Beam @ EHA 2025

June 13, 2025

Beam event participants

TOPIC	PARTICIPANT
Introduction	**Holly Manning** Vice President, Investor Relations & External Communications
Beam Overview & Sickle Cell Disease Strategy	**John Evans** Chief Executive Officer
Clinical Data from BEACON Trial of BEAM-101	**Ashish Gupta, M.D., MPH** University of Minnesota
BEAM-101 Exploratory Biomarker Data	**Amy Simon, M.D.** Chief Medical Officer
Manufacturing Process Performance to Date for BEAM-101	**Giuseppe Ciaramella, Ph.D.** President
Closing Remarks	**Mr. Evans**
Q&A	**Dr. Gupta, Mr. Evans, Dr. Simon, Dr. Ciaramella & John Lo,** Chief Commercial Officer

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, AATD, GSDIa, and ESCAPE; our plans, and anticipated timing, to advance our programs; the clinical trial designs and expectations for BEAM-101, BEAM-301, BEAM-302 and ESCAPE; our potential presentations at the EHA 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, 2024, 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**

Beam THERAPEUTICS

People like Branden, living with sickle cell disease, are at the heart of our vision

> “From age 12, things skyrocketed. I was in the hospital every other month with crises.”

In sixth grade, Branden had his left hip replaced because of damage to his hip joint.

His right hip was replaced not long after.

At age 17, he suffered a life-threatening acute chest syndrome (ACS), with sickled cells blocking blood vessels in his lungs.

Four more ACS episodes that year caused Branden to miss his entire junior year of high school.

From BOSTON CHILDREN’S HOSPITAL ANSWERS BLOG
‘A sickle cell first’
Posted December 9, 2024

Key elements of Beam's leadership position in gene editing

BASE EDITING PLATFORM
Clinical Validation, Fully Integrated Manufacturing

HIGH VALUE FRANCHISES
with Best-in-Class Potential

RAPID EXECUTION
of Clinical Programs

MULTIPLE CATALYSTS
Expected in 2025

Ended first quarter 2025 with $1.2 billion in cash, cash equivalents and marketable securities, including net proceeds from $500 million financing; Cash runway expected to support operating plans into 2028

A comprehensive, fully integrated base editing platform

BASE EDITING PLATFORM
Clinical Validation, Fully Integrated Manufacturing

GENE EDITING AND DELIVERY TECHNOLOGIES

• Base editing	✓ *Clinical proof of concept*
• *ex vivo* cell therapy	✓ *Clinical proof of concept*
• *in vivo* LNP	✓ *Clinical proof of concept*

FULLY INTEGRATED CAPABILITIES

GMP manufacturing at NC facility

>100 GMP batches/ isolations

Global regulatory filings

8 IND/CTA approvals
5 countries
3 designations

Global clinical operations

30+ clinical sites
>40 patients treated

INTERNAL GMP MANUFACTURING FACILITY

Research Triangle, NC

Beam's differentiated, growing liver genetic disease portfolio

HIGH VALUE FRANCHISE
with Best-in-Class Potential

- Best-in-class potential for BEAM-302 in alpha-1 antitrypsin deficiency (AATD) that addresses root cause of disease
- Potential one-time treatment for AATD lung and liver disease under normal gene regulation
- Platform for future liver-targeted pipeline

RAPID EXECUTION
of Clinical Programs

- ✓ Initial data for BEAM-302 demonstrated patients achieved corrected AAT above 11uM, the protective threshold
- ✓ BEAM-302 IND cleared by FDA
- ✓ RMAT Designation and ODD granted to BEAM-302
- ✓ Dosed first patient in BEAM-301 Phase 1/2 study in GSDIa

MULTIPLE CATALYSTS
Expected in 2025

BEAM-302 AATD

- Complete Part A dose escalation and initiate Part B dose escalation
- Present updated data in 2H25

BEAM-301 GSDIa

- Continue to dose and enroll patients

RMAT = Regenerative Medicine Advanced Therapy designation; ODD = orphan drug designation

Beam's multi-wave hematology portfolio

HIGH VALUE FRANCHISE
with Best-in-Class Potential

- Best-in-class potential for BEAM-101 in sickle cell disease (SCD)
- Well established FDA path to BLA
- Lifecycle strategy with ESCAPE and *in vivo* editing
- Platform for future hematology pipeline

RAPID EXECUTION
of Clinical Programs

- ✓ Updated data demonstrating best-in-class potential for BEAM-101 in SCD at EHA
- ✓ Adult and adolescent enrollment in BEACON complete
- ✓ 26 patients dosed as of June 13, 2025
- ✓ ODD granted to BEAM-101

MULTIPLE CATALYSTS
Expected in 2025

BEAM-101 SCD

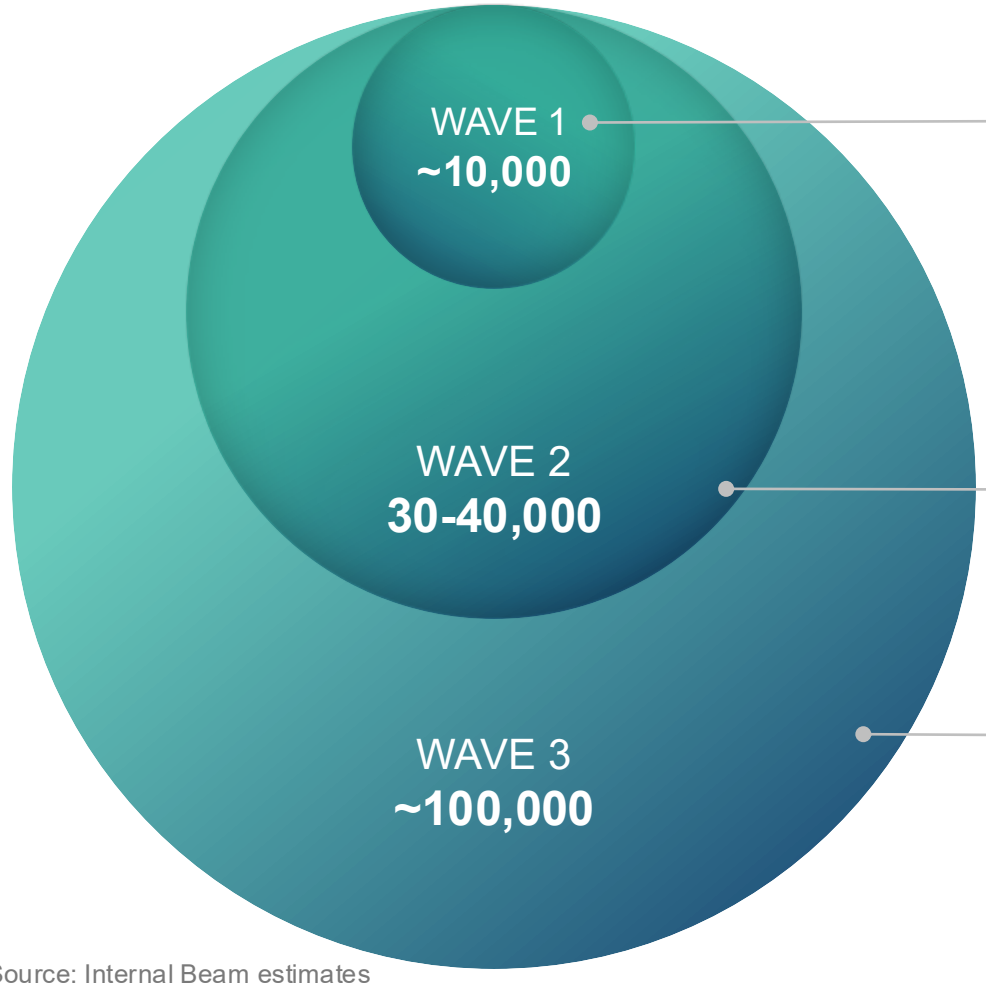
- Dose 30 patients by mid-2025
- Present updated data by YE 2025

ESCAPE SCD & BETA-THAL

- Initiate Phase 1 healthy volunteer trial of BEAM-103 CD117 antibody by YE

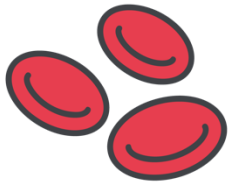
Beam's multi-wave strategy is focused on developing safer, more effective and more accessible treatments for patients with SCD

Potential Eligible SCD Patient Population (U.S.)



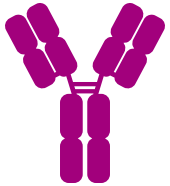
BEAM-101: Precise HbF upregulation

Potentially best-in-class clinical profile
Deepest correction with a non-cutting, non-viral therapy to address most severe SCD



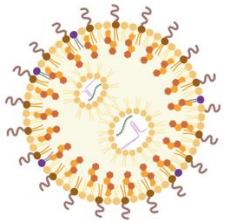
ESCAPE: Multiple edits for non-genotoxic conditioning

Designed to eliminate chemotherapy from transplant and expand patient population



IN VIVO: Direct base editing of stem cells with LNPs

Maximally scalable delivery to reduce infrastructure and maximize patient access



Source: Internal Beam estimates

Severe SCD gene therapy market is poised for steady growth with significant annual demand in U.S.

Projected* peak potential of $3-4 billion revenue per year

- In line with CAR-T experience (10-20% of clinically eligible)
- Demand and capacity analysis suggests 1,200-1,600 patients per year

Viable pricing in place at $2-3 million, and every patient treated is profitable

- Reimbursement environment is improving with no rejections of prior authorizations
- New CMMI model aims to accelerate access; covers 84% of eligible Medicaid patients

Market infrastructure for SCD gene therapy is building momentum

- Qualified treatment centers gaining experience with gene therapy and reimbursement process
- Overall patient experience (# mobilization cycles, days in hospital, time to dose, efficacy/safety) will influence therapy choice and capacity to treat for providers

Early signals that demand is outpacing supply

* Beam market research

Feedback from transplant and SCD KOLs indicates strong patient demand in U.S.

"The demand for gene therapy in SCD will be dictated by the industry's ability to supply."

– Northeast U.S. KOL

"We have patients knocking at the door every day. The industry cannot handle the volume of patients that have shown interest, even if they are able to open up capacity."

– Southeast U.S. KOL

"There are around 500-600 eligible patients in this metropolitan area alone. There is no one company who can handle this volume."

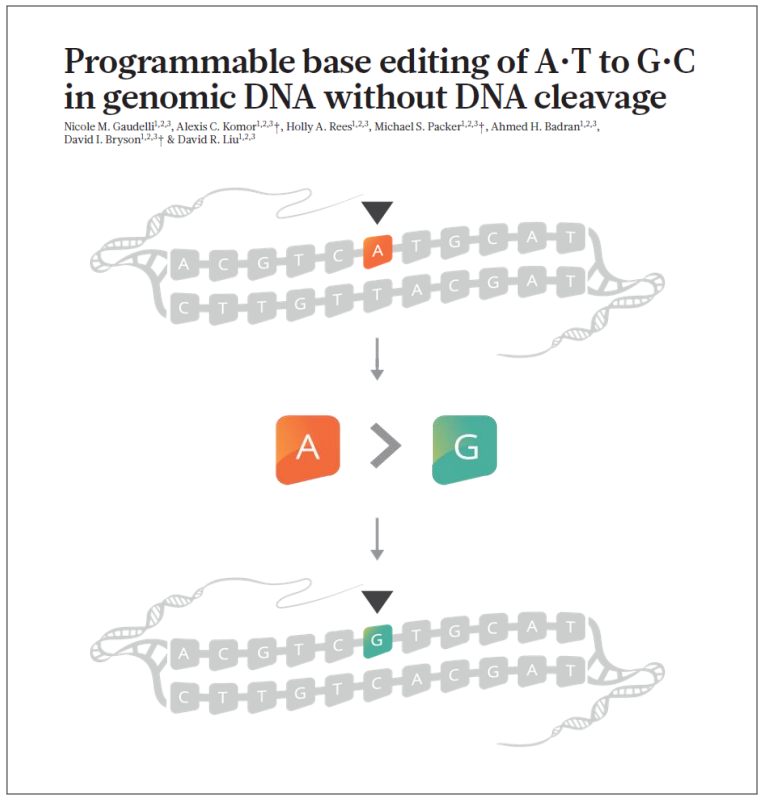
– Northeast U.S. KOL

"We have 40-50 patients on our list currently, and we need to triage who should go forward when."

– Western U.S. KOL

Base editing enables a next-generation investigational genetic therapy for severe SCD

BASE EDITING → BEAM-101 FOR SEVERE SCD



Designed for no double stranded breaks

Potential for healthier cells and faster engraftment

No genetic insertions

Non-viral delivery

High levels of editing

90%+ editing rate for BEAM-101 in preclinical studies

Uniform product edits

Uniform cell editing with higher HbF production and lower residual HbS

Gaudelli, N., et al. Programmable base editing of A•T to G•C in genomic DNA without DNA cleavage. Nature 551, 464–471 (2017).
Mayuranathan, T., Newby, G.A., Feng, R. *et al.* Potent and uniform fetal hemoglobin induction via base editing. *Nat Genet* **55**, 1210–1220 (2023).

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

BEACON trial data provide preliminary evidence of potential differentiation of base editing and BEAM-101 for SCD

HIGH LEVELS OF CORRECTION

- All patients achieving HbF levels >60% and HbS levels <40%, meeting or exceeding levels seen in sickle cell trait
- Anemia resolved, and markers of hemolysis and oxygen delivery normalized or improved in all patients

LESS TIME IN HOSPITAL

- Efficient cell collection process resulting in median of 1 mobilization cycle
- Rapid neutrophil and platelet engraftment with low number of neutropenic days, reducing number of in-hospital days

FLEXIBLE, HIGH YIELD PROCESS

- Beam NC facility allows flexible scheduling for patients and sites
- Consistently high yields and viability, enabling successful manufacturing of BEAM-101 dose

Data from BEACON trial as of Feb 28, 2025

Base Editing for Sickle Cell Disease

Ongoing Results from the BEACON Study Evaluating the Safety and Efficacy of BEAM-101, the First Base-edited Autologous CD34+ HSPC One-time Cell Therapy

ASHISH GUPTA, M.D., MPH
UNIVERSITY OF MINNESOTA

EHA Abstract #PF1151

Beam THERAPEUTICS

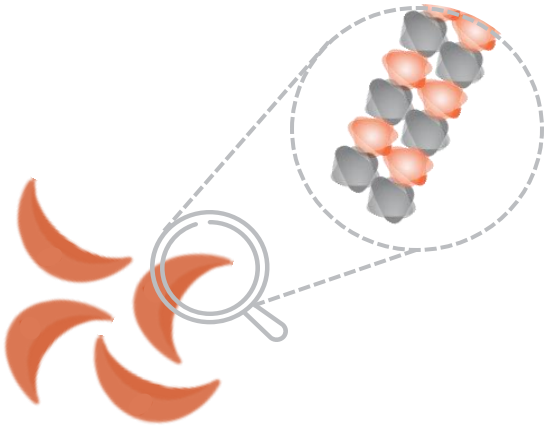
BEACON

BEAM-101 uses precise base editing to increase levels of HbF

Sickle cell disease[1,2]

HbS ↓ HbF ↑

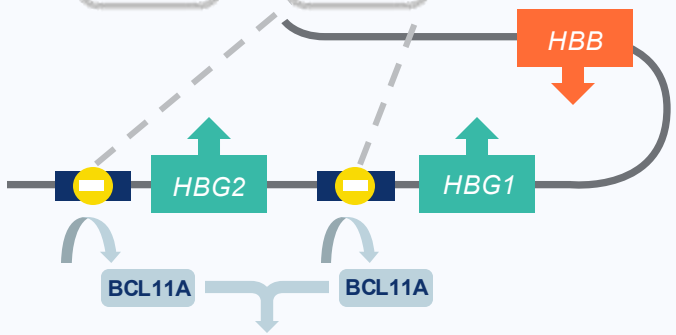
Increased HbS leading to sickled cells



Base editing with BEAM-101[3,4]

Editing of *HBG1/2* promoters is designed to eliminate BCL11A binding sites without disrupting other BCL11A-regulated pathways

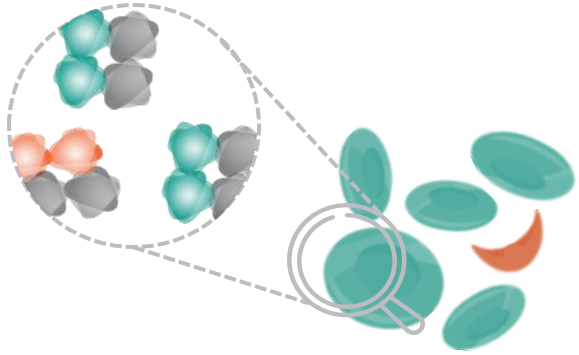
Does not require double-stranded breaks



Following treatment with BEAM-101[2,5]

Increased HbF leading to fewer sickled cells

HbF ↑ HbS ↓



1. Eaton WA, Bunn HF. Blood 2017;129:2719–2726; 2. Akinsheye I, et al. Blood 2011;118:19–27; 3. Beam Therapeutics Inc. Protocol BTX-AUT-001; 4. Beam Therapeutics Inc. Investigator's brochure; 5. Steinberg MH, et al. Blood 2014;123:481–485. A, adenine; BCL11A, transcription factor B-cell lymphoma/leukemia 11A; CRISPR, clustered regularly interspaced short palindromic repeats; G, guanine; HBB, hemoglobin subunit beta; HBG, hemoglobin subunit gamma; HbF, fetal hemoglobin; HbS, sickle hemoglobin; RNA, ribonucleic acid

BEACON is a Phase 1/2 study evaluating the safety and efficacy of BEAM-101 in patients with SCD and sVOCs

Key eligibility criteria

- Age ≥12 to ≤35 years
- SCD with β^S/β^S, β^S/β^0, or β^S/β^+ genotypes
- ≥4 sVOCs in 24 months prior to screening
- No available matched sibling donor
- No history of overt stroke

Key safety endpoints

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

Key efficacy endpoints

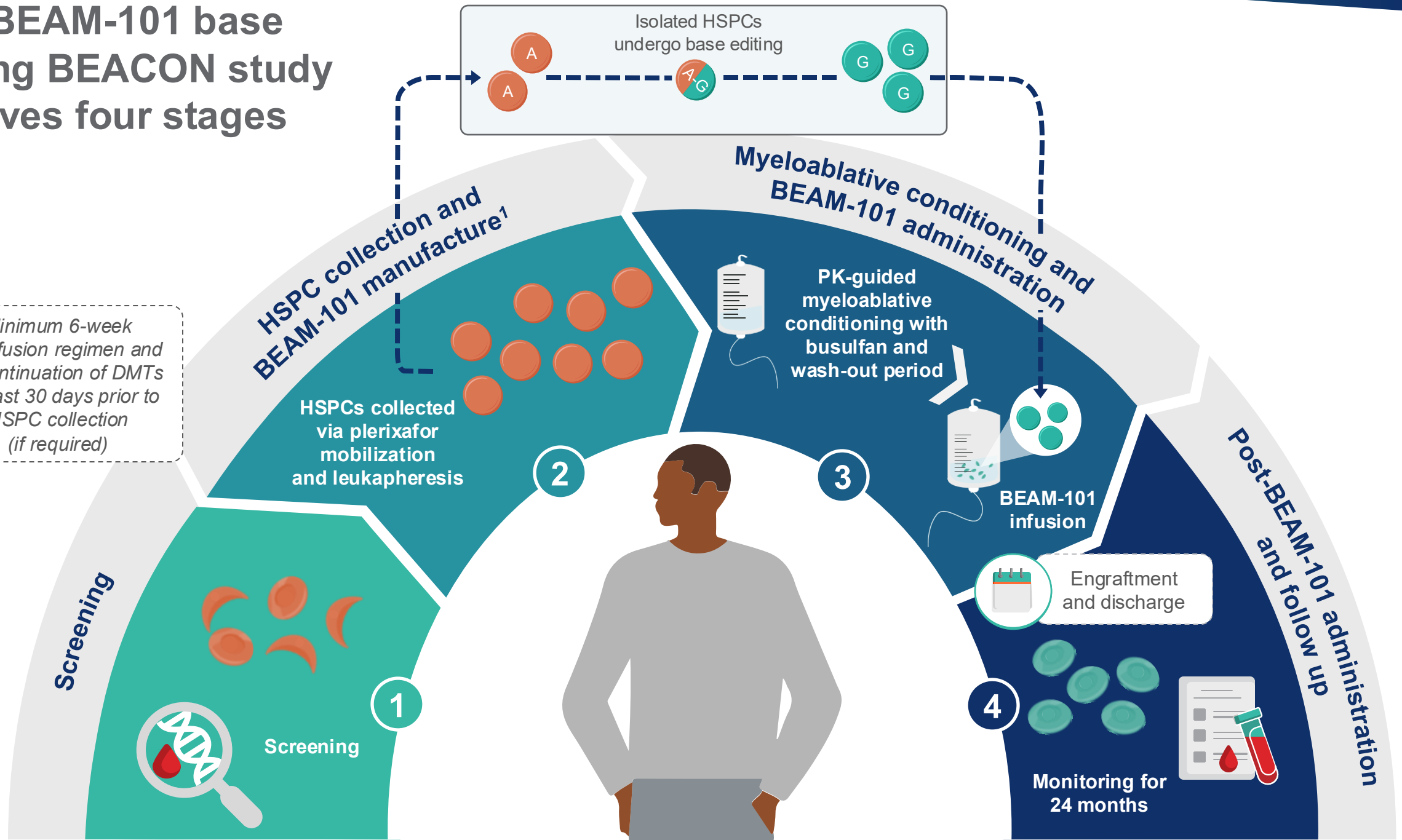
- Proportion of patients sVOC-free for 12 consecutive months*
- Proportion of patients who were hospitalization-free for sVOCs for at least 12 months*
- Total Hb levels
- HbF and HbS levels
- Hemolysis parameters
- RBC function and organ damage

More information on Poster PF1155

Adult enrollment completed; adolescent enrollment initiated; expect to dose 30 patients by mid-year

Phase 1/2, non-randomized, open-label, single-arm, multicenter, safety and efficacy study of the administration of BEAM-101 to patients with SCD (NCT05456880).
To qualify as a sVOC, the event must consist of acute episodes of pain, with no medically determined cause other than a VOC that required at least 24 hours of management in a hospital or observation unit; or a visit to an emergency department, urgent care, or outpatient facility involving therapy with an opioid or IV or IM NSAID; or ACS, as defined by the acute onset of pneumonia-like symptoms (e.g., cough, fever, shortness of breath) along with new pulmonary infiltrates; or splenic sequestration crisis, as defined by left upper quadrant pain, splenic enlargement, and a decrease in Hb of ≥2 g/dL; or priapism episode, defined as a sustained, unwanted, painful erection requiring evaluation and treatment at a medical facility. *From 60 days after last RBC transfusion.
ACS, acute chest syndrome; b, hemoglobin; HbF, fetal hemoglobin; HbS, sickle hemoglobin; IM, intramuscular; IV, intravenous; NSAID, non-steroidal anti-inflammatory drug; RBC, red blood cell; SCD, sickle cell disease; sVOC, severe vaso-occlusive crisis
Beam Therapeutics Inc. Protocol BTX-AUT-001

The BEAM-101 base editing BEACON study involves four stages



1. Kopesky P, et al. Poster presented at EHA; Jun 2024; Madrid, Spain

DMT, disease-modifying therapy; HSPC, hematopoietic stem and progenitor cell; PK, pharmacokinetic

Baseline demographics and characteristics of patients treated with BEAM-101

Baseline characteristics	N=17
Age (years), mean (range)	22.9 (18–34)
Sex, n (%)	
Male	10 (58.8)
Female	7 (41.2)
Genotype, n (%)	
β^S/β^S	16 (94.1)
β^S/β^0	1 (5.9)
Race, n (%)	
Black or African American	15 (88.2)
Previous hydroxyurea use, n (%)*	10 (58.8)
Investigator-reported sVOCs in the 2 years prior to start of study, median (range)	9 (4–18)

Safety and efficacy analysis: N=17

Length of follow up in analysis set: 3.7 months (range: 0.2–15.1)

Data from BEACON trial as of Feb 28, 2025. *Taking at screening.
ACS, acute chest syndrome; Hb, hemoglobin; IM, intramuscular; IV, intravenous; NSAID, non-steroidal anti-inflammatory drug; sVOC, severe vaso-occlusive crisis

BEAM-101 treatment and engraftment characteristics

Treatment	N=17
Number of mobilization and apheresis cycles, median (range)	1 (1–3)
Estimated average AUC of Busulfan for entire conditioning (μg*h/mL), mean (range)*	70.5 (50.2–89.2)
BEAM-101 dose infused (×10^6 CD34+ cells/kg), mean (range)	7.9 (3.2–23.4)
Duration (months) of follow up after BEAM-101 dosing, mean (range)	3.7 (0.2–15.1)
Day of last RBC transfusion, median (range)	15 (1–122†)

Patients required a median of 1 mobilization cycle and 3 days of total mobilization for manufacture

Data from BEACON trial as of Feb 28, 2025. Therapeutic drug monitoring for busulfan was performed and dosing was adjusted based upon plasma busulfan concentrations to maintain a daily target busulfan AUC of 20 μg*h/mL with a cumulative AUC target of 80 μg*h/mL. Neutrophil engraftment defined as ANC ≥500 cells/μL for 3 consecutive days independent of growth factor support. Platelet engraftment defined as post-nadir platelet count ≥50,000/μL on 3 separate days without receiving a platelet transfusion for at least 7 days prior to the first of the three measurements through to the last measurement. *n=14; AUC could not be calculated for the remaining three patients as busulfan pharmacokinetic data were incomplete; †one patient required blood transfusions up to Day 122 as part of ongoing management of critical illness; excluding this patient, the range of last day of RBC transfusion is 1–26 days

ANC, absolute neutrophil count; AUC, area under the curve; RBC, red blood cell

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Duration (months) of follow up after BEAM-101 dosing, mean (range)	3.7 (0.2–15.1)
Day of last RBC transfusion, median (range)	15 (1–122†)
Neutrophil engraftment‡	**N=16**
Achieved neutrophil count ANC ≥500 cells/μL for 3 consecutive days, n (%)	16 (100)
Time to neutrophil engraftment (days), median (range)	16.5 (12–30)
Duration of severe neutropenia (ANC <500 cells/μL) (days), median (range)	7.0 (4–17)
Platelet engraftment§¶	**N=14**
Achieved platelet count ≥50,000/μL on 3 separate days, n (%)‖	14 (100)
Time to platelet engraftment (days), median (range)	19.5 (11–34)
Did not require a platelet transfusion, n (%)	7 (50)

Patients achieved rapid neutrophil and platelet engraftment with few neutropenic days

Data from BEACON trial as of Feb 28, 2025. Therapeutic drug monitoring for busulfan was performed and dosing was adjusted based upon plasma busulfan concentrations to maintain a daily target busulfan AUC of 20 μg*h/mL with a cumulative AUC target of 80 μg*h/mL. Neutrophil engraftment defined as ANC ≥500 cells/μL for 3 consecutive days independent of growth factor support. Platelet engraftment defined as post-nadir platelet count ≥50,000/μL on 3 separate days without receiving a platelet transfusion for at least 7 days prior to the first of the three measurements through to the last measurement. *n=14; AUC could not be calculated for the remaining three patients as busulfan pharmacokinetic data were incomplete; †one patient required blood transfusions up to Day 122 as part of ongoing management of critical illness; excluding this patient, the range of last day of RBC transfusion is 1–26 days; ‡at the time of datacut; one patient achieved neutrophil engraftment post-datacut on Day 19; §at the time of datacut; three patients achieved platelet engraftment post-datacut on Day 27, 28, and 50; ¶four patients experienced ≥Grade 4 thrombocytopenia; ‖three patients' platelets did not drop below 50,000/μL and did not have any platelet transfusions. ANC, absolute neutrophil count; AUC, area under the curve; RBC, red blood cell

BEAM-101 updated safety data were consistent with busulfan conditioning, autologous HSCT, and underlying SCD

Patients with, n (%)	N=17
Any TEAEs	17 (100)
Related to BEAM-101*	1 (5.9)
Any TEAEs ≥Grade 3	15 (88.2)
Related to BEAM-101*	0
AEs leading to discontinuation	0
Serious TEAEs†	6 (35.3)
Related to BEAM-101	0
Death‡	1 (5.9)
Related to BEAM-101	0

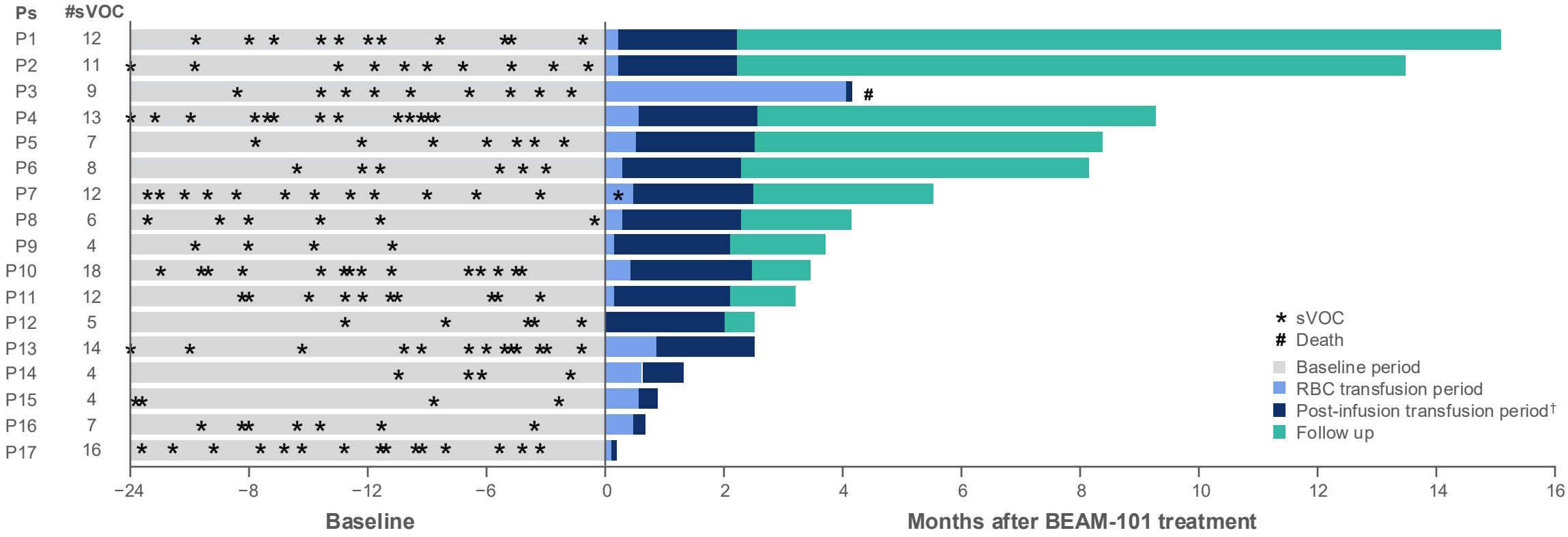
Data from BEACON trial as of Feb 28, 2025. Related events include events where investigator has assessed relationship as possibly or definitely related to BEAM-101. *After the datacut, the number of patients with related TEAEs was one (non-serious Grade 1 dizziness), and the number of patients with ≥Grade 3 related TEAEs was zero, following site data clarification; †serious TEAEs included sickle cell anemia with crisis, retinal hemorrhage, nausea, device-related infection, septic shock, vascular access complication, acute kidney injury, urinary retention, pneumomediastinum, pulmonary fibrosis, and respiratory failure; ‡one patient died due to respiratory failure, likely related to busulfan conditioning, 4 months after infusion. AE, adverse event; TEAE, treatment-emergent adverse event

BEAM-101 updated safety data were consistent with busulfan conditioning, autologous HSCT, and underlying SCD

TEAE (≥3 patients), n (%)	N=17
Stomatitis*	12 (70.6)
Febrile neutropenia*	11 (64.7)
Skin hyperpigmentation	5 (29.4)
Decreased appetite*	5 (29.4)
Hypokalemia	5 (29.4)
Anemia*	4 (23.5)
Constipation	4 (23.5)
Headache	4 (23.5)
Hypertension	4 (23.5)
Platelet count decreased	4 (23.5)
Hypervolemia	3 (17.6)
Hypomagnesemia	3 (17.6)
Nausea	3 (17.6)
Edema (peripheral)	3 (17.6)
Pharyngeal inflammation	3 (17.6)
White blood cell count decreased	3 (17.6)

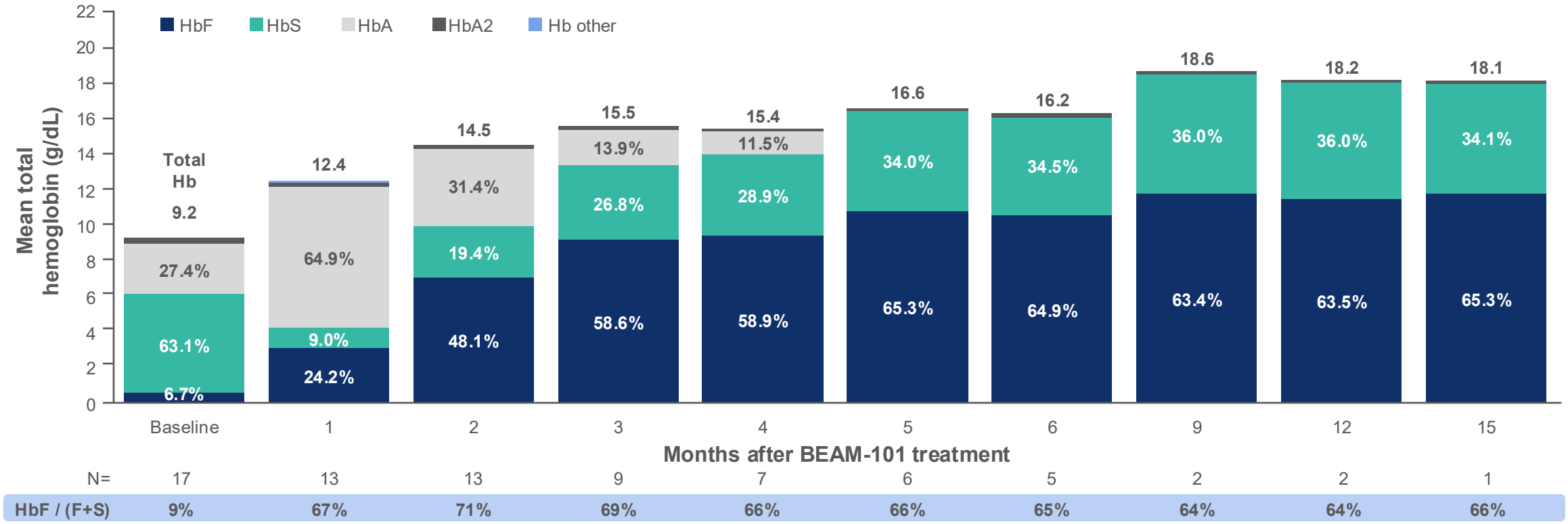
Data from BEACON trial as of Feb 28, 2025.
*Includes events that were ≥Grade 3 in at least three patients. TEAE, treatment-emergent adverse event

No patients have reported any VOCs post-engraftment



Data from BEACON trial as of Feb 28, 2025.
†60 days post last RBC transfusion. Investigator-reported VOCs reported in this figure have not been formally adjudicated. P, patient; RBC, red blood cell; (s)VOC, (severe) vaso-occlusive crisis

All patients achieved rapid and durable increases in HbF >60% and decreases in HbS <40%

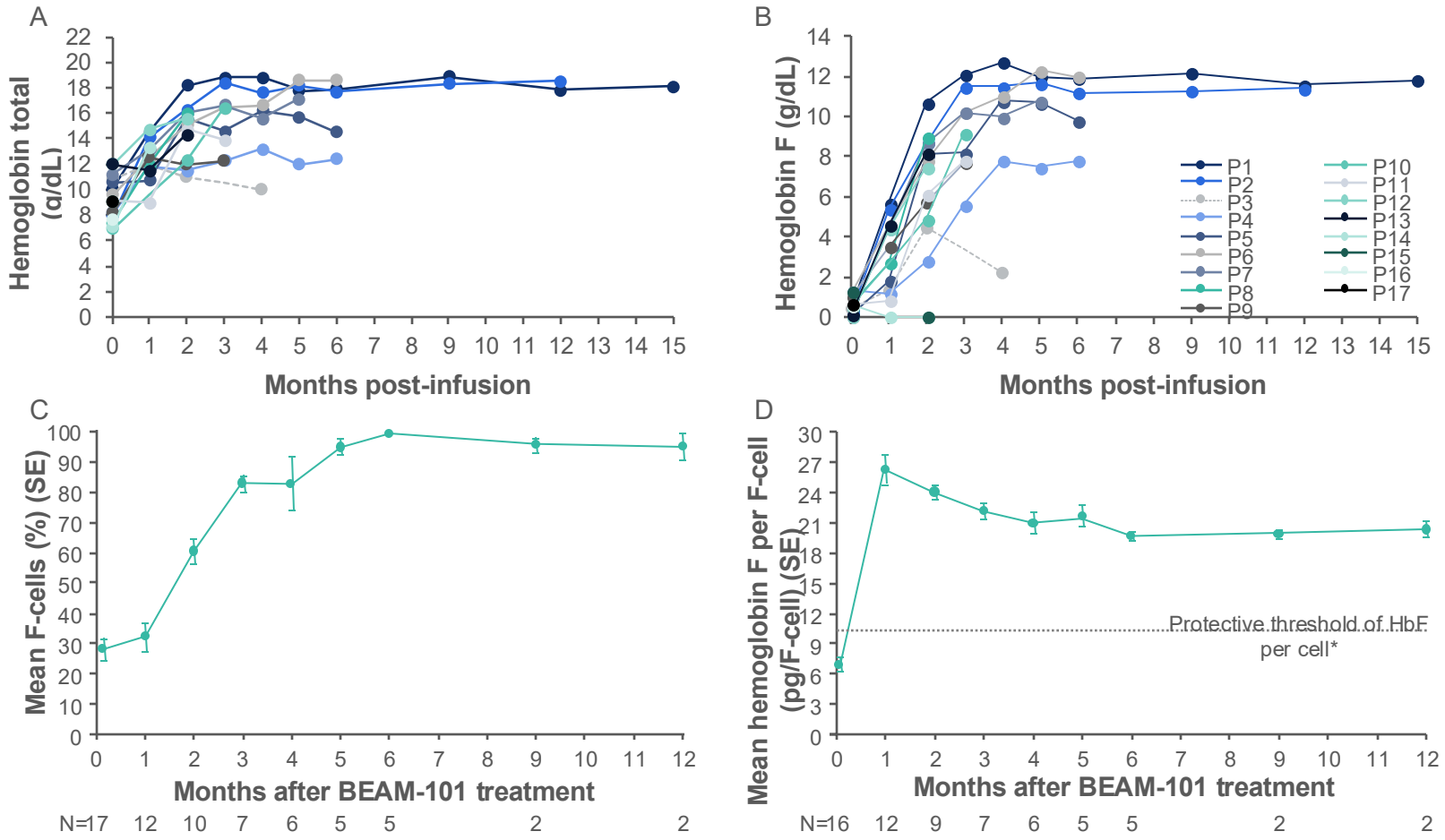


	Baseline	1	2	3	4	5	6	9	12	15
N=	17	13	13	9	7	6	5	2	2	1
HbF / (F+S)	**9%**	**67%**	**71%**	**69%**	**66%**	**66%**	**65%**	**64%**	**64%**	**66%**

Data from BEACON trial as of Feb 28, 2025. Female total Hb LLN-ULN: 11.5–15 g/dL; male LLN-ULN: 13–17 g/dL. HbF % is calculated as a % of untransfused blood (HbF/HbF+HbS). Hb, hemoglobin; HbA, adult hemoglobin; HbF, fetal hemoglobin; HbS, sickle hemoglobin; LLN, lower limit of normal; ULN, upper limit of normal

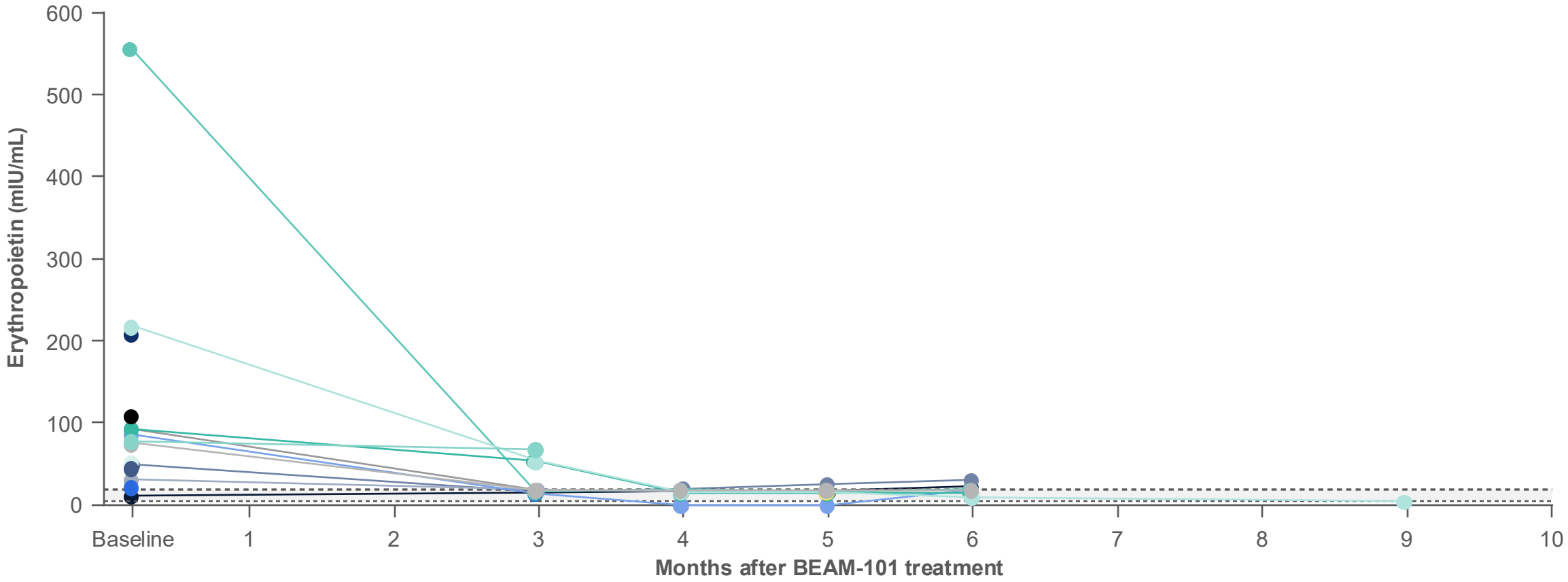
Rapid and robust increases in total Hb and HbF (>60%) were durable through follow up

- Pancellular HbF expression was observed following elimination of transfused blood
- Mean HbF (pg/cell) reached the protective threshold[1] by Month 1 and was sustained through follow up



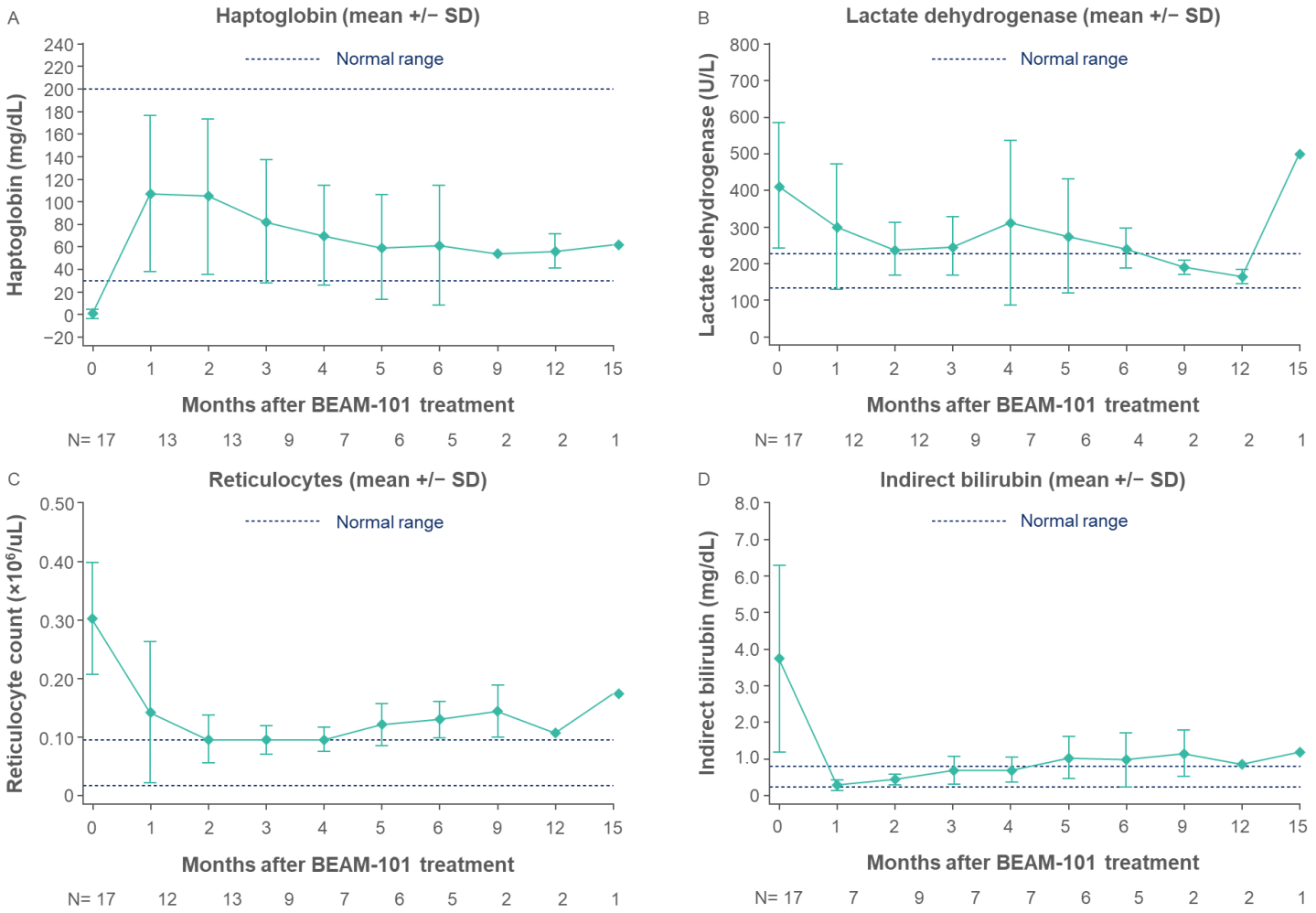
Data from BEACON trial as of Feb 28, 2025. Total Hb (A), HbF (B), mean F-cell (%) over time (C), and mean HbF per F-cell (pg/F-cell) over time (D). Female total Hb LLN-ULN: 11.5-15 g/dL; male LLN-ULN: 13-17 g/dL.
*Defined as the level of HbF that inhibits deoxyHbS polymerization.1 F, female; F-cell, HbF-containing cell
Hb, hemoglobin; HbF, fetal hemoglobin; HbS, sickle hemoglobin; LLN, lower limit of normal; M, male; P, patient; SE, standard error; ULN, upper limit of normal

Erythropoietin levels markedly decreased to normal or near normal, indicating adequate oxygen delivery to tissues



Data from BEACON trial as of Feb 28, 2025. HbA, adult hemoglobin; HbF, fetal hemoglobin; P, patients

Hemolysis markers normalized or improved following BEAM-101 treatment



Data from BEACON trial as of Feb 28, 2025. *The sample for Month 15 data point was hemolyzed; impacts of hemolysis on assay outcomes should be considered when interpreting results. SD, standard deviation

Updated BEACON trial safety data support trial continuation and demonstrated robust and sustained increases in HbF expression and resolution of anemia and VOCs in patients with SCD

- BEAM-101's **efficient collection and manufacturing process** resulted in patients requiring a **median of one mobilization cycle**
- Patients achieved **rapid neutrophil and platelet engraftment** with low number of neutropenic and thrombocytopenic days
- **Updated safety data with BEAM-101 were consistent** with busulfan conditioning, autologous HSCT and underlying SCD
- **No VOCs were reported** by investigators post-engraftment
- All patients achieved **rapid and robust increases in total Hb and HbF (>60%)**; **pancellular** distribution of HbF was **maintained, with HbF/F-cell maintained above the sickling threshold** through follow up
- All patients achieved **rapid and robust decrease in HbS (<40%) with resolution of anemia**, and markers of hemolysis were **normalized or improved** in all patients

Data from BEACON trial as of Feb 28, 2025.

Red Blood Cell (RBC) Health and Function Post BEAM-101 Treatment

Multiple Exploratory Biomarkers Demonstrate Rheology and Sickling Parameters Comparable to Sickle Cell Trait (SCT)

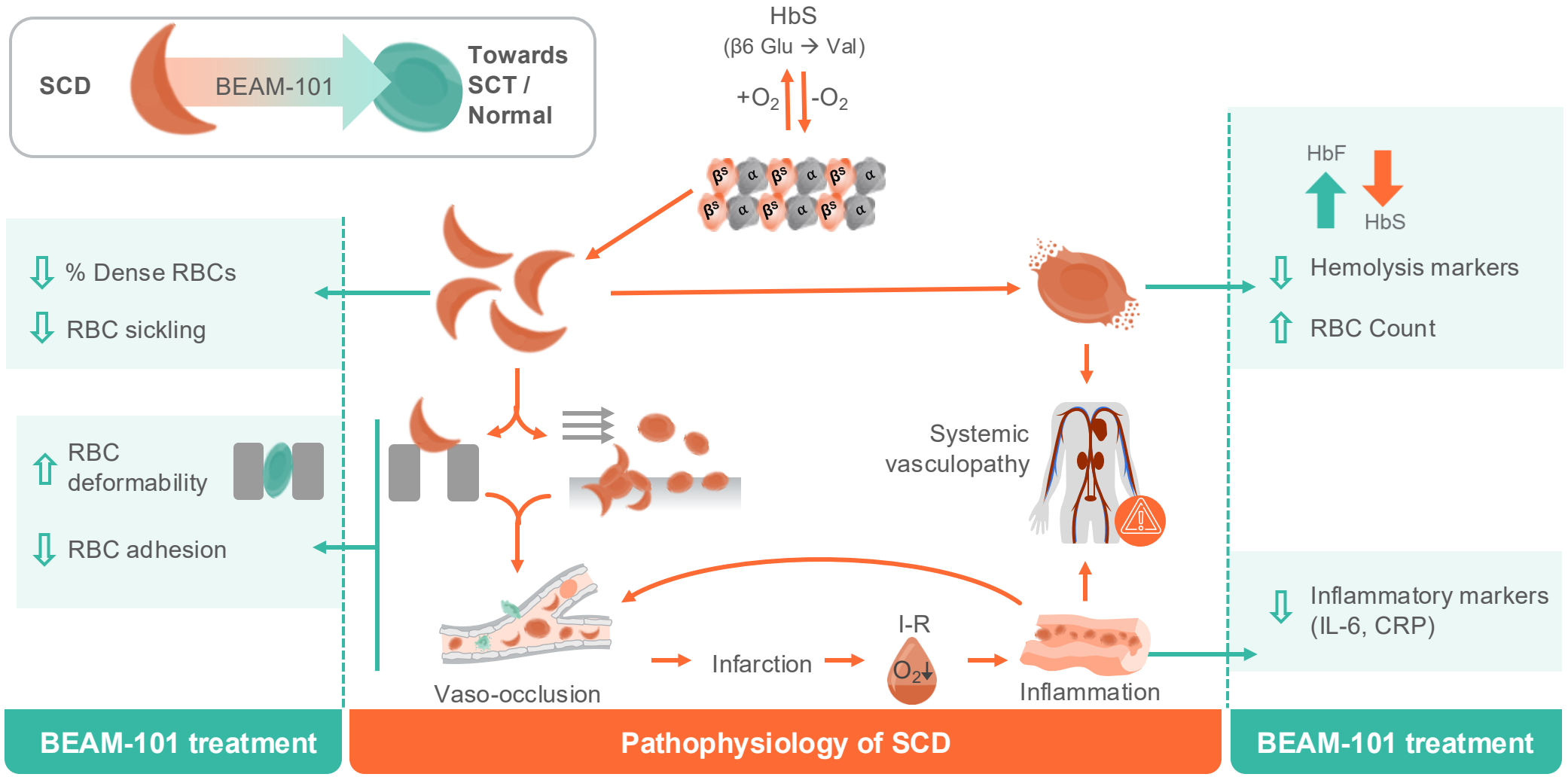
AMY SIMON, M.D.
CHIEF MEDICAL OFFICER

EHA Abstract #PF1155

Beam THERAPEUTICS

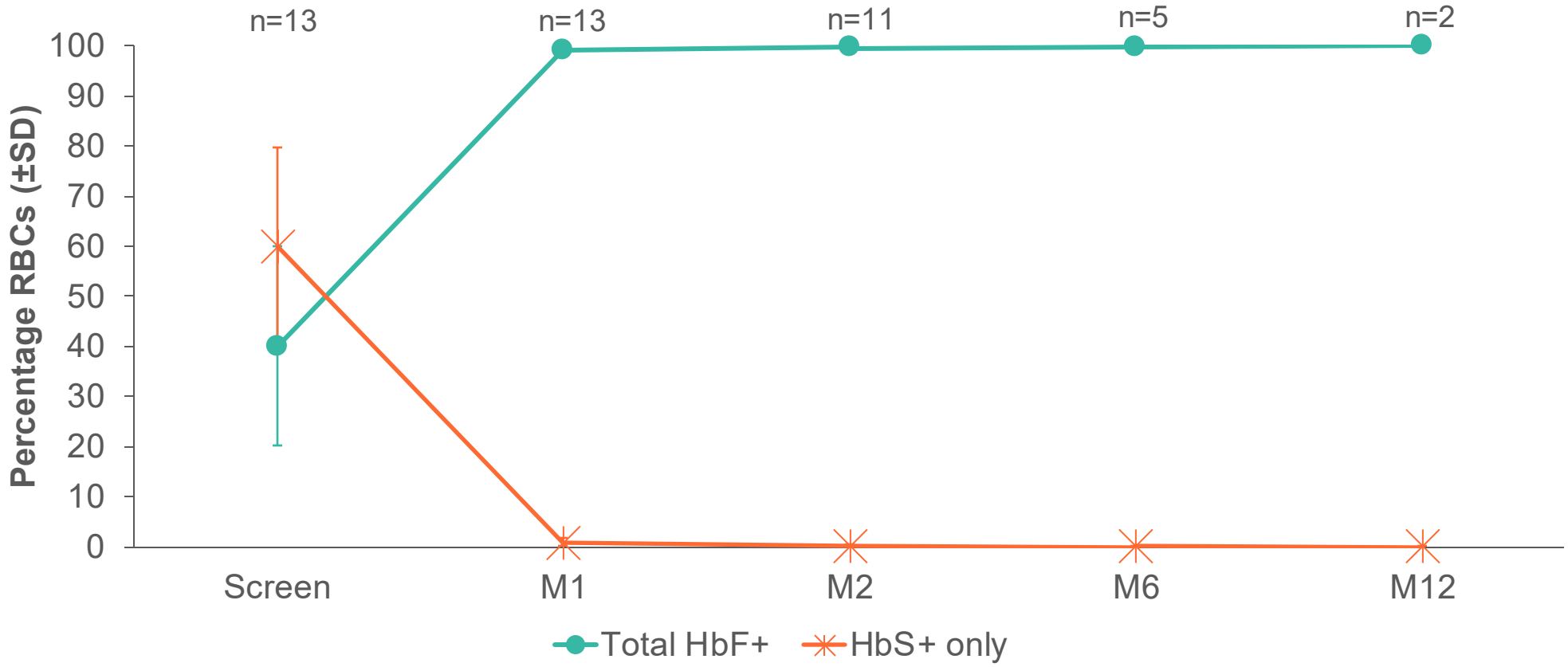
BEACON

What would improved RBC health and function look like post BEAM-101 treatment?



HbF, Fetal hemoglobin; HbS, Sickle hemoglobin; RBC, Red blood cell; SCD, Sickle cell disease; SCT, Sickle Cell Trait; I-R, Ischemia Reperfusion
Data cutoff 28 Oct 2024 for the biomarker data presented here.

Increasing HbF with near elimination of HbS-only cells post BEAM-101



After BEAM-101 treatment:

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

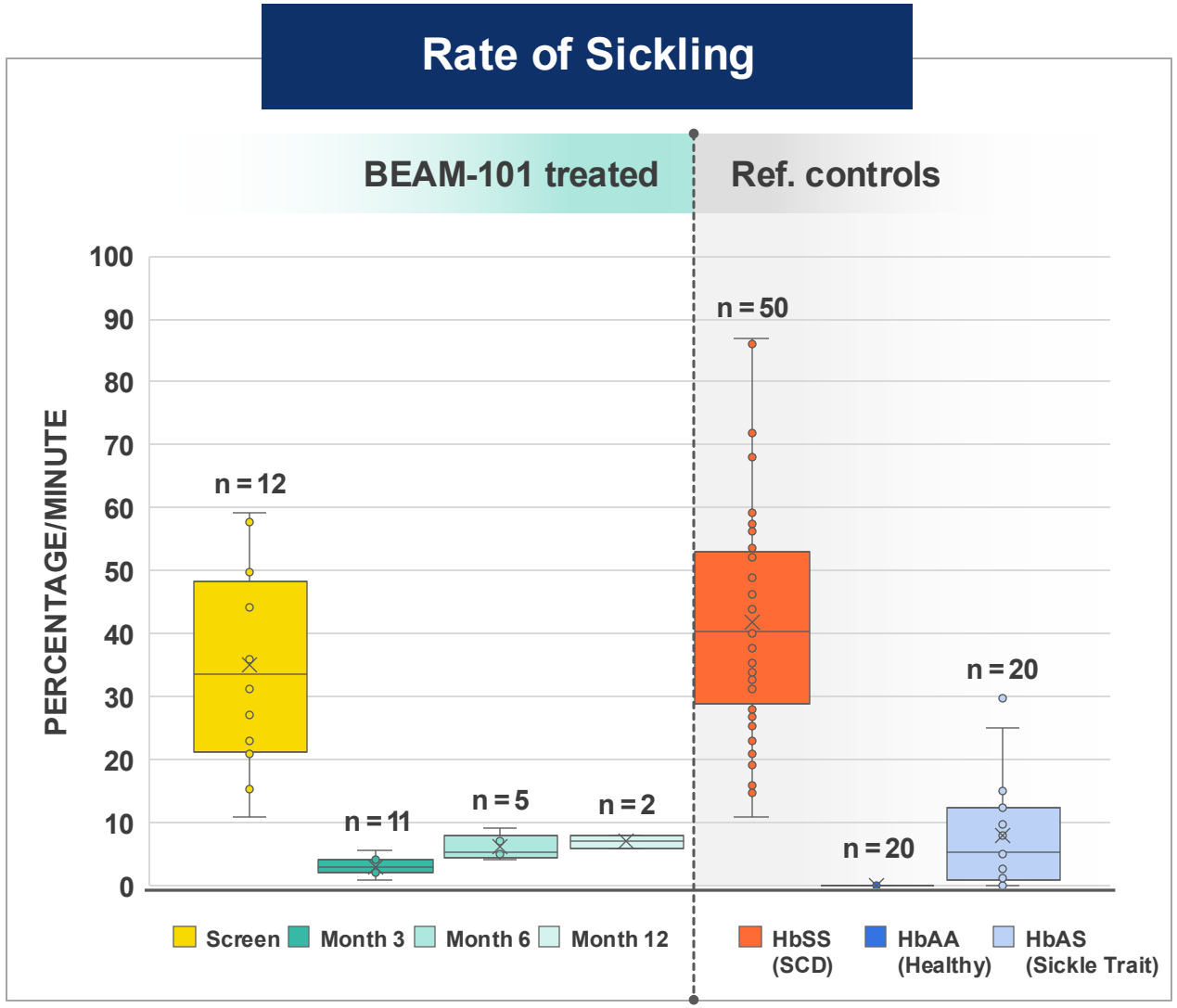
Data from BEACON trial as of April 2025. Duplex flow cytometry performed on un-transfused RBCs
HbF, fetal hemoglobin; HbS, sickle hemoglobin; M, month; RBC, red blood cell; SD, standard deviation

Reduced sickling post BEAM-101, to levels comparable to sickle cell trait

- Reduction in RBC sickling ***(see graph)*** and cell adhesion to levels comparable to SCT samples ***(n = 12)***

- Other RBC function improvements include increased deformability and decreased % dense RBCs ***(n = 5 at M6; n = 2 at M12)***

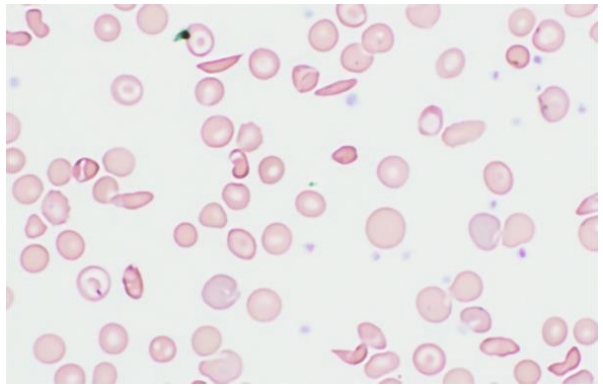
- Reductions in serum CRP and IL-6, indicating less systemic inflammation ***(n = 8 at month 3; n=6 at month 6)***



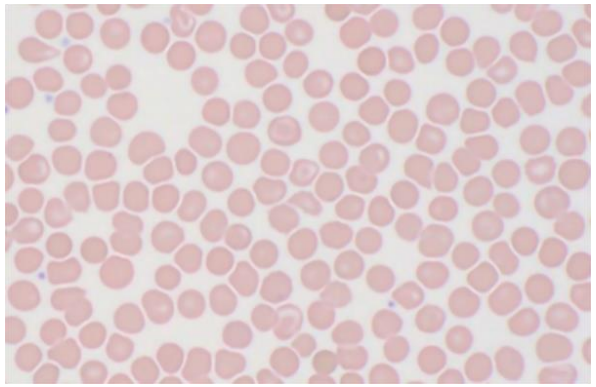
Data cut as of April, 2025
HbAA, healthy volunteer; HbAS/SCT, sickle cell trait; HbSS, sickle cell disease; M, month. o, individual data points; x, mean; -, median

Abnormal RBC morphology and sickle cells not detected post BEAM-101

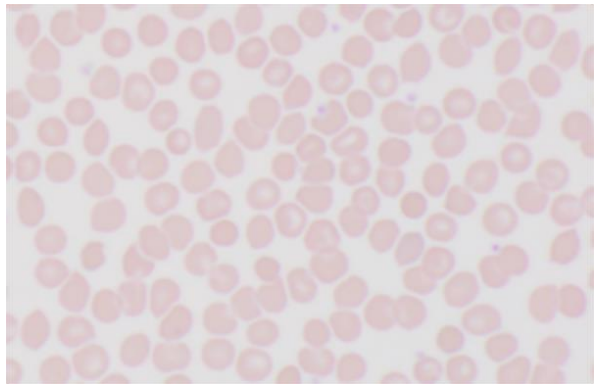
Changes in RBC morphology following treatment with BEAM-101



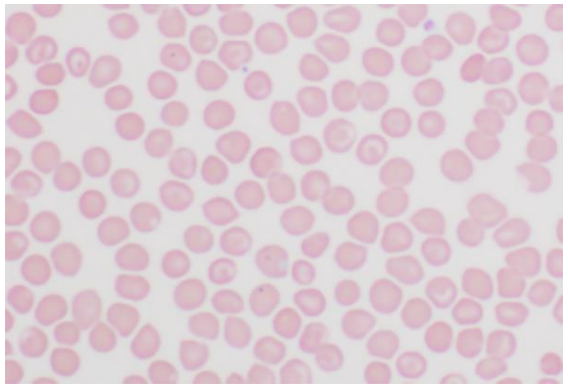
Patient 2
Pre-treatment



Month 3



Month 6



Month 12

Observation	Definition	Grade pre-treatment	Grade post-treatment
Sickle cells	Sickled RBC	1+ to 3+	Not detected
Polychromasia	Immature RBC	1+ to 2+	Not detected
Hypochromia	RBC of less color	1+ to 2+	Not detected
Elongated or crescent-shaped cells	Cells of abnormal shape and size	1+ to 2+	Not detected

3+ = ~≥10%
2+ = ~5–9%
1+ = ~1–4%

Data from BEACON trial as of April 2025.

Exploratory biomarkers in up to 13 patients suggest that BEAM-101 restored RBC health and function

- 99% of non-transfused RBCs expressed HbF, with near-complete elimination of RBCs expressing solely HbS, as early as month 1 post BEAM-101
- Cell adhesion reduced to significantly below the critical SCD threshold post BEAM-101, indicating a reduced risk for VOCs
- Reductions in multiple sickling parameters were comparable to sickle cell trait post BEAM-101
- Percentage dense RBCs, RBC deformability and systemic inflammation improved post BEAM-101
- Increase in RBC cell number and resolution of abnormal RBC morphology observed post BEAM-101

Data from BEACON trial as of Feb 28, 2025.

Manufacturing Process Performance to Date for BEAM-101

GIUSEPPE CIARAMELLA, PH.D.
PRESIDENT

EHA Abstract #PF1165

Beam THERAPEUTICS

Beam process has the potential to reduce time to dose and time in hospital, benefiting patients and the healthcare system

STANDARD PROCESS

Mobilization Cycle 1 → Mobilization Cycle 2 → Manufacture & Release → Transplant & Engraftment

POTENTIAL BEAM PROCESS ADVANTAGES

- Flexible scheduling with internal facility and patient-centric approach
- High yields from base editing
- Closed, automated manufacturing process
- In-house product quality testing
- Rapid time to neutrophil and platelet engraftment
- Less neutropenic days and hospital days

BEAM-101 EXPERIENCE
(as of data cut off, n=17 patients)

Mobilization Cycle 1 → Manufacture & Release → Transplant & Engraftment

Median 1 cycle

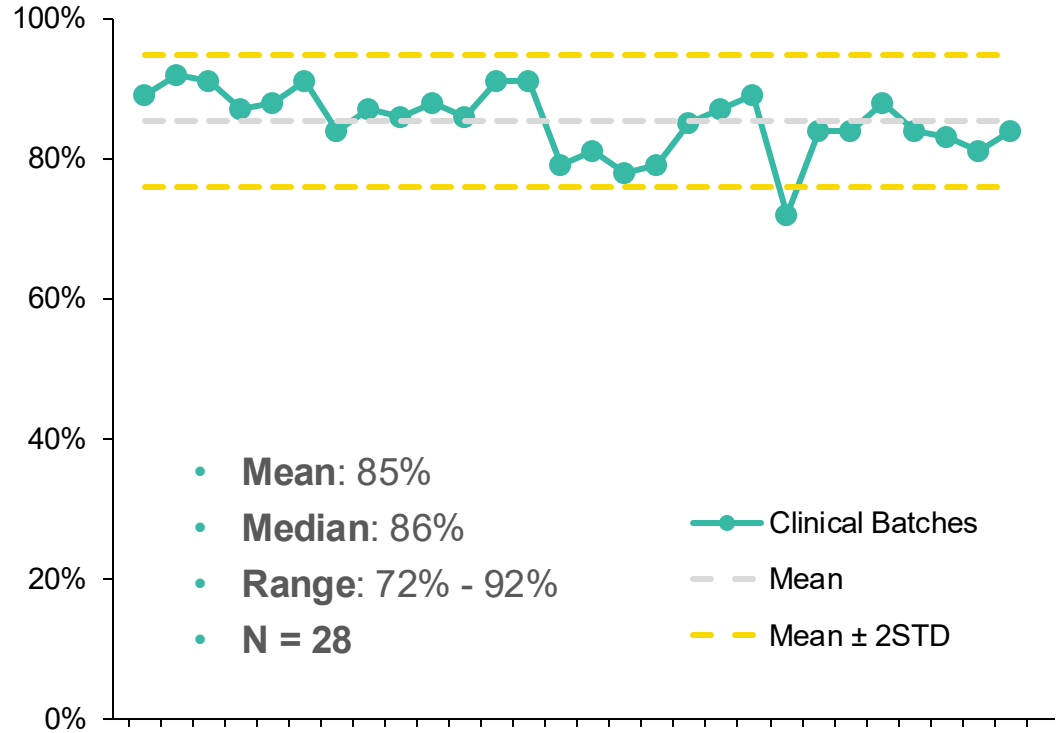
High success rate for release

Neutrophils: 16.5 days
Platelets: 19.5 days

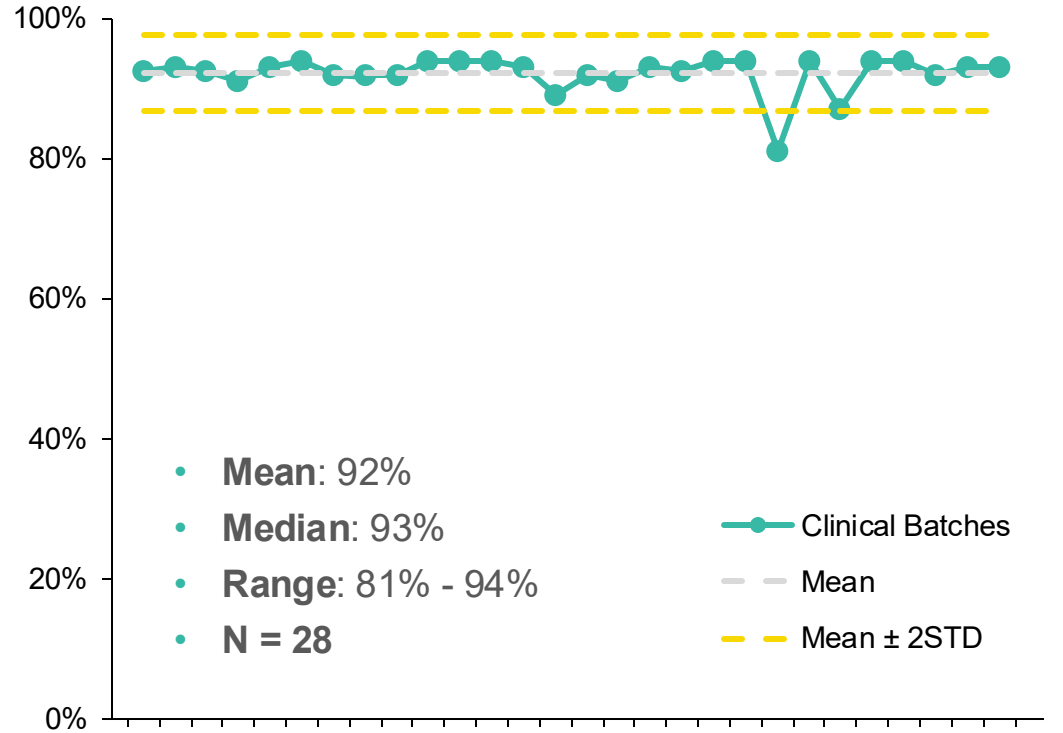
Data from BEACON trial as of Feb 28, 2025

Automation of every unit operation ensures robust and repeatable process performance and produces consistent, high-quality drug product

Viability



On-Target Editing Rate



Data cutoff Feb. 1, 2025

Rapid progress and strong patient demand for BEACON trial, with enrollment now complete

Fully enrolled

Adult and adolescent cohorts in the BEACON trial in under 18 months since first patient dosed

34

Patients with **manufactured and released drug product**

26

Patients dosed with BEAM-101 as of June 13, 2025

Orphan drug designation

Granted to BEAM-101 by U.S. FDA, providing potential regulatory and commercial benefits

Closing

Beam THERAPEUTICS

BEAM-101 positioned to be the best-in-class option for a growing, established severe SCD gene therapy market

Base editing underpins BEAM-101's clinical differentiation in SCD, driving deeper hematologic correction, reduced rounds of mobilization and faster time to engraftment

BEAM-101 uses an **advanced, largely automated and closed manufacturing process with consistently high yields and viability**, enabling successful BEAM-101 manufacturing

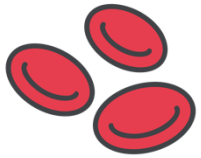
Clear, established path to U.S. BLA submission and launch with low regulatory risk

Positive signals emerging from SCD gene therapy launches: strong patient demand based on transplant and SCD KOL feedback, broad treatment center network now established, viable pricing in place and reimbursement improving

Projected $3-4 billion annual sales potential for gene therapy market in U.S.

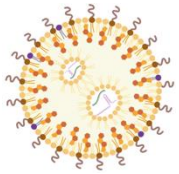
Long-term commitment to transforming sickle cell disease

Beam has assembled a best-in-class portfolio of technologies with potential to deliver curative therapies to sickle cell patients now and in the future



BEAM-101 for severe sickle cell disease

ESCAPE for non-genotoxic conditioning



LNP capabilities for *in vivo* delivery

Patients are at the heart of our vision

Kyle
SICKLE CELL DISEASE

Dan and Kathi
ALPHA-1 ANTITRYPSIN DEFICIENCY

Alyssa and Gayle
GLYCOGEN STORAGE DISEASE IA

Beam THERAPEUTICS