ASH’s Multifaceted Initiative on Sickle Cell Disease (SCD)

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What is the American Society of Hematology (ASH)?

With more than 17,000 members from nearly 100 countries, ASH is the world’s largest professional society serving both clinicians and scientists who are working to conquer blood diseases.

ASH members include clinicians who specialize in treating children and adults with SCD and researchers who investigate the causes and potential treatments of SCD manifestations.
Sickle Cell Across the Lifespan: Infants and Children

• Progress
  – Universal newborn screening to identify infants
  – Evidence-based early interventions that save lives and prevent complications

• Challenges
  – Limited data on whether children are benefitting fully
  – Hydroxyurea likely underutilized

• Opportunities
  – Expand long term newborn screening follow up
  – Insure access of high quality evidenced based medical care
  – New disease-modifying interventions
Sickle Cell Across the Lifespan: Adults

• Progress
  – > 95% of children born today will survive to adulthood

• Challenges
  – Increased healthcare utilization in young adults, age 18-30 y
  – Not clear if early advances are extending the lifespan
    • May be getting shorter?
  – Hydroxyurea, the only FDA-approved medical therapy for SCD, is underutilized
  – Paucity of knowledgeable providers for adults with SCD

• Opportunities
  – NHLBI Evidence-based Management
  – ASH SCD Research Priorities
Additional Issues in Sickle Cell Disease

- Only a minority of SCD patients receive care in specialized centers
  - Numbers of estimated patients with SCD in comprehensive care programs in the US is likely a fraction of the total SCD population
- Limited coordination of community-based services
  - Mental health services
- Increased rates of patient dissatisfaction with care received
Timeline of ASH Work on Sickle Cell

- **2007**: Workshop on SCD Research
- **2011**: SCD Briefing / Release of SCD & SCT Priorities
- **2014**: Workshop on SCT & Athletic Participation
- **2015**: SCD Summit: A Call to Action
- **2016**: SCD Coalition, Release of State of SCD Report, & Call to Action
ASH’s Multifaceted Initiatives on SCD

- ASH SCD Task Force
- ASH SCD Research Priorities
- ASH SCD Management Pocket Guides
- ASH Policy Strategy on SCD
- Sickle Cell Disease Coalition
- ASH SCD Summit: Call to Action
- ASH Sickle Cell Trait (SCT) Policy Statement
- ASH-CDC-SCDAA SCT educational/awareness materials
ASH Sickle Cell Research Priorities

• Identify Predictors of Disease Severity
• Optimize the Use of Existing Therapies
• Develop Novel Therapies
• Strengthen Curative Therapies
• Enhance Pain Research
• Improve Access to Evidence-Based Care Through Innovative Healthcare Delivery Models
• Determine the Effects of Quality of Care on Quality of Life
• Investment in Sickle Cell Trait Research
Goal: Amplify the voice of the SCD stakeholder community, promote awareness and improve outcomes for individuals with SCD

www.scdcoalition.org

• 45 SCDC Members
  – Patient groups – 4
  – Public health, research, and provider organizations – 21
  – Federal agencies – 3
  – Industry – 10
  – Foundations – 3

• Newsletter – SCDC Update

• SCDC Workgroups
  – Access to Care
  – Global
  – Research
Launch of *Call to Action on SCD*

- September 6, 2016 launch event
  - Press Conference and Networking Reception
  - ~3 million impressions of SCDC
    #ConquerSCD hashtag!
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
**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 coordinated 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 healthcare providers with comprehensive knowledge and expertise to care for people with SCD. The unpredictable and often insidious 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.
Current ASH Projects

• Access to Care in the United States
  – Plan for Education Strategy Development

• Research
  – Explore Development of a SCD Clinical Trials Network

• Global Issues
  – Develop Newborn Screening and Early Intervention Consortium in Africa
Access to Care Work Group (U.S.)

• Developing plan to improve health care providers knowledge about SCD management
  – Target audiences: hematologist/oncologists who do not regularly care for SCD patients, primary care physicians, hospitalists, advanced practitioners, and emergency physicians

• Creation of patient-facing ‘pocket-guides’
• Partnerships with other organizations
• Exploring innovative payment models for SCD care
Educational Activities Proposed to Start in 2017

- Hematologists
  - Articulate goals for CME in SCD for hematologists*
  - Dedicate additional focus on SCD within existing CME channels*
  - Explore creation of Away Elective Program in SCD for fellows
  - Develop recommended changes to accreditation requirements for training programs
  - Beta test online training modules for fellows*
  - Explore the development of an ASH external review system to assess the SCD content of hematology-oncology training programs

* already underway
Educational Activities Proposed to Start in 2017

• Patients
  – Recruit patient advocacy organizations into an educational partnership with ASH
  – Produce patient-facing educational resource for hydroxyurea

• Hospitalists
  – Identify hospitalists with existing interest in SCD

• Primary Care Clinicians
  – Identify strategic partners to better articulate ASH’s vision for the roles primary care clinicians can play in SCD
Research: Explore development of a Clinical Trial Network for SCD

• The Work Group on Research is working on a proposal to develop a SCD Clinical Trials Network.

• Goal: Identify the highest priority research questions and the infrastructure needed to support efforts at addressing these questions
Global: Exploration of a SCD Newborn Screening & Early Interventions Consortium

• Goal: establish 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 effect of introduction of these intervention on change the course of treatment and potential improved outcomes for these populations

The Global Burden 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:
  – To 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
Summary

• Unprecedented opportunities to advance research and provide high quality medical care for patients with SCD
• Educational tools for health care providers in all settings and for patients that focus on evidence-based SCD care
• Clinical practice guidelines describing the management of acute and chronic complications of SCD
• Global impact through a consortium in African countries to institute newborn screening and early intervention
• Work with Congress and federal agencies to enhance and expand federal SCD programs
• Without ongoing and, in many areas, even greater investment progress will be lost
Questions?

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