Providing Hope to the Underserved

November 5, 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 and other product 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, 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

$84.6M Revenue

4,000 New Prescriptions\(^1\)

1,150 Unique Prescribers\(^1\)

$535.2 Million cash, cash equivalents and marketable securities (at 9/30/20)

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

90% Broad Payer Coverage\(^2\)
(lives covered at 9/30/20)

- 87% Commercial
- 100% Medicaid
- 86% Medicare

- Fee-for-service Medicaid coverage in 44 states, including all 17 priority states
- Achieved broad coverage one quarter ahead of goal

1. Numbers of new patient prescriptions and unique prescribers are approximate. 2. Payer coverage includes lives covered through verified patient adjudication.

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ONGOING IMPACT OF COVID-19

**SCD Patients**
- Increased risk of severe illness and/or death from COVID-19\(^1\)
- Impacted daily activities and ability to access healthcare
- Average quarterly visits with HCPs down from 1.1 pre-pandemic to 0.8 in Q3\(^2\)

**Healthcare Providers**
- Adopting telemedicine
- Most offices open to virtual engagements with reps
- In-person engagements with reps in Q3 started to return, accounting for ~1/3 of engagements by the end of the quarter

**Oxbryta Launch**
- Q3 new prescriptions up slightly from Q2
- Some regional successes in top SCD states (NY, NJ, CA)
- COVID-19 impact continuing in Q4 and potentially into 2021
- Continued confidence in Oxbryta’s long-term potential

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ASH, American Society of Hematology; Hb, hemoglobin SCD, sickle cell disease.
OXBRYTA IS BEING PRESCRIBED TO A BROAD RANGE OF PATIENTS¹

PATIENT CHARACTERISTICS

+ Almost half had baseline Hb >8 g/dL
+ ~one-third on a combination regimen
+ Almost half had 3+ VOCs in prior year
+ Used across all ages 12 years and older

PHYSICIANS

+ 40% of prescriptions written by non-specialists²

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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# NEW OXBRYTA AND PIPELINE DATA AT ASH 2020

Abstracts Include 72-Week Analysis of Phase 3 HOPE Study Supporting Long-Term Use of Oxbryta and Preclinical Data Highlighting Promise of GBT’s Sickle Cell Disease Pipeline

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SCD: AN UNDERSERVED ORPHAN CONDITION
AN URGENT UNMET NEED

~100,000 SCD patients in the United States¹ / ~52,000 patients in Europe² / >100,000 patients in GCC region³

Lifelong inherited blood disorder

+ Hb polymerization causes deformation and destruction of red blood cells, leading to:
  - Multi-organ morbidity³
  - ~30 years reduction in life expectancy⁴

Historically limited treatment options

+ Drug development focused on pain – only one aspect of the disease

Deej, age 14

Hb, hemoglobin; SCD, sickle cell disease; GCC, Gulf Cooperation Council region (Bahrain, Kuwait, Oman, Qatar, Saudi Arabia, and the United Arab Emirates)

3. Data on file

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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

SCD, sickle cell disease.
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Brain
- Stroke
- Silent cerebral infarct
- Neurocognitive impairment

Lungs
- Pulmonary hypertension

Kidney
- Renal insufficiency
- Renal failure

Liver/gallbladder
- Hepatopathy
- Gallstones

Heart
- Cardiomyopathy

Skin
- Leg ulcers

GU
- Priapism

Chronic Organ Damage: Leading Cause of Death in Adults2

≥ 20 years of age, n=186

Other (13%)
Irreversible Organ Damage (42%) (Lung, Kidney, and/or Liver)
Unknown (8%)
Trauma (8%)
Infection (5%)
Acute Pulmonary Disorders (11%)
Stroke (13%)
MAJORITY OF SCD PATIENTS DO NOT EXPERIENCE VOCs

SCD Patient VOCs per year

- 9%: 10+
- 24%: 2-9
- 15%: 1
- 52%: 0

VOC-targeted therapies

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\(^1\)

- End-organ damage drives major healthcare utilization, with average patient receiving services 30-54 days/year\(^2\)

- ~$700,000 in lost lifetime income per patient\(^3\)

- Major caregiver productivity impact, often creating devastating financial burden

SCD, sickle cell disease.

2. GBT Internal Data.
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

Which safely increases oxygen affinity to create a fraction of non-sickling Hb

Which inhibits HbS polymerization

Hb, hemoglobin; HbS, sickle hemoglobin; oxyHb, oxygenated hemoglobin; RBC, red blood cell; SCD, sickle cell disease.
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
Our Goal: Transform the SCD Treatment Paradigm

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

SCD, sickle cell disease.
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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 effect on VOC rate

VOC, vaso-occlusive crisis.
GBT Market Research: physician respondents (n=248) and patient respondents (n=7).
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95% OF PHYSICIANS AWARE OF OXBRYTA SAID THEY HAVE OR WOULD PRESCRIBE IT

Top Reasons Cited by HCPs to Prescribe

- Improves Anemia
- Increases Hb Levels
- Reduces Hb Polymerization
- Reduces hospitalizations
- Reduces long-term organ damage

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=155, 87) conducted in September-October 2020. Indicates physician would try in at least one patient.

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WE EXPECT EARLY ADOPTION IN SEVERAL PATIENT TYPES

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

SCD U.S. Payer Landscape

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

Broad payer coverage: access for 90% of covered lives in the U.S.

Reimbursement Overview

+ Expect overall gross-to-net to be at the low end of 25-30% range 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.
2. Payer coverage includes lives covered through verified patient adjudication.
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OXBRYTA DISTRIBUTION DESIGNED FOR HIGH-TOUCH PATIENT CARE AND PAYER COVERAGE

Home Delivery (office, school)

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

HCP, healthcare professional; Rx, prescription; SCD, sickle cell disease.
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LOOKING AHEAD
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 patients
+ New age appropriate formulation designed for pediatric population

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 region
+ Targeting countries where vast majority of European SCD patients live

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 region
+ Named Patient Pathways may provide early access to Oxbryta while health authorities conduct reviews

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

Oxbryta 1500 mg¹
N=112

Placebo
N=112

24-week primary analysis treatment period

96-week total treatment period

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

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

- **GU**
  - Priapism

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

Pipeline Opportunities

HbS Polymerization
- Direct HbS inhibitors
  - GBT021601

HbS Polymerization
- HbF Induction
  - Oxbryta®

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 CRISIS

- 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

VOC, vaso-occlusive crises.
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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

WORKING TO IMPROVE CARE FOR SCD PATIENTS AROUND THE WORLD

Global Public Health Problem

Thoughtful and Sustainable Approach


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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

3Q 2020
Achieved broad payer coverage for Oxbryta ahead of expectations

4Q 2020
ASH Oxbryta 72-week data and RWE + pipeline update

Oxbryta U.S. Launch
UPCOMING MILESTONES (CONT.)

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

- **1H 2021**
  - Initiate inclacumab pivotal study

- **Mid 2021**
  - Submit Oxbryta MAA for full marketing authorization

- **Mid 2021**
  - Submit NDA to expand FDA label to include children as young as age 4

- **TBD**
  - Middle East regulatory approvals for Oxbryta

Oxbryta U.S. Commercialization
Thank You