

A young girl with voluminous, curly brown hair is smiling warmly at the camera. She is wearing a light grey, long-sleeved cardigan with a subtle pattern of stars. The background is a soft, out-of-focus indoor setting.

American Society of Hematology

STATE OF SICKLE CELL DISEASE

2016 REPORT



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State of Sickle Cell Disease: 2016 Report include:



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The Emergency
Department Sickle Cell
Care Collaborative*



GLOBAL SICKLE CELL
DISEASE NETWORK



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Summary

Over the past century, great advances have been made in the understanding and treatment of sickle cell disease (SCD). This first “molecular disease,” caused by a single gene mutation, has advanced the field of modern human molecular biology. Many important discoveries have been made, and some treatments developed. These discoveries have identified innumerable questions and opportunities to better understand and treat this complex disease. Yet, many basic scientific processes are still not fully understood, too few treatments have been developed, and most of the people who have SCD do not have access to these treatments which could improve the duration and quality of their lives.

With profound concern about the unmet scientific opportunities and lack

of access to high-quality care, a group of SCD stakeholders has convened and is eager to find

remedies. This document outlines their analysis and details a comprehensive plan to address these issues.



The plan encompasses four priority areas – Access to Care in the United States, Training and Professional Education, Research and Clinical Trials, and Global Issues Related to SCD – which will be the focus of our collective efforts toward ultimately advancing SCD care, early diagnosis, treatment, and research.

For a detailed list of strategies to address these issues visit scdcoalition.org

Introduction

Sickle cell disease (SCD), which causes a wide range of severe and even life-threatening consequences, is caused by a single misspelling in the DNA instructions for hemoglobin, a protein vital for carrying oxygen in the blood. As a result of this mutation, individuals with SCD experience lifelong complications including anemia, infections, stroke, tissue damage, organ failure, intense painful episodes, and premature death. These debilitating symptoms and the complex treatment needs of people living with SCD often limit their education, career opportunities, and quality of life.

The many complications of SCD can make every stage of life extremely challenging for individuals with the disease. For example, approximately 10 percent of children with SCD will have a symptomatic stroke, which can cause learning problems and lifelong disabilities. Pain is the most common clinical manifestation of SCD and results in tremendous suffering, prolonged absences from school, and difficulty maintaining full employment. Individuals with SCD experience chronic pain compounded with acute pain episodes. In a six-month study of adults

with SCD, half of the respondents reported experiencing some level of pain for half of the days, while nearly a third noted having pain the majority of the time.¹ Acute pain episodes can occur multiple times per year and may result in long hospital stays, leading to a complete disruption of the person's life and his or her family's life.

SCD is the first known molecular disorder. However, advances in treatment have been limited compared with later discovered molecular diseases – such as cystic fibrosis – where multiple treatments have benefited patient populations. Over the past century, several important discoveries have been made in the understanding and treatment of SCD. Yet, many very basic scientific processes are not understood, and far too few treatments have been developed. In addition, most people with SCD do not have access to treatments that could improve the duration and quality of their lives.

We know that more can be done for people with SCD to provide improved access to high quality care. While a cure currently exists, it is available to only a



small portion of the patient population and limited primarily to developed countries. The health outcomes and treatment disparities related to SCD make it a public health priority both in the United States and globally.

There is enormous opportunity to improve the state of SCD. There are actions we can take today to address unmet needs – both in the United States and around the world.

In the United States, it is estimated that:

SCD affects approximately
100,000 people

SCD occurs in about
1 in every 365
African-American
births

SCD occurs in about
1 in every 16,300
Hispanic-American
births

About 1 in 13
African-American
babies is born with
sickle cell trait (SCT)

Source: Centers for Disease Control and Prevention; World Health Organization; Piel et al. 2013. Lancet 381: 142-51.

There is enormous opportunity to improve the state of SCD. There are actions we can take today to address unmet needs – both in the United States and around the world.

We must address these disparities by increasing access to diagnostic and therapeutic interventions, and providing comprehensive care and appropriate support services to people living with SCD. Emerging therapies and promising new insights in the treatment of SCD represent a significant step toward improving outcomes and reducing the treatment burden for affected children and adults.

Alleviating the pain and suffering caused by this disease, as well as the socioeconomic costs, is entirely within our grasp. Although conquering SCD is doable, it requires a well-orchestrated plan and a coordinated effort from a range of partners including government agencies,

patient advocacy organizations, health care providers, public health organizations, researchers, foundations, pharmaceutical and biotech companies, and other stakeholders.

In an effort to advance a forward-looking and comprehensive agenda that will make a significant difference, a group of SCD researchers, clinicians, individuals with the disease, and policymakers have come together to develop an organized approach to improving outcomes for people with SCD. From these meetings, four priority areas were identified to focus our collective efforts toward ultimately advancing SCD care, early diagnosis, treatment, and research.

Globally, it is estimated that:

SCD occurs in approximately **300,000 births** annually

SCD is most prevalent in malaria endemic parts of the world, primarily Africa, the Middle East, and South Asia

In many African countries, **10% to 40% of the population** carries the sickle-cell gene, resulting in estimated SCD prevalence of at least 2%.

Source: Centers for Disease Control and Prevention; World Health Organization; Piel et al. 2013. Lancet 381: 142-51.

THE FOUR PRIORITIES

to improving outcomes for people with sickle cell disease



Access to Care in the U.S.



Training and Professional Education



Research and Clinical Trials



Global Issues



ACCESS TO CARE

IN THE UNITED STATES

CURRENT STATE



More than 75 percent of adults with SCD with frequent pain crises fail to get hydroxyurea, which is the recommended treatment.²



Despite universal newborn screening for SCD in the United States, one study found that long-term follow-up after diagnosis was not performed in nearly one-third (30.8%) of cases.³



SCD is also associated with high treatment costs. For an average person with SCD reaching age 45, total lifetime health care costs were estimated to be nearly \$1 million, with annual costs ranging from over \$10,000 for children to over \$30,000 for adults.⁴



Hydroxyurea, the only FDA-approved drug for adults with SCD (often used off-label in children), improves the course of SCD and might lead to significant health care cost reductions. In a two year pediatric study, overall health care costs for children on hydroxyurea were \$1.8 million, compared with \$2.5 million for those who did not receive this treatment.⁵ Unfortunately, despite the National Heart, Lung, and Blood Institute's (NHLBI) recommendations, hydroxyurea is not regularly prescribed and adherence to the therapy is poor.

MILESTONES IN SCD

SCD has been known for hundreds of years by the people of Africa by different tribal names, including nwiwii, nuidudui, ahotuto, and chwechwechwe

1846

First formal case of sickle cell disease reported.

1910

Herrick provides the first formal description of sickle cell anemia, reporting a "pear-shaped and elongated" blood cell from a Chicago dental student.

1922

Mason observes fourth reported case of sickled blood and names the disease "sickle cell anemia."



Though the mortality rate for children with SCD decreased by 3 percent each year in a study from 1979 to 2005, the mortality rate for adults with SCD increased by 1 percent each year during the same period.⁶



People with SCD in the emergency department for pain experienced longer delays to administration of the initial analgesic compared with control patients, despite higher arrival pain scores and triage acuity levels.⁷

- The lack of available specialized providers (e.g., hematologists) plays a significant role in an over-reliance on emergency departments care for some individuals with SCD, especially adults.^{8,9}



Many children with SCD do not receive the necessary services to prevent serious complications from the disease.

- Only one-third of children with SCD receive appropriate monitoring for stroke risk.
- 25 percent of children with SCD do not receive pneumococcal vaccination, which is recommended for all children younger than five years of age.¹⁰
- Children not receiving these necessary services are at greater risk of dying or suffering from cognitive defects as a result of stroke and invasive pneumococcal infections.

1927

Hahn & Gillespie describe the pathophysiology of the disease noting an association between the sickling of red blood cells and low oxygen conditions.

1933

In Memphis, Tenn., 2,500 African Americans were tested to distinguish between sickle cell trait and sickle cell disease.

1934

The painful sickle "crises" are suggested to be the result of blockages of small blood vessels.

Today, individuals with SCD are living longer, but the system of care needs to change to ensure a better quality of life.

In the United States, access to appropriate care is influenced by a number of factors, including health insurance, the availability of health care providers, and provider experience treating people with SCD. Individuals with SCD reported experiencing poor communication between primary-care physicians and hematologists, providers with limited knowledge and experience with pain management, difficulty in scheduling urgent appointments, and lack of follow-up after hospitalization.¹¹ People with SCD and their families have expressed a need for better information, shared decision-making, and strengthened communication with those providing their medical care.

Other factors, such as geography, economic status, and co-existing conditions have an impact on access to care. Those living in or around metropolitan areas are more likely to have access to knowledgeable providers than those living in rural communities; however, people with access to SCD treatment centers may have difficulty affording frequent and expensive treatments. Additionally, people with

conditions related to SCD such as strokes or cognitive impairment struggle to navigate the health care system without assistance.

The transition from pediatric to adult care can be particularly challenging as the focus of care differs. In the United States, children with SCD are more likely than adults to receive care in academic medical centers, which have SCD-specific expertise. Most of these children are seen by primary-care providers – pediatricians, family medicine doctors, and nurse practitioners – and many have access to pediatric hematologists.¹² In contrast, adults with SCD are more likely to receive care in community hospitals, where SCD-specific expertise is much less common.¹³ As more people with SCD are living into adulthood, disease management needs to shift from acute care of complications to a chronic care model that focuses on prevention of crises, and intervention and relief for common complications. A coordinated health care delivery system for adults with SCD is a first step to improve health outcomes.¹⁴

1940

Sherman confirms that sickling of red blood cells in the absence of oxygen is caused by a molecular change in hemoglobin structure.

1948

In *Blood*, Watson suggests a link between HbF levels and the presence of SCD symptoms.

1948

Pauling & Itano showed with protein electrophoresis that hemoglobin from people with SCD is abnormal. SCD is the first disorder described to be the result of a protein abnormality.



GOALS FOR THE FUTURE

Ensure implementation of existing standard-of-care guidelines and best practices in disease management as well as develop new resources

to help all physicians make the best decisions in treating people with SCD

Implement coordinated care models

that incorporate community health workers, ensuring more equal quality of care regardless of where an individual with SCD receives care.

Improve the pediatric-to-adult care transition

by ensuring there are qualified physicians in both pediatric and adult care, through creation of training incentives and retention of providers who treat SCD.

Improve access to evidence-based care

through innovative health care delivery models and incorporate SCD care into the health care system and delivery.

Read more about the goals for the future at scdcoalition.org

1949

Two teams – Neel and Beat – independently publish on SCD inheritance, stating individuals with sickle trait were heterozygous for the gene, whereas those with SCD were homozygous.

1951

Pauling discovers the molecular nature of SCD by showing that the red, oxygen-carrying protein called “hemoglobin” has a different chemical structure in persons with SCD, making SCD the first “molecular disease” discovered.

1953

A diagnostic tool is developed to identify SCD.



TRAINING AND

PROFESSIONAL EDUCATION

CURRENT STATE



In a national survey, only 20.4 percent of family physicians reported that they felt comfortable treating people with SCD.¹⁵



69.4 percent of family physicians reported that clinical decision support tools would be useful for helping to guide their treatment decisions for individuals with SCD.¹⁶



72.6 percent of family physicians believed that more education and clinical support tools would help avoid complications in managing the disease.¹⁷

Currently, there are not enough health care providers with the comprehensive knowledge and expertise to care for people with SCD. This knowledge gap not only exists within specialty (e.g., hematologists) and primary care, but also among other provider groups, such as clinicians who provide hospital-based and outpatient care. This contributes to an enormous unmet medical need, especially for adults with the disease who need coordinated chronic care and ongoing pain management. The unpredictable and often persistent nature of the pain and other complications associated with SCD poses a difficult challenge for providers, especially for those unfamiliar with treating people with this disease.

Although comprehensive programs exist for some children with SCD, many adults do not have access to preventive and comprehensive care. Their health care providers may be inexperienced in treating SCD and the coordination of their care may be insufficient. And while there has been increased access to health insurance in the last few years, the number of providers with SCD expertise has not increased. Consequently, health

1954

It is discovered that sickle cell trait protects against malaria, explaining the prevalence of the sickle gene in regions where malaria is worldwide.

1955

A blood test is developed to identify abnormal hemoglobin, a method still used today to confirm SCD diagnosis.

1956

Ingram and Hunt sequence sickle hemoglobin showing that SCD occurs due to a change in a single amino acid, replacing a glutamic acid with a valine, in hemoglobin.

outcomes for people with the disease have not improved.¹⁸

The issues with access often relate to training and comfort with providing care. The number of hematologists trained and willing to care for children with SCD has increased, but still fewer are providing care for the more complex adults with SCD.¹⁹ In fact, a survey of prominent pediatric SCD centers reported that the largest barrier to transition and continuity of care was the ability to identify an adult provider – specifically a hematologist – with the training, expertise, and comfort in treating this population.²⁰ As a result much of the care of adults with SCD takes place in emergency departments or other non-specialty settings.⁸

Some people with SCD rely exclusively on the hospital or emergency department for care, due to a lack of SCD providers, which can greatly disrupt the continuity of care that they receive. In fact, nearly three-quarters of hospitalization for SCD originates in the emergency

department.¹⁹ Frequency of emergency department visits is substantially higher for adults with SCD than for children. This is potentially problematic because people with SCD often warrant a comprehensive assessment related to their other medical complications (i.e., comorbidities). However, the emergency care system is not designed to care for the chronic problems of an individual with SCD. Relying on uncoordinated episodic care also leads to increased health care costs and the potential for inadequate or inappropriate treatment.⁹

Primary-care providers, such as family physicians, often provide care to individuals with SCD; however, they report feeling that they do not have adequate backgrounds in SCD management due to their lack of experience treating these individuals. Factors currently associated with primary-care physicians' comfort level in treating SCD is whether or not they typically see people with this disease, or from their previous training experiences.²² Greater availability of clinical decision support tools

1963

Hemoglobin 3-D protein structure deciphered using x-ray crystallography.

1971

The National Association for Sickle Cell Disease is founded, now known as the Sickle Cell Disease Association of America (SCDAA).

1972

The *National Sickle Cell Anemia Control Act* is signed into law. It provides for establishment of voluntary SCD screening, education, research, and training.



Many adults are forced to rely on urgent care, leading to increased health care costs and the potential for inadequate or inappropriate treatment.

1972

Researchers find a milder SCD variant that is associated with increased levels of fetal hemoglobin, suggesting that an increased fetal hemoglobin level could serve as a treatment target.

1973

The National Sickle Cell Disease Program is established and National Heart and the Lung Institute begins funding comprehensive sickle cell centers and establishes a sickle cell branch at NIH.

1973

A neonatal screening method is developed that uses blood spots on filter paper.

for health care providers could help those making treatment decisions and provide high-quality care to individuals with SCD.

Despite the NHLBI recommendations on the use of hydroxyurea in the management of SCD, providers are often reluctant to prescribe this therapy or may not be knowledgeable about its use in SCD.²³ As a result, patients are often not well educated on hydroxyurea, leading to misinformation and poor adherence.²⁴

Increasing the focus of training programs on pathology, treatment, and research related to SCD will help expand the number of specialists who are qualified and willing to care for those with this disease. Increased emphasis on training providers who manage individuals with SCD, especially adults, and further implementation of coordinated care models may result in improved standards of care and greater availability of providers with SCD expertise.

GOALS FOR THE FUTURE

Develop an actionable plan to educate health care providers

about best practices in caring for those with SCD, including hematologists, primary-care providers, hospitalists, and emergency department physicians.

Augment pain management expertise through use of best practices and a thorough assessment of reversible conditions known to precipitate pain crises, such as dehydration and infection.

Increase the number of providers

who are able to care for those with SCD through training and certificate programs.

Develop clinical support tools to ensure quality of care for people with SCD.

Cultivate an interest in SCD care among clinicians in early medical training.

Read more about the goals for the future at [scdcoalition.org](https://www.scdcoalition.org)

1975

New York becomes the first state to require newborn screening for SCD.

1977

Gilbert and Sanger's DNA sequencing method allows the mapping of the sickle cell gene.

1978

Flavell maps the human beta and delta hemoglobin genes, pinpointing the DNA mutation resulting in SCD.

Prenatal DNA method of SCD diagnosis reported.



RESEARCH AND

CLINICAL TRIALS

CURRENT STATE



SCD was once associated with early childhood mortality, but today in the United States more than 90 percent of people with SCD live into adulthood, which poses new issues and challenges.²⁵



There is only one medication, hydroxyurea, currently approved by the FDA to treat adults with SCD.²⁶



In the United States, there are currently 36 clinical trials underway related to SCD to evaluate novel agents and approaches. This includes several trials involving bone marrow transplantation and gene therapy, which are potentially curative.



1980

The binding of sickled red blood cells inside blood vessels is shown to block blood flow, suggesting that the extent of stickiness may be a determinant of disease severity.

1984

Bone Marrow Transplant in child with SCD produces the first reported cure of the disease.

1986

Penicillin is recognized as an effective preventive measure in reducing infection and death in children with SCD.

The majority of available treatments manage symptoms of the disease, rather than treating the underlying cause of sickling.

Although the molecular basis of SCD was established more than half a century ago, it has been challenging to translate this research into the development of novel targeted therapies. While new approaches in managing this disease have improved diagnosis and supportive care over the last few decades, people with SCD still have severe complications to overcome.

There is no widely available cure for SCD and few effective treatments. Treatment for those with SCD focuses on disease and pain management, treatment of complications, and acute care during sickling crises, which occurs when red blood cells become rigid and sticky and block the flow through small blood vessels. Only one therapy, hydroxyurea, is approved by the U.S. Food and Drug Administration (FDA), but research shows that it is underused by health care providers and not taken consistently by individuals with SCD.

**1987**

NIH panel recommends screening all newborns in the United States for SCD and giving penicillin prophylaxis to all affected infants by 3 months of age.

1995

Anticancer drug hydroxyurea shown to reduce the frequent, painful complications of SCD.

1996

A new method allowing the use of maternal blood samples for prenatal SCD diagnosis is developed.

Regular blood transfusions are sometimes used as a preventive measure; however, they can lead to abnormally high levels of iron in the blood, which can cause long-term organ damage and reactions due to a mismatch between the donors and recipients.

Currently there are a number of clinical trials underway; however, the majority are single agent trials sponsored by the pharmaceutical industry and it will be years before they will translate into widely available and affordable therapies. Now more than ever there is a need to develop a clinical trial network that could increase the number of patients recruited and allow for assessment of combination therapies. Furthermore, there is great need for increased pain research and exploration of additional curative therapies. While bone marrow and stem cell transplantation are potentially curative, their use is limited due to high cost, the difficulty of locating matched donors, and the high level of risk associated

with the procedure. While still in an exploratory stage, gene therapy and genome editing hold the promise of a future approach for curing SCD and other hemoglobin disorders.

Today, there is an extraordinary opportunity to link research and care more closely. One way to achieve this is through a clinical research network. Some of the most pressing research questions that link to patient care include how to improve dosing and better anticipate response to treatments like hydroxyurea, investigating potential combinations of treatments, better defining the indications for transfusions in SCD, and improving the safety of transfusions by adopting accurate, state-of-the-art blood matching protocols. While clinical trial research helps to move the needle on improving SCD care, often these trials stagnate due to insufficient patient enrollment.^{27, 28} Therefore, it is imperative that individuals have a more active role in the clinical research process.

1996

A study finds that bone marrow transplantation in children with SCD can be curative if their sibling donors share a specific protein.

1997

Periodic blood transfusions in children with SCD are shown to reduce the risk of stroke by 90 percent.

1998

The FDA approves hydroxyurea as the first drug proven to be effective in the treatment of adults with SCD.

GOALS FOR THE FUTURE

Identify predictors of the severity of the disease, including optimizing dosing and treatment response predictors for hydroxyurea, biomarkers for SCD crisis and prognosis, and SCD diagnostic methods.

Optimize the use of existing therapies, better defining the indications for red blood cell transfusions, and identifying principles for accurate blood matching and developing longitudinal studies to determine long-term effectiveness of transfusions and hydroxyurea.

Develop novel therapies, including combination therapies, new drug-delivery modes, and new agents that can be used in combination with hydroxyurea.

Develop clinical trial networks to increase enrollment in clinical trials and share the data with interested stakeholders.

Strengthen curative therapies, such as bone marrow and stem cell transplantation, and support funding for research in SCD gene therapy and genome editing methods.

Enhance pain research for improved outcomes such as pain, fatigue, and infertility, and create a validated SCD-specific functional assessment tool for pain.

Enhance the participation of individuals with SCD in setting the research agenda and increase patient participation in clinical research.

Read more about the goals for the future at [scdcoalition.org](https://www.scdcoalition.org)

For the ASH Research Priorities for SCD and Sickle Cell Trait, click here.

2002

The Health Resources and Services Administration begins the SCD Newborn Screening Program.

2002

The Sickle Cell Adult Provider Network (SCAPN) is founded to establish and support an interactive network for health care providers and investigators who serve adults with SCD.

2003

The *Sickle Cell Treatment Act* of 2003 is signed into law by President George W. Bush.



GLOBAL ISSUES

CURRENT STATE



Prevalence of sickle cell trait varies greatly between different regions but reaches levels as high as 40 percent in some areas of sub-Saharan Africa, eastern Saudi Arabia, and central India.^{29, 30}



In resource-poor countries more than 90 percent of children with SCD do not survive to adulthood.³¹



Approximately 1,000 children in Africa are born with SCD every day and more than half will die before they reach five.³²



SCD has a high prevalence in India, especially in the central and western regions. Approximately 20 percent of children with SCD die by the age of two.³³

2004

U.S. Postal Service issues an SCD awareness postage stamp.

2005

The African Union approves the decision to submit a report to the United Nations about sickle cell anemia.

2006

The NHLBI launches the Sickle Cell Disease Clinical Research Network.

Organizations such as the World Health Organization (WHO) and United Nations (UN) have recognized SCD as a global health issue. In 2006, the World Health Assembly passed a resolution recognizing SCD as a public health priority and called on countries to tackle the disease. This resolution was also adopted by the United Nations in 2009.

The global picture of SCD is similar to that of the disease in the United States before 1970. In countries with poor public health systems and high levels of poverty, SCD remains a major killer of infants and

children, similar to other diseases like malaria and HIV/AIDS. In resource-poor countries, 90 percent of children with SCD do not survive to adulthood. And the problem is growing; by 2050 the number of people with SCD is expected to increase by about 30 percent globally.³⁴

Some middle-income countries are making advancements in both early diagnosis and management of SCD. Brazil, for example, has shown remarkable progress over the past two decades with newborn screening programs being offered across the country.³⁵

**2007**

ASH hosts workshop on SCD Research.

2007

The Global Congress on SCD was established by the WHO and the Thalassemia International Federation with the aim of uniting SCD groups across the globe on SCD efforts.

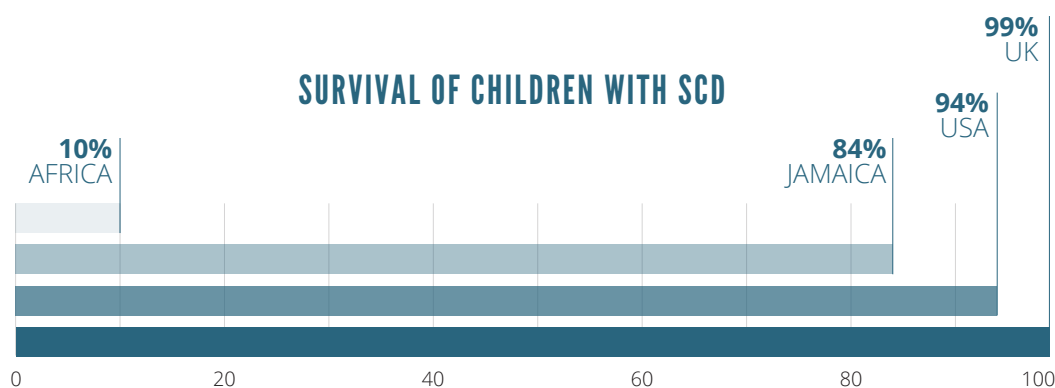
2008

UN passes a resolution recognizing sickle cell anemia as a public health problem, declaring June 19 as World Sickle Cell Disease Day and urging increased awareness and improved care.

A 10-year study in Rio de Janeiro showed that diagnosis through newborn screening programs and treatment was associated with improved survival and quality of life of Brazilian children with SCD. Moreover, in Brazil, health care maintenance for SCD is seen as an essential component of primary care, and the government supplies hydroxyurea free of charge. This type of program has resulted in increased awareness and education for SCD, although there have been only modest improvements in mortality rates. Jamaica represents another example of a middle-income

country with SCD screening and treatment approaches that have resulted in remarkable improvements in the median life expectancy.³⁶

As newborn screening and treatment efforts are implemented around the world, higher survival rates will increase the need for improved treatment options for adults with SCD and ongoing care. In developing our goals for the future, one of our greatest priorities is designing, testing, and implementing sustainable care and pain management approaches for countries with limited resources.



Source: The National Institutes of Health, www.fic.nih.gov/News/GlobalHealthMatters/november-december-2014/Pages/sickle-cell-disease.aspx

2009

NHLBI convenes workshop of SCD stakeholders to discuss key public outreach issues.

2009

NHLBI study finds a modified blood adult stem cell transplant regimen reverses SCD in 90 percent of adults severely affected by disease.

2011

ASH hosts workshop on sickle cell trait (SCT) and athletic participation.

GOALS FOR THE FUTURE

Establish and/or expand newborn screening and early intervention programs.

Increase education of governments and philanthropic groups about the importance of screening and caring for individuals with SCD in heavily burdened countries.

Improve global access to care providers, such as increasing the number of health care providers treating SCD and improving training for health care professionals.

Develop standard-of-care guidelines

that apply to specific, low-resource areas globally including SCD adult care and enhanced use of community-based organizations.

Develop a structured approach to addressing pain in low-resource settings.

Read more about the goals for the future at scdcoalition.org

2012

ASH releases statement on screening for SCT and athletic participation.

2014

NHLBI releases guidelines for managing SCD, stressing the use of the drug hydroxyurea and transfusions for many with the genetic disorder.

2014

ASH releases a list of priorities for SCD and sickle cell trait that seeks to address remaining questions and specific research topics that could move the field forward.

Conclusion

Why is now the right time to focus on this disease?

SCD is a chronic disease that has been neglected for far too long. Those affected by this disease are among the most vulnerable and underserved, and the disease has a profound impact on their lives.

Currently, the only approved drug for adults with SCD – hydroxyurea – reduces the severity and frequency of painful episodes and is used for stroke prevention, but may not prevent acute complications or reverse organ damage that can result in early death or other health problems which affect their quality of life (i.e., comorbidities). There is a need for the development of new treatments and a widely available and affordable cure for this disease. Existing treatments and cures, including bone marrow and stem cell transplantation, are underutilized and do not reach the majority of individuals – especially adults with SCD who could benefit from them. Increased strategies to educate providers and people living

with SCD are also needed to address issues of fragmented care. There is a tremendous disparity in SCD outcomes between individuals in low- and middle-income countries compared to high-income countries.

The status quo is unacceptable. It is imperative that we vastly improve the circumstances under which care is provided. There is opportunity to address these disparities and alter the course of SCD, by improving overall treatment, care, and quality of life for millions of people, especially young children. A number of organizations are coming together to address this issue and each has important assets to help advance a comprehensive SCD action plan. To make real change for those affected by SCD, we need participation from many different stakeholders – including government agencies, patient advocacy organizations, health care providers, researchers, foundations, and the private sector. Those with SCD are waiting, and their well-being depends on our help. Their future is in our hands.

The time is now to change the course of the disease.

Will you join us? For more information visit scdcoalition.org

2015

ASH hosts the ASH SCD Summit: A Call to Action as a first step to a broader initiative, to identify the highest priority actions needed to improve outcomes for individuals with SCD in the U.S. and globally.

2015

2015 Tennessee legislature passed Senate Bill 1074, which authorizes TennCare (Medicaid program) to provide medical assistance for SCD management services and for public education campaign activities related to the disease.

ASH released SCT Educational Toolkit with CDC and SCDA.

2016

Sickle Cell Disease Coalition is established.

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