

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

SCHEDULE 14A INFORMATION

**Proxy Statement Pursuant to Section 14(a) of the
Securities Exchange Act of 1934
(Amendment No.)**

Filed by the Registrant

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Check the appropriate box:

- Preliminary Proxy Statement
- Confidential, for Use of the Commission Only** (as permitted by Rule 14a-6(e)(2))
- Definitive Proxy Statement
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Global Blood Therapeutics, Inc.
(Name of Registrant as Specified In Its Charter)

(Name of Person(s) Filing Proxy Statement, if other than the Registrant)

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- No fee required.
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(1) Title of each class of securities to which transaction applies:

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To our shareholders,

Eight years ago, Global Blood Therapeutics began a journey to transform the treatment of sickle cell disease (SCD) and the lives of people living with this devastating, lifelong condition. In 2015, we first entered clinical testing, and in 2019, we achieved a momentous milestone on our journey with the introduction of Oxbryta[®] (voxelotor) tablets. Oxbryta is the first and only FDA-approved sickle hemoglobin polymerization inhibitor – a new class of therapy. We are proud that our clinical development program advanced so efficiently, and we are delighted that Oxbryta provides patients a medicine that directly inhibits hemoglobin polymerization, an underlying cause of SCD. GBT is driven by our mission to revolutionize the treatment of SCD and other grievous blood disorders.

A New Treatment for Patients in Need

Oxbryta received accelerated approval for the treatment of SCD in adults and children 12 years of age and older on November 25, 2019 – three months ahead of its priority review action date, underscoring the strong Oxbryta data package we provided to the FDA. Along the development path, we worked closely with the FDA and key investigators to design and implement a regulatory and clinical approach that was designed to meet the needs of the SCD community – in a way that had not been seen before.

Oxbryta was approved based on the results of the Phase 3 HOPE (Hemoglobin Oxygen Affinity Modulation to Inhibit HbS PolymErization) Study, which were published in the *New England Journal of Medicine* and presented at the 24th European Hematology Association (EHA) Congress in June 2019. The study demonstrated that Oxbryta provides clinically meaningful and statistically significant improvements in hemoglobin levels as a result of significant reduction in red blood cell destruction (hemolysis).

“Today’s approval provides additional hope to the 100,000 people in the U.S., and the more than 20 million globally, who live with this debilitating disorder.”

– Admiral Brett P. Giroir, M.D.
Former Acting FDA Commissioner
HHS Assistant Secretary for Health ¹

Prior to Oxbryta, the treatment of SCD was focused on alleviating the intense pain, known as vaso-occlusive crises, or VOCs, despite the fact that the majority of patients do not experience these pain crises. ² With Oxbryta, we are providing a therapy that can potentially benefit a much larger population of SCD patients by addressing the underlying causes of SCD with a once-daily, oral medication. This represents a significant shift in the treatment paradigm, with the potential to address the underlying cause of SCD that can lead to severe and undetected long-term harm, such as organ damage and failure, stroke and shortened lifespan.

Delivering for Patients and the SCD Community

Innovative medicines are only impactful if they are accessible. In 2019, we transitioned into a commercial company as we began the successful launch of Oxbryta. We are proud that we were able to make Oxbryta available just days after its early approval by the FDA. In fact, our comprehensive patient support program, GBT Source[™], was up and running the same day Oxbryta was approved.

As an organization, executing flawlessly on our strategy is a top priority. We have received exciting early feedback from patients, physicians and payers, and our planned activities are tracking to our expectations. We believe Oxbryta has the potential to become a new standard of care for people living with SCD.

“After decades of waiting, we now have a treatment option that could change the course of this disease.”

– Beverley Francis-Gibson, President and CEO
Sickle Cell Disease Association of America ³

As we continue to work to make this a reality, our launch strategy is grounded in the following three key elements:

- **Exceptional People and Culture:** We have built a team of smart, experienced and passionate individuals committed to making a difference in SCD. Our field team of sickle cell therapeutic specialists (SCTS) is complemented by our access and patient support, marketing and operations teams.
- **Robust Stakeholder Education:** Prior to approval, our teams began educating physicians, patients, advocates and payers about the importance of preventing sickle hemoglobin polymerization, red blood cell sickling and hemolysis. Immediately following approval, our SCTSs shifted focus to educating healthcare providers on Oxbryta's approved label. This effort is augmented by our medical affairs function, which has a team of medical science liaisons who focus on educating key opinion leaders on the scientific rationale and evidence supporting Oxbryta. On the payer front, we are actively meeting with all key organizations to inform their clinical reviews of Oxbryta, with a goal of achieving broad coverage by end of year.
- **Comprehensive Support Programs:** As mentioned, we launched GBT Source, our high-touch patient support program, on the day of approval. GBT Source is the first SCD support program specifically developed based on input from the patient community and with their needs in mind. It provides wide-ranging support for patients and physicians with comprehensive financial and educational programs, as well as administrative support for physicians and payers, to facilitate patients' ability to access the medicine as quickly as possible. It is supported by our team of field-based patient navigators who provide in-person education and training on GBT Source for patients and physician offices.

Ongoing Community Involvement

In tandem with our efforts to get Oxbryta into the hands of patients, we are also shining a light on SCD, a disease that has long been neglected and ignored. We are building awareness and educating key stakeholders while also advancing initiatives that address disparities in access and quality care that SCD patients face in their daily lives. Since our founding, we have focused on developing deep relationships with the SCD community. We have implemented robust programs, including two SCD-focused conferences that we support every year; our Access to Excellent Care for Sickle Cell Patients (ACCEL) grant program, which has provided more than \$200,000 in funding to U.S.-based nonprofit organizations focused on improving care for those with SCD; and two national awareness campaigns aimed at educating patients and physicians about SCD. In response to the COVID-19 pandemic, we provided more than \$250,000 in support through the newly created GBT Community Fund, as well as a direct donation to the Sickle Cell Disease Association of America, to address critical needs for basic services and funding for patients and families in the SCD community.

Transforming the Treatment Paradigm for SCD

Our vision is to make SCD a well-managed chronic disease. We are actively working to establish Oxbryta as standard of care as well as to advance innovation with next-generation therapies and new pathways for SCD.

We believe that Oxbryta has the potential to improve outcomes for all patients with SCD, given that it targets the underlying cause of the disease. To drive broad adoption over time, we are undertaking a robust post-approval clinical program aimed at expanding Oxbryta's label, including children below the age of 12, and building the evidence to demonstrate Oxbryta's full efficacy. This includes the Phase 3 HOPE-KIDS 2 study, our confirmatory study evaluating the effect of Oxbryta in reducing the risk of stroke. Other key studies that are ongoing or planned include the HOPE-KIDS 1 study, the recently initiated dose-escalation study to explore efficacy and tolerability of higher doses, and the ActiVe study to evaluate the effect of Oxbryta on physical activity and quality of life. As with others in the industry, enrollment in these studies has been paused due to public health measures around COVID-19, but long-term study timelines remain on track.

Beyond Oxbryta, we are building a strong pipeline in SCD. Our most advanced candidate is inclacumab, a novel fully human monoclonal antibody against p-selectin designed to reduce painful VOCs. We expect to begin a pivotal clinical study for inclacumab in the first half of 2021. In addition, in December, we announced a

collaboration with Syros Pharmaceuticals, Inc., to discover, develop and commercialize novel therapies for SCD and beta thalassemia based on the induction of fetal hemoglobin. This gives us the potential to advance other innovative approaches into clinical studies that have the potential to meaningfully improve the lives of people with SCD. We also continue to work in our own labs – where Oxbryta was discovered and developed – on potential next generation small molecule product candidates.

Bringing Hope to SCD Patients Globally

While our current focus is getting Oxbryta to patients in the United States, we are also committed to addressing SCD around the world. It is a major global public health issue, with millions of people of African, Middle Eastern and South Asian descent suffering with limited treatment options. In Europe, we are working to define the regulatory pathway that we hope will enable us to get Oxbryta to patients there. Outside of Europe, we are developing a strategy to ensure long-term, sustainable access across the spectrum of healthcare delivery systems, particularly in the complex and underserved areas of Africa, Asia and South America.

Looking Ahead

While we still have a lot of work to do to achieve our long-term goals, we are off to a promising start with the launch of Oxbryta in the United States, and we feel we are on our way to realize our vision of making SCD a well-managed chronic disease.

2019 was a major step forward, and our optimism for GBT's future carries over to 2020. We must acknowledge that in this current year, we are navigating through an unprecedented time with the COVID-19 pandemic. For GBT, the health and safety of the sickle cell community and of our employees are our highest priority. Proactively, we have taken steps to support public health strategies designed to stem the spread of the virus, including social distancing, while at the same time working to ensure uninterrupted access for patients who are prescribed Oxbryta and support for the entire sickle cell community.

Our entire team is driven by the same purpose: our commitment to patients and the opportunity to change the lives of millions of people around the world. I want to thank our shareholders – along with the SCD community, our employees, clinical investigators, practicing healthcare professionals, our business partners and our Board of Directors – for the ongoing support of our company and our cause.

With kind regards,



Ted W. Love, M.D.
President and Chief Executive Officer
Global Blood Therapeutics, Inc.

1. Food and Drug Administration press release, November 25, 2019.

<https://www.fda.gov/news-events/press-announcements/fda-approves-novel-treatment-target-abnormality-sickle-cell-disease>.

2. Shah, N. et al. *Plos One*. 2019; 14(7): e0214355.

3. Global Blood Therapeutics press release, November 25, 2019. <https://ir.gbt.com/news-releases/news-release-details/fda-approves-oxbrytvm-voxelotor-first-medicine-specifically>.