Providing Hope to the Underserved

May 12, 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, 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, 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 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 certain development, manufacture, distribution and commercialization activities, 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, 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 collaborations, along with those risks set forth in our Annual Report on Form 10-K for the fiscal year ended December 31, 2020, 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.
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
2. Advance SCD Pipeline
3. Leverage Capabilities to Expand Beyond SCD

Leader in SCD and Other Underserved Orphan Disease Communities
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 $482.0M¹

Our Near-Term Goals

- Build on Oxbryta momentum with labeling and geographic expansions
- Initiate 2 inclacumab pivotal trials by mid-2021
- Deliver GBT601 POC data by end of 2021
- Pursue further opportunities to drive growth

SCD, sickle cell disease; POC, proof of concept.
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NEW OXBRYTA AND PIPELINE DATA AT EHA 2021

Includes Phase 2a HOPE-KIDS 1 Study data in children with SCD ages 4 to 11 years treated with Oxbryta

<table>
<thead>
<tr>
<th>Abstract Number</th>
<th>Abstract Title</th>
<th>Presenter / First Author</th>
</tr>
</thead>
<tbody>
<tr>
<td>S260 (oral)</td>
<td>Safety and Efficacy of Voxelotor in Pediatric Patients with Sickle Cell Disease Aged 4-11 Years</td>
<td>Clark Brown</td>
</tr>
<tr>
<td>EP1209 (e-poster)</td>
<td>Real-World Experience of Voxelotor for the Treatment of Patients with Sickle Cell Disease – A Single-Center Study</td>
<td>Alan Anderson</td>
</tr>
<tr>
<td>EP1206 (e-poster)</td>
<td>Real-World Experience of Patients with Sickle Cell Disease Treated with Voxelotor: A Multicenter, Retrospective Study</td>
<td>Biree Andemariam</td>
</tr>
<tr>
<td>PB1769 (abstract book)</td>
<td>Pediatric Patient Reported Outcomes in Patients Receiving Voxelotor for Sickle Cell Disease</td>
<td>Clark Brown</td>
</tr>
</tbody>
</table>

**HOPE-KIDS 1 Key Results (n=45)**

- 47% achieved Hb increase >1 g/dL at 24 weeks
- Reductions in markers of hemolysis
- Well tolerated, no new adverse safety signals detected
- Most common treatment-related AEs: diarrhea (11%), vomiting (11%) & rash (11%)
- Results were consistent with the Phase 3 HOPE Study

Abstracts available on EHA website: [https://ehaweb.org](https://ehaweb.org)

Oral presentation (on demand video) and e-posters available on EHA website on Friday, June 11 at 3:00 am EDT

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EHA, European Hematology Association; SCD, sickle cell disease; Hb, hemoglobin; AEs, adverse events.

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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.

HbS POLYMERIZATION IS THE ROOT PROBLEM IN SCD

**HbS Polymerization**

- **Hemolytic Anemia**
  - Stroke
  - Renal Failure
  - Pulmonary Hypertension
  - Priapism
  - Leg Ulcers
  - Mortality

- **Fatigue**

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

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MULTI-ORGAN DYSFUNCTION IN SCD IS LINKED TO CHRONIC ANEMIA AND HEMOLYSIS

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

Chronic Organ Damage: Leading Cause of Death in Adults

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

≥ 20 years of age, n=186

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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

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Nearly 90% of Patients Achieve Significant Hb Increase (>1 g/dl)
RWE CONSISTENT WITH HOPE STUDY 72-WEEK RESULTS AND DEMONSTRATING ADDITIONAL BENEFITS

**ASH 2020: Symphony Health Claims Analysis (n=1,275)**

- Increased mean Hb by ~1 g/dL
- Statistically significant reduction in:
  - Annualized transfusion rates
  - VOCs

**ASPHO 2021: Single Center Experience (n=76)**

- Increased mean Hb by 2.0 g/dL
- Decreased reticulocytes from 11.6% to 6.5%
- Decreased total bilirubin by 1.4 mg/dL
- PGI-C & CGI-C in most patients were assessed as very much improved or much improved
- Adverse events were rare (3) and resolved with dose modification

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RWE, real-world evidence; VOC, vaso-occlusive crises; PGI-C, patient global impression of change; CGI-C, clinical global impression of change.
ASH 2020 Poster: #2627, Real-World Effectiveness of Voxelotor for Treating Sickle Cell Disease in the U.S.
ASPHO 2021 Poster: Real-World Experience of Pediatric Patients With Sickle Cell Disease Treated With Oxbryta.
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PATIENTS AND HCPs REPORT PATIENT IMPROVEMENT – AT VARYING LEVELS OF Hb INCREASE

### Patient and Clinician Assessment

**Improvement with Oxbryta Therapy**

<table>
<thead>
<tr>
<th>Hemoglobin Change</th>
<th>Greatly Improved</th>
<th>Improved</th>
<th>A Little Improved</th>
<th>No Change</th>
<th>A Little Worse</th>
<th>Much Worse</th>
</tr>
</thead>
<tbody>
<tr>
<td>% Patients</td>
<td></td>
<td></td>
<td></td>
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</tbody>
</table>

**Percentage of Patients**

- Greatly Improved: 60%
- Improved: 50%
- A Little Improved: 40%
- No Change: 30%
- A Little Worse: 20%
- Much Worse: 10%

**Hemoglobin Change and Clinical Improvement**

<table>
<thead>
<tr>
<th>Hemoglobin Level</th>
<th>Greatly Improved or Improved¹</th>
<th>% Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>&lt; 0 g/dL</td>
<td>0%</td>
<td></td>
</tr>
<tr>
<td>0-1 g/dL</td>
<td>10%</td>
<td></td>
</tr>
<tr>
<td>&gt; 1 g/dL</td>
<td>60%</td>
<td></td>
</tr>
</tbody>
</table>

¹The 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 Voxelotor 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

Source: The American Journal of Hematology post hoc analysis valuating 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.

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First-in-class disease modifying therapy approved to treat SCD patients ages 12+

Nearly 5,900 new prescriptions\(^1\)

~1,550 unique prescribers\(^1\)

>90% of covered lives, broad payer coverage\(^2\)

~$165M LTD revenue\(^1\)

Net increase in patients taking Oxbryta each quarter since launch

FDA approval on November 25, 2019. LTD, launch-to-date.
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OXBRYTA PAYER COVERAGE AND GROSS-TO-NET

U.S. SCD Payer Landscape

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

~65% of Oxbryta patients are on government-sponsored plans

Reimbursement Overview

- Channel costs of 8-11% (distribution, returns, copay support)
- Mandatory 23.1% discount for Medicaid and 340B (~10-15% Commercial/Medicare patients)
- Q1 2021 gross-to-net of 15%
- At steady state expect gross-to-net to be on the lower end of 25-30%


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GBT SOURCE PROVIDES HIGH-TOUCH PATIENT SUPPORT

- Home Delivery (office, school)
  - Patient
  - HCPs
  - Disease Education
  - Product & Services Education
  - Adherence & Refill Support
  - Reimbursement Assistance
  - Financial & Copay Support
  - Specialty Pharmacy Network
  - Payers
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-19¹
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
Increased COVID-19 cases impact HCP visits and new Rx's of Oxbryta in certain geographies

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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NEW Rx’s ARE IMPACTED AS COVID-19 CASES INCREASE

NJ, California and Texas continue to show an outsized impact from the pandemic

New Jersey

California

Texas

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New Prescriptions

Conversion rate ~75%
Consistent with analogs

New Patient Starts

Annual adherence (first year)
Well within 50 – 70% range of analogs

On Therapy

Phasing & distributor inventory
Q1 inventory levels:
Decreased by ~4 days from Q4
Higher than early launch levels

Bottles Shipped to Distributor & Revenue

Data as of March 31, 2021
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ENHANCED STRATEGIES TO DRIVE INCREASED ADOPTION AND ADHERENCE

High-touch patient engagement
Nurse support on adherence
Launched GBTSource.com

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

Resource for patients
Access to pharmacist
Schedule refills

New education, marketing materials
New starter kits
In-person HCP engagements where possible
AWARENESS AND SATISFACTION CONTINUES TO INCREASE

**Awareness**
- ~95% Specialist aided awareness of Oxbryta
- Nearly 100% % of HCPs aware of Oxbryta that have or would prescribe it in the future

**Perception**
- 71% % of prescribers extremely satisfied with Oxbryta
- 84% % of current Oxbryta patients who say it works extremely well
- 94% % of current Oxbryta users extremely or very likely to recommend it to others

Data as of March 31, 2021
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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


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ADVANCING THE GBT PIPELINE
## 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>
<th>Ongoing Studies</th>
</tr>
</thead>
</table>
| **Oxbryta (voxelotor)**  
MAA accepted for review Jan. '21 | Include:  
• HOPE-KIDS 1 study  
• HOPE-KIDS 2 TCD post-approval study  
• ActIVe study |
| **Inclacumab**  
P-Selectin Inhibitor | Chronic VOC prevention |  |  |  | Goal: Mid-'21 Phase 3 initiation (two studies) |
| **GBT601**  
Next-Generation HbS Polymerization Inhibitor | Treatment of SCD |  |  |  | Goals: 1H21 enter the clinic, POC data by end of 2021 |
| **HbF Induction**  
(Syros Partnership) |  |  |  |  |  |
| **Anti-sickling**  
(Sanofi In-license) |  |  |  |  |  |
| **Inflammation & Oxidative Stress Reduction**  
(Sanofi In-license) |  |  |  |  |  |

SCD, sickle cell disease; FDA, Food and Drug Administration; MAA, marketing authorization application; TCD, transcranial doppler; VOC, vaso-occlusive crisis; POC, proof of concept; HbF, fetal hemoglobin.

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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 (enrolling now)
- Physical activity (ActIVe Ph4 study) (enrolling now)

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
- Kidney: Renal insufficiency, Renal failure
- Lungs: Pulmonary hypertension
- 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)

- P-selectin
- Binding site on P-selectin

PSGL-1  Inclacumab  Crizanlizumab

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

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 POTENTIAL ADDRESSABLE MARKET

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

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

Lower VOC frequency

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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Next-Generation HbS Polymerization Inhibitor

Potential benefits include:

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

Goal to deliver proof-of-concept data in SCD patients by year-end 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
First patients enrolled in HOPE-KIDS 2 & ActIVe Studies

File with FDA to expand Oxbryta label to ages 4 to 11

Initiate inclacumab pivotal studies & GBT601 clinical trial in SCD

Deliver GBT601 POC data

MAA approval from EMA for Oxbryta for ages 12 and up

Oxbryta Middle East approvals

UPCOMING MILESTONES

Oxbryta U.S. Commercialization

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

1. Projected approvals.

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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