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).
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 $560.9M

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.


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SCD: AN URGENT UNMET NEED

Lifelong inherited blood disorder

Hb polymerization causes / leads to:
Multi-organ morbidity\(^1\)

~30 year reduction in life expectancy\(^2\)

Historically limited treatment options

Drug development was focused on acute pain crisis (VOCs), which impact less than 50% of the patients\(^3\)

Underserved patient population

>350K patients in U.S., Europe, Middle East and Latin America\(^4\)

Millions worldwide, including low-resource countries

---

Hb, hemoglobin; VOC, vaso-occlusive crisis.


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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 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

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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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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 Voxelotor Are Associated with Lower Vaso-Occlusive Crisis Incidence: 72-Week Analysis from the HOPE Study. ASH 2020 Poster #795.

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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.

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<table>
<thead>
<tr>
<th>Key Milestone</th>
<th>Number</th>
</tr>
</thead>
<tbody>
<tr>
<td>New Prescriptions</td>
<td>~5,000</td>
</tr>
<tr>
<td>Unique Prescribers</td>
<td>~1,365</td>
</tr>
<tr>
<td>Covered Lives (90%)</td>
<td>~90%</td>
</tr>
<tr>
<td>2020 Revenue</td>
<td>$123.8M</td>
</tr>
</tbody>
</table>

**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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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)
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ENHANCING STRATEGIES TO DRIVE INCREASED ADOPTION

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

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

- **Specialty Pharmacy Network**
  - Resource for patients
  - Schedule refills
  - Access to pharmacist

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

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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

= 10K patients


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## GBT PIPELINE TARGETS SCD VIA MULTIPLE APPROACHES

<table>
<thead>
<tr>
<th>Program</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Commercial</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Oxbryta (voxelotor)</strong></td>
<td>Treatment of SCD</td>
<td></td>
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<tr>
<td>HbS Polymerization Inhibitor</td>
<td>Ongoing studies:</td>
<td>• HOPE-KIDS 1 study</td>
<td>• HOPE-KIDS 2 TCD post-approval study</td>
<td>• ActIve study</td>
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<tr>
<td><strong>Inclacumab</strong></td>
<td>Chronic VOC prevention</td>
<td></td>
<td></td>
<td></td>
<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>
<td></td>
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<tr>
<td><strong>GBT601</strong></td>
<td>Treatment of SCD</td>
<td></td>
<td></td>
<td></td>
<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. © Global Blood Therapeutics, Inc. 2021
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

OXBRYTA STUDIES: DEMONSTRATING EFFECT ON MULTIPLE ORGANS

Organ Damage in SCD Patients Related to Hemolytic Anemia

- **Brain**
  - Stroke
  - Silent cerebral infarct
  - Neurocognitive impairment

- **Heart**
  - Cardiomyopathy

- **Lungs**
  - Pulmonary hypertension

- **Kidney**
  - Renal insufficiency
  - Renal failure

- **Liver/gallbladder**
  - Hepatopathy
  - Gallstones

- **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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VISION IS TO OPTIMIZE PATIENT OPTIONS AND INCREASE ADDRESSABLE MARKET

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

**Quarterly Dosing**
- Lower VOC frequency

**On-Demand Dosing**
- Reduce re-admissions

**Hospital (Re-)Admission**
- SCD is the leading cause of 30-Day re-admission rates

---

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.
© Global Blood Therapeutics, Inc. 2021
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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## UPCOMING MILESTONES

<table>
<thead>
<tr>
<th>Year</th>
<th>Event Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>2021¹</td>
<td>First patients enrolled in HOPE-KIDS 2 &amp; ActIVe Studies</td>
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<tr>
<td>By Mid 2021</td>
<td>File regulatory application with FDA to expand Oxbryta label to ages 4 to 11</td>
</tr>
<tr>
<td>By Mid 2021</td>
<td>Initiate inclacumab pivotal studies &amp; GBT601 clinical trial in SCD</td>
</tr>
<tr>
<td>Late 2021</td>
<td>Deliver GBT601 POC data</td>
</tr>
<tr>
<td>Q1-Q2 2022</td>
<td>MAA approval from EMA for Oxbryta for ages 12 and up</td>
</tr>
<tr>
<td>2021-2023²</td>
<td>Oxbryta Middle East approvals</td>
</tr>
</tbody>
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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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