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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Dear Shareholders,

2020 was a historic year for GBT, headlined by the successful launch of our first medicine, Oxbryta® (voxelotor) tablets and significant progress on our R&D pipeline, which we believe are important steps towards achieving our goal of transforming sickle cell disease (SCD) into a well-managed condition. Guided by our mission and values, our team demonstrated resilience during a year broadly defined by a global pandemic that had a profound impact on society. The initial months of the Oxbryta launch exceeded expectations, but when the COVID-19 pandemic struck, the disproportionate impact on communities of color and high-risk patient groups, including people living with SCD, became clear. COVID-19 magnified the ongoing disparities in healthcare during a period when issues of racial injustice gained worldwide attention – all of which deeply affected patients with SCD.

As I reflect on 2020, I’m extremely proud of the way GBT responded. In mid-March, with the health and safety of patients, healthcare professionals and employees as a top priority, we made the decision to temporarily withdraw our field teams from all in-person interactions. We took this step in the interest of public health, especially for the most vulnerable populations, including those with SCD.

In an extraordinarily difficult year for the SCD community, we are proud to have helped in a variety of ways, including providing much needed financial support. We established the GBT Community Fund to support the acute needs of SCD patients and their families during the pandemic. GBT also made an immediate donation to the Sickle Cell Disease Association of America (SCDAA) in response to an urgent call for its COVID-19 Emergency Fund.

Our total funding to SCD organizations in 2020 exceeded $2 million, including more than $350,000 in contributions from GBT, our board of directors and our employees. We also proudly supported non-profit organizations through our ongoing Access to Excellent Care for Sickle Cell Patients (ACCEL) Grant Program, which provides grant funding to accelerate the development of sustainable access to care programs for people with SCD.

Key Achievements in 2020
Despite the unique challenges of 2020, we executed on our strategic plan to evolve GBT into a successful commercial-stage organization with a robust pipeline in SCD and a values-driven, people-focused culture.

Based on the strong fundamentals of Oxbryta, which was approved by the U.S. Food and Drug Administration in late 2019 for the treatment of SCD in adults and children 12 years of age and older, in 2020 we built a solid foundation for success – driving broad awareness and early adoption.

We generated nearly 5,000 new prescriptions from launch through 2020 and achieved net revenues of $123.8 million for the year from sales of Oxbryta, making it the most successful launch in SCD history. In addition, we established broad payer coverage one quarter ahead of our goal, demonstrating that payers understand the benefits of Oxbryta. Through our dedicated patient support program, GBT Source®, we've educational efforts to make it easier for healthcare providers to prescribe and patients to start and stay on Oxbryta.

A growing body of data continues to reinforce the potential for Oxbryta as a foundational treatment in SCD. At the 2020 American Society of Hematology (ASH) Annual Meeting, we presented the complete 72-week analysis of the Phase 3 HOPE Study demonstrating durable improvements in hemoglobin and hemolysis. We also presented real-world experience with Oxbryta, which importantly showed that both physicians and patients are seeing improvements.
in overall health status. In 2021, we have rolled out new educational materials to help patients and physicians understand the efficacy and safety profile of Oxbryta.

Expanding Access to Oxbryta

A top priority for GBT is to make Oxbryta available to more patients, including younger children with SCD. We believe that mitigating red blood cell sickling and destruction early in life will modify the course of the disease and alleviate future serious and life-threatening complications. In the U.S., we enrolled the first patients in our pediatric expanded access program, and we plan to submit a regulatory filing to potentially expand the Oxbryta label in the United States to include children ages 4 to 11 years.

We also initiated an early access program in Europe and other regions outside the United States, providing Oxbryta for eligible SCD patients as we work towards our goal of securing regulatory approvals around the world. To this end, we are pleased that the European Medicines Agency (EMA) is currently reviewing our Marketing Authorization Application (MAA) for potential approval of Oxbryta to treat hemolytic anemia in SCD patients ages 12 years and older. In the six Gulf Cooperation Council (GCC) countries in the Middle East, we are working with our distributor, Biopharma-MEA, to make Oxbryta available. We also intend to establish a similar distributorship for Latin America, including Brazil, where the bulk of the region's SCD patients live. We are confident that Oxbryta will become a standard of care for treatment of SCD worldwide, and we believe we can expand access to more than 350,000 patients in the next several years.

Innovative Pipeline

In 2020, GBT advanced our exciting lead pipeline programs – inclacumab for vaso-occlusive crises (VOCs) and GBT021601 (GBT601) – both of which have the potential to be best-in-class SCD therapies.

Inclacumab is a P-selectin inhibitor that binds to a unique epitope on P-selectin that allows it to deliver more potency. We believe this will provide a competitive advantage by potentially enabling quarterly dosing compared to the currently available option, which has monthly intravenous dosing. We plan to initiate two global, randomized, placebo-controlled pivotal Phase 3 trials evaluating the safety and efficacy of inclacumab in the first half of 2021. One trial will focus on the reduction in VOCs and the second will focus on reducing 90-day hospital readmission following an initial VOC hospitalization. The two studies are carefully designed to potentially enable two independent regulatory submissions for separate indications.

GBT601 is a next-generation hemoglobin S polymerization inhibitor, the same mechanism as Oxbryta. Preclinical data shows that it has the potential to normalize hemoglobin through improved red blood cell survival and organ function. We believe this profile gives GBT601 the potential for greater efficacy by achieving higher hemoglobin occupancy at significantly lower doses. In December, we initiated a first-in-human Phase 1 study on the safety and tolerability of GBT601 in healthy volunteers. We plan to begin studying GBT601 in SCD patients in 2021, with a goal of providing proof-of-concept data by the end of the year.

In addition, GBT's drug discovery teams are working on new targets to develop the next wave of treatments for SCD. We plan to expand our pipeline through our active business development efforts and our established collaborations, including our research collaboration with Syros Pharmaceuticals and our recent in-license of two early-stage programs from Sanofi.

In 2020, we continued to strengthen our leadership team with the appointment of Rajiv Patni, M.D., as chief medical officer, and the announcement that SCD thought leader and advocate Kim Smith-Whitley, M.D., will join GBT in May of this year as executive vice president and head of research and development. We also recently added Alexis Thompson, M.D., M.P.H., a world-renowned hematologist and former president of ASH, to our board of directors.

Values-driven Culture

At GBT, we live our cultural values of accountability, inclusion, innovation and community, and, since our inception, we've worked to create a culture fostering workplace development, diversity, and inclusion (WDDI). In 2020, we continued to build a diverse team of talented professionals that enrich our culture and that will drive our future growth and success. We have made diversity a priority for our company, and it is woven into the cultural fabric of the company. More than half of GBT’s employees
are women and more than half are people of color. In addition, we were pleased to be one of only 380 companies, including 22 in healthcare, included in the 2021 Bloomberg Gender Equality Index, which measures gender equality across five pillars: female leadership and talent pipeline, equal pay and gender pay parity, inclusive culture, sexual harassment policies, and pro-women brand.

Other prestigious organizations recognized GBT in 2020 for the progress we've made to improve the lives of people with SCD, who have been ignored for far too long. These included the National Organization for Rare Disease (NORD) Rare Impact Award for Industry Innovation, the Xconomy National Award for Breakthrough Drug of the Year, and the nomination of Oxbryta for the Prix Galien Award for Best Biotechnology Product. These recognitions fuel our ambition to create lasting change for patients.

Delivering on the Mission: 2021 and Beyond
2021 is a milestone year for GBT as we celebrate the 10-year anniversary of our company’s founding. From the beginning, we focused on sickle cell disease, and we are more committed and better equipped than ever before to deliver on our mission in our second decade. We recognize that there is much more to be done. Despite recent progress in innovation, significant barriers remain in helping people with SCD get the high-quality care they need and deserve. We remain humbled and dedicated to helping those living with SCD and their families, and grateful to our shareholders for supporting this critical work.

As we look ahead, our vision is to become the leader in SCD and other underserved orphan disease communities. To help realize this vision, we must establish Oxbryta as standard of care, ensuring optimum real-world experience and excellence throughout our anticipated global launches, as well as advance our R&D pipeline. In addition, our commitment to the SCD community extends to low-resource countries, including the goal of developing distribution and funding approaches in sub-Saharan Africa and other regions. Ultimately, we will not rest until we can offer underserved patients what they have been asking for decades — a chance for a healthier, happier and longer life.

We appreciate all those who continue to support and inspire this mission: our patient advocacy and development partners, healthcare providers and, above all, patients. Our achievements and our future success could not be possible without the contributions of our GBT employees around the world, who continue to demonstrate incredible resolve despite the many challenges from COVID-19. And, I would like to thank you, our shareholders, for your support and being part of our continued growth. We look forward to keeping you informed on our progress.

Sincerely,

Ted W. Love, M.D.
President and Chief Executive Officer
Global Blood Therapeutics, Inc.
March 31, 2021