Sickle Cell Disease: Epidemiology, Surveillance, and Research in the U.S.

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Committee on Addressing Sickle Cell Disease: a Strategic Plan and Blueprint for Action
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“the true number of individuals with SCD remains unknown, and in the absence of a reliable surveillance system, populations will continue to be quantified by estimation”

-Dr. Kathy Hassell, 2010

Additional gaps in knowledge about SCD

- Where do patients with SCD receive care, outside of specialty centers?
- Who takes care of patients with SCD, outside of specialty centers?
- Does ongoing, extended care at a specialty center result in better health outcomes?
- Are patients with SCD receiving recommended SCD therapies?
- Are patients with SCD receiving age-appropriate preventive care?
- Are the SCD-related complications for patients who live past the age of 50 different than for those who die at an earlier age?
SCD surveillance in the U.S.
Sickle Cell Data Collection (SCDC)
Why is population-based data important?
Why is longitudinal data important?

Paulukonis, S., *Episodes of High Emergency Department Utilization among a Cohort of Persons Living with Sickle Cell Disease 2005-2016, ASH 2018 Conference*
Why is SCDC important?

- Provider Education
- Policy Makers
- New Clinics
- New Therapies
- CBO Activities
- Federally-Funded Activities
How can SCDC expand?

Additional States

Additional Data
How can SCDC expand beyond surveillance?

Surveillance

Patient Reports

Health Records

Biospecimens

LINKED

Example: ALS

- A congressionally mandated data collection system for persons in the U.S. with ALS that is maintained by CDC.
- The only population-based data in the U.S. that collects information to help scientists learn more about who gets ALS and its causes.
- Partners: CDC, National Institutes of Health ALS Clinical Trials, ALS Association, Muscular Dystrophy Association ALS Division, Les Turner ALS Foundation, ALS Untangled
- Administrative data, biorepository and self-registration through the web portal

https://www.cdc.gov/als/Default.html
ALS success stories

- Prevalence and demographics of the ALS population in the U.S.
- Patient surveys
- Information sharing with enrolled patients about new clinical trials, research studies, and opportunities to participate
- 16 funded research projects, including R01s
  - Assessing risk factors for health indicators
  - Protective role of biomarkers on disease progression
  - Effect of new therapies on disease progression
  - Role of environmental factors
  - Effect of socioeconomic factors on health outcomes
  - Survival and causes of death
Surveillance and research re-cap

1. National surveillance is needed to accurately determine the number of people with SCD in the United States, their health outcomes, and health care utilization so that changes can be made to health care and health policy in ways that will improve and extend the lives of those living with sickle cell.

2. Longitudinal, population based surveillance has been successfully demonstrated in two states through the SCDC system. The framework can be expanded to include more states, more data sources, and capabilities beyond surveillance.

3. CDC maintains national data collections systems for other health conditions that incorporate many of these characteristics and have succeeded in improving clinical management, outcomes, and access to care. With appropriate resources, time, and support, the same can be done for SCD.
Thank you!

- California SCDC Team
- Georgia SCDC Team
- CDC Foundation
- Doris Duke Charitable Foundation
- Global Blood Therapeutics
  - Pfizer, Inc.
  - Sanofi

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The findings and conclusions in this report are those of the authors and do not necessarily represent the official position of the Centers for Disease Control and Prevention.