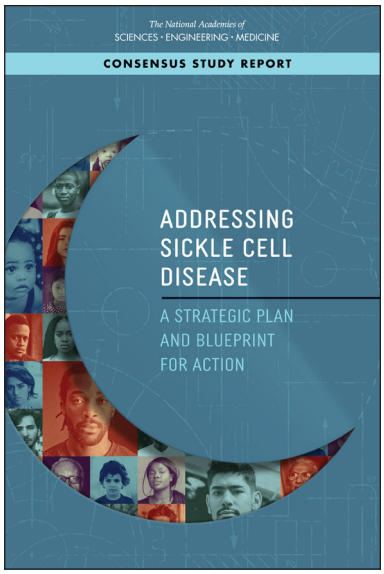
Consensus Study Report
SEPTEMBER 2020
HIGHLIGHTS

Addressing Sickle Cell Disease



There are approximately 100,000 people living with sickle cell disease (SCD) in the United States and millions more globally. Sickle cell trait (SCT) is even more prevalent and occurs in 1–3 million Americans and 8–10 percent of African Americans in the United States. Current estimates indicate that about 300,000 people are born with SCD each year worldwide and that more than 100 million people across the globe live with SCT. The sickle gene is more common in people of African descent, but can also be found in Hispanic-Americans from Central and South America, and people of Middle Eastern, Asian, Indian, and Mediterranean descent.

Since its discovery in 1910, SCD has received relatively little attention and few resources from the scientific, clinical, and public health communities compared with other genetic disorders, such as cystic fibrosis. Until December 2018 there was only one drug approved by the U.S. Food and Drug Administration (FDA) for the condition. A contributing factor to this lack of awareness and resources is that the affected population, which is primarily composed of racial and ethnic minorities, contends with persistent discrimination in the health care system and racism in society at large. Funding for SCD has also historically been low, especially compared to federal and private funding for other conditions, and has decreased over the years.

To accelerate progress for those living with SCD, the Assistant Secretary for Health at the U.S. Department of Health and Human Services (HHS), through the Office of Minority Health, asked the National Academies of Sciences, Engineering, and Medicine to form an ad hoc committee that would develop a strategic plan and blueprint to address SCD in the United States. The resulting committee developed this consensus study report as an answer to that charge.

CONCEPTUAL FRAMEWORK

The inherent health disparities and inequities in SCD and the disease burden on the impacted population underscore the urgency for action. To focus its work, the committee developed a conceptual framework based on two key elements of SCD treatment: addressing health throughout the life span and patient-centered care.

In order to characterize the need for targeted interventions at different life stages, the committee developed a model for understanding the comprehensive needs for SCD across the life span. The dimensions of the life span model include public education and awareness, access to comprehensive health care and therapeutic products, research and longitudinal data, educational support, vocational rehabilitation, and emerging needs, especially for an aging cohort of individuals with SCD.

Because SCD is a lifelong condition, care must be person-centric. Care teams must function as a partnership between providers and patients who are empowered to make decisions about their own health. There is also a need to establish acceptable standards of routine and specialty care that individuals living with SCD require and should receive across the life span.

KEY FINDINGS AND CONCLUSIONS

Overall, the committee found a lack of data to characterize the burden of disease, outcomes, and the needs of those with SCD. The available data are also outdated. Better data collection is necessary to inform care for SCD throughout the life span. This may require longitudinal data systems that will inform evolving care and service needs over time. While most SCD complications are common to both children and adults, specific ones may be more prevalent at different ages. As individuals with SCD survive from childhood into adulthood, information is needed about the appropriate service needs.

Stigma and racism have been shown to negatively affect access to care, treatment, psychological health, and disease outcomes of individuals with SCD in the United States and globally. However, their impacts are often conflated in the literature, and their mechanisms of action are poorly understood. Further studies are needed to understand the separate and combined influences of stigma and racism on individuals with SCD and to develop and implement strategies to address them.

Pediatric care for SCD has improved over the years, but adults with SCD need better systems of comprehensive, team-based care. Many of the approaches to SCD management lack a good evidence base, particularly to support current treatment strategies. The lack of evidence-based guidelines results in inconsistent treatment approaches across care delivery centers and providers, reduced access to care, and poor outcomes.

A STRATEGIC PLAN TO IMPROVE SICKLE CELL DISEASE CARE AND OUTCOMES

Based on its review of the current needs for treating SCD and input from public comments, the committee developed a strategic plan and blueprint for SCD action and identified strategies and specific actions for improving care and outcomes. The vision for the strategic plan is to ensure "long, healthy, productive lives for those living with SCD and those with SCT" and to ensure that SCD populations receive high-quality care equal to that which every person deserves.

The strategic plan consists of eight overarching strategies or "pillars" that support the vision, and seven foundational principles that undergird the strategic plan (see Box 1). Each strategy takes into account the multifaceted needs of the SCD and SCT populations, and each is equally important. Within each pillar, the committee made a number of recommendations on how to transform SCD and SCT care and outcomes. These recommendations include action steps for federal departments and agencies at HHS, the Social Security Administration, the U.S. Department of Education, and corresponding state agencies.

The committee also proposed a blueprint with a timeline for implementing the strategic plan. To ensure meaningful and sustained progress on implementation, the committee recommended that the Office of the Assistant Secretary for Health at HHS should appoint an oversight body with members from across HHS agencies to oversee the rollout of the strategic plan and blueprint. The appointment of the oversight body should be immediate. For a full outline of the committee's strategic plan, blueprint, and recommendations, see the Recommendations insert.

BOX 1 STRATEGIC PLAN FOR ADDRESSING SICKLE CELL DISEASE

Strategic Vision: Long, healthy, productive lives for those living with sickle cell disease (SCD) and those with sickle cell trait (SCT).

- **Strategy A:** Establish a national system to collect and link data to characterize the burden of disease, outcomes, and the needs of those with SCD across the life span.
- **Strategy B:** Establish organized systems of care that ensure both clinical and nonclinical supportive services to all persons living with SCD.

- **Strategy C:** Strengthen the evidence base for interventions and disease management and implement widespread efforts to monitor the quality of SCD care.
- **Strategy D:** Increase the number of qualified health professionals providing SCD care.
- **Strategy E:** Improve SCD awareness and strengthen advocacy efforts through targeted education and strategic partnerships among the U.S. Department of Health and Human Services, health care providers, advocacy groups, community-based organizations, professional associations, and other key stakeholders (e.g., media and state health departments).
- **Strategy F:** Address barriers to accessing current and pipeline therapies for SCD.
- **Strategy G:** Implement efforts to advance understanding of the full impact of SCT on individuals and society.
- **Strategy H:** Establish and fund a research agenda to inform effective programs and policies across the life span.

Foundational Principles: *Safe · Effective · Patient-centered · Timely · Efficient · Equitable · Ethical*

FUTURE DIRECTIONS

Central to the committee's strategic plan is the need for better longitudinal data on care for individuals with SCD throughout the life span. A key component of this effort is a national system to collect and link data to characterize the burden of disease, outcomes, and the needs of those with SCD across the life span. Such a system can be developed by building on current and previous data collection efforts by the Centers for Disease Control and Prevention, developing a clinical registry for SCD, and linking existing datasets on the SCD population.

The committee also recommended that the oversight body established by the Office of the Assistant Secretary for Health at HHS should collaborate with health professional associations, researchers, individuals living with SCD, and funders to develop a robust research agenda with priority topics that need to be studied.

Other priorities for improving care and outcomes include, but are not limited to:

- **Coordinated Care.** Stakeholders throughout the SCD care delivery system should establish organized systems of care that ensure both clinical and nonclinical supportive services to all persons living with SCD.
- **Community-Based Services.** HHS, in collaboration with state health departments and health care providers, should partner with community-based organizations and community health workers to engage the SCD population in designing educational and advocacy programs and policies and in disseminating information on health and community services to individuals living with SCD and their caregivers.
- **Therapies for SCD.** HHS should ensure access to current and pipeline therapies for SCD by identifying approaches for financing the upfront costs of curative therapies and encouraging and reimbursing the practice of shared decision making and the development of decision aids for novel, high-risk, or potentially highly effective therapies for individuals living with SCD.
- **Clinical Trials.** In order to encourage participation in clinical trials, the National Institutes of Health, FDA, the pharmaceutical industry, and the research community should establish an organized, systematic approach that includes affected individuals in the design of trials, working with community-based organizations to disseminate information and recruit participants, and conducting other targeted activities.
- **Newborn Screening.** The Health Resources and Services Administration should work with states to standardize the communication of and use of newborn screening positive results for SCT status in genetic counseling and should create a mechanism for communicating this information across the life span and ensuring access to needed support and services.

Committee on Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action

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