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Recoding in Action

Q3 2020

LET'S RECODE THE STORY

NASDAQ: BLUE

forward-looking statements

These slides and the accompanying oral presentation contain forward-looking statements and information. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward-looking statements. For example, all statements we make regarding the initiation, timing, progress and results of our preclinical and clinical studies and our research and development programs, our ability to advance product candidates into, and successfully complete, clinical studies, the timing or likelihood of regulatory filings and approvals, and the timing and likelihood of entering into contracts with payors for value-based payments over time or reimbursement approvals, and our commercialization plans for approved products are forward looking. All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our most recent quarterly report on Form 10-Q, as well as our subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Must Beat the Odds.

Period.

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Tremendous Progress in Challenging Times

Programs and Pipeline

- ide-cel updated data at ASCO; BLA resubmitted
- Clarity on accelerated US regulatory path for SCD (Updated data at EHA)
- Key 2021 milestones tracking: EU TDT ramp, ide-cel launch, US TDT, ALD & SCD filings & pipeline emergence

Operation Plan

- Optimized BMS collaboration & $200M rights monetization
- Revised operating plan by over $500M through mid-2022
- Extended cash runway into 2023

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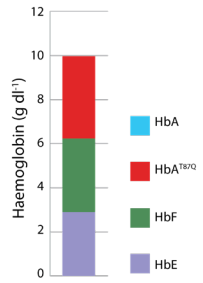
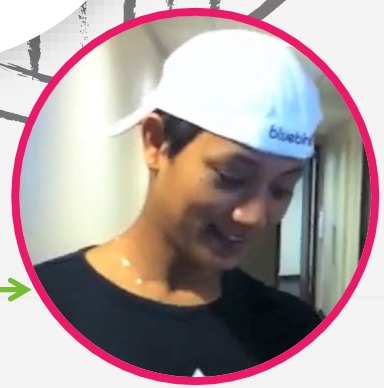
Transfusion-Dependent ß-Thalassemia - reimagined future

2010

nature





RECODE

Vector Potency &

Manufacturing

Enhancement

EHA 2020

- Northstar-2 (HGB-207): All patients treated, 89% TI
- Northstar-3 (HGB-212): 85% of patients have been off transfusions for > 6 months

zynteglo®

(autologous CD34⁺ cells encoding $\beta^{A\text{-}T87Q}$-globin gene)

✓ **EU Approved 2019**

✓ **US rolling BLA initiated 2019**

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Nature 2010

Transfusion-dependent β-thalassemia (TDT): patients achieving transfusion independence across genotypes and ages

ASH 2019

Northstar-2 (HGB-207):

- **Non-β^0/β^0: 90% of patients achieving TI**

Northstar-3 (HGB-212):

- **β^0/β^0 and IVS-I-110: 2 patients evaluable for TI, achieve TI**

EHA 2020

Achieving and maintaining transfusion independence (TI) across ages and genotypes

Northstar-2 (HGB-207):

- **Non-β^0/β^0:** All patients treated
- 89% successfully achieved TI

Northstar-3 (HGB-212):

- **β^0/β^0 and IVS-I-110:** 85% of patients have been off transfusions for > 6 months

Compelling data supports commercial path

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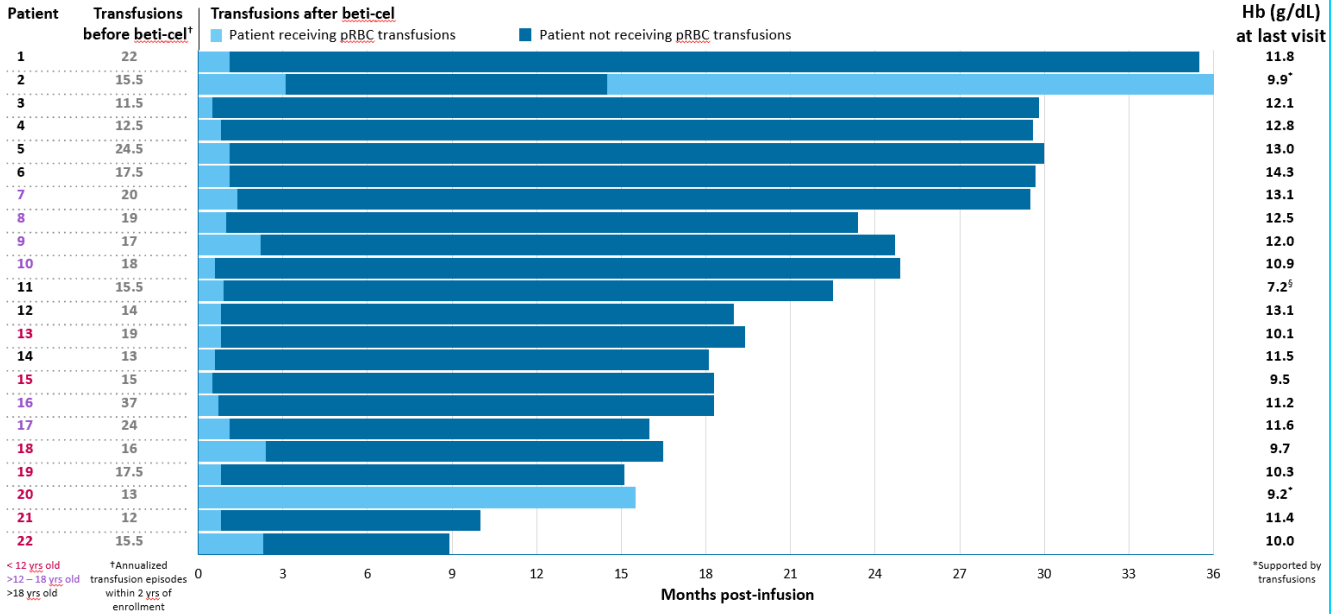
Northstar-2: Non-β^0/β^0 patients achieving & maintaining transfusion independence

91% (20/22) of patients with >3 months of follow-up have stopped pRBC transfusions

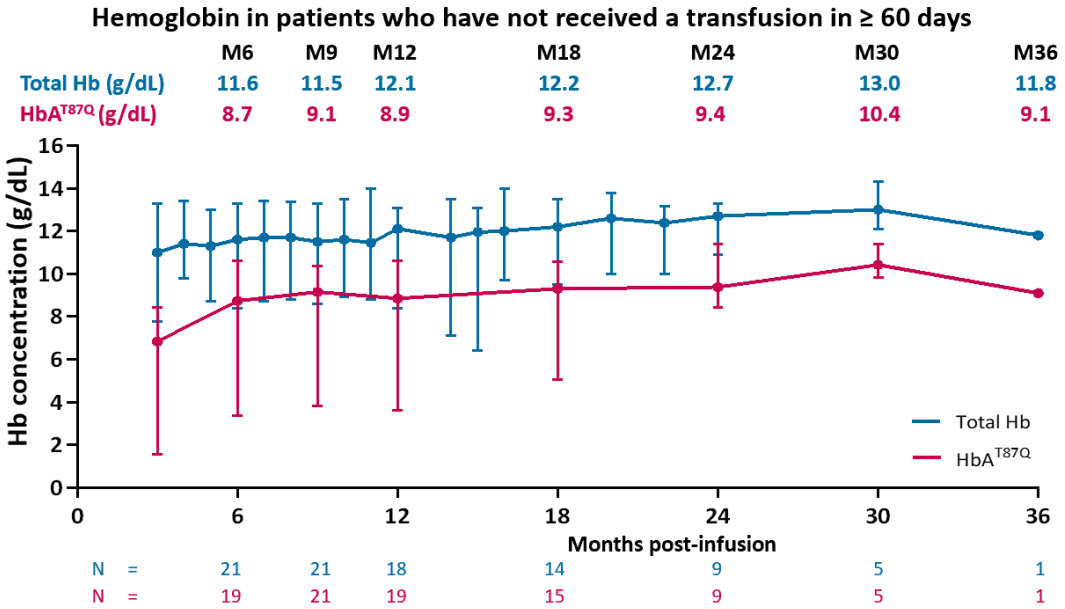


- 89% (17/19) of evaluable patients achieved primary endpoint: transfusion independence
- Patient 2 and Patient 20 had 46% and 16% reduction in pRBC transfusion volume, respectively, from 6 months to last follow-up

§Patient's total Hb level at Month 22 was 13.4 g/dL. Following a planned orthopedic surgery, the patient had blood loss, which required 1 pRBC transfusion; pRBC, packed red blood cell.

Data as of 7 April 2020

Median unsupported total Hb is ≥ 11.5 g/dL



Median, min, max depicted

Data as of 3 March 2020

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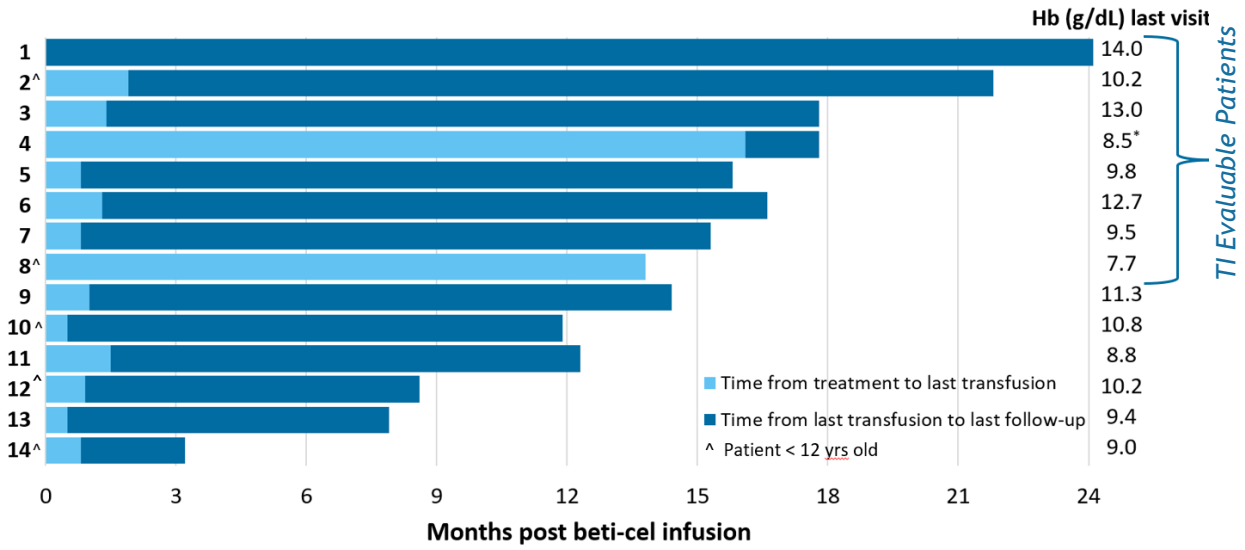
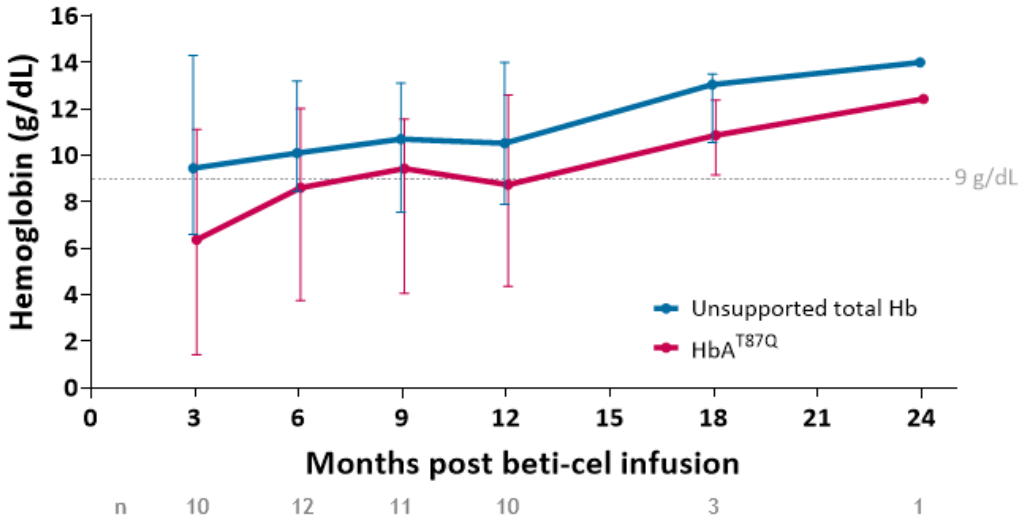
Northstar-3: β^0/β^0 patients continue to show compelling results

Transfusion status in patients with ≥ 3 months follow-up



- 85% (11/13) of patients have been off transfusions for > 6 months; prior to beti-cel infusion, these patients required 11 – 39.5 transfusions/year
- Patient 4 and Patient 8 continue to receive pRBC transfusions and had an 80% and 31% reduction in number of transfusions, respectively

Total Hb and HbA^{T87Q} over time in patients who have not received a transfusion in > 60 days



- As transduced HSCs engraft and produce mature RBCs, HbA^{T87Q} levels increase and stabilize approximately 6 – 9 months after beti-cel infusion

^Patient < 12 years old at consent; *Indicates pRBC transfusion in prior 60 days. Data as of 7 April 2020

Median, min, max depicted; Unsupported total Hb level is defined as Hb without any red blood cell transfusions within 60 days. Hb, hemoglobin. Data as of 3 March 2020

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Robust data supports commercial path forward

zynteglo®
(autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene)

EU: Ready to Go

Ready to treat patients in Germany pending COVID-19 environment

Ongoing engagement with payers in additional EU markets supports access and reimbursement by end of 2020

Plan to pursue expanded label to include patients with β^0/β^0 genotypes and pediatrics

US: Clear Path

Updated data reinforce confidence in pursuing initial approval for patients with TDT and all genotypes

Learnings from FDA engagement leveraged across programs

US BLA Submission Planned for mid-2021 (Q2/Q3)

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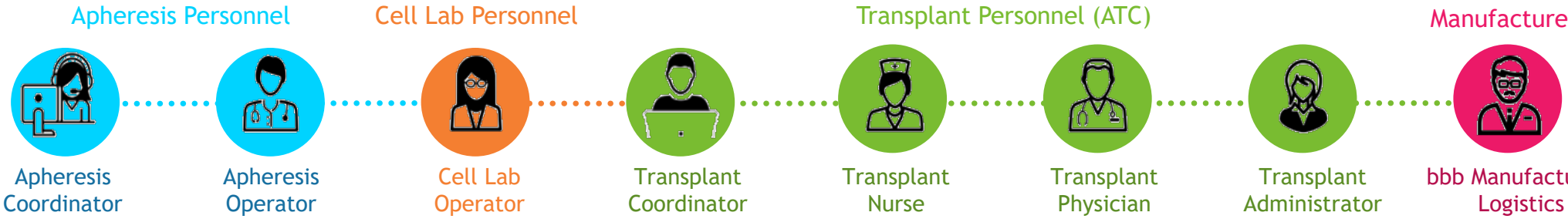
preparing to serve patients in Europe



launch expectations

1. Optimal patient experience through a seamless delivery network
2. Steady country by country launch with progressive build
3. Get the model right for long term success
4. Advance value-based payment over time reimbursement

1 drug product manufacturing

Munich, Germany

initial launch focus

- Germany
- Italy
- UK
- France

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Establishing Promising Access & Value Foundation

EU Launch Readiness

- ✓ First ever at-risk value-based agreement signed with multiple Sick Funds in Germany (~50-70% of patients in Germany covered)
- ✓ Team in place in Zug, UK, France, Italy, Germany, and Nordic Markets
- ✓ Qualified Treatment Centers and manufacturing ready in Germany

U.S. Launch Readiness

- ✓ Team in place for U.S. commercialization
- ○ Payers (Commercial) – Actively engaging to enable access & value-based payment over time at launch
- ○ Policy (State & Federal) - Focused on enabling value-based payment over time in commercial and for Medicaid markets to drive access
- ○ Distribution – Establishing customized distribution model to serve QTC & payer needs

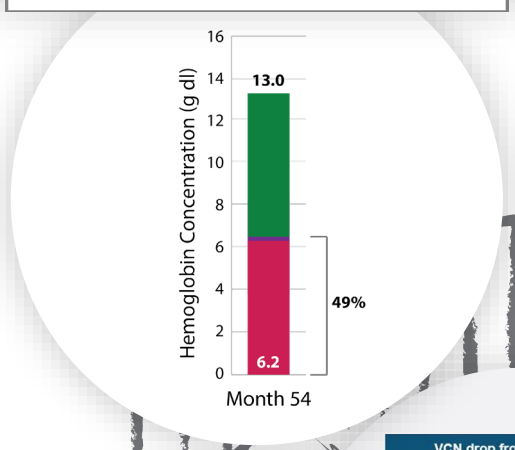
Market and Patient Engagement

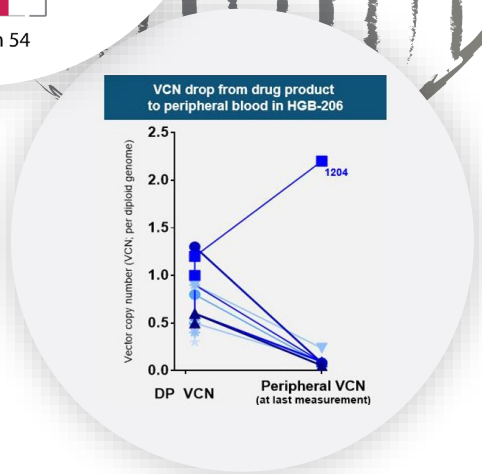
- ✓ Disease Education and outreach in place
- ✓ Patient Advocacy education and initiative support

Sickle Cell Disease – Daring to Dream

2017

The NEW ENGLAND JOURNAL of MEDICINE





RECODE

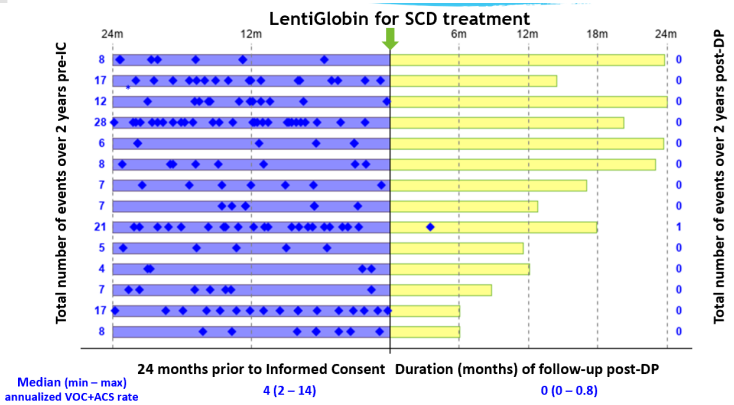
Pre-Tx Transfusions

More Thorough Conditioning

Higher Cell Dose

Higher VCN

EHA 2020



99.5% reduction in annualized rate of VOC + ACS*

- Accelerated development underway

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New England Journal of Medicine 2017

*HGB-206 Group C patients with history of VOCs and ACS who had ≥ 6 months of follow-up; data as of March 3, 2020

Sickle Cell Disease:
Totality of the clinical data validates transformative clinical results

ASH 2019

Early clinical benefit:

- 99% mean reduction in VOC and ACS

Group C patients:

- 17 patients; 9 patients with ≥6 months follow up and ≥4 VOC/ACS at baseline

Improvement in key markers of hemolysis

EHA 2020

Magnitude of clinical benefit:

- 99.5% mean reduction in VOC and ACS

More patients; more follow-up:

- 25 patients; 14 patients with ≥6 months follow up and ≥4 VOC/ACS at baseline

Consistent results across multiple markers:

- Continued improvements in hemolysis markers, HbA^{T87Q} levels and pancellular expression

Clarity on U.S. regulatory path:

- Based on HGB-206 Group C

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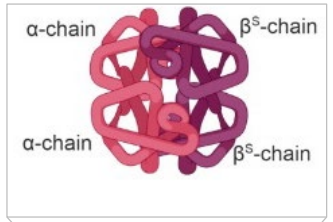
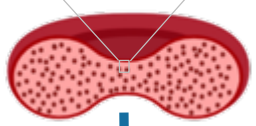
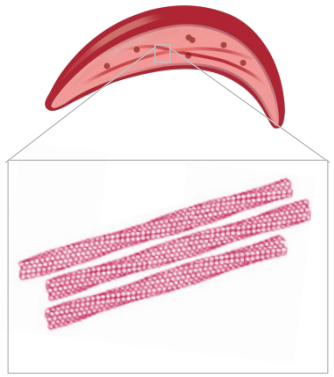
Sickle cell disease is characterized by high morbidity and early mortality

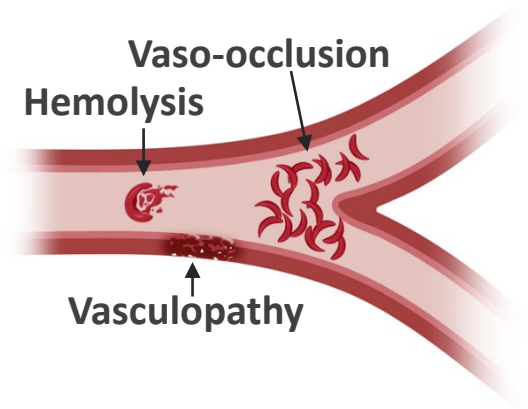


High levels of HbS in RBCs



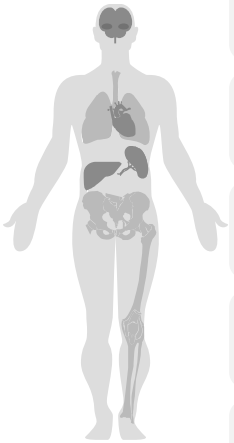
↓O_2 HbS polymerization & sickling





Complications

- 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[1]

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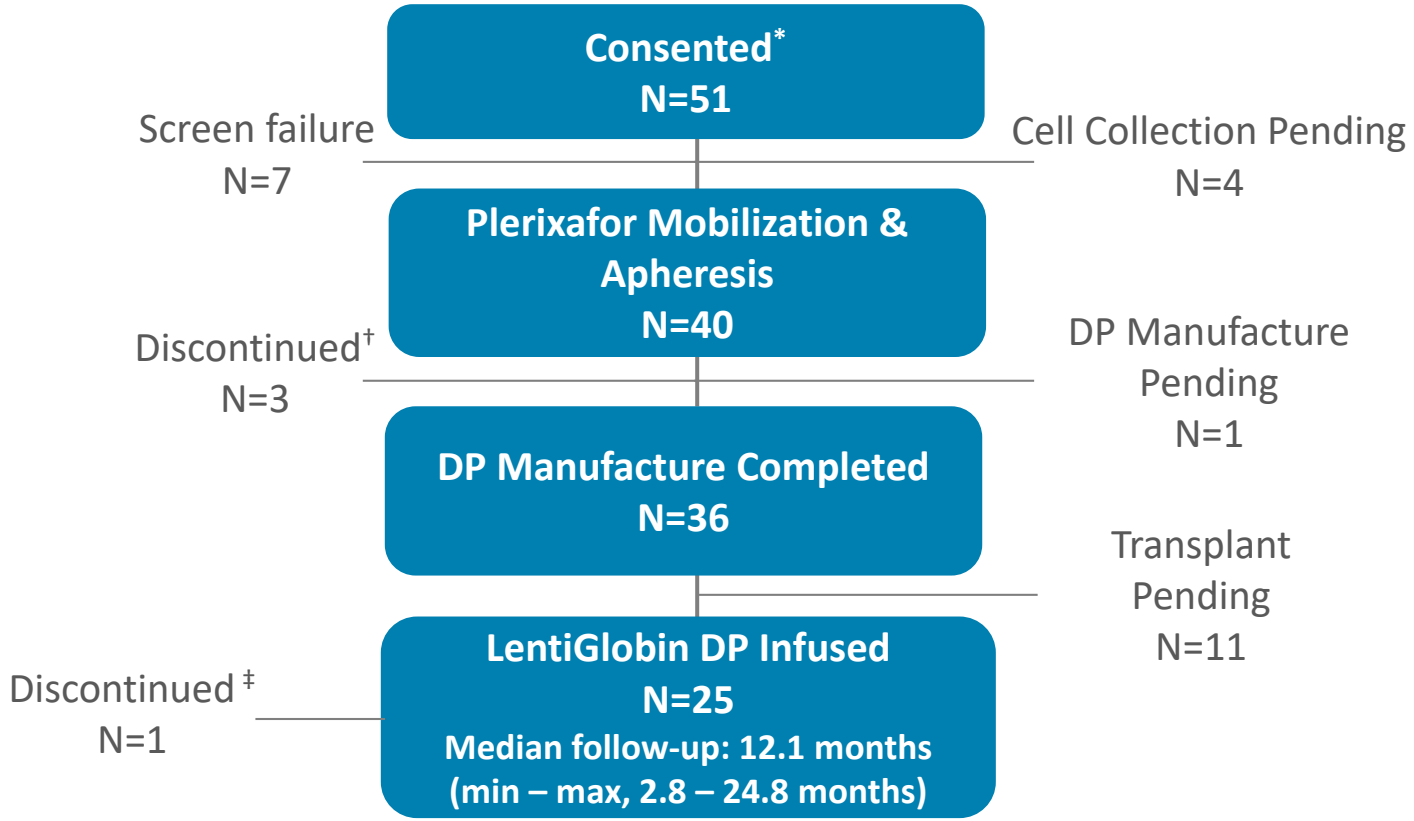
1. Hassell K., Am J Prev Med 2010; CNS, central nervous system; Hb, hemoglobin; RBC, red blood cell

HGB-206 Group C: Patients infused to support BLA submission

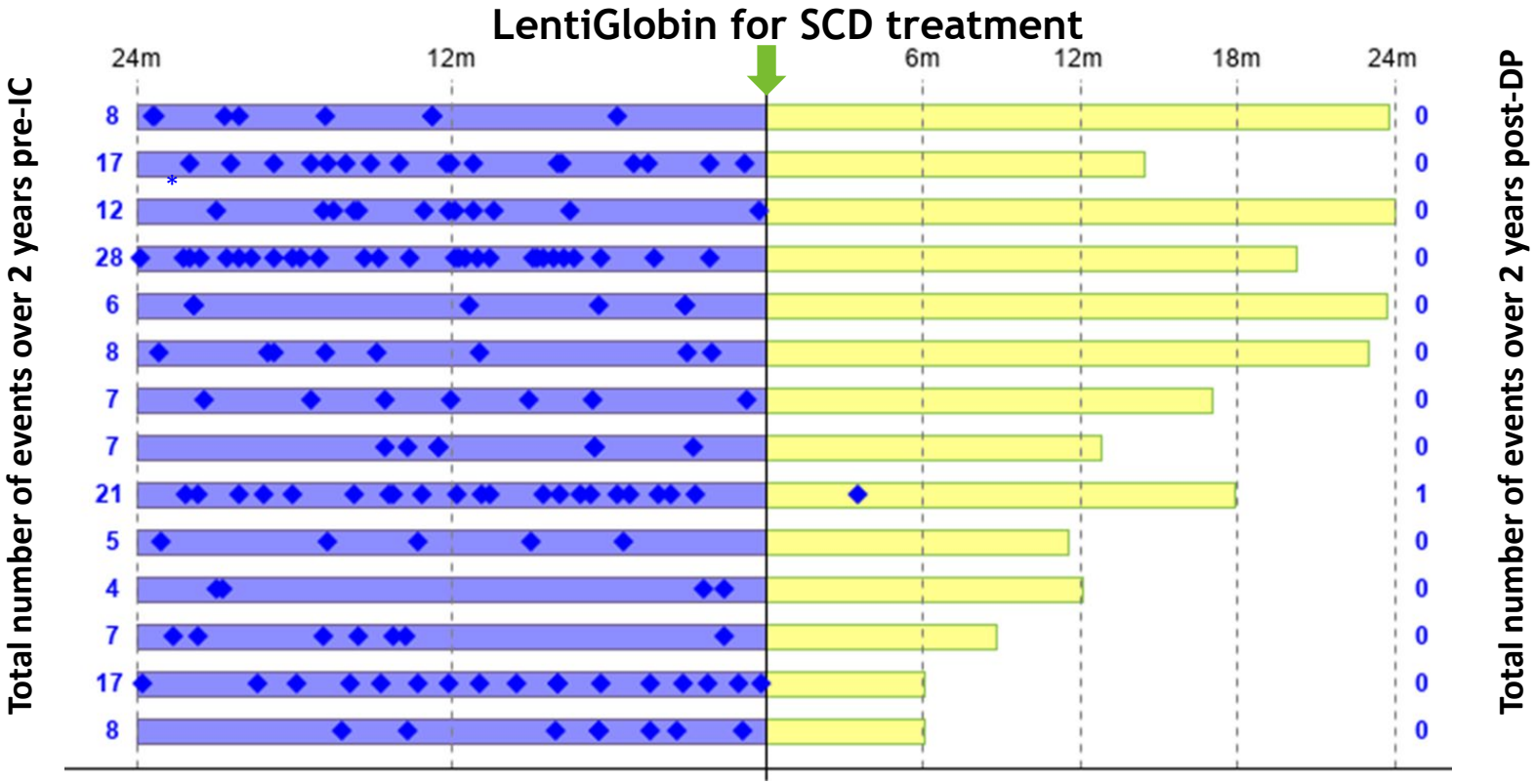


* Currently active, not recruiting; † 1 withdrew consent, 1 at investigator discretion, 1 mobilization failure; ‡ 1 death

DP, drug product

Data as of 3 March 2020

HGB-206 Group C: 99.5% mean reduction of annualized rate of VOCs + ACS post-LentiGlobin treatment



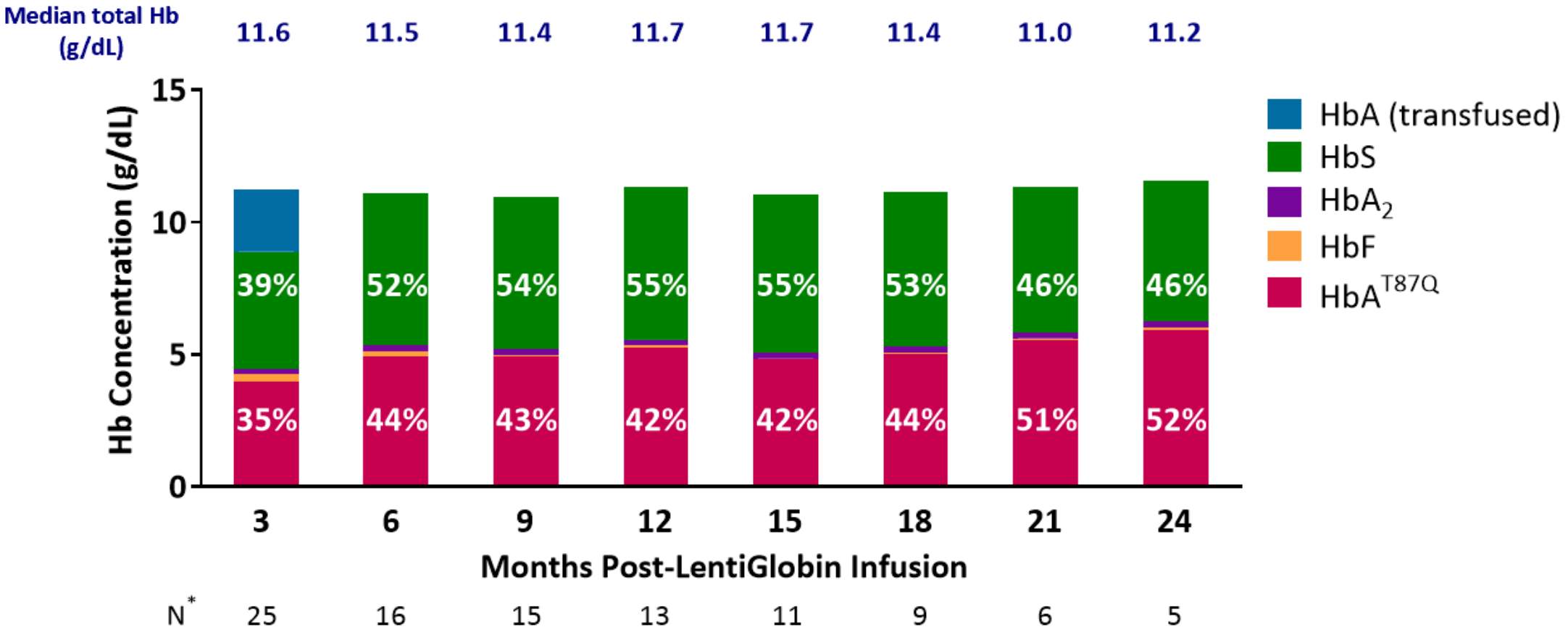
- No ACS or serious VOCs occurred in any Group C patient post-LentiGlobin treatment to date (2.8 – 24.8 months follow-up)
- One previously reported non-serious Grade 2 VOC was observed in 1 patient ~ 3.5 months post-LentiGlobin treatment

Investigator-reported AEs of VOC or ACS are shown; Patients with ≥ 4 VOC/ACS at baseline before IC and with ≥ 6 months of follow-up post-DP infusion are included

ACS, acute chest syndrome; CI, confidence interval; DP, drug product; IC, informed consent; VOC, vaso-occlusive crisis

Data as of 3 March 2020

HGB-206 Group C: Median HbS ≤60% and HbA^{T87Q} ≥40% at ≥6 months post-LentiGlobin treatment



% represents median Hb fraction as % of total Hb; Hb, hemoglobin; * Number of patients with data available

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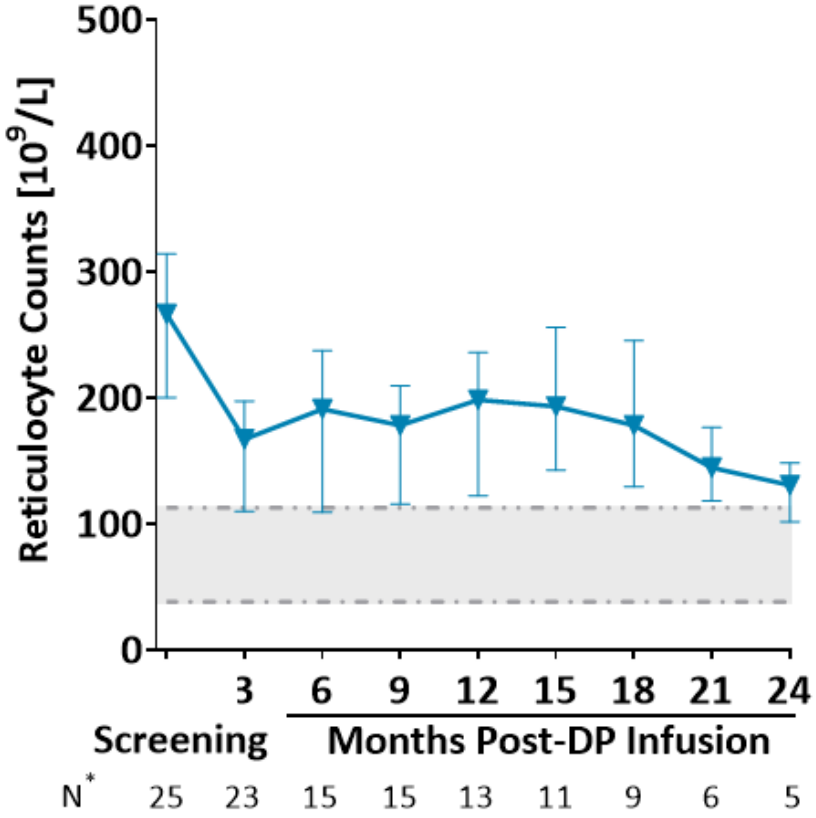
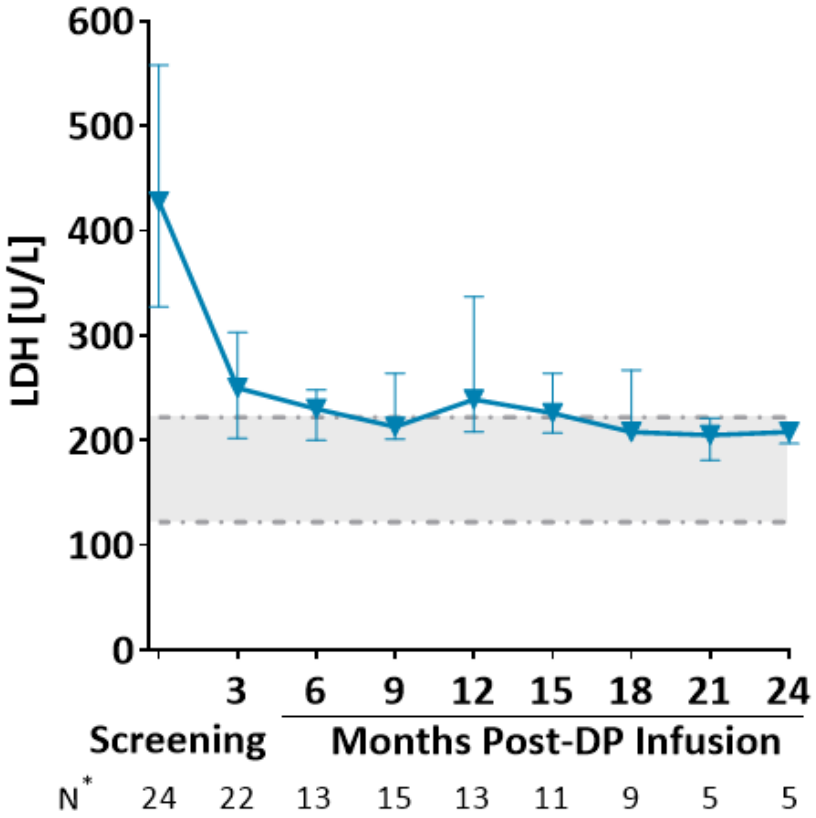
Data as of 3 March 2020

HGB-206 Group C: Decrease in hemolysis markers post-LentiGlobin treatment

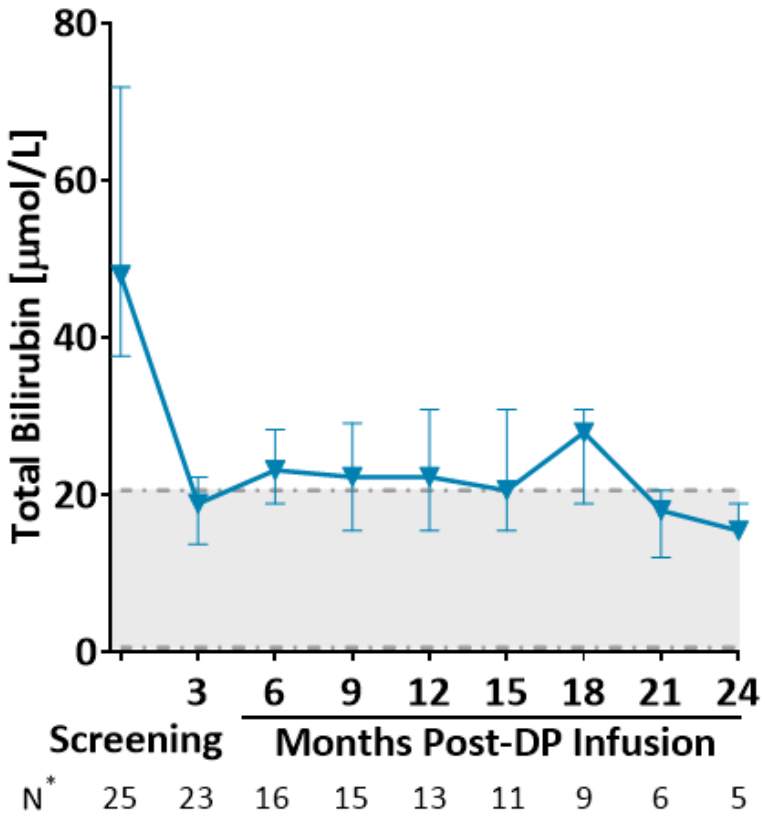
Reticulocyte Counts



Lactate Dehydrogenase



Total Bilirubin

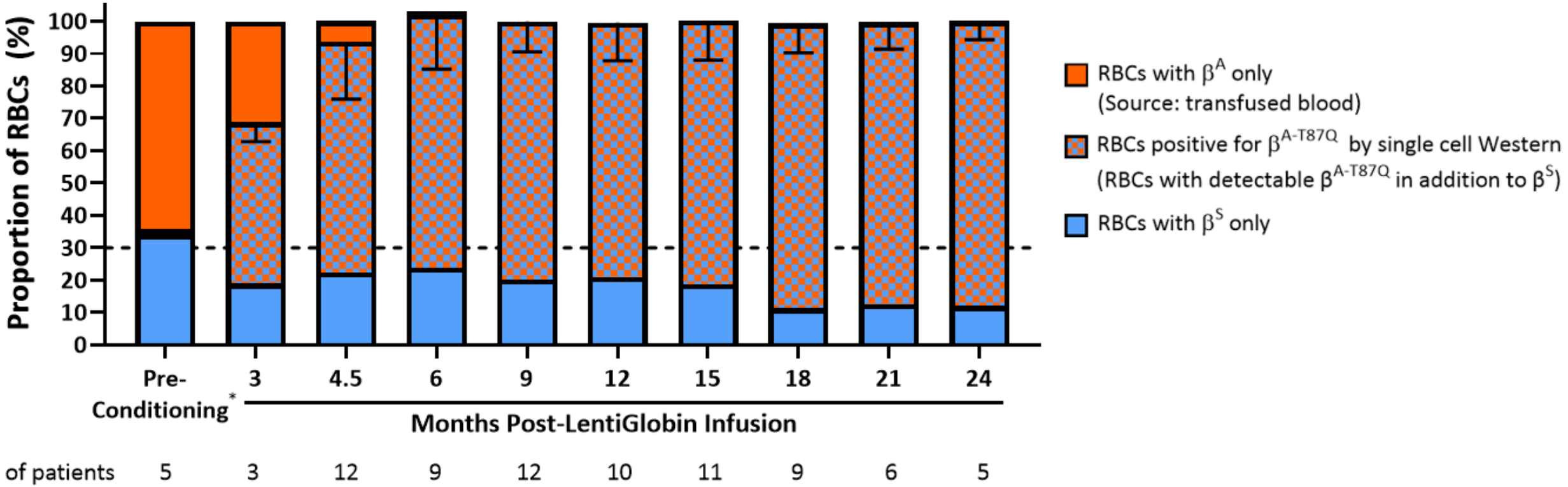


Median (Q1, Q3) depicted; Dot-dash lines denote lower and upper limits of normal values; * Number of patients with data available

Data as of 3 March 2020

Average proportion of RBCs containing $\beta^{A\text{-}T87Q}$ from LentiGlobin-treated patients is ≥70% by month 6 and ~90% by month 18

- Single RBC western assay was performed in subset of HGB-206 Group C patient samples



- **Median (min – max) HbA^{T87Q}/RBC was 15.3 (11.7 – 20)† pg in patients with ≥ 6 months follow-up, which is comparable to the 13 – 18 pg of HbA/RBC in individuals with sickle cell trait‡ and higher than 10 pg of HbF/RBC in those with HPFH§**

Mean & SD are depicted; Reducing HbS to < 30% is recommended by guidelines for exchange RBC transfusions for patients with SCD (indicated by dashed line);* Pre-conditioning sample does not contain any $\beta^{A\text{-}T87Q}$, signal is due to error rate of multiples; † Calculated as (% HbA^{T87Q} of total Hb/% RBCs containing $\beta^{A\text{-}T87Q}$) x MCH; ‡ Calculated to 13-18 pg/RBC using 50% HbA/RBC for the lower end of the range and 60% HbA/RBC for the upper end of the range; § Estimated in Steinberg MH et al., Blood. 2014;123(4):481-5.

HPFH, hereditary persistence of fetal hemoglobin; MCH, mean corpuscular hemoglobin; RBCs, red blood cells; SD, standard deviation

HGB-206 Group C: Safety profile post-LentiGlobin infusion

Non-hematologic ≥ Grade 3 AEs *Post-DP infusion in ≥ 2 patients**	**N=25** n (%)
Stomatitis	15 (60)
Febrile neutropenia	11 (44)
Increased ALT	3 (12)
Increased AST	3 (12)
Increased GGT	3 (12)
Increased total bilirubin	3 (12)
Nausea	3 (12)
Premature menopause	2 (8)
Upper abdominal pain	2 (8)
Serious AEs *Post-DP infusion in ≥ 2 patients*	
Nausea	2 (8)
Opioid withdrawal syndrome	2 (8)
Vomiting	2 (8)

* Hematologic AEs commonly observed post-transplantation have been excluded; AE, adverse events; ALT, alanine aminotransferase; AST, aspartate aminotransferase; GGT, gamma-glutamyl transferase

- 3 patients with DP-related AEs (all nonserious and ≤ Grade 2)†
- No cases of veno-occlusive liver disease
- No graft failure
- No vector-mediated RCL and no insertional oncogenesis
- One death, unlikely related to LentiGlobin: A 27-year-old patient with history of VOC/ACS, pulmonary hypertension, and venous thrombosis died ~20 months post-treatment after sudden onset of shortness of breath followed by cardiac arrest
  - Post-DP: No VOCs/ACS (vs 28 episodes in 2 years pre-study); no sickle-related adverse events or ≥ Grade 3 AEs
    - At last study visit, Hb was 13.9 g/dL, with HbA^{T87Q} 36% and HbS 56%
  - Autopsy showed no evidence of pulmonary embolism, stroke or clinically significant sickling
    - Death was due to CV disease, with findings of cardiomegaly, cardiac fibrosis and pulmonary congestion
  - Per PIs, pre-existing SCD-related cardiac disease and pulmonary hypertension may have been contributing factors

† 1 pt with Grade 2 nonserious neutropenic fever on study day 10 (resolved on study day 18); 1 pt with post-DP infusion Grade 2 AEs of nail discoloration and constipation as well as Grade 1 AEs of runny nose and cough. This pt also had 3 AEs with onset pre-DP infusion (nonserious Grade 2 alopecia, Grade 1 vomiting and Grade 1 fatigue) which were initially assessed as DP-related, but attribution was changed to not DP-related after datacut date; 1 pt with 1 event of nonserious Grade 2 back pain

ACS, acute chest syndrome; CV, cardiovascular; DP, drug product; Hb, hemoglobin; PIs, principal investigators; RCL, replication competent lentivirus; VOC, vaso-occlusive crisis

Updated, accelerated plan based on compelling VOE data

HGB-206 Group C

Sickle Cell Disease, history of vaso-occlusive events (VOEs) over 24 months

Ongoing Phase 1/2, single arm, multi-center, U.S. study
N=41 (Group C)

- Primary Endpoint: Complete resolution of severe VOEs
- Key Secondary Endpoint:
  - HbA^{T87Q} and total Hb
- ≥ 12 years of age - ≤ 50 years of age

HGB-210

Sickle Cell Disease, history of VOEs over 24 months

Phase 3, single arm, multi-center, global study

- Primary Endpoint: HbA^{T87Q} and Total Hb
- Key Secondary Endpoint:
  - Reduction in severe VOEs

1. HGB-206 Group C: Basis of BLA submission in 2H 2021

2. Primary endpoint: VOEs

3. HGB-210: Serving as confirmatory study

Multiple Myeloma – changing what's possible

Standard of Care*

- ~4 months PFS
- ~30% ORR
- ~3% CR

RECODE

BCMA Target &

Next-Gen CAR

ASCO 2020

- ✓ mPFS of 12.1 months at 450x10^6 dose
- ✓ CAR+ T cell persistence observed up to 1yr
- ✓ KarMMa N=128; CRB-401 N=67

2020

- U.S. BLA submitted July 2020
- Ongoing studies in 3L, 2L and 1L (Newly Diagnosed)

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*Lonial et al, Lancet 2016 (Dara); Siegel et al, Blood 2012 (Kyprolis); Hajek et al, Leukemia 2017 (Kyprolis); Chari et al, NEJM 2019 (Selinexor); Richardson et al, Blood 2014 (PomDex)

Multiple Myeloma - ide-cel:
Broad oncology strategy and development program supported by clinical data

BCMA Program

BMS Alignment

- U.S. 50/50 co-co
- Ex-U.S. BMS wholly-owned

Regulatory path enabling near-term launch:

- BLA submitted
- MAA submission accepted

Broad clinical development program enabling potential expansion into earlier lines

ASCO 2020

KarMMa Data

Mature and consistent data demonstrate deep and durable responses:

- CAR+ T cell persistence observed up to 1yr with meaningful detectable vector
- mPFS of 12.1 months at $450x10^6$ dose
- KarMMa N=128; CRB-401 N=67

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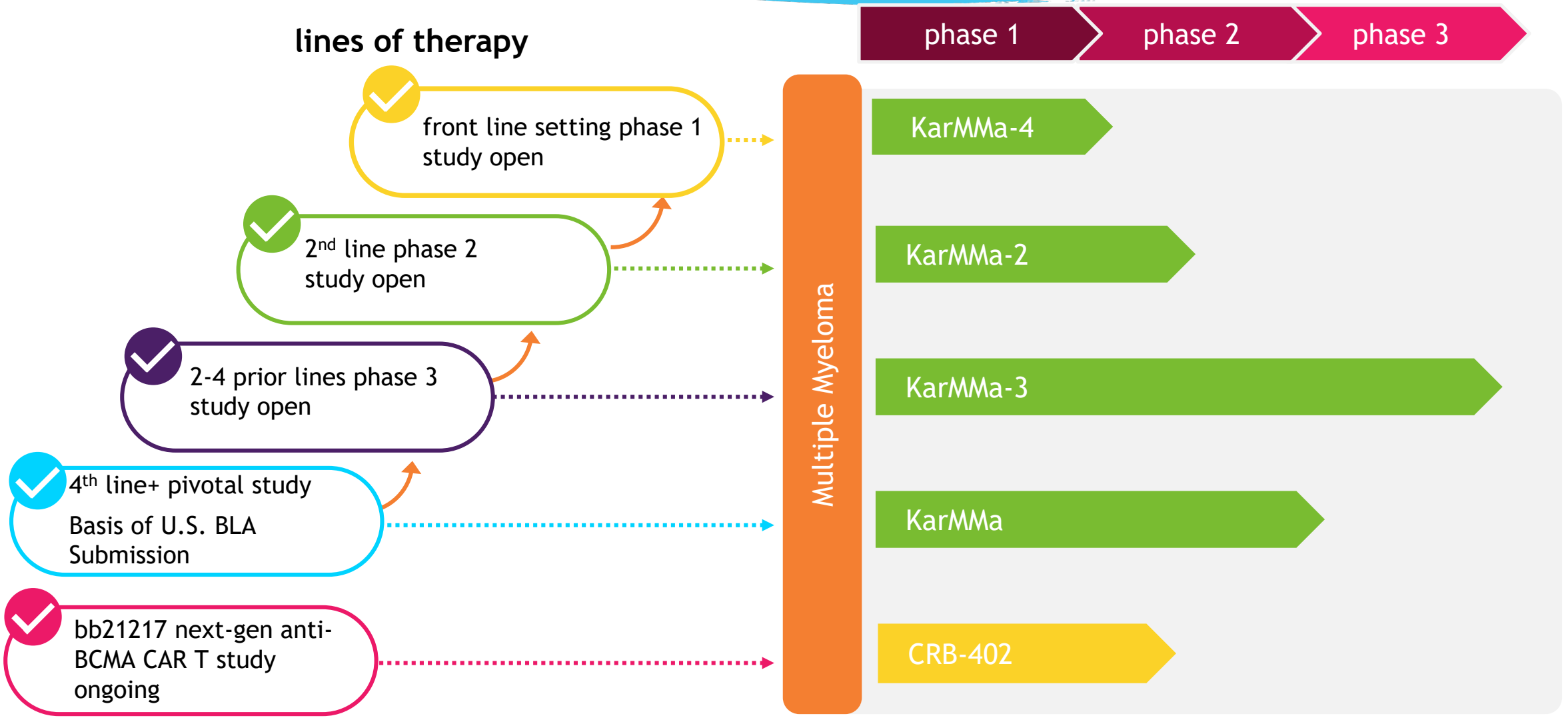
Advancing into earlier lines of therapy and continuing to innovate



Studies ongoing in partnership with BMS

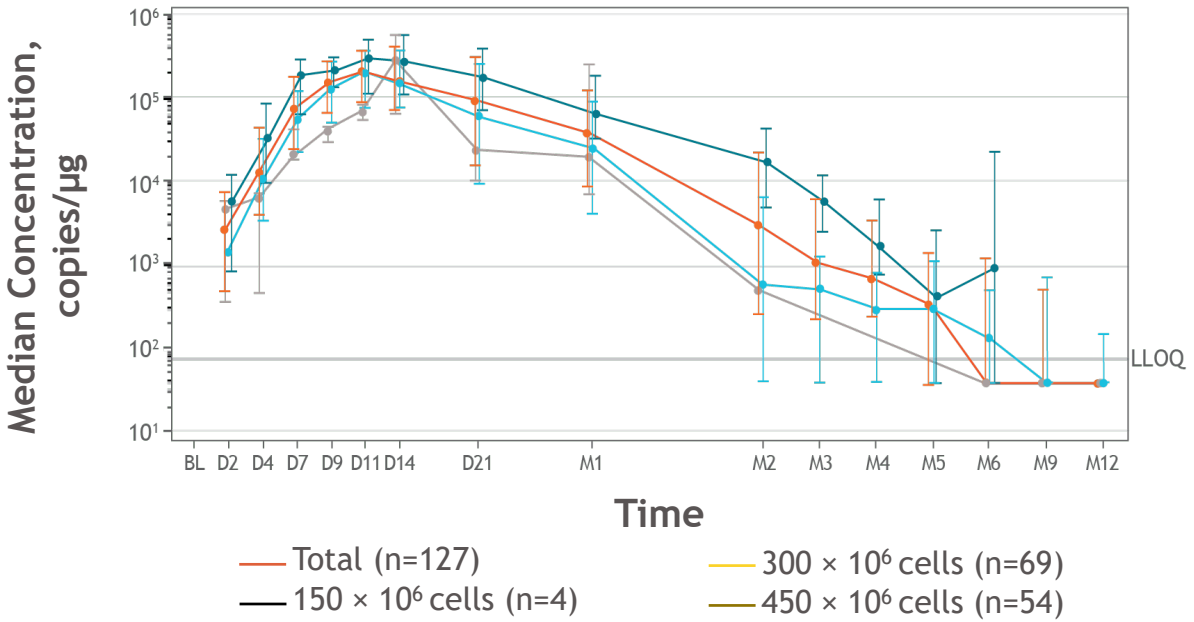
KarMMa: heavily pretreated, refractory patient population

Characteristics		Ide-cel Treated (N=128)
Age, median (range), y		61 (33–78)
Male, %		59
ECOG PS, %	0 1 2	45 53 2
R-ISS Stage,* %	I II III	11 70 16
High-risk cytogenetics [del(17p), t(4;14), t(14;16)],† %		35
High tumor burden (≥50% BMPCs), %		51
Tumor BCMA expression (≥50% BCMA+),‡ %		85
Extramedullary disease, %		39
Time since initial diagnosis, median (range), y		6 (1–18)
No. of prior anti-myeloma regimens, median (range)		6 (3–16)
Prior autologous SCT, %	1 >1	94 34
Any bridging therapies for MM, %		88
Refractory status, %	Anti-CD38 Ab-refractory Triple-refractory	94 84

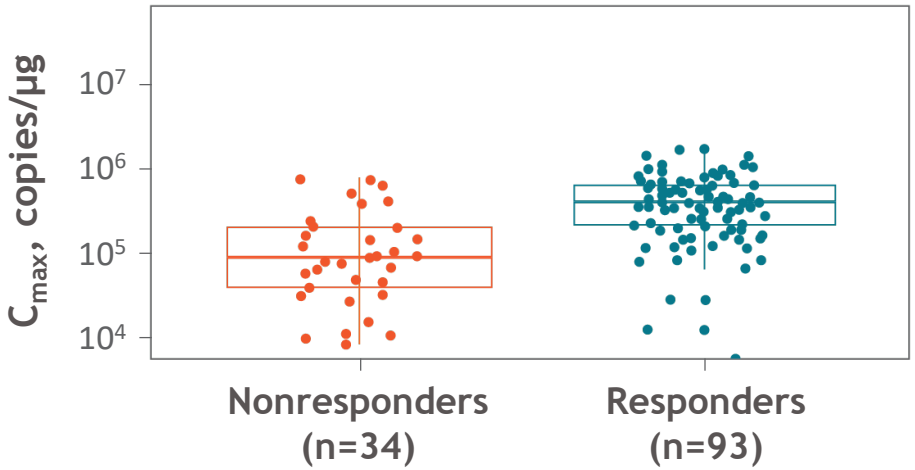
- Patients were heavily pretreated, refractory to last line per IMWG criteria, and mostly refractory to all 3 major MM drug classes
- The majority had high tumor burden and more than one third had extramedullary disease and high-risk cytogenetics
- Tumor BCMA expression identified by IHC in all patients
- Most patients (88%) received bridging therapy during CAR T cell manufacturing
  - Only 4% of patients responded (4 PR, 1 VGPR) to bridging therapy

CAR+ T cell expansion, persistence, and peak exposure

CAR+ T Cell Expansion and Persistence



Peak Vector Copies in Responders (≥PR) vs Nonresponders (<PR)

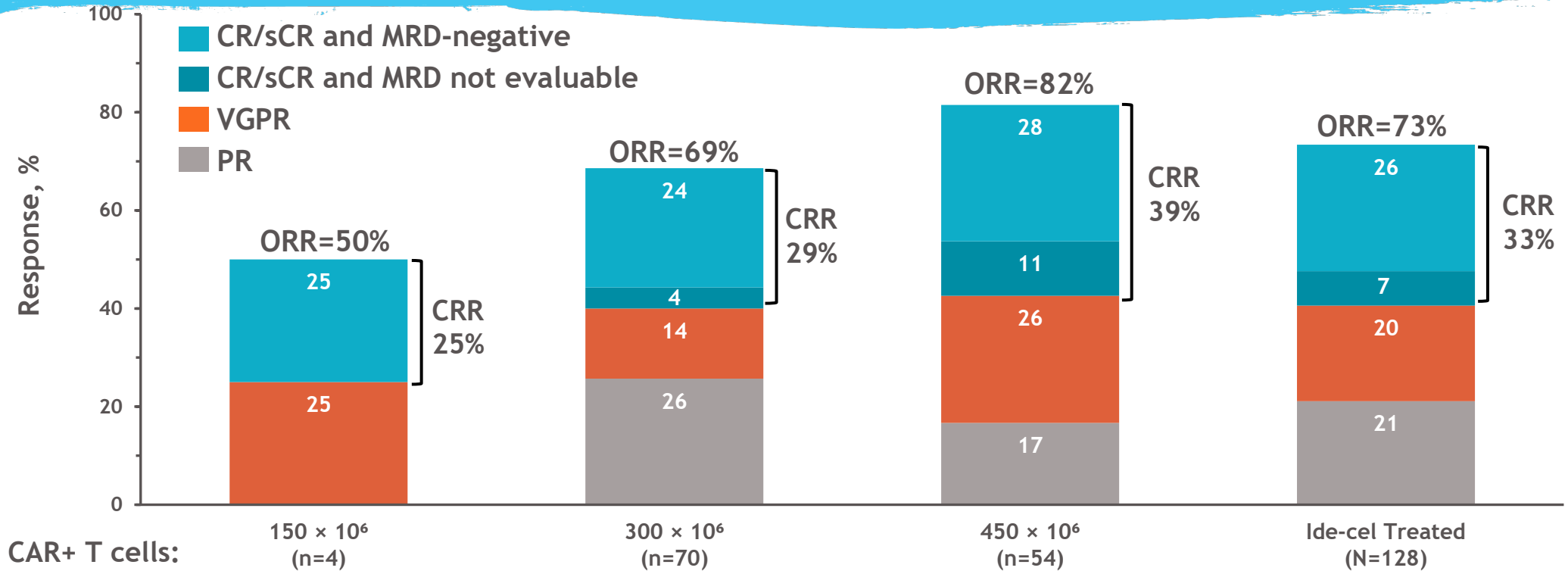


	Mo 1	Mo 3	Mo 6	Mo 9	Mo 12
Evaluable patients, n	118	100	49	27	11
Patients with detectable vector, n (%)	117 (99)	75 (75)	29 (59)	10 (37)	4 (36)

- Median peak CAR+ T cell expansion was at 11 d
- Median expansion increased at higher target doses with overlapping profiles
- Peak exposure higher in responders than nonresponders
- Durable persistence was observed up to 1 y

Data cutoff: 19 April 2019. Pharmacokinetic (PK) analysis population (N=127). One patient died on day 4 and had no evaluable PK samples and was therefore excluded. Error bars represent interquartile range. BL, baseline; C_{max}, maximum concentration; LLOQ, lower limit of quantitation; M, month.

82% ORR and 39% CR rate at 450 x 10^6 dose level



- Primary (ORR >50%) and key secondary (CRR >10%) endpoints met in the ide-cel treated population
  - *ORR of 73% (95% CI, 65.8–81.1; P<0.0001*)*
  - *CRR (CR/sCR) of 33% (95% CI, 24.7–40.9; P<0.0001)*
- Median time to first response of 1.0 mo (range, 0.5–8.8); median time to CR of 2.8 mo (range, 1.0–11.8)
- Median follow-up of 13.3 mo across target dose levels
- All patients with CR or sCR and were evaluable for MRD, were MRD-negative

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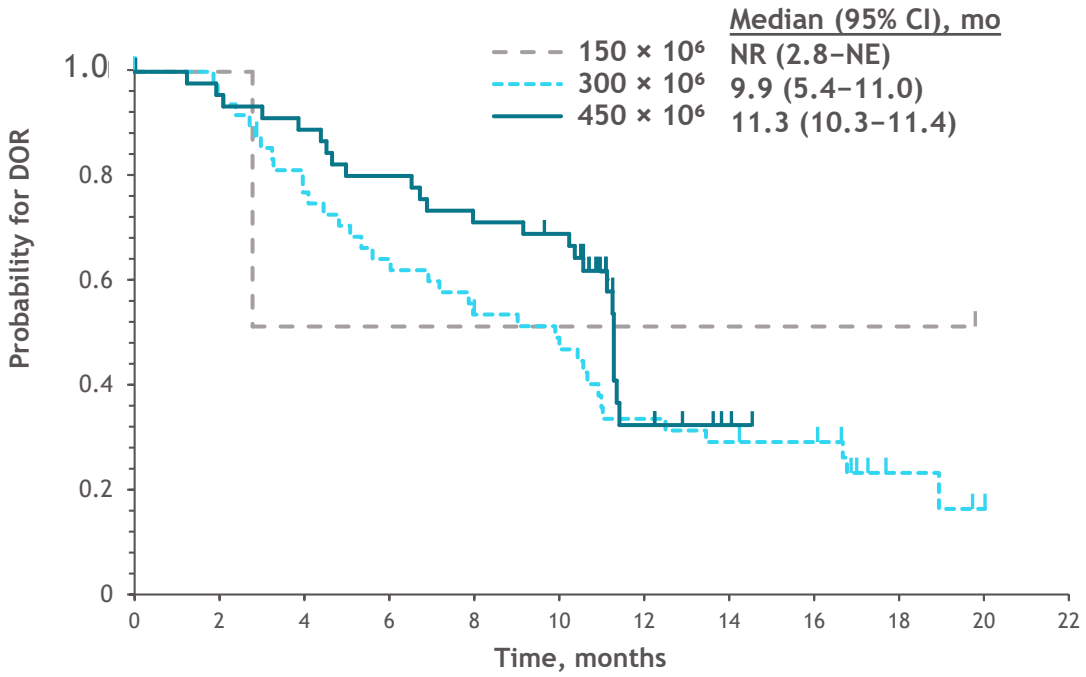
Data cutoff: 14 Jan 2020. MRD-negative defined as $<10^{-5}$ nucleated cells by next generation sequencing. Only MRD values within 3 mo of achieving CR/sCR until progression/death (exclusive) were considered.
Values may not add up due to rounding.
CR/sCR, complete response/stringent CR; CRR, CR rate; MRD, minimal residual disease; ORR, overall response rate (≥PR); PR, partial response; VGPR, very good PR. **P* value at the primary data cutoff with same ORR and 95% CI.

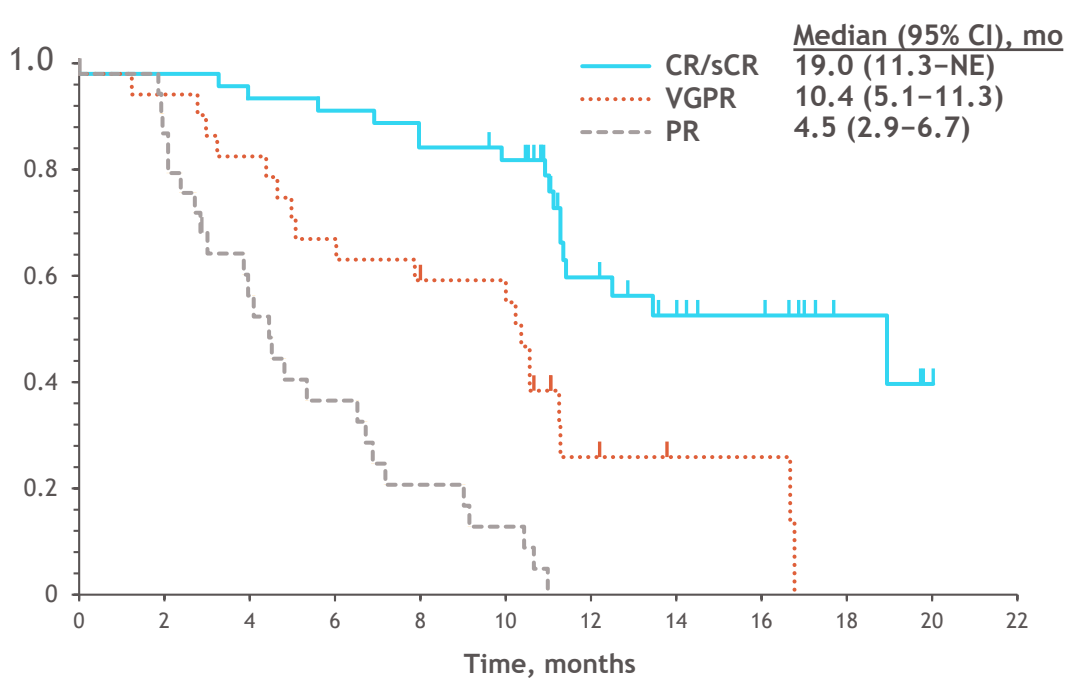
mDOR of 11.3 mo at 450 × 10^6 dose; mDOR of 19 mo in patients achieving CR/sCR

DOR by Target Dose



At risk, N												
150 × 10^6	2	2	1	1	1	1	1	1	1	1	0	
300 × 10^6	48	45	35	29	24	21	14	12	11	3	1	0
450 × 10^6	44	42	39	35	31	29	7	2	0	0	0	

DOR by Best Response



At risk, n												
CR/sCR	42	42	40	39	36	34	18	13	10	4	1	0
VGPR	25	24	21	17	15	14	4	2	2	0	0	
PR	27	23	14	9	5	3	0	0	0	0	0	

- Durable responses were observed across all target doses; DOR increased with depth of response

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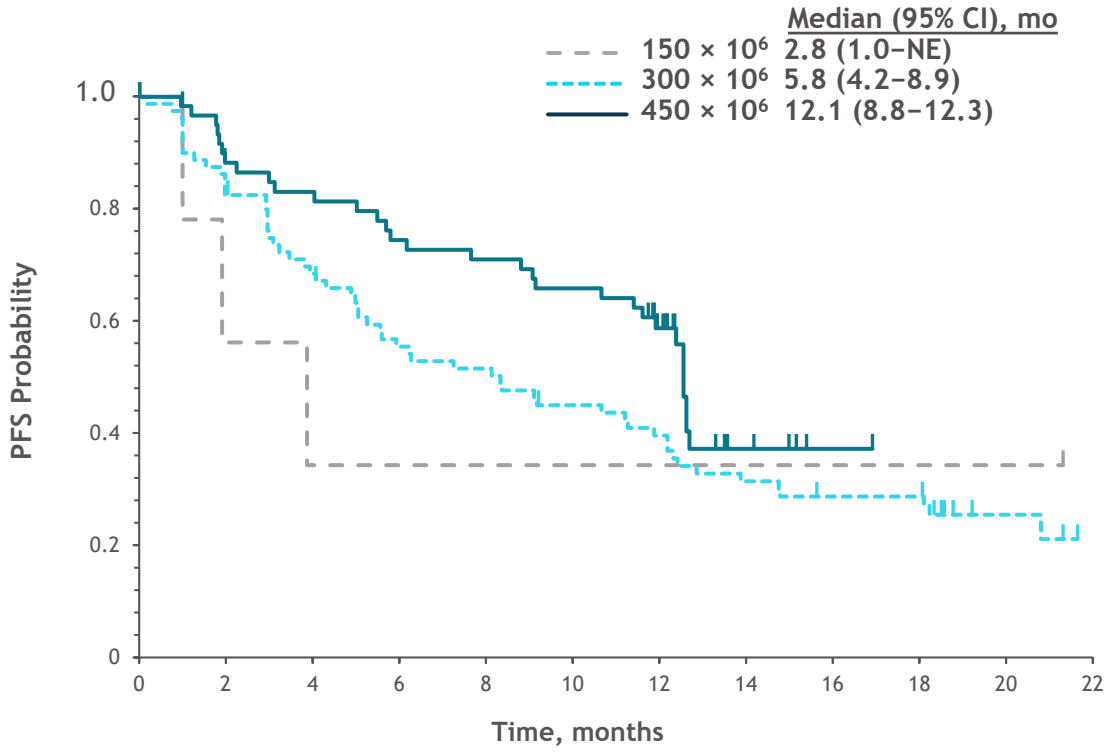
Data cutoff: 14 Jan 2020. CR/sCR, complete response/stringent CR; DOR, duration of response; NE, not estimable; NR, not reached; PR, partial response; VGPR, very good PR.

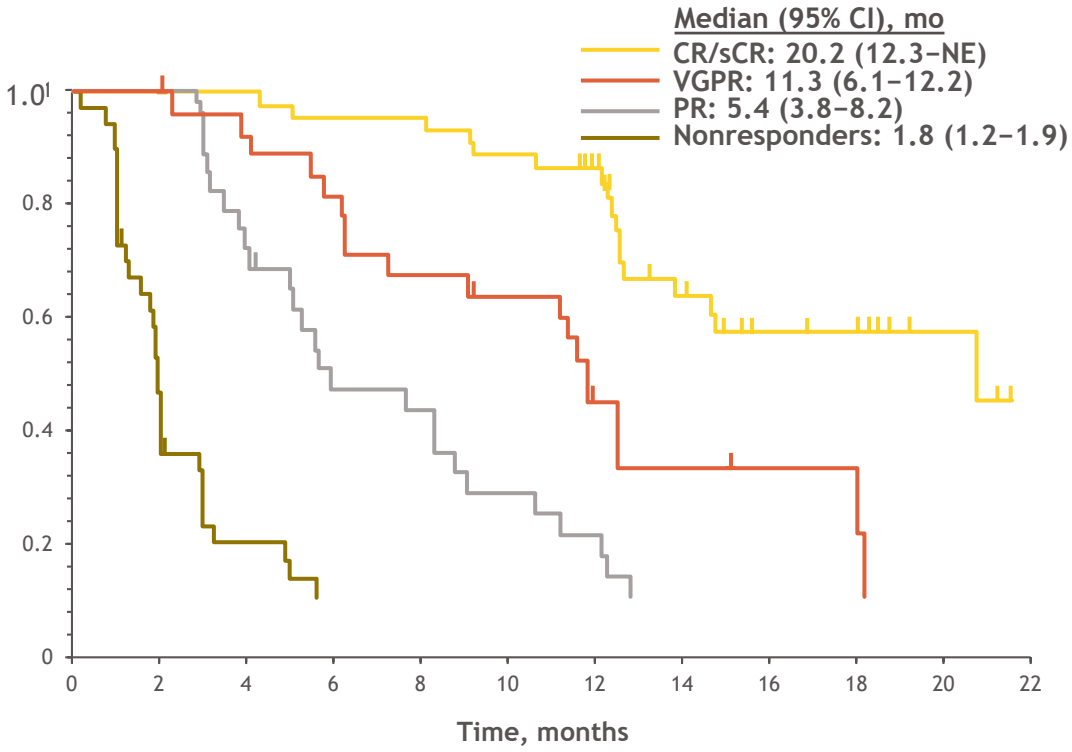
mPFS of 12.1 months at 450 x 10^6 dose level; mPFS of 20.2 months in patients with a CR/sCR

PFS by Target Dose



At risk, N												
150 × 10^6	4	2	1	1	1	1	1	1	1	1	1	0
300 × 10^6	70	56	42	33	29	24	17	14	11	7	2	0
450 × 10^6	54	44	40	36	34	31	17	4	1	0	0	

PFS by Best Response



CR/sCR	42	42	42	40	39	37	26	16	11	8	4	0
VGPR	25	25	22	20	16	14	8	3	2	0	0	
PR	27	16	10	9	5	1	0	0	0	0	0	
Nonresponders	34	8	83	70	64	56	35	19	13	8	4	0

- PFS increased with higher target dose; median PFS was 12 mo at 450 × 10^6 CAR+ T cells
- PFS increased by depth of response; median PFS was 20 mo in patients with CR/sCR

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Data cutoff: 14 Jan 2020. NE, not estimable; PFS, progression-free survival.

Safety profile consistent with known toxicities of CAR T therapy

CRS

Ide-cel Treated (N=128)	
≥1 CRS event, n (%)	107 (84)
Max. grade (Lee Criteria)* 1/2 3 4 5	100 (78) 5 (4) 1 (<1) 1 (<1)
Median onset, d (range)	1 (1–12)
Median duration, d (range)	5 (1–63)
Tocilizumab, n (%)	67 (52)
Corticosteroids, n (%)	19 (15)

Neurotoxicity

Ide-cel Treated (N=128)	
≥1 NT event, n (%)	23 (18)
Max. grade (CTCAE)* 1 2 3	12 (9) 7 (5) 4 (3)
Median onset, d (range)	2 (1–10)
Median duration, d (range)	3 (1–26)
Tocilizumab, n (%)	3 (2)
Corticosteroids, n (%)	10 (8)

- Ide-cel was tolerable across the dose range
- Grade ≥3 CRS or iiNT ≤6% at target dose of 450 × 10^6 CAR+ T cells
  - CRS frequency increased with dose, but mostly low grade
- Cytopenias were common; not dose related
- Infections (including bacterial, viral, fungal) were common (69%); not dose-related
- 5 deaths (4%) within 8 wk of ide-cel infusion (2 following disease progression, 3 from AEs) and 1 from an AE within 6 mo of ide-cel infusion

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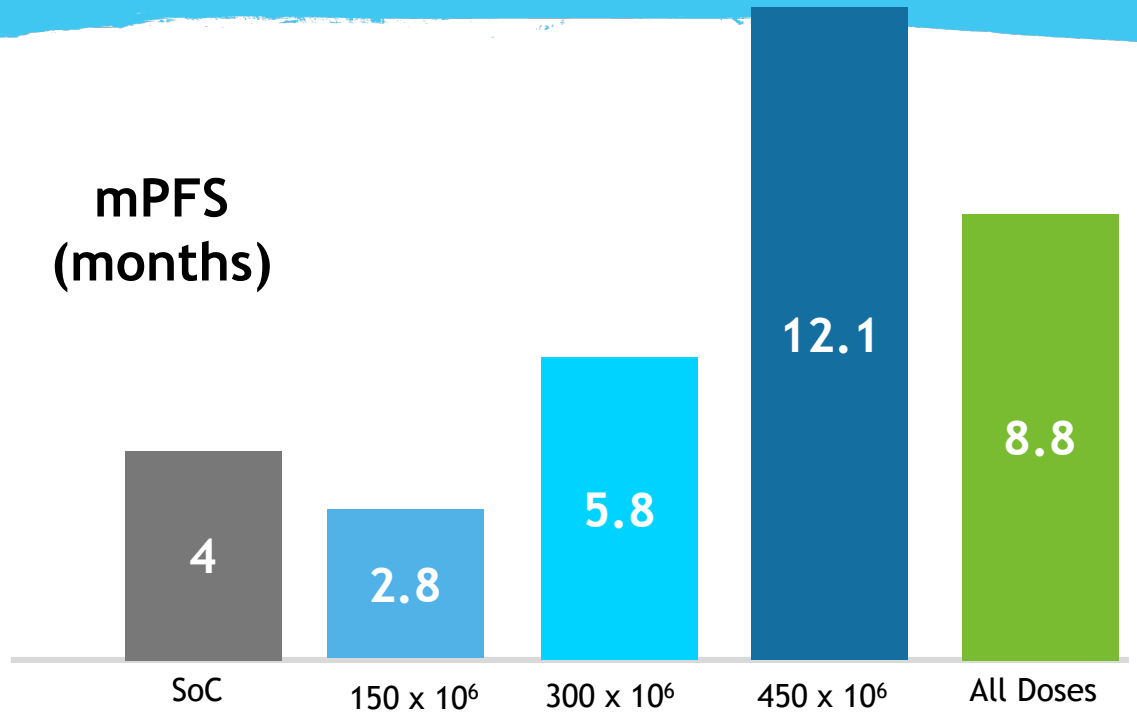
Data cutoff: 14 Jan 2020; CRS, cytokine release syndrome, iiNT, investigator identified neuotoxicity

ide-cel (bb2121) - Positive Pivotal Data at ASCO



	150 x 10^6 CAR+ T cells (N=4)	300 x 10^6 CAR+ T cells (N=70)	450 x 10^6 CAR+ T cells (N=54)	All Doses (N=128)
ORR, n (%)	2 (50.0)	48 (68.6)	44 (81.5)	94 (73.4)
CR/sCR, n (%)	1 (25.0)	20 (28.6)	21 (39)	42 (33)
Median DoR, mo	---	9.9	11.3	10.7

Heavily pretreated population

- Median 6 prior lines of therapy, 94% refractory to anti-CD38, 84% triple refractory
- All patients were refractory to their last treatment (progression during or within 60 days of last therapy)

Deep and durable responses across dose levels

- mPFS of >12mo at the 450 x 10^6 dose
- All patients who had CR or sCR, who were evaluable for minimal residual disease (MRD), were MRD-negative
- Durability is consistent across doses

Safety consistent with the Ph1 data

- Gr ≥ 3 CRS and iiNT were reported in <6% of subjects at each target dose
- CRS and iiNT of any grade occurred in 83.6% and 18% of patients, respectively

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iiNT: investigator identified neurotoxicity
Ide-cel is being developed in collaboration with Bristol-Myers Squibb

Revised BMS Collaboration: Aligned to Support ide-cel Commercialization

shared commitment

- U.S. co-promote/ co-develop intact
- KarMMa development program underway in earlier lines

monetization

- bluebird to receive $200m for ex-U.S. milestones and royalties

manufacturing alignment

- BMS to manufacture vector ex-U.S. over time
- bluebird to continue U.S. vector manufacturing

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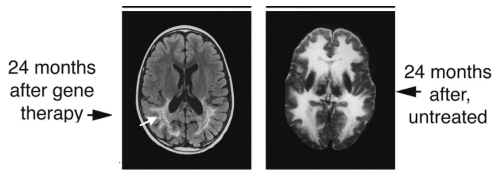
Cerebral Adrenoleukodystrophy - From Tragedy to Hope

2009

Science
AAAS



RECODE

Enhanced Construct
&
Manufacturing

EPNS: 2019

- ✓ 15/17 patients alive and MFD-free at 24 months follow up and continue to be MFD-free with up to 5 years of follow-up
- ✓ 32 total patients treated

Data as of April 25, 2019

2020

- **2H 2020 anticipated MAA submission**
- **Newborn screening active in 14 US states; several pilot programs in EU**

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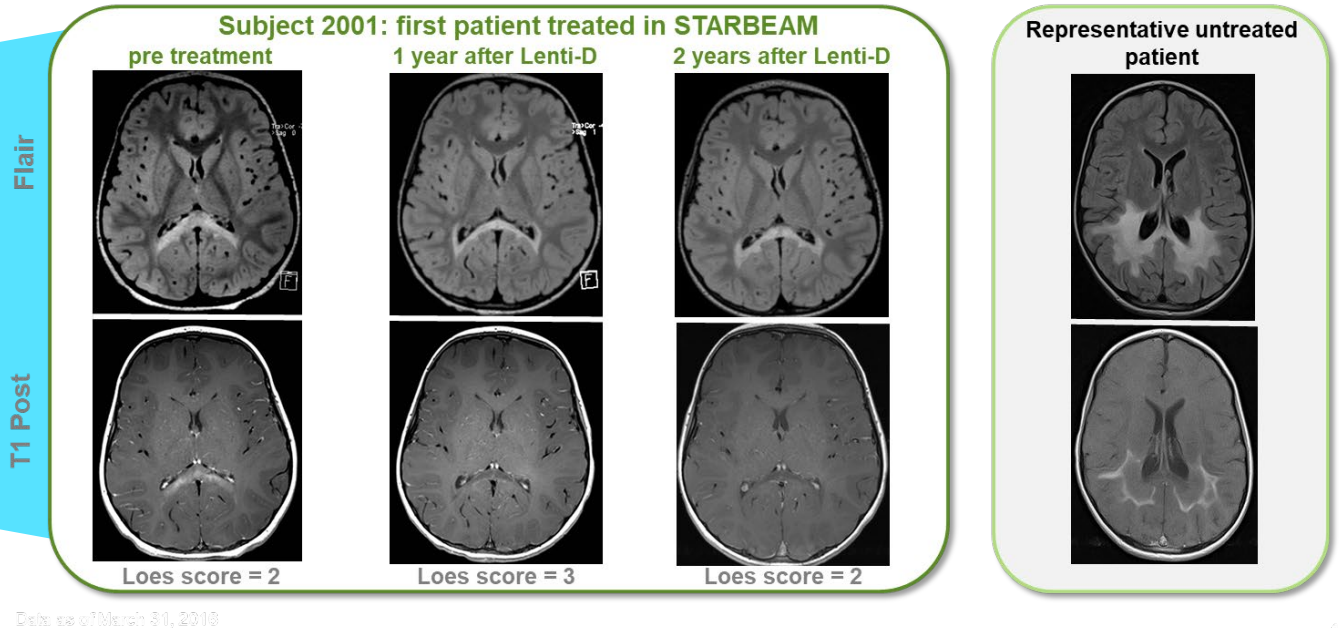
Science 2009

Lenti-D treatment halts CALD disease progression



Data as of March 31, 2018

- **All patients who were alive and MRD-free at 24 months follow up (15/17; 88%) continue to be MFD-free with up to 5 years of follow-up**
  - 32 patients have been treated with Lenti-D with a median follow-up time of 21.2 months
  - 14 patients are still on study with less than 24 months of follow-up and show no evidence of MFDs
  - Three patients did not or will not meet the primary efficacy endpoint; two patients withdrew from the study at investigator discretion, and one experienced rapid disease progression early on-study resulting in MFDs and death.
- **Safety profile consistent with autologous transplantation**
  - No GvHD, no graft rejection
- **Enrollment completed in Starbeam study**
  Phase 3 ALD-104 study currently enrolling

Data as of April 25, 2019

R&D BLUE style: what do we work on?

Core Research Principles

Programs with the Potential to Transform Patient Lives	Diseases with Definitive Endpoints of Clinical Success	Targets with Human Genetic and/or Functional Validation	Disruptive Solutions to the Problems that Need to be Solved
We tackle diseases with a clear unmet medical need based on the magnitude of impact and not necessarily the number of patients	Clinical success should be objective, measurable, un-incremental, and rapid	Biology may be complex but the role of the target in the disease must be definitive	We don't do incremental science. We take on the big problems that, if successful, will disrupt our field

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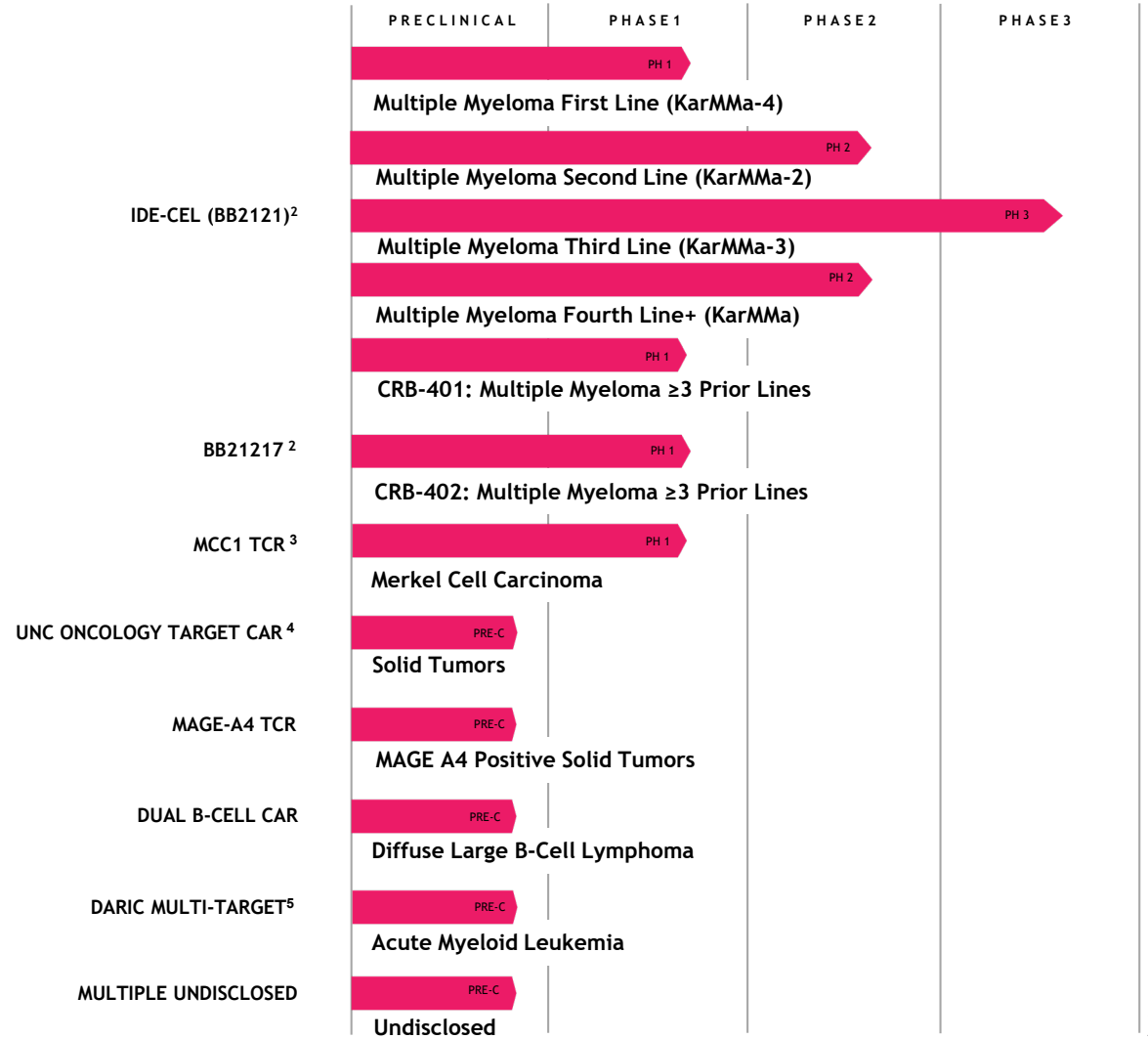
pipeline overview

[1] Dev is led by Dana-Farber/Boston Children's Cancer and Blood Disorders Center
[2] Dev is led in collaboration with Bristol Myers Squibb
[3] Dev is led by Fred Hutch Cancer Research Institute
[4] Dev is led by University of North Carolina
[5] Dev is led by Seattle Children's Research Institute

Severe Genetic Diseases

Oncology



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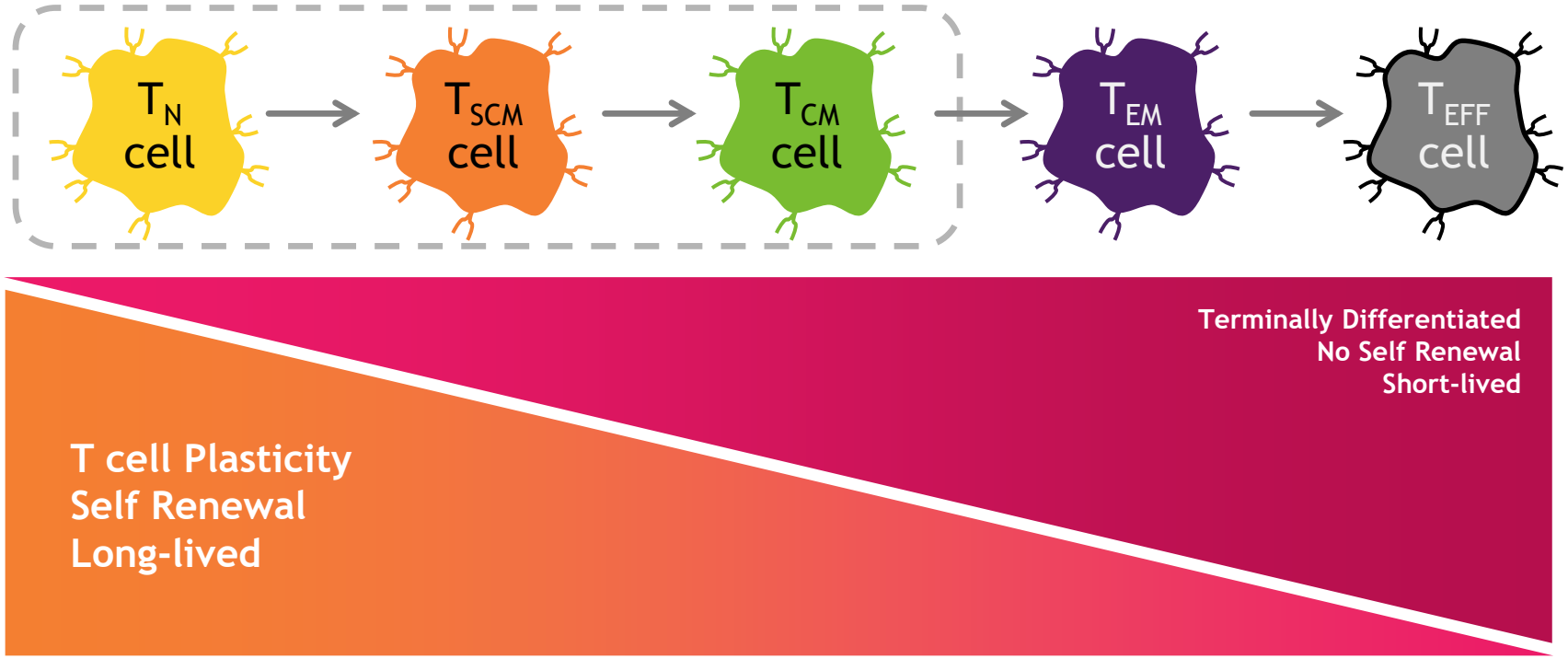
bb21217: PI3K inhibition during manufacturing drives increase in long-lived, memory-like T cells



Hypothesis: Increasing long-lived, memory-like T cell subsets in the drug product may result in enhanced persistence of functional anti-BCMA CAR T cells *in vivo*

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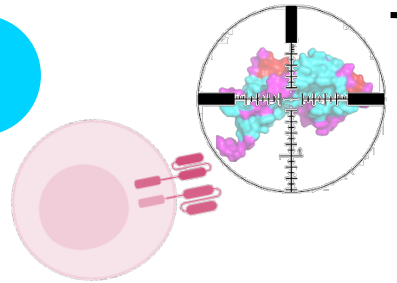
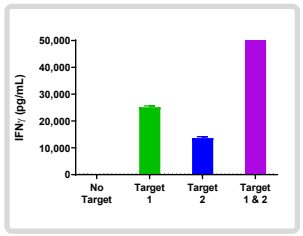
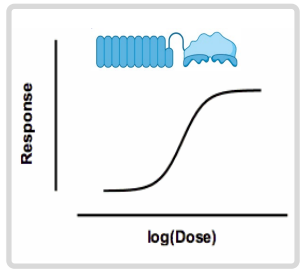
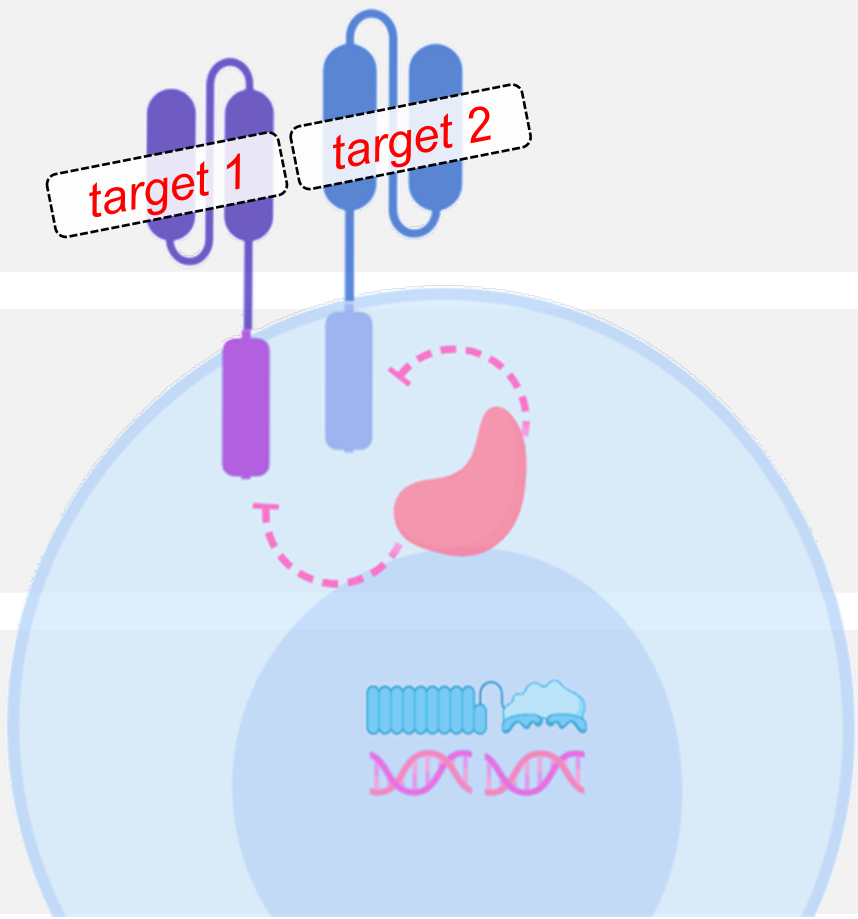
Diffuse Large B-Cell Lymphoma -Triple Threat Approach

	LAYER	PURPOSE
1	*dual-CAR targeting*	prevent escape
2	*signal extension*	enhance T cell activation
3	*signal amplification*	improve T cell persistence









EACH LAYER INFORMS 1:MANY PLATFORM

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2020-2021: BLUE is Prepared and On Track for the Catalysts Ahead

	2020	2021
Regulatory	✓ LentiGlobin SCD Regulatory Update ✓ Ide-cel (bb2121) MM U.S. BLA submission ▪ Lenti-D CALD EU MAA Submissions	▪ LentiGlobin SCD U.S. BLA submission (2H) ▪ LentiGlobin TDT U.S. BLA submission (Q2/Q3) ▪ Lenti-D CALD U.S. BLA submission (mid-year)
Clinical Updates	✓ Ide-cel (bb2121) KarMMa data at ASCO, CRB-401 by EOY ✓ SCD: HGB-206 data at EHA, EOY ✓ TDT: HGB-207, HGB-212 Data at EHA ▪ Lenti-D ALD-102 data update by EOY	▪ Ide-cel KarMMa studies progressing and evolving ▪ Building and evolving clinical data set on SGD programs
Commercial & Foundation Building	▪ ZYNTEGLO Access and Reimbursement established in additional EU countries ▪ ZYNTEGLO first commercial patients treated (2H) ▪ Ide-cel U.S. launch ready	▪ Ide-cel U.S. launch underway ▪ ZYNTEGLO geographic expansion ▪ LentiGlobin TDT U.S. launch ready and SCD gearing up

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