Remarks to the

Global Blood Therapeutics Access to Care Summit
OFFICE OF THE ASSISTANT SECRETARY FOR HEALTH

• **Provide trusted data and information**
  to serve HHS, the federal government, states and localities, and the general public

• **Convene partners**
  federal agencies, state and local, professional societies, NGOs, academia, civil society, commercial partners, patient advocates

• **Develop novel initiatives**
  gain situational awareness, identify gaps, build teams, set a common agenda, support infrastructure, transition to Operational Divisions

• **Organize and Lead National Initiatives**
OPPORTUNITIES TO IMPROVE THE LIVES OF PEOPLE LIVING WITH SICKLE CELL DISEASE
AWARENESS
NATIONAL SICKLE CELL AWARENESS MONTH
SEPTEMBER 2019
OMH’s #SickleCellStories
"We will always stand strong with those battling this condition and remain committed to improving the quality of life of those living with SCD."
SICKLE CELL DISEASE
NEW FEDERAL WORKING GROUP
The National Academies will provide recommendations related to the barriers that exist in healthcare for SCD patients, limitations or possible opportunities to develop SCD specific registries and/or surveillance systems, new research innovations, and the importance of patient advocacy and community engagement groups.
QUESTIONS TO FORM BASIS FOR A NATIONAL ACTION PLAN

• What specific initiatives will lead to substantially greater compliance with SCD care and treatment guidelines?

• How do we facilitate the seamless transition of care for SCD patients when they exit a comprehensive, specialty pediatrics center and enter college and/or the adult internal medicine environment?

• Is there a need for a national patient registry to consolidate data on genetics, epigenetics, microbiome, treatments, and social determinants of health with patients’ outcomes, function, etc.?

• What steps are needed to facilitate the development and implementation of clinical trials and broad access/enrollment of SCD patients in these clinical trials, specifically to include the potential establishment of a clinical trials network?
QUESTIONS TO FORM BASIS FOR A NATIONAL ACTION PLAN

• What changes could be made in FDA regulations or outcome measures to catalyze the approval of new therapies for SCD?

• How do we assure that SCD patients have their pain treated effectively and without stigma or discrimination?

• There is robust pipeline of new therapies, including potential cures, for SCD:
  ▪ Do we have agreement on study endpoints necessary for approval of SCD therapies?
  ▪ Are we sufficiently accelerating testing and approval by available regulatory pathways?
  ▪ Do we have the payment mechanisms to assure that patients can receive the therapy?
  ▪ How do we enhance availability of effective, expensive treatments via state Medicaid programs, or other programs in addition to/instead of such programs?
ACTION
OASH OBJECTIVE:

Increase the life expectancy of Americans with sickle cell disease (SCD) by at least 10 years within 10 years (by 2028)
AUTHORIZING LEGISLATION (WITHOUT APPROPRIATIONS)

The Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention, and Treatment Act of 2018 (S. 2465)

- Signed into law by President Trump on December 18, 2018
- Reauthorizes a SCD prevention and treatment program and provides grants for research, surveillance, prevention, and treatment of heritable blood disorders
- Eligible entities include states, state or local health departments, and institutions of higher education

Senator Tim Scott
Representative Michael Burgess M.D.
EXPANSION OF THE CDC SCD DATA COLLECTION PROGRAM

- Collects health information about people with SCD to study long term trends in diagnosis, treatment, and healthcare access
- Georgia and California only two states participating since 2015
- OASH and CDC added $1 million in funding in 2019, and will add an additional $1 million in 2020
- Expansion of data collection program to 9 states representing up to 32% of US SCD population
IMPROVING CARE FOR PEOPLE LIVING WITH SCD
UPCOMING INITIATIVES LED BY OASH OFFICE OF MINORITY HEALTH

• Stakeholder engagement workshop in advance of a possible Sickle Cell Disease National Quality Improvement Effort
  - Will solicit input and feedback from the SCD community on the proposed American Society of Hematology (ASH) and American Board of Pediatrics (ABP) Quality Improvement Effort

• Sickle Cell Disease Training and Mentoring Program for Primary Care Providers (STAMP)
  - A new pilot program focused on targeted outreach to primary care providers, including providers at federally quality health centers (FQHCs) and student health clinics at Historically Black Colleges and Universities (HBCUs)
FOCUS ON PAIN IN SCD

“\textit{I've been taking this medication since '95 and I take a really high amount. But I only take enough to be able to function. ...But the main thing is, we need opioid medications to be an option in the tool box.}”
NIH WILL DEVOTE APPROXIMATELY $110M TO RESEARCH RELATED TO SICKLE CELL DISEASE IN FY2020

- Support implementation research that promotes innovative approaches to ensure all patients receive evidence-based care,
- Catalyze development of innovative therapies that enhance health outcomes and show promise for the eventual cure of SCD, and
- Promote capacity building and research that enables SCD diagnosis, surveillance, and care delivery globally, especially in Africa.
SICKLE CELL DISEASE: THE “CURE” WORD

60 Minutes Featuring
NIH’s Efforts to Cure
Sickle Cell Disease
Sunday, March 10 at 7:00 p.m. EDT

National Institutes of Health
PAYMENT REFORM: INTEGRATED CARE FOR KIDS (InCK) MODEL

**Opportunity to define a new SCD comprehensive care model**

The InCK Model is a child-centered local service delivery and state payment model aimed at reducing expenditures and improving the quality of care for children covered by Medicaid and CHIP, especially those with or at-risk for developing significant health needs.

**GOALS**

1. Improving performance on priority measures of child health
2. Reducing avoidable inpatient stays and out-of-home placements
3. Creation of sustainable Alternative Payment Models (APMs)

Up to 8 cooperative agreement awards anticipated December 2019
ADDITIONAL ACTIONS

• HHS Office of Minority Health
  will devote $3M dollars (FY2020) for the development and implementation of an
  innovative care model for adolescents and young adults with SCD to improve
  longevity and quality of life

• Centers for Medicare and Medicaid Services (CMS)
  will include SCD as a key topic in its network in order to educate physicians,
  hospitals and patients about the best course of treatment, incentivize increased
  access to care and to provide additional flexibilities on how care is delivered
POTENTIAL NEW DEMONSTRATION MODELS OF INTEGRATED CARE FOR SCD
GLOBAL REACH
SCD IN AFRICA

300,000 - 400,000 children are born each year with SCD
Mortality: 50% - 80% by age 5 years

• Hosted Roundtable on SCD in Africa
  - Attended by seven African Delegations
  - Professional Societies
  - NGOs
  - Commercial Corporations
  - WHO Chief Scientist

• Follow Up Meeting WHO-AFRO in Brazzaville, Congo, August 2019

• Meeting at the UN General Assembly in New York, September 2019
WHAT IS POSSIBLE FOR AFRICAN CHILDREN?

December 1, 2018

Hydroxyurea for Children with Sickle Cell Anemia in Sub-Saharan Africa

- 635 Children 1-10 years of age
- 4 clinical sites in Sub-Saharan Africa: Angola, DRC, Kenya, Uganda
- Provided standard doses of Hydroxyurea
- 94.2% retained in treatment through 3 years

RESULTS

- VOC Decreased 54%
- Infections Decreased 37%
- Malaria Decreased 50%
- Transfusions Decreased 67%
- Deaths Decreased 69%

And this is just the beginning of what is possible!
