

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): December 6, 2020**

**GLOBAL BLOOD THERAPEUTICS, INC.**  
(Exact name of registrant as specified in its charter)

**Delaware**  
(State or Other Jurisdiction of Incorporation)

**001-37539**  
(Commission File Number)

**27-4825712**  
(I.R.S. Employer Identification No.)

**181 Oyster Point Blvd.**  
**South San Francisco, California 94080**  
(Address of Principal Executive Offices) (Zip Code)

**(650) 741-7700**  
(Registrant's telephone number, including area code)

**Not Applicable**  
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

<b>Title of each class</b>	<b>Trading Symbol(s)</b>	<b>Name of each exchange on which registered</b>
Common Stock, par value \$0.001 per share	GBT	The NASDAQ Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

**Item 8.01. Other Events.**

On December 6, 2020, Global Blood Therapeutics, Inc. issued two press releases titled “GBT Presents New Data on the Long-Term and Real-World Use of Oxbryta® (voxelotor) Tablets in Patients with Sickle Cell Disease at 62nd ASH Annual Meeting and Exposition” and “GBT Presents Data on New Sickle Cell Disease Pipeline Therapies with Best-in-Class Potential – Inclacumab and GBT021601.” Copies of the press releases are attached as Exhibits 99.1 and 99.2, respectively, to this Current Report on Form 8-K and are incorporated herein by reference.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

**Exhibit****No.****Description**

<a href="#"><u>99.1</u></a>	<a href="#"><u>Press Release, dated December 6, 2020, titled “GBT Presents New Data on the Long-Term and Real-World Use of Oxbryta® (voxelotor) Tablets in Patients with Sickle Cell Disease at 62nd ASH Annual Meeting and Exposition”</u></a>
<a href="#"><u>99.2</u></a>	<a href="#"><u>Press Release, dated December 6, 2020, titled “GBT Presents Data on New Sickle Cell Disease Pipeline Therapies with Best-in-Class Potential – Inclacumab and GBT021601”</u></a>
104	Cover Page Interactive Data File (embedded within Inline XBRL document)

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**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

**Global Blood Therapeutics, Inc.**

Date: December 7, 2020

By: /s/ Jeffrey Farrow  
Jeffrey Farrow  
Chief Financial Officer  
(Principal Financial Officer)

## GBT Presents New Data on the Long-Term and Real-World Use of Oxbryta® (voxelotor) Tablets in Patients with Sickle Cell Disease at 62nd ASH Annual Meeting and Exposition

*Final 72-Week Analyses of Phase 3 HOPE Study Demonstrate Durable Improvements in Hemoglobin Levels and Significant Improvements in Overall Health Status*

*Real-World Experience Study Results Consistent with HOPE Study and Show Improved Patient Health Status*

**SOUTH SAN FRANCISCO, Calif., Dec. 06, 2020 (GLOBE NEWSWIRE)** -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced new data from the 72-week analyses of the Phase 3 HOPE Study of Oxbryta® (voxelotor) tablets in patients with sickle cell disease (SCD). These data, as well as new findings from real-world experience studies of Oxbryta, are being presented at the all-virtual 62<sup>nd</sup> American Society of Hematology (ASH) Annual Meeting and Exposition.

“We are pleased that the longer term, 72-week HOPE Study data are consistent with the previously reported 24-week primary analyses, confirm the durability of effect and justify the sustained use of Oxbryta for treatment of sickle cell disease,” said Ted W. Love, M.D., president and chief executive officer of GBT. “Since Oxbryta was approved in late 2019, we are also excited by the growing body of real-world evidence that shows similar increases in hemoglobin levels as were observed in the HOPE study and demonstrates that Oxbryta has the potential to significantly improve overall health status for patients with this devastating disease.”

### 72-Week Analyses of Phase 3 HOPE Study

The analyses of the complete data from the Phase 3 HOPE Study support the long-term use of Oxbryta to reduce anemia and hemolysis, with the potential to mitigate the associated morbidity and mortality of SCD.

An analysis of the 72-week data (Abstract #1716) demonstrated that Oxbryta at 1500 mg resulted in durable improvements in hemoglobin levels and markers of hemolysis over 72 weeks of treatment. A large majority of patients (approximately 90 percent) achieved a Hb improvement of >1 g/dL from baseline at one or more time points during the study as compared to placebo (approximately 25 percent). The study also found:

- Significant improvements in markers of hemolysis in indirect bilirubin and reticulocyte percentage.
- Consistent with the 24-week data previously reported, treatment with Oxbryta remained well tolerated. The most common side effects reported were headache, diarrhea, abdominal pain, nausea, arthralgia, rash and pyrexia.

“The underlying cause of sickle cell disease and the root of the devastating, life threatening complications of the disease is hemoglobin polymerization and the resulting anemia and hemolysis,” said Elliott Vichinsky, M.D., director of hematology/oncology at UCSF Benioff Children’s Hospital in Oakland, Calif. “The longer-term 72-week data presented at ASH this week provide additional support for the chronic use of this novel disease modifying therapy in the treatment of this serious condition.”

Another HOPE Study analysis (Abstract #795) found that higher hemoglobin levels achieved with Oxbryta are associated with a lower incidence of vaso-occlusive crises (VOCs) over 72 weeks. While the HOPE Study was not designed or powered to show an effect on VOCs, these results suggest the importance of reducing hemolysis and raising hemoglobin in individuals with SCD through inhibition of polymerization. Specifically:

- The annualized incidence rates of VOCs were numerically lower in patients receiving Oxbryta 1500 mg (2.4) than placebo (2.8); this numerical difference was greater in patients who had experienced two or more VOCs in the year prior to the study.
- Patients with the highest average hemoglobin levels over 72 weeks experienced the fewest VOCs with Oxbryta, with a stepwise reduction in VOC rate as hemoglobin levels increased.

A third analysis from the HOPE Study (Abstract #802) used the Clinical Global Impression of Change (CGI-C) scale, a validated outcomes measure that provides a holistic assessment of the effect of treatment. Results showed that treatment with Oxbryta compared to placebo resulted in a statistically significant higher rating of improved overall patient health status after 72 weeks by the treating physician.

### Real-World Experience with Oxbryta

Since its approval in November 2019, Oxbryta has been prescribed to thousands of patients. Analyses from two studies of real-world experience with Oxbryta showed hemoglobin levels increased similarly to what was reported from the HOPE study.

An analysis (Abstract #2627) evaluating Symphony Health claims data from a subset of 1,275 SCD patients ages 12 and older treated with Oxbryta showed statistically significant reductions in annualized transfusion rates and a reduced annual rate of VOC events following the initiation of Oxbryta therapy.

An additional study (Abstract #1723) from a single-center case series showed that both patients and clinicians observed improved health status based on the Patient Global Impression – Improvement (PGI-I) and the Clinical Global Impression – Improvement (CGI-I) scales to examine patient and clinician perception of health status in patients treated with Oxbryta. In addition, while

cases of gastrointestinal side effects were reported at a rate of incidence similar to that as the HOPE Study, patients were successfully managed with adjustments to dosing regimens and persisted on treatment.

“After such an extended period with no new treatments for people with SCD, our hope for Oxbryta was that patients would finally have a therapeutic option that could lessen the daily and often invisible burden of this disease,” said Modupe Idowu, M.D., medical director at UT Physicians Comprehensive Sickle Cell Center at Houston. “I am encouraged by the analysis of CGI-I and PGI-I scores, which demonstrate that both physicians and patients see improvements in overall health with Oxbryta treatment.”

### **About Sickle Cell Disease**

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States,<sup>1</sup> an estimated 52,000 people in Europe,<sup>2</sup> and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.<sup>1</sup> It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.<sup>1</sup> SCD is a lifelong inherited rare blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.<sup>3</sup> Due to a genetic mutation, individuals with SCD form abnormal hemoglobin known as sickle hemoglobin. Through a process called hemoglobin polymerization, red blood cells become sickled – deoxygenated, crescent-shaped, and rigid.<sup>3-5</sup> The sickling process causes hemolytic anemia (low hemoglobin due to red blood cell destruction) and blockages in capillaries and small blood vessels, which impede the flow of blood and oxygen throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.<sup>4-7</sup>

### **About Oxbryta<sup>®</sup> (voxelotor) Tablets**

Oxbryta (voxelotor) is an oral, once-daily therapy for patients with sickle cell disease (SCD). Oxbryta works by increasing hemoglobin’s affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, GBT believes Oxbryta blocks polymerization and the resultant sickling and destruction of red blood cells, which are primary pathologies faced by every single person living with SCD. Through addressing hemolytic anemia and improving oxygen delivery throughout the body, GBT believes that Oxbryta has the potential to modify the course of SCD. On Nov. 25, 2019, Oxbryta received U.S. Food and Drug Administration (FDA) accelerated approval for the treatment of SCD in adults and children 12 years of age and older.<sup>8</sup>

As a condition of accelerated approval, GBT will continue to study Oxbryta in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of the therapy to decrease stroke risk in children 2 to 15 years of age.

In recognition of the critical need for new SCD treatments, the FDA granted Oxbryta Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD. Additionally, Oxbryta has been granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), and the European Commission (EC) has designated Oxbryta as an orphan medicinal product for the treatment of patients with SCD.

GBT plans to seek regulatory approvals to expand the potential use of Oxbryta in the United States for the treatment of SCD in children as young as 4 years old, and to treat hemolytic anemia in SCD patients ages 12 years and older in Europe.

### **Important Safety Information**

Oxbryta should not be taken if the patient has had an allergic reaction to voxelotor or any of the ingredients in Oxbryta. See the end of the patient leaflet for a list of the ingredients in Oxbryta.

Oxbryta can cause serious side effects, including serious allergic reactions. Patients should tell their health care provider or get emergency medical help right away if they get rash, hives, shortness of breath or swelling of the face.

Patients receiving exchange transfusions should talk to their health care provider about possible difficulties with the interpretation of certain blood tests when taking Oxbryta.

The most common side effects of Oxbryta include headache, diarrhea, stomach (abdominal) pain, nausea, tiredness, rash and fever. These are not all the possible side effects of Oxbryta.

Before taking Oxbryta, patients should tell their health care provider about all medical conditions, including if they have liver problems; if they are pregnant or plan to become pregnant as it is not known if Oxbryta can harm an unborn baby; or if they are breastfeeding or plan to breastfeed as it is not known if Oxbryta can pass into breastmilk or if it can harm a baby. Patients should not breastfeed during treatment with Oxbryta and for at least two weeks after the last dose.

Patients should tell their health care provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins and herbal supplements. Some medicines may affect how Oxbryta works. Oxbryta may also affect how other medicines work.

Patients are advised to call their doctor for medical advice about side effects. Side effects can be reported to the FDA at 1-800-FDA-1088. Side effects can also be reported to Global Blood Therapeutics at 1-833-428-4968 (1-833-GBT-4YOU).

Full Prescribing Information for Oxbryta is available at [Oxbryta.com](http://Oxbryta.com).

## About Global Blood Therapeutics

Global Blood Therapeutics (GBT) is a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments that provide hope to underserved patient communities. Founded in 2011, GBT is delivering on its goal to transform the treatment and care of sickle cell disease (SCD), a lifelong, devastating inherited blood disorder. The company has introduced Oxbryta<sup>®</sup> (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of red blood cell sickling in SCD. GBT is also advancing its pipeline program in SCD with inclacumab, a P-selectin inhibitor in development to address pain crises associated with the disease, and GBT021601, the company's next generation hemoglobin S polymerization inhibitor. In addition, GBT's drug discovery teams are working on new targets to develop the next wave of treatments for SCD. To learn more, please visit <https://gbt.com> and follow the company on Twitter @GBT\_news.

## Forward-Looking Statements

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995, including statements containing the words "will," "anticipates," "plans," "believes," "forecast," "estimates," "expects," and "intends," or similar expressions. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements in this press release may include statements that are not historical facts and are considered forward-looking 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. GBT intends these forward-looking statements, including statements regarding GBT's priorities, dedication, focus, goals and vision; safety, efficacy and mechanism of action of Oxbryta and other product characteristics; significance of reducing hemolysis and raising hemoglobin; commercialization, delivery, availability, use, and commercial and medical potential of Oxbryta; inferences drawn from study results and related analyses, including with respect to continued use of Oxbryta; growth of real-world evidence; ongoing and planned studies of Oxbryta and related protocols, activities and expectations; potential expansion of the approved use of Oxbryta for more patients in the U.S., and potential regulatory approval for Oxbryta to treat patients in Europe; altering the treatment, course and care of SCD and mitigating related complications; potential of GBT's pipeline, including inclacumab and other product candidates; and advancing GBT's pipeline, working on new targets and discovering, developing and delivering treatments, to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act, and GBT makes this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect GBT's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to the company and on assumptions the company has made. GBT 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 GBT's control including, without limitation, risks and uncertainties relating to the COVID-19 pandemic, including the extent and duration of the impact on GBT's business, including commercialization activities, regulatory efforts, research and development, corporate development activities and operating results, which will depend on future developments that are highly uncertain and cannot be accurately predicted, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease; the risks that GBT is continuing to establish its commercialization capabilities and may not be able to successfully commercialize Oxbryta; risks associated with GBT's dependence on third parties for development, manufacture and commercialization activities related to Oxbryta; government and third-party payor actions, including those 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; and the timing and progress of GBT's and Syros' research and development activities under their collaboration; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, and in GBT's 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 GBT's subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, GBT assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

## References

1. Centers for Disease Control and Prevention website. Sickle Cell Disease (SCD). <https://www.cdc.gov/ncbddd/sicklecell/data.html>. Accessed June 3, 2019.
2. European Medicines Agency. <https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182125>. Accessed June 12, 2020.
3. National Heart, Lung, and Blood Institute website. Sickle Cell Disease. <https://www.nhlbi.nih.gov/health-topics/sickle-cell-disease>. Accessed August 5, 2019.
4. Rees DC, et al. *Lancet*. 2010;376(9757):2018-2031.
5. Kato GJ, et al. *Nat Rev Dis Primers*. 2018;4:18010.
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8. Oxbryta (voxelotor) tablets prescribing information. South San Francisco, Calif. Global Blood Therapeutics, Inc.; November 2019.

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## GBT Presents Data on New Sickle Cell Disease Pipeline Therapies with Best-in-Class Potential – Inclacumab and GBT021601

*Two Inclacumab Pivotal Phase 3 Clinical Trials Expected to Begin in First Half of 2021*

*GBT021601 – Potent Next-Generation Hemoglobin S Polymerization Inhibitor Shown to be Highly Effective in SCD Animal Models*

**SOUTH SAN FRANCISCO, Calif., Dec. 06, 2020 (GLOBE NEWSWIRE)** -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced new preclinical data on its sickle cell disease (SCD) pipeline therapies – inclacumab, a novel P-selectin inhibitor in development to reduce the frequency of vaso-occlusive crises (VOCs) in patients with SCD, and GBT021601, a next-generation hemoglobin S (HbS) polymerization inhibitor. These data are being presented at the all-virtual 62<sup>nd</sup> American Society of Hematology (ASH) Annual Meeting and Exposition.

“In pursuit of our mission to transform the treatment of and care for people living with sickle cell disease, our research and development pipeline is targeting multiple pathologies, including vascular occlusion and hemoglobin polymerization,” said Ted W. Love, M.D., president and chief executive officer of GBT. “We’re very excited about the best-in-class potential of both inclacumab and GBT021601. In 2021, we plan to initiate two pivotal Phase 3 clinical trials evaluating inclacumab for its ability to reduce the frequency of VOCs and hospital readmissions caused by VOCs. In addition, this is the first time we are presenting data on GBT021601. These preclinical data are very promising, and we look forward to studying the safety and efficacy of this potentially innovative therapy in SCD patients once we enter the clinic as planned in the near future.”

### **Inclacumab: *In Vitro* Analysis and Phase 3 Clinical Study Program**

*In vitro* study results (Abstract #1707) demonstrated that inclacumab has the potential to be a best-in-class P-selectin inhibitor for reducing the frequency of VOCs in patients with SCD. When characterized alongside crizanlizumab, an FDA-approved P-selectin inhibitor for treatment of VOCs, inclacumab:

- Binds P-selectin at the natural ligand binding site and has an affinity similar to crizanlizumab,
- Demonstrated rapid binding kinetics to P-selectin and remained bound for longer, and
- Inhibited platelet-leukocyte aggregation to a greater extent than crizanlizumab.

Additionally, prior clinical experience with inclacumab in more than 700 non-SCD participants demonstrated the potential for a substantially longer duration of exposure and near complete inhibition of platelet-leukocyte aggregation over a 12-week period. Taken together, we believe these characteristics will translate into quarterly dosing, improved patient adherence, and the potential to expand use to a broader patient population.

In 2021, GBT plans to initiate two global, randomized, placebo-controlled pivotal Phase 3 trials evaluating safety and efficacy of inclacumab. These trials are designed to enhance understanding of how P-selectin inhibitors could provide clinical benefit for patients with SCD and reduce overall healthcare utilization. One study is designed to reduce the frequency of VOCs over one year in patients with SCD when treated with inclacumab (30 mg/kg) or placebo every 12 weeks. The second study will evaluate inclacumab based on a primary endpoint of 90-day hospital readmission rates following a VOC hospitalization. Participants in that trial will receive either a single dose of inclacumab (30 mg/kg) or placebo, peri-discharge following a VOC hospitalization. Approximately 50 percent of U.S. SCD patients with least two annual VOC events are re-admitted within 90 days following a VOC hospitalization.<sup>1</sup> Initiation of both trials is expected in the first half of 2021.

### **GBT021601: Preclinical Analysis of Next Generation HbS Polymerization Inhibitor**

Preclinical data (Abstract #1704) on GBT021601, a molecule discovered and designed by scientists at GBT, demonstrated its potential as a potent next-generation HbS polymerization inhibitor. GBT021601 has the same mechanism of action as Oxbryta<sup>®</sup> (voxelotor) tablets, but with the potential for greater efficacy by achieving higher hemoglobin (Hb) occupancy at significantly lower doses. The study showed that GBT021601 normalized Hb levels in Townes sickle cell mice. In addition, in this study GBT021601 was highly effective in:

- Reducing hemolysis,
- Prolonging red blood cell (RBC) lifespan,
- Improving RBC health, and
- Potentially improving organ function.

Following treatment with GBT021601, levels of erythropoietin (EPO), a hormone that plays a key role in the production of RBCs, did not change – indicating that the observed increase in Hb levels was safe and was not due to hypoxic response.

A Phase 1 clinical study on the safety and tolerability of GBT021601 in SCD patients is expected to begin by mid-2021.

GBT presentations from the ASH Annual Meeting will be available on the GBT website.

### **GBT Analyst & Investor Day Webcast Details**

GBT is hosting a virtual Analyst & Investor Day event tomorrow, Monday, December 7, at 4 p.m. PT to review data on Oxbryta



in patients with SCD and preclinical studies on inclacumab and GBT021601 presented at the 62<sup>nd</sup> ASH Annual Meeting and Exposition. The event will provide additional background on the Phase 3 study program for inclacumab. The webcast can be accessed directly at [www.gbtinvestorday.virtualeventsite.com](http://www.gbtinvestorday.virtualeventsite.com). Participants are requested to register in advance. A replay will be available for three months following the event on GBT's investor webpage at [www.gbt.com](http://www.gbt.com).

### **About Sickle Cell Disease**

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States,<sup>2</sup> an estimated 52,000 people in Europe,<sup>3</sup> and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.<sup>2</sup> It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.<sup>2</sup> SCD is a lifelong inherited rare blood disorder that impacts hemoglobin, a protein carried by red blood cells that delivers oxygen to tissues and organs throughout the body.<sup>4</sup> Due to a genetic mutation, individuals with SCD form abnormal hemoglobin known as sickle hemoglobin. Through a process called hemoglobin polymerization, red blood cells become sickled – deoxygenated, crescent-shaped, and rigid.<sup>4-6</sup> The sickling process causes hemolytic anemia (low hemoglobin due to red blood cell destruction) and blockages in capillaries and small blood vessels, which impede the flow of blood and oxygen throughout the body. The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.<sup>5-8</sup>

### **About Oxbryta<sup>®</sup> (voxelotor) Tablets**

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As a condition of accelerated approval, GBT will continue to study Oxbryta in the HOPE-KIDS 2 Study, a post-approval confirmatory study using transcranial Doppler (TCD) flow velocity to assess the ability of the therapy to decrease stroke risk in children 2 to 15 years of age.

In recognition of the critical need for new SCD treatments, the FDA granted Oxbryta Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD. Additionally, Oxbryta has been granted Priority Medicines (PRIME) designation from the European Medicines Agency (EMA), and the European Commission (EC) has designated Oxbryta as an orphan medicinal product for the treatment of patients with SCD.

GBT plans to seek regulatory approvals to expand the potential use of Oxbryta in the United States for the treatment of SCD in children as young as 4 years old, and to treat hemolytic anemia in SCD patients ages 12 years and older in Europe.

### **Important Safety Information**

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Patients receiving exchange transfusions should talk to their health care provider about possible difficulties with the interpretation of certain blood tests when taking Oxbryta.

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Patients should tell their health care provider about all the medicines they take, including prescription and over-the-counter medicines, vitamins and herbal supplements. Some medicines may affect how Oxbryta works. Oxbryta may also affect how other medicines work.

Patients are advised to call their doctor for medical advice about side effects. Side effects can be reported to the FDA at 1-800-FDA-1088. Side effects can also be reported to Global Blood Therapeutics at 1-833-428-4968 (1-833-GBT-4YOU).

Full Prescribing Information for Oxbryta is available at [Oxbryta.com](http://Oxbryta.com).

### **About Global Blood Therapeutics**

Global Blood Therapeutics (GBT) is a biopharmaceutical company dedicated to the discovery, development and delivery of life-changing treatments that provide hope to underserved patient communities. Founded in 2011, GBT is delivering on its goal to transform the treatment and care of sickle cell disease (SCD), a lifelong, devastating inherited blood disorder. The company has

introduced Oxbryta<sup>®</sup> (voxelotor), the first FDA-approved treatment that directly inhibits sickle hemoglobin polymerization, the root cause of red blood cell sickling in SCD. GBT is also advancing its pipeline program in SCD with inclacumab, a P-selectin inhibitor in development to address pain crises associated with the disease, and GBT021601, the company's next generation hemoglobin S polymerization inhibitor. In addition, GBT's drug discovery teams are working on new targets to develop the next wave of treatments for SCD. To learn more, please visit <https://gbt.com> and follow the company on Twitter @GBT\_news.

### Forward-Looking Statements

Certain statements in this press release are forward-looking within the meaning of the Private Securities Litigation Reform Act of 1995, including statements containing the words "will," "anticipates," "plans," "believes," "forecast," "estimates," "expects," and "intends," or similar expressions. These forward-looking statements are based on GBT's current expectations and actual results could differ materially. Statements in this press release may include statements that are not historical facts and are considered forward-looking 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. GBT intends these forward-looking statements, including statements regarding GBT's priorities, dedication, focus, goals, mission and vision; safety, efficacy and mechanism of action of Oxbryta and other product characteristics; commercialization, delivery, availability, use, and commercial and medical potential of Oxbryta; inferences drawn from study results and related analyses, including with respect to the potential of inclacumab and GBT021601; ongoing and planned studies of Oxbryta and drug candidates and related protocols, activities, timing and other expectations; potential expansion of the approved use of Oxbryta for more patients in the U.S., and potential regulatory approval for Oxbryta to treat patients in Europe; altering the treatment, course and care of SCD and mitigating related complications; potential of GBT's pipeline, including inclacumab and GBT021601; and advancing GBT's pipeline, working on new targets and discovering, developing and delivering treatments, to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act, and GBT makes this statement for purposes of complying with those safe harbor provisions. These forward-looking statements reflect GBT's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to the company and on assumptions the company has made. GBT 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 GBT's control including, without limitation, risks and uncertainties relating to the COVID-19 pandemic, including the extent and duration of the impact on GBT's business, including commercialization activities, regulatory efforts, research and development, corporate development activities and operating results, which will depend on future developments that are highly uncertain and cannot be accurately predicted, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the U.S. and in other countries, and the effectiveness of actions taken globally to contain and treat the disease; the risks that GBT is continuing to establish its commercialization capabilities and may not be able to successfully commercialize Oxbryta; risks associated with GBT's dependence on third parties for development, manufacture and commercialization activities related to Oxbryta; government and third-party payor actions, including those 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; and the timing and progress of GBT's and Syros' research and development activities under their collaboration; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2019, and in GBT's 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 GBT's subsequent filings with the U.S. Securities and Exchange Commission. Except as required by law, GBT assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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