



American Society of Hematology  
Helping hematologists conquer blood diseases worldwide



# **ASH's Multifaceted Initiative on Sickle Cell Disease (SCD)**

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# ASH's Multifaceted Initiative on SCD

- Development and Implementation of ASH Projects to Address:
  - Access to Care in the United States
  - Global Issues
  - Research
- Development of ASH's New SCD Guidelines
- ASH Policy Strategy on SCD
- SCD Coalition
  - *Call to Action on SCD*



# Development and Implementation of ASH Projects

- Access to Care in the United States
  - Implementation of Educational Strategy
- Global Issues
  - Develop Newborn Screening and Early Intervention Consortium in Africa
- Research
  - Explore Development of a SCD Clinical Trials Network (SCDCTN)



# Access to Care in US: Implementation of SCD Education Strategy

- Health Provider Focused Education and Training Group
  - Development and Implementation of Educational Strategies to Improve the Care of Individuals with SCD
  - Target Audiences: Hematologists; PCPs; Hospitalists; and Patients



# Global: Exploration of a SCD Newborn Screening & Early Interventions Consortium

- Goal: Establish a registry study modeled after the ASH International Consortium on Acute Leukemia (ICAL) for SCD in sub-Saharan Africa:
  - Set standard protocols for newborn screening in participating countries/institutions
  - Implement standard early interventions, treatment and registry protocols
- Aim: Examine the effect of introduction of these interventions on changing the course of treatment and outcomes for these populations



The Global Burden of SCD

# Research: Explore Development of a SCD Clinical Trials Network (SCDCTN)

- Development of a SCD Clinical Trials Network (CTN) was the top priority among the research priorities identified.
- Goal: Identify the Highest Priority Research Questions for the SCD CTN
  - Scope: To Facilitate the Clinical Study of New and Existing Therapies to Cure and Manage the Symptoms of SCD



# Development of ASH's New SCD Guidelines

## New ASH Clinical Practice Guidelines on the Management of Acute & Chronic Complications of Sickle Cell Disease

- Topics addressed include:
  - *Pain*
  - *Cerebrovascular Disease*
  - *Stem Cell Transplantation*
  - *Transfusion support*
  - *Cardiopulmonary & Kidney Disease*
- Publication of the five guidelines are anticipated in 2019



# ASH SCD Policy Priorities

- Goals:
  - Enhance federal government activities in SCD research, training and services
  - Improve reimbursement for care and treatment of SCD patients to assure adherence to recognized standards of care





# Sickle Cell Disease Coalition



***[www.scdcoalition.org](http://www.scdcoalition.org)***

*Goal: Amplify the voice of the SCD stakeholder community, promote awareness and improve outcomes for individuals with SCD*

- 50 SCDC Members
  - Patient groups – 4
  - Public health, research, and provider organizations – 29
  - Federal agencies – 3
  - Industry – 11
  - Foundations – 3
- Newsletter – *SCDC Update*
- SCDC Working Groups
  - Access to Care
  - Global
  - Research



# The Current State of SCD

Four priorities to advance SCD care:

1. Access to Care in the United States
2. Training and Professional Education
3. Research and Clinical Trials
4. Global Issues Related to SCD



American Society of Hematology

## STATE OF SICKLE CELL DISEASE

2016 REPORT

**STATE OF SICKLE CELL DISEASE | 2016 REPORT CARD**

Sickle cell disease (SCD) — an inherited disorder that causes a person's red blood cells to become deformed and get stuck in veins, blocking oxygen flow throughout the body — can cause severe pain, stroke, organ failure, and even death.

While there's no widely available cure for SCD, care is obtainable. But that care is inconsistent in the United States and wholly absent in large parts of the world. To understand where SCD care stands today, the American Society of Hematology (ASH) has polled individuals with SCD, health care providers, and global health leaders. Though advances have been made, their scores show us that we have much to do to improve the state of care for those living with SCD.

Priority	Score
ACCESS TO CARE (U.S.)	3.7
TRAINING AND PROFESSIONAL EDUCATION	3.2
RESEARCH AND CLINICAL TRIALS	4.7
GLOBAL ISSUES	3.2

**ACCESS TO CARE (U.S.)**  
**CURRENT STATE**  
In the United States, access to appropriate care is limited by a number of factors, including health insurance, availability of knowledgeable health care providers, provider experience, geography, economic status, and co-existing conditions. Additionally, the transition from pediatric to adult care can be especially challenging, and the focus needs to shift from acute care of complications to a chronic care model.  
**GOALS FOR THE FUTURE**  
• Develop evidence-based guidelines and consistent health care delivery models to ensure that individuals with SCD can access quality care regardless of age, location, and socioeconomic status.

**TRAINING AND PROFESSIONAL EDUCATION**  
**CURRENT STATE**  
There are not enough health care providers with comprehensive knowledge and expertise to care for people with SCD. The unpredictable and often persistent nature of the pain and complications associated with SCD poses a difficult challenge for providers, especially those inexperienced with treating people with this disease. Many family physicians feel they do not have adequate background in SCD management, making it essential to train more providers.  
**GOALS FOR THE FUTURE**  
• Increase the number of providers able to care for those with SCD by educating clinicians to treat symptoms and complications while encouraging medical trainees to pursue careers in SCD care.

**RESEARCH AND CLINICAL TRIALS**  
**CURRENT STATE**  
There is only one FDA-approved treatment (hydroxyurea) for adults with SCD — it is often used off-label in children. Hydroxyurea is under-prescribed in the United States and largely unavailable abroad. This, coupled with limited funding to research and test new cures, severely limits the care individuals can receive.  
**GOALS FOR THE FUTURE**  
• Invest in strategies to expand use of existing treatments, develop novel therapies, and strengthen curative options while accelerating their discovery.

**GLOBAL ISSUES**  
**CURRENT STATE**  
SCD is a major killer of infants and children in the developing world, especially in Africa and India where the disease is more common. A lack of resources has limited global progress in increasing awareness and education of SCD.  
**GOALS FOR THE FUTURE**  
• Expand newborn screening and early intervention programs, increase SCD awareness and education, and improve access to quality care in developing regions.

Available at [www.scdcoalition.org/report](http://www.scdcoalition.org/report)

For a more detailed look at the state of SCD, and how a coalition of groups with an interest in SCD are working to improve care worldwide, visit [scdcoalition.org](http://scdcoalition.org)



# Summary

- Developing tools to educate health care providers in all settings to treat people with SCD.
- Working to build a consortium of African countries to institute a population-based registry study for newborn screening and early intervention
- Exploration of a Clinical Trials Network for SCD
- Clinical practice guidelines describing the management of acute and chronic complications of SCD.
- Working with Congress and federal agencies to enhance and expand federal SCD programs (US).





***[www.hematology.org](http://www.hematology.org)***

***[www.scdcoalition.org](http://www.scdcoalition.org)***

