# New GBT Multinational Survey Shines a Needed Light on the Misunderstood Realities, Unseen Burden and Care Challenges of Sickle Cell Disease

*Sickle Cell Health Awareness, Perspectives and Experiences (SHAPE) survey developed with international steering committee of medical experts and patient advocates*

**SOUTH SAN FRANCISCO, Calif., – June 13, 2022 –** Global Blood Therapeutics, Inc. (GBT) (NASDAQ: GBT) today announced results from the **S**ickle Cell **H**ealth **A**wareness, **P**erspectives and **E**xperiences (SHAPE) survey, a multinational survey on the burden of sickle cell disease (SCD) and unmet needs as reported by more than 1,300 patients, caregivers and healthcare professionals (HCPs) in 10 countries. The findings highlight the significant impact of SCD on everyday life for patients, including fatigue, pain and mental health problems, in addition to impacting their ability to attend and be successful at school or work, and, thereby, reducing their earning potential.

One of the largest global burden of disease surveys conducted in SCD, SHAPE identified long-term health complications of SCD as a key concern among patients and HCPs. The survey also revealed that SCD patient caregivers face profound physical, psychosocial, and economic burdens resulting from taking care of people living with the disease.[1,2]

"Sickle cell disease is a lifelong condition that causes damage in the body and has a profound impact on the quality of life of those who suffer from it and their caregivers. The SHAPE survey is important because it illustrates how vital it is that we understand our patients' needs, and it suggests what we within the medical community can do to help change perspectives, increase education and awareness, and improve care," said Dr. Baba Inusa, professor and consultant of paediatric haematology, Guy's and St Thomas' NHS Foundation Trust, London and chair of the National Haemoglobinopathy Panel in England. "These results are a wake-up call, and I believe that the actions that follow can enable us to help drive a better dialogue and improved conversations around the management and care of this long-neglected and devastating disease."

**SHAPE Survey HCP Results**

The findings from the HCP portion of the survey were presented during a poster presentation on Friday, June 10, 2022, at the European Hematology Association (EHA) 2022 Hybrid Congress.[1] SHAPE highlighted the complex environment of treating people living with SCD. The survey results suggest the need for more education and tools to address the long-term impact of the disease and underpin the need for improved overall awareness and understanding of SCD. Key results from this portion of the study include:

- Despite fatigue/tiredness being identified as having the biggest impact on patients' lives (84%), only half (53%) of HCPs felt they have effective tools to treat this symptom.

- Most HCPs (84%) believed that treating hemolytic anemia (low hemoglobin due to red blood cell destruction) can improve most aspects of SCD, including reducing organ damage.
- Most HCPs (78%) felt confident in their knowledge of the long-term effects of SCD, but 83% wanted more support in educating their patients on this topic.
- Nearly one third (31%) of HCPs found it challenging to understand patients' concerns, with 43% citing difficulties due to having different ethnic background from their patients.
- Two-thirds (66%) of HCPs felt that patients do not always understand the long-term effects of SCD, and more than one third (37%) were reluctant to raise potential future challenges or complications with a patient or their family.
- Most HCPs (82%) agreed that SCD causes financial difficulties for patients, which may consequently affect their access to care.

**SHAPE Survey Patient and Caregiver Results**

The findings from patients and caregivers highlight that the realities of SCD for both patients and caregivers extend beyond the significant pain often experienced by patients and comprise a broad, lifelong series of physical, emotional and social barriers.

"SCD is the reality of so many people around the world, and yet patients often experience poor care, especially by non-specialist healthcare providers who may lack the training to provide good comprehensive care. In recent years, we've started to see some positive change, but it is still not enough. The challenges for patients seem endless and this survey highlights that, in spite of this, patients do not feel heard. They continue to face acute physical but also emotional damage, stigma and discrimination." said Dianaba Ba, who lives with SCD and is director of operations, SOS GLOBI: The Federation of Sickle Cell and Thalassemic Patients, an SCD patient advocacy organization in France.

Results from the patient and caregiver portions of the survey, which will be featured in an oral presentation on Friday, June 17, 2022 (16:00 – 17:45 CEST), at the 4th Global Congress on Sickle Cell Disease,[2] include:

- Most patients (93%) agreed that reducing their risk of long-term health issues due to SCD, such as organ damage, was important to them.
- Fatigue/tiredness was a symptom experienced by most patients (84%) in the past year, which also had the greatest negative impact on their ability to attend and be successful at school or work, and, thereby, on their earning potential.
    - Patients reported that due to SCD they missed an average of 7.5 days of school/work in the past month.
- Mental health symptoms affected the majority of patients with SCD in the past year, including 62% who reported feeling depressed, low mood or down, and 57% who indicated they felt worry or nervousness.
- Patients reported more positive experiences with specialist HCPs as compared with emergency departments and general HCPs.
    - More than half (54%) of SCD patients reported they believe they had experienced poor care in the past from emergency medicine professionals due to such providers' lack of knowledge about SCD.
- Over half of caregivers reported that patient symptoms negatively impact their own lives, such as their career and education (56%), long-term health prospects (55%) and overall well-being (53%).

“Our aim for this survey is to add to the body of knowledge around better understanding and characterizing the misunderstood, under-reported experiences and unmet needs of people living with SCD, as well as those caring for and treating people with the condition,” said Kim Smith-Whitley, M.D., executive vice president and head of research and development of GBT. “These results provide the opportunity to leverage multinational perspectives and experiences to help drive more attention about sickle cell disease and improve dialogue around the treatment, management and care of these patients. GBT is committed to being part of shaping change within the global SCD community, and we are grateful to all those who participated in and contributed to this important research.”

Members of the SHAPE survey Steering Committee of SCD expert clinicians and patient advocates include:

- Alan Anderson, M.D., University of South Carolina School of Medicine Greenville; Comprehensive Sickle Cell Program, Prisma-Health Upstate, Greenville, SC, USA
- Ashley Clark, Sickle Cell Association of America (SCDAA) (formerly)
- Baba Inusa, M.D., Paediatric Haematology, Evelina Children's Hospital, Guy's and St Thomas' Hospital, London, United Kingdom
- Biba Tinga, Sickle Cell Disease Association of Canada
- Dianaba Ba, SOS GLOBI: The Federation of Sickle Cell and Thalassemic Patients, France; sickle cell patient
- Elvie Ingoli, German Sickle Cell Disease and Thalassaemia Association
- Fernando Ferreira Costa, M.D., Ph.D., Haematology and Haemotherapy Centre, School of Medicine, University of Campinas - UNICAMP, Campinas, São Paulo, Brazil
- Isaac Odame, MB, ChB, Department of Hematology/Oncology, The Hospital for Sick Children, Toronto, Canada
- Joachim B. Kunz, M.D., Department of Pediatric Oncology, Hematology and Immunology, Hopp Children’s Cancer Center (KiTZ) Heidelberg, University of Heidelberg, Heidelberg, Germany
- John James, OBE, Sickle Cell Society, London, UK
- Mariane de Montalembert, M.D., Hôpital universitaire Necker-Enfants maladies, Paris, France
- Wasil Jastaniah, MBBS, King Faisal Specialist Hospital & Research Center, Jeddah, Kingdom of Saudi Arabia
- Zakareya Al Kadhem, Bahrain Society for Sickle Cell Anaemia Patient Care

**About the SHAPE Survey**

Commissioned by GBT, the SHAPE survey was conducted by Ipsos Healthcare, a global health research agency dedicated to understanding the motivations, behaviors and influences of the multiple actors in the healthcare sector. Quantitative surveys of 919 patients, 207 caregivers and 219 HCPs were conducted in 10 countries (Bahrain, Brazil, Canada, France, Germany, Oman, Saudi Arabia, United Arab Emirates, United Kingdom, and the United States of America), with fieldwork conducted between September 2021 and February 2022. Eligible patients had to be aged 12 or over, caregivers had to be 18 or over and HCPs had to care for at least 10 SCD patients.

For more information on the SHAPE survey visit https://www.gbt.com/shape.

**About Sickle Cell Disease**
Sickle cell disease (SCD) affects more than 100,000 people in the United States,[3] an estimated 52,000 people in Europe,[4] up to 100,000 people in Brazil,[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. When sickle hemoglobin becomes deoxygenated, it polymerizes to form rods, which deforms the red blood cells into sickled – crescent-shaped, rigid – cells.[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 silent and overt stroke, among other serious issues.[12]

**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, dedication, commitment, focus, goals, mission, vision and positioning; safety, efficacy and mechanism of action of Oxbryta and other product characteristics; significance of the SHAPE survey and related findings and possible future activities; commercialization, delivery, availability, use and commercial and medical potential of Oxbryta; impacting the treatment, course and care of SCD; safety, efficacy, mechanism of action, advancement and potential of GBT's drug candidates and pipeline; 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.

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