Providing Hope to the Underserved

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GBT
Hope + Science + Community

LIVING OUR MISSION

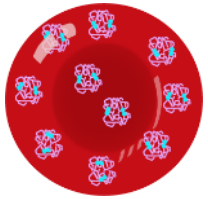
To truly make SCD a well-managed disease, we must continue to advance innovations in care and address long-standing gaps in health equity.

Muyiwa
Age 36
Durham, NC
Actual Patient

SCD, sickle cell disease.

SCD: A DEVASTATING DISEASE IMPACTING RED BLOOD CELL HEALTH

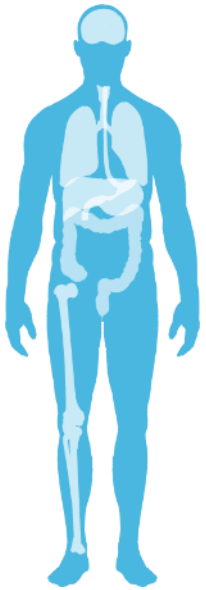
Normal RBC with oxygenated Hb



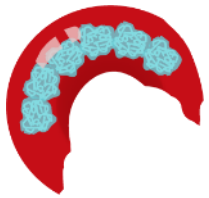
Normal levels of Hb

RBCs reaching tissue

Normal oxygen content and delivery



Sickle RBC with polymerization of deoxygenated Hb

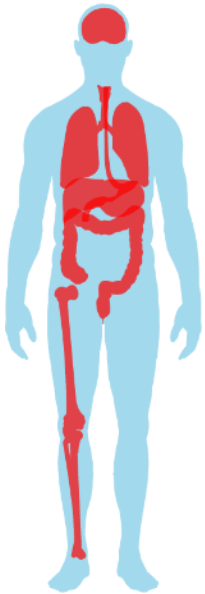


Hemolysis

Anemia

Vaso-occlusion

Decreased oxygen content and impaired delivery



Hb = hemoglobin; HbS = sickle hemoglobin; RBC = red blood cell; SCD = sickle cell disease.

Rees DC et al. *Lancet.* 2010;376:2018-2031.

MAJOR BURDEN ON U.S. PATIENTS AND SOCIETY

Up to **$286,000** annually in **cost of medical care**[1]

End-organ damage drives **major healthcare utilization**, average of **30-54 days/year**[2]

~$700,000 in lost lifetime income per patient[3]

Major caregiver productivity impact; often **devastating financial burden**

1. Song, X, et al. Economic Burden of End Organ Damage Among Patients with Sickle Cell Disease in the US. 2019 American Society of Hematology Annual Meeting. Poster #3388. 2. GBT Internal Data.
3. Lubeck, D. et al. Estimated Life Expectancy and Income of Patients With Sickle Cell Disease Compared With Those Without Sickle Cell Disease. JAMA Netw. Open. 2019 Nov 1;2(11):e1915374.

RACE AND DISEASE DISCRIMINATION IMPACT CARE

Misconceptions can lead to undertreatment[1]

- HCPs have misconceptions that lead to undertreatment, including:
  - Fear that the patient is a drug abuser
  - Disbelief in the patient's pain severity
  - Reluctance to prescribe opioids

Interactions with the healthcare system can be challenging

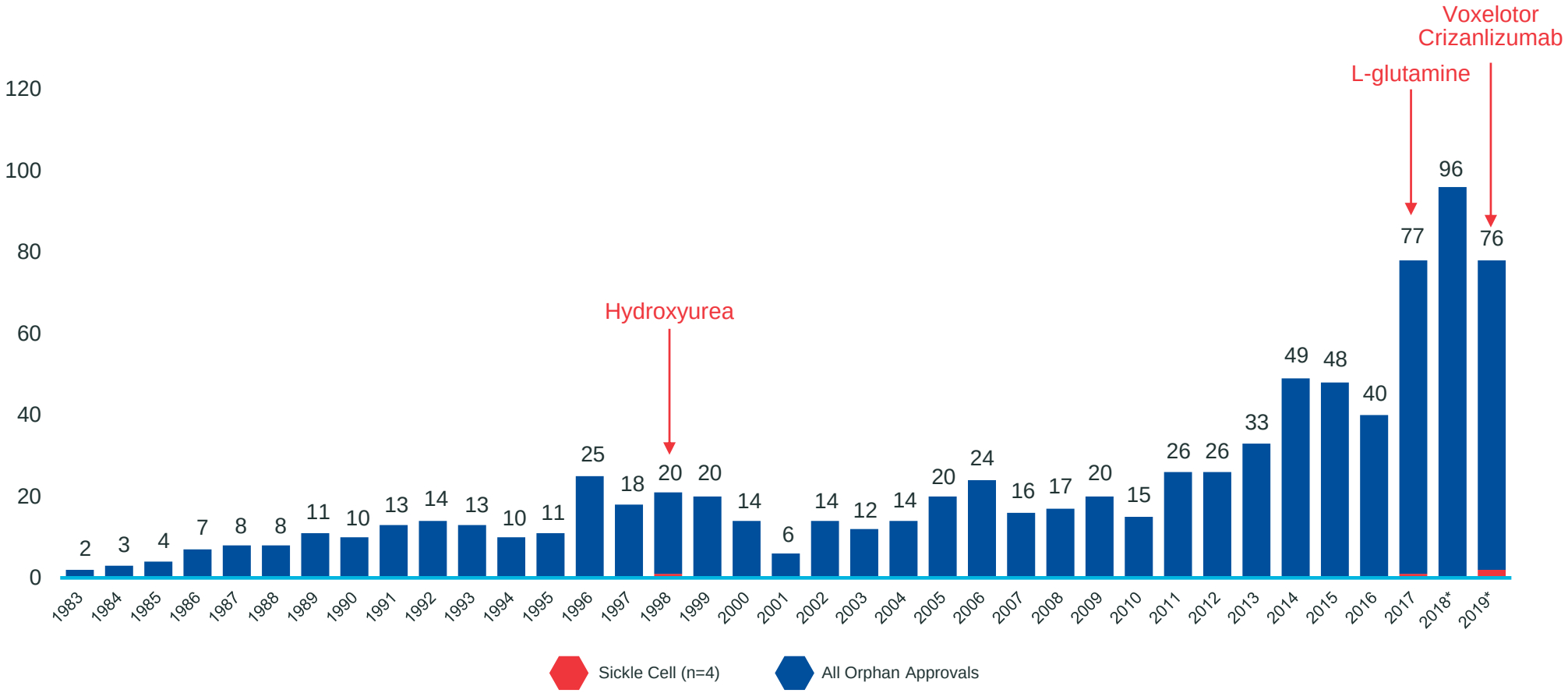
- ED healthcare providers harbor negative attitudes about patients with SCD[2]
- SCD patients experienced longer ED wait times[3]
- HCPs provide poor communication, demonstrating less respect and spending less time[4]

ED = emergency department; HCP = healthcare provider;

1. Adams-Graves P and Bronte-Jordan L. Expert Rev Hematol. 2016;9:541-552; 2. Glassberg J et al. Am J Hematol. 2013;88:532-533; 3. Haywood C Jr et al. Am J Emerg Med. 2013;31:651-656; 4. Haywood C Jr et al. *Patient Educ Couns*. 2014;96:159-164.

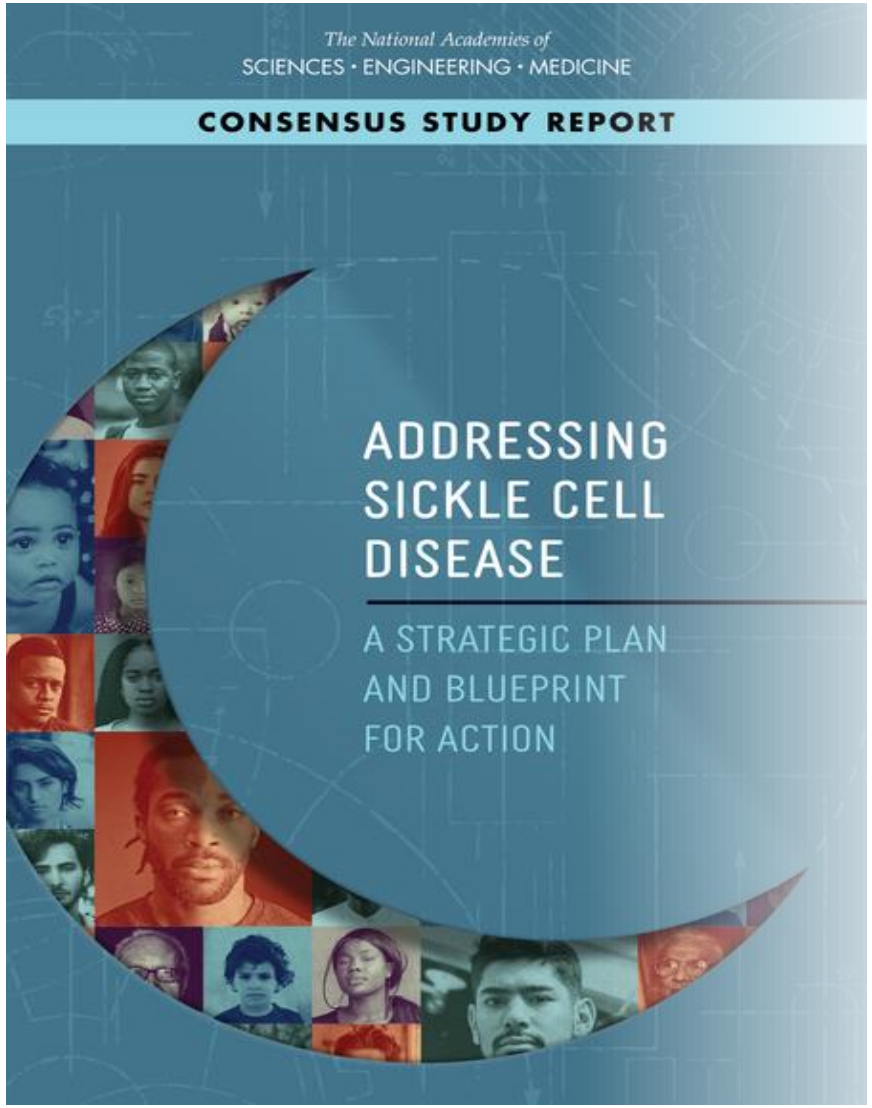
INNOVATION IN SCD THERAPIES HAS LAGGED OTHER RARE DISEASES

Number of FDA Orphan Drug Approvals



Adapted from Miller, KL. Investigating the landscape of US orphan product approvals. Orphanet J Rare Dis. 2018; 13: 183.
*Food and Drug Administration (FDA). Search orphan drug designations and approvals. http://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm. Accessed October 2020.

SCD MOMENTUM AT THE NATIONAL AND STATE LEVELS



Increased investment in innovative medicines

Growing focus on need to fill health equity gaps

Policy initiatives to improve SCD patient care

SCD now receives more attention, but more is needed to ensure lasting and impactful change.

SCD, sickle cell disease.

10 YEARS OF INNOVATION & COMMITMENT TO THE FUTURE

First-in-class therapy

Global expansion underway

Robust pipeline of potential SCD therapies

Commitment to ending health inequality

SCD, sickle cell disease.

FIRST AND ONLY FDA APPROVED MEDICINE THAT DIRECTLY INTERFERES WITH RED BLOOD CELL SICKLING

INDICATION

What is OXBRYTA®?

OXBRYTA is a prescription medicine used for the treatment of sickle cell disease in adults and children 12 years of age and older.

It is not known if OXBRYTA is safe and effective in children below 12 years of age.

This indication is approved under accelerated approval based on increase in hemoglobin (Hb). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

IMPORTANT SAFETY INFORMATION

Do not take OXBRYTA if you have had an allergic reaction to voxelotor or any of the ingredients in OXBRYTA. See the end of the patient leaflet for a list of the ingredients in OXBRYTA.

If you are receiving exchange transfusions, talk to your healthcare provider about possible difficulties with the interpretation of certain blood tests when taking OXBRYTA.

Before taking OXBRYTA, tell your healthcare provider about all of your medical conditions, including if you:

+ have liver problems
+ are pregnant or plan to become pregnant. It is not known if OXBRYTA can harm your unborn baby
+ are breastfeeding or plan to breastfeed. It is not known if OXBRYTA can pass into your breastmilk and if it can harm your baby. Do not breastfeed during treatment with OXBRYTA and for at least 2 weeks after the last dose

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Some medicines may affect how OXBRYTA works. OXBRYTA may also affect how other medicines work.

For more information about Oxbryta, please see the Full Prescribing Information and Patient Information, which is available from your presenter or by visiting www.Oxbryta.com

Source: Oxbryta PI 2021; Oxbryta Patient PI 2021.

OXBRYTA® INTERFERES IN THE SICKLING PROCESS REDUCING SICKLING AND IMPROVING RBC HEALTH

Oxbryta® (voxelotor) 500mg tablets

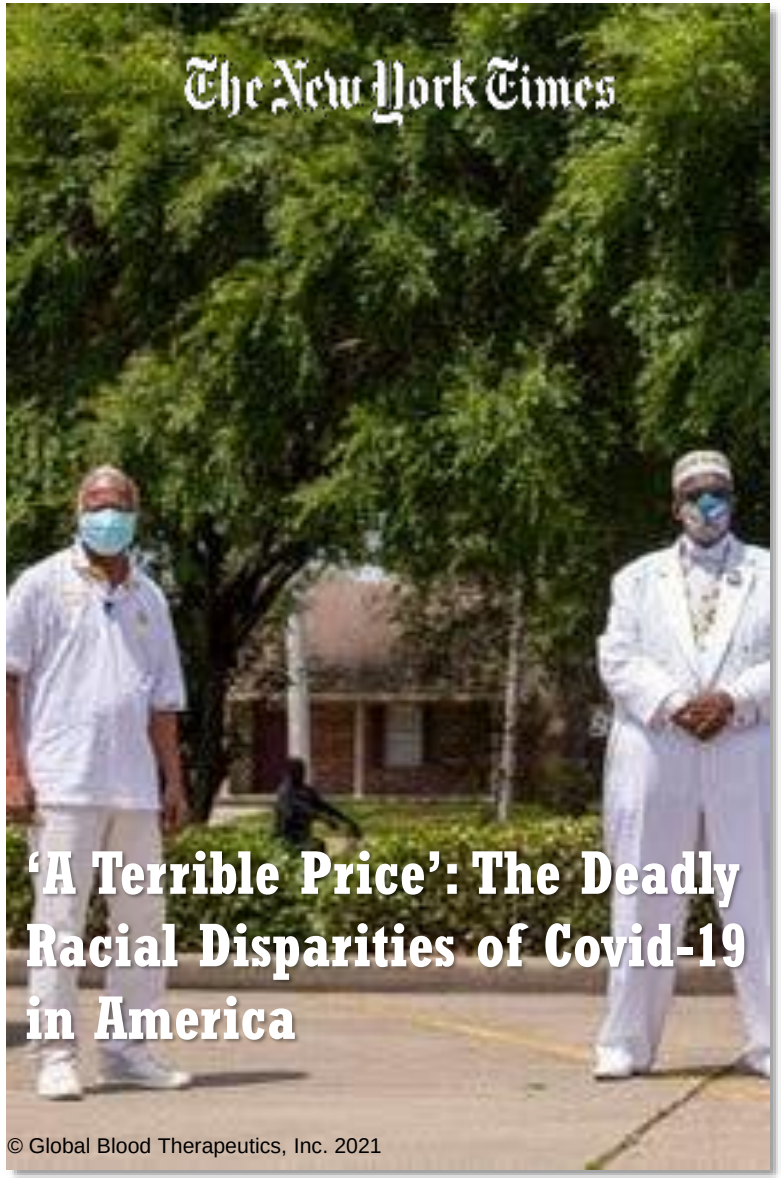
With less RBC destruction, we anticipate an increase in hemoglobin, which is expected to deliver oxygen to your body's tissues

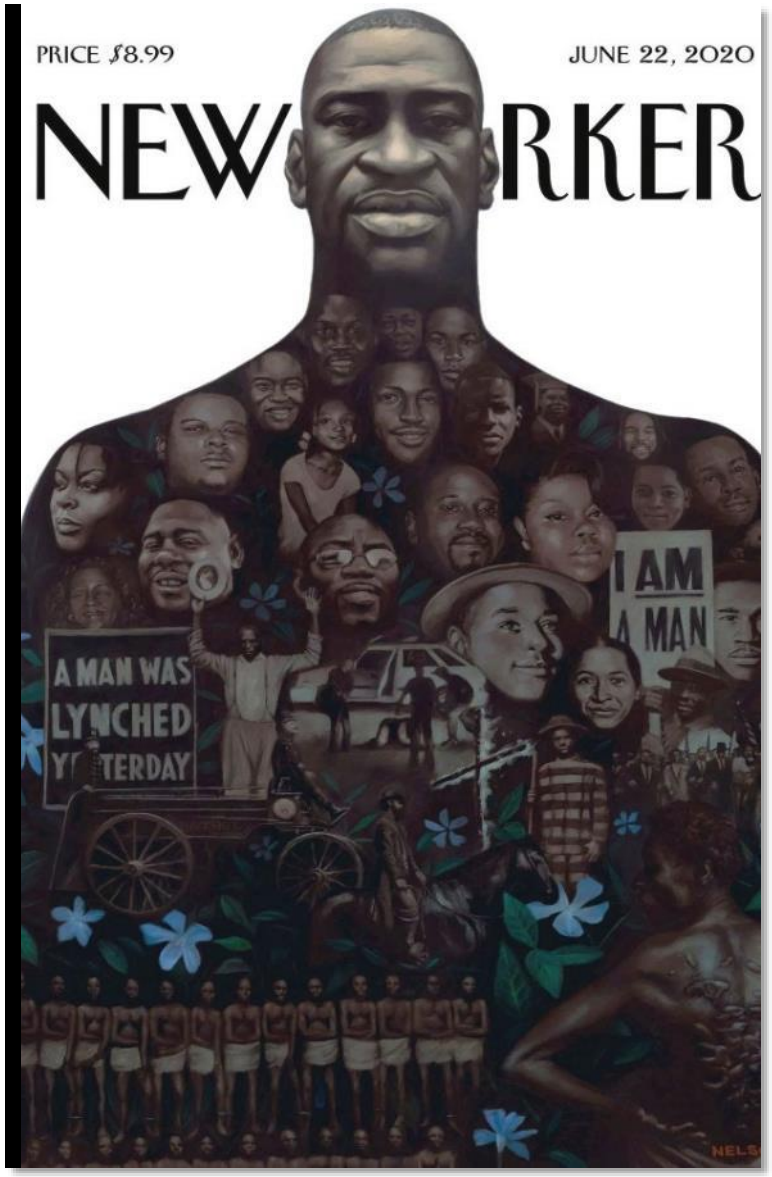
For more information about Oxbryta, please see the Full Prescribing Information and Patient Information, which is available from your presenter or by visiting www.Oxbryta.com

Oxbryta® (voxelotor) 500mg tablets

Source: Howard et al. (2019). Blood, 133. 1865-1875.; Oxbryta PI 2021; Oxbryta Patient PI 2021.

UNPRECEDENTED CHALLENGES





SCD COMMUNITY IS THE CENTER OF EVERYTHING WE DO

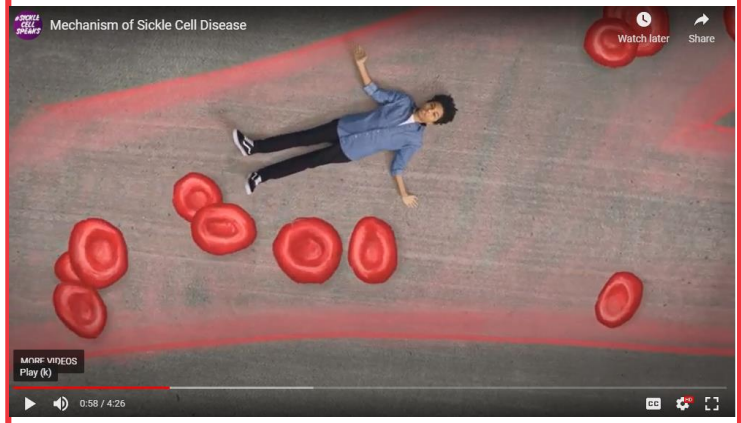
Engaging through clinical studies, access, and advocacy

ACCEL

Access to Excellent Care for Sickle Cell Patients

Addressing long-standing inequities

Closing the knowledge gap



ACCELERATING HIGHER QUALITY CARE IN SCD

Access to Excellent Care for Sickle Cell Patients (ACCEL) Grant Program 2021 Grantees

ACCEL
Access to Excellent Care for Sickle Cell Patients

- Improving the Transition from Pediatric to Adult Care for SCD Patients in Rural Areas of California
- Increasing COVID-19 Vaccine Awareness in the SCD Community across the U.S.
- Empowering SCD Patients to Educate Healthcare Providers about the Impact of Race and Healthcare Disparities
- Connecting SCD Patients and Families with Health and Social Service Resources
- Building a Collaborative Community Network to Mitigate Social Determinants of Health
- Offering Disease Education and Improving Communication with Patients and Families
- Improving Transitions from Pediatric to Adult Care
- Helping SCD Patients Better Manage Pain with Palliative Care
- Increasing Nurses' Theoretical and Clinical Expertise in the Care of SCD Patients

SCD, sickle cell disease

IMPROVING THE HEALTH OF UNDERSERVED PATIENT COMMUNITIES WORLDWIDE

GBT | THE GBT FOUNDATION

Supporting programs that aim to create meaningful and sustainable change

- **SCD education/awareness, empowerment, and access to care**
- **Innovative solutions to improve healthcare equity**

SCD, sickle cell disease.

GBT COUNCIL FOR SCD HEALTHCARE EQUITY

Uniting SCD community leaders to create initiatives to improve SCD care, anchored in the broad vision of NASEM recommendations

Biree Andemariam, MD
Director, NE Sickle Cell Institute
University of Connecticut

Beverly Francis-Gipson
President & CEO, Sickle Cell Disease Association of America

Terry Jackson, PhD
Jaxson Enterprises

Diane Nugent, MD
Chief, Hematology
Children's Hospital of Orange County

Mattie Robinson
Micromattie Consulting

Wanda Whitten-Shurney, MD
CEO, Michigan Chapter
SCDAA

Latasha Lee, PhD, MPH
Vice President of Clinical and Social Research & Development NMQF

Betty Pace, MD
Tedesco Distinguished Chair, Ped Hematology
Augusta University

Emma Andelson
Program Manager
Sick Cells

Matt Powers
Managing Director, MMS
Health Management Associates

Donnell Ivy, MD
Vice-CMO
SCDAA

Mary Brown
President & CEO
Sickle Cell Disease Foundation

Charlotte Curtis
Founder, Sickle Cycle

SCD, sickle cell disease; NASEM, National Academies of Sciences, Engineering, and Medicine

A DEEP COMMITMENT TO THE MISSION

Thank You

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