

1Q 2020 Company Update

May 11, 2020

LET'S
RECODE
THE STORY

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.**



Tremendous Progress in Challenging Times

Programs and Pipeline

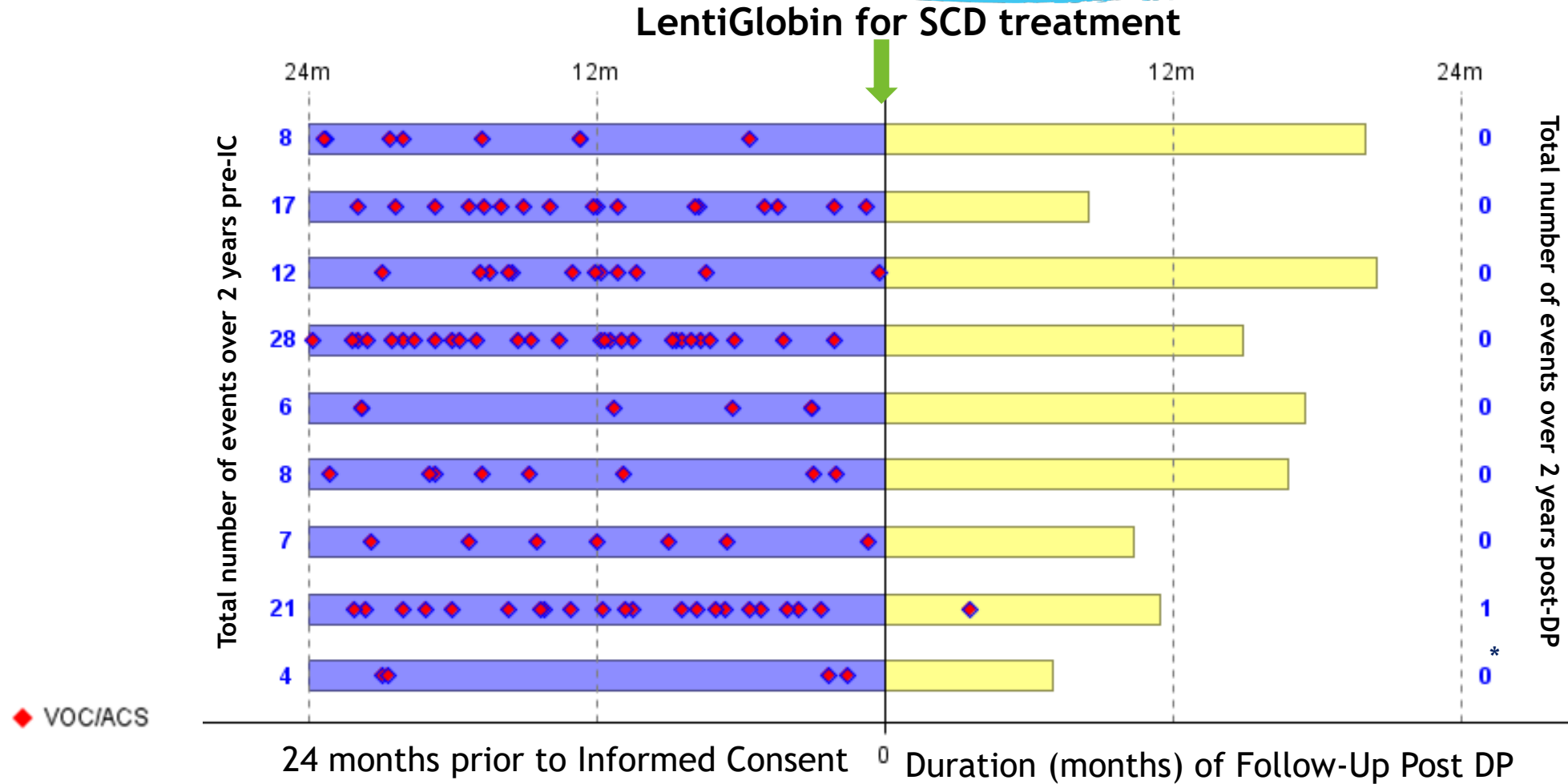
- ▶ BLA submitted for ide-cel (Updated data at ASCO)
- ▶ Clarity on accelerated US regulatory path for SCD (Updated data at EHA)
- ▶ Key 2021 milestones tracking: EU TDT ramp, ide-cel launch, US TDT, ALD & SCD filings & pipeline emergence



Operation Plan

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

Compelling VOC+ACS Data at ASH 2019



*As previously reported, 1 non-serious Grade 2 VOC was observed in 1 patient ~3.5 months post-LentiGlobin treatment
Investigator-reported adverse events of VOC or ACS are shown; *Patients with ≥ 4 VOC/ACS at baseline before Informed Consent and with $\sim \geq 6$ months of follow-up post-DP infusion
ACS, acute chest syndrome; VOCs, vaso-occlusive crises; DP, drug product

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:
 - HbAT87Q and total Hb
- ≥ 12 years of age - ≤ 50 years of age

Updated HGB-206 Data to be Presented at EHA 2020

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: The basis of the
BLA submission in 2H 2021

2. Primary endpoint:
VOEs

3. HGB-210: Serving as
confirmatory study

Core Four Programs Continue to Progress

ide-cel

**U.S. BLA Submitted
KarMMA Data at ASCO**

LentiGlobin TDT

**Ongoing Engagement in EU
Phase 3 Data at EHA**

LentiGlobin SCD

**Updated Regulatory Path
HGB-206 Group C
Data at EHA**

Lenti-D

**Updated Clinical Data and
Planned MAA filing in 2020**

Revised Operating Plan

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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

Revised Operating Plan: Committed to Financial and Operational Sustainability

Critical to Plan:

- ▶ Core 4: Vision to launch 4 commercial therapies
- ▶ Manufacturing: Deep supply chain and established suspension LVV
- ▶ Robust Research Engine: Supporting Core 4, expanding Core 4, and promising preclinical programs
- ▶ People & Culture: Keeping bluebird BLUE!

Prioritization & Cost Saving:

- ▶ Facilities: Taking actions to reduce our facility footprint and fixed cost overhead
- ▶ Reduced / Deferred SG&A Build: In line with commercial timing and forecast
- ▶ Label-Expanding Studies: HGB-211 indefinitely paused
- ▶ Research: Prioritization of preclinical programs



EXTEND CASH RUNWAY INTO 2022

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

	2020	2021
Regulatory	<ul style="list-style-type: none"> ✓ Ide-cel (bb2121) MM U.S. BLA submission ✓ LentiGlobin SCD Regulatory Update ▪ Lenti-D CALD EU MAA Submissions 	<ul style="list-style-type: none"> • 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	<ul style="list-style-type: none"> • Ide-cel (bb2121) KarMMa data at ASCO, CRB-401 by EOY • SCD: HGB-206 Data at EHA • TDT: HGB-207, HGB-212 Data at EHA • Lenti-D ALD-102 data update by EOY 	<ul style="list-style-type: none"> • Ide-cel KarMMa studies progressing and evolving • Building and evolving clinical data set on SGD programs
Commercial & Foundation Building	<ul style="list-style-type: none"> • ZYNTEGLO Access and Reimbursement established in additional EU countries • ZYNTEGLO first commercial patients treated (2H) • Ide-cel U.S. launch ready 	<ul style="list-style-type: none"> • Ide-cel U.S. launch underway • ZYNTEGLO geographic expansion • LentiGlobin TDT U.S. launch ready and SCD gearing up