



Q3 Financial Results & ASH Abstracts Investor Webcast

November 5, 2024

Beam conference call participants



TOPIC	PARTICIPANT
Introduction	Holly Manning VP Investor Relations & External Communications
Q3 Business Update	John Evans Chief Executive Officer
Beam's Sickle Cell Disease Strategy	Mr. Evans
ASH Abstracts: BEAM-101 Clinical Data	Amy Simon, M.D. Chief Medical Officer
ASH Abstract: ESCAPE Preclinical Data	Giuseppe Ciaramella, Ph.D. President
Closing Remarks	Mr. Evans
Q&A	Mr. Evans, Dr. Simon, Dr. Ciaramella

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 therapeutic applications and potential of our technology, including with respect to SCD, T-ALL/T-LL, AATD, GSD1a, and ESCAPE; our plans, and anticipated timing, to advance our programs; the clinical trial designs and expectations for BEAM-101, BEAM-201, BEAM-301, BEAM-302 and ESCAPE; our potential presentations at the ASH annual meeting; our current expectations and anticipated results of operations, including our expected use of capital; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; 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," "strategy," "possibilities," "promise," "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 uncertainty that our product candidates will receive regulatory approval necessary to initiate human clinical trials; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that initiation and enrollment of our clinical trials may take longer than expected; that our product candidates or the delivery modalities we rely on to administer them may cause serious adverse events; 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, 2023, our quarterly reports on Form 10-Q, and in any subsequent filings with the Securities and Exchange Commission (the "SEC") which are available on the SEC's website at www.sec.gov. Additional information will be made available by our annual and quarterly reports and other filings that we make from time to time with the SEC. These forward-looking statements speak only as of the date of this presentation. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

OUR VISION IS TO PROVIDE LIFE-LONG CURES for patients suffering from serious diseases



POTENTIAL FOR
one-time, curative
therapies



GENE EDITING FOR
rare and common
diseases



PLATFORM FOR
rapidly programmable
precision medicines

The central hypothesis behind Beam: Base editing is more precise, efficient, predictable and versatile than nucleases

NUCLEASE CRISPR, ZFN, TALENs

Precision targeting with CRISPR



Double-stranded breaks



Lack of control of gene sequence outcomes

...ACG --- GCAT...
...ACGTC **GCTT** ATGCAT...
...A --- TGCAT...
...ACGTC **T** ATGCAT...
...AC --- AT...
...ACGTC **AAC** --- GCAT...
etc

----- Deletions
A G C T Insertions

BASE EDITING BEAM THERAPEUTICS

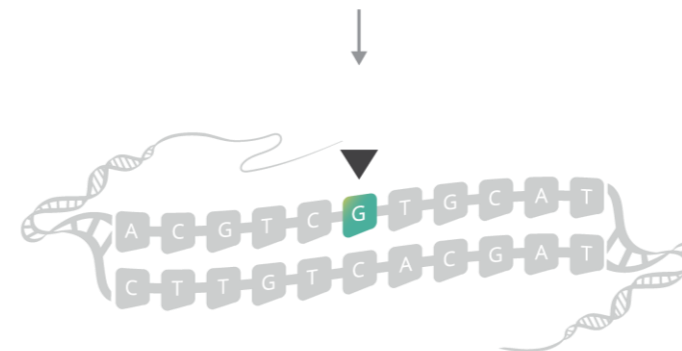
Precision targeting with CRISPR



Enzymatic base conversion



Highly efficient with predictable gene sequence outcomes



Two platforms with potential to create transformative therapies and significant value creation

Hematology

- Best-in-class potential for BEAM-101 for sickle cell disease (SCD)

- Increased probability of technical success for *ex vivo* gene editing and fetal hemoglobin (HbF) upregulation

- Validated FDA regulatory pathway

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

- Platform for future hematology pipeline

Initial data today

Liver Genetic Diseases

- Best-in-class potential for BEAM-302 for alpha-1 antitrypsin deficiency (AATD)

- Increased probability of technical success for *in vivo* lipid-nanoparticle (LNP) gene editing in liver

- Potential for rapid clinical proof of concept

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

- Platform for future liver-targeted pipeline

Data expected in 2025

Significant execution momentum in Q3 across our priority hematology and liver genetic disease programs

Hematology



35 patients enrolled and 8 patients dosed in BEACON Phase 1/2 trial of BEAM-101 in SCD



Development candidate nominated for ESCAPE technology in SCD:

- BEAM-103 (anti-CD117 mAb)
- BEAM-104 (CD34 cells with a CD117 edit and HBG1/2 edit)



\$925.8M in cash with expected **operating runway into 2027** (excluding commercialization expenses for BEAM-101)

Liver Genetic Diseases



First cohort dosing completed in the Phase 1/2 trial of BEAM-302 in AATD



IND approved by FDA and site activation activities underway for BEAM-301 Phase 1/2 study in glycogen storage disease 1a (GSD1a)

Four Beam abstracts accepted for presentation at the 66th American Society of Hematology (ASH) Annual Meeting



ORAL

Initial Results from the BEACON Clinical Study of BEAM-101 in Sickle Cell Disease

Sunday, Dec. 8, 10 a.m. PT
Abstract #513

POSTER

Impact of BEAM-101 Treatment on Red Blood Cell Hemoglobin Expression, Rheology and Sickling Properties

Monday, Dec. 9, 6-8 p.m. PT
Abstract #4957

ORAL

Preclinical Data for ESCAPE in a Rhesus Autologous Transplantation Model

Sunday, Dec. 8, 10:45 a.m. PT
Abstract #516

POSTER

Initial Data from the Phase 1/2 Study of BEAM-201, Multiplex Base-Edited Allogeneic Anti CD7 CAR-T-Cells

Monday, Dec. 9, 6-8 p.m. PT
Abstract #4838

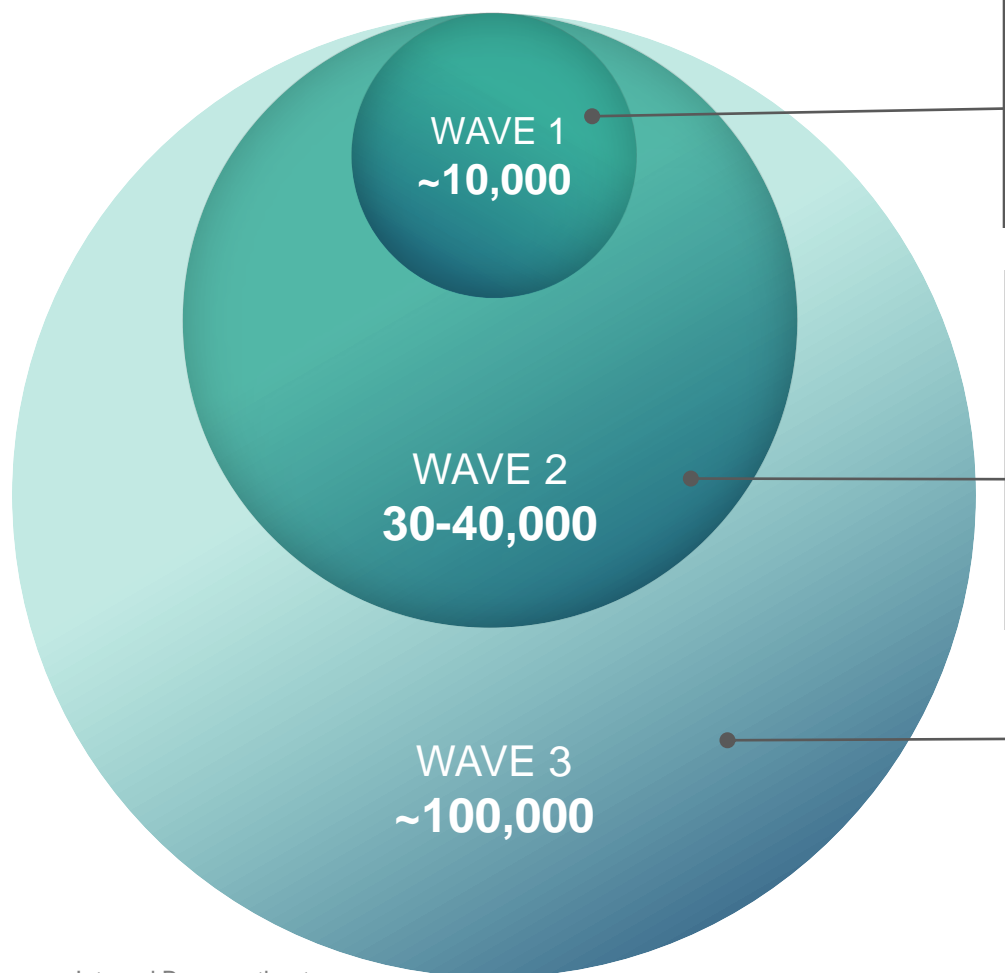
Beam to host investor event on Sunday, December 8, 2024, at 8 p.m. PT

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

SICKLE CELL DISEASE

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

Potential Eligible SCD Patient Population (U.S.)



BEAM-101: Precise HbF upregulation

Potentially best-in-class gene editing

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

ESCAPE: Multiple edits for non-genotoxic conditioning

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

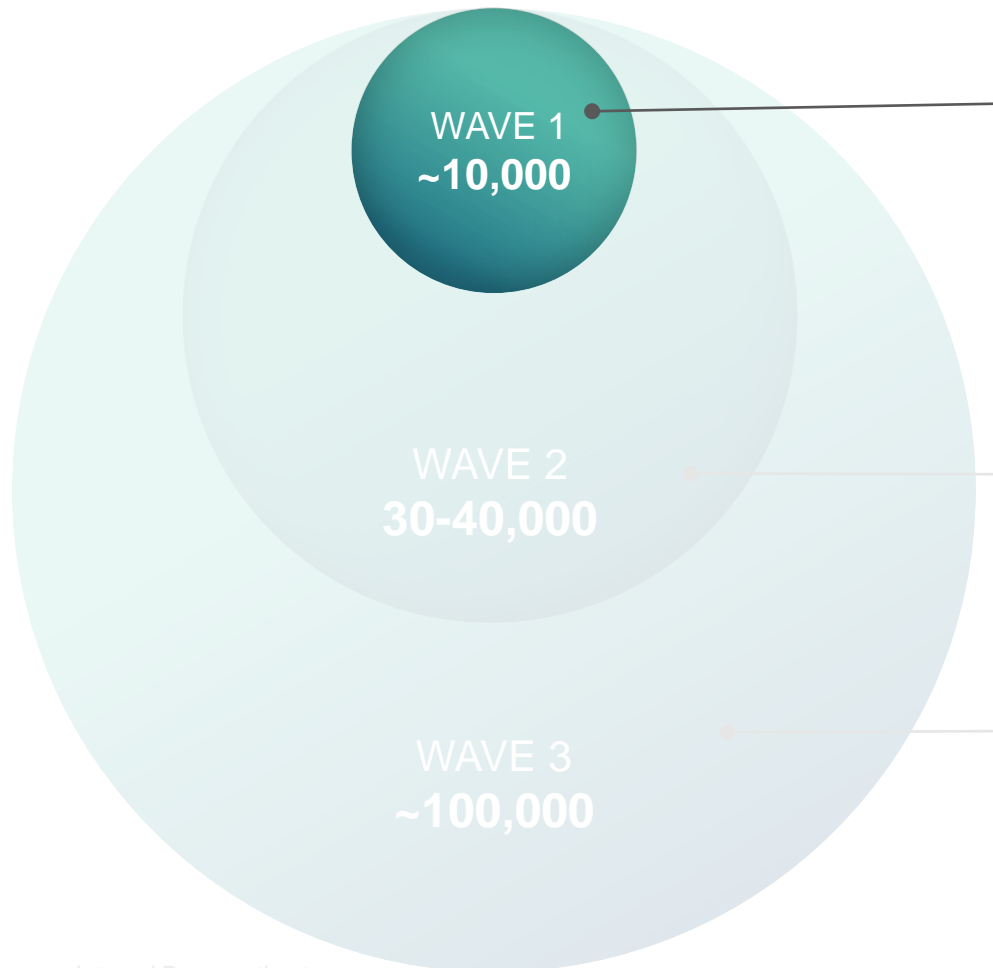
- Broader range of disease severity
- Broader age range
- Increased willingness-to-treat

In vivo: Base editing with hemopoietic stem cell (HSC)-targeted LNPs

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

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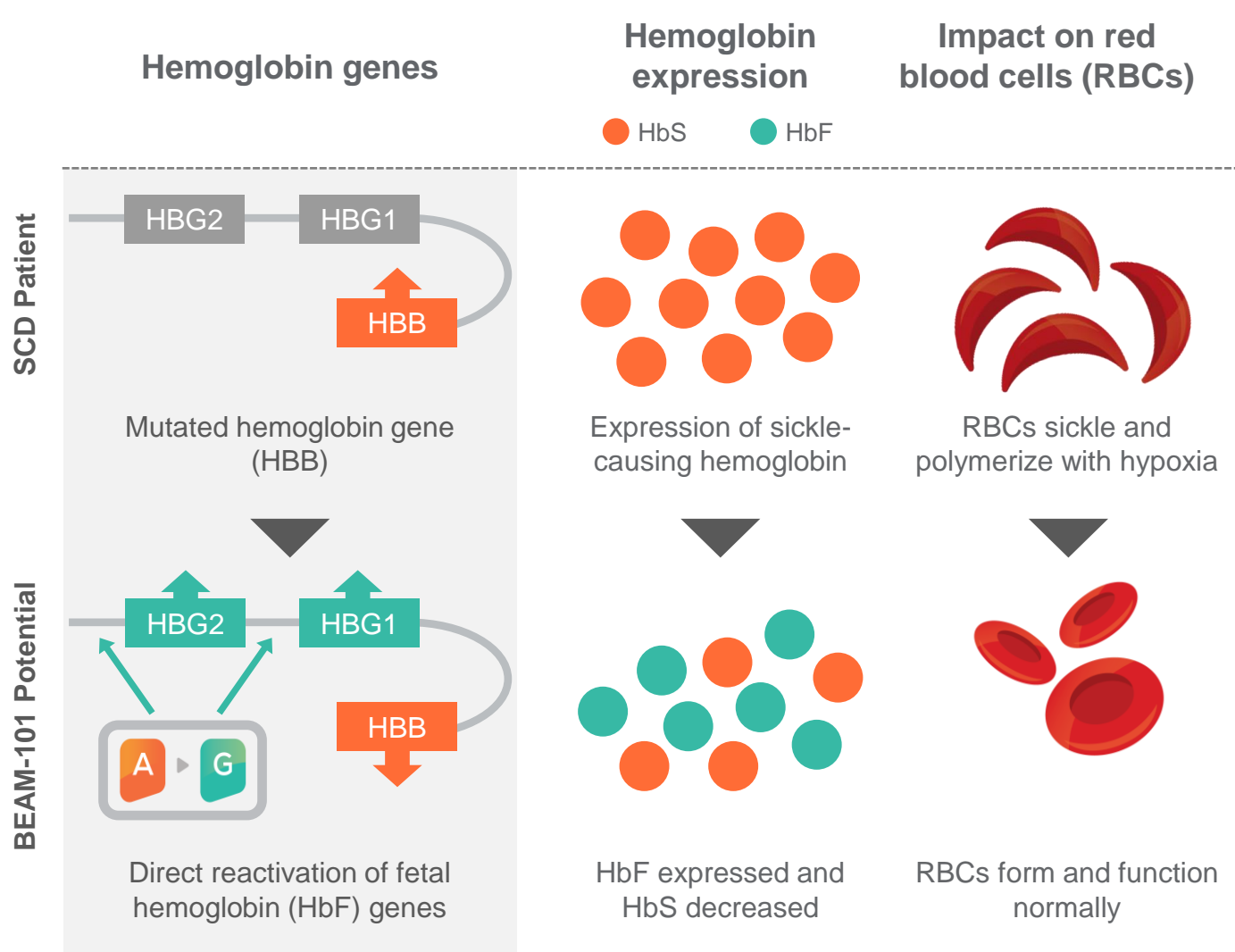
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BEAM-101: Designed to be best-in-class genetic medicine for sickle cell disease



SCD Unmet Need

- Sickle hemoglobin (HbS) polymerization is root cause of sickle cell pathophysiology
- Affects millions of people worldwide and ~100K in U.S.
- Median survival in the U.S. is ≥ 20 years shorter

Current Available Treatments

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

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

Disease



Sickle cell disease (two mutations)

- 0% normal Hb (HbA)
- 100% sickle Hb (HbS)
- All circulating cells with HbSS genotype

Non-disease



Sickle cell “trait” (SCT) (carrier with one mutation, typically asymptomatic)

- 60% HbA
- 40% HbS
- No circulating cells with HbSS genotype



Normal (no mutations)

- 100% HbA
- 0% HbS
- No circulating cells with HbSS genotype

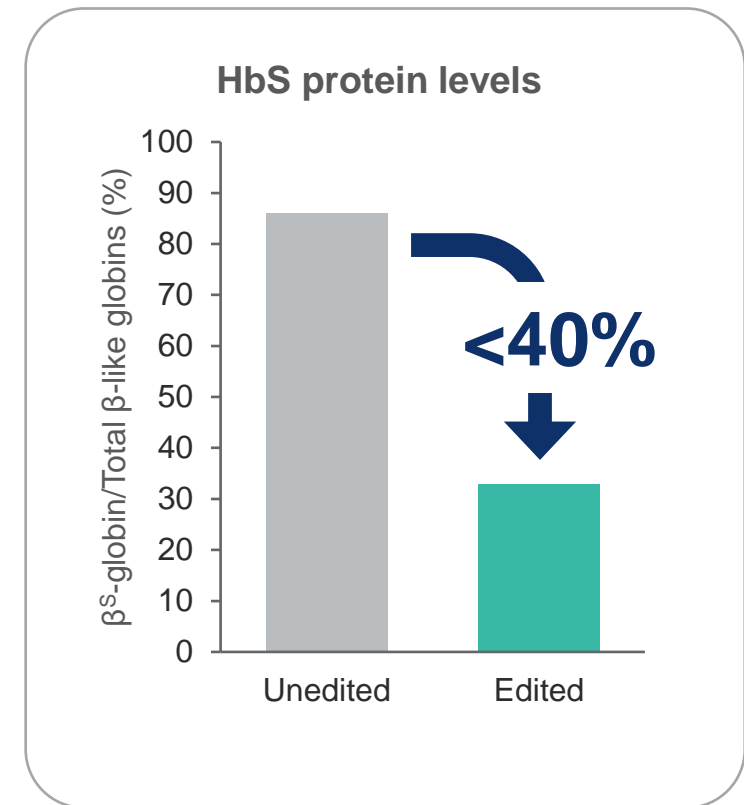
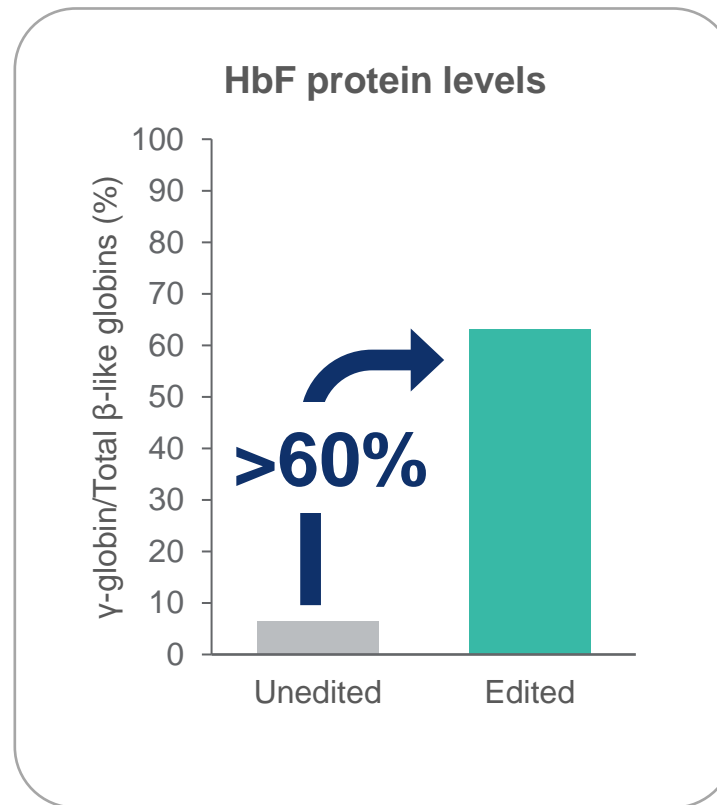
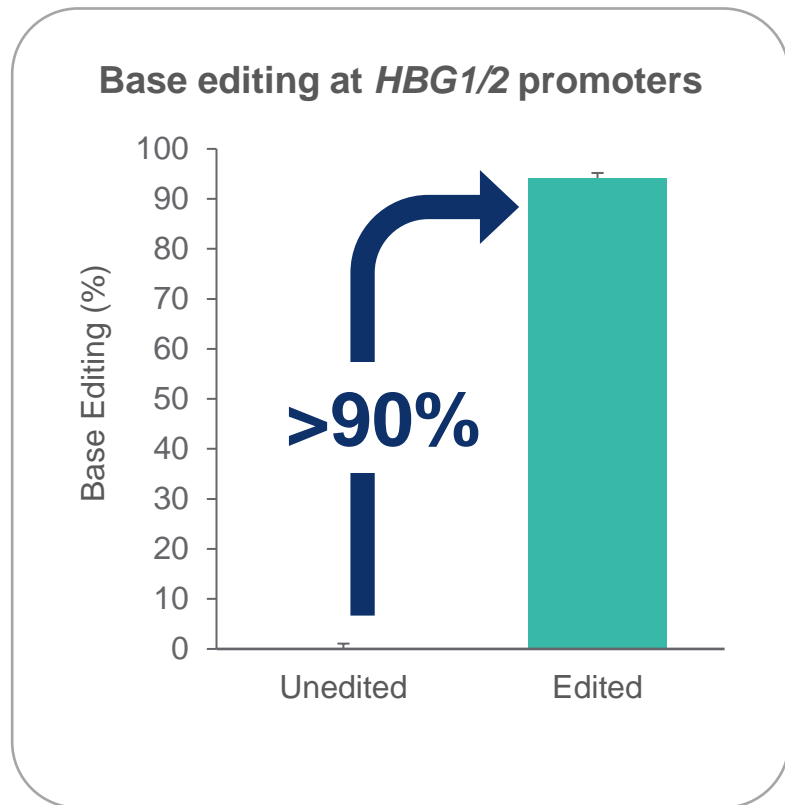
Base editing

>60% HbF (anti-sickling)

<40% HbS

Minimize cells expressing only HbS

Preclinical data suggest BEAM-101 has potential for highest HbF induction and lowest residual HbS levels vs. other approaches



Preclinical data presented at ASGCT 2020

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

BEAM-101 Phase 1 Clinical Data

AMY SIMON, M.D., CHIEF MEDICAL OFFICER

BEACON Phase 1/2 study: Evaluating safety and efficacy of BEAM-101 in patients with SCD and severe vaso-occlusive crises

Sentinel Cohort (N = 3)

- ✓ Enrollment complete
- ✓ Dosing complete



Expansion Cohort (N = 42)

- ✓ 35 patients cleared screening and enrolled
- ✓ 8 patients dosed, with the remaining in process



Key Eligibility Criteria

- 18-35 years age
- SCD (β^S/β^S ; β^S/β^0 ; β^S/β^+ genotypes) with ≥ 4 severe vaso-occlusive crises (sVOCs) in 24 mos pre-screening
- No available matched sibling donor
- No history of overt stroke

Select Safety Endpoints

- Proportion of patients with successful neutrophil engraftment
- Time to neutrophil engraftment
- Time to platelet engraftment

Select Efficacy Endpoints

- Proportion of patients sVOC-free for 12 consecutive months
- Total Hb levels
- HbF and HbS levels
- Hemolysis parameters
- Patient-reported outcomes
- RBC function and organ damage

ASH ABSTRACT:

safety n=6
efficacy n=4

ASH PRESENTATION:

safety n=7
efficacy n=7

Patient demographics and treatment characteristics



PATIENT DEMOGRAPHICS (N=6)

Genotype, n	β^S / β^S	5
	β^S / β^0	1
Gender, n	Male	3
	Female	3
Age in years	Range	19-27
Race	African American	6

TREATMENT CHARACTERISTICS (N=6)

Mobilization cycles	1 cycle	3	Mean: 1.5 cycles
	2 cycles	3	
BEAM-101 dose, viable CD34+ cells x 10 ⁶ /kg	Mean (range)	11.9 × 10 ⁶ (5.2–23.4) viable CD34+ cells/kg	

Preliminary safety data (N=6)

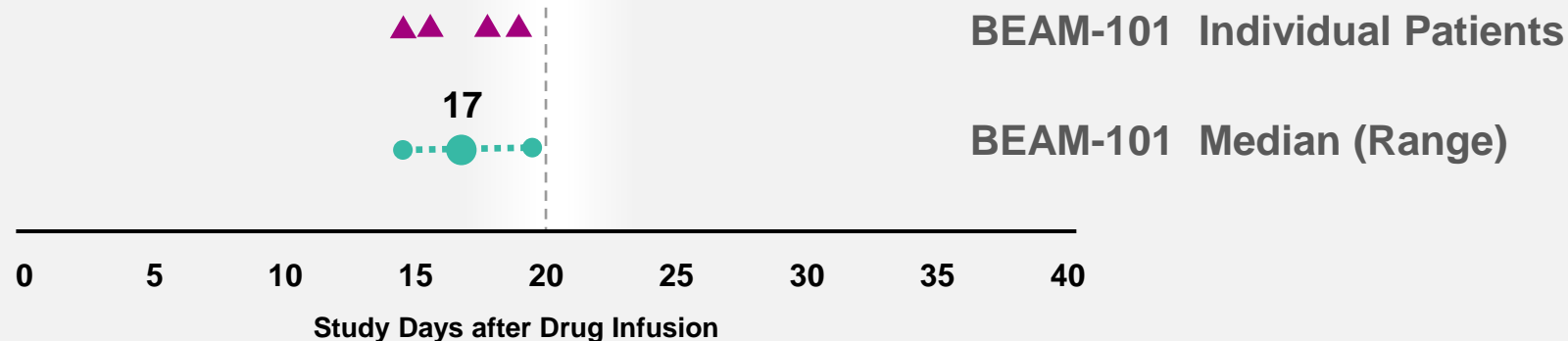


- Initial safety profile consistent with myeloablative conditioning with busulfan and autologous hematopoietic stem cell transplant (HSCT)
- One patient died due to respiratory failure likely related to busulfan conditioning 4 months after BEAM-101 infusion
 - Unrelated to BEAM-101 as determined by investigator
 - Case reviewed by Data Safety Monitoring Committee and FDA
- In all patients dosed, there were no \geq Grade 3 adverse events (AEs) or serious AEs related to BEAM-101

Preliminary safety data: Time to engraftment (N=4*)

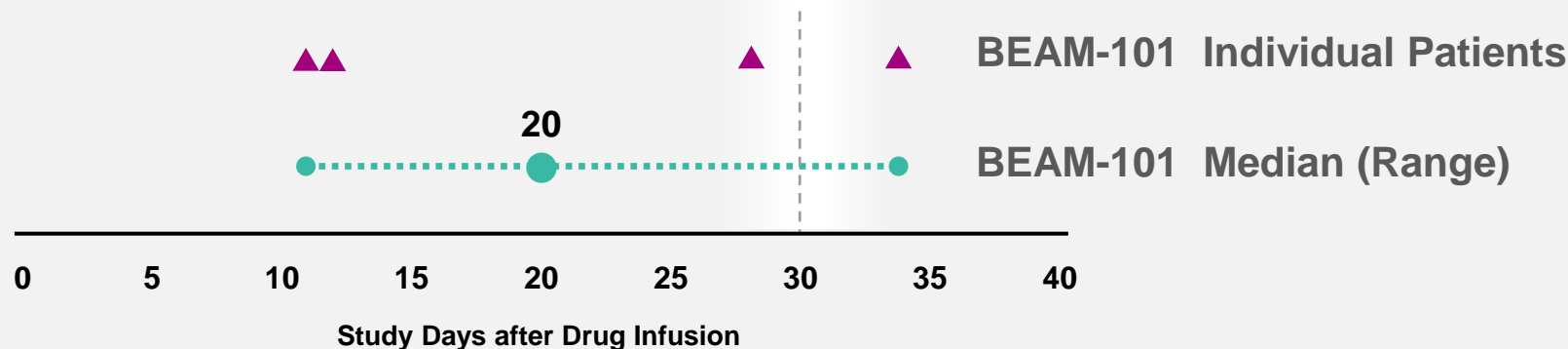


Neutrophil Engraftment
N=4



--- >90% allotransplant patients (peripheral blood stem cell) achieve neutrophil engraftment by Day 20¹

Platelet Engraftment
N=4



--- Approximately 60% of allotransplant patients (peripheral blood stem cell) achieve platelet engraftment by Day 30²

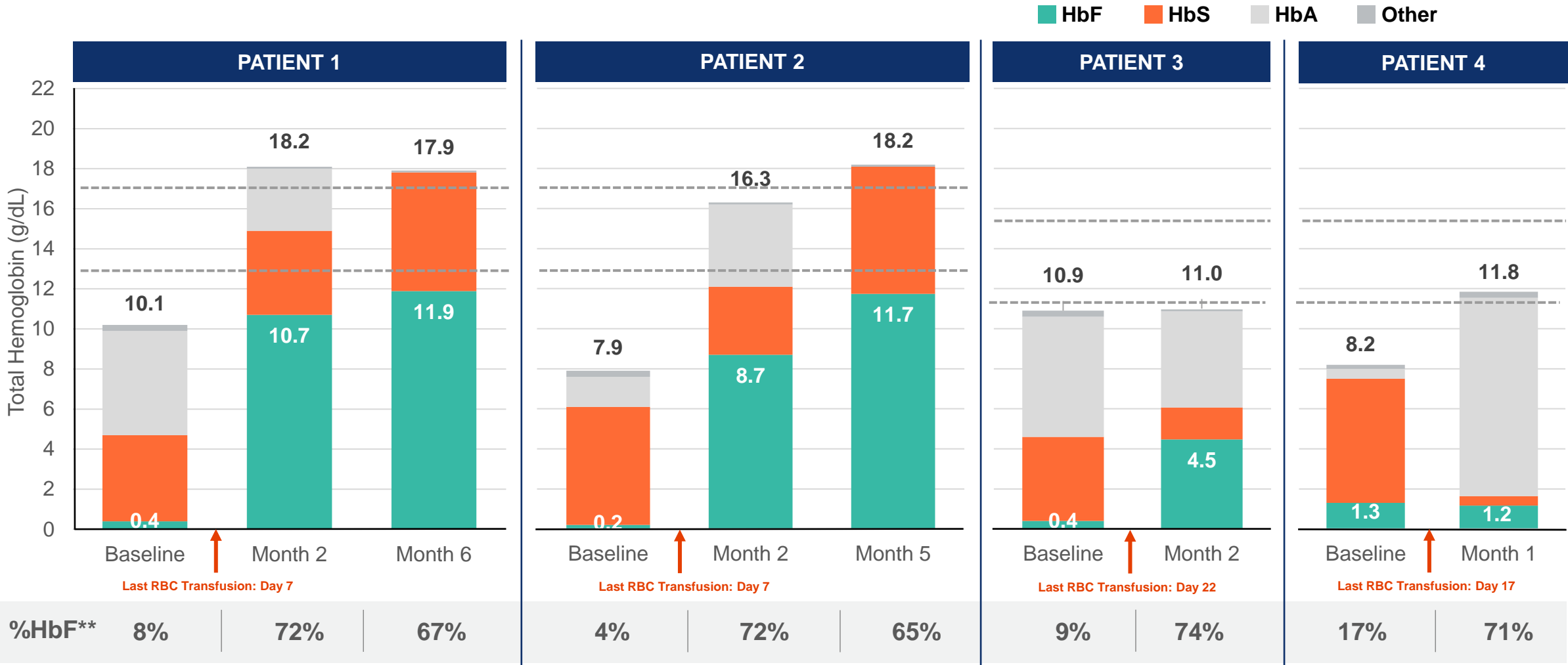
¹Kawajiri A et al., Blood Cell Therapy, 2019. 2: 22-30

²Ramirez P et al., BMT 2011, 46:981-986

Data cut as of July 2, 2024

*Data from patients with a follow-up period of one month or more¹⁹

Preliminary efficacy data (N=4*)



- Potent induction of HbF and reduction of HbS, consistent with preclinical results

**%HbF / (F + S)

----- Normal range of total hemoglobin for males (13-17 g/dL) and females (11.5-15.5 g/dL), per the central laboratory

Data cut as of July 2, 2024

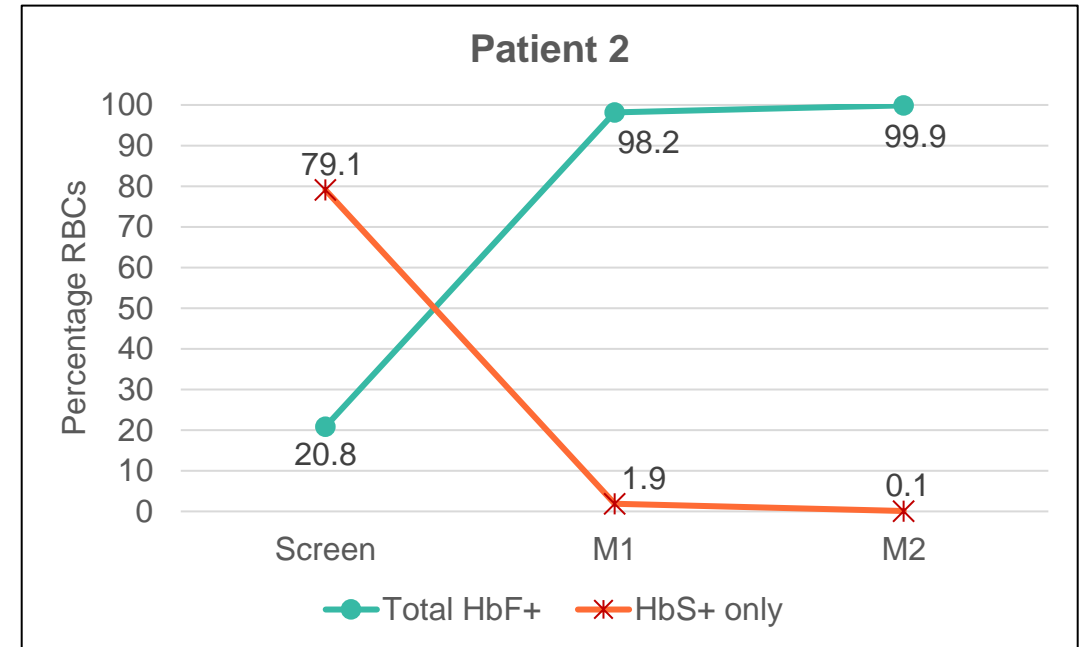
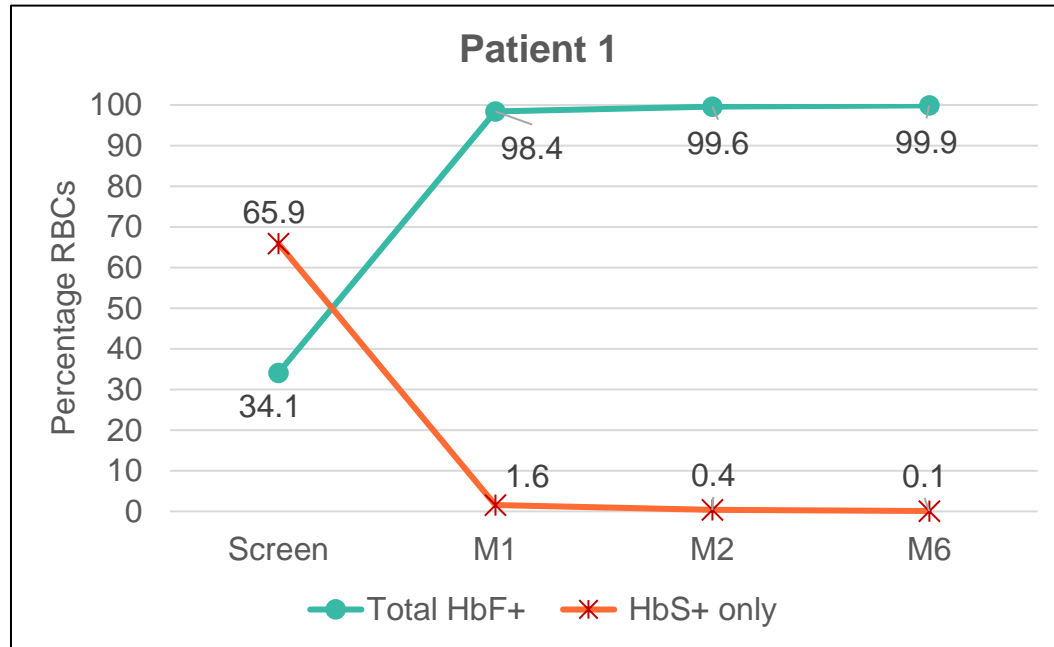
*Data from patients with a follow-up period of one month or more²⁰

Preliminary efficacy data (N=4*)



- All patients achieved >60% HbF and <40% HbS of non-transfused hemoglobin at Month 1, sustained through all time points
- Markers of hemolysis (lactate dehydrogenase, indirect bilirubin, haptoglobin and reticulocyte counts) normalized or improved for all patients
- No VOCs were reported by investigators following BEAM-101 treatment

Exploratory red blood cell (RBC) function assays

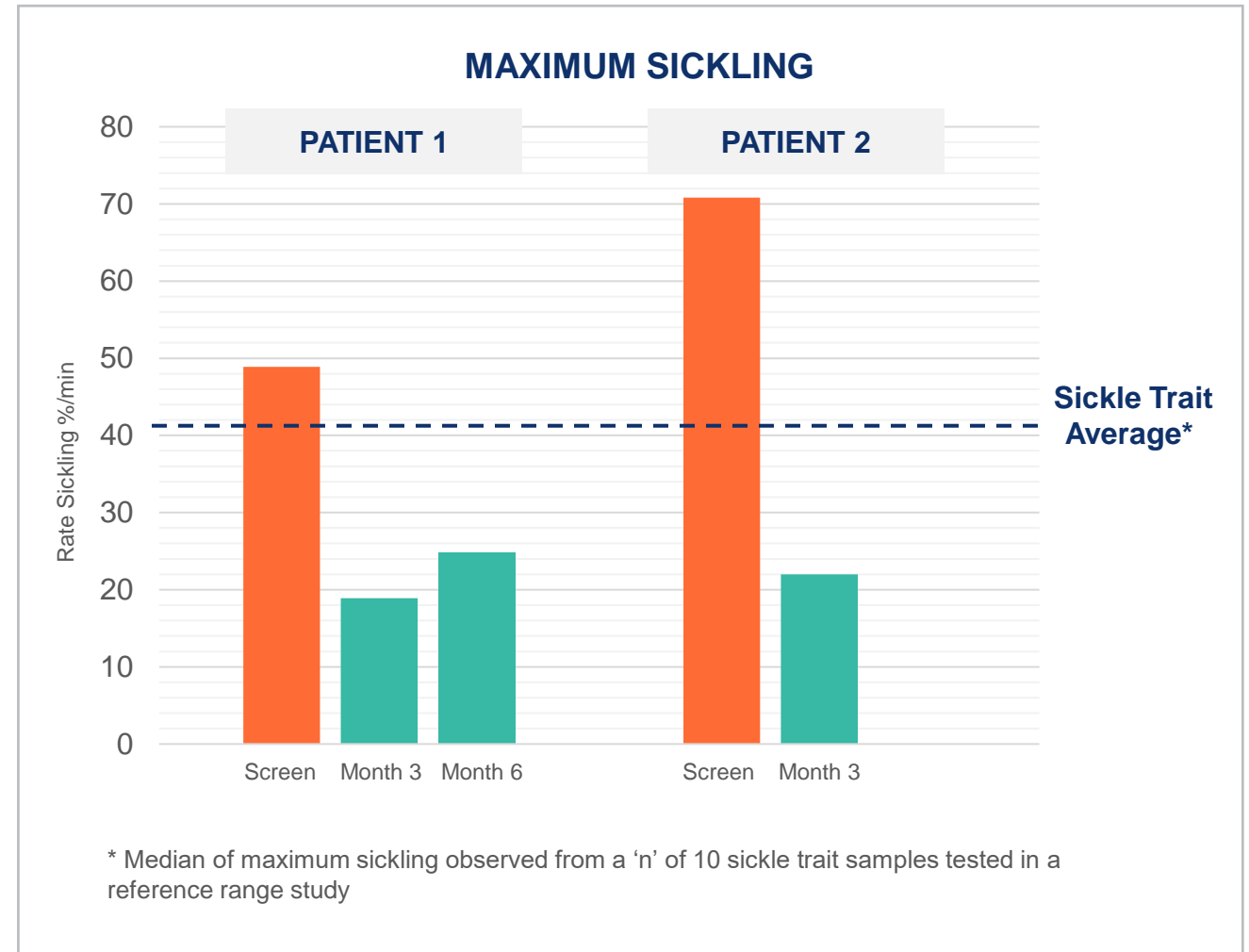


After BEAM-101 treatment:

- Nearly all RBCs were expressing HbF by Month 1
- Nearly all RBCs expressing solely HbS were eliminated by Month 2

Exploratory red blood cell (RBC) function assays

- Reduction in RBC sickling (see graph) and cell adhesion to levels comparable to SCT samples (N=2)
- Other RBC function improvements include increased deformability and decreased density (N=1 at Month 6)
- Resolution of abnormal cell morphology and sickle cells by Month 6 and 4 in patients 1 and 2, respectively



Summary: Emerging BEACON data show potential for significant differentiation of base editing and BEAM-101 for SCD



Efficient cell collection process resulting in 1-2 cycles

- Potential to enable fewer hospital days



Initial potent HbF induction and HbS reduction, consistent with preclinical results: >60% HbF / <40% HbS

- Similar to sickle cell trait profile



Initial safety profile consistent with myeloablative conditioning with busulfan and autologous HSCT

- No SAEs related to BEAM-101



Initial biomarker data demonstrate near elimination of RBCs solely expressing HbS and improved RBC function after BEAM-101 treatment



Neutrophil engraftment following busulfan conditioning occurred <20 days

- Potential to enable fewer hospital days



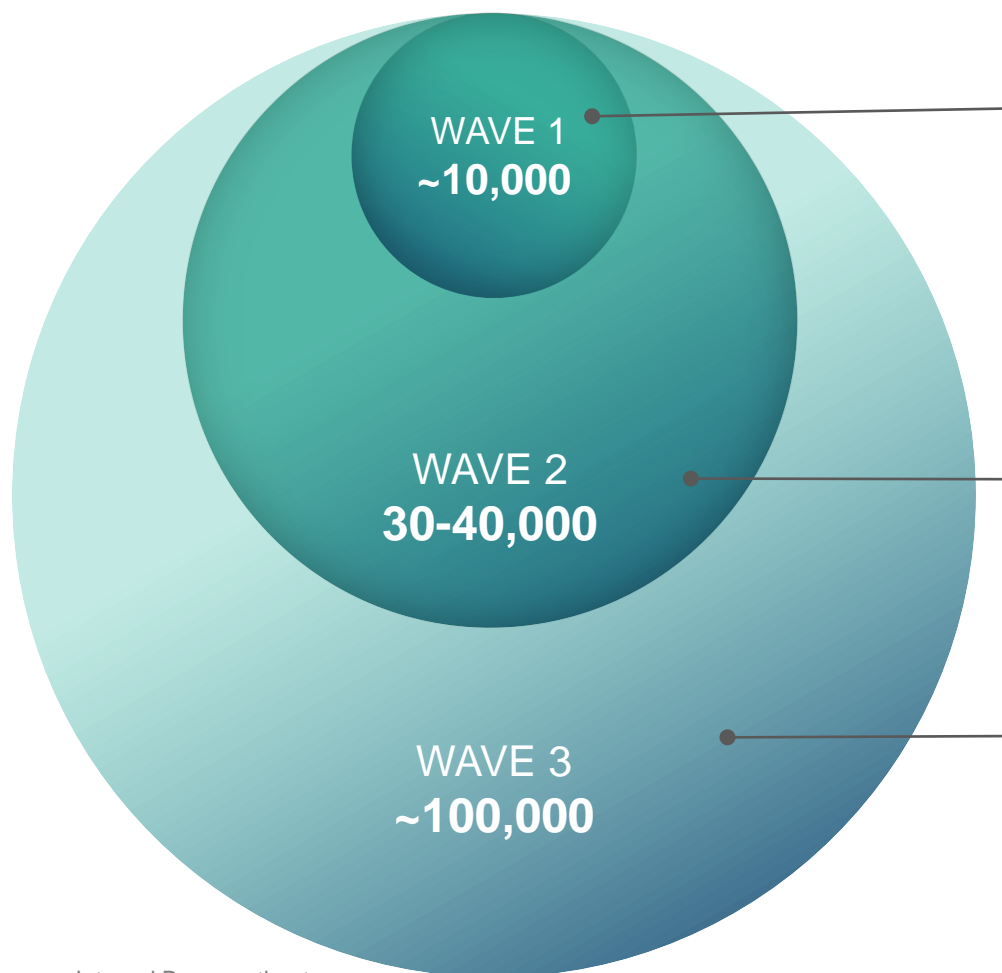
Presentation at ASH to include additional data with more patients and longer follow-up

ESCAPE Program Non-human Primate Data

GIUSEPPE CIARAMELLA, PH.D., PRESIDENT

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

Potential Eligible SCD Patient Population (U.S.)



BEAM-101: Precise HbF upregulation

Potentially best-in-class gene editing

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

ESCAPE: Multiple edits for non-genotoxic conditioning

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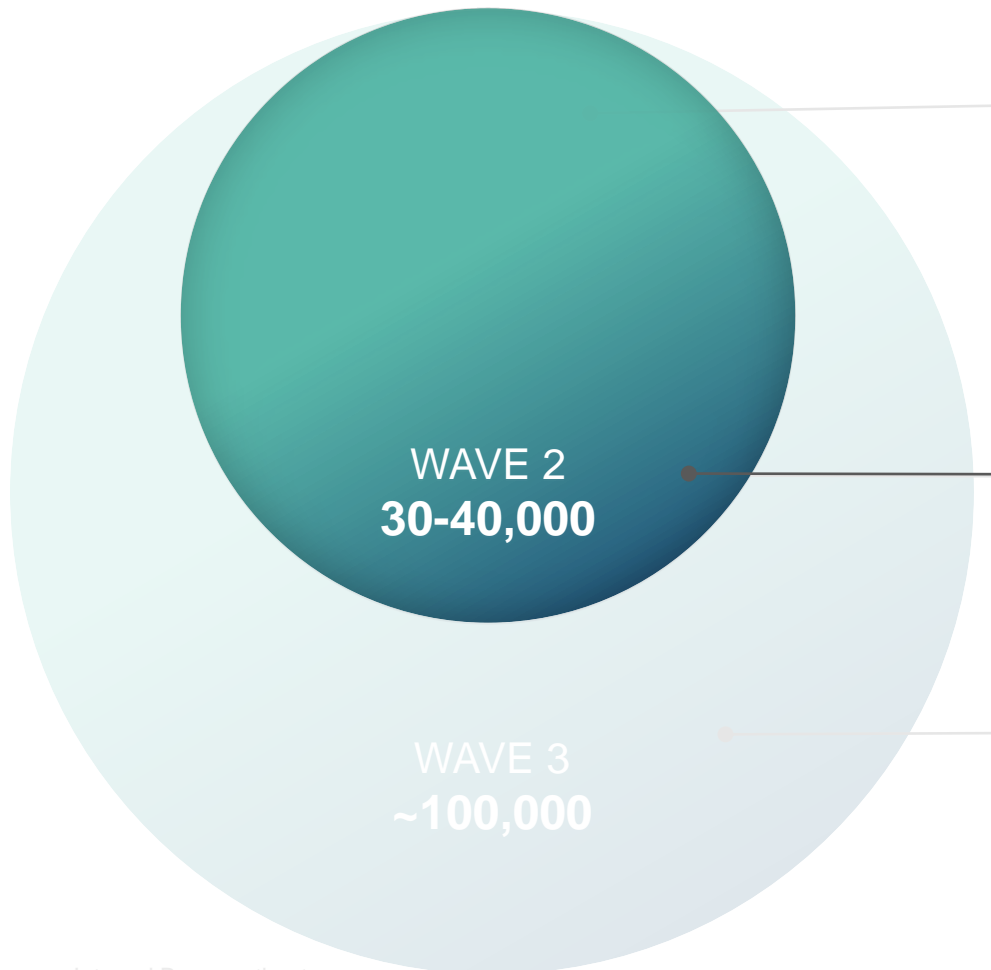
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- Broader age range
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In vivo: Base editing with HSC-targeted LNPs

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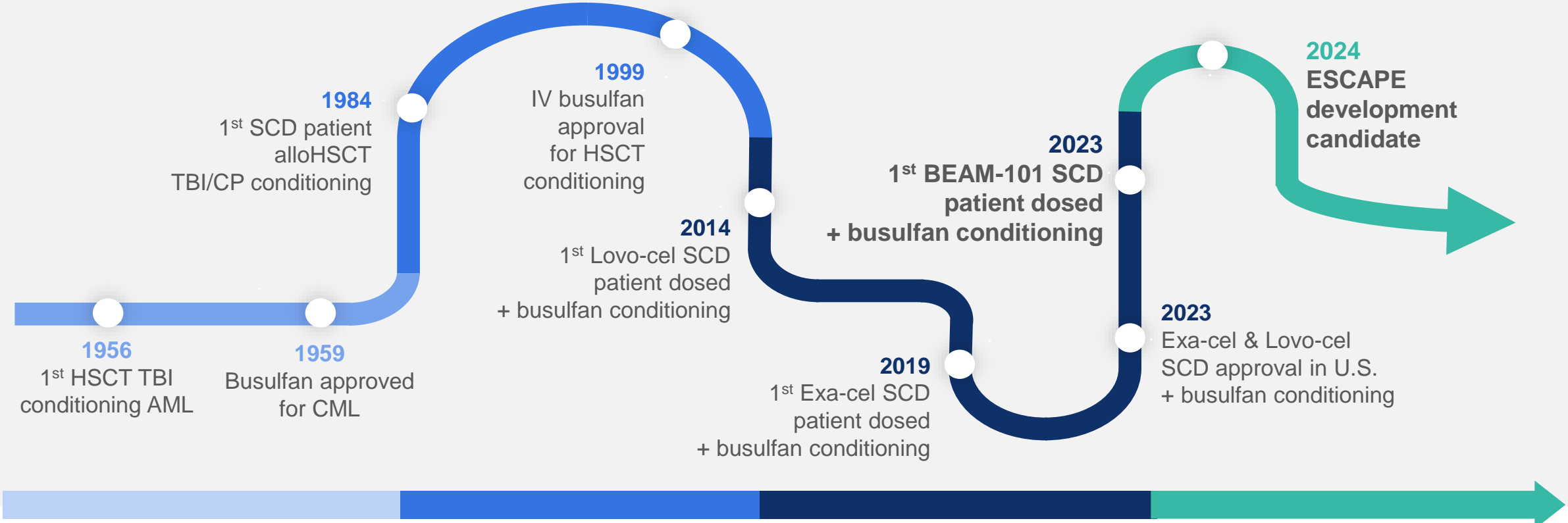
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ESCAPE technology designed to bring a paradigm shift to conditioning for the first time in nearly 70 years



68 years of genotoxic conditioning:

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

Promise of non-genotoxic conditioning

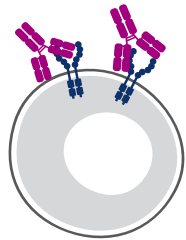
- Prevent acute and chronic toxicities
- Preserve fertility
- Potential to be outpatient

ESCAPE technology designed for selective depletion of diseased cells to enable non-genotoxic conditioning

ESCAPE technology for hemoglobinopathies is comprised of two components:

BEAM-103

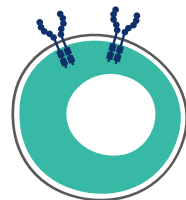
Anti-CD117 conditioning monoclonal antibody (mAb), which binds to the specific epitope edited by ESCAPE



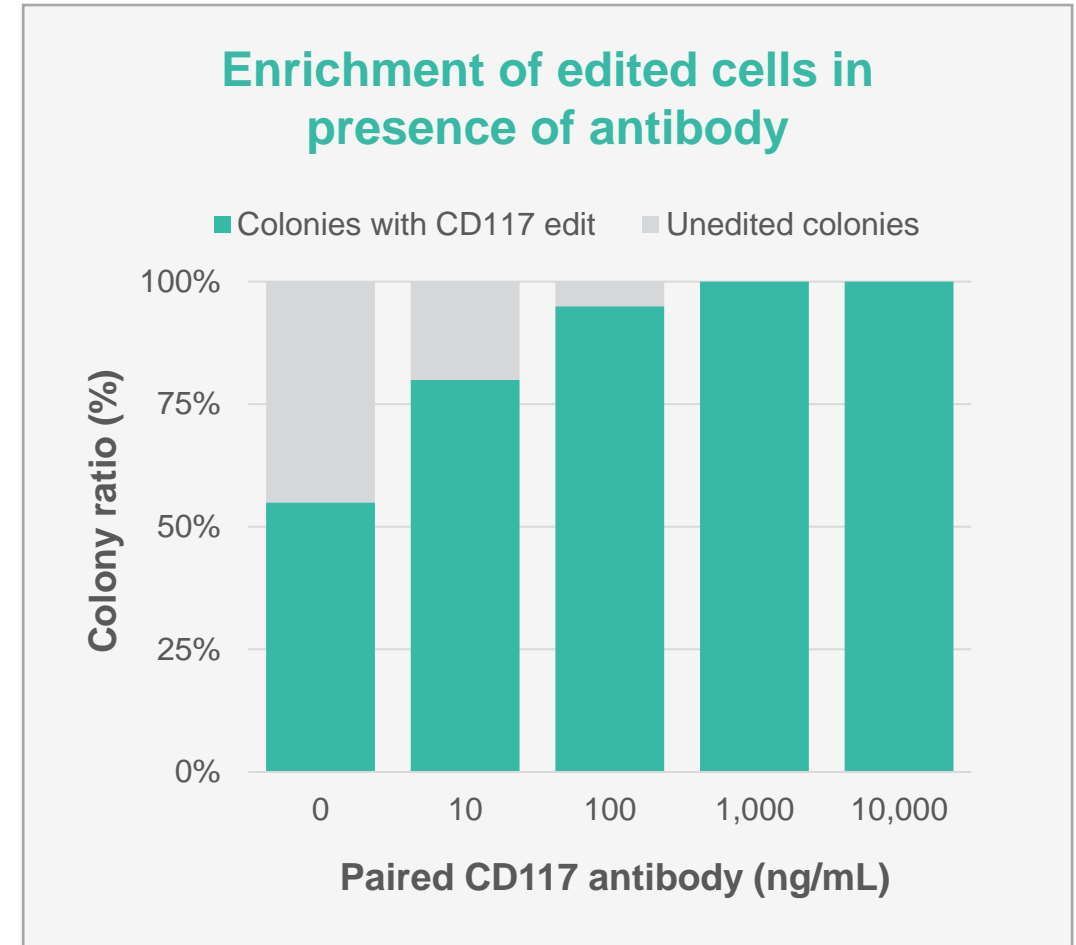
Old diseased cells are suppressed and eliminated by BEAM-103, creating space for the graft

BEAM-104

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



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



Data presented at FASEB 2022

Assessing ESCAPE in a rigorous non-human primate (NHP) model of autologous transplant



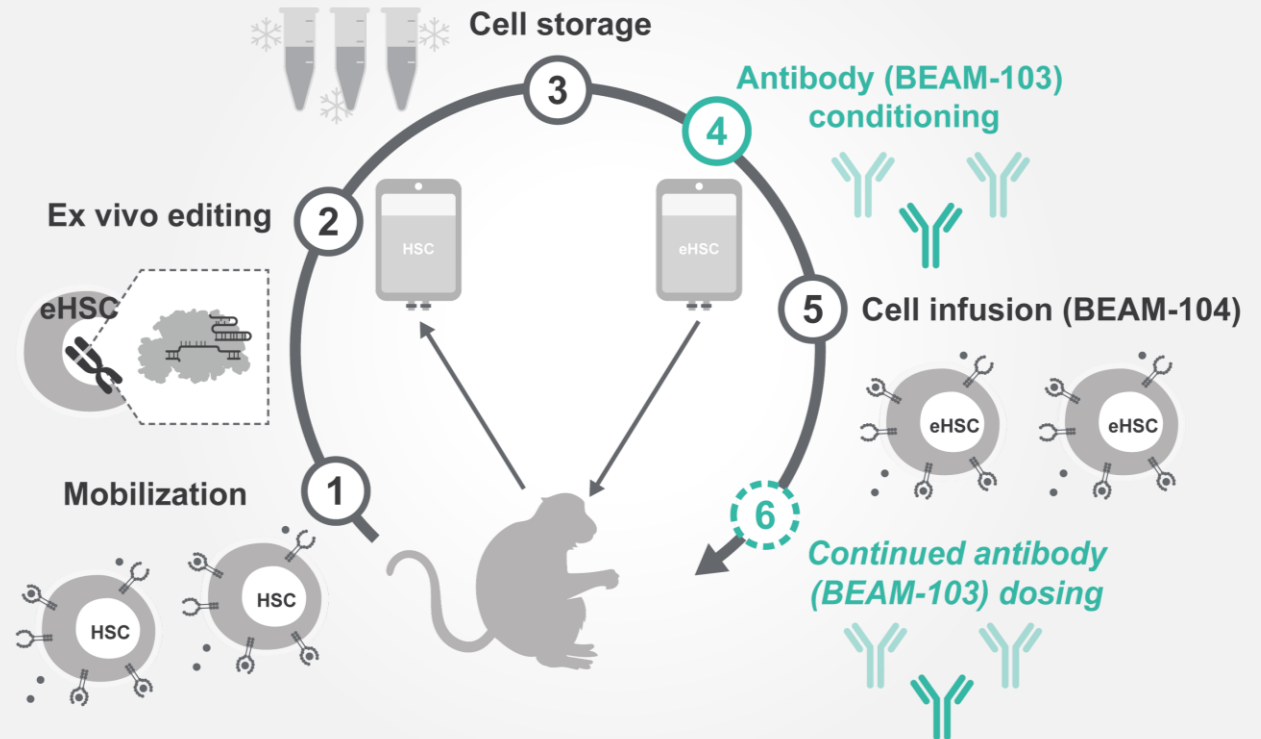
ASH 2024



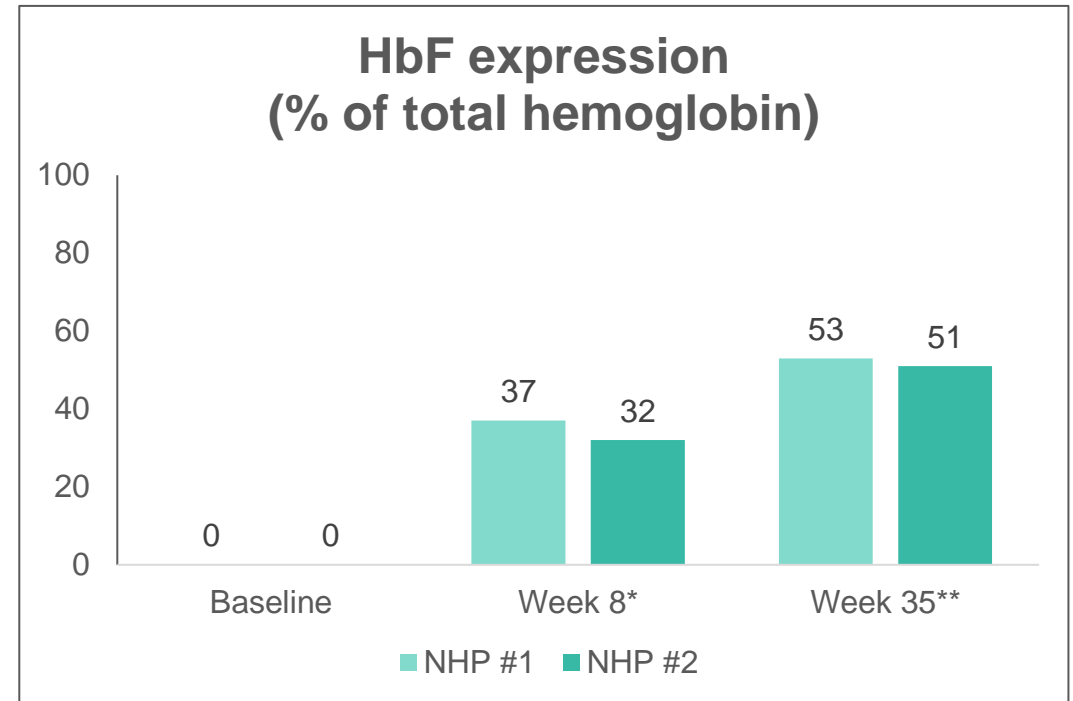
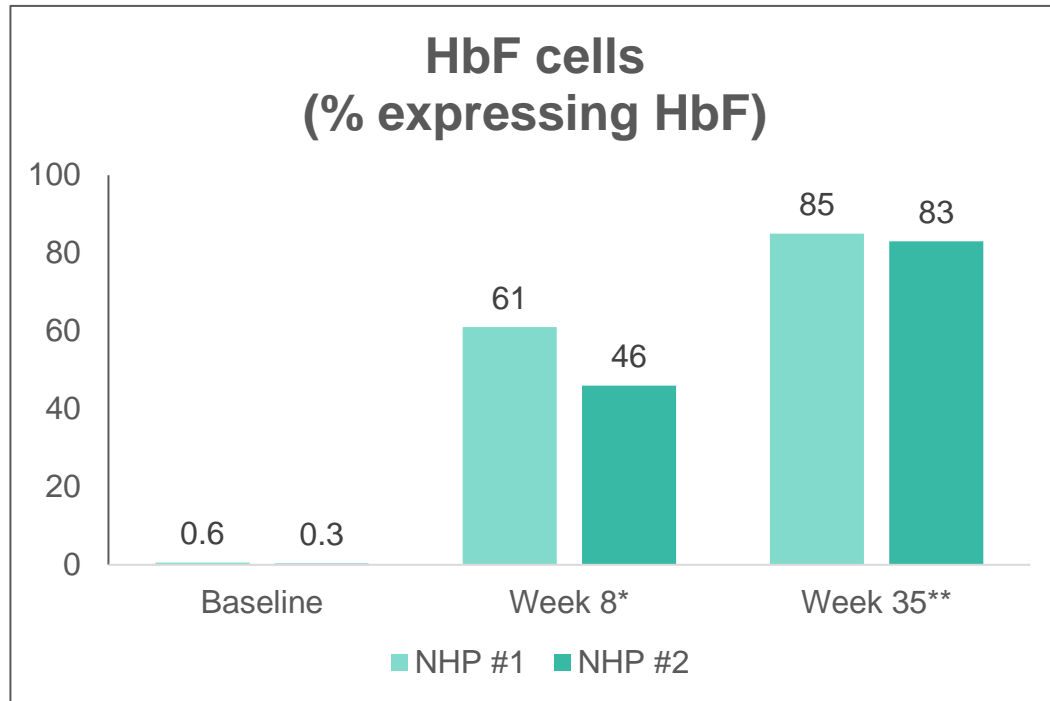
Proof-of-concept of non-genotoxic antibody conditioning

- Can engraftment of HSCs be achieved without chemotherapy?
- Can a therapeutic level of fetal hemoglobin induction be achieved?

Study design: Complete *ex vivo* gene editing transplant with antibody conditioning



Long-term engraftment of base edited CD34 cells after antibody conditioning in NHPs led to high % F cells and HbF %



Antibody dosed prior to infusion and monthly through week 35

- Proof of concept of long-term engraftment after antibody conditioning, without chemotherapy, in NHPs
- % F-cells and HbF % at therapeutic thresholds comparable to gene therapy after 3-6 months
- mAb well tolerated – no myeloablation, no supportive care necessary

*9 weeks for NHP #2

**34 weeks for NHP #2

Anticipated next steps for ESCAPE



1

- Follow-up NHP studies to optimize antibody dose regimen, dose response and chimerism
 - Additional data expected in 2025

2

- GMP manufacturing initiated
- Initiate Phase 1-enabling tox studies by YE 2024



3

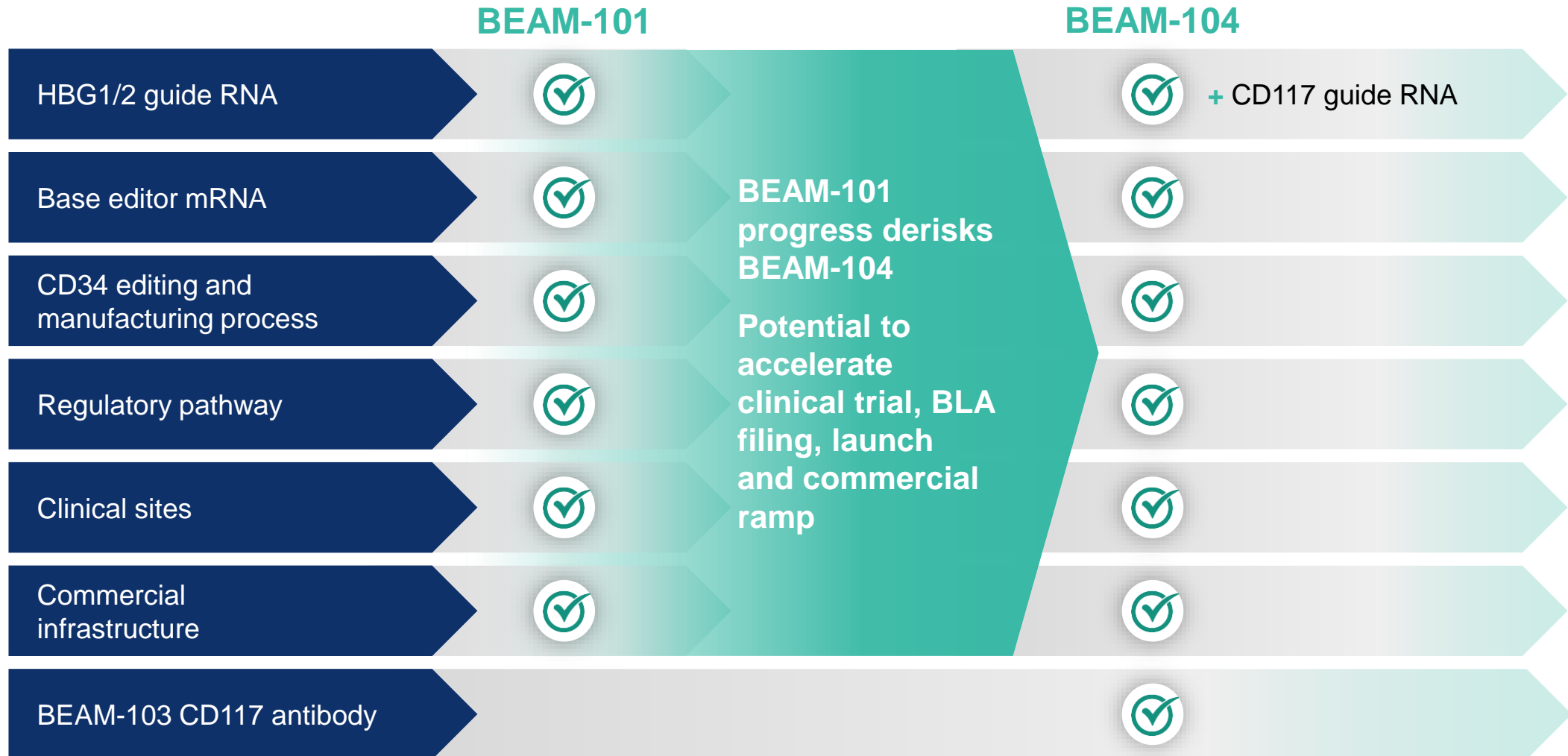
- Phase 1 study of BEAM-103 antibody to evaluate PK/PD and safety in healthy volunteers



4

- Efficacy study of BEAM-103 and BEAM-104 in SCD and beta-thalassemia patients

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



Significant step forward for Beam's hematology vision and base editing platform



BEAM-101 in SCD

Potentially differentiated clinical profile, comparable to sickle cell trait

ESCAPE NHP data

Potential to eliminate chemotherapy from transplant and expand the SCD market

Base editing

Technology validated, with strong translation from preclinical to clinical

BEAM-201 Phase 1 Clinical Data

MULTIPLEX BASE EDITING FOR CELL THERAPY

BEAM-201 abstract highlights: Data demonstrate proof of concept of 1st quad-edited, allogeneic CAR-T cell therapy



BEAM-201 safety profile consistent with underlying disease, lymphodepletion and AEs associated with CAR-T therapy



Pharmacokinetic (PK) and safety data support continued Phase 1 dose exploration



Early evidence of clinical efficacy with CAR-T cell doses <200 million, as measured by CRi/CR in 2/3 patients



Additional data will be presented at ASH Annual Meeting

Recent and Anticipated Catalysts

BEAM-101 SCD

Completed  sentinel dosing and initiated expansion


Present initial clinical data at ASH


ESCAPE SCD & BETA- THALASSEMIA

Initiate Phase 1-enabling preclinical studies in 2024

Present NHP preclinical data at ASH

BEAM-302 AATD

CTA cleared in the UK 

Initiate Phase 1/2 clinical trial 

Present initial data in 2025

BEAM-301 GSD1a

Obtained U.S. IND clearance 

Dose first patient in Phase 1/2 study in early 2025

BEAM-201 T-ALL / T-LL

Present initial clinical data at ASH



Q & A