



Building Trust in Sickle Cell Clinical Development Through Authentic Patient Partnership

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Contents

03 Foreword	10 Introduction	34 Recommendations
04 Disclosures & Acknowledgments	22 Methodology	36 Calls-to-action
05 Snapshot	26 Results	38 Authors
06 Executive Summary	33 Conclusion	42 References

GLOSSARY	
ASCAT	Academy for Sickle Cell and Thalassemia
EMA	European Medicines Agency
EU	European Union
FDA	Food & Drug Administration
LISTEN	Learning and Insights into Sickle Cell Trial Experiences
QoL	quality of life
SCD	Sickle cell disease
SCDAA	Sickle Cell Disease Association of America
US	United States

Foreword

No Progress Without Partnership

For far too long, people living with sickle cell disease (SCD) have existed at the intersection of profound clinical need and systemic inequity.

The result is a fracture in the relationship between communities and the systems meant to serve them.

This fracture has been shaped by more than just a shortage of effective treatments; it has been shaped by lived experience. Experiences that have been well documented, but have long been known within SCD communities. Moments of not being believed. Care that arrived too late. The need to fight to be seen, heard, and treated equitably.

It is no coincidence that many people living with SCD describe themselves as “warriors,” having spent years fighting not only the disease itself, but also for recognition, belief, and equitable care. Over time, these experiences accumulate, shaping expectations of healthcare and research.

This is where progress must begin.

In many areas of healthcare, trust is assumed to start from a neutral position. In SCD, and in many communities that have experienced marginalization, that is often not the case. Instead, interactions begin with a trust deficit, shaped by history, lived experience, and unmet expectations.

This reality is often described as mistrust. Yet mistrust is not simply a barrier to overcome. It is often a rational response to what people have seen and experienced. Framing it solely as mistrust risks placing responsibility on communities rather than on the systems, structures, and decisions that contributed to its development.

Acknowledging that the trust deficit is the responsibility of the healthcare system reframes the challenge. It recognizes that accountability extends across industry, clinicians, researchers, and institutions alike. It makes clear that trust cannot be requested or presumed. It must be earned through consistent action. Progress in SCD, as in other areas marked by inequity, will depend on the willingness of the system to first establish credibility and demonstrate a genuine commitment to improving outcomes.

Partnership is central to that effort. The trust deficit can be addressed only through relationships built step by step, grounded in shared purpose, mutual respect, and accountability. Without such partnership, progress will continue to fall short.

This paper is therefore a call to action. Drawing on RISE UP, a clinical development program co-created through partnership between Agios and people living with SCD, it demonstrates what meaningful partnership can look like in practice. It calls on industry to commit to partnership from the outset, ensuring that people living with SCD help shape decisions rather than simply respond to them. It challenges stakeholders to reconsider where trust begins, how it is rebuilt, and whose voices are essential to that process. The path forward is not obscure. It is already visible. What remains is the collective willingness to follow it and make partnership the standard on which progress depends.

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Disclosures & Acknowledgements

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The RISE UP clinical trial and campaign would not have been possible without the generosity, honesty, and commitment of the people who shared their lived experience to help shape this work.

To the warriors, advocates, caregivers, healthcare professionals, and community leaders who contributed throughout this journey, thank you. Your insights helped ensure RISE UP was grounded in the real-world experiences of people living with sickle cell disease and shaped a more meaningful approach to research, care, and community engagement.

We are deeply grateful for your trust, collaboration, and partnership. This clinical trial, campaign, and white paper are a reflection of your voices, experiences, and shared commitment to a better future for the sickle cell community.

Thank you!



Snapshot

Sickle cell disease (SCD) is a rare, inherited blood disorder that affects millions of people worldwide, disproportionately impacting communities of color and representing a significant area of unmet medical need.

Hemolysis (or destruction of red blood cells) is the underlying driver of SCD complications, resulting from the release of cellular debris and harmful byproducts that contribute to the blockage of blood vessels essential for delivering oxygen to vital organs. As a result, patients may experience debilitating symptoms, including chronic anemia, acute and chronic pain, fatigue, and an increased risk of life-threatening complications resulting from organ damage.

Until recently, despite this significant burden, progress in SCD research and treatment development has historically been slow. Decades of neglect and underinvestment, driven in part by systemic inequities and racial disparities in healthcare, have limited innovation and industry engagement. This history of discrimination has also been reflected in clinical development, through interrupted programs and trials that often faced challenges to adequately address the realities of living with SCD. Together, these setbacks have contributed to a longstanding trust deficit among affected communities and created additional barriers to clinical trial participation.

Against this backdrop of systemic discrimination, a changing regulatory environment, and a more active patient community advocating for change, Agios, a pharmaceutical company focused on rare diseases, recognized that meaningful progress in SCD would require more than scientific innovation alone.

The company understood that a longstanding challenge to consistently listen to and partner with patients had contributed to mistrust and missed opportunities to design research around what matters most to those living with the disease. Rather than viewing patients solely as trial participants, Agios sought to engage them as active partners throughout the development process.

The experience suggests that when patients are engaged as equal partners in shaping meaningful decisions, trust is strengthened, trial design becomes more relevant, and research is better aligned with patient needs and priorities. This initiative demonstrates the value of patient partnership, particularly in rare diseases and underserved communities where meaningful patient involvement continues to lag.

Agios calls on industry, regulators, healthcare professionals, and patient advocacy organizations to move beyond acknowledging the importance of patient engagement and take action to embed patient partnership as a standard practice in research and development.



RISE UP

Executive Summary

Sickle cell disease (SCD) is an inherited blood disorder that affects millions of people worldwide, disproportionately impacting communities of color and representing a significant area of unmet medical need.

SCD is caused by mutations in hemoglobin, a protein that carries oxygen in red blood cells throughout the body.¹ Accumulation and destruction (“hemolysis”) of these abnormal red blood cells result in the release of cellular debris and harmful byproducts that contribute to the blockage of blood vessels essential for delivering oxygen to vital organs. Hemolysis is one of the key drivers of SCD complications associated with debilitating symptoms, such as episodes of severe pain, fatigue, and life-threatening complications resulting from organ damage.^{1,2} In the United States (US), life expectancy of US adults with SCD continues to be more than two decades shorter than the general population.²

100,000 - 120,000

SCD affects approximately 100,000–120,000 people in the United States (US).^{3,4}

7.7m

Affects an estimated 7.7 million people worldwide.⁵

Its impact is disproportionately high in certain regions, including sub-Saharan Africa, the Middle East, India, the eastern Mediterranean, and parts of South and Central America.⁶

SCD is associated with a multitude of health challenges, exacerbated by slow development of new medicines, poor or delayed access to screening, treatment, and/or comprehensive care, lack of physician education on effective treatment strategies, and lack of research on the physical and psychosocial impacts of SCD beyond pain.^{7,8}

“**People often think SCD is just about pain, but it’s so much more. The disease can affect anywhere there’s blood flow—so it’s all-encompassing, physically and mentally.”**



Blaze Armon Eppinger
Sickle Cell Warrior

Despite the significant unmet need, progress in SCD research has historically been limited by neglect and underfunding, driven in part by deep-rooted systemic inequities and racial disparities within healthcare systems, which have contributed to longstanding mistrust among affected communities.

Historic challenges with SCD clinical trial enrollment have been attributed to a well-documented history of stigma, racism, inequity, and medical mistreatment within healthcare systems, leading to deep mistrust among predominantly African American communities. These communities have experienced both complicit and implicit bias in the healthcare system, where they have been dismissed, not believed, or labelled as “drug-seeking” rather than receiving care.⁹ Feelings of being marginalized have fueled the belief that their lives are undervalued within the healthcare system, thus eroding trust into a deficit and inclining them to avoid participation in clinical research.⁹

These factors compound the burden of an already debilitating disease, creating a “double burden” and contributing to high clinical trial termination rates, which hinder the development of promising therapies.

“This is a community that has endured years of injustice. There is a protective barrier they’ve learned to erect and getting through that barrier is a matter of proving yourself in some way, showing yourself to be trustworthy, demonstrating that you care.”



Wally R. Smith
Sickle Cell Healthcare
Provider

“We knew we needed to take an entirely new approach if we were to begin to rebuild trust and co-create research that will benefit SCD patients and their caregivers.”



Holly John
Agios

Against this backdrop of systemic discrimination, and a changing regulatory environment, Agios, a pharmaceutical company focused on rare diseases, recognized the need for a different approach to clinical development in sickle cell.

When Agios started its SCD drug development program, it recognized a convergence of significant unmet need and longstanding systemic challenges—healthcare and regulatory expectations were evolving, with patients calling for more meaningful involvement, and agencies such as the US Food & Drug Administration (FDA) and European Medicines Agency (EMA) placing increased emphasis on integrating patient perspectives.^{10, 11} Agios understood that successful clinical development in SCD required partnering with patients (called “warriors” due to their daily battles against SCD), recognizing that patient engagement needed to move beyond traditional, transactional models, instead involving patients as partners.

With this in mind, Agios established an advisory board of warriors and advocates who were involved across the entire process, from naming the clinical trial to contributing to the development of meaningful endpoints and shaping clinical trial recruitment and awareness programs.

The experience suggests that when patients are engaged as partners, shaping meaningful decisions, trust rebuilds and clinical trials are more likely to prioritize patients’ needs and reach study completion. The encouraging results from this initiative underscore the importance of patient partnership, particularly in rare diseases and diverse communities, where patient involvement continues to lag.

Contributed to Naming of the Clinical Program, “RISE UP”, to Reflect the Desire to Change SCD Research from the Inside

Included Patient-Relevant Clinical Endpoints, Fatigue and Health-Related Quality of Life and Performance Outcome Assessments, in the Trial Protocol

Increased Community Engagement and Interest in RISE UP Trial by Featuring Warriors in a Clinical Trial Recruitment Campaign

Rapidly Achieved Patient Recruitment Goals

Decreased Burden of Trial Participation by Easing Financial and Logistical Obstacles

Enhanced Patient Engagement with Trial-Related Information

Recognized by International and National Medical Communities

Engaging patients as equal partners from the outset contributed to the design of a SCD patient-centric clinical trial in an area where many have historically faced challenges, to support the investigation of a potential new treatment option for patients. The experience in SCD reflects a broader, well-established shift across healthcare, supported by a growing body of evidence showing that patient partnership leads to stronger engagement, and more informed research.

These findings highlight the importance of early and active patient participation, particularly in rare diseases where patient collaboration remains low.

Agios calls on stakeholders across the healthcare ecosystem to move beyond acknowledgment and take action to embed patient partnership as standard practice.

Introduction

FACTS AND FIGURES

WHAT IS SCD?

- An inherited blood disorder that causes abnormal, sickle-shaped red blood cells.¹
- Through a process called “hemolysis”, cellular debris and harmful byproducts are released into the bloodstream, contributing to the blockage of blood vessels and driving SCD complications, including damage to vital organs, the leading cause of death in adults with SCD.^{12, 13}

SYMPTOMS & COMPLICATIONS^{1, 14, 15}

Key symptoms:

- Anemia
- Delayed growth
- Chronic fatigue
- Pain crises (episodes of severe pain)
- Painful swelling
- Jaundice

Key complications:

- Cardiopulmonary disease
- Central nervous system disease
- Eye complications
- Infections
- Kidney disease

GLOBAL AND UNITED STATES IMPACT

Worldwide:

- Affects an estimated 7.7 million people
- Disproportionately high impact in certain regions, including sub-Saharan Africa, Middle East, India, eastern Mediterranean, and parts of South and Central America^{6, 16}
- 300,000 babies born annually with SCD, 80% in sub-Saharan Africa
- 12th leading cause of death in children under 5 years of age⁵

In United States:

- Affects an estimated 100,000-120,000 people³
- Disproportionate birth prevalence in Black or African American people:
 - 1 in 350 non-Hispanic Black/African American (vs. 1 in 2,070 overall)¹⁷
 - 1 in 13 are carriers
- More than two decades’ shorter life expectancy than general population: 52.6 vs. 79.0 years, respectively^{2, 18}

Sickle cell disease (SCD) is a serious genetic blood disorder that continues to affect millions globally, despite medical advances.

SCD is an inherited blood disorder caused by a genetic mutation that results in abnormal, sickle- (or crescent-) shaped red blood cells when the cells are depleted of oxygen.¹ SCD occurs in people who inherit two mutated sickle cell genes, one from each parent. Those who inherit only one sickle cell gene are known as “carriers” and do not have symptoms of SCD but can pass the gene to their children.^{6, 15}

In people with SCD, premature destruction (“hemolysis”) of abnormal red blood cells leaks cellular debris and harmful byproducts into the bloodstream, triggering cycles of inflammation within the blood vessels.¹²

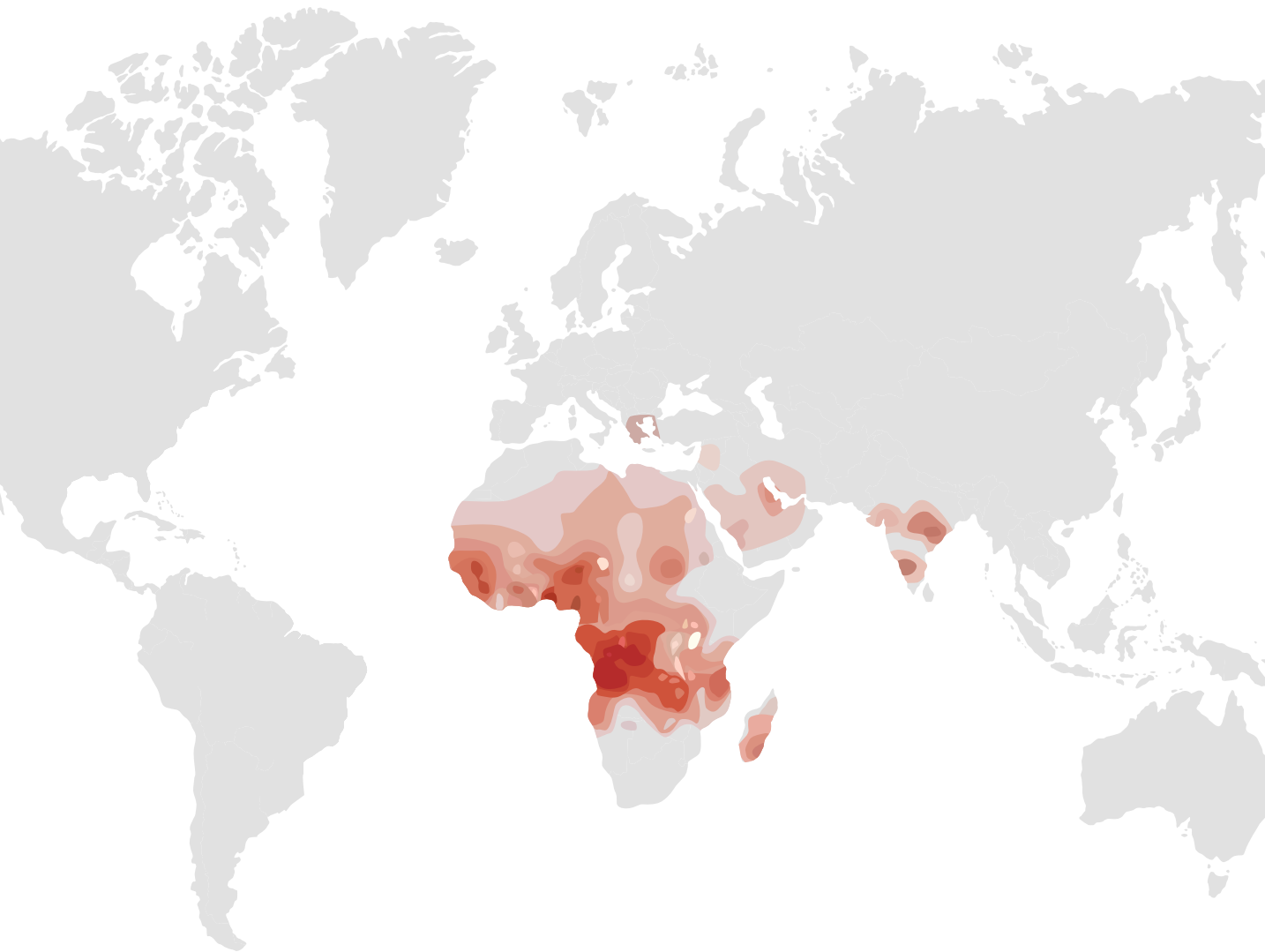
As part of the inflammatory process, both the damaged red blood cells and the tissues lining the blood vessels become sticky, trapping cells within the vessels and causing blood thickening and loss of oxygen supply, which collectively lead to chronic anemia, acute and chronic pain, fatigue, and chronic damage to vital organs, the most common cause of death in adults with SCD.^{12, 19, 20} For example, complications affecting the organs can include diseases affecting the heart and/or lungs, central nervous system disease, eye complications, infections,

and kidney disease.^{1, 14} The impact on survival is substantial, with life expectancy for individuals with SCD in the United States (US) estimated to be more than 20 years shorter than average (52.6 vs. 79.0 years, respectively),^{2, 13} while quality-adjusted life expectancy is reduced by more than 30 years.^{2, 13} Aside from mortality, SCD is a serious and debilitating condition that can substantially impair quality of life due to recurring episodes of severe pain (known as pain crises), anemia, ongoing fatigue, jaundice, painful swelling, and delayed growth.^{1, 14, 15}

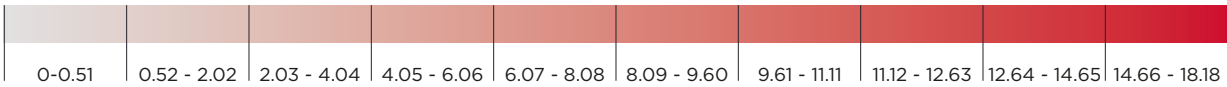
SCD affects an estimated 7.7 million people worldwide.⁵ Its prevalence overlaps with malaria-ridden regions of sub-Saharan Africa, the Middle East, India, the eastern Mediterranean, and parts of South and Central America (Figure 1), where being a carrier of SCD may offer protection from malarial infection.^{6, 16} Of approximately 300,000 babies born with SCD each year, 80% are in sub-Saharan Africa.²¹ Prevalence of SCD is expected to increase to approximately 400,000 annual SCD births by 2050, due to global migration of at-risk populations, along with improved surveillance and disease management in high-income countries.^{21, 22} Within the US, approximately 100,000-120,000 people have SCD,³ 90% of whom are non-Hispanic black or African American.¹⁷ While the overall SCD birth prevalence is 1 in 2,070 US newborns, Black or African American babies are disproportionately affected: 1 in 350 are born with SCD,^{3, 17} and 1 in 13 are carriers.^{6, 16, 23}



FIGURE 1: GLOBAL DISTRIBUTION OF THE SICKLE CELL DISEASE



HbS allele frequency (%)

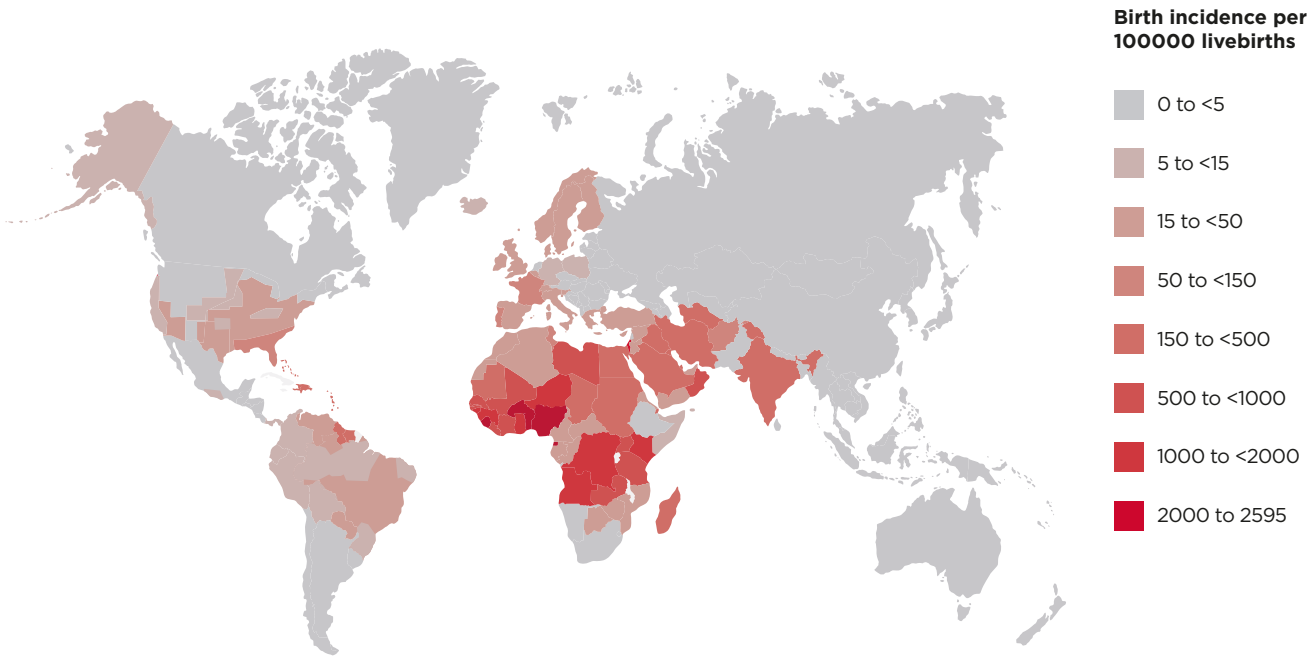


Left Panel: Adapted from Global distribution of the sickle cell gene and geographical confirmation of the malaria hypothesis by Piel et al.,¹⁶ licensed under Creative Commons (CC) BY-NC-SA 3.0.

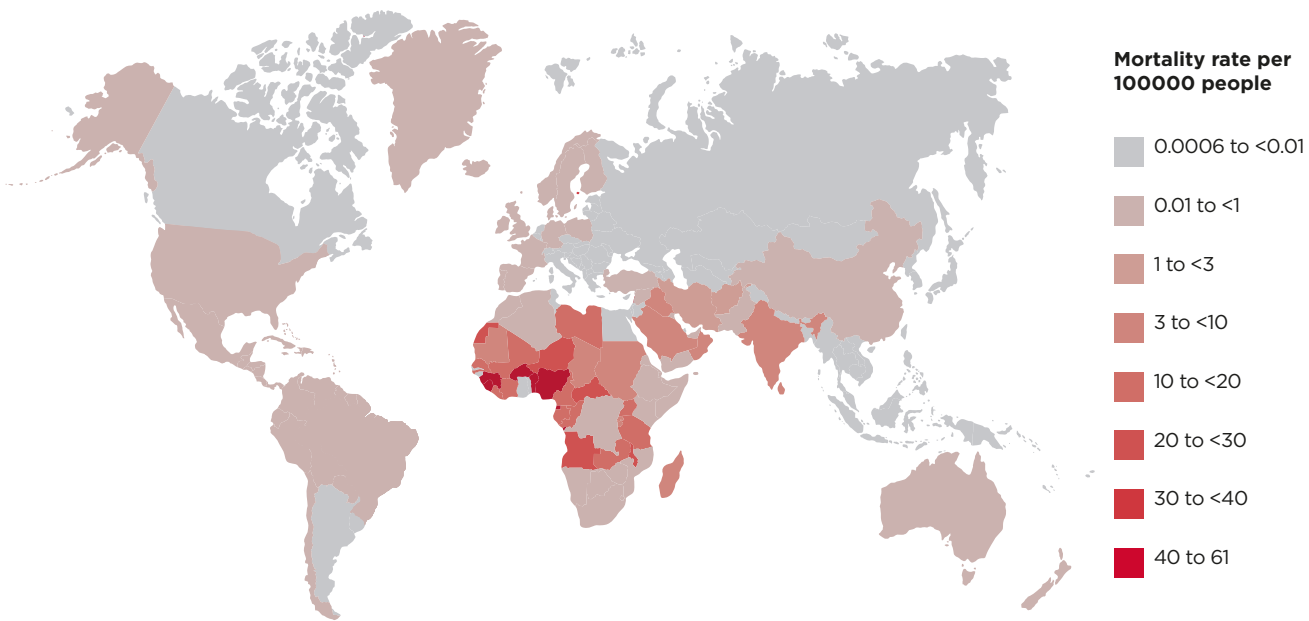
Right Panels: Adapted from Global, regional, and national prevalence and mortality burden of sickle cell disease, 2000–2021: a systematic analysis from the Global Burden of Disease Study 2021 by Global Burden of Disease 2021 Sickle Cell Disease Collaborators,⁵ licensed under CC-BY.

FIGURE 2: GLOBAL DISTRIBUTION OF SICKLE CELL DISEASE MEASURES OF (A) BIRTH INCIDENCE AND (B) MORTALITY RATE

A. BIRTH INCIDENCE, MALES AND FEMALES, 2021



B. ALL-AGE TOTAL SICKLE CELL DISEASE MORTALITY RATE, MALES AND FEMALES, 2021



SCD is a life-threatening disorder that causes premature mortality.

In low- and middle-income countries, the vast majority (50-90%) of children with SCD die before the age of 5 years, with bacterial sepsis being the leading cause of childhood mortality.^{24, 25}

Globally, an estimated 81,100 children with SCD under 5 years of age died in 2021, making SCD the 12th leading cause of death in that age group.⁵

In high-income countries, research and clinical practice milestones over the last 50 years (e.g., universal newborn screening, early education for parents of SCD children,

prophylactic antibiotics, disease management strategies, and development of new treatments) have transformed SCD from a life-threatening pediatric disease into a chronic disease of adults.²⁶ Yet, life expectancy of US adults with SCD remains more than two decades shorter than the general population, at 52.6 years of age compared with 79.0 years, respectively.^{2, 18} SCD-related deaths are most commonly caused by complications of the heart or other vital organs, driven by underlying hemolysis, blocked blood vessels, and inflammation in tissues.^{12, 19, 20}

People with SCD live with a range of debilitating impacts.

For people with SCD who survive into adulthood, SCD can significantly diminish quality of life (QoL), with chronic pain, hospitalizations, and potential disabilities contributing to anxiety, depression, and other mental health issues.²⁷ In addition, a 2019 global survey of over 2,000 warriors showed substantial QoL impact of SCD-related pain and fatigue on activities of daily living, including housework, exercise, social life, and relationships, with symptom burden (particularly pain crises) having the

highest impact on daily life.²⁸ Survey participants also reported SCD had a negative impact on education and the ability to work, resulting in negative economic consequences.²⁸ In another 2011 published US-based study of 1,046 warriors, QoL worsened with specific complications (pain crisis, asthma), as well as dependence on opioids and anti-depressants, with acute and chronic pain impairing QoL more than any other SCD-related complication.²⁹

SCD is complex to manage, underscoring the need for more treatment options.

Because symptoms and complications of SCD can vary from person to person,²⁷ its treatment can be complicated, requiring special care from different types of healthcare professionals to handle both immediate and long-term problems. Persons affected by SCD also need help with the physical, emotional, and social challenges that the disease brings to them and their families.¹⁰ They also need specialists who can help coordinate management strategies for SCD, including both comprehensive and preventive care, such as pain management, vaccination, genetic counseling, and specialized monitoring based on organ involvement.²⁷

“

You can do everything in your power to try to limit your crises, but sometimes you can't out-hydrate or out-rest the disease. It's a war going on in your body that you can't always control.”



Golie Lorenzo Green
Sickle Cell Warrior

Hydroxyurea, a type of medicine that helps the body produce more fetal hemoglobin to reduce red blood cell sickling, has been the mainstay of treatment in SCD since 1995.³⁰⁻³² It has been used to reduce complications and mortality rates.³⁰⁻³² More recent treatment advances include the introduction of two disease-modifying therapies, but post-marketing evaluation of their risk-benefit profiles resulted in their removal from US and/or European Union (EU) markets.³³⁻³⁶ Another disease-modifying therapy shown to reduce the frequency of pain crises in SCD patients, was approved by the FDA in July 2017, but was not approved by the EMA due to insufficient data on clinical efficacy and was subsequently withdrawn from consideration for marketing authorization in 2019.^{37, 38}

New medicines have predominantly included disease-modifying therapies, which aim to change how the disease affects the body, alleviate symptoms or reduce complications, such as chronic anemia, fatigue, or pain.^{1, 38, 39} Development of anti-hemolytic agents are being investigated due to their potential to improve red blood cell survival and function.⁴⁰ In 2023, two gene therapies were approved for SCD.⁴¹ By modifying a patient's own cells to address the underlying genetic cause of the disease, these treatments offer the potential for a cure.¹ However, gene therapies and other potentially curative options such as, bone marrow/stem cell transplants are costly and may carry significant risks.¹

Entrenched factors, including racial inequity, have been associated with delays in the development and delivery of new treatments for SCD.

SCD is widely documented as being underfunded, under-researched and underserved, with few approved treatments, especially when compared to other rare diseases.⁴²

SCD disproportionately affects persons of African descent. Histories of racism, both broadly and within the healthcare system itself, still haunt people of color in the US, leading many within the SCD community to mistrust research studies.⁴²⁻⁴⁴ Inequities in pain management, research funding, and stigma have all been linked to underlying structural racism, with many of those with SCD reporting that their race compromises their quality of care in healthcare settings (e.g., inadequate pain management, long wait times in emergency departments).^{5, 43}

These communities have experienced complicit and implicit bias in the healthcare system when they have been dismissed, not believed, or labelled as “drug-seeking” rather than receiving care.⁹ Feelings of being marginalized have fueled the belief that their lives are undervalued within the healthcare system, thus eroding trust into a deficit.⁹

As a result, patients can feel trapped in a power dynamic where the system is against them, dialog with doctors is not always empowering, and access to care can be an uphill battle, with their voices getting lost in a system that studies them but does not include them. Social determinants of health, i.e., non-medical factors that influence health outcomes, such as poverty, unemployment or housing instability, add further complexity.

“

When it comes to pain relief we are often treated very differently to someone with cancer. These inequities shape how you experience the whole health system.”



Dominique Friend
Sickle Cell Warrior

“

We could not expect trust. Historic and ongoing marginalization had already created a trust deficit in companies like ours and across the healthcare system. We had to earn it, so we started by listening to the community and understanding their experiences.



Abdulafeez Oluyadi
Agios

Although people living with SCD have benefited from past clinical trials, several barriers to enrollment have led to premature trial closures, with factors such as travelling to trial centers, travel costs, taking time off work, fatigue, debilitating pain, and the unpredictability of the disease contributing to clinical recruitment difficulties.^{45, 46} In the 2022-2023 Learning and Insights into Sick Cell Trial Experiences (LISTEN) survey, which evaluated 1,145 warriors and 361 healthcare providers from 17 countries, the biggest barriers to trial participation by warriors were identified as the potential for side effects, missing work or school or loss of income.^{47, 48} Commonly reported motivators for trial participation included the feeling of supporting new treatment developments that may benefit themselves or others with SCD, the potential to better manage symptoms and/or side effects, the opportunity to try a better-working treatment, and increasing knowledge about SCD; other important factors included having information about safety measures for the trial, how the treatment works, and the ability to speak with the researchers running the trial or patients already involved in the trial.⁴⁷ When these practical and logistical concerns are not adequately addressed, the anxiety around participation may leave patients reluctant to get involved.⁴⁵ Furthermore, when studies focus on meeting research goals rather than the needs of patients, skepticism about joining a clinical trial is further heightened.⁴⁹

Consequently, clinical trials in SCD have historically experienced high trial termination rates, with over 25% of interventional SCD clinical trials having closed early and 57% of those citing low patient enrollment as the primary reason for termination.⁵⁰ Challenges with enrollment have delayed the development of new therapies and premature abandonment of novel drugs. As a result, many of these treatments never reach SCD communities.



Warriors are joining the fight to overcome the ‘double burden’ in SCD research.

Lack of trust by patients with SCD has been attributed to structural and systemic issues, i.e., deep-rooted problems in how healthcare systems are set up. A comprehensive report from the National Academies of Science, Engineering, and Medicine in 2020 revealed patient experiences of SCD pain not being believed, false accusations of SCD patients being drug-seeking, delayed care, and care systems requiring SCD patients to fight in order to be seen, heard, and treated equitably.⁵¹ Racism in medical systems has understandably led to historic and ongoing mistrust within the predominantly African American community, contributing to the significant burden of trial participation on patients already managing a chronic, debilitating disease. The high rate in SCD trial closures, compounded by historic racism within the healthcare system and resulting mistrust, has resulted in a “double burden”, which has stalled the development of potential therapies.

Warriors are resisting being passive recipients of care. Instead, they are becoming active advocates and asserting their desire to be seen, heard, and respected as true partners. Simultaneously, regulatory bodies like the FDA and the EMA are encouraging companies to ensure that authentic, meaningful patient involvement is an integral part of the drug development process.^{10, 11} FDA issued its first draft guidance on use of patient-reported outcomes in clinical trials in 2006⁵² which was followed by publication of several guidance documents from 2018 onward on collecting comprehensive input from patients during drug development.^{10, 53-55} Likewise, EMA has promoted public engagement since 2014, emphasizing patient involvement in clinical trial decision-making.^{11, 53, 56} These examples underscore the efforts made by global health authorities who share the consensus that if organizations want to develop trust, improve participation, and create interventions that work for the SCD community, the only credible path forward will require creating pathways to meaningfully connect pharmaceutical companies with the community at large.

Early and meaningful patient participation in clinical trial design may contribute to more relevant, efficient, and trusted research.

Patients and advocates have reported that patient engagement often occurs too late in the clinical trial design process or sometimes not at all, and many studies focus almost solely on meeting research goals, not fully accounting for the human needs of their participants.⁵⁷ In fact, when the patient community isn’t engaged, the consequences can undermine trust and damage public perception, resulting in inadequate trial designs that measure irrelevant endpoints, have low recruitment, and/or waste resources.⁵⁸

NEGATIVE CONSEQUENCES OF NOT ENGAGING THE PATIENT COMMUNITY INCLUDE:

Implementation challenges

- Logistical challenges may not be adequately addressed, resulting in poor enrollment and retention.⁴⁵
- Informed consent processes may be overly complex, culturally insensitive, or stigmatizing.⁵⁷
- Resources for trial participants may be inadequate without user input and validation.⁵⁹
- Clinical trials may progress inefficiently, causing budgetary and reputational consequences.⁴⁵

Diminished utility of trial findings

- Regulatory decisions may be delayed due to the lack of demonstrable patient involvement.^{10, 53, 60}
- Clinical trial designs may have limited real-world relevance.⁵⁷
- Poorly communicated trial results may alienate patients and the public by creating an unnecessary barrier to understanding.⁵⁷

CONVERSELY, INVOLVING PATIENTS IN THE DESIGN OF CLINICAL TRIALS HAS BEEN ASSOCIATED WITH SUBSTANTIAL BENEFITS:

Benefits to patients

- Informational material is more relevant and understandable to patients and their peers, family, and caregivers. This may help support decisions on whether or not to participate in a trial.⁶¹
- Communication of trial findings and their potential importance is improved, helping build trust and engagement in research.⁶¹
- Trial participants are more likely to complete a trial when logistical barriers are identified and addressed early in the trial design process.⁶²
- Clinical endpoints are reduced by 30%, reducing patient burden during data collection by focusing on endpoints that are meaningful to patients.⁶³

Benefits to health care systems

- Trials run more efficiently, with faster trial initiation, shorter trial duration, and quicker trial close-out.^{58, 62, 63}
- Clinical procedures are significantly reduced, with up to 50% fewer data points collected when patient advisory boards are used.⁶³
- Greater resource efficiency reduces operational expenses, lowers development costs, and reduces waste and redundancy.⁶¹⁻⁶³

To better help people whose lives are affected by SCD, clinical trials should include patient opinions and involvement right from the start of the research process. This approach could lead to results that matter more to patients, making studies easier for them to join, enabling collection of more useful information, and helping researchers find and retain participants more easily.⁶⁴



Agios partnered with warriors and advocates to co-create the RISE UP clinical program, with patient perspectives shaping every stage of trial design and communication.

Agios recognized that the challenges facing SCD research were never going to be solved through typical forms of patient engagement, such as consultation only after issues arise on a trial, such as difficulty recruiting. Addressing the persistent challenges facing SCD research demands candid acknowledgment of the historical and systemic factors that have eroded trust within affected communities, as described above. Recognizing the history and challenges the healthcare system and pharmaceutical industry faced, Agios set out to redefine its approach to clinical development in SCD. The company prioritized early, authentic partnership with warriors and advocates. This shift was grounded in the belief that patients are experts in their own lived experience, and that their partnership is essential for designing research that is both credible and relevant.

To put these principles into practice, and drawing on the conviction, “nothing for us without us”, the company assembled a group of warriors and advocates to form an advisory board to help shape Agios’ clinical program “RISE UP”—named to reflect the Board’s desire to change SCD research from the inside out.

This collaborative model helped ensure that patient perspectives shaped every stage of the clinical program, from trial design to the trial recruitment campaign. By centering the voices and priorities of those most affected, the RISE UP initiative aimed to rebuild trust, reduce barriers to participation, and set a new standard for partnership in rare disease research.

“

So many times we (patients) have been left out of the equation. For Agios to say, we want to build something with you that made it feel... authentic.”



Blaze Armon Eppinger
Sickle Cell Warrior

Patients are experts in their own conditions and in their lived experience. Involving them from the outset ensures research is more relevant, credible, and impactful. Rather than fitting warriors into a predefined trial design, the RISE UP program was built around their experiences and priorities from the start. Partnership with the SCD community helped ensure their voice was embedded throughout the trial process, to address anticipated challenges, and raise genuine awareness. This was felt to be the only way to address the effects of the ‘double burden’ that has hampered SCD research.

Members of the advisory board had the opportunity to communicate what life is like for people with SCD and their advocates. These interactions informed the development of the Phase 2 and 3 clinical trial designs for an investigational oral treatment (developed by Agios) to help improve the function of red blood cells and reduce hemolysis. It was essential to ensure co-creation from the start and align with community expectations by incorporating patient insights and recommendations into the trial’s protocol design and implementation plan, as well as its dissemination and communication plan.



The clinical program

Warriors were engaged as partners from the outset to help ensure the clinical trials considered real-world needs, fostered transparency, and ultimately delivered for the SCD community.

To better help people whose lives are at risk or affected by SCD, clinical trials should include patient opinions and involvement right from the start of the research process. This approach may contribute to results that matter more to patients, making studies easier for them to join, collecting more useful information, and helping studies find and retain participants more easily.⁶⁴

This work was guided by four core principles that served as non-negotiable foundations for the initiative:

- CO-CREATING
- INCLUSION
- TRANSPARENCY
- TRUST-BUILDING

To build understanding of the real needs and expectations of clinical trial participation, an Advisory Board of SCD, caregivers, and community members was convened.

Twelve warriors and advocates (Bahrain n=1, France n=1, United Kingdom n=2, United States n=8 [n=7 in the campaign]) took part in a series of clinical trial design workshops on the RISE UP Phase 2 and Phase 3 clinical trial design. A mix of male and female warriors was recruited since the viewpoints of males have typically been poorly represented. A broad age range (16–54 years) ensured a range of experience. In total, a series of 4 virtual patient advisory board interviews were held with this group.

PARTICIPANTS WERE ASKED FOR THEIR OPINIONS IN THESE AREAS:

- Meaningful trial parameters
- Study duration
- Post-study access to treatment
- Study endpoints
- Eligibility criteria
- Assessments and procedures
- Operational support needs (e.g., visit frequency and locations)
- Compensation considerations
- Pain reporting

Importantly, Advisory Board members were treated as subject-matter experts. They spoke to what was feasible, what was burdensome, and what mattered most to them as outcomes—information that traditional clinical experts alone could not provide. This was the only way to ensure the complexity and diversity of the SCD experience was captured.

Feedback from these discussions was used to help shape the trial protocol design, which was then shared with the FDA to understand if these recommendations were feasible from a regulatory perspective. Once the trial protocol and the trial design were finalized, feedback from the FDA was presented back to the Advisory Board, indicating where their input was incorporated, and, in cases where it was not possible, provided the reason why. This ensured transparency in communication. The warriors were later updated on progress at each important trial milestone and consulted on next steps.

The recruitment campaign

Warriors and advocates co-created the RISE UP clinical trial recruitment campaign, shaping the creative concept and communication strategy from the earliest stages.

Seven warriors (5 US warriors from the clinical trial design workshops and 2 US patient advocates) were convened to co-create the RISE UP clinical trial recruitment campaign, which aimed to educate the community about the trial and the value of participation. Co-creation drew from their strong knowledge on what messaging would resonate, how to build trust, and how to communicate authentically in their communities. Their expertise was essential in determining the right channels, tone, and approach to reach people effectively.

A creative agency was consulted to develop a creative concept for the clinical trial recruitment campaign. To inform planning, warriors first met with the creative agency for a ‘listening session’ during which they described their lived experience and their community’s strengths.

This early involvement was welcomed by the patients who are typically invited to provide input only after campaign ideas have been developed. That information was used to develop a campaign incorporating aspects such as:

- How to encourage warriors to learn about the RISE UP study and what it would mean to enroll
- How warriors want to be represented, in comparison to campaigns from past years
- What information is most helpful and important to inform potential participants
- How this information should be shared in a way that builds trust

Among several compelling creative concepts, a campaign focused on boxing was selected as a symbol of strength, reflecting an attribute the warriors felt had not been traditionally portrayed.

“We named the trial ‘RISE UP’ because like in boxing, you get knocked down by a crisis, but you keep getting back up. It’s about the strength in our community.”

RISE UP
Sickle Cell Warriors



FIGURE 3: PATIENT ENGAGEMENT PROCESS



Results

When Agios partnered with people with lived experience, the results suggested that authentic patient engagement contributed to on-time enrollment and a clinical trial that better reflected community needs.

Patient engagement efforts in RISE UP exceeded the expectations of those involved. The impacts of including warriors in the trial design also have implications for other diseases and settings, yielding a set of imperatives and transferable insights that are described in this section.

Collaborate with patients from the earliest stages of developing a trial.

When patients are brought on board at the beginning, rather than halfway through, conversations can truly shape the direction and design of trials.

“If there’s one thing to remember, it’s that patients must be included in defining treatments for them. True partnership and transparency are essential.”



In RISE UP, prioritizing patient input and involvement from the earliest stages (before protocol writing) enhanced trial design, ensuring the protocol was grounded in lived experience and focused on relevant outcomes (like patient-reported outcomes for daily fatigue). When consulted on protocol design, patients provided detailed input on study duration, post-study access, study endpoints, and eligibility criteria (see Table 1 below for feedback and details on how this was put into practice).

KEY PATIENT CONTRIBUTIONS TO THE PROTOCOL DESIGN INCLUDED:

- 2:1 randomization to best address the community’s preference to maximize the number of participants who receive the study treatment
- Allowing hydroxyurea during the study period to reflect real-world practice and increase the pool of eligible patients
- Addition of fatigue as a study outcome to better reflect how people actually live with SCD (warriors brought up “fatigue” as a critical concern in their daily lives)

Involving patients early ensured that the trial structure was inherently feasible and credible. Potential obstacles to successful recruitment could be anticipated and addressed, thus mitigating the need for revisions to the trial protocol during recruitment. For example, addressing participation barriers by offering support for travel, lodging, and some appropriate non-trial medical costs ensured participation did not depend on income or access, making participation more realistic for people already carrying a heavy economic or logistical burden.

When a suggestion could not be implemented, the company provided a clear rationale for why and addressed any associated concerns. This communication fostered both education and mutual respect. This experience adds to a body of evidence demonstrating that patient engagement in research leads to efficiencies, including in recruitment and retention, prevention of disruptive protocol amendments, enhanced trial conduct, and increased likelihood of success.^{45, 62, 65}

TABLE 1: SUMMARY OF COMMUNITY FEEDBACK ON THE RISE UP TRIAL DESIGN

STUDY ASPECTS	COMMUNITY FEEDBACK	PROTOCOL DESIGN ELEMENTS
Study Duration	12-month study duration may hamper participation and/or compromise treatment adherence	Approximately monthly study visits for 7 months, followed by visits every 3 months
Study Design	Preference for all participants to receive study treatment during study period	Patients enrolled were twice as likely to receive study drug than placebo (2:1 randomization to investigational drug or placebo).
Post-Study Access	Important to provide patients access to treatment after completion of the clinical trial	An open-label extension period of 216 weeks (~4 years) was added, which allowed patients to continue to receive treatment, with monthly visits for the first 6 months of the extension period, followed by every 3 months up to 1.5 years and every 6 months thereafter.
Study Endpoints	- Evaluate effect as assessed by high quality patient-reported outcomes - Evaluate pain and fatigue - Provide flexibility with questionnaire and minimize questionnaire burden	Key secondary and other endpoints in the trial include both self-reported measures of Health-Related Quality of Life and objective Performance Outcome Assessments.
Eligibility Criteria	- Assessing effect in patients with hemoglobin <8 g/dL could be beneficial - Prohibiting concomitant therapies (e.g., hydroxyurea or exchange transfusion) may preclude patient participation	- Subgroup analyses were specified in the protocol based on baseline hemoglobin (<8 g/dL, ≥8 g/dL). - Concomitant hydroxyurea (sickle cell disease standard of care) use was allowed and was used as a stratification factor.

WHAT THIS TELLS US - TRANSFERABLE INSIGHT:

By grounding the protocol design process in the lived experience of the warrior community, clinical studies may be made more effective, focusing on outcomes that are relevant and with parameters that are realistic for patients to adopt and adhere to.

This experience further reinforces the evidence that patient engagement in research delivers tangible benefits, including stronger recruitment and retention, fewer protocol amendments, more efficient trial delivery, and an increased likelihood of success.^{45, 62, 65}



Partnership means being treated as a true part of the process—a two-way street where our opinions are heard, and we're given context for decisions. Transparency made all the difference. If something couldn't be done, they explained why. We were always kept informed, never an afterthought."



Teonna Woolford
Sickle Cell Warrior



Co-create with community to enable awareness and engagement

Patients and the public are often excluded or left confused by poorly communicated trial information. Resources made for patients are often inadequate as their input was not sought. Clinical trial recruitment campaigns better resonate with the community if the community plays a central role in designing them.

In RISE UP, the creative agency (the team responsible for creating the campaign content) was brought together with community members before concept development to help ensure the campaign was truly built with them in mind. The creative agency did not develop a single campaign concept without listening to the perspectives of patients and advocates and understanding how they wanted to be seen. Due to this, the RISE UP campaign was uniquely impactful and succeeded in building awareness and engagement, contributing to increased recruitment.

When consulted on how to design, plan, and execute the RISE UP campaign, patients advised a combination of communication approaches, including the following:

- Use language that resonates with the community and align with their priorities to drive engagement and action. When asked how they wanted to be represented, the response was definitive: "Show our strength, not just our struggle."
- Set a tone of empowerment rather than illness. This inspired the boxing gloves imagery, a symbol of resilience for "sickle cell warriors." The impact was reflected in one warrior's child's response: "Dad, you look strong," a powerful illustration of shifting perceptions and fostering empowerment.
- Accurately reflect how they want to be represented and which channels to select, to drive higher engagement levels with trial information and visuals.
- Reach/engage with individuals and local community groups, foster relationships, and build trust to gain support.
- Connect with local and national thought leaders and influencers whose voices resonate with the community. Such individuals have insight into how to help build relationships with patients, families and advocacy groups.
- Feature people living with SCD, not actors.

Implementing this advice meant that the final campaign was grounded in the vision of patients in partnership with the company. It used their language, stories, and visuals, with imagery selected to represent the full spectrum of disease, age, gender, and experiences.

CAMPAIGN IMPACT

The campaign video launched on World Sickle Cell Day and received over 3 million views in six months. Compared with the pre-campaign period, the campaign achieved:

- 124% increase in daily visits to the RISE UP website
- 67% more click-throughs to the study page
- 32 new click-throughs to the physician site

In addition, the RISE UP campaign’s social media and digital efforts were reflected in an average of 2-3 clicks per day on clinicaltrials.gov, with nearly 278,000 people visiting the clinical trial website.

“The boxing ring photo shoot really captured who we are as a community. We are fighters. It felt genuine, like they took the time to get to know us and made sure we were true to ourselves. The resulting imagery was different to the random faces you often see in ads for clinical trials or new therapies.”



DeMitrrious Wyant
Sickle Cell Warrior



FIGURE 4: CAMPAIGN CO-CREATED WITH SICKLE CELL WARRIORS

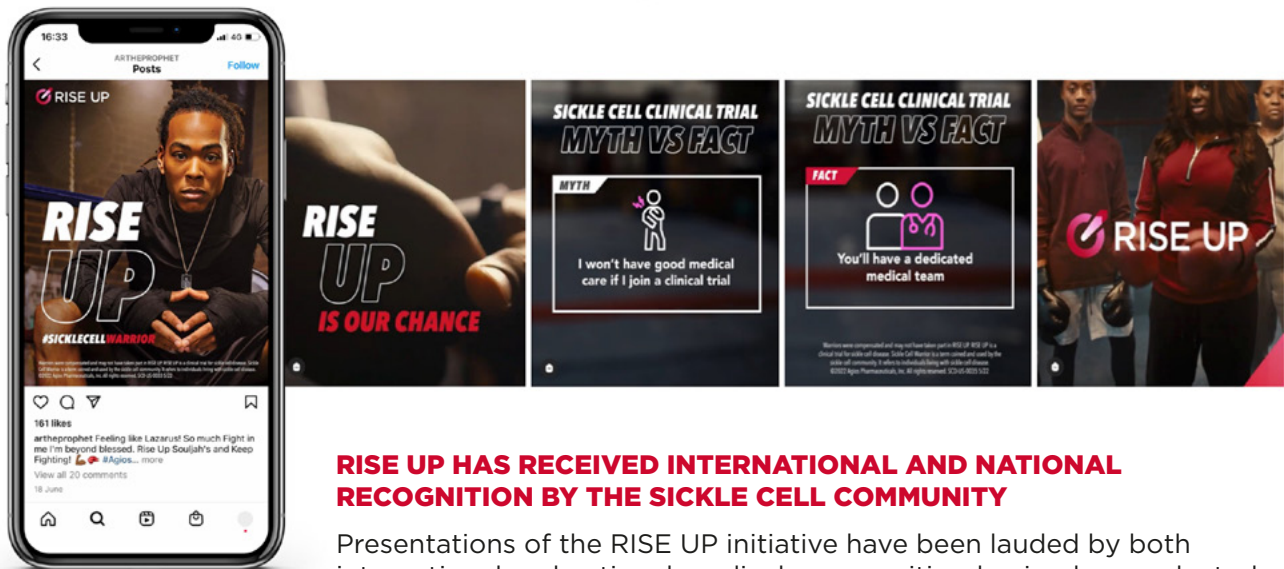


WHAT THIS TELLS US - TRANSFERABLE INSIGHT:

Prioritizing and amplifying the community’s own narrative not only builds authentic engagement but can also help develop trust by demonstrating respect and fostering a sense of shared

purpose, which may contribute to enhanced recruitment. This approach transforms potential participants from passive subjects into active, respected partners in scientific advancement.

FIGURE 5: EXAMPLES OF INSTAGRAM POSTS BY SCD ADVISORY BOARD MEMBERS



RISE UP HAS RECEIVED INTERNATIONAL AND NATIONAL RECOGNITION BY THE SICKLE CELL COMMUNITY

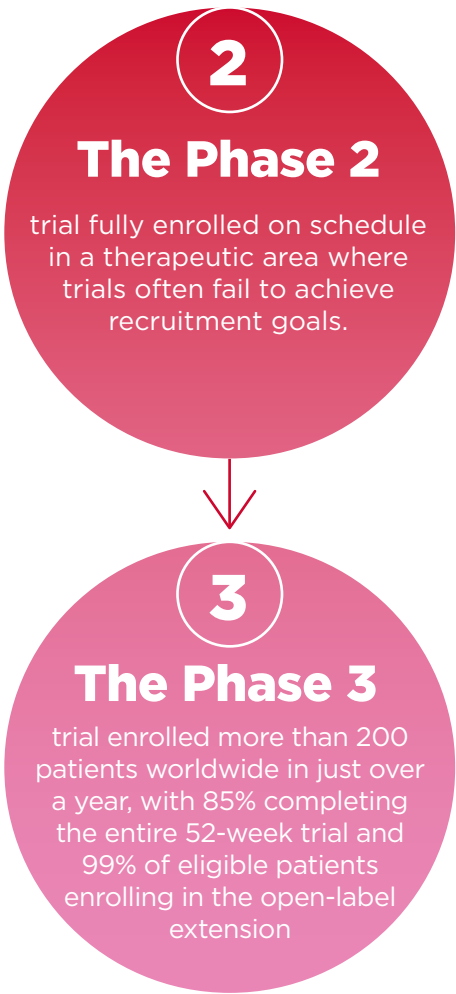
Presentations of the RISE UP initiative have been lauded by both international and national medical communities, having been selected as 1 of 3 plenary presentations at the 2023 annual conference of the Academy for Sickle Cell and Thalassemia (ASCAT)⁶⁶ and awarded the “best oral presentation” at the 51st Annual National Convention of the Sickle Cell Disease Association of America (SCDAA) in 2023.⁶⁷

True partnership starts with recognizing patients as lived experience experts.

Patients are experts in their own conditions and in their lived experience and therefore bring deeper disease insight than clinical experts alone can provide. Involving them from the outset ensures research is more relevant, credible, and impactful. Active listening is key to making the right decisions and helps fill critical information gaps that might not even be noticed until patients are allowed to speak. Other hallmarks of strong partnership are early collaboration, transparency, and clear feedback loops.

In RISE UP, when warriors and advocates were empowered to speak about their real lives—their work schedules, their health challenges, their fears—practical barriers surfaced early. It was then possible to design trials and materials that supported full participation.

Integrating SCD patient feedback contributed to the successful, on-time completion of the Phase 2 trial and rapid enrollment of over 200 patients in the Phase 3 trial—a significant milestone in a historically recruitment-challenged field. In the Phase 3 trial, 85% of enrolled patients completed the entire 52-week trial, and 99% of eligible patients enrolled in the open-label extension.^{68, 69} With recruitment and retention being common clinical challenges and high mistrust in clinical research, the enrollment milestones in the Phase 2 and Phase 3 trials demonstrate the positive power of authentic patient partnership in fostering trust and inclusivity.



WHAT THIS TELLS US - TRANSFERABLE INSIGHT:

Collaborating with patients helps ensure operational success. By investing early in collaborative trial designs and operational processes, organizations can ensure that the trial is inherently feasible and credible, which can mitigate the risk of recruitment failure and support enrollment.

Conclusions

The RISE UP program suggests partnering with patients from the start of clinical trial planning leads to more efficient and relatable research.

By prioritizing patient input before protocol development, the trial design was grounded in lived experience and focused on outcomes that matter most to the community. Early involvement allowed for practical adjustments like flexible trial visits, post-trial access

to treatment, and support for travel and lodging-making participation more accessible and realistic. This approach also fostered transparency, as patient feedback was addressed directly, building mutual respect and trust.

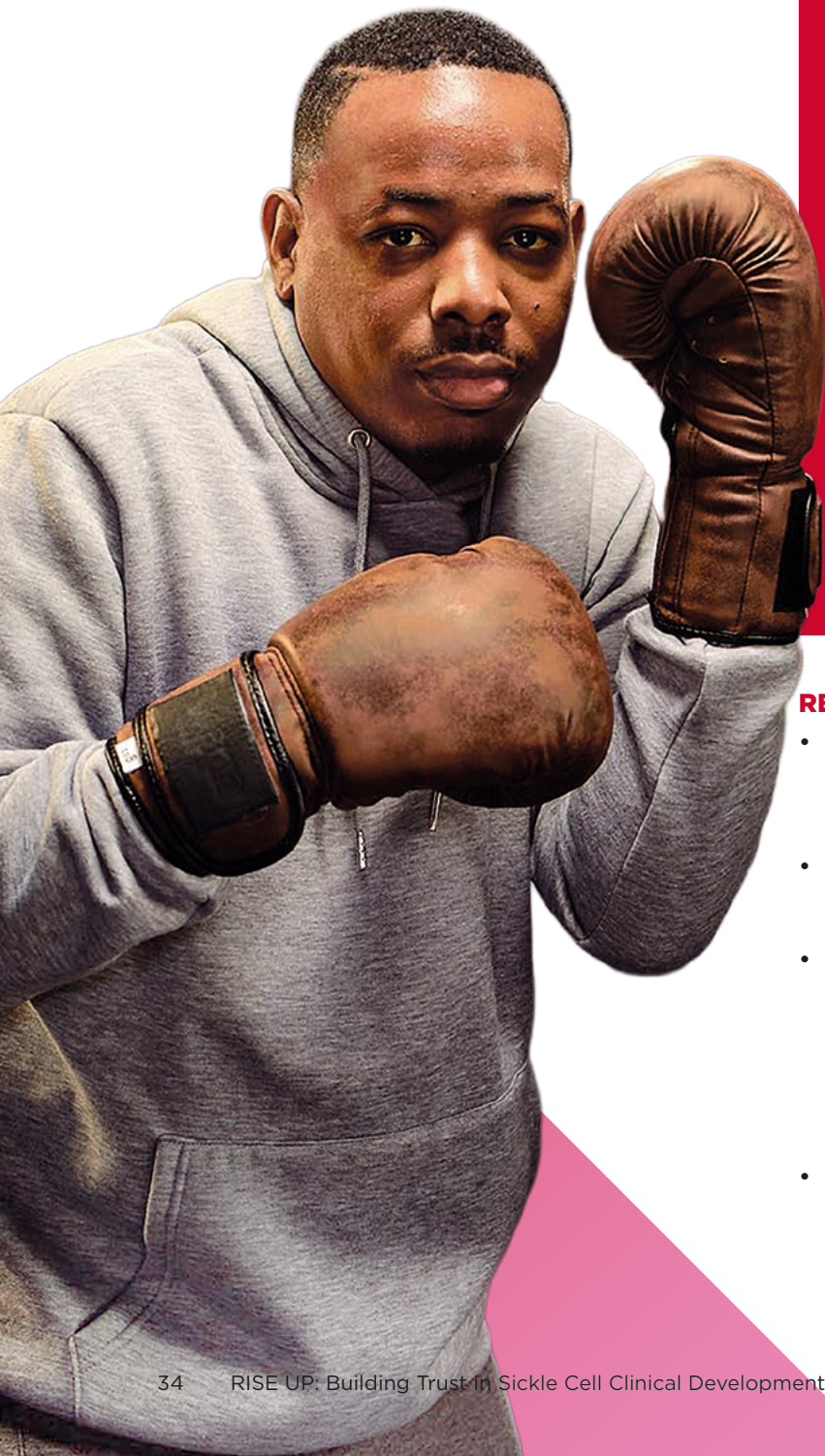
FIGURE 5. A SNAPSHOT OF RISE UP CLINICAL TRIAL RECRUITMENT CAMPAIGN ACHIEVEMENTS

Contributed to Naming of the Clinical Program	 Named “RISE UP” to Reflect the Desire to Change SCD Research from the Inside	
Relevant Clinical Endpoints Included	Fatigue was added as an endpoint, reflecting a critical concern in the daily lives of patients	Relevant patient-centered outcomes were included (patient-reported measures of Health-Related Quality of Life and Performance Outcome Assessments)
Burden of Trial Participation Reduced	Potential obstacles to successful recruitment were anticipated and addressed	Support for travel and lodging ensured participation didn't depend on income or access
A Clinical Trial Recruitment Campaign Connected with the Community	Video launched on World Sickle Cell Day: 3M+ views in 6 months	Nearly 278,000 people visited the clinical trial website
Greater Engagement with Trial-Related Information	Compared with pre-campaign period: 124% increase in daily visits to the RISE UP website 67% more click-throughs to the study page	
Real Impact on Enrollment and Timelines	The Phase 2 study fully enrolled on schedule in a therapeutic area where trials often fail to achieve recruitment goals.	The Phase 3 trial enrolled more than 200 patients worldwide in just over a year.
International and National Recognition by Medical Communities	Selected as 1 of 3 plenary presentations at the 2023 ASCAT Conference*	Awarded “Best Oral Presentation” at the 51st (2023) Annual National Convention of the SCDA Convention*

* 51st Annual National Convention of the Sickle Cell Disease Association of America (2023)

Recommendations

The experiences of those who made the RISE UP clinical development program successful have contributed to actionable recommendations for others committed to building trust with patients through authentic partnership. By applying these insights, organizations can help drive research that is more meaningful, credible, and impactful, within SCD and across other disease areas.



Insight 1:

By grounding the protocol design process in the lived experience of the SCD patient community, clinical studies may be more effective, focusing on outcomes that are relevant and with parameters that are realistic for patients to adopt and adhere to. This experience adds to a body of evidence demonstrating that patient engagement in research leads to efficiencies, including in recruitment and retention, prevention of disruptive protocol amendments, enhanced trial efficiency, and increased likelihood of success.^{45, 62, 65}

RECOMMENDATIONS:

- At the earliest stages of the Research & Development process, create a patient advisory board to inform decision-making processes related to the trial design.
- Prioritize convenience and ease of patient compliance, based on their lived experience, during protocol development.
- Implement a system for transparent feedback, explaining how feedback is used, or if it can't be, clearly explaining the scientific or regulatory reasons why not. Act on the group's recommendations and communicate how regulatory requirements and patient needs are being balanced.
- Keep engagement central throughout the study. Establish a mandate for early and constant partnership, to move beyond token consultation, and ensure patients' influence is integrated at every stage.

Insight 2:

Prioritizing and amplifying the community's own narrative builds authentic engagement, creates space to better understand patients' experiences and what has led to mistrust of the medical system, and helps ensure those mistakes are not repeated. This demonstrates respect and fosters a sense of shared purpose, may contribute to enhanced recruitment. This approach transforms potential participants from passive subjects into active, respected partners in scientific advancement.

RECOMMENDATIONS:

- Authentically engage local and national thought leaders and influencers whose voices resonate with the community, and allow them to show you how to help build relationships with patients, families, and advocacy groups. Listen to what they say and follow their advice where feasible.
- Leverage social media platforms for broad campaign reach and engagement.
- Find out what patients want to know about a clinical trial and any potential concerns so these can be addressed. Don't assume you know what these will be.

Insight 3:

Collaborating with patients can help ensure operational success. By investing early in collaborative trial design and operational processes, organizations can ensure that the trial structure is inherently feasible and trusted, which can mitigate the risk of recruitment failure and support enrollment.

RECOMMENDATIONS:

- Define what patient-centered means to your organization and ensure the support of leadership. Creating a true culture of partnership increases efficiency and the ability to act decisively on patient-provided input and insights.
- Patient engagement and collaborative trial design should be prioritized early in research and development. This organizational change ensures feasibility and trust are established from the start, thereby increasing the chances of reaching completion of a study that generates meaningful findings for patients.

Calls-to-action

For patients	For patient advocacy organizations	For healthcare professionals	For the pharmaceutical industry
1 Be open to joining an advisory board or patient panels when invited and be specific about what matters most in your daily life so trials measure outcomes that reflect your lived experience. Your voice matters and can help improve the progress of clinical trials.	1 Help members understand the value of clinical research and the potential role they can play in shaping future treatments. Offer resources and support to navigate the clinical trial journey, from exploring opportunities to understanding expectations and rights as a participant.	1 Encourage open dialog from patients to raise awareness about clinical trials and drive engagement at the grassroots level. Create space in clinic visits and community forums for patients to voice concerns about trials, and respond with concrete information about protections, support, and options.	1 Make partnership an integral component throughout the clinical trial process, from protocol development to operational planning.
2 Tell researchers and healthcare teams what matters most to you, from travel and accommodation to scheduling and family commitments, so they can design trials that work better for patients.	2 Support members to share their experiences and challenges with trial sponsors. Where individuals do not feel comfortable providing feedback directly, act as a trusted intermediary to ensure their perspectives, concerns, and needs are heard and addressed.	2 Use your influence with trial sponsors to promote trial designs and support provisions that reflect patients' lived experiences, reduce participation burdens, and enable more equitable access.	2 Partner with patients at the earliest stages of research and development to listen and better understand the community's experiences and disease-related needs. Use the insights gained to address potential barriers to participation.
	3 When giving input to companies or researchers, request a clear process for reporting back: what was adopted, what wasn't, and why. Share this information with your community to build understanding and trust in the research process.		3 Report back to patients on the full spectrum of feedback, explaining what has been incorporated and why certain aspects were not possible to address.

**It is important to note that the complications of SCD are age-specific, with different age cohorts affected by the disease in different ways. In the RISE UP clinical trials, the patients recruited were all aged 16 years and older; therefore, the recommendations contained in this white paper may need to be adapted when considering the needs of younger patients and their families. Furthermore, the engagement insights in this white paper are informed primarily by experiences in the US. The RISE UP studies were conducted globally, with adaptations made for different countries. The key learnings in this report may also require contextual adaptation across regions.*

Authors



DeMitrious Wyant
Rare Care Foundation

Living with sickle cell has taught me resilience and given me a sense of purpose. Whether through education, advocacy, or creative expression, I've tried to use my experiences to help others better understand the often unseen realities of life with sickle cell. Every obstacle I've faced has strengthened my commitment to helping people not just live with sickle cell, but live well. By listening to patients and learning from our lived experiences, research can help create a brighter future for the sickle cell community.



Golie Lorenzo Green
Sickle Cell Association of Houston

One of the greatest lessons sickle cell has taught me is that some of the biggest challenges are often the least visible. Sickle cell can have big impacts on your plans, your energy, and even how you think about the future. Through my work in the community, I've seen what can happen when patients, researchers, healthcare teams, and industry come together and truly listen to one another. Those conversations have reinforced my belief that lived experience belongs at the heart of research.



Dominique Friend
Sickle International Family Coalition

What began as navigating my own experiences with sickle cell inspired me to become an advocate not just for myself, but for others in our community. Through sharing my story and creating opportunities for others to share theirs, I've seen how powerful patient voices can shape conversations about care, research, and even broader policy decisions. These experiences have reinforced my belief that every patient deserves to feel heard, respected, and valued.



Teonna Woolford
Sickle Cell Reproductive Health Education Directive

My journey with sickle cell has included pain crises, major surgeries, and unexpected challenges that have changed the course of my life. Those experiences inspired me to advocate for issues like reproductive health and quality of life that are often overlooked. While there is still much work to do, I am encouraged by the growing recognition that patients must help define what meaningful progress looks like. When research reflects the full lived experience of sickle cell, it has the power to create lasting change.



Blaze Armon Eppinger
Sickle Cell Foundation of Georgia

Working with children and families affected by sickle cell has shown me the incredible strength that exists within our community. I've seen young people go from feeling isolated to finding confidence, connection, and a sense of purpose when they realize they are not alone. Those experiences reinforce my belief that progress starts with listening. When people, families, researchers, and healthcare professionals come together as partners, we can drive research that reflects real-life experiences and helps create a brighter future for everyone living with sickle cell.



Dr. Wally Smith
MD, Virginia Commonwealth University

The sickle cell community has taught me far more than any textbook ever could. Over the years, I have seen the impact of both sickle cell and the systemic barriers that many patients continue to face. Despite these challenges, I am continually inspired by the resilience, wisdom, and determination of the people I serve. Their experiences have shaped not only my understanding of medicine, but also my understanding of what good care should look like. I remain committed to advocating for a healthcare system that listens, responds, and delivers the quality care this community deserves. Lasting progress will only come when trust, respect, and partnership are at the center of everything we do.



Ahmar Zaidi
Senior Medical Director, Agios

Working with the sickle cell community has reinforced a fundamental truth: patients are experts in their own lived experience. As researchers and clinicians, we bring scientific expertise, but patients bring insights that no study or dataset can fully capture. Some of the most valuable lessons I've learned have come directly from listening to patients and understanding what matters most in their daily lives. The future of sickle cell research will be strongest when patients continue to help shape the decisions that affect them.



Holly John
Senior Director, Global Patient Advocacy & Strategy, Agios

One of the greatest privileges of my role has been working alongside the sickle cell community and learning directly from the people whose lives are affected every day. Time and again, patients and advocates have reminded me that trust is not built through good intentions alone. It is built through meaningful action, transparency, and genuine partnership. They have reinforced my belief that when patients are involved from the beginning, we can create solutions that are more relevant, inclusive, and impactful for the community.



Abdulafeez (Deji) Oluyadi
Senior Director, Clinical Science, Agios

The sickle cell community has continually reminded me that research is about people first. While data helps us understand the science, patients help us understand the lived experience behind it. Their insights have challenged us to think more broadly about what meaningful progress looks like and how we can better address the needs of the community. I believe the strongest research is built through partnership, where scientific expertise and lived experience work hand in hand.



Janie Davis
Director, Patient Advocacy Liaisons, Agios

Throughout my work with the sickle cell community, I have been reminded that meaningful advocacy begins with listening. People living with SCD and their families have shared their experiences, challenges, and aspirations with remarkable honesty, offering insights that have shaped the way I think about partnership and engagement. Their voices have reinforced the importance of creating opportunities for people to contribute not just as participants, but as partners whose perspectives help guide decisions.

“

For many people affected by sickle cell, the journey has been a long one. While there is still work to do, RISE UP gives me hope. It shows what is possible when we listen, build trust, and work alongside patients as partners. My hope is that we continue to raise the bar and do better.”



Janie Davis
Agios



EVERY
DAY
WE
RISE



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42 RISE UP: Building Trust in Sickle Cell Clinical Development Through Authentic Patient Partnership

43

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