Updated Results from HGB-206 LentiGlobin for Sickle Cell Disease Gene Therapy Study: Group C Data and Group A AML Case Investigation

John F Tisdale, Alexis A Thompson, Janet L Kwiatkowski, Markus Y Mapara, Lakshmanan Krishnamurti, Banu Aygun, Kimberly A Kasow, Stacey Rifkin-Zenenberg, Manfred Schmidt, Alex Miller, Xinyan Zhang, Dennis Kim, Dustin Whitney, Hans Bitter, Phillip D Gregory, Melissa Bonner, Sunita Goyal, Jennifer J Jaroscak, Julie Kanter, Mark C Walters

Disclosure

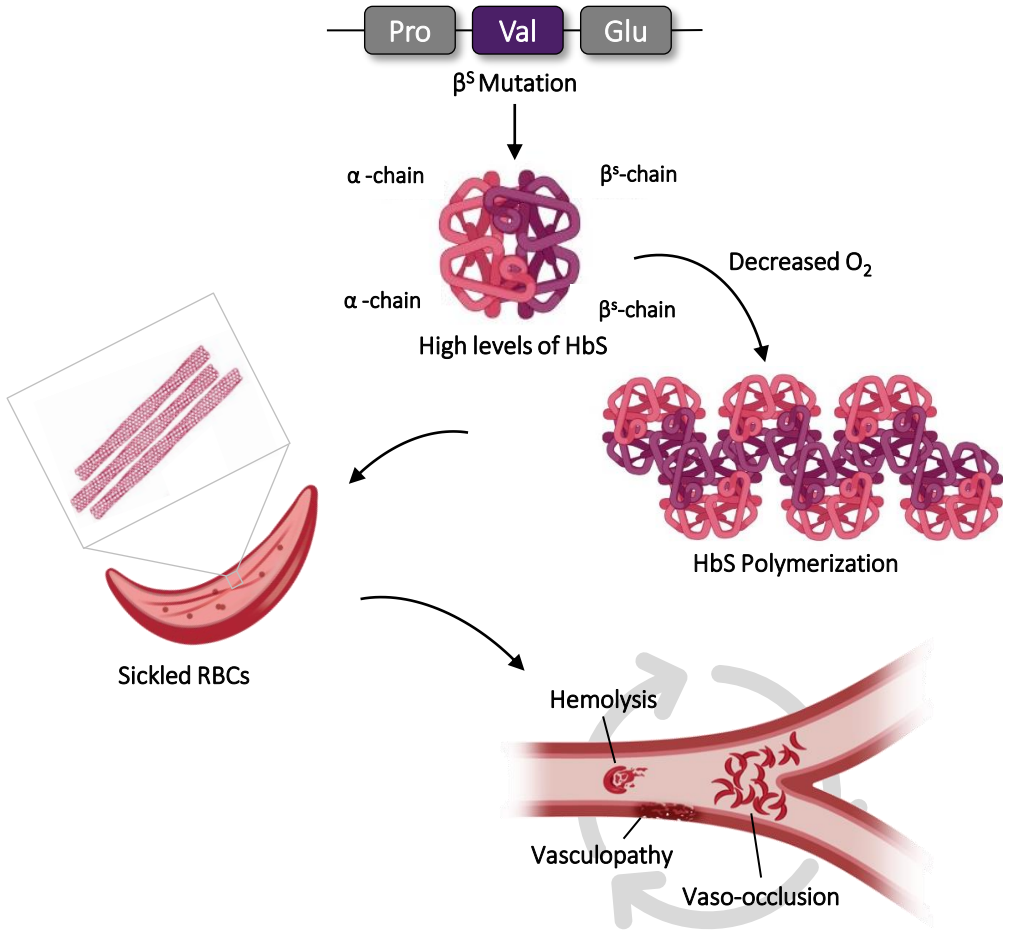
John F Tisdale

Nothing to Disclose

SCD is characterized by high morbidity and early mortality

For video

Pathophysiology of SCD[1]



Complications[2,3]

- Chronic daily pain
- Vaso-occlusive pain
- Anemia
- Cerebral vasculopathy/stroke
- Retinopathy
- Acute chest syndrome
- Pulmonary hypertension
- Hepato-splenic sequestration
- Cardiovascular complications
- Priapism
- Kidney disease
- Sudden death
- Leg ulcers
- Organ failure
- Osteonecrosis

> 50% of patients with SCD die before 45 years of age[2]

Glu, glutamic acid; Hbs, sickle hemoglobin; Pro, proline; RBC, red blood cell; SCD, sickle cell disease; Val, valine.

1. Kato GJ, et al. *Nat Rev Dis Primer*. 2018;4:18010; 2. Hassell K., *Am J Prev Med.* 2010; 3. Kanter, et al. *Blood Rev*. 2013;27(6):279- 287.

HGB-206: Study design

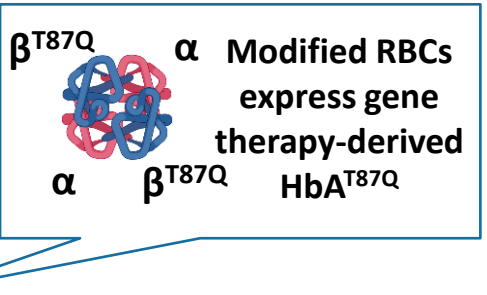
For video

HSC collection
Bone marrow harvest or mobilization with plerixafor & apheresis

Busulfan myeloablative conditioning

DP infusion

Transduced HSCs engraft and contribute to reconstitution of functional RBCs

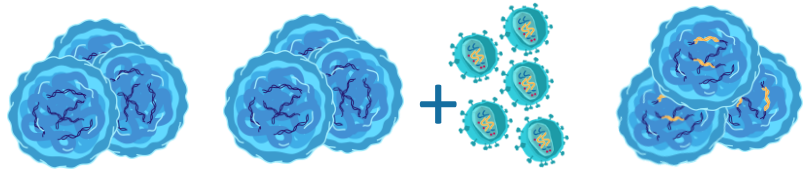


2-yr follow-up

Long-Term Follow-Up Study

	Group A	Group B	Group C
Pre-collection transfusion regimen	Optional	Required	Required
HSC source	Bone marrow	Bone marrow	Mobilized PB
Manufacturing process	Original	Orig → Refined	Refined

LentiGlobin DP centralized manufacturing



Select CD34+ cells

Transduce with BB305 lentiviral vector

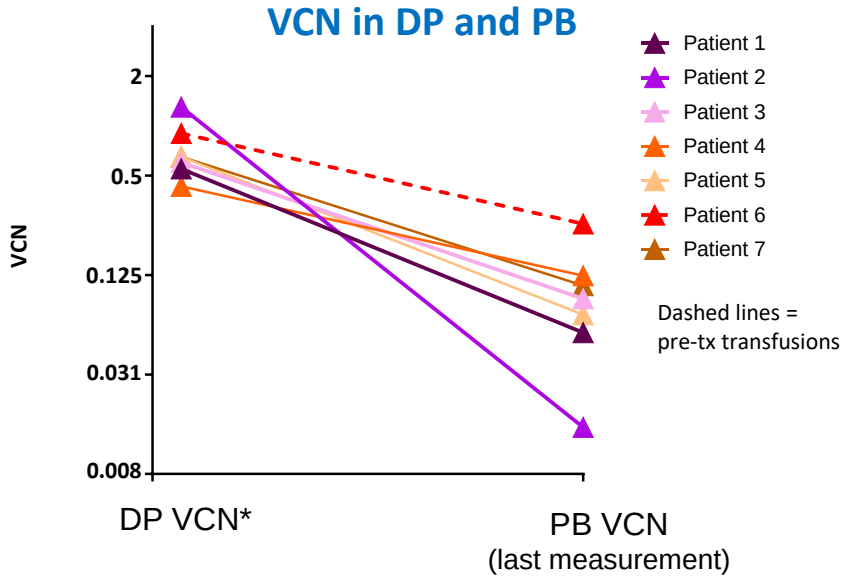
Cryopreserve, test, release DP

DP, drug product; HSCs, hematopoietic stem cells, PB, peripheral blood; RBCs, red blood cells.

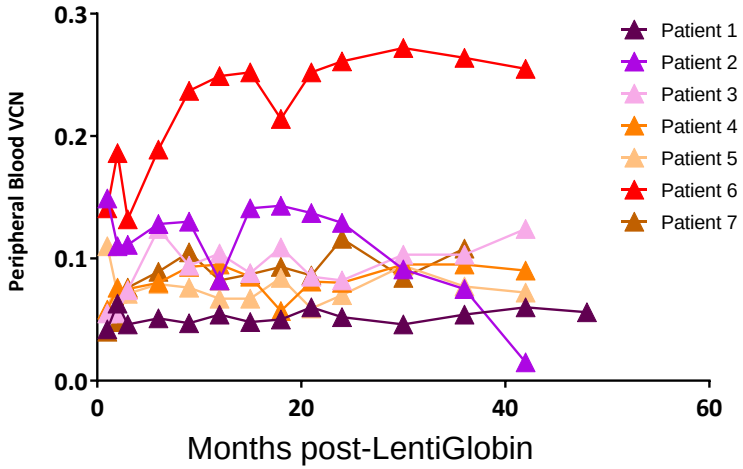
HGB-206 Group A: Decline in PB VCN after product infusion resulted in low HbA^{T87Q} production

For video

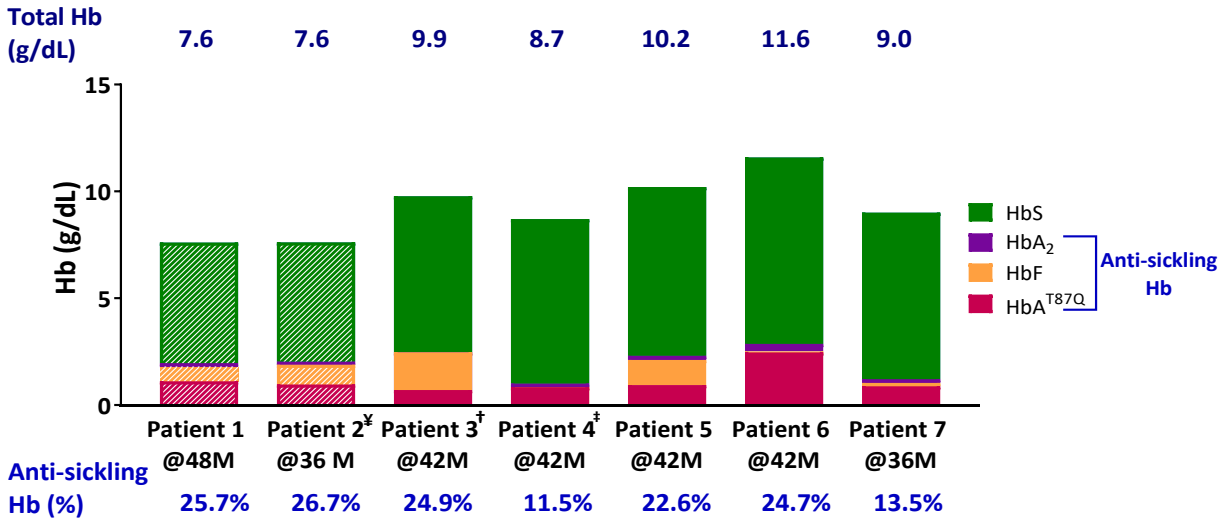
VCN in DP and PB



PB VCN over time



Hb fractions at last visit[1]



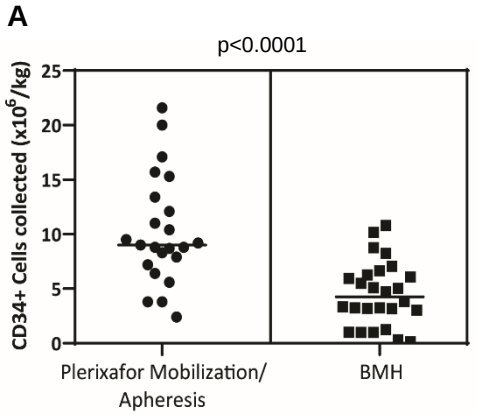
Hatched bars show patients on regular transfusions and HU post-engraftment

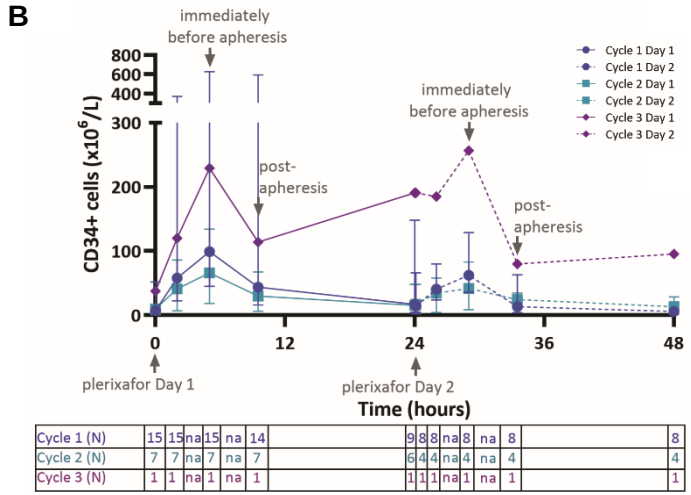
*Mean DP VCNs used for patients with >1 DP lot; ¥Given confounding effect of myelodysplastic syndrome and subsequent chemotherapy on the Hb fractions data, these laboratory values were censored from the start of chemotherapy administered after MDS diagnosis; †HbA_2 levels are pending; ‡Group A pt 4 had 2 transfusions post-engraftment. PB, peripheral blood; Hb, hemoglobin; MDS, myelodysplastic syndrome; pt, patient.

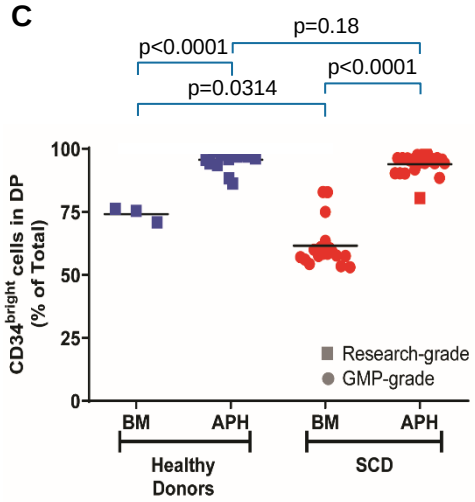
1. Walters M et al. Presented at ASH; December 7–10, 2019: Orlando, FL.

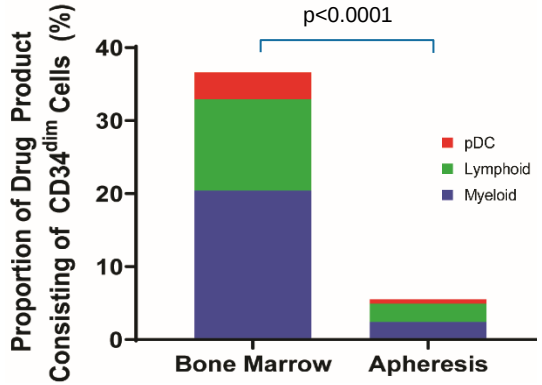
HGB-206: Plerixafor mobilization improves HSC collection compared with BMH

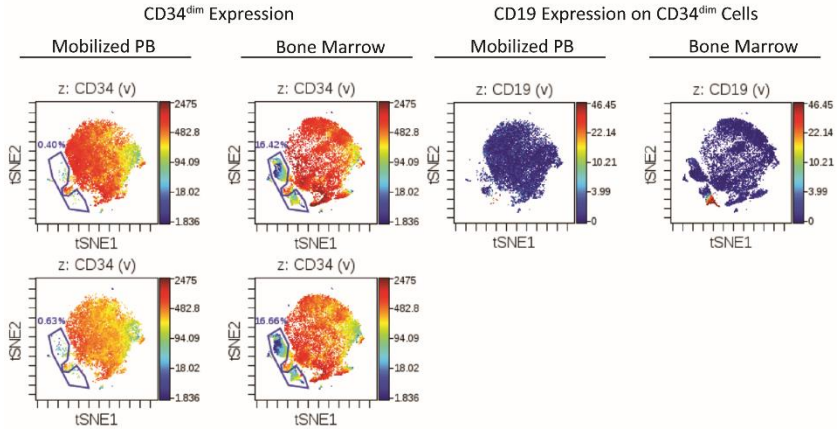
For video











HSC, hematopoietic stem cell; BMH, bone marrow harvest.

Tisdale, JF, et al. *Am J Hematol*, 2020; 95: 239-242.

Protocol evolution in the open-label, phase 1/2 HGB-206 study of LentiGlobin GT (bb1111) in severe SCD

For video

HSC Source Shift

	Group A[1-6]	Group B[1-6]	Group C[1-6]
Pre-collection transfusion regimen	Optional	Required	Required
HSC Source	Bone marrow	Bone marrow	Plerixafor-mobilization and apheresis
Conditioning AUC Target, µM*min per dose[a]	4,000 (Median AUC: 4747)	4,500 (median AUC: 5136)	5,000 (median AUC: 4843)
Manufacturing Process	Original	Original → Refined	Refined
Total Cell Dose, x10^6 cells/kg (Median total CD34+, CD34hi/+ HSCs)	Low (2.1, 1.6)	Medium (2.7, NA)	High[b] (6.9, 5.8)
Transduction Efficiency (Median DP VCN, Median % Transduced)	Low (0.6, 25.0%)	High (3.1, 78.4%)	High (3.8, 80.2%)

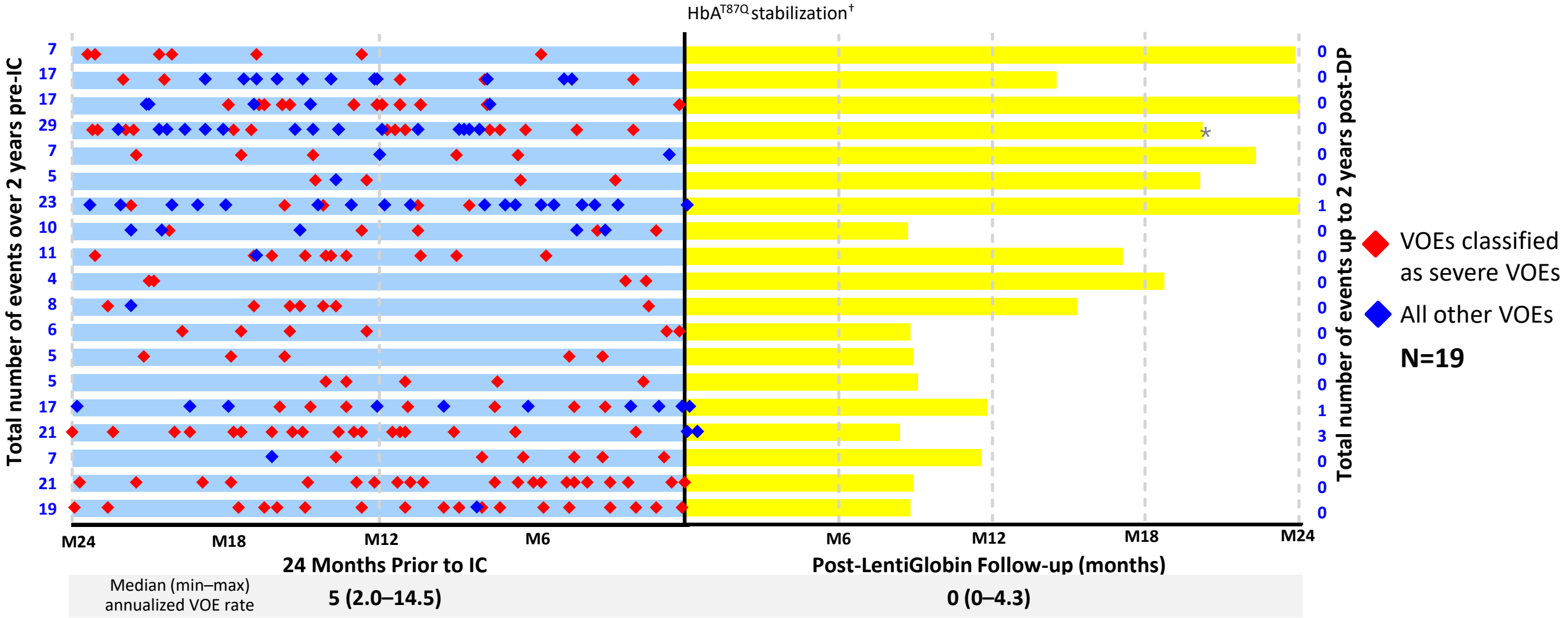
[a]For a once daily dosing regimen[5,6]

[b]Following transduction of isolated CD34+ HSCs with BB305 lentiviral vector, the bb1111 cell dose will be ≥3.0 × 10^6 CD34+ cells/kg for each subject[3]

AUC, area under the curve; DP, drug product; GT, gene therapy; HSC, hematopoietic stem cell; VCN, vector copy number. 1. bluebird bio data on file. 2. Kanter J, et al. *Blood*. 2017;130 (Supplement 1):527. 3. Kanter J, et al. Blood. 2018; 132 (Supplement 1): 1080. 4. Kanter J. EHA *Library*. 2020; Abstract #S282. 5. Thompson AA, et al. ASH 2020. Abstract #677. 6. Tisdale JF, et al. *Am J Hematol*. 2020;95(9):E239-E242.

HGB-206 Group C: Complete resolution of VOEs ≥ 6 months post-LentiGlobin treatment

For video



Protocol VOEs are shown; Patients with ≥ 4 sVOEs at baseline before IC and with ≥ 6 months of follow-up post-DP infusion are included. A VOE includes episodes of acute pain with no medically determined cause other than a vaso-occlusion, lasting more than 2 hours and severe enough to require care at a medical facility, a VOE includes acute episodes of pain, acute chest syndrome, acute hepatic sequestration, and acute splenic sequestration; †HbA^{T87Q} expression stabilizes within 6 months; *One death, unlikely related to LentiGlobin, > 18 months post treatment in a patient with significant baseline SCD-related cardiopulmonary disease.
Note: In the last datacut, one patient had a non-serious VOC at Day 107. The event is recorded as an investigator reported VOE but does not meet the definition of a protocol VOE.

DP, drug product; IC, informed consent; max, maximum; min, minimum; sVOEs, severe VOEs; VOC, vaso-occlusive crisis; VOE, vaso-occlusive event.

HGB-206 Group C: Safety profile

For video

Treatment-emergent ≥ Grade 3 AEs *Reported in ≥ 2 patients*[*]	N=32 n (%)
Stomatitis	21 (65.6)
Febrile neutropenia	14 (43.8)
Increased ALT	4 (12.5)
Increased AST	4 (12.5)
Increased GGT	4 (12.5)
Nausea	4 (12.5)
Increased blood bilirubin	2 (6.3)
Premature menopause	2 (6.3)
Upper abdominal pain	2 (6.3)
Serious treatment-emergent AEs *Reported in ≥ 2 patients*	
Abdominal pain	2 (6.3)
Nausea	2 (6.3)
Drug withdrawal syndrome	2 (6.3)
Vomiting	2 (6.3)

[*]Hematologic AEs commonly observed post-transplantation have been excluded.

- 1 patient with a nonserious Grade 2 DP-related neutropenic fever (resolved)
- No cases of veno-occlusive liver disease, graft failure
- No vector-mediated RCL and no insertional oncogenesis
- One death, attributed to cardiopulmonary disease and unlikely related to LentiGlobin, > 18 months post treatment in a patient with significant baseline SCD burden

AE, adverse event; ALT, alanine aminotransferase; AML, acute myeloid leukemia; AST, aspartate aminotransferase; DP, drug product; GGT, gamma-glutamyl transferase; MDS, myelodysplastic syndrome; RCL, replication competent lentivirus; SCD, sickle cell disease.

HGB-206 clinical trial hold following two suspected unexpected serious adverse reactions (SUSARs)

For video

- Beyond the 20 Aug 2020 data cut, two SUSARs were reported and as of February 2021, HGB-206 is on a clinical hold
  - **Group C: Initially reported MDS diagnosis revised to transfusion-dependent anemia**
    - A patient had persistent anemia 6 months after transplant and was found to have trisomy 8 in 6% of cells scored on a 6-month BM aspirate but no blasts or dysplastic cells
    - Investigator assessed as serious, Grade 3, ongoing, and possibly related to LentiGlobin for SCD
    - A follow-up BM aspirate revealed no genetic or chromosomal abnormalities and no evidence of myeloid neoplasm, and the diagnosis was changed to transfusion-dependent anemia with investigations ongoing
  - **Group A: Patient diagnosed with AML > 5 years post-LentiGlobin treatment.**
    - Investigator assessed as serious, Grade 4, ongoing, and possibly related to LentiGlobin for SCD

SUSAR, suspected unexpected serious adverse reactions; MDS, myelodysplastic syndrome; BM, bone marrow; AML, acute myeloid leukemia.

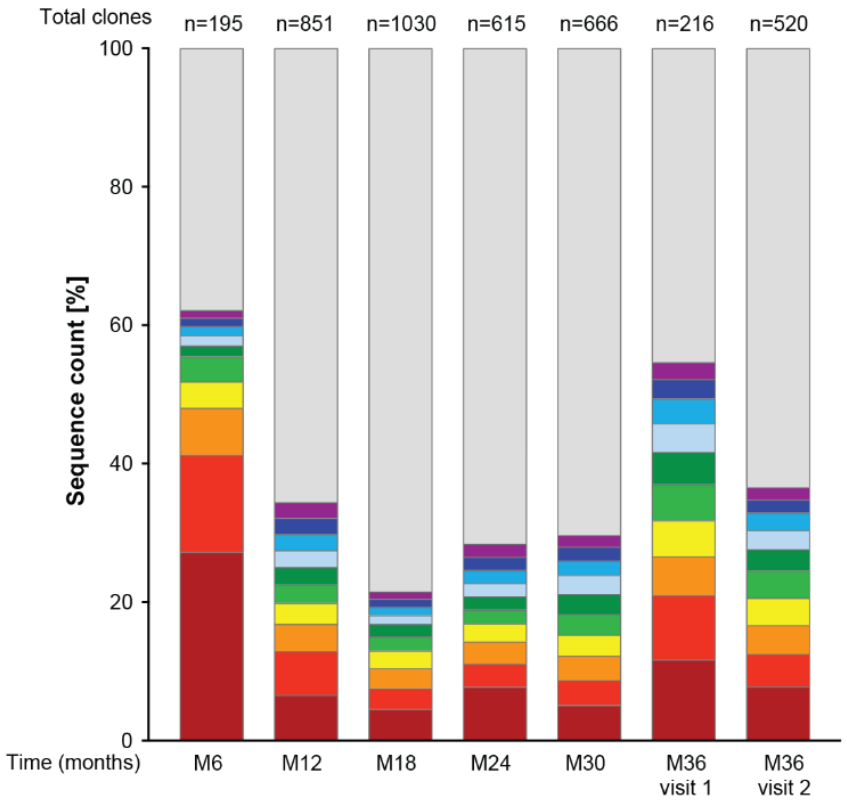
HGB-206 Group A: 2018 MDS/AML case unrelated to vector

For video

- At ~36 months post LentiGlobin GT, a patient was diagnosed with MDS in 2018
- BM biopsy showed 15% myeloblasts and dysplasia
- Cytogenetics showed monosomy 7 and abnormal chromosome 19p in 8 of 20 metaphases
- Blast cells (CD34+) had low VCN consistent with the absence of LVV integration
- The patient passed away due to relapsed AML in 2020

No evidence of clonal predominance by ISA

Frequencies of top 10 integration sites



BM, bone marrow; c/dg, vector copies per diploid genome; DP, drug product; GT, gene therapy; HSCT, hematopoietic stem cell transplant; IV, intravenous; LVV, lentiviral vector; MDS, myelodysplastic syndrome; N/A, not applicable; VCN, vector copy number.
1. Rege KP et al., BMT 1998; 2. Howe R et al., BMT 2003; 3. McNerney ME et al., *Nat Rev Cancer.* 2017; 4. Hsieh MM et al. *Blood Adv.* 2020; 4: 2058–2063.

HGB-206 Group A: Two cases of AML

For video

	2018 MDS/AML case[1]	2021 AML case
Mutations at baseline	No mutations or cytogenetic abnormalities (microarray, NGS)	No mutations or cytogenetic abnormalities (microarray, NGS)
Mutations post-treatment prior to AML diagnosis	NA*	No mutations M3, M6, M18, M24 (microarray, NGS)
Mutations at AML diagnosis	• Monosomy 7 • Abnormal 19p • *RUNX1* (NP_001745.2:p.Asp198Gly), • *PTPN11* (NP_002825.3:p.Phe71Leu), • *KRAS* NP_203524.1:p.Gly12Ala	• Monosomy 7 • Partial loss of 11p • *RUNX1* Exon 5 stop gained p.A149*fs • *PTPN11* Exon 3 missense: p.A72V
Vector in blasts?	No	Yes; insertion in 4th intron of *VAMP4*

*Only baseline mutations available. AML, acute myeloid leukemia; NGS, next generation sequencing; NA, not available.

1. Hsieh MM et al. *Blood Adv*. 2020; 4: 2058–2063

HGB-206 Group A 2021 AML case: Is the vector a “passenger” or “driver” of oncogenesis?

For video

Is vector present in tumor cells?

NO — **Not vector-mediated**

YES

Driver alterations existed and are present in samples pre-treatment

- ❑ Classical driver alterations consistent with AML phenotype
- ❑ ISA reveals oligoclonal pattern consistent with pre-existing tumor
- ❑ Driver alteration(s) identified (somatic or germline) prior to infusion

IS does not cause transcriptional or mechanistic changes that would likely contribute to oncogenesis

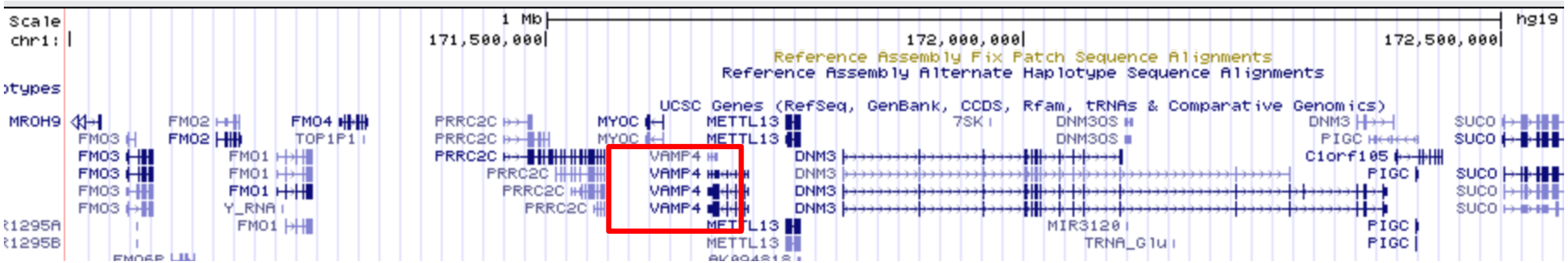
- ❑ Classical driver alterations consistent with AML
- ❑ No substantial change in gene expression around IS
- ❑ Vector is NOT transcriptionally active in tumor cells
- ❑ Transcriptional profile consistent with properties of known AML driver alterations
- ❑ IS(s) found in other patients without sequelae
- ❑ IS(s) unremarkable with respect to cancer-associated genes
- ❑ IS(s) does not disrupt genomic elements

All these profiles would identify the vector as a “passenger”

AML, acute myeloid leukemia; IS, insertion site.

HGB-206 Group A 2021 AML case: What role does *VAMP4* play?

For video



What role does *VAMP4* play?

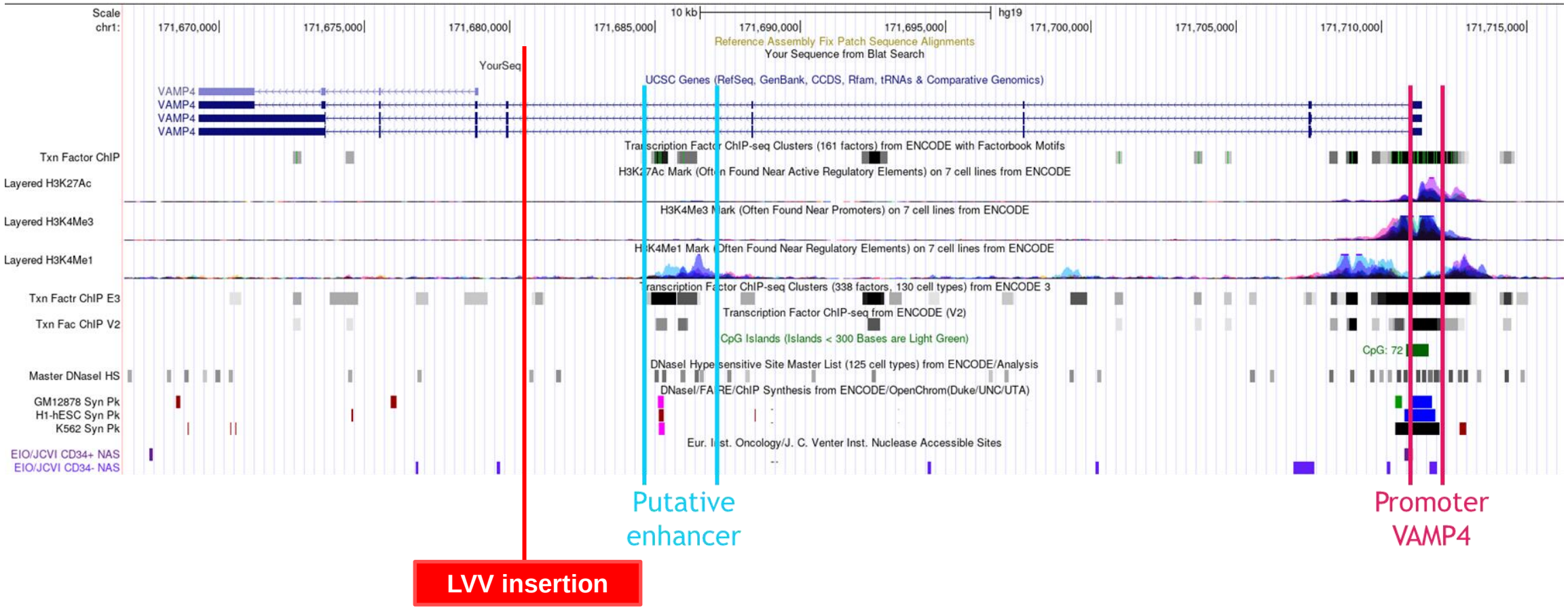
- Located on chromosome 1q
- Implicated in Golgi ribbon formation, transport from the Golgi to the membrane, and neurotransmission[1,2]
- No role in cellular proliferation or survival based on analysis of gene databases (COSMIC, cBioPortal, DepMap, and CCGD)

***VAMP4* has no reported role in cellular proliferation or oncogenesis**

AML, acute myeloid leukemia; IS, integration site; LVV, lentiviral vector; VAMP4, vesicle-associated membrane protein 4. 1. Shitara A, et al. *Mol Cell Biochem*. 2013;380:11-21; 2. Tran TH et al. 2007 *J Cell Sci*. 120:1028-41.

HGB-206 Group A 2021 AML case: Did the *VAMP4* IS disrupt any genomic features?

For video



IS mapped to the non-coding region of *VAMP4* in a location with no annotated genomic features*

*Evaluated genomic features included enhancer elements, promoters, boundary elements (eg, CTCF binding sites), areas of chromatin accessibility (incl. ATAC-seq), and regions of chromosomal looping (Hi-C contact matrix)

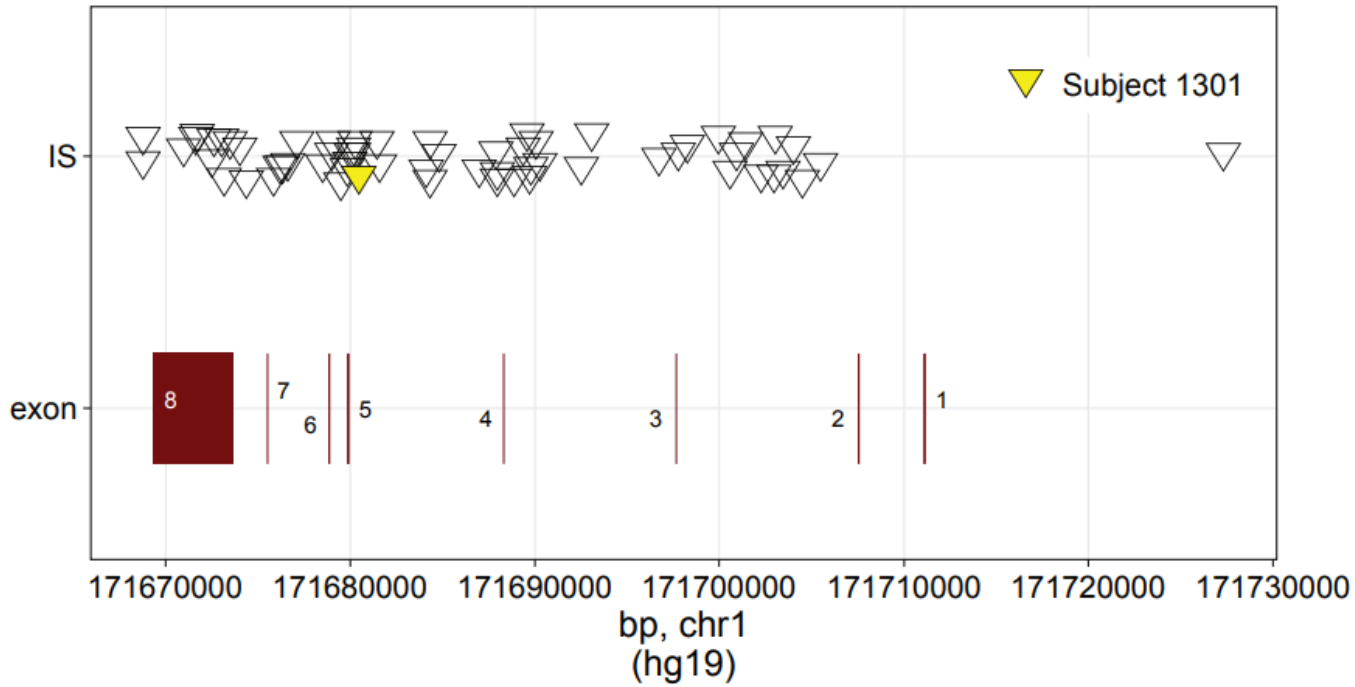
AML, acute myeloid leukemia; IS, integration site; LVV, lentiviral vector; VAMP4, vesicle-associated membrane protein 4.

HGB-206 Group A 2021 AML case: Is *VAMP4* IS associated with clonal predominance in other patients with SCD?

For video

Frequency and location of VAMP4 insertions

- Of 35 patients with SCD with ISA data, 25 had IS in *VAMP4*
- 60 unique IS were mapped
- 14 unique IS in intron between exons 4 and 5
- Only the patient diagnosed with AML had *VAMP4* IS >0.05% at any time point



***VAMP4* IS was present in the majority (71%) of patients in HGB-206 without sequelae in any other patient**

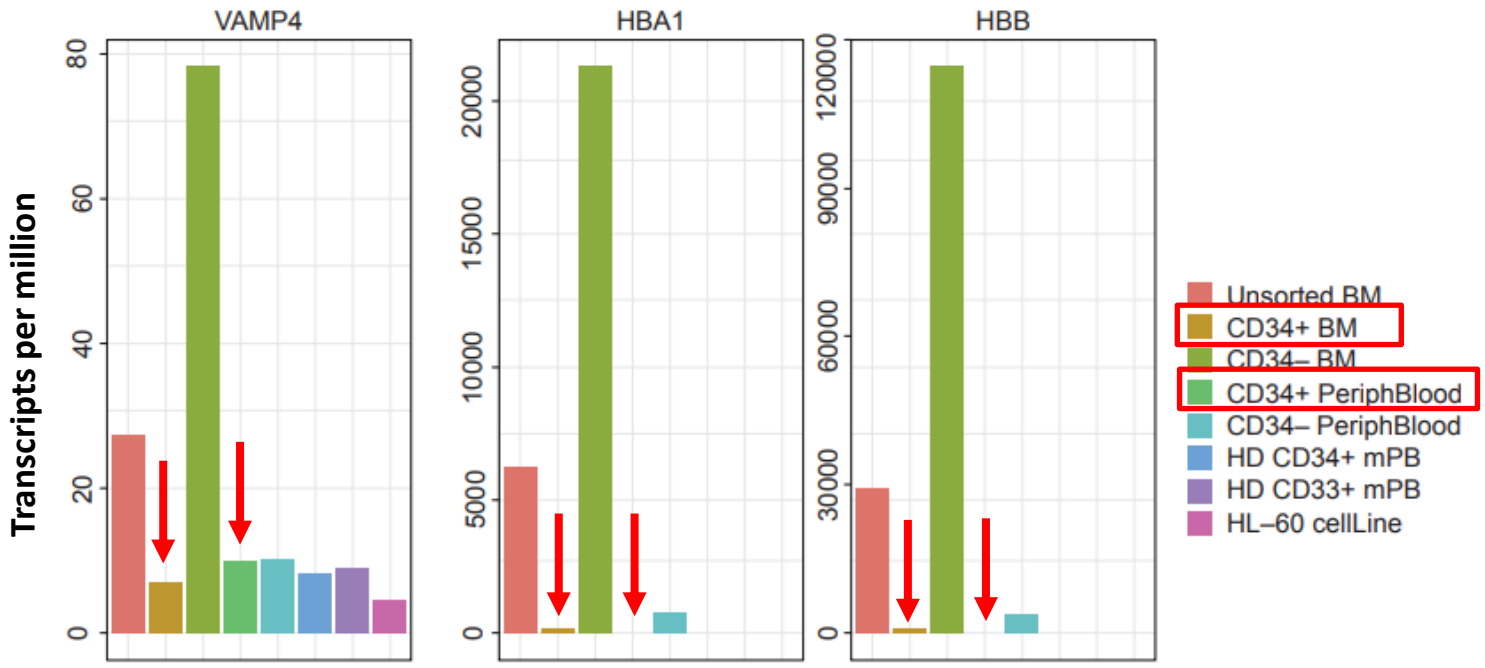
AML, acute myeloid leukemia; IS, integration site; ISA, integration site analysis; VAMP4, vesicle-associated membrane protein 4.

HGB-206 Group A 2021 AML case: Is the vector transcriptionally active in tumor cells?

For video

- Highest expression levels of *HBA1* and *HBB* were in unsorted and CD34- BM samples that include erythroid progenitors
- *HBB* expression in CD34+ BM is 0.6% of the CD34– BM expression
  - Of those, the majority (60%) were endogenous β^S transcripts and 40% were from the β^{T87Q} transgene

Gene expression analysis*



Vector is minimally transcriptionally active in tumor cells and does not significantly affect *VAMP4* expression

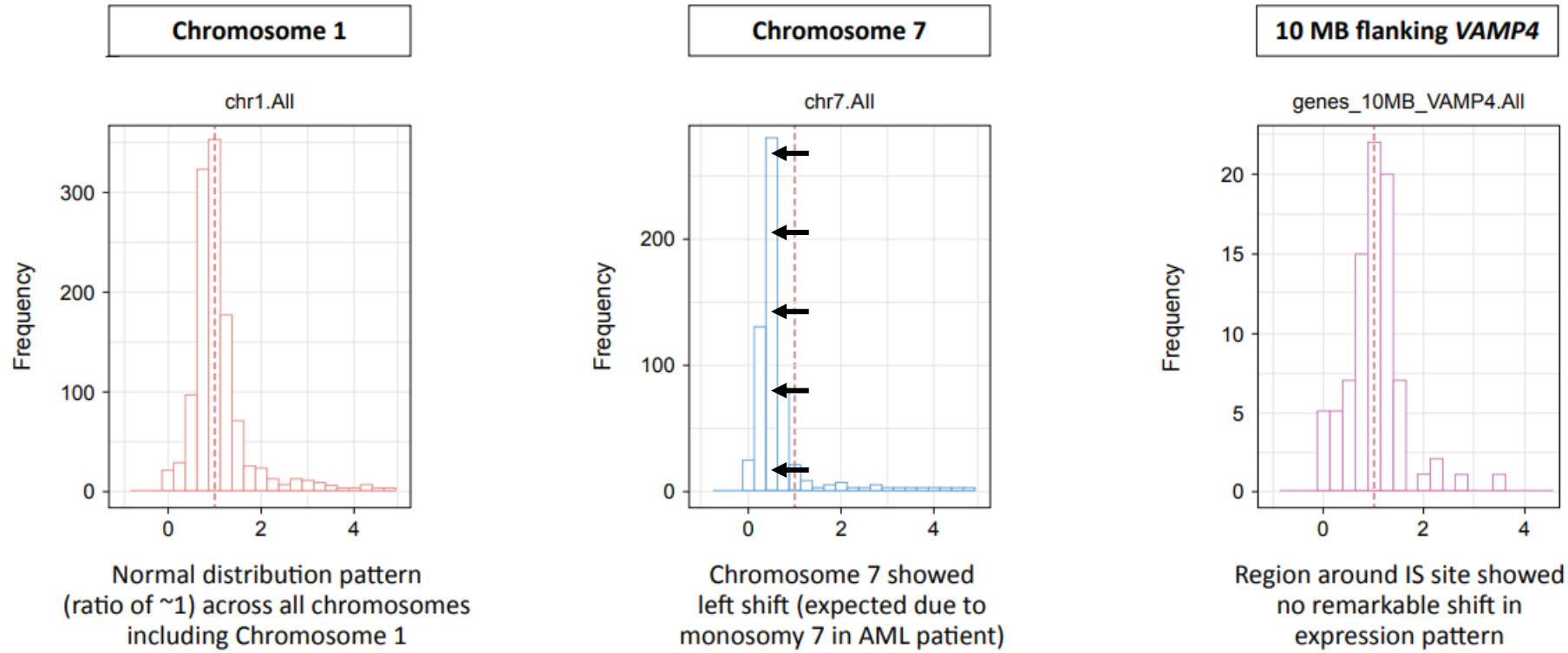
*Blast cells indicated by red arrows and boxes in figure and legend.

AML, acute myeloid leukemia; BM, bone marrow; IS, integration site; VAMP4, vesicle-associated membrane protein 4. Y-axes differ to accommodate large expression differences.

HGB-206 Group A 2021 AML case: Did *VAMP4* IS impact expression of nearby genes or chromosome-wide expression?

For video

Gene expression ratios in AML vs healthy donor CD34+ BM cells



Normal distribution pattern (ratio of ~1) across all chromosomes including Chromosome 1

Chromosome 7 showed left shift (expected due to monosomy 7 in AML patient)

Region around IS site showed no remarkable shift in expression pattern

***VAMP4* IS did not impact gene expression proximal to the vector insertion**
Normal pattern of distribution (ratio of ~1) was detected across all chromosomes

AML, acute myeloid leukemia; BM, bone marrow; IS, integration site; VAMP4, vesicle-associated membrane protein 4.

HGB-206 Group A 2021 AML case: Unlikely related to vector-mediated insertional oncogenesis

For video

Criteria	Findings
1. Classical driver alterations consistent with AML	✓ Monosomy 7, partial loss of 11p, *RUNX1*, *PTPN11*
2. No substantial change in gene expression around IS	✓ No remarkable expression changes in 10 MB region around *VAMP4* IS
3. Vector is NOT transcriptionally active in tumor cells	✓ Very low level *HBB* detected in CD34+ cells
4. Transcriptional profile consistent with properties of known AML driver alterations	✓ RNAseq data consistent with monosomy 7 and contains *PTPN11* and *RUNX1* mutations
5. IS(s) found in other patients without sequelae	✓ *VAMP4* IS common and this patient is the only one with *VAMP4* IS >0.05% at any point
6. IS(s) unremarkable with respect to cancer-associated genes	✓ *VAMP4* has no known association with cellular proliferation or oncogenesis
7. IS(s) does not disrupt genomic elements	✓ *VAMP4* IS does not disrupt mapped genomic features

AML, acute myeloid leukemia; IS, integration site; VAMP4, vesicle-associated membrane protein 4.

HGB-206 Group A 2021 AML case: What may have contributed to the development of AML?

For video

- **Patients with SCD have a higher rate of hematological malignancies compared with the general population**[1,2] and age, gender, race, and ethnicity matched non-SCD patients[3]
- There may be **higher risk for the development of malignancy following graft rejection/failure**[4-7]
- In SCD patients with unsuccessful or partly successful transplantation, hematopoietic stress from continued disease and proliferative stress from initial reconstitution of the BM by engrafting HSPCs may also contribute to risk of hematologic malignancies[4,5,7]
- The **underlying increased risk of hematologic malignancies in SCD**, **combined with the transplant procedure and associated proliferative stress, as well as continued hematopoietic stress due to minimal clinical benefit** in these two Group A patients (DP manufactured using stem cells collected via BMH and using a previous manufacturing process which has since been discontinued) may have contributed to the development of AML

Elevated risk of hematological malignancies in patients with SCD and following transplant, particularly suboptimal transplant

AML, acute myeloid leukaemia; MDS, myelodysplastic syndrome SCD, sickle cell disease; BM, bone marrow; HSPC, hematopoietic stem and progenitor cells; DP, drug product; BMH, bone marrow harvest.

1. Brunson A, et al, *Blood*. 2017;130:1597-9; 2. Seminog OO et al, *J R Soc Med*. 2016;109:303-9; 3. Jain S et al, *Blood*. 132; 1087; 4. Eapen M et al. *Lancet Hematol*. 2019: 6:e585-e596; 5, Ghannam JY et al. *Blood*. 2020:135:1185-1188; 6. Janakiram M et al. *Leuk Lymphoma*. 2018;59:241-244; 7. Fitzhugh et al. *Blood Adv*. 2017;1:652–661.

MDS/AML documented in patients following transplantation with different conditioning regimens and with different donor sources in SCD

For video

	Matched 0170 TBI/Campath	Matched 0077 TBI/Campath Pentostatin/Cy	Matched (Chicago,Riyadh) TBI/Campath	Haplo 0225 TBI/Campath -/+ Cy	Haplo 0069 TBI/Campath/Cy Pentostatin/Cy	Eapen CIBMTR Many types	Gene Therapy with BB305 LVV Busulfan	
							LentiGlobin for SCD‡	Beti-cel§ for TDT
N in study	58	26	64	21	19	910	**49** Median follow up (min –max) months **23.1 (0.8–74.9)**	**63** Median follow up (min –max) months **29.54 (0.9–76.4)**
MDS, AML	2 (3.5%)	1 (3.8%)	1 (1.6%)	3 (14%)*	0	6† (1.0%)	2 (4.3%)	0
	1 graft failure (MDS)	1 low engraft (AML)	1 graft failure (MDS)	2 graft failure MDS, AML		Details not available	2 in Group A[1,2]	

*Two patients with preexisting TP53 mutations; †overlap with NIH reporting in n=2; ‡as of 17 Feb 2021; §as of 30 Nov 2020.

- MDS/AML more common in patients with graft failure
- Improved engraftment in NIH haplo trial appears to have improved risk of MDS/AML
- Median follow up not sufficient to be conclusive

AML, acute myeloid leukaemia; MDS, myelodysplastic syndrome; SCD, sickle cell disease; TDT, transfusion-dependent β-thalassemia; TBI, total body irradiation; CY, cyclophosphamide.

Data from NIH Intramural Program; 1. Hsieh MM et al. *Blood Adv*. 2020; 4: 2058–2063; 2. Kwiatkowski J et al. Presented at ASPHO. April 20–23, 2021. Virtual. 3. Eapen M et al. *Lancet Hematol*. 2019: 6:e585-e596; 4, Ghannam JY et al *Blood*. 2020:135:1185-1188

Summary

For video

- The ongoing Phase 1/2 HGB-206 study is the largest clinical trial of GT in SCD to date with longest follow up > 5 years
- Data in the initial 7 patients (Group A) showed low PB VCN and HbA^{T87Q} expression, prompting protocol improvements (eg, HSC mobilization with plerixafor and improved transduction efficiency) for Group C patients who demonstrated positive outcomes:
  - Complete resolution of severe VOEs, median total Hb was consistently ≥ 11 g/dL, median anti-sickling HbA^{T87Q} ≥ 40%
- Investigation into 2 AML cases in Group A indicates they were unlikely related to vector-mediated insertional oncogenesis
  - We demonstrated the absence of vector integration in the blast cells of an MDS/AML case in 2018[1]
  - In the 2021 case, we report the IS was in *VAMP4*, a gene with no reported role in oncogenesis, and had no significant effects on regional or global gene expression, indicating that vector insertion was a passenger to AML development and not a driver of oncogenesis
- The underlying increased risk of hematologic malignancies in SCD, combined with the transplant procedure and associated proliferative stress, as well as continued hematopoietic stress due to minimal clinical benefit in these two Group A patients may have contributed to the development of AML
- The benefit-risk balance of LentiGlobin for SCD remains positive, and the safety profile post-LentiGlobin for SCD remains generally consistent with the risks of autologous stem cell transplant, myeloablative single-agent busulfan conditioning, and underlying SCD

AML, acute myeloid leukemia; GT, gene therapy; Hb, hemoglobin; HSC, hematopoietic stem cell; IS, insertion site; MDS, myelodysplastic syndrome; PB, peripheral blood; SCD, sickle cell disease; VCN, vector copy number; VOE, vaso-occlusive event.

1. Hsieh MM et al. *Blood Adv*. 2020; 4: 2058–2063; 2. Eapen M et al *Lancet Hematol*. 2019: 6:e585-e596; 3. Ghannam JY et al *Blood*. 2020:135:1185-1188; 4. Kahn JM et al. *Blood Adv*. 2020:4:2084-94.

Thank you to all for their important contributions to the AML case investigation

For video

- **Staff at HGB-206 clinical site for their support in the care of the patient described in this presentation**
- **Members of the bluebird bio team for their contributions:**
  - Alberto de Iaco
  - Brandon Gentile
  - Brandon Nguyen
  - Chetanya Pandya
  - Chris Mucci
  - Cynthia Rogers
  - James Rottman
  - Katie Groglio
  - Lauryn Christiansen
  - Madhu Mahajan
  - Maggie Chen
  - Mauris Nnamani
  - Megan Hoban
  - Nick Fenger
  - Nick Rouillard
  - Noel Namai
  - Omar Elbetanony
  - Russell Goetze
  - Ryan Leenay
  - Shelby LaBarre
  - Steph Hintzen
  - Swathi Krishnan
  - Tiffany Hu
  - Geoff Parsons
  - Sarah Voytek
  - Marianna Foos
  - Ashish Yeri
  - Maple Gioia
  - Marisa Gayron
  - Suus Jonkheer
  - Briana Deary
  - Natalia Mackowiak
  - Leanne Ianiello
  - Heidi Elliot
  - Laura Demopoulos
  - Jessie Lynch
  - Fran Smith
  - Joyce O'Connell
  - Perrine Geoffroy
  - Kate Lewis

Thank you to the study site members as well as the study participants and their families

For video

Ann and Robert H. Lurie Children's Hospital of Chicago, Northwestern University
- Alexis Thompson
- Peter Chase

Medical University of South Carolina
- Jennifer Jaroscak
- Brandi Day

Children's Hospital of Philadelphia
- Janet Kwiatkowski
- Pranaya Venkatapuram

UCSF Benioff Children's Hospital
- Mark Walters
- Cyrus Bascon
- Marci Moriarty
- Frans Kuypers

Emory University
- Lakshmanan Krishnamurti
- Megan Hanby

Hackensack University Medical Center
- Stacey Rifkin-Zenenberg
- Elana Smilow

Cohen Children's Medical Center
- Banu Aygun
- Alicha Paul
- Judene Mavrikis

National Institutes of Health, Molecular and Clinical Hematology Branch
- John Tisdale
- Matt Hsieh
- Naoya Uchida
- Stephanie Helwing
- Rick Gustafson
- Wynona Coles

Columbia University Medical Center
- Markus Mapara
- Monica Bhatia
- Beatriz Raposo Corradini
- Matt Chiaramonte

University of North Carolina
- Kimberly Kasow
- Catherine Cheng

University of Alabama
- Julie Kanter
- Michele Blue

GeneWerk GmbH
- Manfred Schmidt

bluebird bio, Inc.
- Suus Jonkheer
- Brandi Blount
- Jessie Lynch
- Xinyan Zhang
- Jean-Antoine Ribeil
- McKinley Nickerson
- Manisha Pradhananga
- Marisa Gayron
- Ketaki Kadam
- Pamela Chan