

Global Blood Therapeutics, Inc.
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April 29, 2019

UNITED STATES
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SCHEDULE 14A INFORMATION
Proxy Statement Pursuant to Section 14(a) of the
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To my fellow shareholders,

Fueled by compassion, the GBT team made tremendous advances in support of the sickle cell disease (SCD) community last year. Two significant regulatory achievements, the strength of data from our Phase 3 HOPE Study of voxelotor and our conviction that SCD patients deserve far better treatment options continue to propel us. I would like to recap highlights from 2018 and preview our plans for 2019.

Our Accelerated Approval Pathway

We began 2018 with exciting news. The U.S. Food and Drug Administration (FDA) granted voxelotor, our lead product candidate, Breakthrough Therapy Designation – the first ever for an investigational treatment for SCD. Eleven months later, we announced that the FDA agreed with our proposal seeking approval for voxelotor under an accelerated approval pathway. We plan to file a New Drug Application (NDA) for voxelotor in the second half of 2019 under this pathway on the basis of hemoglobin increase as our primary endpoint. I am proud of the progress we have made toward our goal of making voxelotor, a potentially disease-modifying therapy, commercially available to the SCD community. We believe voxelotor has the potential to become the new standard of care in the treatment of SCD.

Significance of Hemolytic Anemia and Red Blood Cell Destruction

SCD is a chronic, life-limiting, inherited blood disorder affecting an estimated 100,000 people in the United States and 60,000 people in Europe. SCD causes the destruction of red blood cells (hemolysis). SCD is also known as sickle cell anemia because every SCD patient suffers from anemia (too few red blood cells). Voxelotor works by stopping the sickle hemoglobin from destroying red blood cells. As a result, we believe voxelotor improves hemolytic anemia and oxygen delivery to body organs.

Medical and scientific literature indicate that improvement in anemia can be life-changing for SCD patients. Specifically, in December 2018, at the 60th American Society of Hematology (ASH) Annual Meeting & Exposition, data were presented from a literature review showing that a 1 gram per deciliter (g/dL) increase in hemoglobin could reduce the risk of stroke by 41 percent, the risk of kidney failure by 53 percent and the risk of pulmonary hypertension by 57 percent. Even more significant, the risk of death might decrease by 64 percent. Thus, we are very excited about voxelotor's potential to be transformative for SCD patients.

In 2018, we presented positive clinical trial results from our Phase 2a HOPE-KIDS 1 Study and Phase 3 HOPE Study of voxelotor in SCD:

In June, at the 23rd European Hematology Association (EHA) Congress, we presented 24-week data from the ongoing HOPE-KIDS 1 Study in adolescents ages 6 to 17 treated with a 900 mg dose of voxelotor. Results demonstrated sustained and durable improvements in hemoglobin levels and a reduction in hemolysis with voxelotor. Based on the encouraging safety profile, the Data Safety Monitoring Board recommended expanding dosing to children as young as four years of age.

Also in June, we announced 12-week data from the HOPE Study in patients ages 12 and older. Results demonstrated that 58 percent of patients on a 1500 mg dose of voxelotor exceeded a 1 g/dL increase in hemoglobin at week 12. Statistically significant improvements were observed in hemoglobin, reticulocytes and bilirubin, demonstrating an improvement in hemolysis and anemia.

In December, at ASH, we presented 24-week data from the HOPE Study. Results demonstrated that 65 percent of patients who received a 1500 mg dose of voxelotor had a greater than 1 g/dL increase in hemoglobin at week 24. Overall, voxelotor produced statistically significant, rapid and robust improvements in anemia and hemolysis.

Changing the Course of SCD in Children

Children do not show signs of SCD at birth. Up until approximately nine months of age, they are protected by the presence of fetal hemoglobin. We anticipate that our ongoing pediatric clinical program will, over time, evaluate voxelotor in children as young as nine months old. Our vision is that, as this natural protection from SCD wanes, administering voxelotor as early as nine months of age will protect against progression of the disease.

Voxelotor also holds promise for minimizing the risk of stroke in children, which is 250 times more common in children with SCD than in the general pediatric population. Anemia is the single most important predictor of stroke in SCD patients who have no prior history. We plan to conduct a post-approval confirmatory study to demonstrate the stroke risk reduction benefit of voxelotor. The FDA agreed that using transcranial doppler (TCD) flow velocity is an acceptable primary endpoint for this study. TCD ultrasound is a noninvasive technique that uses sound waves to evaluate blood flow to the brain.

We are excited by the potential to modify the progression of SCD at an early age, and the potential to minimize the risk of stroke in children. Our commitment to children with SCD further deepens our commitment to the SCD community.

Community Involvement Informs Our Mission

Community involvement gives us insight into the challenges people living with SCD face. We have also made it a practice to listen and facilitate knowledge sharing among healthcare providers, drug developers, community-based organizations, people living with SCD and policymakers. In September 2018, for example, we hosted the first Access to Care Summit. We brought together 100 members of the SCD community to share success stories and discuss solutions to address barriers faced by patients in receiving comprehensive, life-long, quality healthcare. We also convened the 7th Annual SCD Therapeutics Conference, which focused on the latest medical advances and potential future treatments for SCD.

In our view, there is not enough coordinated, comprehensive care for people with SCD. To change that, we are putting the person with SCD at the center of how we approach patient care. We want to foster a greater understanding of the disease with the hope of reducing the discrimination and stigma that people with SCD frequently encounter when they seek care. We want to encourage government policy makers to allocate resources to enable the delivery of high-quality care that people with SCD deserve. Our approach is focused on education, outreach and advocacy.

Building Our Commercial Capabilities

We are building our team in anticipation of the potential FDA approval and launch of voxelotor in the first half of 2020. In 2018, we recruited two industry veterans to lead our commercial organization and access-to-care efforts, including David Johnson, a former Gilead Sciences leader who we hired in March 2018 as our chief commercial officer. In August, Heidi Wagner joined us from Alexion Pharmaceuticals as senior vice president, government affairs and policy to help SCD patients gain access to voxelotor and coordinated health care services. In addition, we appointed Dawn Svoronos to our board of directors in December 2018. Dawn brings invaluable expertise from more than 30 years of global experience in the biopharmaceutical industry, including 25 years at Merck, where she helped commercialize many new treatments.

In the coming months, we plan to fill many commercial positions as well as medical science liaisons to efficiently cover the states where SCD is most prevalent. Our growing team will help build awareness of the implications of hemolytic anemia and hemolysis, as well as the potential of voxelotor as a first-in-class, disease-modifying therapy that could benefit the SCD community.

Expanding our Pipeline

Our goal is to be an independent and sustainable company by expanding our development pipeline through our business development and internal research efforts. In 2018, we expanded our SCD pipeline by in-licensing inclacumab, a novel anti-P-selectin monoclonal antibody from Roche. We plan to develop inclacumab as a treatment for vaso-occlusive crises in patients with SCD. We anticipate beginning our pivotal study with inclacumab in early 2021. Inclacumab is a strong complement to voxelotor, providing us with potentially two new therapies for SCD patients.

In addition, we recently hired Brian Cathers, Ph.D., a seasoned discovery and research leader from Celgene, as our chief scientific officer. Brian will lead our in-house research team.

Our business development and in-house research teams have a common set of criteria to identify potential therapeutic opportunities. Both teams are focused on well-validated targets that have the potential like voxelotor to address unmet medical needs and change the standard of care.

Our Hope

I have the great fortune to lead a company of diverse, talented and dedicated employees who are the best in the biotech industry. I am grateful for their tireless innovations and contributions. As we prepare to file our first NDA, we imagine the day when SCD is well treated, and the devastating consequences of SCD are no longer. We are hopeful this day will come soon for the SCD community, and that GBT plays an important role in transforming the way SCD is treated. We appreciate your ongoing support of our company and our cause.

With kind regards,

Ted W. Love, M.D.

President and Chief Executive Officer

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