional CAR-T and liver programs, and Stargardt disease program in multiple preclinical studies; our current expectations and anticipated results of operations, including our estimated cash balance as of the end of 2021 and our expected use of capital; the potential activities 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 the COVID-19 pandemic; 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, 2020, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, 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](http://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

- ▶ Coming era of **one-time, curative therapies**
- ▶ Gene editing for **rare and common diseases**
- ▶ Platform for **rapidly-programmable precision medicines**

# Base editing is a next-generation approach to gene editing with single base precision

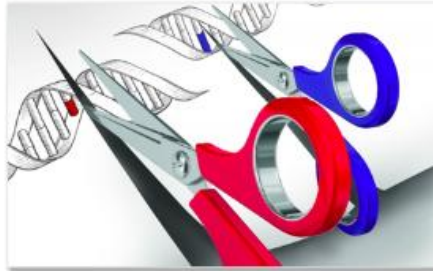
## Nuclease editing

CRISPR, Zinc Fingers, TALEs

## Base editing



Precise targeting?	▶ <b>Yes (guide RNA or ZF/TALE)</b>	▶ <b>Yes (guide RNA)</b>
Double strand breaks?	▶ <b>Yes</b>	▶ <b>No</b>
Editing predictability?	▶ <b>Random insertions and deletions</b>	▶ <b>Predictable single base changes</b>





## Rare disease

Over half of genetic disease mutations are point mutations



## Common disease

Single base changes drive risk and protection from common diseases

THE NEW ENGLAND JOURNAL OF MEDICINE

ORIGINAL ARTICLE

### Sequence Variations in PCSK9, Low LDL, and Protection against Coronary Heart Disease

Jonathan C. Cohen, Ph.D., Eric Boerwinkle, Ph.D., Thomas H. Mosley, Jr., Ph.D., and Helen H. Hobbs, M.D.

### A Protein-Truncating HSD17B13 Variant and Protection from Chronic Liver Disease

Nouso S. Abul-Husn, M.D., Ph.D., Xipang Cheng, M.D., Ph.D., Alexander H. Li, Ph.D., Yuning Xie, Ph.D., Claudia S. Schumann, Ph.D., Panayiotis Steiris, Ph.D., Yashu Liu, Ph.D., Julia Kozlinska, Ph.D., Stefan Stender, M.D., Ph.D., G. Craig Wood, M.S., Ann N. Stepanchik, Ph.D., Matthew D. Selk, et al.

### Gene mutation defends against Alzheimer's disease

Rare genetic variant suggests a cause and treatment for cognitive decline.

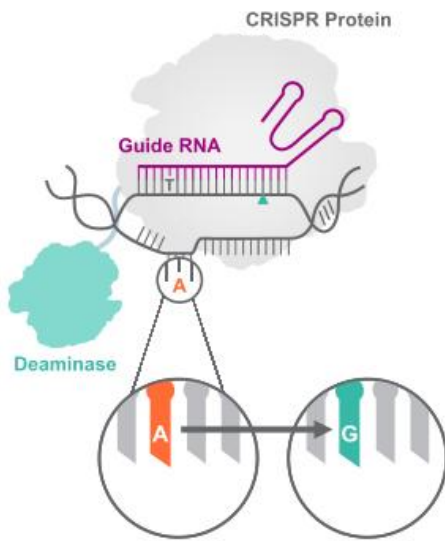


International Journal of  
Neonatal Screening



Review  
**Sickle Cell Disease—Genetics, Pathophysiology,  
Clinical Presentation and Treatment**

# Base editing is a highly-differentiated, potentially best-in-class gene editing technology



## **B** Biology

- ▶ Direct, durable editing of single DNA base pairs

## **A** Application

- ▶ Gene correction, activation, silencing, modification
- ▶ Simultaneous “multiplex” editing at many sites

## **S** Specificity

- ▶ Highly specific and predictable editing profile
- ▶ Avoid genotoxicity and chromosomal aberrations associated with double-stranded DNA breaks

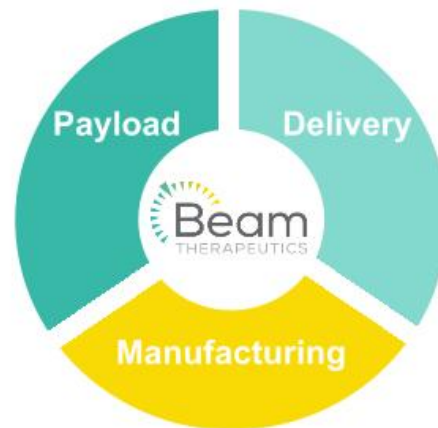
## **E** Efficiency

- ▶ High levels of editing in any cell type, including non-dividing cells

# We are establishing a leading platform for precision genetic medicine

## Suite of gene editing technologies

- ▶ Base editing
- ▶ Nuclease editing
- ▶ RNA editing
- ▶ Prime editing



## Suite of delivery technologies

- ▶ Autologous cell therapy
- ▶ Allogeneic cell therapy
- ▶ mRNA
- ▶ LNP vectors
- ▶ Viral vectors

## Wholly-owned manufacturing capability

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

## Strategic collaboration with Pfizer for *in vivo* base editing



Leadership in next-generation base editing technology and mRNA/LNP delivery platform



Global leader in design, development, and commercialization of novel medicines, including mRNA/LNP expertise

- ▶ \$300M upfront
- ▶ \$1B+ in potential milestones
- ▶ 4-year research term; Pfizer option at DC nomination
- ▶ 3 targets, not included in Beam's current programs
- ▶ Leverages Beam delivery technologies to target liver, muscle, CNS
- ▶ Beam option at end of P1/2 for 35% WW cost/net profit split on any one program

**Including the upfront payment from this deal, our cash<sup>1</sup> balance as of year-end 2021 was ~\$1.2 billion<sup>2</sup>**

1. Cash, cash equivalents and marketable securities; 2. Amount is preliminary, has not been audited and is subject to change upon completion of the audit of our consolidated financial statements as of and for the year ended December 31, 2021.

## Additional strategic collaborations broaden therapeutic opportunities and unlock value in Beam platform



- ▶ Base editing for the prevention of cardiovascular disease
- ▶ Beam opt-in to 50% of US rights after Phase 1



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



- ▶ Non-exclusive out-license of Cas12b nuclease applications\* (eg, CAR insertion) for certain engineered cell therapies
- ▶ \$50M upfront



- ▶ Non-exclusive collaboration for non-genotoxic conditioning in combination with BEAM-101 and BEAM-102



- ▶ Exclusive rights to prime editing for transition mutations (~30% of all mutations) and sickle correction

\*Excludes base editing

# Diversified portfolio of base editing programs



DELIVERY	PROGRAM / DISEASE		EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
Ex vivo HSCs	BEAM-101	Sickle Cell Disease Beta Thalassemia	Activation of fetal hemoglobin					
	BEAM-102	Sickle Cell Disease	Correction of HbS sickle mutation					
Ex vivo T cells	BEAM-201	T-cell ALL CD7+ AML	Multiplex silenced CD7 CAR-T					
		T-cell Lymphoma	Multiplex silenced CD5 CAR-T					
In vivo LNP	BEAM-301	Glycogen Storage Disease Ia	Correction of R83C mutation					
		Alpha-1 Antitrypsin Deficiency	Correction of E342K mutation					
		Glycogen Storage Disease Ia	Correction of Q347X mutation					
		Hepatitis B Virus	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; ALL = Acute Lymphoblastic Leukemia; AML = Acute Myeloid Leukemia

# Key progress and anticipated milestones



## 2021 Achievements

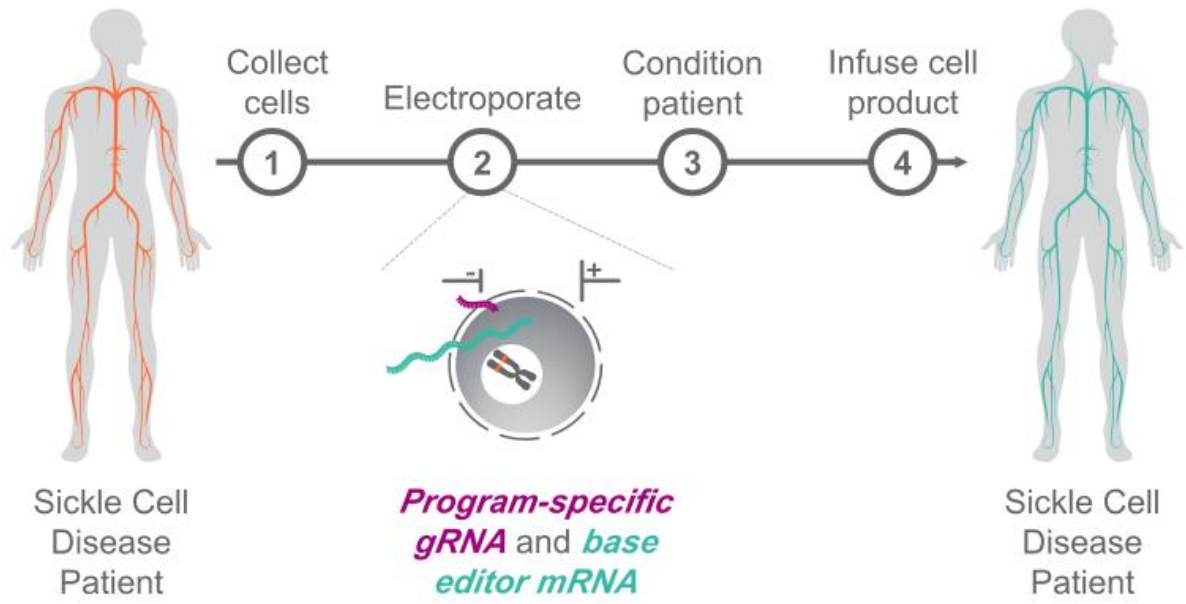
- FDA clearance of the **BEAM-101** IND
- IND-enabling studies for **BEAM-102**
- IND-enabling studies for **BEAM-201**
- Beam LNP data in non-human primates
- First liver DC, **BEAM-301**: GSDIa R83C
- Non-human primate studies for Stargardt
- Apellis and Sana partnerships

## 2022 Milestones

- First subject enrolled for **BEAM-101** in 2H 2022
- Submit IND for **BEAM-102** in 2H 2022
- Submit IND for **BEAM-201** in 2H 2022
- Nominate 2<sup>nd</sup> CAR-T development candidate
- Initiate IND-enabling studies for **BEAM-301**
- Nominate 2<sup>nd</sup> liver development candidate
- Form additional strategic platform partnerships (Pfizer)



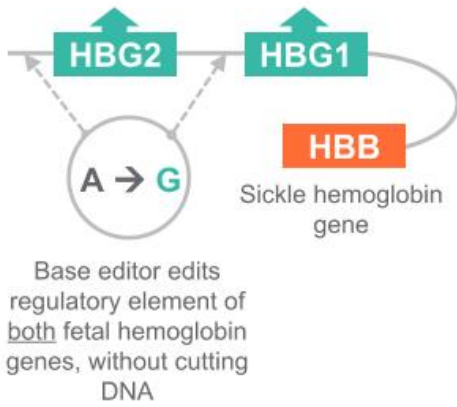
# Autologous *ex vivo* cell process for editing hematopoietic stem cells



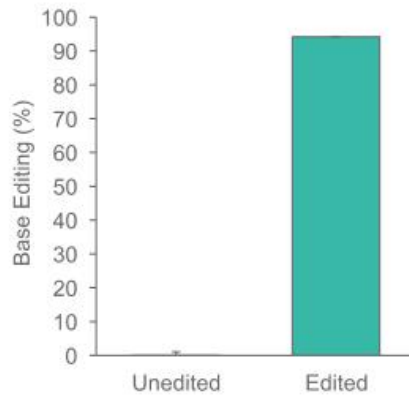


# BEAM-101: High levels of editing and robust HbF induction after long-term *in vivo* engraftment

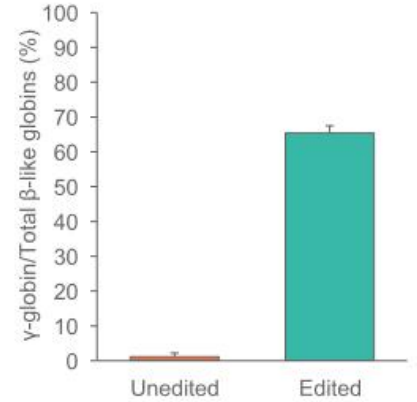
Sickle Cell Disease: 100,000 patients in the US; severe pain, organ damage, early mortality



>90% base editing at *HBG1/2* promoters in multilineage cells<sup>1</sup>



>65% gamma globin protein levels in sorted erythroid cells<sup>2</sup>

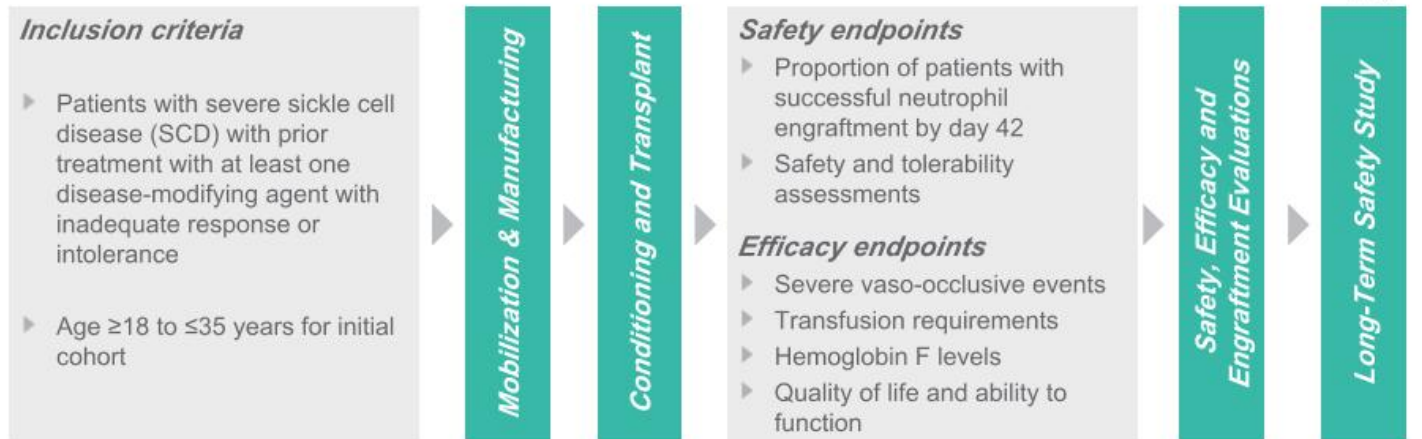


Presented at ASGCT 2020: Edited human HSPCs analyzed 16 weeks after infusion in NBSGW mice (Mean±SEM, n=4-6);  
1. Sorted human Lineage-CD34+ bulk bone marrow; 2. Sorted erythroid cells (GlyA+)

# BEAM-101 is the first clinical base editing program

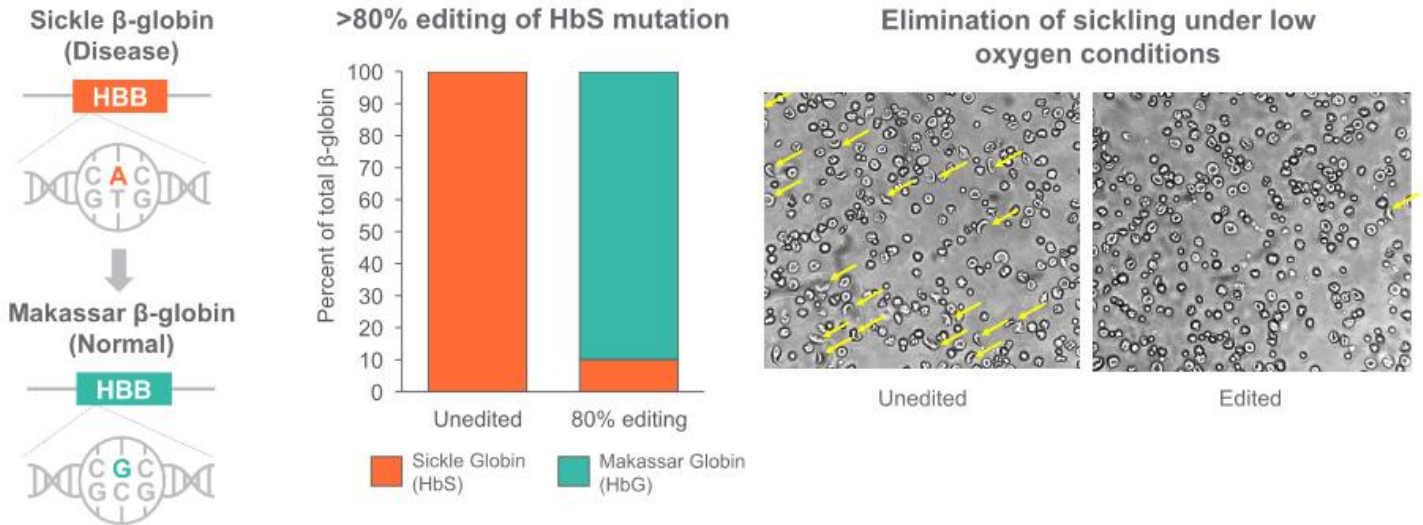


## BEACON-101 Phase 1/2 Study Design



# BEAM-102: High editing of sickle mutation led to significant elimination of HbS globin in patient donor cells

Sickle Cell Disease: 100,000 patients in the US; severe pain, organ damage, early mortality

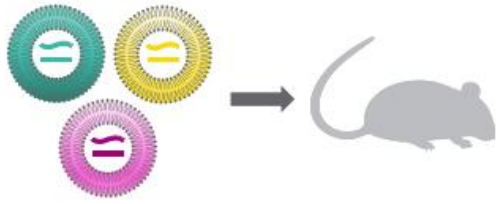


Presented at ASGCT 2020; Sickle (HbS) and Makassar variant globin protein, at varying bulk levels of Makassar editing assessed by NGS, was measured by UPLC and expressed as a fraction of total beta globin in 18 day mature RBCs derived from edited HbSS CD34+s. UPLC was conducted on n = 2 for each bulk editing condition. CD34+ HbSS cells were edited and subsequently differentiated to generate mature erythroid red blood cells and exposed to low oxygen conditions (<2%) in a hypoxic chamber. Image is representative of n=2 different sickling assays from n=2 independent donors that were successfully edited at high levels (>80% by NGS) and confirmed to have near 90% Makassar globin by UPLC.

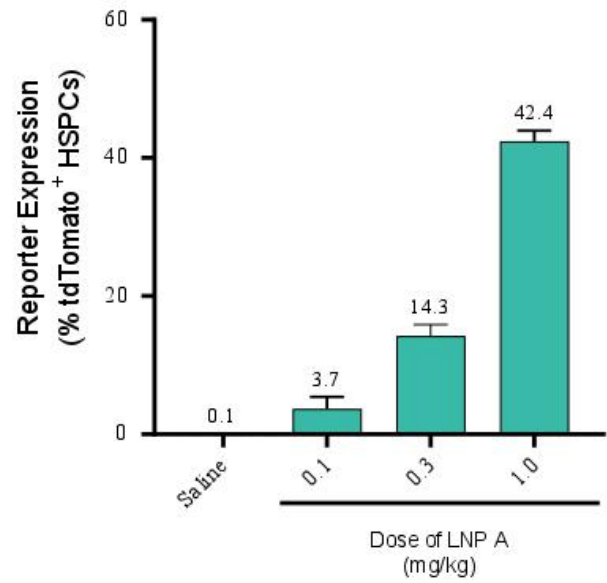
# Uniquely positioned to potentially create best-in-class regimens for SCD patients, now and in the future



# Developing LNPs for the delivery of mRNA to Hematopoietic Stem & Progenitor Cells (HSPCs)

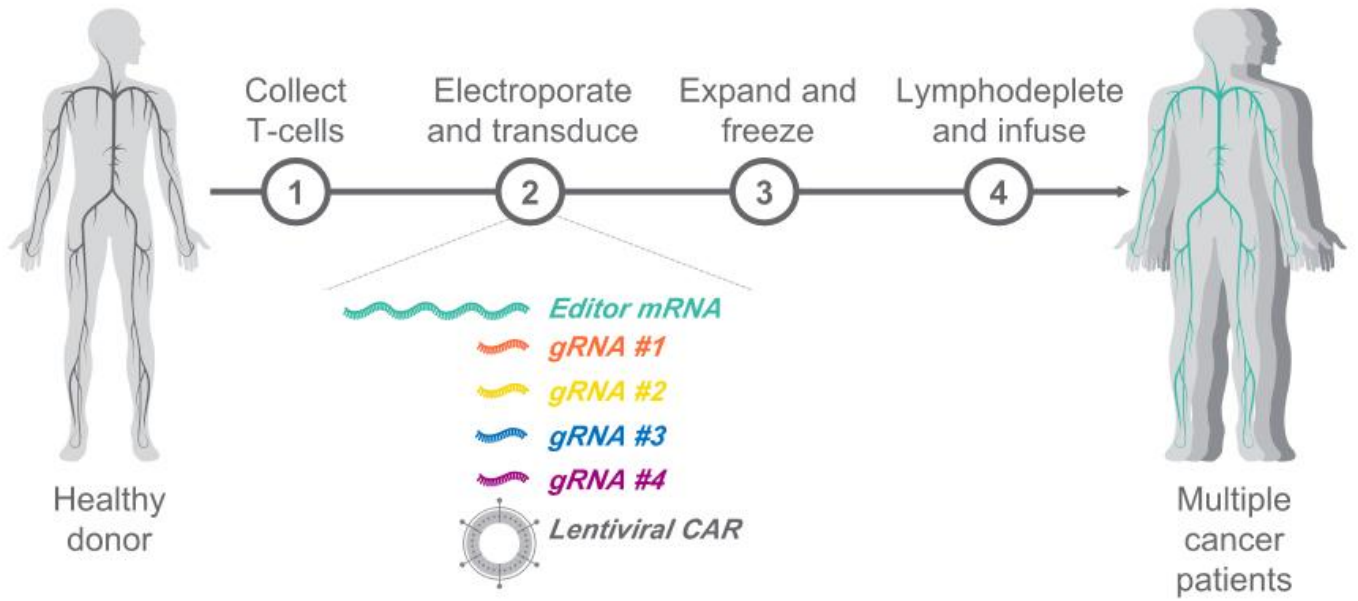


- ▶ Proprietary technology for high throughput LNP screening for delivery beyond the liver
- ▶ Each nanoparticle contains mRNA payload plus a unique DNA barcode
- ▶ Simultaneous in vivo screening of LNPs to select formulations capable of targeting diverse tissues



Presented at TIDES 2021; Cre-reporter mice (N=2-4);

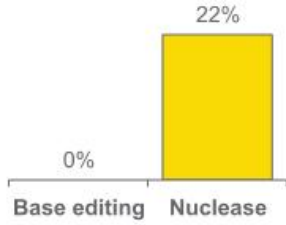
# Allogeneic multiplex edited CAR-T cell process



# Significant advantages of multiplex base editing without double strand breaks

## Chromosomal rearrangements

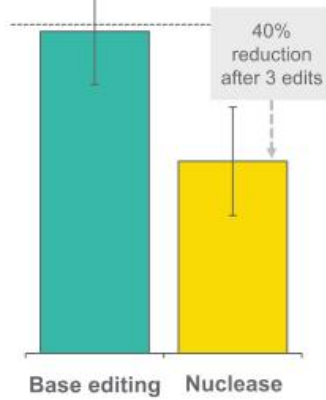
Percent of cells with translocations<sup>1</sup>



4 edits: TRAC, CD52, PD1, CD7

## Impact on cell expansion

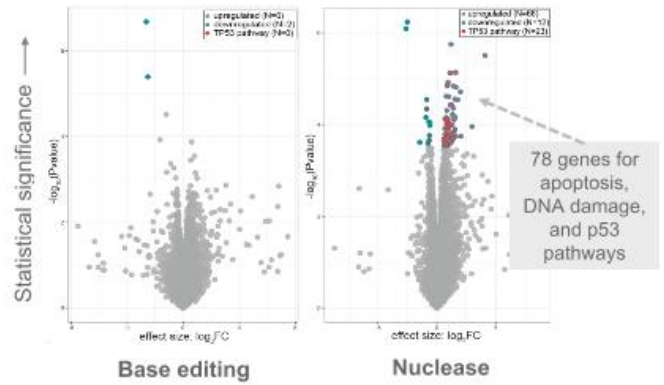
Percent yield after editing<sup>2</sup>



3 edits: TRAC, B2M, PD1

## DNA damage response to editing (apoptosis and p53 pathway)

Gene expression changes after editing



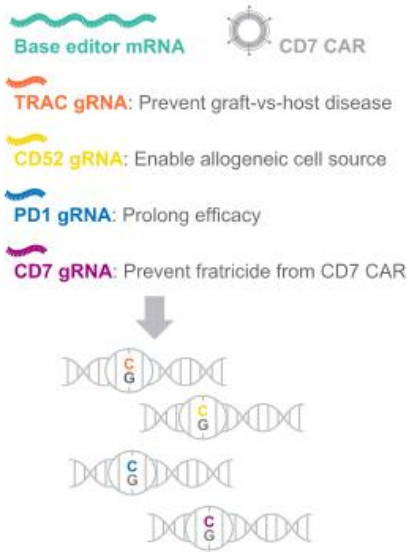
4 edits: TRAC, CD52, PD1, CD7

1. Base editing versus nuclease editing with the same four guide RNAs measured via G-banded karyotypes from 100 cells; 2. Extensive guide screen across three targets, with BE4 and spCas9 sgRNAs selected for high editing efficiency and expansion in single-plex test, final cell yields compared between 3 edits, normalized to electroporation only control;

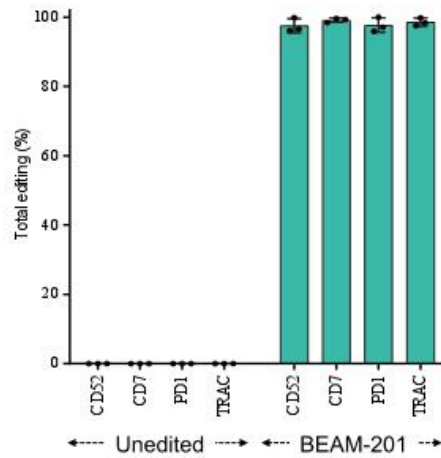


# BEAM-201: High level of cell engineering enabled by simultaneous multiplex base editing of four genes

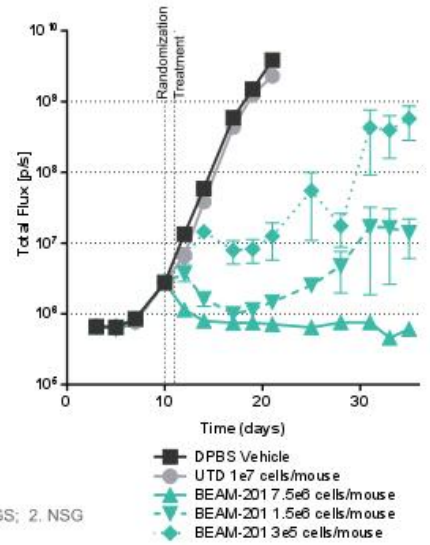
## T-Cell Acute Leukemia: 15% of ALL, not treated by B-cell CARTs



Clinical process yields 96-99% editing, >90% quad edited<sup>1</sup>



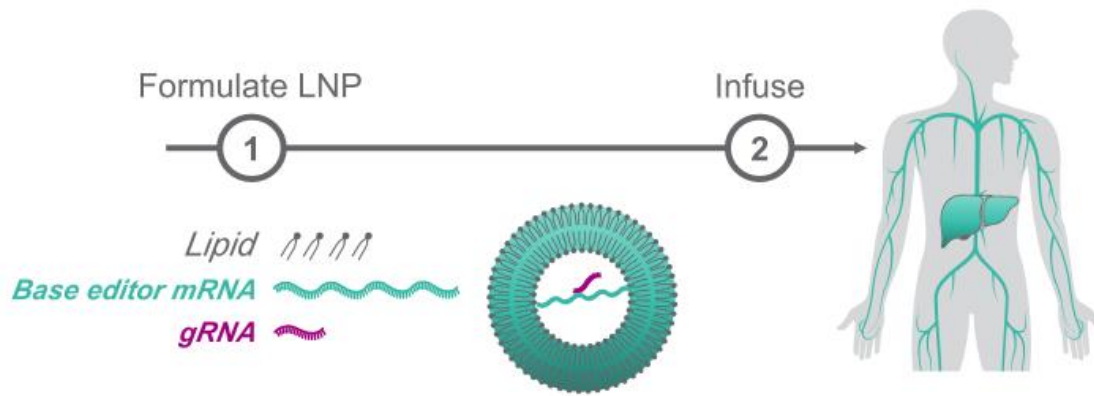
Potent in vivo tumor clearance or control across a 25-fold dose<sup>2</sup>



Presented at SITC 2020; 1. Simultaneous base editing at four target loci using clinical-scale process as measured by NGS; 2. NSG mice bearing CCRF-CEM-GFP-Luc tumors (Gomez-Silva et al, 2017)



# Non-viral delivery for *in vivo* base editing in liver

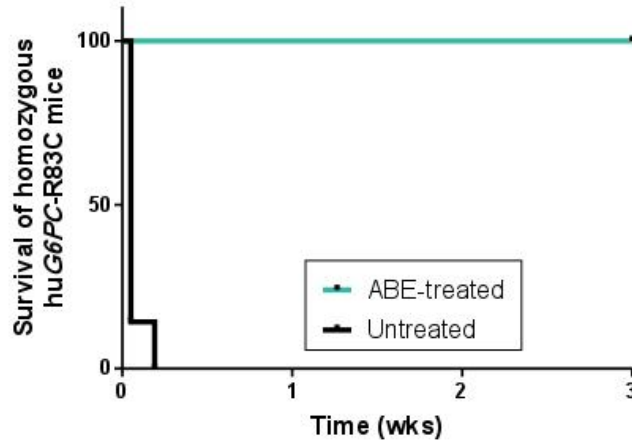
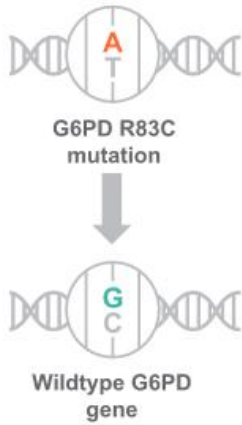


- ▶ Clinically validated technology for transient, *in vivo* delivery to the liver
- ▶ Scalable manufacturing with lower COGS
- ▶ **Proprietary Beam formulation showed up to 60% editing in NHPs at clinically-relevant dose of 1.0 mpk**

# BEAM-301: ABE correction of GSDIa R83C mutation associated with improved survival of R83C mice

Glycogen Storage Disease Ia: 900 patients in US with R83C; life-threatening hypoglycemia

## Kaplan-Meier Survival Estimates<sup>1</sup>

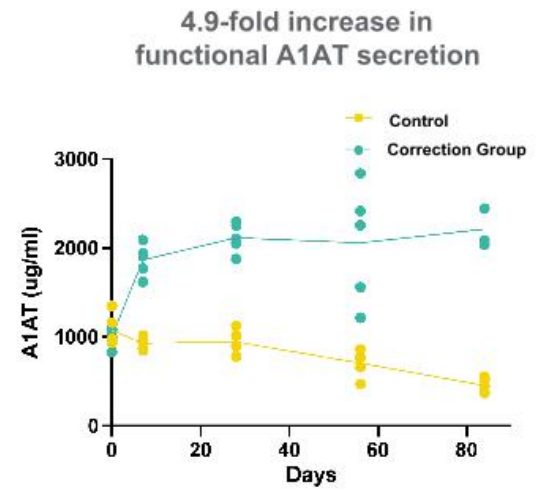


- ▶ DC nomination in Dec 2021 – Beam's first *in vivo* DC
- ▶ Near-normal serum metabolites, G6PC activity, hepatic morphology and lipid deposition
- ▶ Animal models suggest 11% editing may be sufficient for clinical benefit<sup>2</sup>

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; 2. Chou & Mansfield. 2007. Curr. Gen. Ther.

# In vivo direct correction of A1AT mutation with base editing designed to address liver and lung disease

Alpha-1 Anti-trypsin Deficiency: 60,000 ZZ patients in US<sup>1</sup>; severe progressive lung and liver disease



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

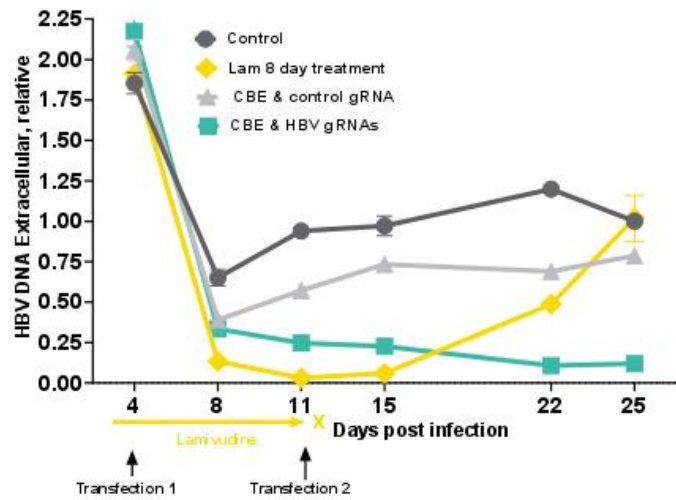
1. The most severe form of Alpha-1 arises when a patient has an E342K (PiZ) point mutation in both copies of the SERPINA1 gene, where two copies are designated ZZ.

# Multiplex base editing of hepatitis B virus genome reduced viral markers and prevented rebound

Hepatitis B: 850,000 US patients living with chronic hepatitis B; nearly 300 million worldwide

- ▶ Current antivirals do not eliminate the HBV genome, leading to viral rebound and preventing cure
- ▶ Multiplex base editing has potential to silence covalently closed circular DNA (cccDNA)
- ▶ Base editing also has potential to silence HBV integrated in human genome, without fear of chromosomal rearrangements caused by double-stranded DNA breaks

Base editing caused reduction of viral antigens and prevention of viral rebound, unlike lamivudine



Presented at International HBV Meeting 2021; Data shown from primary hepatocyte co-cultures;

# Meet the Beam Team



**John Evans**  
Chief Executive Officer



**Giuseppe Ciaramella, PhD**  
President, Chief Scientific Officer



**Terry-Ann Burrell**  
Chief Financial Officer



**Amy Simon, MD**  
Chief Medical Officer



**Courtney Wallace**  
Chief Business Officer



**Christine Bellon, PhD, JD**  
Chief Legal Officer



**Susan O'Connor**  
Chief Human Resources Officer



**Suzanne Fleming**  
Chief Accounting Officer



**Brian Riley**  
SVP, Technical Operations



**Manmohan Singh, PhD**  
SVP, Pharmaceutical Sciences and Delivery Technologies



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

Thank you





## Beam Therapeutics Reports Progress Across *Ex Vivo* and *In Vivo* Pipeline of Base Editing Therapeutics and Outlines Key Anticipated 2022 Milestones

*First Subject Anticipated to be Enrolled in BEAM-101 Phase 1/2 Clinical Trial for the Treatment of Sickle Cell Disease in the Second Half of 2022*

*BEAM-301 Named as Fourth Development Candidate for the Treatment of Glycogen Storage Disease Type Ia*

*Nomination of Two Additional Development Candidates Anticipated in 2022*

*Company to Present Pipeline and Business Updates at 40<sup>th</sup> Annual J.P. Morgan Healthcare Conference on January 10, 2022, at 2:15 p.m. ET.*

**CAMBRIDGE, Mass., January 09, 2022** - Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today outlined anticipated 2022 milestones across its *ex vivo* programs targeting editing of hematopoietic stem cells (HSCs) and T cells and *in vivo* programs targeting editing of liver cells leveraging lipid nanoparticles (LNPs) for delivery. Updates include that the company has selected its fourth development candidate and first *in vivo* base editing candidate, BEAM-301, which aims to correct the R83C mutation for the potential treatment of patients with glycogen storage disorder Ia (GSDIa).

“We made significant progress across our base editing portfolio in 2021, which culminated in U.S. Food and Drug Administration clearance of the first investigational new drug application of a base editing therapeutic, BEAM-101. We also further expanded our platform, particularly with LNP delivery of base editors to the liver and our proprietary technology for accelerating LNP delivery to other tissues, including HSCs,” said John Evans, chief executive officer of Beam. “We believe 2022 is set to be our most important year yet, with preparations underway to launch the BEACON-101 clinical trial with BEAM-101 for the treatment of sickle cell disease and to complete our transition to becoming a clinical-stage company. We believe we are well positioned today, with four development candidates, a rich pipeline of earlier stage programs, and an industry-leading platform of editing and delivery technologies enabling us to bring forward a new class of precision genetic medicines. None of this would be possible without the commitment of our remarkable team of fearless innovators. We look forward to the year ahead and continuing our work to bring potentially life-changing medicines to as many patients as possible.”

### ***Ex Vivo* HSC Programs**

- BEAM-101 is a patient-specific, autologous HSC investigational therapy, which incorporates base edits that are designed to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin. BEAM-101 aims to potentially alleviate the effects of mutations causing sickle cell disease (SCD) or



beta-thalassemia by leading to increases in fetal hemoglobin, which inhibits hemoglobin S (HbS) polymerization. The BEACON-101 trial is a Phase 1/2 clinical trial designed to assess the safety and efficacy of BEAM-101 for the treatment of SCD. The trial is expected to include an initial “sentinel” cohort of three patients, treated one at a time to confirm successful engraftment, followed by dosing in up to a total of 45 patients. Beam has begun site selection and the institutional review board approval processes for the BEACON-101 trial and plans to enroll the first subject in the second half of 2022.

- BEAM-102 is designed to treat SCD by directly editing the causative HbS point mutation to recreate a naturally occurring normal human hemoglobin variant, HbG-Makassar. The Makassar variant has been reported to have the same function as the more common HbA variant and does not cause SCD. Beam plans to submit an investigational new drug (IND) application for BEAM-102 in the second half of 2022.

#### **Ex Vivo T Cell Programs**

- BEAM-201 is a multiplex base edited anti-CD7 CAR-T cell investigational therapy designed to treat relapsed/refractory T-cell acute lymphoblastic leukemia, a severe disease affecting children and adults. Beam plans to submit an IND application for BEAM-201 in the second half of 2022.
- Beam plans to nominate a second CAR-T development candidate in 2022.

#### **In Vivo LNP Liver-targeting Programs**

- BEAM-301, the company’s newest development candidate, is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation. R83C is the most common disease-causing mutation of GSDIa, a life-altering genetic disease with no approved disease-modifying treatments available today. Beam anticipates initiating IND-enabling studies for BEAM-301 in 2022.
- Beam plans to nominate a second liver-targeted development candidate in 2022.

#### **J.P. Morgan Healthcare Conference**

Mr. Evans will present Beam’s pipeline and business updates during a presentation at the 40<sup>th</sup> Annual J.P. Morgan Healthcare Conference on Monday, January 10, 2022, at 2:15 p.m. ET. A live webcast will be available in the investor section of the company’s website at [www.beamtx.com](http://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 enables precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This enables a wide range of potential therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is

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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 plans, and anticipated timing, to nominate additional development candidates, initiate IND-enabling studies, and submit IND applications; the therapeutic applications and potential of our technology, including with respect to sickle cell disease, beta-thalassemia, T-ALL, GSDIa, and LNPs; the planned initiation and design of our BEACON-101 clinical trial, including the timing of enrolling the first subject in the trial; our planned presentations at an upcoming conference; 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; 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 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” in our Annual Report on Form 10-K for the year ended December 31, 2020, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, 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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