Providing Hope to the Underserved

March 3, 2021
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, strategy, positioning and future activities, achievements and impact, 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 standard of care and disease-modifying therapy, to address top priorities, transforming the treatment paradigm, and the significance of reducing hemolysis and increasing hemoglobin, Oxbryta awareness and education, the impact of the COVID-19 pandemic and our related response and expectations, the commercial supply of Oxbryta, the availability, use and impact of GBT Source®, payer coverage, implementing and completing clinical development plans, generating and reporting data and analyses from past, ongoing and potential future studies, inferences drawn from studies and related analyses, regulatory review, our manufacturing and commercial infrastructure, our R&D pipeline, the attributes, potential and future development of drug candidates, 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, 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 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 are continuing to establish our commercialization capabilities and may not be able to successfully commercialize Oxbryta, risks associated with our dependence on third parties for development, manufacture, distribution 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 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, 2020, 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.
LIVING OUR MISSION

GBT discovers, develops and delivers life-changing treatments for people living with grievous blood-based disorders, starting with sickle cell disease (SCD).
OUR LONG-TERM VISION

1. Establish Oxbryta as Standard of Care
   - Strategic Focus

2. Advance SCD Pipeline
   - Leverage Capabilities to Expand Beyond SCD

3. Leader in SCD and Other Underserved Orphan Disease Communities

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FOCUS ON NEAR-TERM GROWTH

Where We Are Today

Successful Oxbryta launch, despite COVID-19 pandemic

Advancing pipeline of potential best-in-class SCD therapies

Solid balance sheet with $560.9M¹

Our Near-Term Goals

Build on Oxbryta momentum with labeling and geographic expansion

Initiate 2 inclacumab pivotal trials mid-2021
Deliver GBT601 POC data by year end

Pursue investment opportunities to drive growth

SCD, sickle cell disease; POC, proof of concept.
¹ Cash, cash equivalents, and marketable securities as of December 31, 2020.
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SCD: AN UNDERSERVED ORPHAN CONDITION
SCD: AN URGENT UNMET NEED

Lifelong inherited blood disorder
Hb polymerization causes / leads to:
Multi-organ morbidity¹
~30 year reduction in life expectancy²

Historically limited treatment options
Drug development was focused on acute pain crisis (VOCs), which impact less than 50% of the patients³

Underserved patient population
>350K patients in U.S., Europe, Middle East and Latin America⁴
Millions worldwide, including low-resource countries

Hb, hemoglobin; VOC, vaso-occlusive crisis.

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HbS POLYMERIZATION IS THE ROOT PROBLEM IN SCD

- HbS, sickle hemoglobin.
- © Global Blood Therapeutics, Inc. 2021
MULTI-ORGAN DYSFUNCTION IN SCD IS LINKED TO CHRONIC ANEMIA AND HEMOLYSIS

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MAJOR BURDEN ON U.S. PATIENTS AND SOCIETY

Up to $286,000 annually in cost of medical care

End-organ damage drives major healthcare utilization, average of 30-54 days/year

~$700,000 in lost lifetime income per patient

Major caregiver productivity impact; often devastating financial burden

OXBRYTA: FIRST-IN-CLASS SCD THERAPY
OXBRYTA ATTACKS THE ROOT CAUSE OF SCD

- Once-daily, oral treatment
- Binding to Hb stabilizes the oxyHb (R) state
- Increases oxygen affinity safely to create non-sickling Hb
- Inhibits HbS polymerization

oxyHb, oxygenated hemoglobin; Hb, hemoglobin; HbS, sickle hemoglobin.
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OXBRYTA IMPACTS RBCs RAPIDLY

Pre-Treatment

Day 21 of Treatment

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HOPE STUDY: DURABLE IMPROVEMENTS AT 72 WEEKS

Nearly 90% of Patients Achieve Significant Hb Increase (>1 g/dl)

Source: Long-Term Efficacy and Safety of Voxelotor in Adolescents and Adults with Sickle Cell Disease: HOPE Trial 72-Week Analysis, ASH 2020 Poster #1716
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PATIENTS AND HCPs REPORT PATIENT IMPROVEMENT – AT VARYING LEVELS OF Hb INCREASE

Patient and Clinician Assessment Improvement with Oxbryta Therapy

Hemoglobin Change and Clinical Improvement

1. The remaining 19% of patients measured rated as a little improved or no change (one patient of 0-1 g/dL improvement and 2 patients with >1 g/dL improvement).

Source: Patient Perception of Oxbryta Treatment Benefit, ASH 2020 Poster #1723.

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VOCs LOWEST IN PATIENTS ACHIEVING HIGHEST Hb LEVELS

MCF, mean cumulative function; HU, hydroxyurea.
Summary excludes VOC events after treatment discontinuation and events after HU initiation post randomization for patients with no HU use at baseline. Summary excludes patients without post-baseline Hb lab assessment. Hb values are as observed based on assessments collected through the end of the week 72 visit window. Hb values collected after treatment discontinuation (for patients with last dose prior to the week 72 visit window), after withdrawal of consent, after study discontinuation, and after HU initiation post randomization for patients with no HU use at baseline were excluded.

Source: Higher Hemoglobin Levels Achieved with Voselotor Are Associated with Lower Vaso-Occlusive Crisis Incidence: 72-Week Analysis from the HOPE Study. ASH 2020 Poster #795.

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DEMONSTRATING CLINICAL BENEFIT IN LEG ULCERS

Post-Hoc Analysis of HOPE Study
Published in American Journal of Hematology

>90% voxelotor patients had leg ulcer improvement/resolution by week 72

100% voxelotor 1500 mg patients had leg ulcer resolution by week 72

75% voxelotor 1500 mg patients had leg ulcer resolution by week 24

% Patients with Leg Ulcers that Resolved or Improved by Week 24 & 72

1. The American Journal of Hematology post hoc analysis evaluating the incidence of leg ulcers and outcomes in patients enrolled in the HOPE trial across the 72-week treatment period. Published January 2021.
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OXBRYTA CAN IMPROVE SCD PATIENT LIVES

“I feel like I’m more able to be the parent and the person that I want to be.”
Lakesha D.

“I feel like I’m more able to help take care of my kids and my family.”
Muyiwa S.

“I feel like I’m able to do more with my family and friends.”
Michelle P.

Individual patient results may vary.
First-in-class disease modifying therapy approved to treat SCD patients ages 12+

- ~5,000 new prescriptions\(^1\)
- ~1,365 unique prescribers\(^1\)
- ~90% of covered lives, broad payer coverage
- $123.8M 2020 revenue

Launch progress despite headwinds from COVID-19

FDA approval on November 25, 2019. As of December 31, 2020 unless otherwise stated.
1. From launch through December 31, 2020.
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TARGETING HCPs AND KOLs

17 states represent ~85% of SCD patients

~60 sickle cell therapeutic specialists targeting ~5,000 HCPs

12 medical science liaisons targeting the top 500 KOLs

KOL, key opinion leader.
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ADAPTING TO COVID-19 ENVIRONMENT

COVID-19 Impact is Real

SCD patients at increased risk of severe illness and death from COVID-19

HCP/patient interactions down significantly from pre-pandemic averages

Industry-wide, HCPs often less comfortable initiating new therapies virtually

Significantly reduced in-person field engagements

Direct correlation between COVID-19 cases and new Rx’s of Oxbryta

How GBT is Adapting

Encouraging increased telemedicine adoption

Enhancing capabilities for virtual field engagements

Executing in-person field engagements, wherever appropriate

Increasing HCP/patient education and real-world evidence

Augmenting patient support services and patient communication

Rx, prescription.
1. Centers for Disease Control and Prevention (CDC)
© Global Blood Therapeutics, Inc. 2021
GBT SOURCE PROVIDES HIGH-TOUCH PATIENT SUPPORT

- Patient
- HCPs
- Disease Education
- Reimbursement Assistance
- Financial & Copay Support
- Adherence & Refill Support
- Product & Services Education
- Home Delivery (office, school)
- Specialty Pharmacy Network
- Payers

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ENHANCING STRATEGIES TO DRIVE INCREASED ADOPTION

- **High-touch patient engagement**
- Nurse support on adherence
- Weekly updates for HCPs

- **Reimbursement support**
- Patient follow-ups and reminders
- HCP and patient education

- **Resource for patients**
- Schedule refills
- Access to pharmacist

- **HCP engagement**
- New education, marketing materials

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DEPLOYING NEW AND UPDATED EDUCATION MATERIALS

WHAT YOU WILL FIND IN YOUR JOURNAL

Inside your journal there are different sections to help you through your treatment:

Planning for success

- Nausea tips (p.5)

- My lab results
  - Simple charts to record your lab results so you’ll know your numbers (p.6)

- Oxbryta™ monthly journal
  - Monthly exercises to track your progress on oxbryta (p.13)

- Questions for my healthcare provider
  - Room to write down questions you may want to ask your healthcare provider (p.14-15)

- My upcoming appointments
  - A place to keep track of upcoming appointments

WHAT IS A TREATMENT JOURNAL?

Your treatment journal can help you track your progress, monitor your health, and review any information your healthcare provider explains about your medication.

- Preventing your medication from getting lost or stolen
  - Keep this journal with you and review it along with each healthcare appointment.

OXBRYTA MAY DECREASE THE NUMBER OF SICKLED RED BLOOD CELLS

Blood smear shows the different shapes of red blood cells. Oxbryta™ reduces the fraction of sickle cell in red blood cells or a normal patient (left) (A) and in a patient on day 1 (B) (right).

- Before Oxbryta
  - Sickle red blood cells (sickled cell disease)
  - Normal red blood cells (A)
  - Normal red blood cells (B)

- After Oxbryta
  - Fewer sickle red blood cells (C)

SELECTED SAFETY INFORMATION

Before taking Oxbryta, tell your healthcare provider about all of your medical conditions, including:

- If you are pregnant or plan to become pregnant
- If you are breastfeeding or plan to be breastfed

- Nausea
  - Limit caffeine consumption
  - Eat a healthy diet, and reduce your intake of highly processed foods and sugar
  - Maintain a regular sleep schedule and avoid sleeping too little or too much
  - Reduce and manage stress whenever possible
  - Consult your healthcare provider to see if an over-the-counter medicine is okay for you to take

CALL YOUR DOCTOR FOR MEDICAL ADVICE ABOUT SIDE EFFECTS. YOU MAY REPORT SIDE EFFECTS TO FDA AT 1-800-FDA-1088. YOU MAY ALSO REPORT SIDE EFFECTS TO Global Blood Therapeutics at 1-833-425-4966 (1-833-GBT-4YOU).

INDICATION

OXYGENA™

OXBRYTA is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older. It is not known if OXBRYTA is safe and effective in children below 12 years of age. This indication is approved under accelerated approval based on change in hemoglobin HbA₂. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

Please see Important Safety Information on back and full Prescribing Information in pocket.
WE BELIEVE OXBRYTA WILL BECOME STANDARD OF CARE

Opportunity to Reach >350K Patients by 2022

**U.S.**
- Current Oxbryta label
  - 86K Patients Age 12+
  - 17K Age 4-11
- Planning to file regulatory application to expand label to ages 4 to 11

**Latin America**
- 100K Patients
- Seeking to partner with distributor for Brazil

**Europe**
- 52K Patients
- MAA under review to treat hemolytic anemia in SCD patients 12+ years old

**Middle East**
- 100K Patients
- Partnered with distributor for six GCC countries


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ADVANCING THE GBT PIPELINE
## GBT PIPELINE TARGETS SCD VIA MULTIPLE APPROACHES

### Ongoing studies:
- Goal: 1H 2021 Phase 3 initiation (two studies)
  - HOPE-KIDS 1 study
  - HOPE-KIDS 2 TCD post-approval study
  - ActIVe study

### Goals:
- 1H 2021 enter the clinic, POC data by end of 2021

### Programs:

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<th>Program</th>
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<th>Phase 1</th>
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<th>Phase 3</th>
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<td><strong>Oxbryta (voxelotor)</strong></td>
<td>Treatment of SCD</td>
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<td>Commercial</td>
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<tr>
<td>HbS Polymerization Inhibitor</td>
<td>Ongoing studies:</td>
<td>• HOPE-KIDS 1 study</td>
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<td><strong>Inclacumab</strong></td>
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<td>Goal: 1H 2021 Phase 3 initiation (two studies)</td>
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<tr>
<td>P-Selectin Inhibitor</td>
<td>Acute VOC re-admission prevention</td>
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<tr>
<td><strong>GBT601</strong></td>
<td>Treatment of SCD</td>
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<td>Goals: 1H 2021 enter the clinic, POC data by end of 2021</td>
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<tr>
<td>Next-Generation HbS Polymerization Inhibitor</td>
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<td><strong>HbF Induction</strong></td>
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<td>(Syros Partnership)</td>
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POC, proof of concept; HbF, fetal hemoglobin.
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OXBRYTA STUDIES: DEMONSTRATING EFFECT ON MULTIPLE ORGANS

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

Investigator Initiated:
Chronic kidney disease
Organ damage (brain/cardiac/kidney)
Voxelotor-MRI study

GBT-Sponsored:
HOPE-KIDS 2: TCD confirmatory study
Physical activity (ActIVe Ph4 study)

Planned Investigator-Initiated Studies of Neurological Complications of SCD

Stroke epidemiology in adults with SCD
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

Lungs
- Pulmonary hypertension

Skin
- Leg ulcers

GU
- Priapism

MRI, magnetic resonance imaging; TCD, transcranial doppler.
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INCLACUMAB: BEST-IN-CLASS POTENTIAL FOR VOCs

Goal: Quarterly infusion dosing

Encouraging safety
(>700 patients in non-SCD studies)

VOC, vaso-occlusive crisis.
Source: Inclacumab, a Fully Human Anti-P-selectin Antibody, Directly Binds to PSGL-1 Binding Region and Demonstrates Robust and Durable Inhibition of Cell Adhesion. ASH 2020 Poster #1707.
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P-selectin
Binding site on P-selectin

PSGL-1
Inclacumab
Crizanlizumab

Inclacumab more closely mimics the natural binding site on P-selectin

Patients with SCD

Concentration, µg/mL
PLA inhibition, %

Inclacumab
Crizanlizumab
VISION IS TO OPTIMIZE PATIENT OPTIONS AND INCREASE ADDRESSABLE MARKET

**Goals:**
- Improve health
- Maintain daily activities
- Reduce healthcare utilization & cost

**SCD is the leading cause of 30-Day re-admission rates**

**Home**

- Quarterly Dosing
  - Lower VOC frequency

**Physician Office**

**Hospital (Re-)Admission**

- On-Demand Dosing
  - Reduce re-admissions

1. SCD readmissions / number of index stays: Elixhauser A and Steiner C, HCUP Statistical Brief #153, April 2013.
Source: Inclacumab, a Fully Human Anti-P-selectin Antibody, Directly Binds to PSGL-1 Binding Region and Demonstrates Robust and Durable Inhibition of Cell Adhesion. ASH 2020 Poster #1707.
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PHASE 3 PROGRAM WILL STUDY THE VOC CONTINUUM

**Chronic Prevention Protocol**

**N = 240**

Primary Endpoint: VOC rate during 48-week treatment period

**Acute Re-Admission Protocol**

**N = 280**

Primary Endpoint: Proportion of participants with at least 1 re-admission for VOC within 90 days of hospitalization for VOC

Open-Label Extension Protocol

VOC, vaso-occlusive crisis.

Source: Inclacumab, a Fully Human Anti-P-selectin Antibody, Directly Binds to PSGL-1 Binding Region and Demonstrates Robust and Durable Inhibition of Cell Adhesion. ASH 2020 Poster #1707.

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Potential benefits include:

- Normalized Hb
- Improved RBC survival, health and organ function
- One pill per day
- Functional cure as single agent

Goal to advance into the clinic with SCD patients by 1H 2021

HbS, sickle hemoglobin; RBC, red blood cell.

Source: GBT021601 Inhibits HbS Polymerization, Prevents RBC Sickling and Improves the Pathophysiology of Sickle Cell Disease in a Murine Model. ASH 2020 Poster #1704.

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THOUGHTFUL & SUSTAINABLE APPROACH TO ACHIEVING WORLDWIDE ACCESS OVER TIME

- **Execute** on U.S. launch of Oxbryta
- **Expand** U.S. label and secure ex-U.S. approvals
- **Advance** pipeline and continue investing in innovation
- **Develop** distribution and funding approaches in sub-Saharan Africa and India
UPCOMING MILESTONES

2021¹
First patients enrolled in HOPE-KIDS 2 & ActIVe Studies

By Mid 2021
File regulatory application with FDA to expand Oxbryta label to ages 4 to 11

By Mid 2021
Initiate inclacumab pivotal studies & GBT601 clinical trial in SCD

Late 2021
Deliver GBT601 POC data

Q1-Q2 2022
MAA approval from EMA for Oxbryta for ages 12 and up

2021-2023²
Oxbryta Middle East approvals

Oxbryta U.S. Commercialization

FDA, Food & Drug Administration; POC, proof of concept; MAA, marketing authorization application; EMA, European Medicines Agency.
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OUR LONG-TERM VISION

1. Establish Oxbryta as SOC
   - More-real world experience
   - Label expansion
   - Global launches
   - Access in low resource countries

2. Advance SCD Pipeline
   - Inclacumab
   - GBT601
   - HbF inducers
   - Novel targets

3. Leverage Capabilities to Expand Beyond SCD
   - Benign hematology
   - Orphan diseases

Leader in SCD and Other Underserved Orphan Disease Communities

SOC, standard of care.
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Thank You