Providing Hope to the Underserved

September 8, 2020
SAFE HARBOR STATEMENT

Statements we make in this presentation may include statements that are not historical facts and are considered forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended (collectively, the “Acts”). We intend these forward-looking statements, including statements regarding our mission, vision, goals, plans, milestones and future activities, the safety, efficacy, mechanism of action, other product characteristics, availability, use, commercialization and commercial and therapeutic potential of Oxbryta® (voxelotor), including the potential to reduce morbidity and mortality, to be a disease-modifying therapy, to address top priorities, transforming the treatment paradigm, delivering to patients, and the significance of increasing hemoglobin, the impact of the COVID-19 pandemic, the commercial supply of Oxbryta, the availability and use of GBT Source™, payer coverage, implementing and completing clinical development plans for voxelotor, generating and reporting data and analyses from past, ongoing and potential future studies of voxelotor, regulatory review, our manufacturing and commercial infrastructure, our pipeline, the attributes, potential and future development of inclacumab, actual and potential partnerships and distribution arrangements, expanding access to Oxbryta for patients in the U.S. and globally, our financial position, guidance and expectations, available funds and related expectations, and intellectual property rights, to be covered by the safe harbor provisions for forward-looking statements contained in the Acts and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect our views as of the time made about our plans, intentions, expectations, strategies and prospects, which are based on the information then available to us and on assumptions we have made. We can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved, and, furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control, including, without limitation, risks and uncertainties relating to the COVID-19 pandemic, including the extent and duration of the impact on our business, the risks that we have only recently established our commercialization capabilities and may not be able to successfully commercialize Oxbryta, risks associated with our dependence on third parties for development, manufacture and commercialization activities related to Oxbryta, government and third-party payer actions, including relating to reimbursement and pricing, risks and uncertainties relating to competitive products and other changes that may limit demand for Oxbryta, the risks regulatory authorities may require additional studies or data to support continued commercialization of Oxbryta, the risks that drug-related adverse events may be observed during commercialization or clinical development, and that data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, compliance with the funding and other obligations under the Pharmakon loan, progress under our distribution agreement for select Middle East countries, and progress of our collaboration with Syros, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2019, and in our most recent Quarterly Report on Form 10-Q filed with the U.S. Securities and Exchange Commission, as well as discussions of potential risks, uncertainties and other important factors in our subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.
OUR VISION: TRANSFORM SICKLE CELL DISEASE

Delivering on our Mission

Oxbryta U.S. Launch Progress

Looking Ahead
OUR MISSION

GBT discovers, develops and delivers life-changing treatments for people living with grievous blood-based disorders, starting with SCD.
OXBRYTA NOW AVAILABLE FOR U.S. PATIENTS

+ First FDA-approved medicine that directly inhibits the sickling and destruction of red blood cells in sickle cell disease (SCD)
  – First-in-class HbS polymerization inhibitor; first SCD medicine to gain accelerated approval
  – Approved Nov. 25, 2019, three months ahead of PDUFA date

+ Approved to treat SCD in adults and children age 12 and older
  – Improves hemoglobin and other clinical measures of hemolysis
  – May be given with or without hydroxyurea

+ Post-approval confirmatory study underway – using transcranial Doppler (TCD) flow velocity to demonstrate a decrease in stroke risk in children 2-15 years of age

HbS, sickle hemoglobin.

Important Safety Information
The Prescribing Information for Oxbryta includes Warnings and Precautions for hypersensitivity reactions and laboratory test interference. Please see additional Important Safety Information at the end of this presentation and the full Prescribing Information for Oxbryta at https://www.oxbryta.com/pdf/prescribing-information.pdf.

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OXBRYTA U.S. LAUNCH KEY METRICS

Launch-to-Date Progress

$47.7M
Revenue

3,000
New
Prescriptions$1

920
Unique
Prescribers$1

$574.2 Million
cash, cash equivalents
and marketable
securities
(at 6/30/20)

• Access to $75 million under term loan facility
• Potential runway to achieve positive cash flow

Payer Coverage
(lives covered
at 6/30/20)

53%

44%
Commercial

62%
Medicaid

53%
Medicare

• Fee-for-service Medicaid coverage in 42
states, including all 17 priority states
• Payer coverage includes lives covered
through medical exceptions

1. Numbers of new patient prescriptions and unique prescribers are approximate.
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ASSESSING THE IMPACT OF COVID-19

**SCD Patients**
- Increased risk of severe illness and/or death from COVID-19\(^1\)
- Impacted ability to conduct daily activities, meet basic needs, and secure access to healthcare
- ASH guidance lists Oxbryta as option for patients with symptomatic low Hb levels or who are difficult to transfuse\(^2\)

**Healthcare Providers**
- Adopting telemedicine
- Some not prescribing new therapies without an in-person visit\(^3\)
- Fewer new Oxbryta prescribers added in Q2
- 500-600 field team interactions (mostly virtual) exiting Q2

**Oxbryta Launch**
- Q2 new prescriptions stabilized at ~40% decrease from Q1
- COVID-19 impact continuing in Q3 and potentially longer
- Commercial supply into late 2021
- Continued confidence in Oxbryta’s long-term potential

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ASH, American Society of Hematology; Hb, hemoglobin; SCD, sickle cell disease.


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OXBRYTA IS BEING PRESCRIBED TO A BROAD RANGE OF PATIENTS\(^1\)

**PATIENT CHARACTERISTICS**

- Almost half had baseline Hb >8 g/dL
- More than half on a combination regimen
- Almost half had 3+ VOCs in the prior year
- Used across all ages 12 years of age and older

**PHYSICIANS**

- 40% of prescriptions written by non-specialists\(^2\)

Hb, hemoglobin; VOC, vaso-occlusive crisis.

1. GBT analysis of claims and lab data and chart audit. 2. GBT analysis of enrollments into GBT Source™.

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SCD: AN UNDERSERVED ORPHAN CONDITION
AN URGENT UNMET NEED

~100,000 SCD patients in the United States\(^1\) / ~52,000 patients in Europe\(^2\)

**Lifelong inherited blood disorder**
+ Hb polymerization causes deformation and destruction of red blood cells, leading to:
  - Multi-organ morbidity\(^3\)
  - ~30 years reduction in life expectancy\(^4\)

**Historically limited treatment options**
+ Drug development focused on pain – only one aspect of the disease

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Hb, hemoglobin; SCD, sickle cell disease.


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HbS POLYMERIZATION IS THE ROOT CAUSE OF RBC SICKLING AND DESTRUCTION IN SCD

HbS polymerization

Hemolytic Anemia
- Organ Damage
  + Stroke
  + Renal failure
  + Pulmonary hypertension
  + Priapism
  + Leg ulcers
  + Mortality

Fatigue

Vaso-occlusion
- Organ Damage
  + Osteonecrosis
  + Retinopathy
- Pain / Vaso-occlusive crisis (VOC)

HbS, sickle hemoglobin; RBC, red blood cell; SCD, sickle cell disease.
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MULTI-ORGAN DYSFUNCTION IN SCD IS LINKED TO CHRONIC ANEMIA AND HEMOLYSIS

Chronic Organ Damage: Leading Cause of Death in Adults

- Stroke (13%)
- Unknown (8%)
- Trauma (8%)
- Infection (5%)
- Acute Pulmonary Disorders (11%)
- Stroke (13%)
- Irreversible Organ Damage (42%) (Lung, Kidney, and/or Liver)

≥ 20 years of age, n=186

SCD, sickle cell disease.
MAJORITY OF SCD PATIENTS DO NOT EXPERIENCE VOCs

SCD Patient VOCs per year

9%

24%

15%

52%

SCD, sickle cell disease; VOC, vaso-occlusive crisis.
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SCD PLACES MAJOR BURDEN ON U.S. PATIENTS AND
SOCIETY BEYOND DIRECT MEDICAL COSTS

Up to $286,000 annually in cost of medical care for patients with complications

End-organ damage drives major healthcare utilization, with average patient receiving services 30-54 days/year

~$700,000 in lost lifetime income per patient

Major caregiver productivity impact, often creating devastating financial burden

SCD, sickle cell disease.
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OXBRYTA INHIBITS HbS POLYMERIZATION, THE ROOT CAUSE OF RBC SICKLING AND DESTRUCTION IN SCD

Once-daily, oral treatment

Binding to Hb stabilizes the oxyHb (R) state\(^1\)

Which safely increases oxygen affinity to create a fraction of non-sickling Hb\(^2\)

Which inhibits HbS polymerization\(^3\)

Hb, hemoglobin; HbS, sickle hemoglobin; oxyHb, oxygenated hemoglobin; RBC, red blood cell; SCD, sickle cell disease.


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OXBRYTA IMPACT ON RED BLOOD CELLS SEEN AFTER ONLY TWO WEEKS OF TREATMENT

Before Oxbryta
Hb ~6.5 g/dL

After Oxbryta (~2 weeks)
Hb 7.6 g/dL

Hb, hemoglobin.

28-year-old woman with sickle cell anemia (HbSS) and infrequent pain crises (VOCs) enrolled in expanded access program. Received hydroxyurea for many years without improvement in hemolysis or anemia and a fetal hemoglobin (HbF) level of 6%. Hb increased by 1.1 g/dL with concordant reductions in bilirubin and lactate dehydrogenase (LDH).

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OXBRYTA ACHIEVED RAPID, ROBUST AND SUSTAINED IMPROVEMENT IN Hb AND HEMOLYSIS

Pivotal Phase 3 HOPE Study

+ Hb increase of >1 g/dL in 51.1% of patients
+ Oxbryta was safe and well tolerated
+ Fewer VOCs were observed, with a substantial increase in Hb

“The increase in hemoglobin level and reduction in hemolysis observed with Oxbryta support its use as a new, potentially disease-modifying therapy for SCD.”

– NEJM editorial by Alexis Thompson, M.D., M.P.H.

Hb, hemoglobin; SCD, sickle cell disease; VOC, vaso-occlusive crisis.
Approximately 82% of all randomized patients completed 24 weeks of treatment.
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OXBRYTA U.S. LAUNCH
KEYS TO A SUCCESSFUL LAUNCH

Commercial Organization
- Highly Experienced Team

Stakeholder Education
- Understand the Needs
- Educate Patients, Physicians and Payers

Patient Access
- Support Patients and Access
- Establish Payer Coverage

Our Goal: Transform the SCD Treatment Paradigm
TARGETED AND EFFICIENT LAUNCH UNDERWAY

+ 17 states represent ~85% of SCD patients
+ 75 sickle cell therapeutic specialists targeting nearly 6,000 HCPs
+ 12 medical science liaisons targeting the top 500 KOLs

HCP, healthcare professional; KOL, key opinion leader; SCD, sickle cell disease.
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OXBRYTA HAS POTENTIAL TO ADDRESS TOP TWO PHYSICIAN AND ADVOCATE PRIORITIES

1. Long-term organ damage
   + Sustained improvements in anemia and hemolysis
   + Potential to reduce morbidity (including long-term organ damage and stroke) and mortality

2. Risk of stroke and silent infarct

3. VOCs
   + HOPE Study was not designed to evaluate VOC rate

VOC, vaso-occlusive crisis.
GBT Market Research: physician respondents (n=248) and patient respondents (n=7). © Global Blood Therapeutics, Inc. 2020
90% of physicians said they would prescribe Oxbryta; ~60% within next 5 months

% of physicians that would prescribe Oxbryta
- Total: 95% (n=183)
- Hem Onc: 94% (n=69)
- Pediatric / Hem Onc: 100% (n=14)
- PCP: 94% (n=47)
- Pediatric: 95% (n=41)
- NP / PA: 92% (n=12)

% that would adopt Oxbryta within next 5 months
- Total: ~60% (n=117)
- Hem Onc: 100% (n=14)
- Pediatric / Hem Onc: 94% (n=47)
- PCP: 95% (n=41)
- Pediatric: 92% (n=12)

Top Reasons Cited by HCPs to Prescribe
- Improves Anemia
- Increases Hb Levels
- Efficacy Related to VOCs
- Reduces Hb Polymerization
- Improved O₂ Delivery

Hb, hemoglobin; HCP, healthcare professional; Hem Onc, hematologist-oncologist; PA, physician assistant; PCP, primary care provider; NP, nurse practitioner; VOC, vaso-occlusive crisis.

GBT Market Research (n=183, 117) conducted in June-July 2020. Indicates physician would try in at least one patient.

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WE EXPECT EARLY ADOPTION IN SEVERAL PATIENT TYPES

107K
Diagnosed

103K
4+ Years of Age
(potential U.S. label expansion)

86K
12+ Years of Age
(Oxbryta U.S. label)

58K
12+ Years of Age
Hb ≤ 10.5 g/dL

22K
12+ Years of Age
Hb ≤ 8 g/dL

Hb, hemoglobin.
April 2018 – March 2019 Quest Lab Data, SHA Claims (80% 12 years and older) and internal analysis.
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COMMITTED TO MAXIMIZING PAYER COVERAGE AND ACCESS FOR SCD PATIENTS

SCD U.S. Payer Landscape

- Medicaid: 50%
- Commercial: 32%
- Others: 3%
- Medicare: 15%

Reimbursement Overview

- Expect overall gross-to-net of 25-30% at steady state
- Mandatory 23.1% discount for Medicaid and 340B (~10-15% Commercial/Medicare patients)
- Channel costs of 8-11% (distribution, returns, copay support)
- No price increase for 3 years; thereafter, increases limited to inflation

SCD, sickle cell disease.
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BROAD PAYER COVERAGE BY END OF 2020

Broad Coverage

+ 1H progress: 53% covered lives¹
+ Medicaid FFS: all 17 top states

Q1 – Q2

- Initiation of our national rebate agreement with HHS
- Some medical exceptions while formulary reviews occur
- Patient visits starting but will take time to ramp up

Q3

- Increasing volume as formularies are updated

Exiting Q4

+ Goal: Broad coverage among public and private payers

Limited Coverage

FFS, fee for service; HHS, U.S. Department of Health and Human Services.
1. Payer coverage includes lives covered through medical exceptions
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OXBRYTA DISTRIBUTION DESIGNED FOR HIGH-TOUCH PATIENT CARE AND PAYER COVERAGE

Home Delivery (office, school)

- Disease Education
- Adherence & Refill Support
- Financial & Copay Support
- Reimbursement Assistance
- Product & Services Education
- Specialty Pharmacy Network
- Payers
- HCPs
- Rx

HCP, healthcare professional; Rx, prescription; SCD, sickle cell disease. © Global Blood Therapeutics, Inc. 2020
LOOKING AHEAD
PLANS TO EXPAND ACCESS FOR PATIENTS IN U.S., EUROPE AND MIDDLE EAST

Expanding U.S. FDA Label

+ Plan to submit NDA to FDA by mid-2021
  - Accelerated approval pathway submission to treat children as young as 4 years of age
+ Potential to treat additional ~17K patients¹
+ New age appropriate formulation designed for pediatric population

Europe Regulatory and Market Strategy

+ Plan to submit MAA to EMA by mid-2021
  - Full marketing authorization pathway submission to treat hemolytic anemia in SCD patients 12+ years of age
+ ~52K SCD patients in the region²
+ Targeting countries where vast majority of European SCD patients live

Middle East Distribution Partner

+ Agreement to distribute Oxbryta in Bahrain, Kuwait, Oman, Qatar, Saudi Arabia and the United Arab Emirates
+ ~100K SCD patients 12+ years of age in the region¹
+ Named Patient Pathways may provide early access to Oxbryta while health authorities conduct reviews

EMA, European Medicines Agency; FDA, Food & Drug Administration; MMA, marketing authorization application; NDA, new drug application; SCD, sickle cell disease.
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HOPE-KIDS 2: TCD CONFIRMATORY STUDY UNDERWAY

STUDY POPULATION

+ N=224
+ Age (2-15)
+ Conditional TCD flow velocity (170-199 cm/s, elevated stroke risk)
+ Sites in U.S., Europe and Africa

Primary Endpoint

+ Mean change in TCD at 24 weeks

Secondary Endpoints

+ Conversion to normal or abnormal TCD at 96 weeks
+ Change in Hb over time and clinical measures of hemolysis

Once-daily, oral dosing

Oxbryta 1500 mg¹
N=112

Placebo
N=112

24-week primary analysis treatment period

96-week total treatment period

Hb, hemoglobin; HU, hydroxyurea; TCD, transcranial Doppler.
¹ Or weight-based equivalent.
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ADDITIONAL OXBRYTA STUDIES: DEMONSTRATING EFFECT ON MULTIPLE ORGANS

Planned Sponsored and Investigator-Initiated Studies of Oxbryta (voxelotor)

Investigator Initiated:

+ Pilot Study of Voxelotor for SCD Patients at Highest Risk for Progression of Chronic Kidney Disease
+ Open Label Study to Evaluate Hemolysis and Organ Damage in Adult SCD Patients Treated with Voxelotor (Brain/Cardiac/Kidney)
+ The Effect of Voxelotor on Cerebral Perfusion and Oxygenation (Voxelotor-MRI Study)

GBT-Sponsored:

+ Actigraphy Improvement with Voxelotor (ActIVe): Ph4 Study to Evaluate Effect of Voxelotor on Physical Activity in Adolescents and Adults with SCD

Planned Investigator-Initiated Studies of Neurological Complications of SCD

+ The Epidemiology of Silent and Overt Strokes in Adults with SCD: a Prospective Cohort Study
+ Nigeria Collaborative Research Proposal: Young adults with SCD – prospective cohort study to estimate the prevalence and short term incidence of neurological morbidity

Organ Damage in SCD Patients Related to Hemolytic Anemia

Brain
Stroke
Silent cerebral infarct
Neurocognitive impairment

Heart
Cardiomyopathy

Liver/gallbladder
Hepatopathy
Gallstones

Kidney
Renal insufficiency
Renal failure

Skin
Leg ulcers

Lungs
Pulmonary hypertension

GU
Priapism

MRI, magnetic resonance imaging; SCD, sickle cell disease.
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SCD DEVELOPMENT PIPELINE TARGETING MULTIPLE PATHOLOGIES

HbS Polymerization
- Direct HbS inhibitors

HbS Polymerization
- HbF Induction

Vascular Inflammation
- Cellular Adhesion / Occlusion
  - Inclacumab

HbF, fetal hemoglobin; HbS, sickle hemoglobin; SCD, sickle cell disease.
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INCLACUMAB: IN DEVELOPMENT FOR PATIENTS WITH FREQUENT PAIN CRISSES

+ P-selectin inhibition is clinically validated to reduce VOCs
  - A novel fully human monoclonal antibody
  - Potential for less frequent dosing
  - Established PK, safety and tolerability in more than 500 patients

+ Exclusive worldwide licensing agreement with Roche

+ Manufacturing underway

+ Initiation of pivotal study anticipated in 1H 2021
WORKING TO IMPROVE CARE FOR SCD PATIENTS AROUND THE WORLD

Global Public Health Problem

Thoughtful and Sustainable Approach

- Execution of Europe strategy
- Established distributor partnership to provide access in select Middle East countries
- Potential partnership and distribution opportunity in Latin America
- Exploring ways to distribute and fund Oxbryta in sub-Saharan Africa and India


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Reached agreement with FDA on design of TCD post-approval confirmatory study

UPCOMING MILESTONES

1Q 2020
First patient enrolled in dose optimization study

2Q 2020
Update on Oxbryta pediatric label expansion in United States

2Q 2020
Update on Oxbryta go-forward plan in Europe

TBD
First patients enrolled in HOPE-KIDS 2 and ActIVe Studies

4Q 2020
 Broad payer coverage for Oxbryta

1H 2021
Initiate inclacumab pivotal study

Oxbryta U.S. Launch
Thank You