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

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# FORM 8-K

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## CURRENT REPORT

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

**Date of Report (Date of earliest event reported): October 21, 2019**

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## GLOBAL BLOOD THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

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| **Delaware** | **001-37539** | **27-4825712** |
|---|---|---|
| (State or Other Jurisdiction of Incorporation) | (Commission File Number) | (I.R.S. Employer Identification No.) |

**171 Oyster Point Blvd., Suite 300**
**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)

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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 October 21, 2019, Global Blood Therapeutics, Inc. issued a press release titled “GBT Announces Results of Post-hoc Analysis of Phase 3 HOPE Study Showing Improvement in Leg Ulcers in Patients with Sickle Cell Disease Treated with Voxelotor”. 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 No. | Description |
|---|---|
| 99.1 | Press Release, dated October 21, 2019 |
| 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: October 21, 2019

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

**EXHIBIT 99.1**

# GBT Announces Results of Post-hoc Analysis of Phase 3 HOPE Study Showing Improvement in Leg Ulcers in Patients with Sickle Cell Disease Treated with Voxelotor

*First of Several Post-hoc Analyses Planned Using HOPE Study Clinical Data*

*Findings Presented in Poster Session at 13th Annual Academy for Sickle Cell and Thalassemia Conference*

SOUTH SAN FRANCISCO, Calif., Oct. 21, 2019 (GLOBE NEWSWIRE) -- Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced results of a post-hoc analysis of data from the Phase 3 HOPE (Hemoglobin Oxygen Affinity Modulation to Inhibit HbS PolymErization) Study of voxelotor, an oral, once-daily therapy in development for the treatment of sickle cell disease (SCD). Results of this exploratory analysis suggest that voxelotor resolved or reduced the severity of existing leg ulcers and decreased the incidence of new leg ulcers in patients with SCD. The findings are being presented in a poster session at the 13th Annual Academy for Sickle Cell and Thalassemia (ASCAT) Conference in London.

Leg ulcers are a painful and often debilitating chronic complication of SCD, affecting up to 25% of patients. Chronic, nonhealing leg ulcers in SCD are thought to be caused by severe anemia and intravascular hemolysis (rupture or destruction of red blood cells within blood vessel walls). Although hydroxyurea and chronic transfusions are used to treat SCD, no controlled clinical study data have demonstrated the efficacy of these therapies in the treatment of leg ulcers in patients with SCD.

“We are encouraged by the results of this analysis of an exploratory endpoint of the HOPE Study because they not only suggest potential clinical benefit of voxelotor in leg ulcers, a serious complication of SCD, but they are also consistent with our understanding of how the improvement in anemia and hemolysis with voxelotor can potentially translate to an improvement in related clinical morbidity,” said Josh Lehrer, M.D., chief medical officer of GBT. “This is the first of several post-hoc analyses of the HOPE Study that we intend to conduct.”

The randomized, placebo-controlled HOPE Study compared voxelotor (1500 mg and 900 mg daily) with placebo in patients ages 12 and older with SCD. Results, which were published in *The New England Journal of Medicine* , showed rapid and sustained improvements in hemoglobin levels and markers of hemolysis with voxelotor compared with placebo.

The exploratory post-hoc analysis included data from 17 patients who had at least one SCD-related leg ulcer either at the time of trial initiation (n=13) or developed one over the 24-week treatment period (n=4). Results showed the following:

- All four patients treated with voxelotor 1500 mg had their leg ulcers resolve or improve. Three patients had their leg ulcers resolve, and one patient showed improvement from moderate to mild. Additionally, no patients receiving voxelotor 1500 mg reported new leg ulcers after starting treatment.
- Among six patients with leg ulcers at the start of treatment with voxelotor 900 mg, three patients had their leg ulcers resolve, one patient showed improvement from moderate to mild, and two patients showed no change in ulcer severity. Additionally, two patients who did not have leg ulcers at the start of treatment developed them during the study.
- None of the patients treated with placebo had their leg ulcers improve. Three patients in the placebo group who had leg ulcers at the start of treatment showed no change in ulcer severity, and two patients who did not have leg ulcers at the start of treatment developed them during the study.
- Resolution of leg ulcers was associated with a hemoglobin occupancy of approximately 20% or higher, within the target range for inhibition of sickle hemoglobin polymerization.

GBT’s New Drug Application (NDA) for voxelotor is currently under Priority Review by the U.S. Food and Drug Administration (FDA), which provides for a six-month review, and has been assigned a Prescription Drug User Fee Act (PDUFA) target action date of February 26, 2020. The NDA for voxelotor is supported by data from the Phase 3 HOPE Study.

**About Sickle Cell Disease**
SCD is a lifelong inherited blood disorder caused by a genetic mutation in the beta-chain of hemoglobin, which leads to the formation of abnormal hemoglobin known as sickle hemoglobin (HbS). In its deoxygenated state, HbS has a propensity to polymerize, or bind together, forming long, rigid rods within a red blood cell (RBC). The polymer rods deform RBCs to assume a sickled shape and to become inflexible, which causes hemolytic anemia (low hemoglobin due to RBC destruction) that can lead to multi-organ damage and early death. This sickling process also causes blockage in capillaries and small blood vessels. Beginning in childhood, SCD patients typically suffer unpredictable and recurrent episodes or crises of severe pain due to blocked blood flow to organs, which often lead to psychosocial and physical disabilities.

**About Voxelotor in Sickle Cell Disease**
Voxelotor (previously called GBT440) is being developed as an oral, once-daily therapy for patients with SCD. Voxelotor works by increasing hemoglobin's affinity for oxygen. Since oxygenated sickle hemoglobin does not polymerize, voxelotor blocks polymerization and the resultant sickling and destruction of red blood cells. With the potential to improve hemolytic anemia and oxygen delivery, GBT believes that voxelotor may potentially modify the course of SCD. In recognition of the critical need for new SCD treatments, the U.S. Food and Drug Administration (FDA) has granted voxelotor Breakthrough Therapy, Fast Track, Orphan Drug and Rare Pediatric Disease designations for the treatment of patients with SCD. The European Medicines Agency (EMA) has included voxelotor in its Priority Medicines (PRIME) program, and the European Commission (EC) has designated voxelotor as an orphan medicinal product for the treatment of patients with SCD.

GBT is currently evaluating voxelotor in the HOPE (Hemoglobin Oxygen Affinity Modulation to Inhibit HbS PolymErization) Study, a Phase 3 clinical study in patients age 12 and older with SCD. Additionally, voxelotor is being studied in the ongoing Phase 2a HOPE-KIDS 1 Study, an open-label, single- and multiple-dose study in pediatric patients (age 4 to 17) with SCD. The HOPE-KIDS 1 Study is assessing the safety, tolerability, pharmacokinetics and exploratory treatment effect of voxelotor.

**About GBT**
GBT is a clinical-stage biopharmaceutical company determined to discover, develop and deliver innovative treatments that provide hope to underserved patient communities. GBT is developing two therapies for the potential treatment of sickle cell disease, including its late-stage product candidate, voxelotor, as an oral, once-daily therapy. 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 about GBT's development plans for voxelotor and the potential benefits of voxelotor for SCD patients and other statements containing the words "anticipate," "planned," "believe," "forecast," "estimated," "expected," and "intend," among others. 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 the availability of, and sufficiency of data to support, accelerated regulatory approval, the therapeutic potential and safety profile of voxelotor, including the potential to be a disease-modifying therapy for SCD, the potential for voxelotor to be approved and to become a new standard of care for treating adolescents and adults with SCD, the ability to implement and complete clinical development plans for voxelotor, the ability to generate and report data from our past, ongoing and potential future studies of voxelotor, regulatory review and actions relating to voxelotor, the potential commercial launch of voxelotor, and the timing of these events, 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 GBT's 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, the risks that GBT's clinical and preclinical development activities may be delayed or terminated for a variety of reasons, that results of clinical trials may be subject to differing interpretations, that regulatory authorities may disagree with GBT's clinical development plans or require additional studies or data to support further clinical investigation of GBT's product candidates, that drug-related adverse events may be observed in clinical development, and that data and results may not meet regulatory requirements or otherwise be sufficient for further development, regulatory review or approval, along with those risks set forth in GBT's Annual Report on Form 10-K for the fiscal year ended December 31, 2018, and in GBT's Quarterly Report on Form 10-Q for the quarter ended June 30, 2019, 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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