Beam Therapeutics

PRECISION GENETIC MEDICINES THROUGH BASE EDITING

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 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.

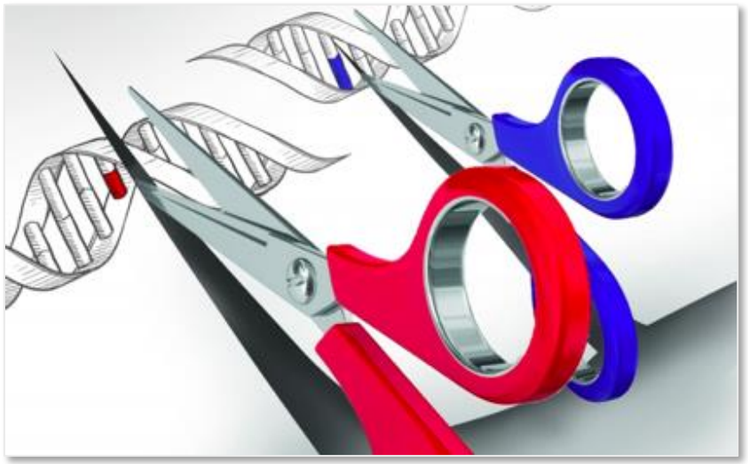
Each forward-looking statement is subject to important risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval 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. 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

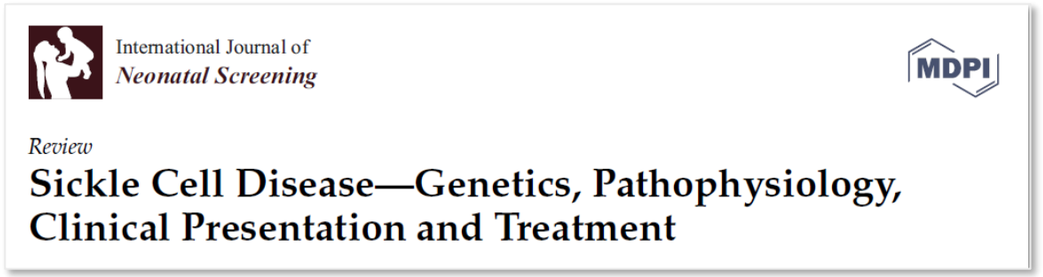
	Nuclease editing CRISPR, Zinc Fingers, TALEs	Base editing
Precise targeting?	Yes (guide RNA or ZF/TALE)	Yes (guide RNA)
Double strand breaks?	Yes	No
Editing predictability?	Random insertions and deletions	Predictable single base changes



Single base DNA variants drive health outcomes

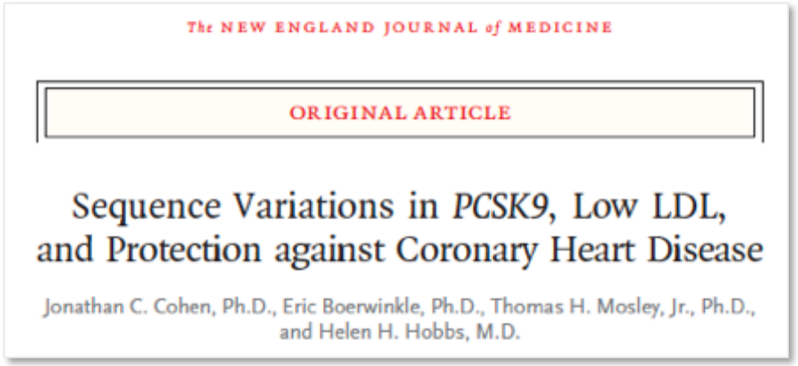
Rare disease

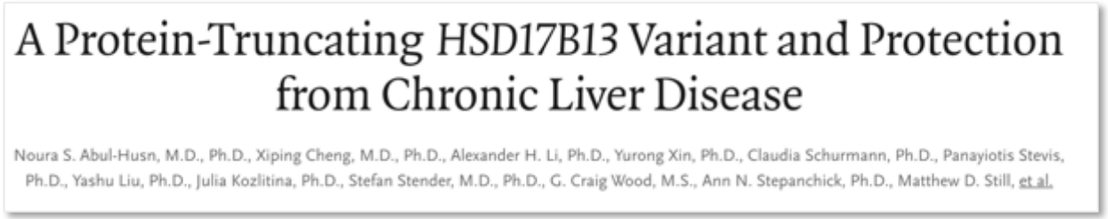
Over half of genetic disease mutations are point mutations



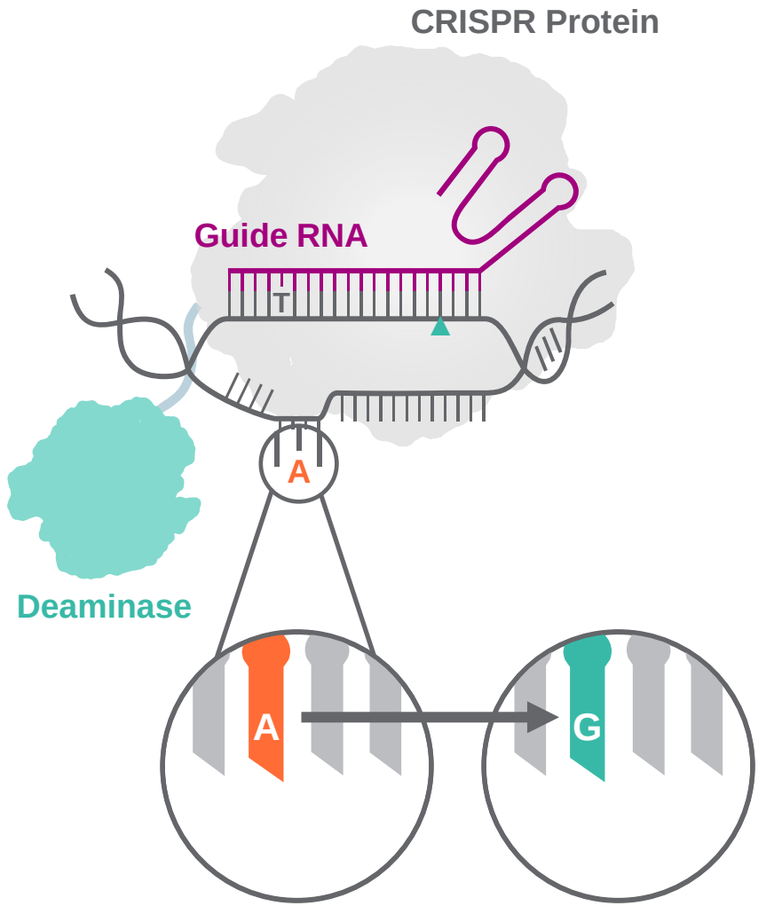
Common disease

Single base changes drive risk and protection from common diseases





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

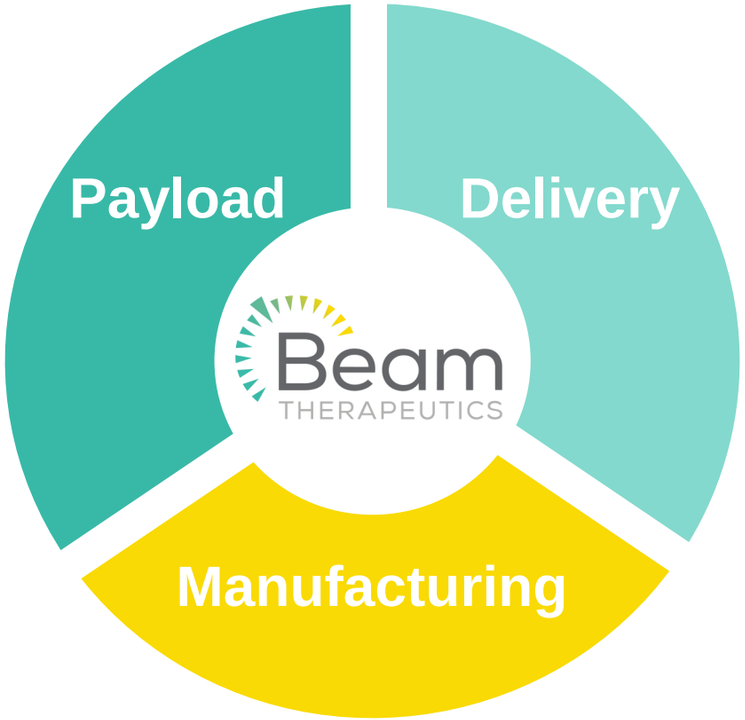
E Efficiency

- 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

Beam THERAPEUTICS

Beam THERAPEUTICS

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

Pfizer

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[1] balance as of year-end 2021 was ~$1.2 billion[2]

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

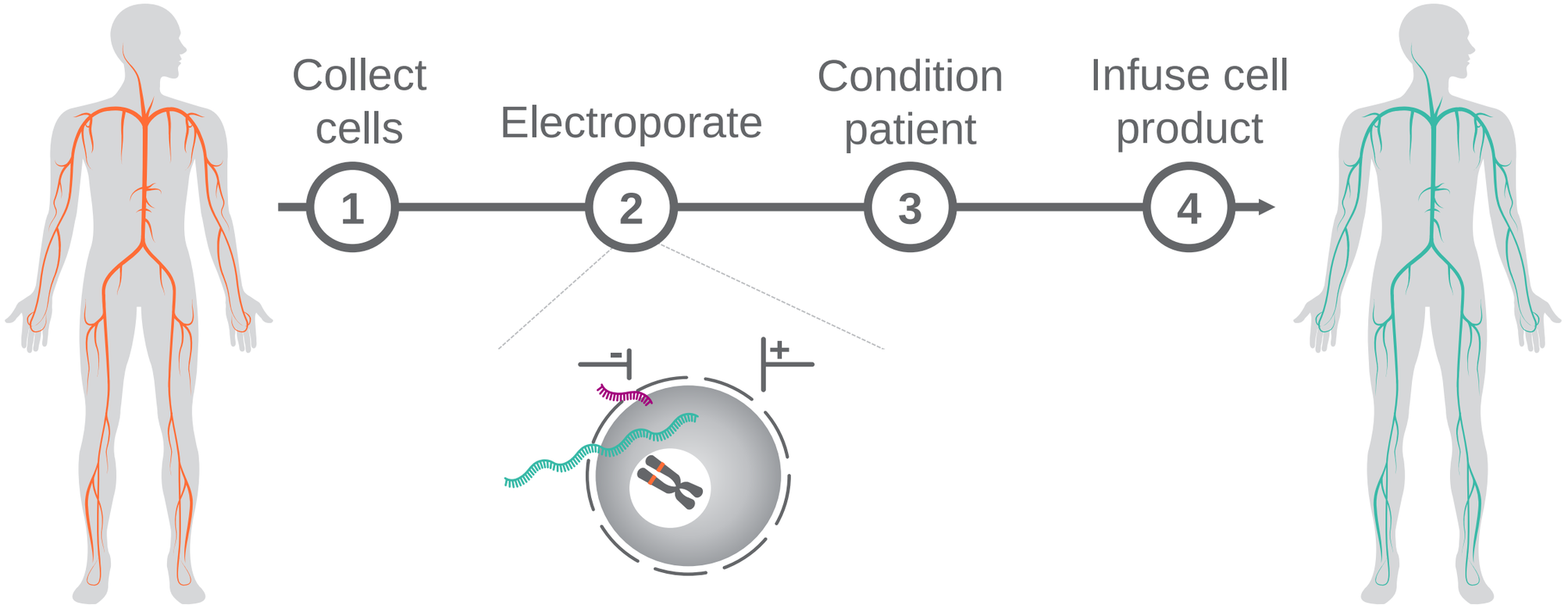
2021 Achievements

- [x] FDA clearance of the **BEAM-101** IND
- [x] IND-enabling studies for **BEAM-102**
- [x] IND-enabling studies for **BEAM-201**
- [x] Beam LNP data in non-human primates
- [x] First liver DC, **BEAM-301**: GSDIa R83C
- [x] Non-human primate studies for Stargardt
- [x] 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 2nd CAR-T development candidate
- [ ] Initiate IND-enabling studies for **BEAM-301**
- [ ] Nominate 2nd liver development candidate
- [x] Form additional strategic platform partnerships (Pfizer)

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



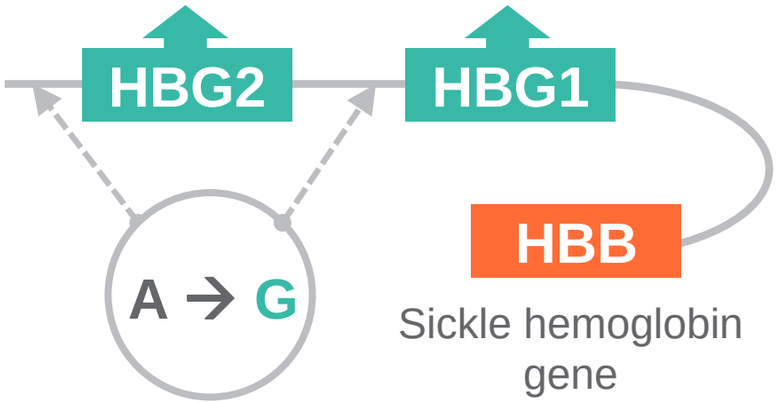
Sickle Cell Disease Patient

Program-specific gRNA and ***base editor mRNA***

Sickle Cell Disease Patient

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

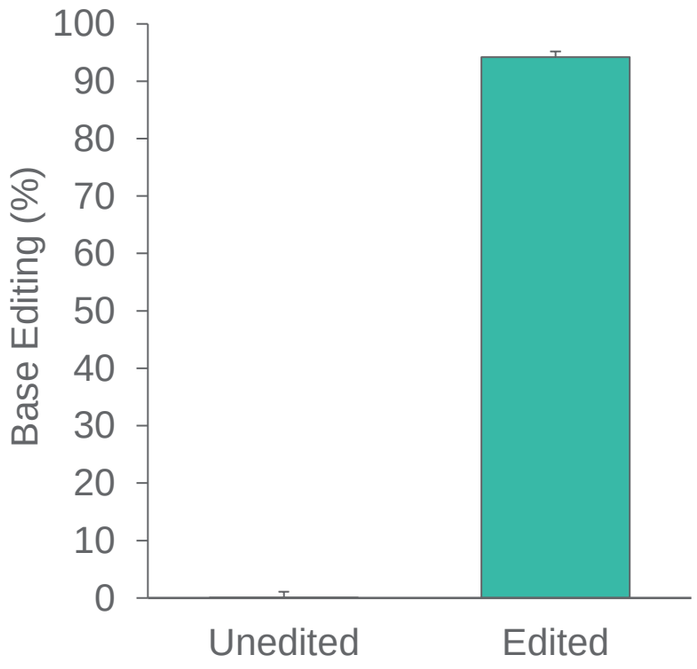
Sickle Cell Disease: 100,000 patients in the US; severe pain, organ damage, early mortality



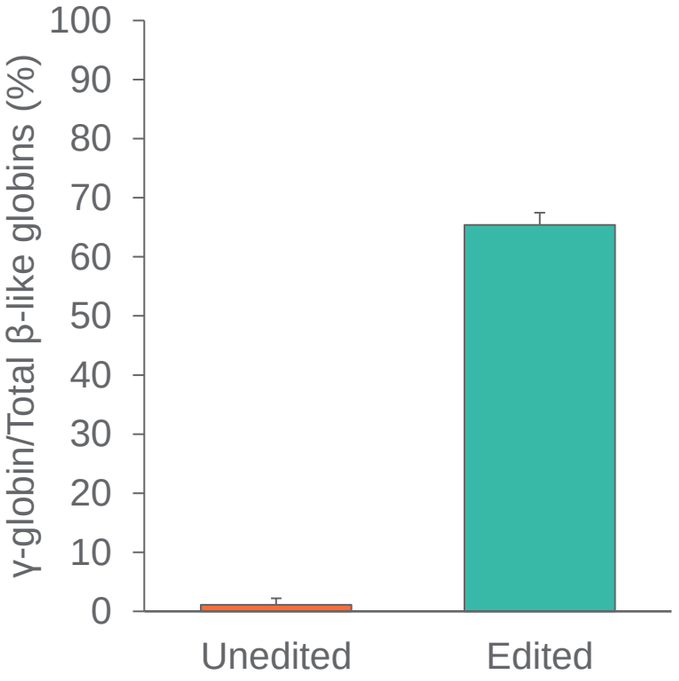
Sickle hemoglobin gene

Base editor edits regulatory element of <u>both</u> fetal hemoglobin genes, without cutting DNA

>90% base editing at *HBG1/2* promoters in multilineage cells[1]



>65% gamma globin protein levels in sorted erythroid cells[2]



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

Inclusion criteria

- Patients with severe sickle cell disease (SCD) with prior treatment with at least one disease-modifying agent with inadequate response or intolerance
- Age ≥18 to ≤35 years for initial cohort

Mobilization & Manufacturing

Conditioning and Transplant

Safety endpoints

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

Efficacy endpoints

- Severe vaso-occlusive events
- Transfusion requirements
- Hemoglobin F levels
- Quality of life and ability to function

Phase 1/2 study

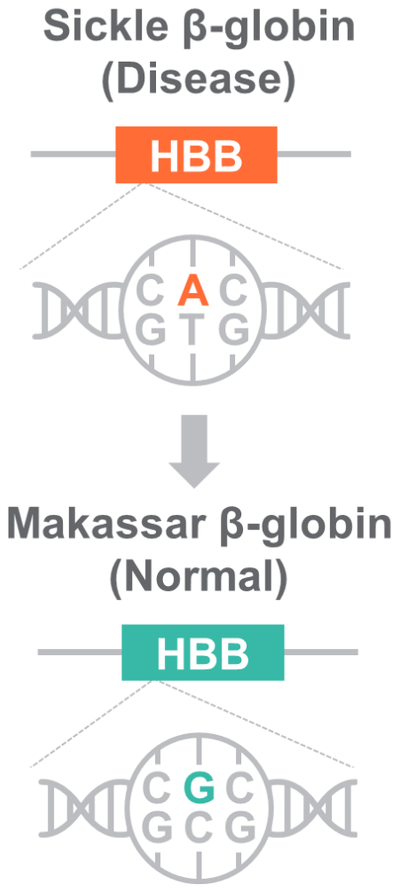
Safety, Efficacy and Engraftment Evaluations

Follow up study

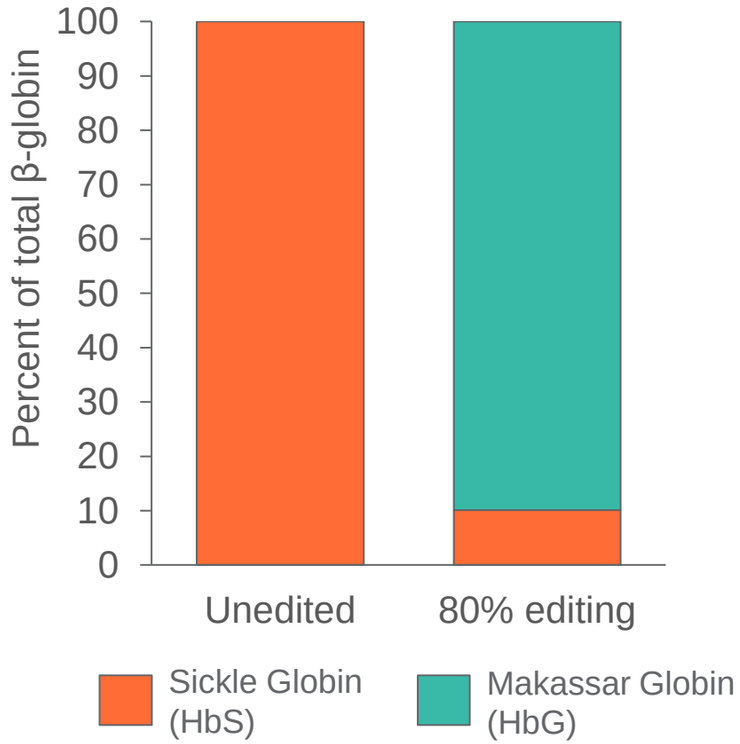
Long-Term Safety Study

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

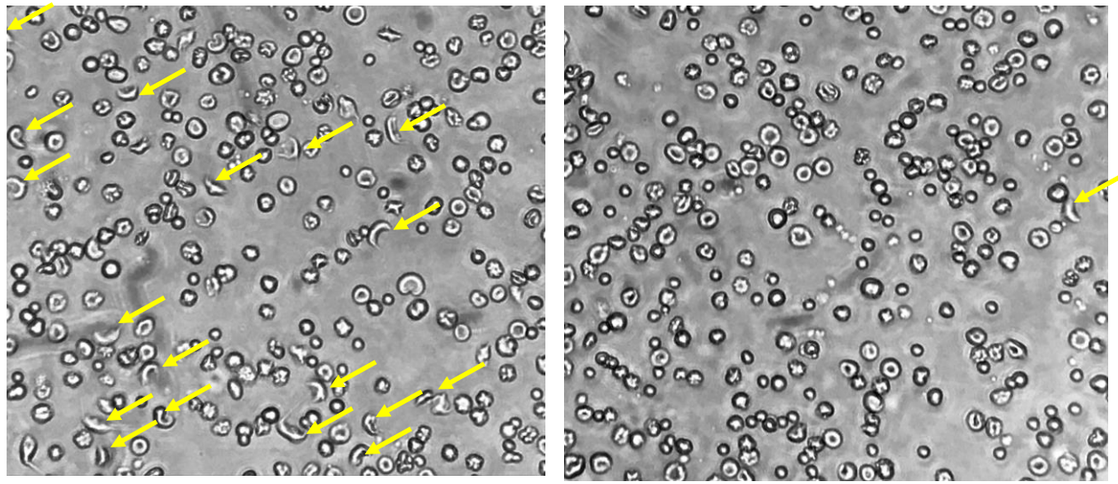
Sickle Cell Disease: 100,000 patients in the US; severe pain, organ damage, early mortality



>80% editing of HbS mutation



Elimination of sickling under low oxygen conditions



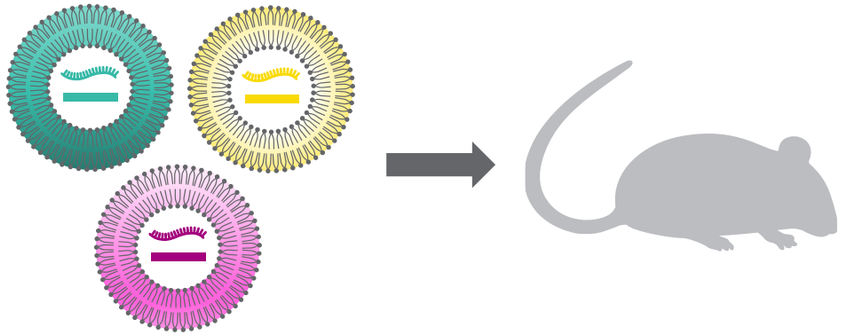
Unedited Edited

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.

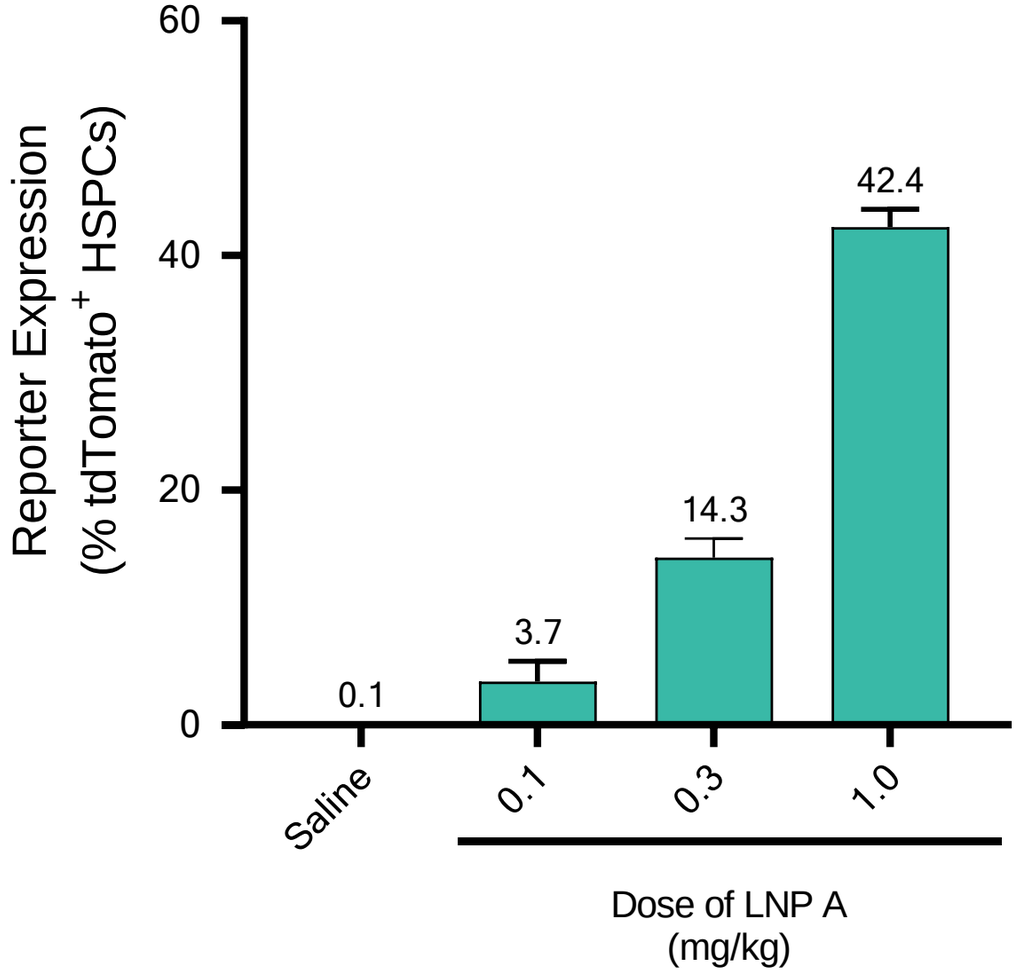
Uniquely positioned to potentially create best-in-class regimens for SCD patients, now and in the future

Wave 1 **Base Editing + HSC Transplant**	Wave 2 **Improved Conditioning**	Wave 3 ***In vivo* Delivery**
Precise gene editing (non-cutting, non-viral) BEAM-101 BEAM-102	HSC conditioning with targeted antibodies (less toxic) magenta THERAPEUTICS Beam THERAPEUTICS	*In vivo* editing after infusion of HSC-targeted LNPs (no transplant)

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

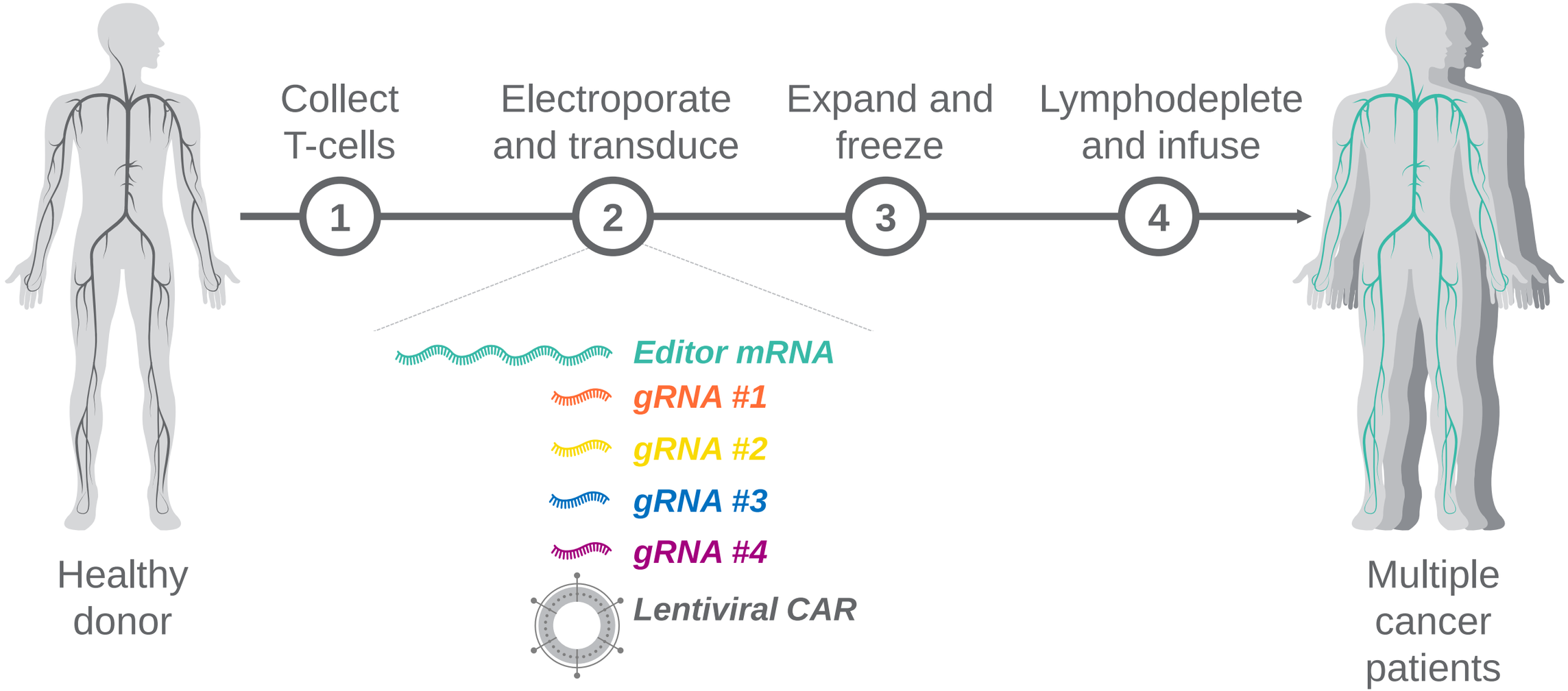


- Proprietary technology for high throughput LNP screening for delivery beyond the liver
- Each nanoparticle contains mRNA payload plus a unique DNA barcode
- Simultaneous in vivo screening of LNPs to select formulations capable of targeting diverse tissues



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

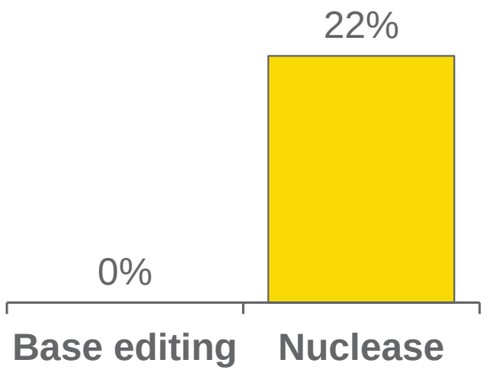
Allogeneic multiplex edited CAR-T cell process



Significant advantages of multiplex base editing without double strand breaks

Chromosomal rearrangements

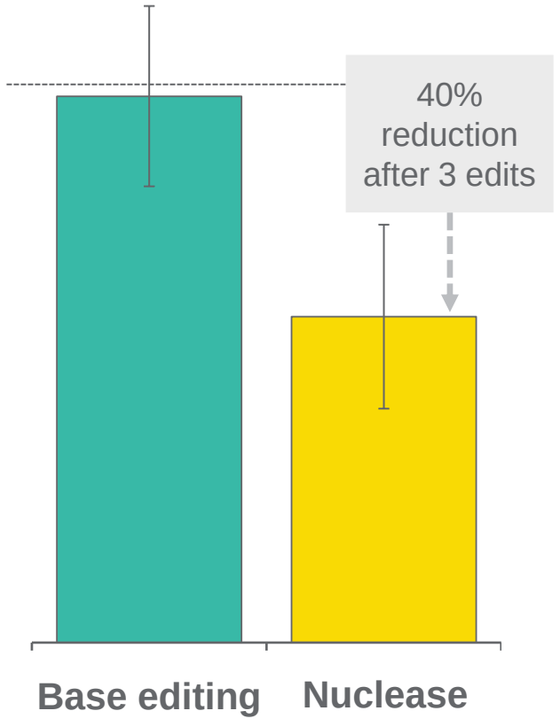
Percent of cells with translocations[1]



4 edits: TRAC, CD52, PD1, CD7

Impact on cell expansion

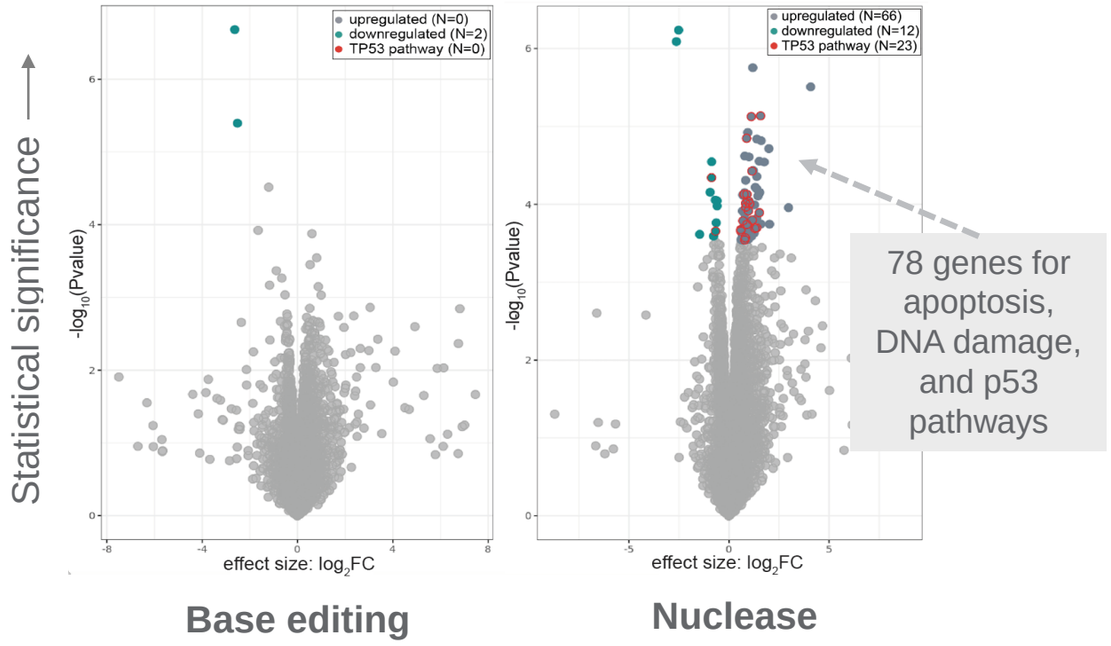
Percent yield after editing[2]



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

Base editor mRNA

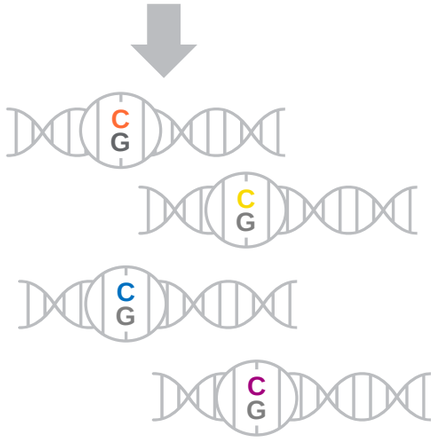
CD7 CAR

TRAC gRNA: Prevent graft-vs-host disease

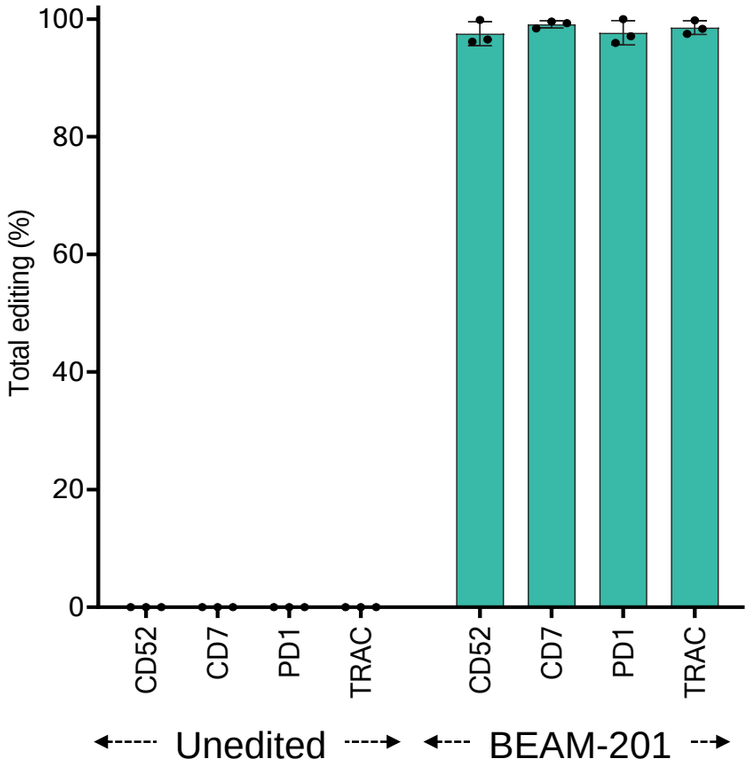
CD52 gRNA: Enable allogeneic cell source

PD1 gRNA: Prolong efficacy

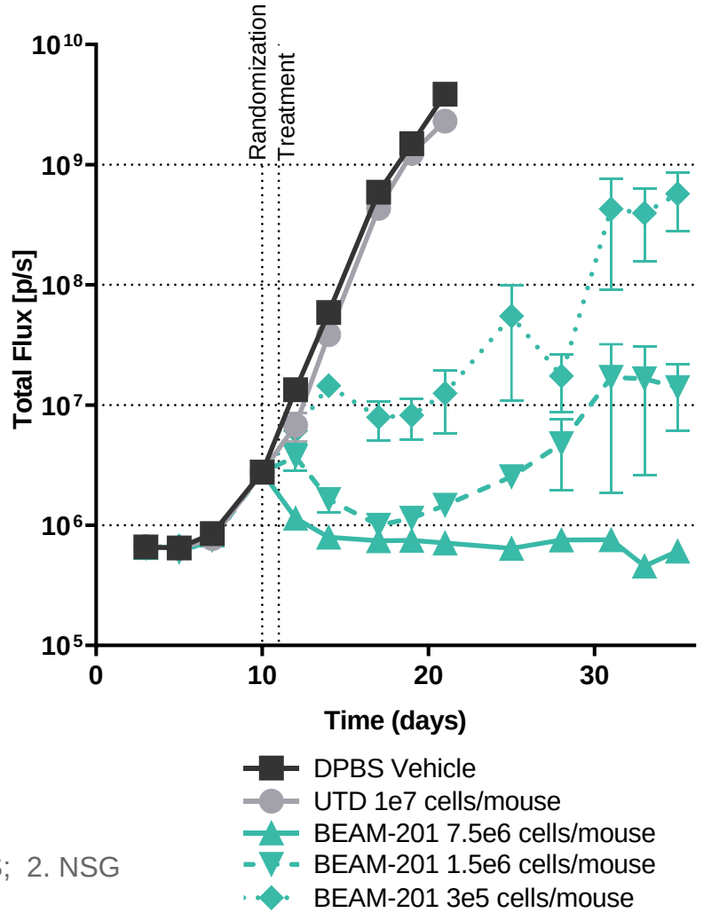
CD7 gRNA: Prevent fratricide from CD7 CAR



Clinical process yields 96-99% editing, >90% quad edited[1]

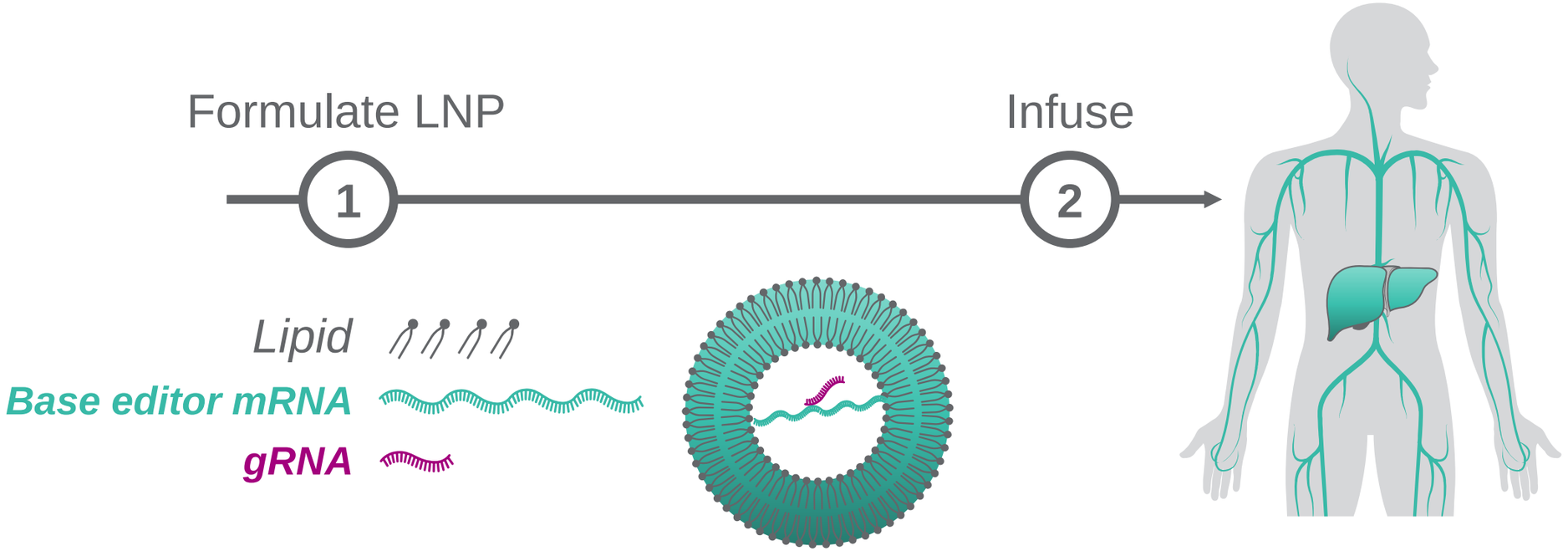


Potent in vivo tumor clearance or control across a 25-fold dose[2]



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

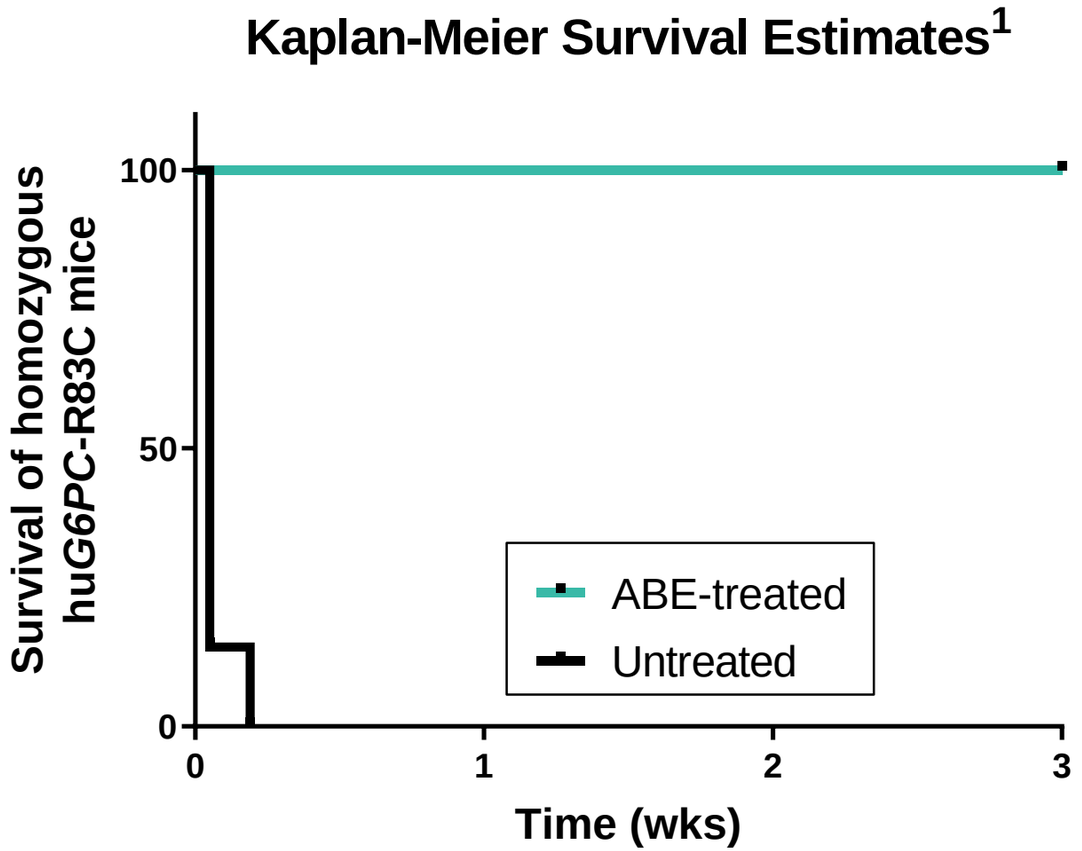


- Clinically validated technology for transient, *in vivo* delivery to the liver
- Scalable manufacturing with lower COGS

- **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

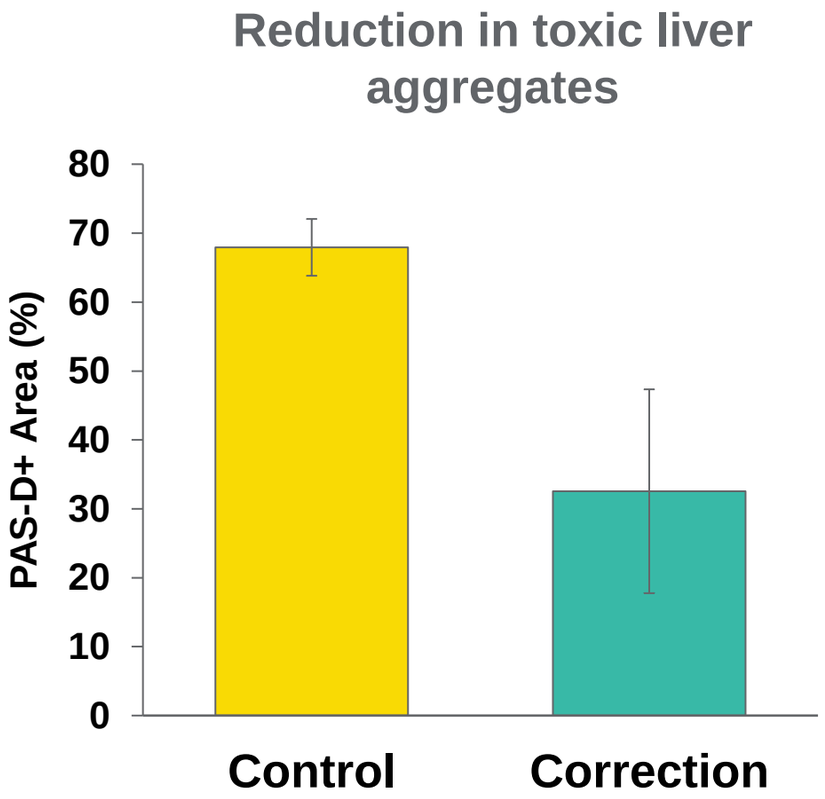


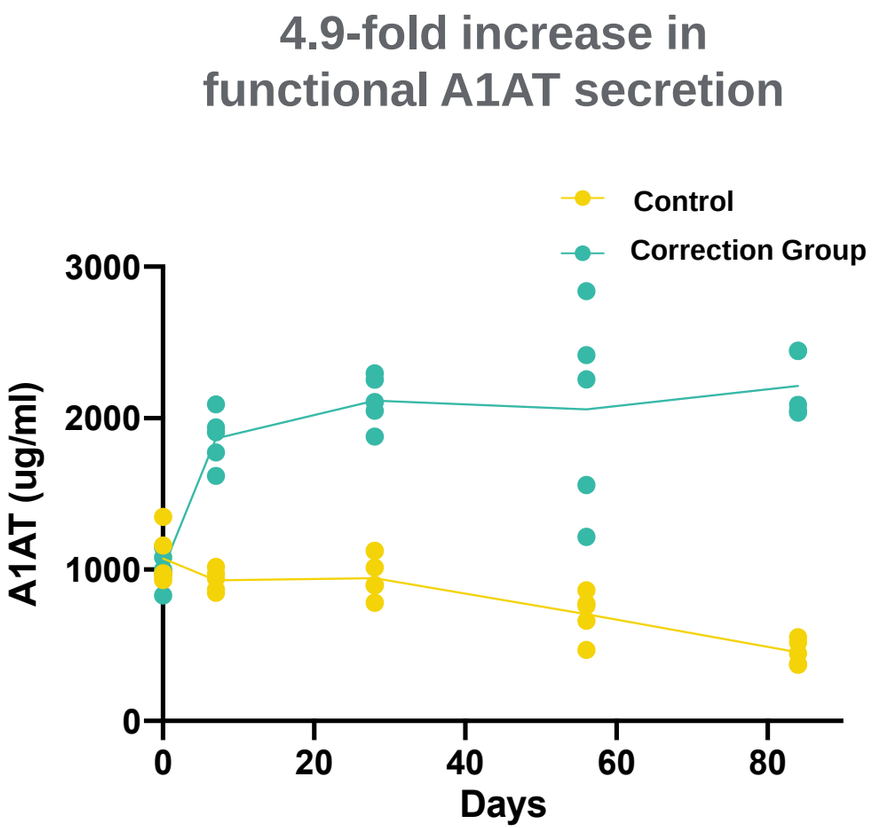
- **DC nomination in Dec 2021 – Beam's first *in vivo* DC**
- Near-normal serum metabolites, G6PC activity, hepatic morphology and lipid deposition
- Animal models suggest 11% editing may be sufficient for clinical benefit[2]

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[1]; 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

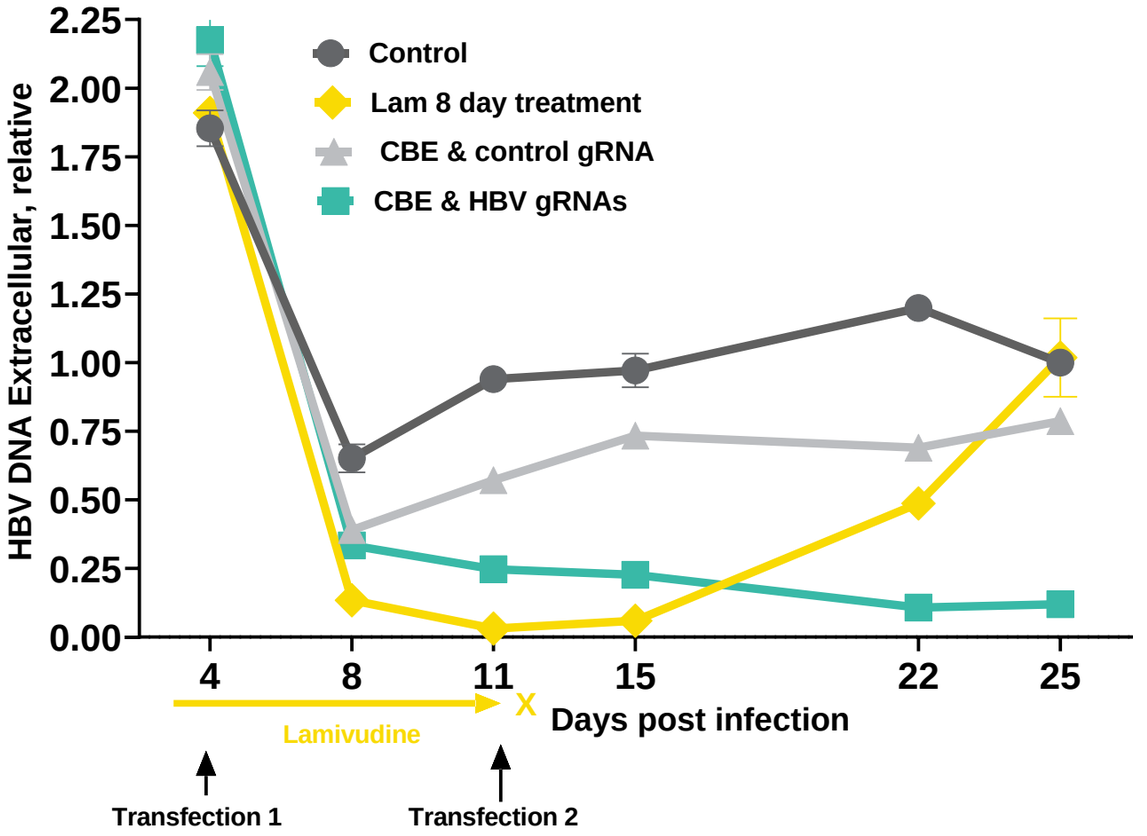
1. The most severe form of Alpha-1 arises when a patient has an E342K (PiZ) point mutation in both copies of the SERPINA1 gene, where two copies are designated ZZ.

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