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): June 6, 2022

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. ☐

In this report, "GBT," "Company," "we," "our," and "us" means Global Blood Therapeutics, Inc., and/or one or more of our subsidiaries, unless the context otherwise provides.

Item 8.01. Other Events.

On June 6, 2022, Global Blood Therapeutics, Inc. issued a press release titled "GBT's Inclacumab and GBT601 Receive U.S. FDA Orphan Drug and Rare Pediatric Disease Designations for the Treatment of Sickle Cell Disease." 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 June 6, 2022
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: June 6, 2022

By: /s/ Jeffrey Farrow
Jeffrey Farrow
Chief Financial Officer

GBT's Inclacumab and GBT601 Receive U.S. FDA Orphan Drug and Rare Pediatric Disease Designations for the Treatment of Sickle Cell Disease

Inclacumab is a novel P-selectin inhibitor currently in Phase 3 clinical trials to reduce vaso-occlusive crises (VOCs) and readmissions due to VOCs in patients with sickle cell disease

GBT601 is a next generation sickle hemoglobin (HbS) polymerization inhibitor expected to advance into Phase 2/3 trial by mid-year

SOUTH SAN FRANCISCO, Calif., June 06, 2022 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced that the U.S. Food and Drug Administration (FDA) has granted both orphan drug and rare pediatric disease designations for inclacumab and GBT021601 (GBT601) for the treatment of sickle cell disease (SCD). Inclacumab is a novel P-selectin inhibitor currently in Phase 3 clinical trials to evaluate its potential to reduce the occurrence of vaso-occlusive crises (VOCs) and readmissions due to VOCs in patients with SCD. GBT601, a next generation sickle hemoglobin (HbS) polymerization inhibitor, is being studied in a restarted Phase 1 clinical trial and is expected to advance into the Phase 2 portion of a Phase 2/3 trial by mid-year.

"The FDA's orphan drug and rare pediatric disease designations for both inclacumab and GBT601 are an acknowledgment of the critical and ongoing unmet need in sickle cell disease and the potential of GBT's innovative pipeline of investigational medicines," said Kim Smith-Whitley, M.D., executive vice president and head of research and development at GBT. "We believe that both inclacumab and GBT601 have the potential to be best-in-class therapeutic options for the treatment of this devasting disease. We are excited to continue these clinical development programs to make progress on our goal of transforming sickle cell disease into a well-managed condition via multiple therapeutic approaches."

VOCs take a significant toll on patients and are a leading cause of hospital readmissions in SCD.[1] Patient enrollment is currently ongoing in two randomized, double-blind, placebo-controlled Phase 3 clinical trials evaluating the safety and efficacy of inclacumab – dosed quarterly – for the potential treatment of VOCs associated with SCD. Both studies are enrolling individuals with SCD ages 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. The first registrational trial, GBT2104-131 (NCT04935879), is evaluating the effect of inclacumab on the frequency of VOCs. The second registrational trial, GBT2104-132 (NCT04927247), is evaluating the effect of a single dose of inclacumab on hospital readmission rates.

A Phase 1 study of six SCD patients demonstrated that GBT601, which has the same mechanism of action as Oxbryta® (voxelotor), achieved a high target hemoglobin occupancy at daily doses lower than 500 mg, while maintaining a favorable safety and tolerability profile. After patients who participated in the earlier Phase 1 study of GBT601 expressed interest in participating in a restart of the study at a higher dose, GBT recently restarted the Phase 1 study to assess a 150 mg daily dose, greater than the 100 mg daily maintenance dose previously studied. Upon completion, the company plans to submit these new data for presentation at a medical meeting later this year.

The FDA's orphan drug designation provides orphan status to drugs and biologics that are being developed to address rare diseases or disorders that affect fewer than 200,000 people in the U.S.[2] Orphan drug designation qualifies GBT for various incentives, including tax credits for qualified clinical trials and market exclusivity if it receives FDA approval. A rare pediatric disease designation is granted for rare diseases that primarily affect children under 18 years old, with recipients of this designation qualifying for a priority review voucher if the drug is approved and certain conditions are met. The priority review voucher may be redeemed, transferred, or sold.[3]

About Sickle Cell Disease

Sickle cell disease (SCD) affects more than 100,000 people in the United States,[4] an estimated 52,000 people in Europe,[5] and millions of people throughout the world, particularly among those whose ancestors are from sub-Saharan Africa.[6] It also affects people of Hispanic, South Asian, Southern European and Middle Eastern ancestry.[7] 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.[7] 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, crescent-shaped and rigid.[7,8,9] The recurrent sickling process causes destruction of the red blood cells and hemolytic anemia (low hemoglobin due to red blood cell destruction) and blockages in capillaries and small blood vessels (vaso-occlusion), which impede the flow of blood and oxygen delivery throughout the body, commonly referred to as vaso-occlusive crises (VOCs). The diminished oxygen delivery to tissues and organs can lead to life-threatening complications, including stroke and irreversible organ damage.[8,9,10,11] Complications of SCD begin in early childhood and can include neurocognitive impairment, acute chest syndrome, and overt stroke, among other serious issues.[12]

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 due to VOCs in people with 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 Roche. The safety, tolerability and pharmacokinetics of inclacumab have been evaluated by Roche in more than 700 non-SCD patients.

About GBT021601

Discovered and designed by GBT's research and development team, GBT021601 (GBT601) has the same mechanism of action as Oxbryta® (voxelotor), with the potential for greater efficacy by achieving higher hemoglobin levels and occupancy at lower doses. GBT601 is being studied in a single and multiple ascending dose Phase 1 study assessing the safety, tolerability, pharmacokinetics and pharmacodynamics of GBT601 in patients with SCD ages 18 to 60 years. The Phase 2 portion of a Phase 2/3 study of GBT601 is expected to initiate in mid-2022.

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, starting with sickle cell disease (SCD). Founded in 2011, GBT is delivering on its goal to transform the treatment and care of SCD, a lifelong, devastating inherited blood disorder. The company has introduced Oxbryta® (voxelotor), the first FDA-approved medicine that directly inhibits sickle hemoglobin (HbS) 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 HbS polymerization inhibitor. In addition, GBT's drug discovery teams are working on new targets to develop the next generation of 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, commitment, dedication, focus, goals, mission, vision, and positioning; the safety, efficacy, and mechanism of action of Oxbryta, and other product characteristics; the commercialization, awareness, delivery, availability, use, and commercial and medical potential of Oxbryta; ongoing and planned studies, clinical trials and registries, and related protocols, activities, timing, and other expectations; future presentations; impacting the treatment, care, and course of SCD and mitigating related complications; continuing clinical development programs and making progress on GBT's goals; safety, efficacy, mechanism of action, advancement and potential of inclacumab, GBT601 and GBT's other drug candidates and pipeline; the significance and potential benefits of the FDA's orphan drug and rare pediatric disease designations; 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, 2021, 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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