Recoding in Action
Q3 2020

LET'S RECODE THE STORY
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.
Tremendous Progress in Challenging Times

Programs and Pipeline

- EHA, ASCO, EBMT data support upcoming regulatory submissions and launches
- ide-cel BLA accepted with Priority Review
- Clarity on accelerated approval for SCD based on complete resolution of VOEs
- Continuing to treat patients in clinical studies at consistent pace despite COVID

Operation Plan

- Revised operating plan by over $500M through mid-2022
- Raised approx. $540m in equity offering
- Optimized BMS collaboration & $200M rights monetization
- Extended cash runway into 2023
Transfusion-Dependent β-Thalassemia - reimagined future

**RECODE**
Vector Potency & Manufacturing Enhancement

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

**EHA 2020**
- EU Approved 2019
- US rolling BLA initiated 2019
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
Northstar-2: Non-β²/β² 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

Data as of 7 April 2020

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 3 March 2020

Median unsupported total Hb is ≥ 11.5 g/dL

89% (17/19) of evaluable patients achieved primary endpoint: transfusion independence

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.
Northstar-3: $B^0/B^0$ patients continue to show compelling results

- 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

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**Transfusion status in patients with ≥ 3 months follow-up**

- Time from treatment to last transfusion
- Time from last transfusion to last follow-up
- Patient < 12 yrs old

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**Total Hb and HbAT87Q over time in patients who have not received a transfusion in > 60 days**

- As transduced HSCs engraft and produce mature RBCs, HbAT87Q levels increase and stabilize approximately 6 – 9 months after beti-cel infusion

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

**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 $\beta^0/\beta^0$ 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
preparing to serve patients in Europe

1 drug product manufacturing

- Munich, Germany

initial launch focus

- Germany
- Italy
- UK
- France

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
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

STRONG FOUNDATION FORMING
Sickle Cell Disease – Daring to Dream

2017

**RECODE**
- Pre-Tx Transfusions
- More Thorough Conditioning
- Higher Cell Dose
- Higher VCN

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

EHA 2020

- 99.5% reduction in annualized rate of VOC + ACS*

- Development plans under accelerated approval underway

New England Journal of Medicine 2017
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, primary endpoint of complete resolution of VOEs
Sickle cell disease is characterized by high morbidity and early mortality

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

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

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

> High levels of HbS in RBCs lead to O₂ polymerization & sickling, resulting in vaso-occlusion, hemolysis, and vasculopathy.

> 50% of patients with SCD die before 45 years of age.
HGB-206 Group C: Patients infused to support BLA submission

**Consented**
N=51

- Screen failure
  N=7

- Discontinued†
  N=3

**Plerixafor Mobilization & Apheresis**
N=40

- Cell Collection Pending
  N=4

**DP Manufacture Completed**
N=36

- DP Manufacture Pending
  N=1

**LentiGlobin DP Infused**
N=25
Median follow-up: 12.1 months (min – max, 2.8 – 24.8 months)

- Transplant Pending
  N=11

- Discontinued‡
  N=1

* 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

Data as of 3 March 2020
HGB-206 Group C: Decrease in hemolysis markers post-LentiGlobin treatment

- **Reticulocyte Counts**
  - Screening: 25
  - Months Post-DP Infusion: 23, 15, 15, 13, 11, 9, 6, 5

- **Lactate Dehydrogenase (LDH)**
  - Screening: 24
  - Months Post-DP Infusion: 22, 13, 15, 13, 11, 9, 5, 5

- **Total Bilirubin**
  - Screening: 25
  - Months Post-DP Infusion: 23, 16, 15, 13, 11, 9, 6, 5

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 $\beta^{A-T87Q}$ 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$^8$

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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 $\beta^{A-T87Q}$, signal is due to error rate of multiples;$^†$ Calculated as (% HbA$^{T87Q}$ of total Hb)% RBCs containing $\beta^{A-T87Q}$ 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

Data as of 3 March 2020
HGB-206 Group C: Safety profile post-LentiGlobin infusion

### Non-hematologic ≥ Grade 3 AEs

<table>
<thead>
<tr>
<th>Non-hematologic ≥ Grade 3 AEs</th>
<th>N=25</th>
<th>n (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Post-DP infusion in ≥ 2 patients</strong>*</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Stomatitis</td>
<td>15 (60)</td>
<td></td>
</tr>
<tr>
<td>Febrile neutropenia</td>
<td>11 (44)</td>
<td></td>
</tr>
<tr>
<td>Increased ALT</td>
<td>3 (12)</td>
<td></td>
</tr>
<tr>
<td>Increased AST</td>
<td>3 (12)</td>
<td></td>
</tr>
<tr>
<td>Increased GGT</td>
<td>3 (12)</td>
<td></td>
</tr>
<tr>
<td>Increased total bilirubin</td>
<td>3 (12)</td>
<td></td>
</tr>
<tr>
<td>Nausea</td>
<td>3 (12)</td>
<td></td>
</tr>
<tr>
<td>Premature menopause</td>
<td>2 (8)</td>
<td></td>
</tr>
<tr>
<td>Upper abdominal pain</td>
<td>2 (8)</td>
<td></td>
</tr>
</tbody>
</table>

### Serious AEs

<table>
<thead>
<tr>
<th>Serious AEs</th>
<th>Post-DP infusion in ≥ 2 patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nausea</td>
<td>2 (8)</td>
</tr>
<tr>
<td>Opioid withdrawal syndrome</td>
<td>2 (8)</td>
</tr>
<tr>
<td>Vomiting</td>
<td>2 (8)</td>
</tr>
</tbody>
</table>

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

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

- 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

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

† 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 data cut date; 1 pt with 1 event of nonserious Grade 2 back pain
**Updated plan for accelerated approval based on compelling VOE data**

1. **HGB-206 Group C**: Basis of BLA submission in 2H 2021
   - **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
     - \(\geq 12\) years of age - \(\leq 50\) years of age

2. **Primary endpoint**: VOEs

3. **HGB-210**: Serving as confirmatory study
   - **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
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)

*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
Advancing into earlier lines of therapy and continuing to innovate

lines of therapy

- Front line setting phase 1 study open

- 2nd line phase 2 study open

- 2-4 prior lines phase 3 study open

- 4th line+ pivotal study (Basis of U.S. BLA Submission)

- bb21217 next-gen anti-BCMA CAR T study ongoing

Multiple Myeloma

- phase 1
  - KarMMa-4

- phase 2
  - KarMMa-2
  - KarMMa-3
  - KarMMa

- phase 3
  - CRB-402

Studies ongoing in partnership with BMS
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.

### Characteristics Ide-cel Treated (N=128)

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

- 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
• 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

Data cutoff: 14 Jan 2020. MRD-negative defined as <10^-5 nucleated cells by next generation sequencing. Only MRD values within 3 m 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

• Durable responses were observed across all target doses; DOR increased with depth of response
mPFS of 12.1 months at 450 x 10^6 dose level; mPFS of 20.2 months in patients with a CR/sCR

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

Data cutoff: 14 Jan 2020. NE, not estimable; PFS, progression-free survival.
Safety profile consistent with known toxicities of CAR T therapy

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### CRS

<table>
<thead>
<tr>
<th>Ide-cel Treated (N=128)</th>
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</thead>
<tbody>
<tr>
<td>≥1 CRS event, n (%)</td>
<td>107 (84)</td>
</tr>
<tr>
<td>Max. grade (Lee Criteria)*</td>
<td></td>
</tr>
<tr>
<td>1/2</td>
<td>100 (78)</td>
</tr>
<tr>
<td>3</td>
<td>5 (4)</td>
</tr>
<tr>
<td>4</td>
<td>1 (&lt;1)</td>
</tr>
<tr>
<td>5</td>
<td>1 (&lt;1)</td>
</tr>
<tr>
<td>Median onset, d (range)</td>
<td>1 (1−12)</td>
</tr>
<tr>
<td>Median duration, d (range)</td>
<td>5 (1−63)</td>
</tr>
<tr>
<td>Tocilizumab, n (%)</td>
<td>67 (52)</td>
</tr>
<tr>
<td>Corticosteroids, n (%)</td>
<td>19 (15)</td>
</tr>
</tbody>
</table>

### Neurotoxicity

<table>
<thead>
<tr>
<th>Ide-cel Treated (N=128)</th>
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</thead>
<tbody>
<tr>
<td>≥1 NT event, n (%)</td>
<td>23 (18)</td>
</tr>
<tr>
<td>Max. grade (CTCAE)*</td>
<td></td>
</tr>
<tr>
<td>1</td>
<td>12 (9)</td>
</tr>
<tr>
<td>2</td>
<td>7 (5)</td>
</tr>
<tr>
<td>3</td>
<td>4 (3)</td>
</tr>
<tr>
<td>Median onset, d (range)</td>
<td>2 (1−10)</td>
</tr>
<tr>
<td>Median duration, d (range)</td>
<td>3 (1−26)</td>
</tr>
<tr>
<td>Tocilizumab, n (%)</td>
<td>3 (2)</td>
</tr>
<tr>
<td>Corticosteroids, n (%)</td>
<td>10 (8)</td>
</tr>
</tbody>
</table>

- Ide-cel was tolerable across the dose range
- Grade ≥3 CRS or iiNT ≤6% at target dose of $450 \times 10^6$ 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
**ide-cel (bb2121) - Positive Pivotal Data at ASCO**

**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

**mpFSS (months)**

<table>
<thead>
<tr>
<th></th>
<th>150 x 10^6</th>
<th>300 x 10^6</th>
<th>450 x 10^6</th>
<th>All Doses (N=128)</th>
</tr>
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<tr>
<td>SoC</td>
<td>4</td>
<td>2.8</td>
<td>5.8</td>
<td>8.8</td>
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<tr>
<td>ORR, n (%)</td>
<td>2 (50.0)</td>
<td>48 (68.6)</td>
<td>44 (81.5)</td>
<td>94 (73.4)</td>
</tr>
<tr>
<td>CR/sCR, n (%)</td>
<td>1 (25.0)</td>
<td>20 (28.6)</td>
<td>21 (39)</td>
<td>42 (33)</td>
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<tr>
<td>Median DoR, mo</td>
<td>...</td>
<td>9.9</td>
<td>11.3</td>
<td>10.7</td>
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</tbody>
</table>

ide-cel is being developed in collaboration with Bristol-Myers Squibb

iiNT: investigator identified neurotoxicity
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
Cerebral Adrenoleukodystrophy – From Tragedy to Hope

**RECODE**
Enhanced Construct & Manufacturing

- 20/23 patients alive and MFD-free at 24 months follow up, all continue to be MFD-free with up to 5 years of follow-up
- 32 total patients treated
  
  Data as of January 2020

- 2H 2020 anticipated MAA submission
- Newborn screening active in 17 US states; several pilot programs in EU

ALD-102 EBMT: 2020
Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy

Florian Eichler, M.D., Christine Duncan, M.D., Patricia L. Musolino, M.D., Ph.D., Paul J. Orchard, M.D., Sefirot De Oliveira, M.D., Adrian J. Thirsk, M.D., Myriam Arnot, Ph.D., Colleen Domenicos, M.N., R.N., Troy C. Lund, M.D., Weston P. Miller, M.D., Gerald V. Raymond, M.D., Raman Sankar, M.D., Ann J. Shah, M.D., Caroline Sevo, M.D., Ph.D., H. Bully Cagiar, M.D., Paul Gissen, M.D., Heman Amareno, M.D., Gregor Battrick, M.D., Nicholas J. Smith, M.D., Ash M. Paker, M.D., Esther Shnitir, M.P.H., Tara O’Meala, B.S., David Davidson, M.D., Patrick Aubourg, M.D., and David A. Williams, M.D.


ALD-102: all patients who were alive and MRD-free at 24 months follow up (20/23; 87%) continue to be MFD-free with up to 5 years of follow-up
- 32 patients have been treated with eli-cel with a median follow-up time of 30.0 months
- 9 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 or graft failure

Enrollment completed in ALD-102 Starbeam study Phase 3 ALD-104 study currently enrolling

Data as of January 2020
R&D BLUE style: what do we work on?

Core Research Principles

**Programs with the Potential to Transform Patient Lives**

We tackle diseases with a clear unmet medical need based on the magnitude of impact and not necessarily the number of patients.

**Diseases with Definitive Endpoints of Clinical Success**

Clinical success should be objective, measurable, un-incremental, and rapid.

**Targets with Human Genetic and/or Functional Validation**

Biology may be complex but the role of the target in the disease must be definitive.

**Disruptive Solutions to the Problems that Need to be Solved**

We don’t do incremental science. We take on the big problems that, if successful, will disrupt our field.
### Severe Genetic Diseases

<table>
<thead>
<tr>
<th>Product Candidate</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>ELI-CEL</td>
<td>PRE-C</td>
<td>PH 1/2</td>
<td>PH 2/3</td>
</tr>
<tr>
<td>LENTIGLOBIN™</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>TDT Non-80/80</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>TDT, Including</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>SCD (HGB-210)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>SCD (HGB-206)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>BCL11A shRNA(mir)</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>MPSI GENE THERAPY</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>MULTIPLE UNDISCLOSED</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
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</table>

### Oncology

<table>
<thead>
<tr>
<th>Product Candidate</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
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<tbody>
<tr>
<td>IDE-CEL (BB2121)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>Multiple Myeloma First Line (KarMMa-4)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>Multiple Myeloma Second Line (KarMMa-2)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>Multiple Myeloma Third Line (KarMMa-3)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>Multiple Myeloma Fourth Line+ (KarMMa)</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>CRB-401: Multiple Myeloma ≥3 Prior Lines</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
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<tr>
<td>CRB-402: Multiple Myeloma ≥3 Prior Lines</td>
<td>PRE-C</td>
<td>PH 2/3</td>
<td>PH 3</td>
</tr>
<tr>
<td>MCC1 TCR</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
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<tr>
<td>Merkel Cell Carcinoma</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>UNC ONCOLOGY TARGET CAR</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>Solid Tumors</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>MAGE-A4 TCR</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>MAGE A4 Positive Solid Tumors</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>DUAL B-CELL CAR</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>Diffuse Large B-Cell Lymphoma</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>DARIC MULTI-TARGET</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>Acute Myeloid Leukemia</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
<tr>
<td>MULTIPLE UNDISCLOSED</td>
<td>PRE-C</td>
<td>PH 1</td>
<td>PH 3</td>
</tr>
</tbody>
</table>

---

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
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*
Diffuse Large B-Cell Lymphoma - Triple Threat Approach

<table>
<thead>
<tr>
<th>LAYER</th>
<th>PURPOSE</th>
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<tbody>
<tr>
<td>1</td>
<td>dual-CAR targeting</td>
</tr>
<tr>
<td>2</td>
<td>signal extension</td>
</tr>
<tr>
<td>3</td>
<td>signal amplification</td>
</tr>
</tbody>
</table>

EACH LAYER INFORMS 1: MANY PLATFORM
2020-2021: BLUE is Prepared and On Track for the Catalysts Ahead

<table>
<thead>
<tr>
<th>2020 Complete</th>
<th>2020 Upcoming</th>
<th>2021</th>
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<tbody>
<tr>
<td>✓ LentiGlobin SCD Regulatory Update</td>
<td>✓ Eli-cel CALD EU MAA Submission</td>
<td>✓ LentiGlobin SCD U.S. BLA submission (2H)</td>
</tr>
<tr>
<td>✓ Ide-cel (bb2121) MM U.S. BLA submission</td>
<td></td>
<td>✓ LentiGlobin TDT U.S. BLA submission (mid-year)</td>
</tr>
<tr>
<td>✓ Ide-cel (bb2121) KarMMa data at ASCO</td>
<td>✓ SCD: HGB-206 data by end of year</td>
<td>✓ Ide-cel KarMMa studies progressing and evolving</td>
</tr>
<tr>
<td>✓ SCD: HGB-206 data at EHA</td>
<td>✓ Ide-cel CRB-401 data by end of year</td>
<td>✓ Building and evolving clinical dataset on SGD programs</td>
</tr>
<tr>
<td>✓ TDT: HGB-207, HGB-212 Data at EHA</td>
<td>✓ ZYNTEGLO Access and Reimbursement established in additional EU countries</td>
<td>✓ Ide-cel U.S. launch underway</td>
</tr>
<tr>
<td>✓ Eli-cel ALD-102 data update by EOY</td>
<td>✓ ZYNTEGLO first commercial patients treated</td>
<td>✓ ZYNTEGLO geographic expansion</td>
</tr>
<tr>
<td>✓ SCD: HGB-206 data at EHA</td>
<td>✓ Ide-cel U.S. launch ready</td>
<td>✓ LentiGlobin TDT U.S. launch ready and SCD gearing up</td>
</tr>
<tr>
<td>✓ SCD First patients treated with sLVV</td>
<td>✓ ZYNTEGLO Launch in Germany</td>
<td></td>
</tr>
</tbody>
</table>