Providing Hope to the Underserved

May 4, 2022

GBT
Hope + Science + Community

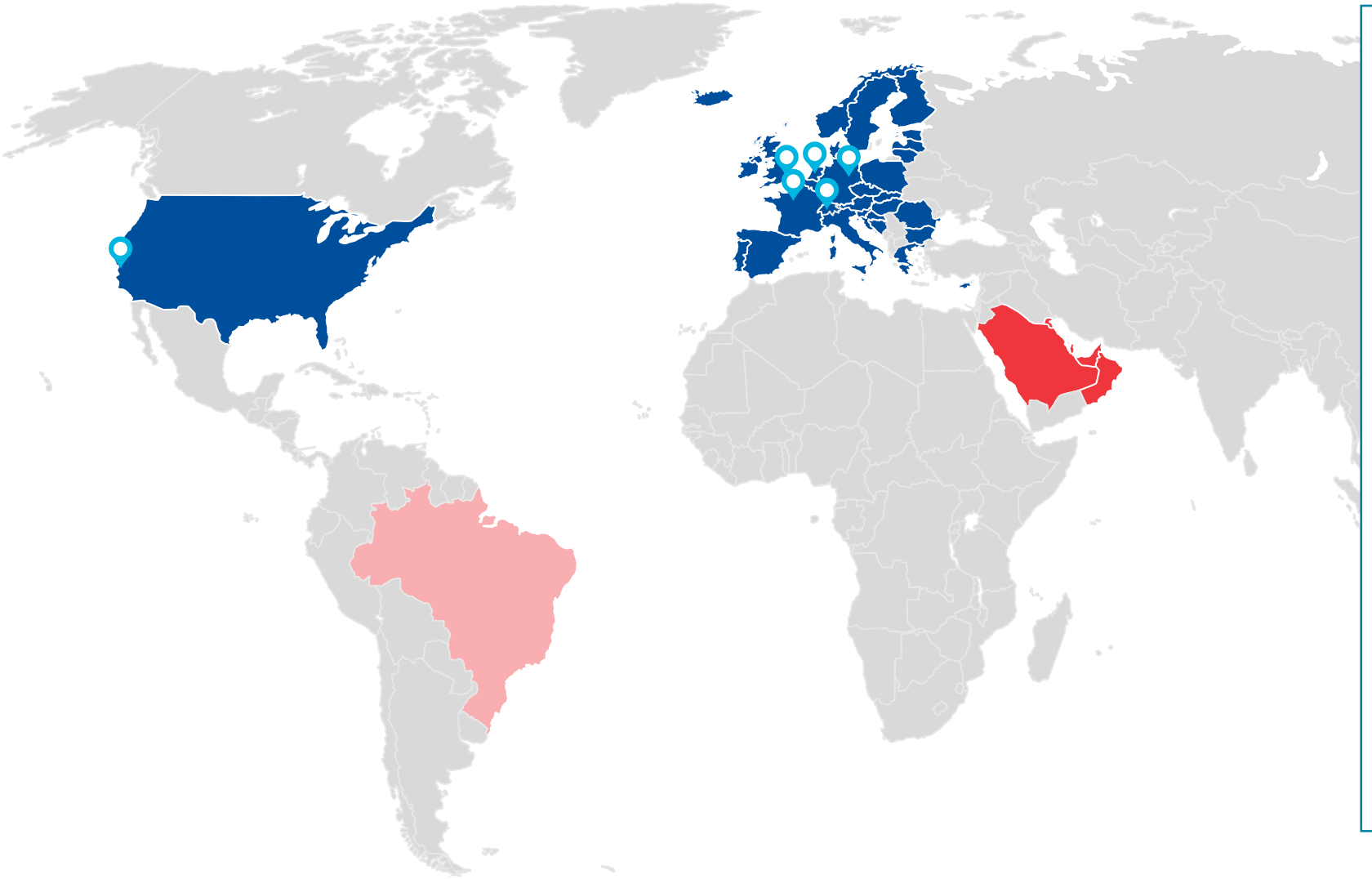
SAFE HARBOR STATEMENT

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 priorities, commitment, dedication, focus, goals, mission, vision, milestones, strategy, positioning, opportunities, 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 improve patient lives, reduce morbidity and mortality and to be a standard of care and disease-modifying therapy; the significance and potential of commercial strategy and related initiatives; Oxbryta awareness and education; the availability, use and impact of GBT Source® and other digital materials; payer coverage; expanding access to Oxbryta for patients in the U.S. and globally, including the commercial potential, timing and other expectations; implementing and completing clinical development plans and registries; generating and reporting data and analyses from past, ongoing and potential future studies; inferences drawn from studies and related analyses; regulatory filing, review and approval; our manufacturing and commercial infrastructure; safety, efficacy, mechanism of action, potential and advancement of our drug candidates and pipeline; discovering, developing and delivering treatments; the significance of reducing hemolysis and increasing hemoglobin, making SCD a well-managed condition and developing a functional cure for SCD; actual and potential partnerships and distribution arrangements; 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 research, development, manufacture, distribution and commercialization activities; government and third-party payer actions, including relating to reimbursement and pricing; risks and uncertainties relating to competitive treatments 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; and the timing and progress of activities under our collaboration, license and distribution agreements; along with those risks set forth 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).

GBT COMPANY OVERVIEW



- Founded in 2011 and now 450+ employees[1]
- Headquarters in South San Francisco with offices in Europe
- Direct operations in U.S. and Europe; partnering in select markets
- 2021 revenue of ~$195 million
- Robust R&D pipeline in SCD; active business development

Direct | Current / Planned Distribution Partner | GBT Offices

1. Full-time employees globally as of December 31, 2021

SCD: AN UNDERSERVED ORPHAN CONDITION

SCD: AN URGENT UNMET NEED

Millions of patients worldwide[1]

Historically limited treatment options; most focused on pain

Varying clinical manifestations

30-year reduced life expectancy[2]

Mapillar, mother

President & Founder, MTS Sickle Cell Foundation

***One family, three different experiences*:**

Hajar, age 12

Cognitive issues

- Major impact on performance in school

Deej, age 16

10 surgeries, one stroke, but no VOCs

- Undergoes regular blood transfusions

Tully, age 17

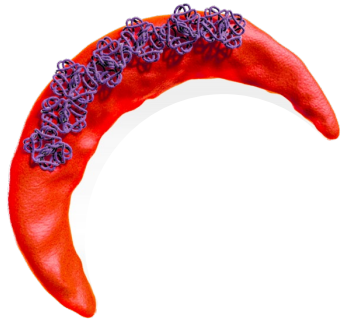
Sustained fatigue and VOCs

- Pain impacts ability to go to school and do activities

VOC, vaso-occlusive crisis.

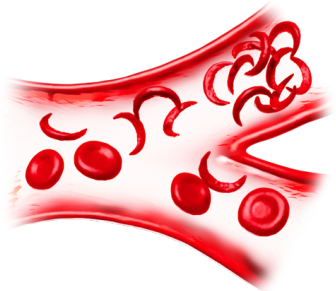
1. Population data: Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). Accessed February 23, 2022; European Medicines Agency. Accessed February 23, 2022. Data on file. 2. Akinsheye, I. et al. Fetal hemoglobin in sickle cell anemia. Blood. 2011. 118:19-27.

SCD IS DRIVEN BY HEMOGLOBIN POLYMERIZATION



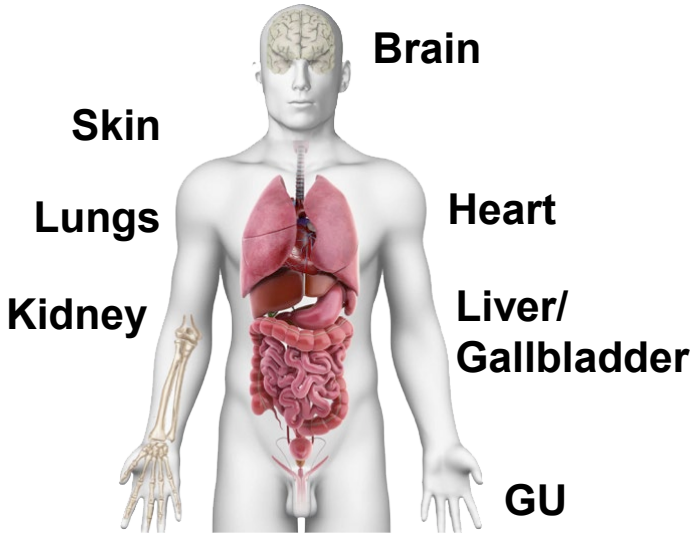
SICKLING
caused by polymerization – sickle hemoglobin forms long, rigid rods

HEMOLYSIS AND ANEMIA
caused by the breakdown of RBCs



VOCs / PAIN CRISES
caused by blockage of blood vessels

LINKED TO MULTI-ORGAN DYSFUNCTION



RBC, red blood cell; GU, genitourinary.

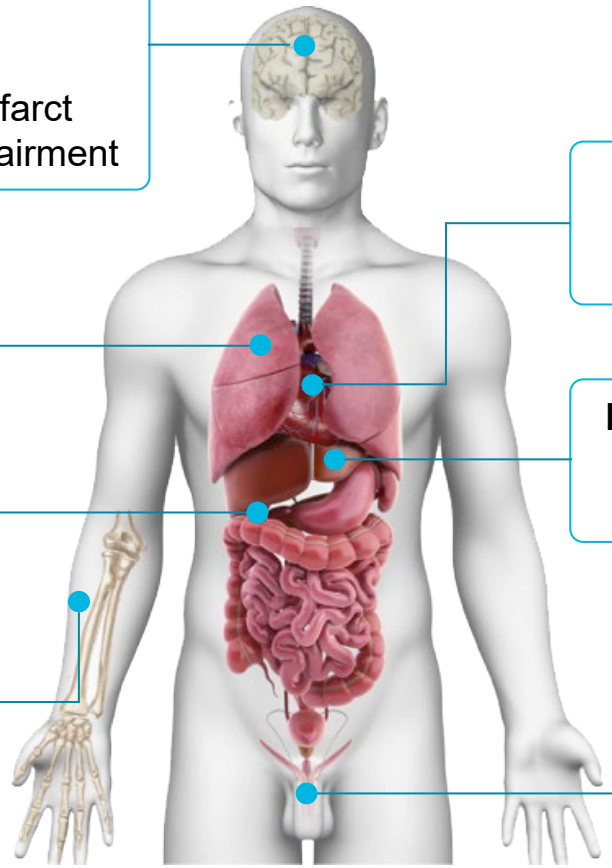
MULTI-ORGAN DYSFUNCTION IN SCD IS LINKED TO CHRONIC ANEMIA AND HEMOLYSIS

Brain
Stroke
Silent cerebral infarct
Neurocognitive impairment

Lungs
Pulmonary hypertension

Kidney
Renal insufficiency
Renal failure

Skin
Leg ulcers

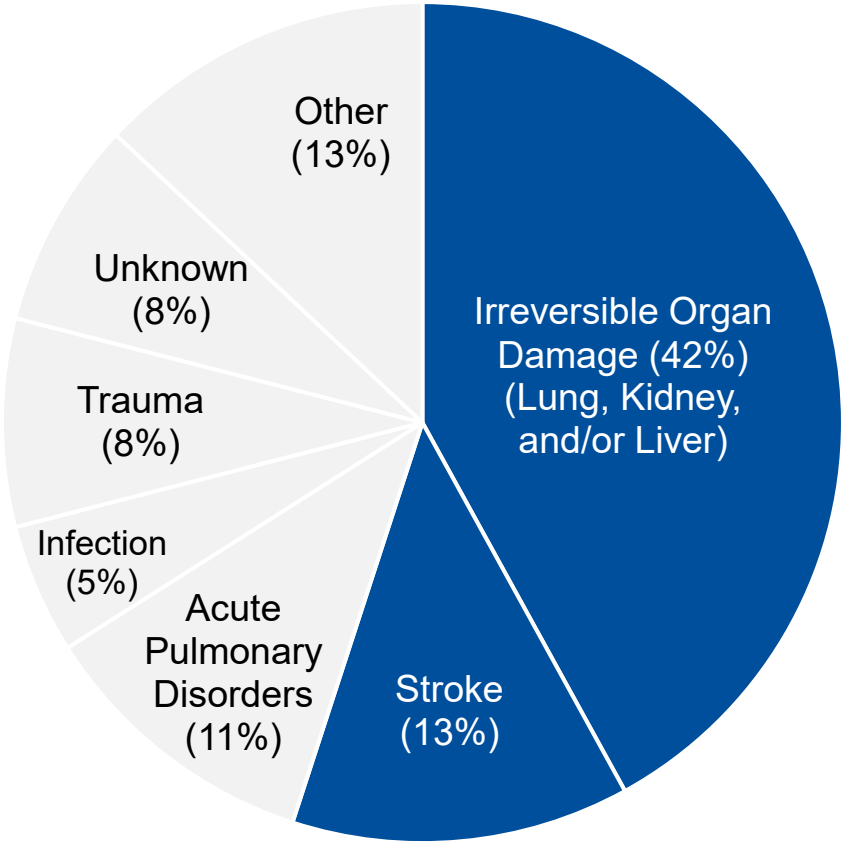


Heart
Cardiomyopathy

Liver/Gallbladder
Hepatopathy
Gallstones

GU
Priapism

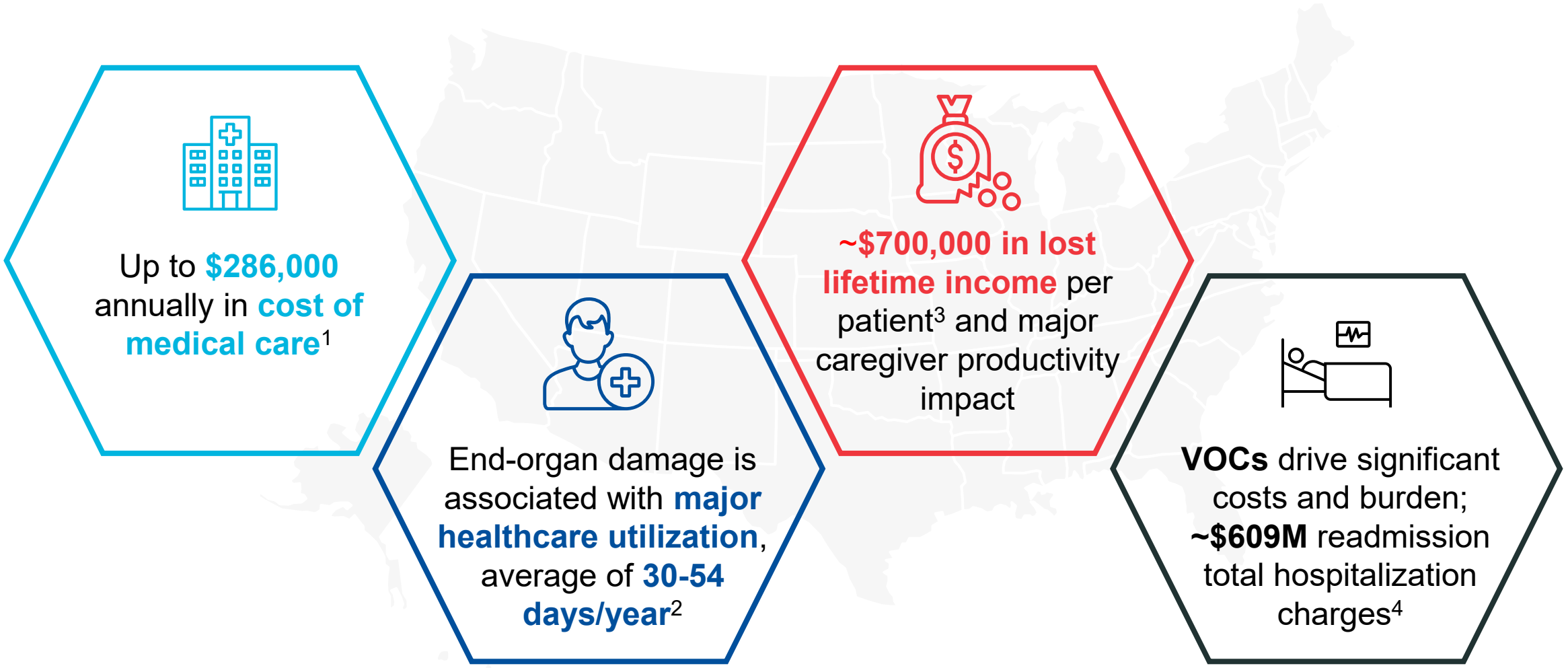
Chronic Organ Damage: Leading Cause of Death in Adults[1]



≥ 20 years of age, n=186

Image adapted from Kato GJ et al. *Nat Rev Dis Primers.* 2018;4:18010.
1. Powars, DR et al. *Medicine.* 2005;84:363–376.

MAJOR BURDEN ON U.S. PATIENTS AND SOCIETY

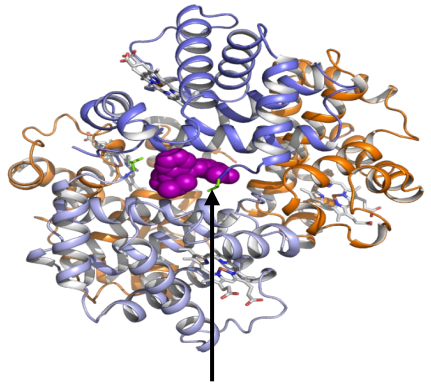


1. Song, X, et al. Economic Burden of End Organ Damage Among Patients with Sickle Cell Disease in the US. ASH 2019 Poster #3388. 2. Campbell, A, et al. Patients With Sickle Cell Disease and Major End-Organ Damage Spend Significant Time Receiving Healthcare Services With High Associated Indirect Costs. SCDAA Annual National Convention, 2021. 3. Lubeck, D. et al. Estimated Life Expectancy and Income of Patients With Sickle Cell Disease Compared With Those Without Sickle Cell Disease. JAMA Netw. Open. 2019 Nov 1;2(11):e1915374. 4. Vivek Kumar, Neha Chaudhary & Maureen M. Achebe. Epidemiology and Predictors of all-cause 30-Day readmission in patients with sickle cell crisis. Nature. 2020.

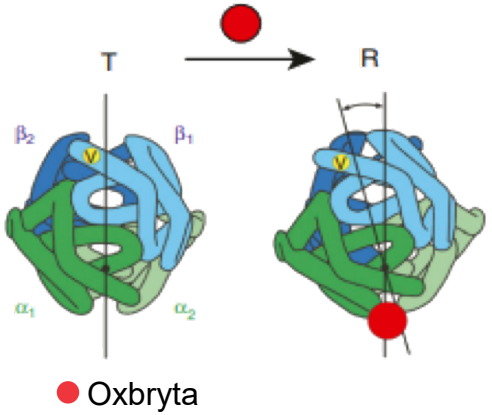
OXBRYTA: FIRST-IN-CLASS SCD THERAPY

OXBRYTA INHIBITS Hb POLYMERIZATION

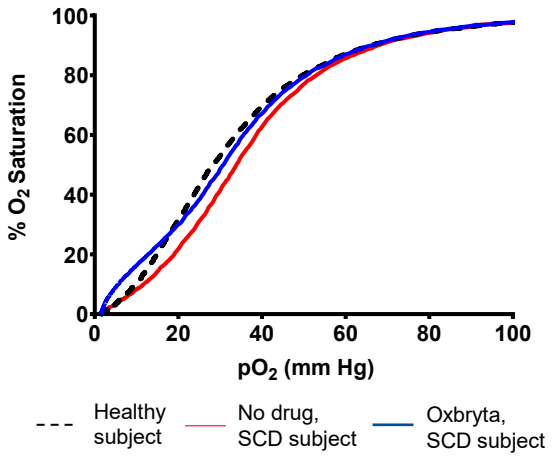
Once-daily, oral treatment



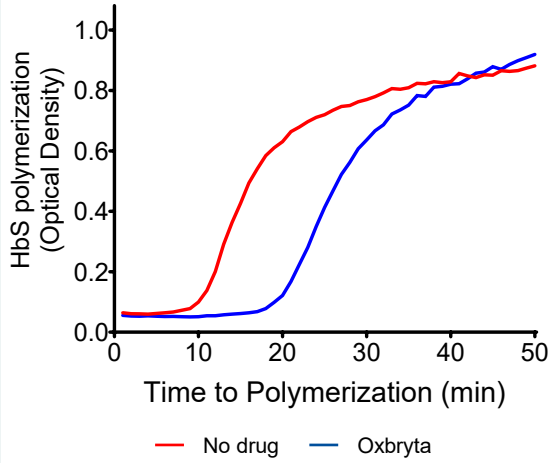
Binding to Hb stabilizes the oxyHb (R) state[1]



Increases oxygen affinity safely to create non-sickling Hb[2]



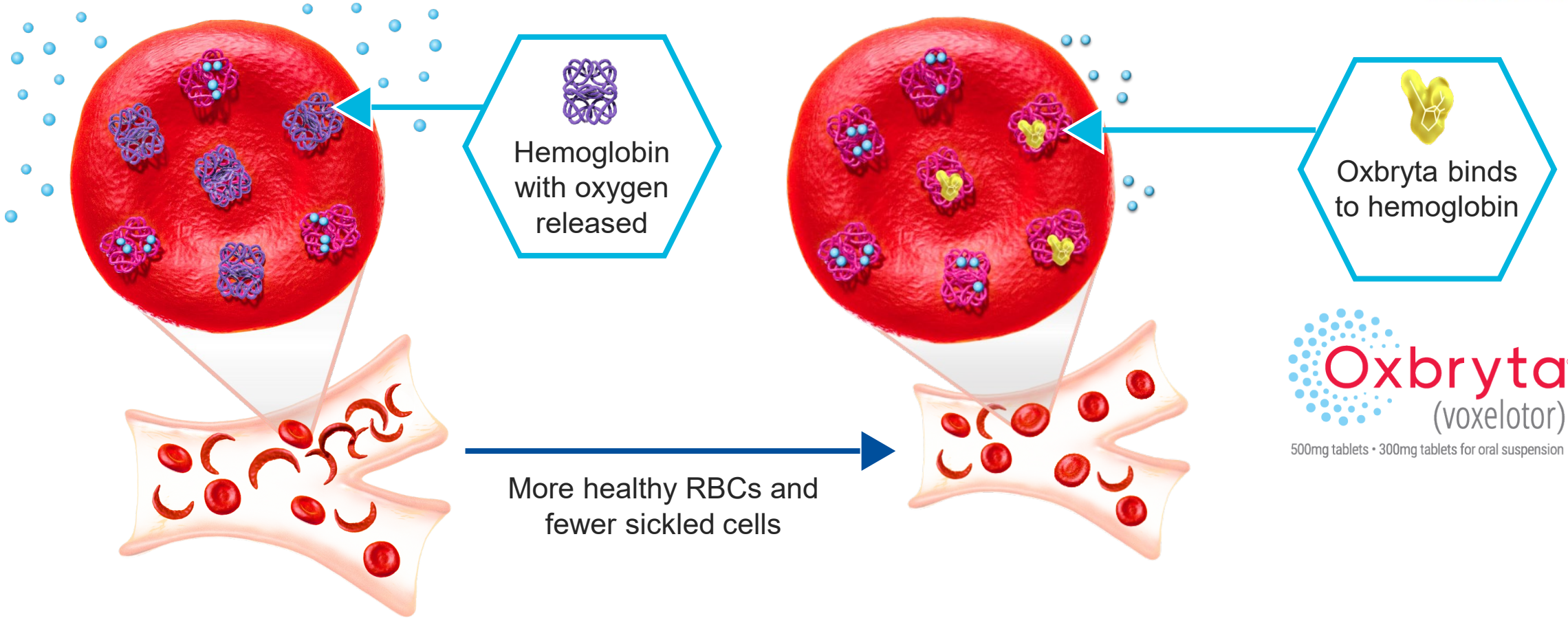
Inhibits HbS polymerization[3]



oxyHb, oxygenated hemoglobin; Hb, hemoglobin.

1. Adapted from Bunn and Eaton, *Blood.* 2017. 2. Hutchaleelaha, A. et al., *British Journal of Clinical Pharmacology.* 2019. 3. Oksenberg , D. et al., *Br J. Haematol.* 2016.

OXBRYTA HELPS HEMOGLOBIN DO ITS JOB—DELIVER OXYGEN THROUGHOUT THE BODY

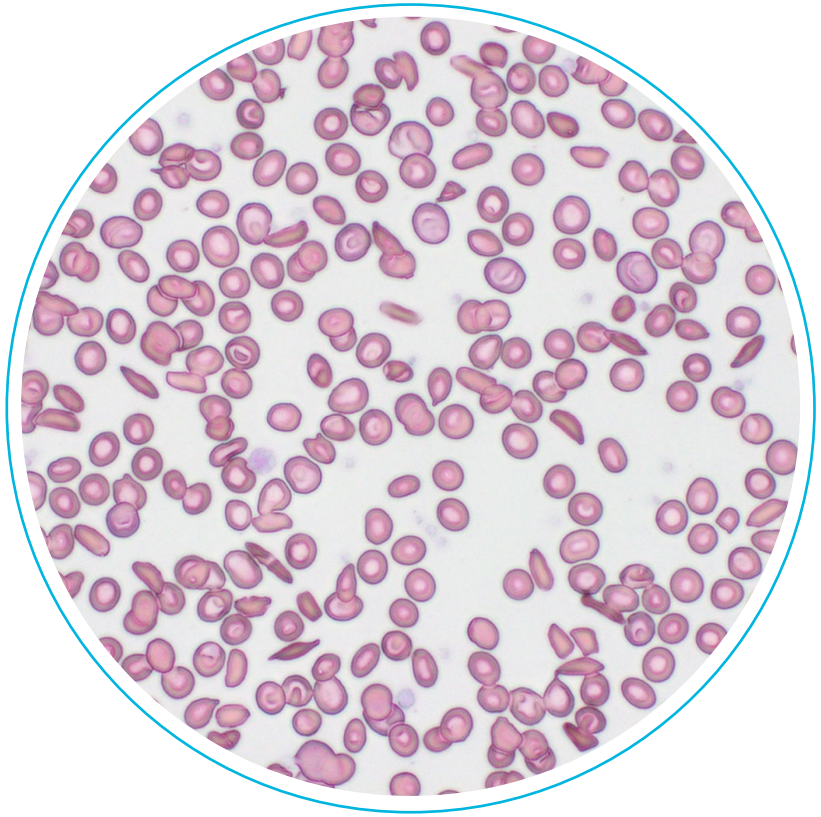


Oxbryta® (voxelotor)
500mg tablets • 300mg tablets for oral suspension

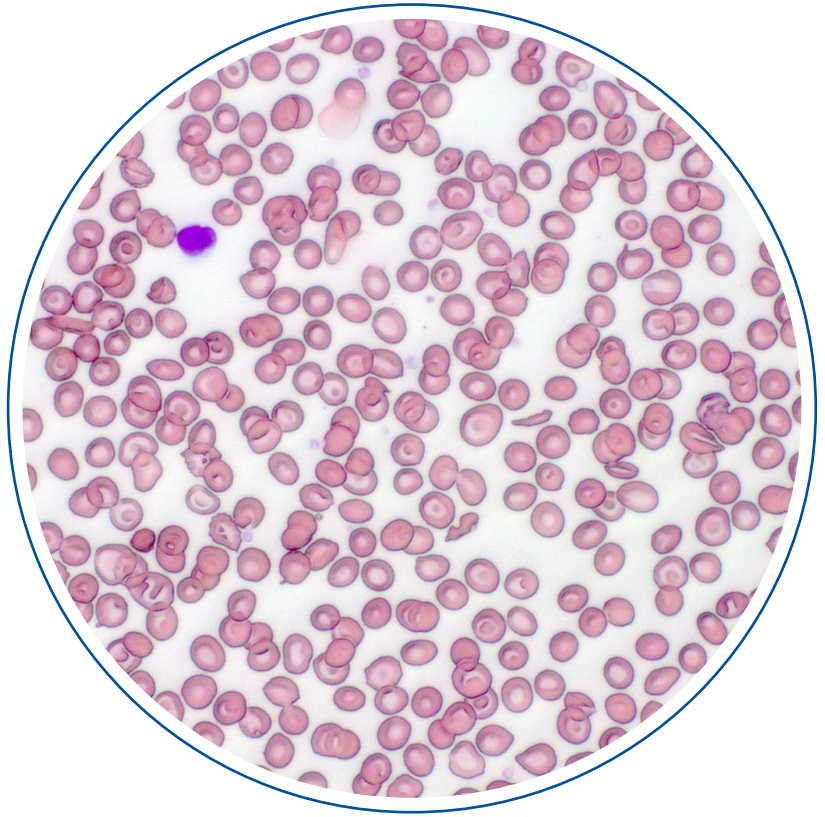
RBC, red blood cell.

OXBRYTA RAPIDLY INCREASES RBC COUNT & HEALTH

Pre-Treatment



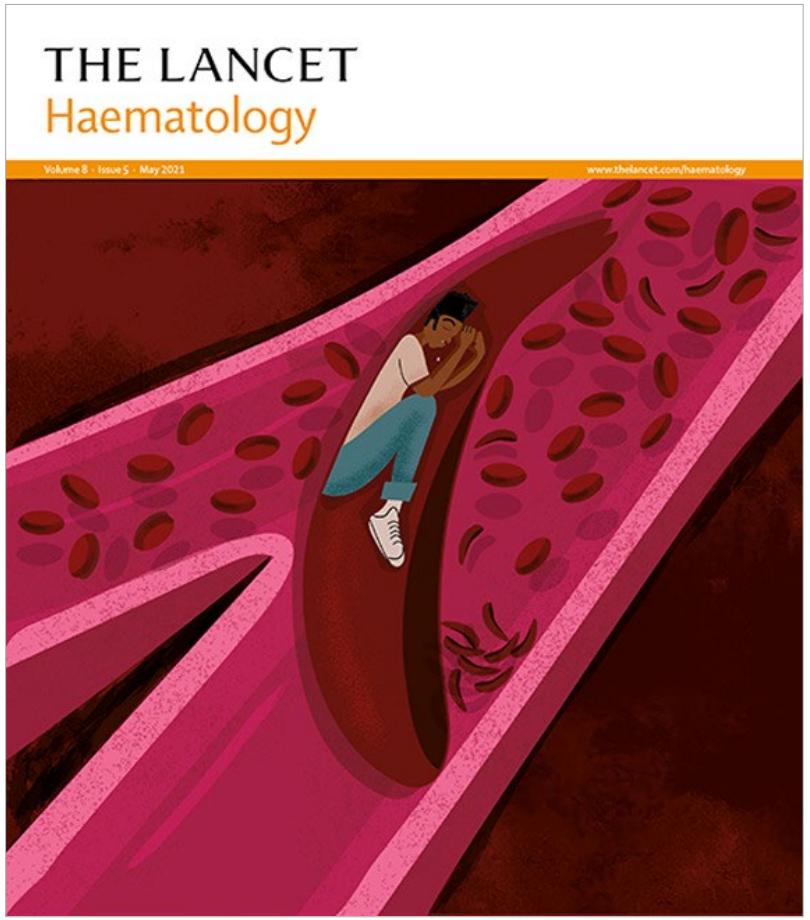
Day 21 of Treatment

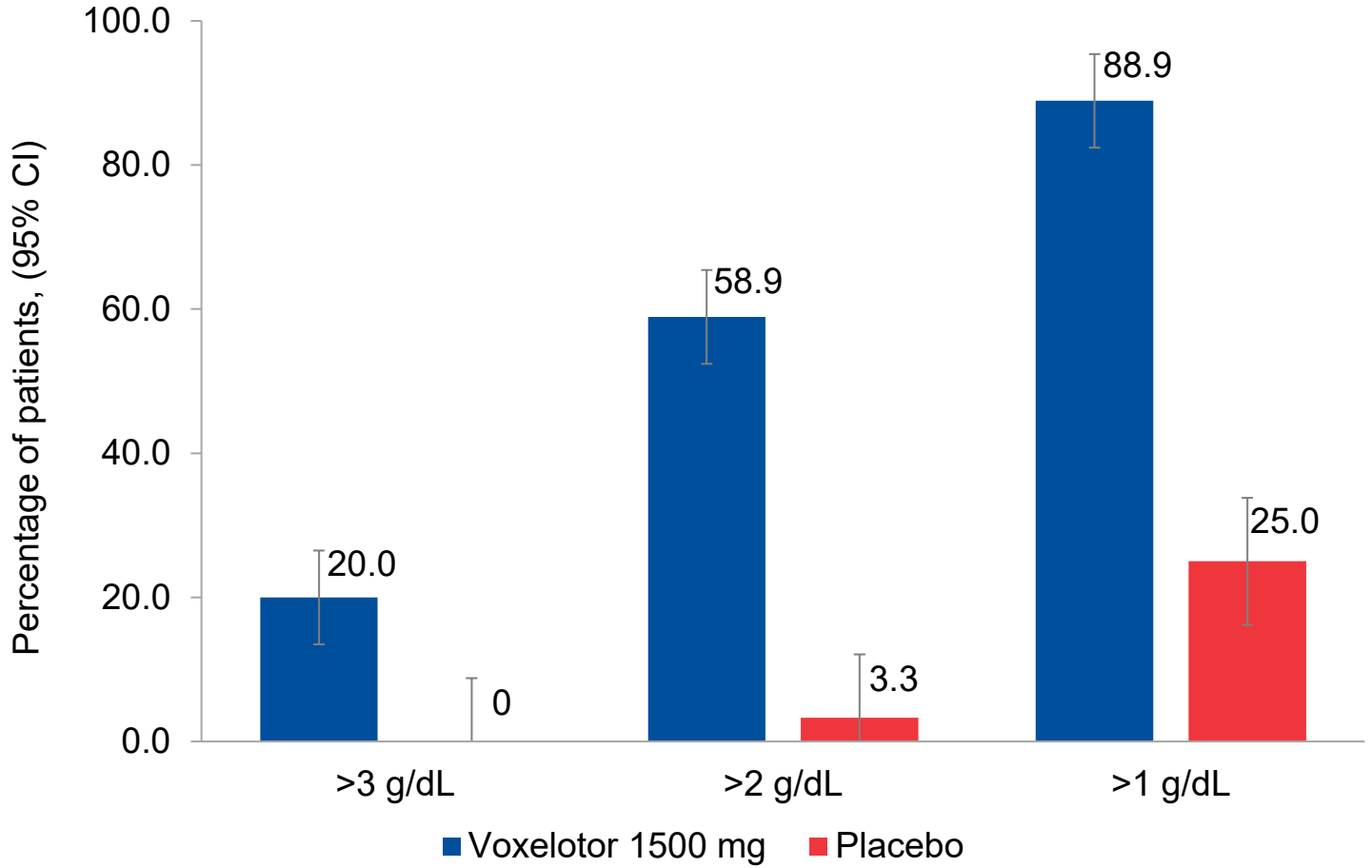


ASH 2020 Poster #1723: Patient Perception of Oxbryta Treatment Benefit. Typical peripheral blood smear. Before and after Oxbryta treatment.

HOPE STUDY: DURABLE IMPROVEMENTS AT 72 WEEKS

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





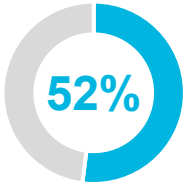
The Lancet Haematology, April 2021. Voxelotor in adolescents and adults with sickle cell disease (HOPE): long-term follow-up results of an international, randomised, double-blind, placebo-controlled, phase 3 trial.
ASH 2020 Poster: #1716, Long-Term Efficacy and Safety of Voxelotor in Adolescents and Adults with Sickle Cell Disease: HOPE Trial 72-Week Analysis.

RWE CONSISTENT WITH HOPE STUDY AND DEMONSTRATES ADDITIONAL BENEFITS

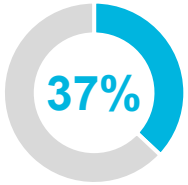
Symphony Claims Analysis Takeaways

N = 3,128; 40% male / 60% female; mean age of 34.7

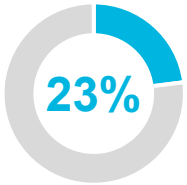
Mean Hb increase of 1.1 g/dL
60.8% of patients had Hb change >1 g/dL during follow up[1]



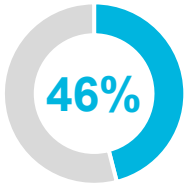
↓ in **transfusions***



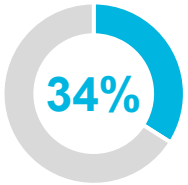
↓ in **all-cause hospitalizations***



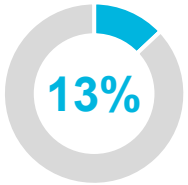
↓ in the number of **VOCs[2]***



↓ in **iron chelation***



↓ in **VOC-related hospital admissions***



↓ in **opioid prescriptions***

* = p<0.001
RWE, real-world evidence; Hb, hemoglobin; VOC, vaso-occlusive crisis.
Shah, N. et al. Real-world effectiveness of voxelotor for treating sickle cell disease in the US: a large claims data analysis. *Expert Review of Hematology*. February 2022.

EXPANDING OXBRYTA CLINICAL DATA

~4,000 patients in clinical trials or RWE studies

GBT Clinical Trials

HOPE Phase 3:

- 24 & 72 weeks
- Post-hoc analyses
- HOPE-KIDS 1
- ✓✓ HOPEKids2 GBT Sickle Cell Disease Clinical Study (enrolling)

Real World Experience

Completed:

- UT Health (ASH 2020)
- ✓ Claims Analysis (ASH 2020/2021)*
- Prisma Health (ASPHO & EHA 2021)
- ✓ RETRO registry (data to be published)

Enrolling / Planned:

- ✓✓ PROSPECT registry
- ✓✓ Activity and sleep quality
- ✓✓ Neurocognition
- ✓✓ Cerebral Blood Flow

✓ Clinical endpoints ✓✓ Prospective clinical endpoints *Ongoing

OXBRYTA'S POTENTIAL TO IMPROVE SCD PATIENT LIVES

Abraham

I like to ride my bike and go places, so I make sure to eat well, drink lots of water, and let my body rest every day.

Arelys

I enjoy time with my family and my kids – it's something that is truly priceless.

Actual Oxbryta patients. Individual patient results may vary.

OXBRYTA: COMMERCIAL UPDATE & OPPORTUNITY

DELIVERING FOR PATIENTS AGES 4+

Net increase in patients taking Oxbryta each quarter since launch[1]

~9,600
new prescriptions[2]

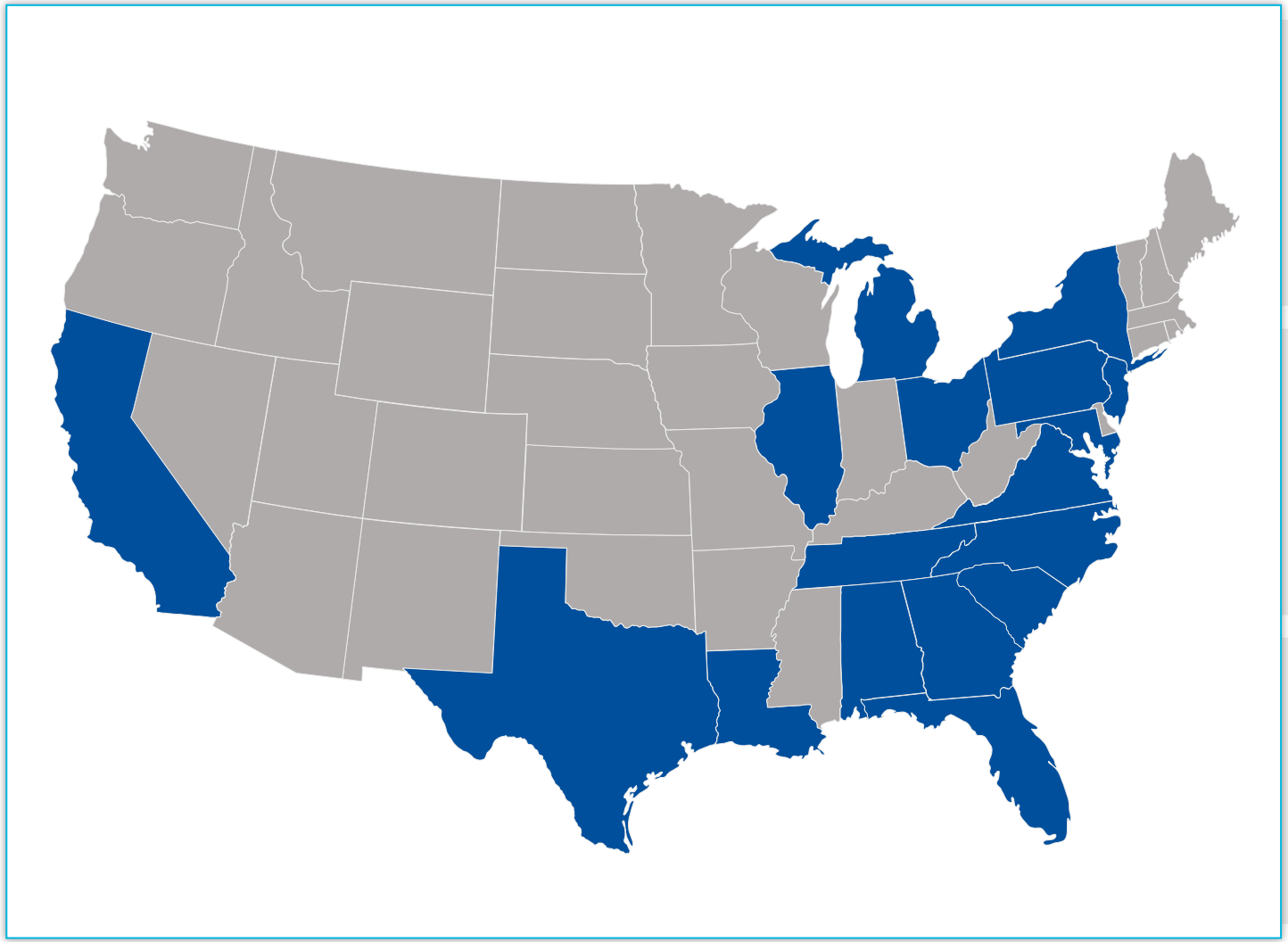
>90%
of covered lives for ages 12+, broad payer coverage[3]

~2,000
unique prescribers[2]

~$375M
LTD revenue[2]

FDA approval for ages 12+ on November 25, 2019 and for ages 4-11 on December 17, 2021. LTD, launch-to-date. Figures may vary from actual due to rounding.
1. For ages 4+ patient population as of March 31, 2022. 2. As reported from launch through March 31, 2022. 3. As of March 31, 2022.

SYNERGISTIC TARGETING OF HCPs AND KOLs



17 states represent **~85%** of ages 12+ SCD patients; patients ages 4-11 concentrated in same 17 states

~55 sickle cell therapeutic specialists targeting **~4,700** HCPs; team already calling on the top SCD pediatric centers

~10 medical science liaisons targeting the top **500 KOLs**

KOL, key opinion leader.
Symphony Health Claims Data, 2012-2018. GBT data as of December 31, 2021.

OXBRYTA PAYER COVERAGE AND GROSS-TO-NET

U.S. SCD Payer Landscape

Ages 12+ SCD payer mix: Medicaid (~50%), Commercial (~30%) and Medicare (~15%)[1]

- ~65% of Oxbryta patients are on government-sponsored plans[2]

Ages 4-11 SCD payer mix: Medicaid (~70%) and Commercial (~30%)[3]

Reimbursement Overview

Channel costs of 8-11% (distribution, returns, copay support)

Mandatory 23.1% discount for Medicaid and 340B (~10-15% Commercial/Medicare patients)

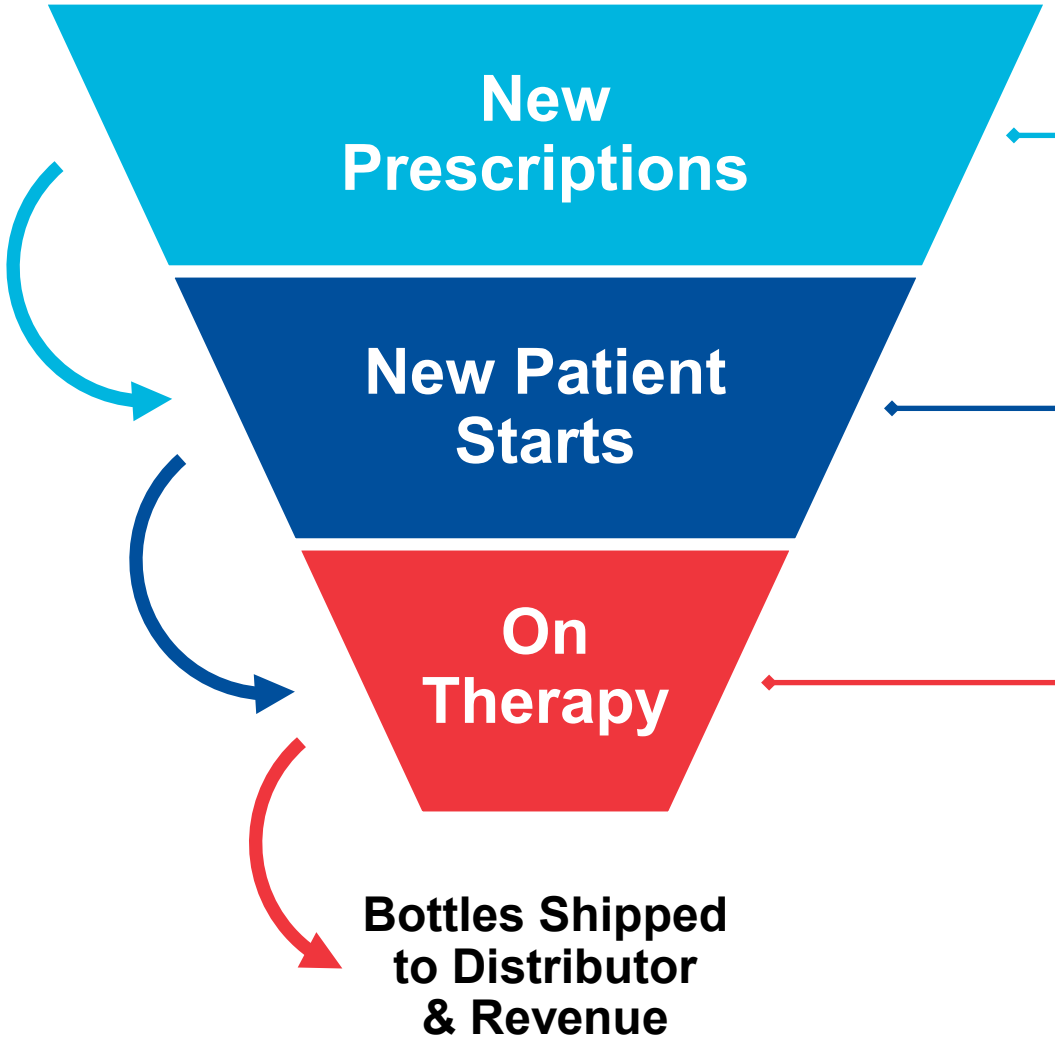
Q1 2022 U.S. gross-to-net of 16.3%

Broad payer coverage for patients ages 12+; anticipate achieving broad coverage for the 4-11 age group by mid-year 2022

Anticipate GBT's aggregate (age 4+) gross-to-net to be approximately 25% at steady state

1. Shared Health Alliance Rx Claims for Hydroxyurea and Endari, Nov. 2015-Oct. 2018. 2. Average based on first on-label fill by patient. Payer mix will fluctuate quarter to quarter. 3. Ped Payer mix based on SHA Claims as of June 2021.

OXBRYTA PRESCRIPTION FUNNEL



Conversion rate ~75%
Consistent with analogs

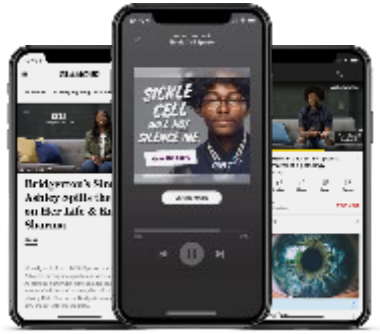
Annual adherence (first year)
Within 50 – 70% range of analogs

Phasing & distributor inventory
Q1 inventory days on hand decreased from Q4; returned to historical levels

Data as of March 31, 2022.

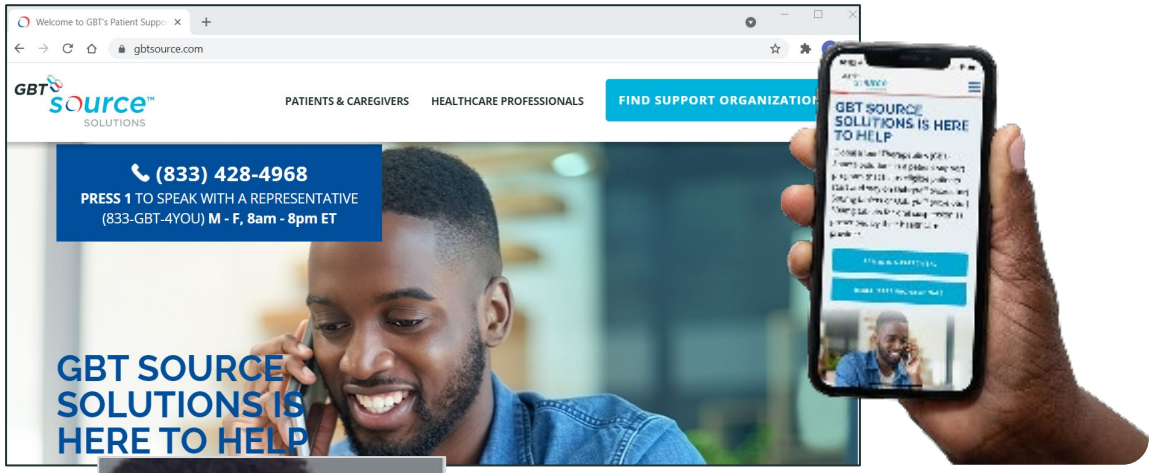
DTC CAMPAIGN AND OTHER DIGITAL MATERIALS DRIVE INCREASED INTEREST IN OXBRYTA

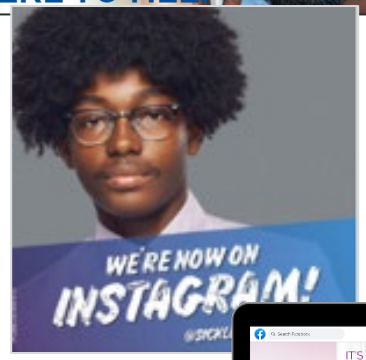
Oxbryta Commercial



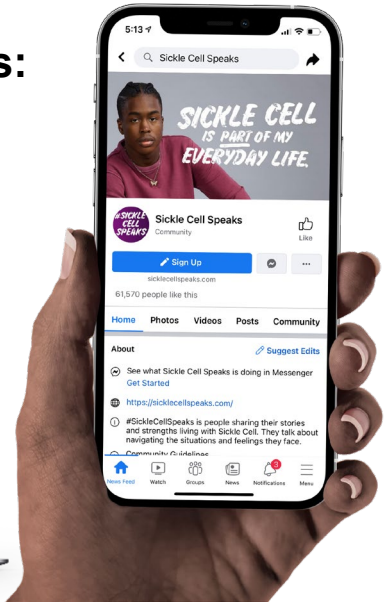
Online Video
YouTube
Pandora
Podcasts

GBT Source Solutions®

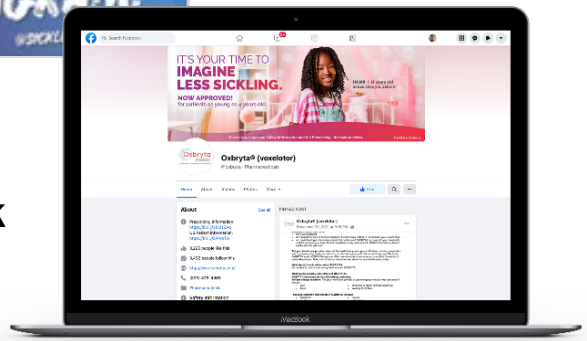




Sickle Cell Speaks: Instagram



Oxbryta: Facebook



HIGH AWARENESS AND SATISFACTION

Awareness

Specialist aided awareness of Oxbryta

Specialists aware that Oxbryta is now approved for children ages 4 to 11 years

Perception

% of prescribers satisfied with Oxbryta

% of current Oxbryta patients who say it **works extremely well**

% of current Oxbryta users are likely to recommend it to others

Data as of March 31, 2022

NEAR-TERM OPPORTUNITY TO REACH >350K PATIENTS

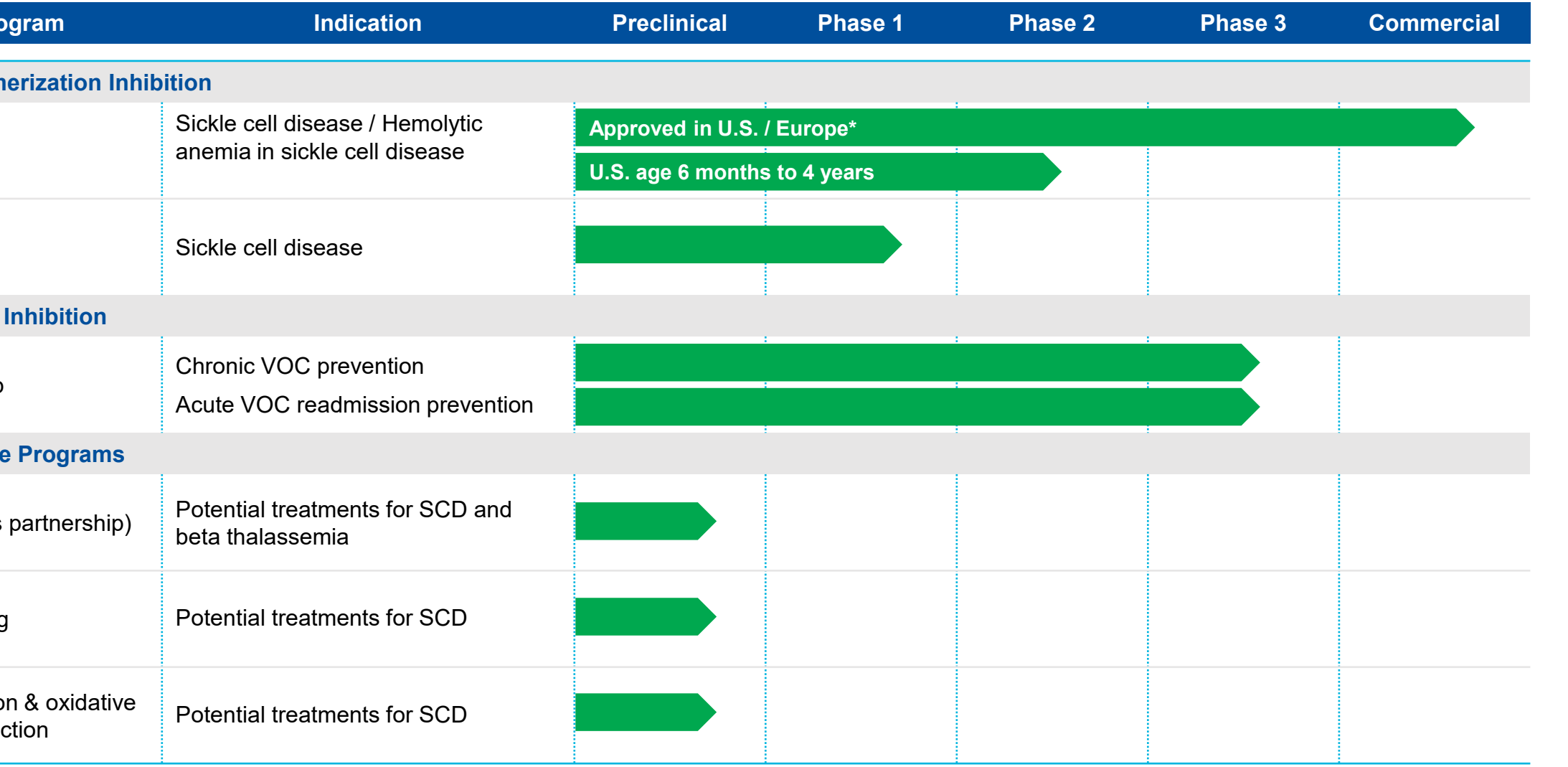
U.S.	Europe	Middle East	Latin America
Oxbryta label			
>100K Patients (4+)	**~52K** Patients	**~100K** Patients	**~100K** Patients
FDA approval for ages 12+ on November 25, 2019 and for ages 4-11 on December 17, 2021	**EU approval for ages 12+ on February 14, 2022** **United Kingdom under review for potential approval**	**Partnered with distributor for six GCC countries** **Approval secured in United Arab Emirates**	**Planning to partner with distributor for Brazil**

Early access programs in Europe and Middle East underway and planned for Latin America

EU, European Union.
Population data: Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). https://www.cdc.gov/ncbddd/sicklecell/data.html. Accessed February 24, 2021; Symphony Health Claims Data, May 2021; European Medicines Agency. https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182125. Accessed February 24, 2021. Data on file.

ADVANCING THE GBT PIPELINE

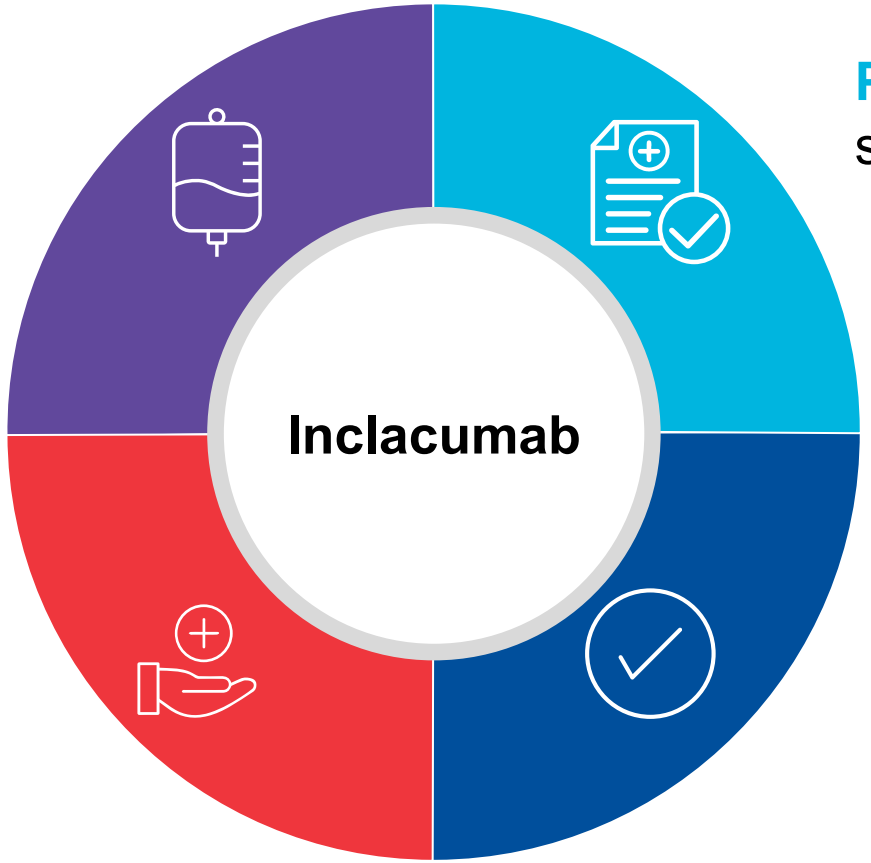
GBT PIPELINE TARGETS SCD VIA MULTIPLE APPROACHES



HbF, fetal hemoglobin. *Approval includes EU member states and Iceland, Liechtenstein and Norway. For the UK, GBT has submitted an application to the Medicines and Healthcare products Regulatory Agency (MHRA) for a Great Britain Marketing Authorisation using the EC Decision Reliance Procedure.

INCLACUMAB HAS BEST-IN-CLASS POTENTIAL

Quarterly dosing
vs. monthly



Potentially favorable
safety profile

Unique opportunity via
chronic or on-demand dosing

Phase 3 THRIVE
clinical studies underway

TWO PIVOTAL PHASE 3 STUDIES OF INCLACUMAB WITH OPPORTUNITY TO IMPROVE OUTCOMES AND REDUCE COSTS

Chronic Prevention Protocol
quarterly dosing

N = 240 (enrolling)

Primary Endpoint: VOC rate during 48-week treatment period

thrive
THerapy for Reduction with Inclacumab of VOC Episodes

Acute Re-Admission Protocol
on-demand dosing

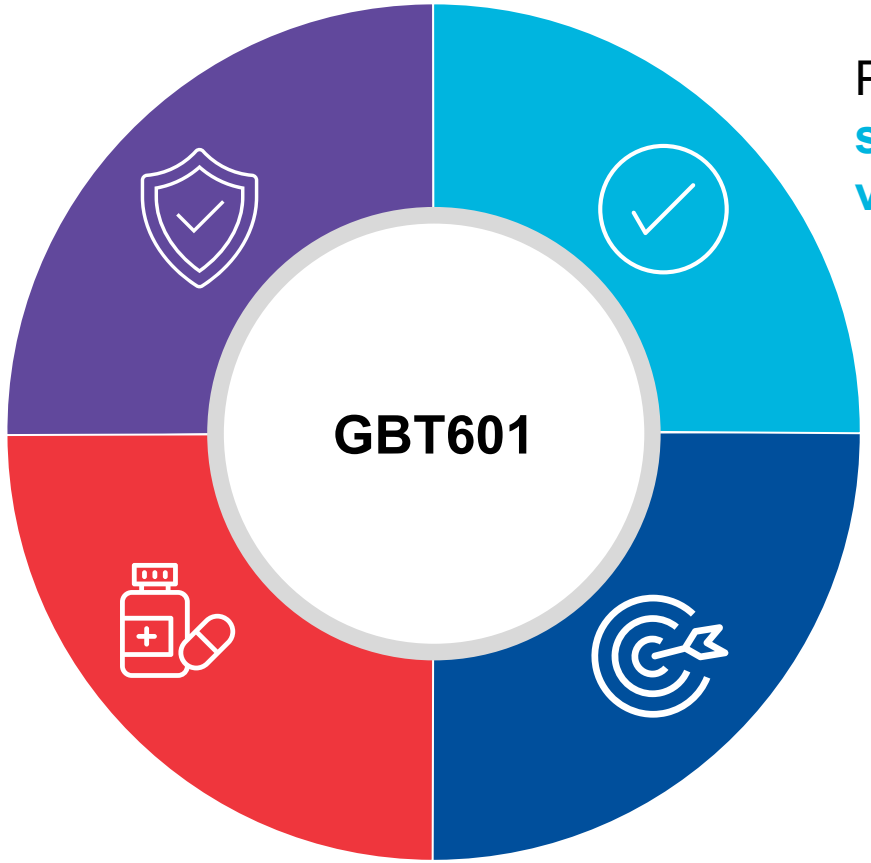
N = 280 (enrolling)

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

Treatment Goals:

GBT601: POTENTIAL FOR BETTER EFFICACY AT A LOWER DOSE

Based on Oxbryta's established **mechanism of action**

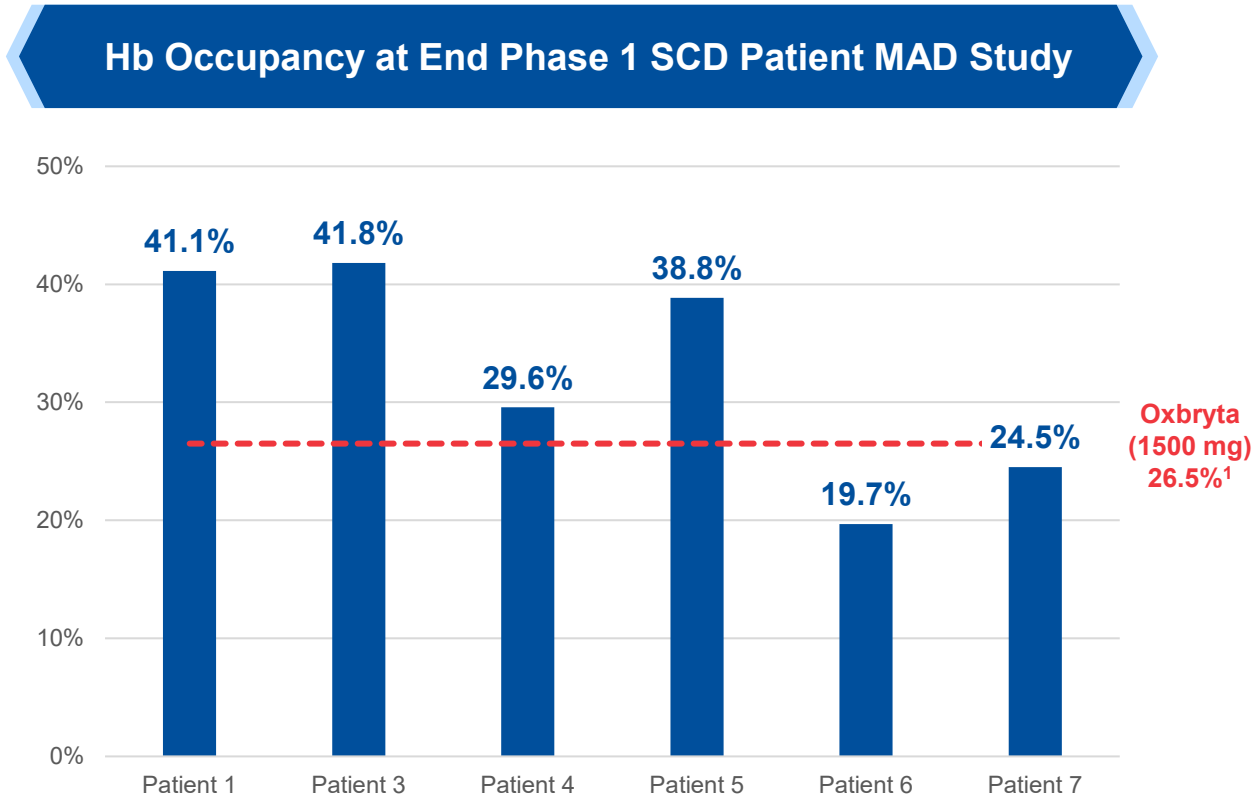


Potential to demonstrate **superior efficacy on a variety of clinical outcomes**

Potential for **single pill** per day dose and **better tolerability**

Targeting **30%+ Hb occupancy**

GBT601 ACHIEVED >30% MEAN Hb OCCUPANCY WITH 100 mg DAILY DOSE



Mean Hb occupancy of 32.6%

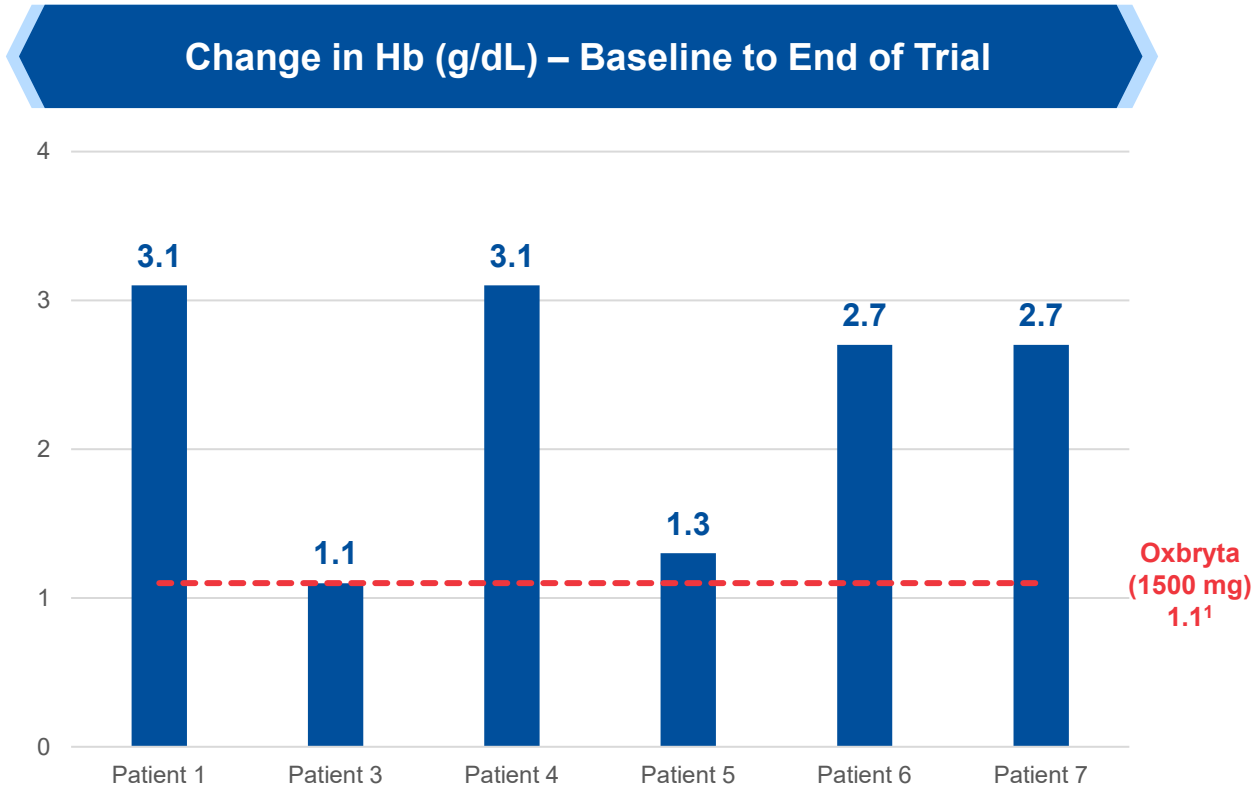
Hb occupancy measured at end of trial, which included 3 weeks at 100 mg daily dose (MAD-2)

Dose proportionality observed from MAD-1 (50 mg daily dose) to MAD-2 (100 mg daily dose)

Note: Patient #2 withdrew during screening before study initiation and was replaced by patient #7.

1. https://www.nejm.org/doi/full/10.1056/nejmoa1903212
ASH 2021 Poster #3099: GBT021601, a Next Generation HbS Polymerization Inhibitor: Results of Safety, Tolerability, Pharmacokinetics and Pharmacodynamics in Adults Living with Sickle Cell Disease and Healthy Volunteers.

Hb IMPROVED UP TO 3.1 g/dL WITH 100 mg DOSE OF GBT601



	Patient 1	Patient 3	Patient 4	Patient 5	Patient 6	Patient 7
Baseline	8.3	8.7	8.4	8.3	7.6	7.8
End of Trial	11.4	9.8	11.5	9.6	10.3	10.5
Hb Occupancy	41.1%	41.8%	29.6%	38.8%	19.7%	24.5%

Mean increase in Hb 2.3 g/dL

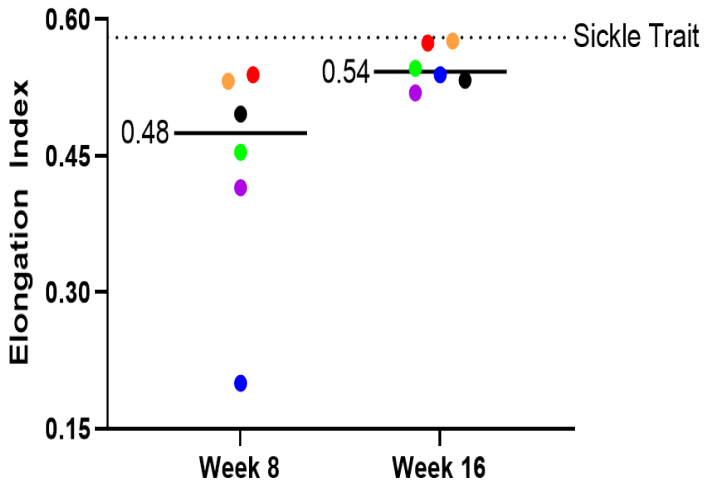
Hb change measured at end of trial, which included 3 weeks at 100 mg daily dose (MAD-2)

Note: Patient #2 withdrew during screening before study initiation and was replaced by patient #7.

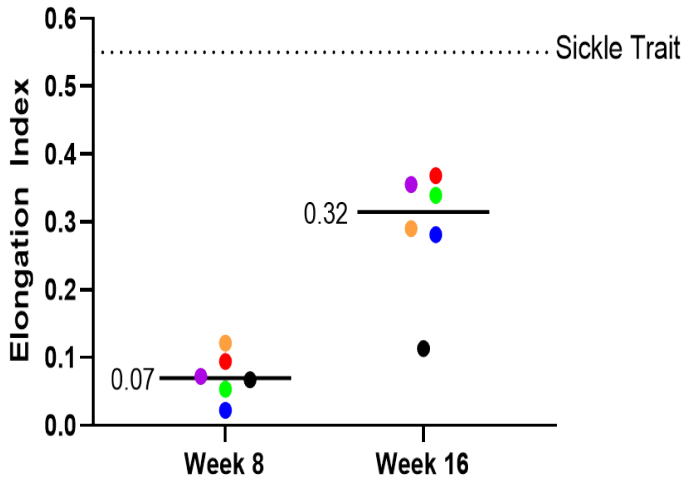
1. https://www.nejm.org/doi/full/10.1056/nejmoa1903212
ASH 2021 Poster #3099: GBT021601, a Next Generation HbS Polymerization Inhibitor: Results of Safety, Tolerability, Pharmacokinetics and Pharmacodynamics in Adults Living with Sickle Cell Disease and Healthy Volunteers.

GBT601 DEMONSTRATED SIGNIFICANT IMPROVEMENTS IN RBC HEALTH

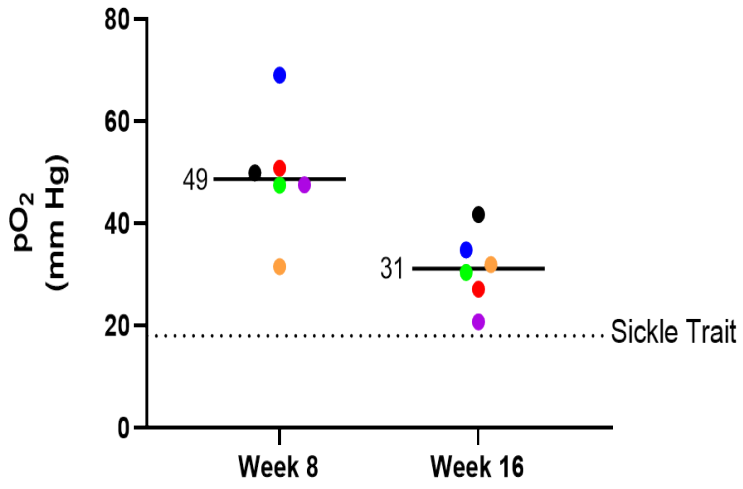
Elmax



Elmin



PoS



Patient 1 Patient 3 Patient 4 Patient 5 Patient 6 Patient 7

RBC, red blood cell.
Note: Patient #2 withdrew during screening before study initiation and was replaced by patient #7.

WE HAVE SEVERAL CATALYSTS TO DRIVE GROWTH

Underpinned by a strong balance sheet with ~$662 million[1]

Oxbryta

Strong launch fundamentals anticipated to drive growth in NRxs in 2H 2022

Launch for patients ages 4-11 underway; targeting broad payer coverage by mid-year

EU marketing authorization for patients ages 12+; anticipate GB authorization by mid-year

Launch in Germany anticipated in near term; reimbursement negotiations in France, England and Germany underway

Pipeline

Enrolling two Phase 3 trials for inclacumab

Advancing GBT601 to Phase 2/3, with Phase 2 portion targeted to be initiated by mid-year

Restarting GBT601 Phase 1 SCD patient trial to study 150 mg daily dose; goal to present data at medical meeting by end of year

Research exploring additional MOAs in anticipation of potential combination therapies

NRxs, new prescriptions; EU, European Union; GB, Great Britain.
1. Cash, cash equivalents, and marketable securities as of March 31, 2022.

OUR LONG-TERM VISION

Leadership in SCD and Other Underserved Orphan Disease Communities

- More-real world experience
- Label expansion
- Global launches
- Access in low resource countries

Establish Oxbryta as SOC

- Inclacumab
- GBT601
- HbF inducers
- Novel targets

Advance SCD Pipeline

- Benign hematology
- Orphan diseases

Leverage Capabilities to Expand Beyond SCD

VALUE

SOC, standard od care.

Thank You

GBT
Hope + Science + Community