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): July 22, 2021

GLOBAL BLOOD THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**001-37539**	**27-4825712**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(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:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
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 July 22, 2021, Global Blood Therapeutics, Inc. issued a press release titled "GBT Provides Regulatory and Pipeline Updates in Sickle Cell Disease (SCD)." A copy of the press release is attached as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Press Release dated July 22, 2021
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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: July 22, 2021

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

EXHIBIT 99.1

GBT Provides Regulatory and Pipeline Updates in Sickle Cell Disease (SCD)

Submits Supplemental New Drug Application to the U.S. FDA for Oxbryta® (voxelotor) in Children with SCD Ages 4 to 11

Initiates Phase 3 Clinical Trials of Inclacumab and Enrolls First SCD Patient in Phase 1 Study of GBT021601

SOUTH SAN FRANCISCO, Calif., July 22, 2021 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced updates across several of its development programs in sickle cell disease (SCD). The company has submitted a supplemental New Drug Application (sNDA) to the U.S. Food and Drug Administration (FDA) seeking accelerated approval for Oxbryta® (voxelotor) for the treatment of SCD in children ages 4 to 11 years, together with a related separate New Drug Application (NDA) required to seek approval for a pediatric weight-based formulation of Oxbryta. Additionally, GBT initiated two global, randomized, placebo-controlled, pivotal Phase 3 clinical trials of inclacumab, a novel P-selectin inhibitor. The company also enrolled the first SCD patient in a Phase 1 study evaluating GBT021601 (GBT601), a next-generation hemoglobin S (HbS) polymerization inhibitor, in people with SCD.

“Starting at a very early age, sickle cell disease has a serious and life-altering impact on children, and current therapeutic options for children under 12 years of age are limited. We believe there is significant potential to impact the longer-term outcomes by addressing the root cause of red blood cell sickling at a young age,” said Ted W. Love, M.D., president and CEO of GBT. “Our team is committed to working closely with the FDA to potentially expand access to Oxbryta, thereby providing hope to families.”

The Oxbryta pediatric sNDA and NDA include data from the open-label Phase 2a HOPE-KIDS 1 Study (GBT440-007). An analysis of data presented at the European Hematology Association (EHA) 2021 Virtual Congress in 45 children with SCD ages 4 to 11 years enrolled in the study showed that weight-based treatment with Oxbryta resulted in rapid and sustained improvements in hemoglobin. Concurrent improvements in markers of hemolysis also were observed. GBT requested priority review for the sNDA and NDA, which, if granted, could result in a six-month review process. The FDA has a 60-day filing review period to determine whether the sNDA and the NDA are complete and acceptable for filing.

Design and Initiation of Pivotal Phase 3 Clinical Trials of Inclacumab

The two randomized, double-blind, placebo-controlled Phase 3 clinical trials are evaluating the safety and efficacy of inclacumab for the treatment of vaso-occlusive crises (VOCs) associated with SCD. Both studies are enrolling individuals with SCD age 12 years and older who have experienced between two and 10 VOCs in the previous year. Inclacumab selectively targets P-selectin, a protein that mediates cell adhesion and is clinically validated to reduce pain crises.

GBT2104-131 (NCT04935879) is evaluating the effect of inclacumab on the frequency of VOCs. Approximately 240 patients will be randomized to an IV infusion of 30 mg/kg inclacumab or placebo every 12 weeks. The primary outcome of this Phase 3 trial is the rate of VOCs during the 48-week study period. Secondary outcomes include time to first and second VOCs, proportion of participants with no VOCs, rate of VOCs that required admission to a healthcare facility and duration of inpatient hospitalization for VOCs.

The second trial, GBT2104-132 (NCT04927247), is evaluating the effect of a single dose of inclacumab on hospital readmission rates. VOCs in SCD are a leading cause of hospital readmissions[1] and a significant burden on patients and healthcare resources. In this Phase 3 trial, approximately 280 participants who have been admitted to a healthcare facility due to a VOC will be randomized to receive a one-time IV infusion of 30 mg/kg of inclacumab or placebo in the peri-discharge window (just prior to, during or around discharge). The primary outcome is the rate of hospital readmissions for a VOC within 90 days following an initial hospitalization for a VOC. Secondary outcomes include readmission within 30 days, time to first hospital readmission for VOC and rate of VOCs leading to healthcare provider visits.

“Our goal is to transform sickle cell disease into a well-managed condition, and we are committed to advancing our pipeline of innovative therapies that address the multiple pathologies of this inherited disease in parallel with working to expand access to Oxbryta. Vaso-occlusive crises take a significant toll on patients and are a leading cause of hospital readmissions. We believe inclacumab could be a best-in-class therapeutic option, with the potential for less frequent dosing than existing treatments,” said Kim Smith-Whitley, M.D., executive vice president and head of research and development at GBT. “Our pipeline is the engine of our commitment to improving the lives of people living with this terrible disease. We are excited to initiate enrollment in our two pivotal studies for inclacumab, in parallel with our Phase 1 study of GBT601 in people with sickle cell disease.”

Design of Phase 1 Clinical Trial of GBT601

GBT is currently enrolling patients in GBT021601-012. This single and multiple ascending dose Phase 1 study is assessing the safety, tolerability, pharmacokinetics and pharmacodynamics of GBT601 in up to six people with SCD ages 18 to 60 years. Patients with hemoglobin levels between 5.5 g/dL and 10.5 g/dL are eligible for enrollment. The primary outcome is the safety and tolerability of GBT601 as assessed at 14 weeks. Secondary outcomes include measures of pharmacokinetics and pharmacodynamics, as well as an assessment of the relationship between GBT601 and measures of anemia and hemolysis. GBT plans to submit preliminary proof-of-concept data for GBT601 for presentation at a medical meeting this year.

Discovered and designed by GBT’s research and development team, GBT601 has the same mechanism of action as Oxbryta, with the potential for greater efficacy by achieving higher hemoglobin levels and occupancy at lower doses, as demonstrated in

preclinical studies. This study follows an ongoing first-in-human trial to determine safe and tolerable dosing.

About Sickle Cell Disease

Sickle cell disease (SCD) affects an estimated 100,000 people in the United States,[2] an estimated 52,000 people in Europe,[3] and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.[2] It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.[2] 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.[4] 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.[4-6] 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.[5-8]

About Oxbryta® (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, Oxbryta inhibits sickle hemoglobin 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 November 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.[9]

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. Also, in May 2021, Oxbryta was granted Promising Innovative Medicine (PIM) designation in the United Kingdom from the Medicines and Healthcare Products Regulatory Agency (MHRA).

The EMA has accepted for review GBT's Marketing Authorization Application (MAA) seeking full marketing authorization of Oxbryta in Europe to treat hemolytic anemia in SCD patients ages 12 years and older.

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 healthcare 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 healthcare 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 healthcare 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 2 weeks after the last dose.

Patients should tell their healthcare 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 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.

About Inclacumab

Inclacumab is a novel, fully human monoclonal antibody that selectively targets P-selectin, a protein that mediates cell adhesion and is clinically validated to reduce pain crises,[10] known as vaso-occlusive crises or VOCs, in people with sickle cell disease (SCD). Preclinical results suggest that inclacumab has the potential to be a best-in-class option for reducing VOCs in people with SCD, with the potential for quarterly, rather than monthly dosing. GBT has exclusive worldwide rights to inclacumab as part of

the company's licensing agreement with F. Hoffmann-La Roche Ltd. The safety, tolerability and pharmacokinetics of inclacumab have been evaluated by Roche in more than 700 non-SCD patients.

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® (voxelotor) tablets, 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 Phase 3 development to address pain crises associated with the disease, and GBT021601 (GBT601), 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 potential treatments for SCD. To learn more, please visit www.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, commitment, focus, goals, mission and vision; safety, efficacy and mechanism of action of Oxbryta and other product characteristics; significance of reducing sickling and hemolysis and raising hemoglobin; commercialization, delivery, availability, use and commercial and medical potential of Oxbryta; significance of data presented at the EHA Congress, including support for the use of Oxbryta in children; ongoing and planned studies and clinical trials and related protocols, activities and expectations; expanding access to Oxbryta, including related activities and expectations; regulatory submissions to potentially expand the approved use of Oxbryta for more patients and in a pediatric formulation in the U.S. and to treat patients in Europe and other territories, including potential review, timing and approval; altering the treatment, course and care of SCD and mitigating related complications; potential impact of addressing the root cause of red blood cell sickling at a young age; safety, efficacy, mechanism of action, advancement and potential of GBT's drug candidates and pipeline; and 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 research, development, manufacture, distribution and commercialization activities; government and third-party payer actions, including those 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 GBT's collaboration, license and distribution agreements; along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2020, 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

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Contact:
Steven Immergut (media)
650.410.3258
simmergut@gbt.com

Courtney Roberts (investors)
650.351.7881
croberts@gbt.com