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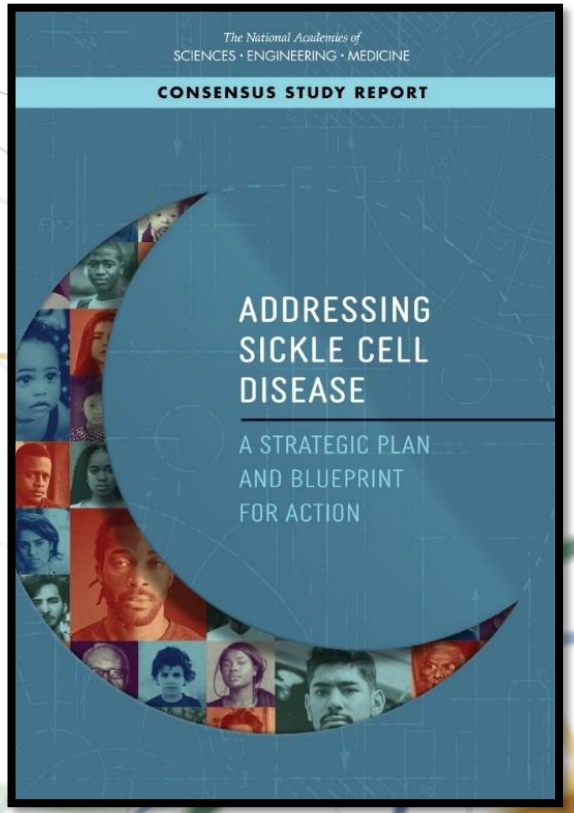

Public Release Webinar

September 10, 2020

Committee

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Sickle Cell

- Genetic mutation identified in 1910
- ~100,000 people living with Sickle Cell Disease (SCD) in the U.S
- ~ 1-3 million Americans have Sickle Cell Trait (SCT)
- ~8-10% of African Americans have SCT

Characteristics of SCD

- Historically, the population affected by SCD has contended with racism and implicit bias within and outside the health care system; socially, they are stigmatized because of cultural beliefs and the lack of general understanding of the disease.

Characteristics of SCD (cont.)

- Pain
- Pediatric and adult health care delivery vary considerably
- Mortality increases with age
- Limited resources to address SCD (e.g. research on treatments, care delivery)

Statement of Task

The Department of Health and Human Services, Office of Minority Health requested that NASEM **develop a strategic plan and blueprint for addressing sickle cell disease (SCD) in the United States**. The plan should include a review of:

- the epidemiology, health outcomes, genetic implications, and societal factors associated with SCD and sickle cell trait (SCT), including serious complications of SCD such as stroke, kidney and heart problems, acute chest syndrome, and debilitating pain crises;
- current guidelines and best practices for the care of patients with SCD;
- to the extent possible, the economic burden associated with SCD; and
- current federal, state, and local programs related to SCD and SCT, including screening, monitoring and surveillance, treatment and care programs, research, and others.

Statement of Task (cont.)

The report should provide guidance on priorities for programs, policies, and research and make recommendations as appropriate, regarding:

- limitations and opportunities for developing national SCD patient registries and/or surveillance systems;
- barriers in the healthcare sector associated with SCD and SCT, including access to care and quality of care, workforce development, pain management, and transitions from pediatric to adult care;
- needed innovations in research, particularly for curative treatments such as gene replacement/gene editing and increasing awareness and enrollment of SCD patients in clinical trials; and
- the expanded and optimal role of patient advocacy and community engagement groups.

Committee guidance should be formulated around strategic objectives (strategic plan) and action steps (blueprint). Throughout all the deliberations, the committee will give consideration to ethical issues related to SCD and SCT.

Committee Approach

Data Gathering Activities:

- Review of the literature (primarily U.S. based)
- Five deliberative committee sessions
- Five public data gathering meetings
  - Received input from patients and patient advocates, providers, pharmaceutical manufacturers, researchers and representatives from relevant SCD programs
- Visit to Georgia Comprehensive Sickle Cell Center located at Grady Memorial Hospital

Conceptual Approach

- Life-span approach: the needs and specific challenges of individuals with SCD may vary according to different stages in life.
- The experience of SCD is shaped by sociocultural factors, environmental factors, and socioeconomic factors, which can exacerbate the disease's impact for people from racial and ethnic minority groups living with the disease.
- Person-centric care: SCD must be managed as a chronic disease, which requires an ongoing person-centric, collaborative approach to care management.

Organization of the Report

Summary

Chapter 1:Introduction

Chapter 2: Societal and Structural Contributors to Disease Impact

Chapter 3: Screening, Registries, and Surveillance

Chapter 4: Complications of Sickle Cell Disease and Current Management Approaches

Chapter 5: Health Care Organization and Use

Chapter 6: Delivering High-Quality Sickle Cell Disease Care With a Prepared Workforce

Chapter 7: Developing and Delivering the Next Generation of Therapies

Chapter 8 Community Engagement and Patient Advocacy

Chapter 9: Strategic Plan and Blueprint For Sickle Cell Disease Action

Appendixes

The Impetus for Action

- SCD exemplifies the experience and consequences of health disparities and inequities in the U.S.
- The burden of SCD is immense – for the individual and their family, for their community and for the larger society.

Committee's Vision and Foundational Principles for the Strategic Plan

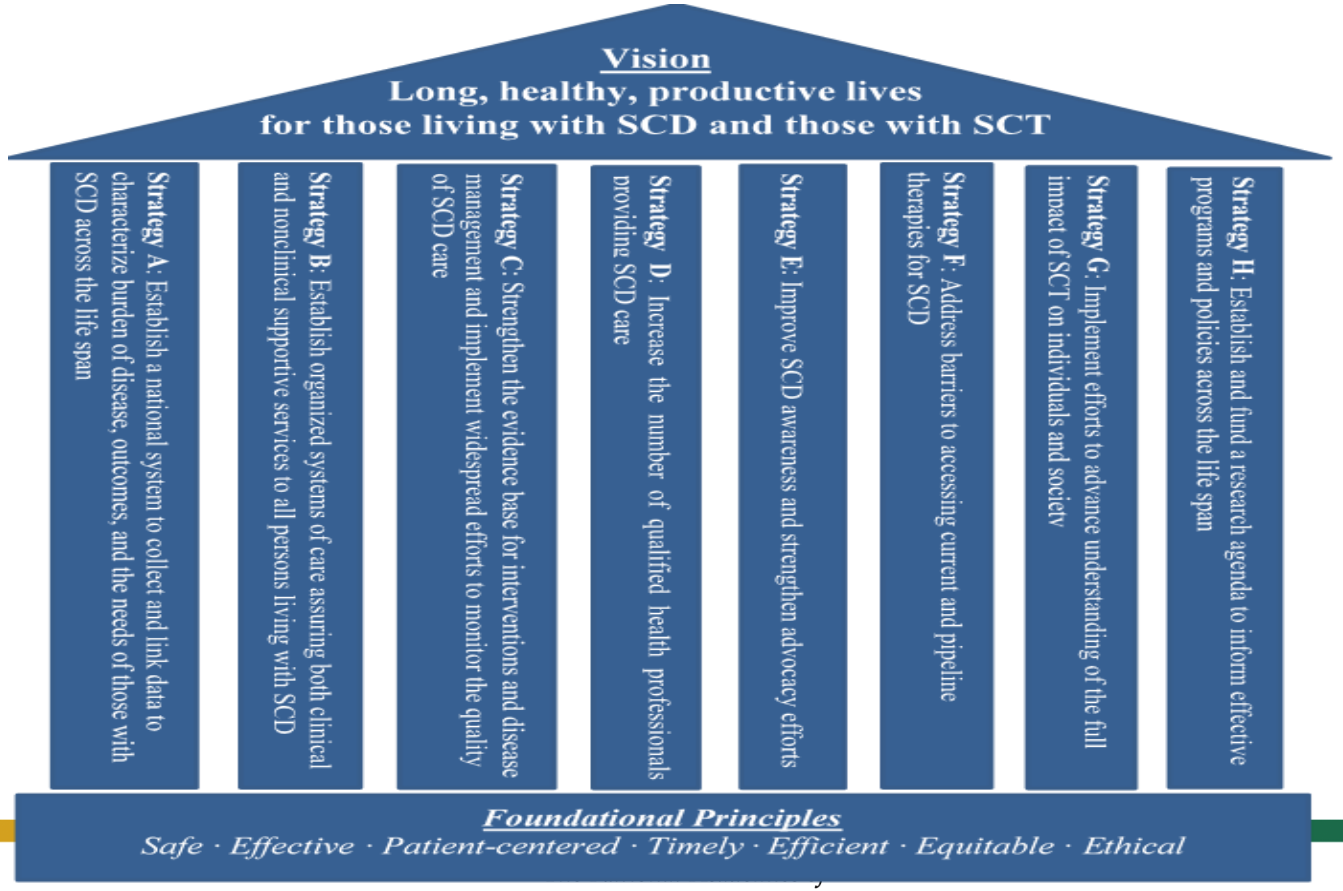
Vision

- Ensure long, healthy, productive lives for those living with SCD and those with SCT.

Foundational Principles

- Health care be safe, effective, patient-centered, timely, efficient, equitable, and **ethical**.

Strategic Plan for Improving SCD Care and Outcomes



Strategic Plan and Blueprint for Action

Eight strategies or “pillars” that support the vision

- **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.

Strategic Plan and Blueprint for Action (cont.)

- **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 key stakeholders.
- **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.

Strategy A: Findings

- Important data gaps exist for SCD. Longitudinal data collection systems would provide data for decision making and evaluation of needed changes in SCD care.
- Inconsistent communication of SCD results to parents/guardians and providers and in follow-up once diagnosed across state newborn screening (NBS) programs. Thus, newborns with SCD and their families do not receive standardized quality care and familial support across state programs.
- Inconsistent follow-up and communication of positive SCT status to parents, relevant providers, and young adults seeking trait status from NBS systems. Thus, some people with SCT are unaware of their status; this can affect future reproductive decisions and/or health.

Strategy A: Action Steps

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.

- Develop state public health surveillance systems to support a national longitudinal registry of all persons with SCD *(1-2 years)*.
- Develop a clinical data registry for SCD *(1-2 years)*.
- Establish a working group to identify existing and disparate sources of data that can be immediately linked and mined *(1-2 years)*.

Strategy B: Findings

- Vaso-occlusive crises and frequent hospitalizations increase the risk for learning difficulties, poor performance, and absenteeism among children. Educational supports may be needed but parents and others may be uninformed of the process for obtaining them.
- Silent cerebral infarcts into adulthood may impact cognitive function; vocational rehabilitation services may open door to job market.
- Existing SSI disability qualifications for SCD do not reflect the full impact of the disease on functional status. Criteria penalize patients that receive high-quality care that reduces disability but then lose access services.

Strategy B: Findings (cont.)

- A comprehensive, multidisciplinary system of SCD care is needed that includes a collaborative process for delineating the elements of care and providers. Models exists to learn from. Comprehensive SCD centers could be certified.
- Novel payment approaches are needed to support a coordinated system of SCD care.

Strategy B: Action Steps

Establish organized systems of care that ensure both clinical and nonclinical supportive services to all persons living with SCD.

- Develop educational materials to provide guidance to a broad range of education staff and providers to support the medical and academic needs of students with SCD *(1-2 years)*.
- Review SSI disability insurance qualifications *(1-2 years)*.
- Expand and enhance vocational rehab programs for individuals living with SCD *(2-3 years)*.
- Convene a panel of relevant stakeholders to delineate the elements of a comprehensive system of SCD care*(2-3 years)*.
- Develop and pilot reimbursement models for the delivery of coordinated SCD health care and support services *(3-4 years)*.

Strategy C: Findings

- There is excess SCD mortality in adulthood attributed to not receiving appropriate care or high-quality care.
- Quality improvement efforts in SCD have lagged behind those in other diseases.
- There is a need to generate evidence to address gaps and standardize and promote the delivery of high-quality SCD care.
- Quality indicators for SCD are needed that are consensus driven.
- SCD could benefit from the development of quality improvement collaboratives.

Strategy C: Action Steps

Strengthen the evidence base for interventions and disease management and implement widespread efforts to monitor the quality of SCD care.

- Fund efforts to identify and mitigate potentially modifiable disparities in mortality and health outcomes *(1-2 years)*.
- Develop and track a series of indicators to assess the quality of SCD care *(1-2 years; 3-5 years)*.
- Fund and conduct research to close the gaps in the existing evidence base for SCD care *(3-5 years)*.
- Foster the development of quality improvement collaboratives *(3-5 years)*.
- Require the reporting of expert consensus-driven SCD quality measures and other metrics of high-quality health care for persons with SCD *(3-5 years)*.

Strategy D: Findings

- A number of health professions training and research support opportunities exist that could be better used to incentivize professionals in to SCD care delivery and research.
- Health professional training could be enhanced through a SCD Academy. The HIV Academy is one model.
- Fellows in hematology/oncology training programs have reported few mentorship opportunities, especially for SCD.

Strategy D: Action Steps

Increase the number of qualified health professionals providing SCD care.

- Disseminate information on loan repayment opportunities to incentivize health care professionals interested in conducting research on SCD *(1-2 years; 3-5 years)*.
- Convene an Academy of SCD Medicine to support SCD providers through education, credentialing, networking, and advocacy *(2-3 years)*.
- Develop early and effective mentoring programs to link early career health professionals with seasoned providers *(3-5 years)*.

Strategy E: Findings

- Lack of public knowledge and understanding about SCD continue to perpetuate stigma that confronts individuals living with the disease.
- There is a need for culturally and linguistically relevant educational materials for individuals living with SCD, especially on care management approaches and emerging clinical research.
- Several community-based programs (such as camps, peer-mentoring, and transitions programs) have been proven to be effective in supporting care management for those with SCD. Effective programs need to be identified and replicated.

Strategy E: Action Steps

Improve SCD awareness and strengthen advocacy efforts through targeted education and strategic partnerships among key stakeholders.

- Engage with media to improve awareness about the disease and address misconceptions about the disease and those affected *(1-2 years)*.
- Engage the SCD population in designing educational and advocacy programs and policies and in disseminating information on health and community services *(1-2 years)*.
- Translate and disseminate emerging clinical research information to people living with SCD and their families *(2-3 years)*.

Strategy F: Findings

- The high cost of novel curative therapies for SCD pose a substantial barrier to access which should be addressed to ensure access for all individuals with SCD.
- There are several unanswered questions about novel curative therapies. Emphasis should be placed on counseling patients on the uptake of therapies that are high risk and equipping them with tools and knowledge that foster provider–patient shared decision making.
- Several entities (PCORI, ASH, FDA, and NIH) have existing activities to foster patient-centric clinical trials design and encourage participation in clinical trials. Best practices from these efforts need to be standardized and scaled.

Strategy F: Action Steps

Address barriers to accessing current and pipeline therapies for SCD.

- Encourage and reimburse the practice of shared decision making and the development of decision aids *(1-2 years; 3-5 years)*.
- Identify approaches to financing the upfront costs of curative therapies *(2-3 years)*.
- Establish an organized, systematic approach to encourage participation in clinical trials *(2-3 years)*.

Strategy G: Findings

- Misconceptions about SCT perpetuate stigma about trait carriers and associated risks of SCT status.
- Research indicates that SCT may be a risk factor for health complications and sudden death in certain rare, extreme instances; there is a need for further studies to understand the extent of these complications and how to eliminate them.
- SCT status information is collected as part of NBS in all 50 states, the District of Columbia, and U.S. territories. However, communication of trait status is not standardized and passed along to individuals or families across the life span for use in future decision-making.

Strategy G: Action Steps

Implement efforts to advance understanding of the full impact of SCT on individuals and society.

- Disseminate information to promote awareness and education about the potential risks associated with SCT *(1-2 years)*.
- Fund research to elucidate the pathophysiology of SCT *(2-3 years)*.
- Standardize the communication of and use of newborn screening positive results in genetic counseling *(2-3 years)*.

Strategy H: Findings

Persistent lack of funding for SCD has resulted in substantial gaps in knowledge about the disease. Federal and private funders, health professional associations, researchers and patients should collaborate to develop a robust research agenda with priority topics that need to be studied.

Strategy H: Action Steps

Establish and fund a research agenda to inform effective programs and policies across the life span *(1-2 years; 3-5 years)*. Areas for future research include:

- Societal and structural challenges;
- Current management approaches;
- Health care organization and utilization;
- Developing and delivering the next generation of therapies.

Blueprint for Implementation

In order to make meaningful and sustained progress on implementation, the committee strongly suggests the following:

- **Oversight body:** appointed by the Office of the Assistant Secretary for Health (OASH) with representation from across HHS. The current HHS Sickle Cell Disease Workgroup is one option for such a body.
- **Regular assessments**: to evaluate progress of implementation. The first should occur no later than 5 years after the release of this report.
- **Multiple timeframes:** divided into short-term (1-2 years), mid-term (2-3 years) and longer-term (3-5 years).

Timeframe for Implementation

26 recommendations/action steps implemented over a period of 5 years

short-term (1-2 years)	mid-term (2-3 years)	long-term (3-5 years)
9 recommendations	8 recommendations	9 recommendations*

* Include 4 recommendations with short-term/long-term actions.

QUESTIONS?

Public release date:
September 10, 2020
2-3pm ET

Download the free report from https://www.nap.edu/