



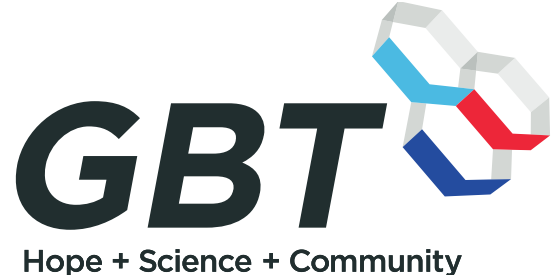
hope

Science

Community

State of the State of Sickle Cell Disease

Ted W. Love, M.D.
President and CEO
Global Blood Therapeutics, Inc.
March 30, 2022



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OUR AGENDA TOGETHER

- **Sickle Cell Disease Overview**
- **GBT's Commitment to the Community**
- **Needs & Momentum at Federal Level**
- **SCD in Georgia**
- **A Call to Action**



LIVING OUR MISSION

To truly make sickle cell disease 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





AN URGENT UNMET NEED

Millions of patients worldwide¹

Historically limited treatment options;
most focused on pain

30-year reduced life expectancy²



Varying Clinical Manifestations

Patient A, age 12

Cognitive issues

- Major impact on performance in school

Patient B, age 16

10 surgeries, one stroke,
but no VOCs

- Undergoes regular blood transfusions

Patient C, age 17

Sustained fatigue and VOCs

- Pain impacts ability to go to school and do activities

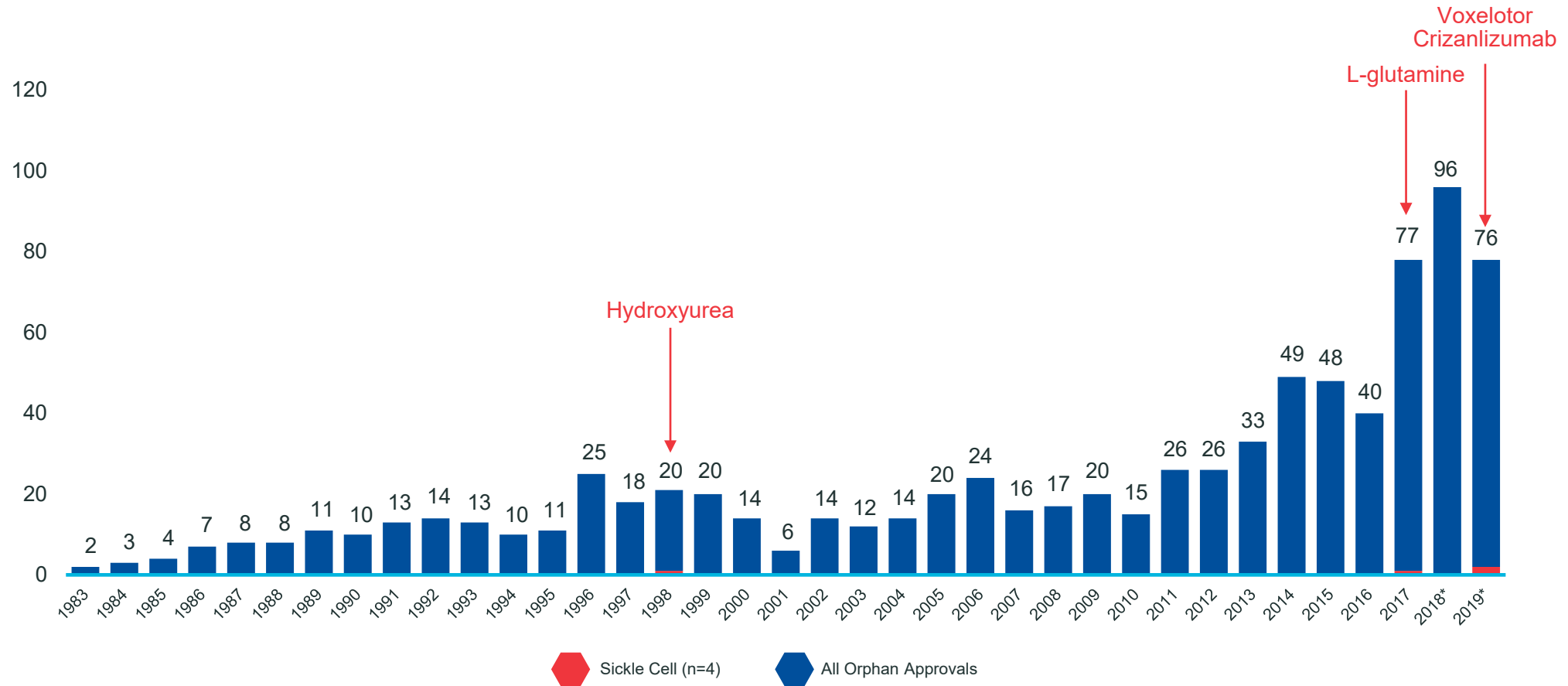
VOC, vaso-occlusive crisis.

1. Population data: [Centers for Disease Control and Prevention website](#). Sickle Cell Disease (SCD). Accessed February 23, 2022; [European Medicines Agency](#). Accessed February 23, 2022. Data on file. 2. Piel FB, Steinberg MH, Rees DC. Sickle cell disease. *New Engl J Med*. 2017;376;16:1561-1574.

INNOVATION IN SCD 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/loopd/index.cfm>. Accessed October 2020.



ADDRESS

FOUNDED TO ADDRESS SICKLE CELL DISEASE





10 YEARS OF INNOVATION & COMMITMENT TO THE FUTURE



**Developing
first-in-class
therapies**

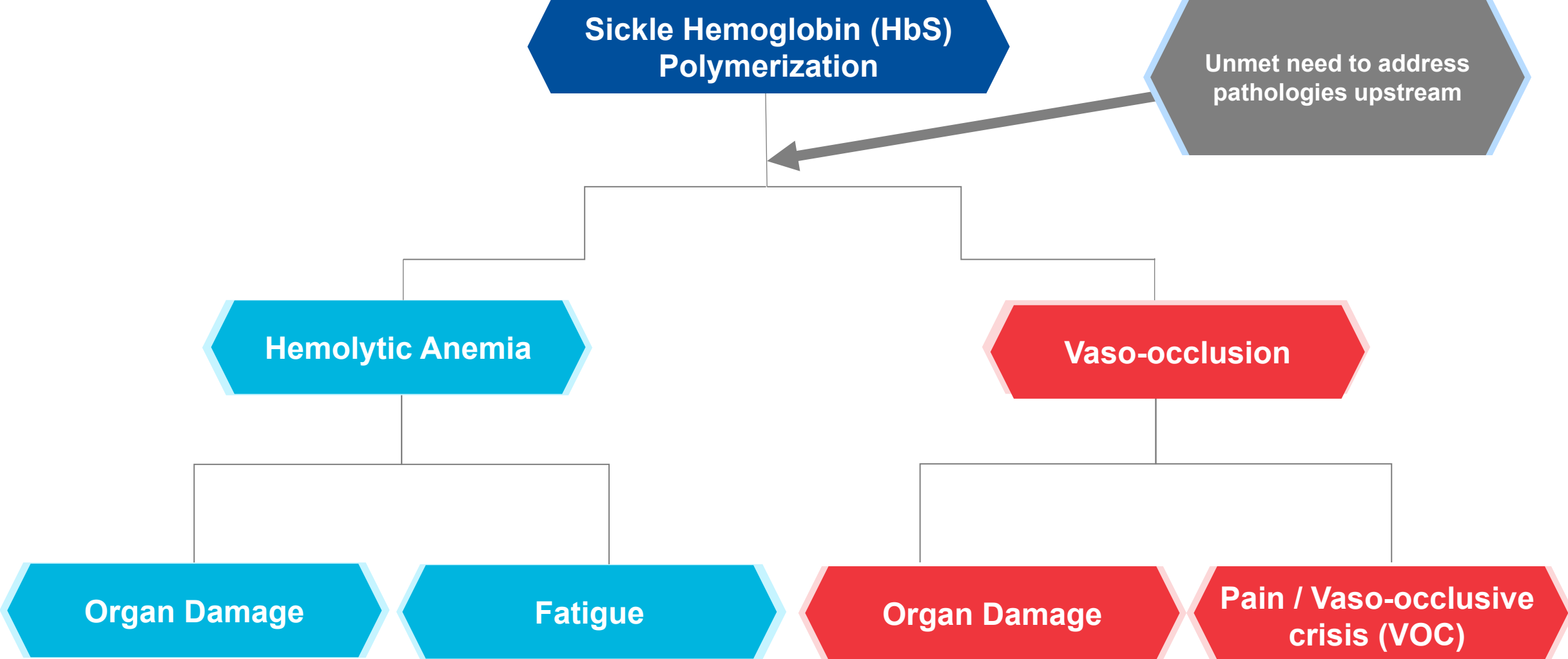
**Global expansion
underway**

**Robust pipeline of
potential
SCD therapies**

**Commitment to
ending health
inequality**



ADDRESSING THE UNDERLYING PATHOLOGIES



HbS, sickle hemoglobin.
Adapted from Eaton, W. and Bunn, F. Targeting HbS Polymerization. *Blood*. 2017.
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RACE AND DISEASE DISCRIMINATION IMPACT CARE

Provider misconceptions can lead to undertreatment¹

- + Fear that the patient is a drug abuser
- + Disbelief in the patient's pain severity
- + Reluctance to prescribe opioids

Interactions with healthcare system can be challenging

- + Negative attitudes about patients with SCD²
- + Longer wait times in the ER³
- + Poor communication, spending less time, less respect⁴

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.

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SCD COMMUNITY IS THE CENTER OF EVERYTHING WE DO



Funding access to care and
advocacy initiatives



Community engagement



Closing the knowledge gap



ACCELERATING HIGHER QUALITY CARE IN SCD



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



Improving the Transition from Pediatric to Adult Care for SCD Patients in Rural Areas of California



Offering Disease Education and Improving Communication with Patients and Families



Increasing COVID-19 Vaccine Awareness in the SCD Community across the U.S.



Improving Transitions from Pediatric to Adult Care



Empowering SCD Patients to Educate Healthcare Providers about the Impact of Race and Healthcare Disparities



Helping SCD Patients Better Manage Pain with Palliative Care



Connecting SCD Patients and Families with Health and Social Service Resources



Increasing Nurses' Theoretical and Clinical Expertise in the Care of SCD Patients



Building a Collaborative Community Network to Mitigate Social Determinants of Health



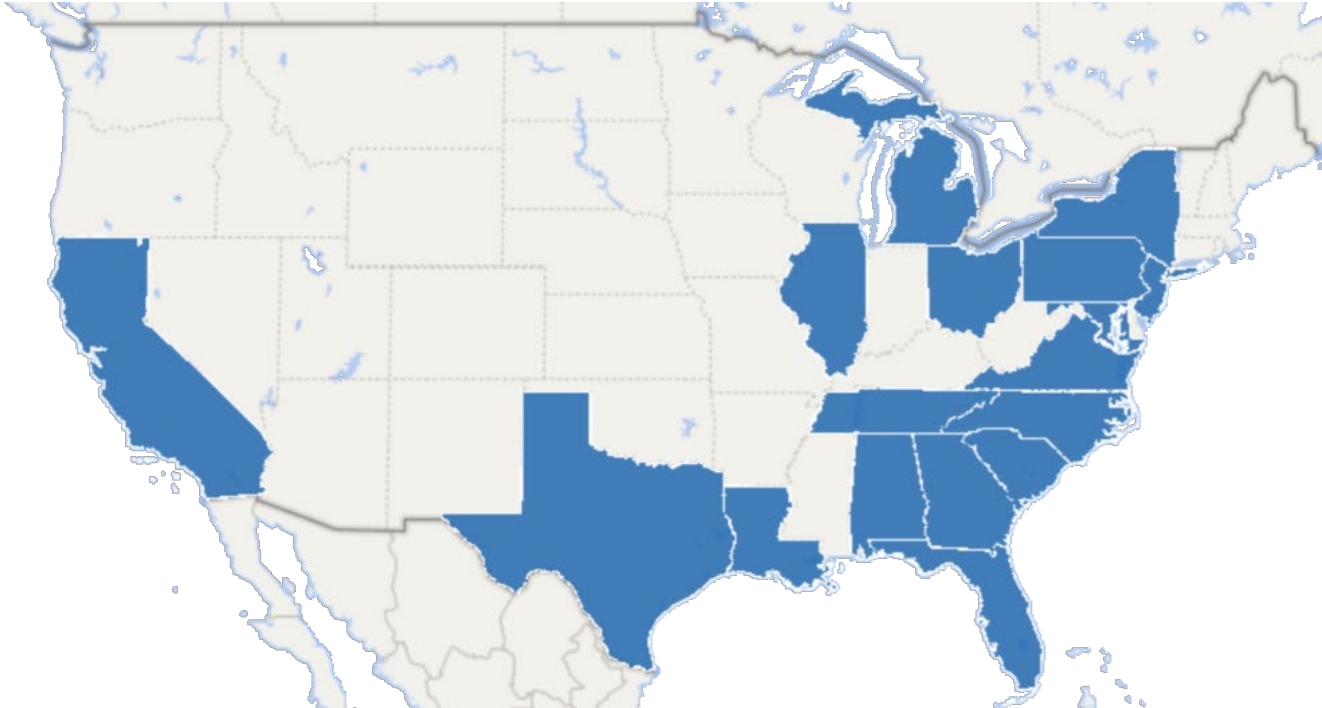
SCD IN THE US & GEORGIA





MEETING SCD PATIENTS WHERE THEY ARE

~84% of SCD Patients (age 12+) Live in 17 States



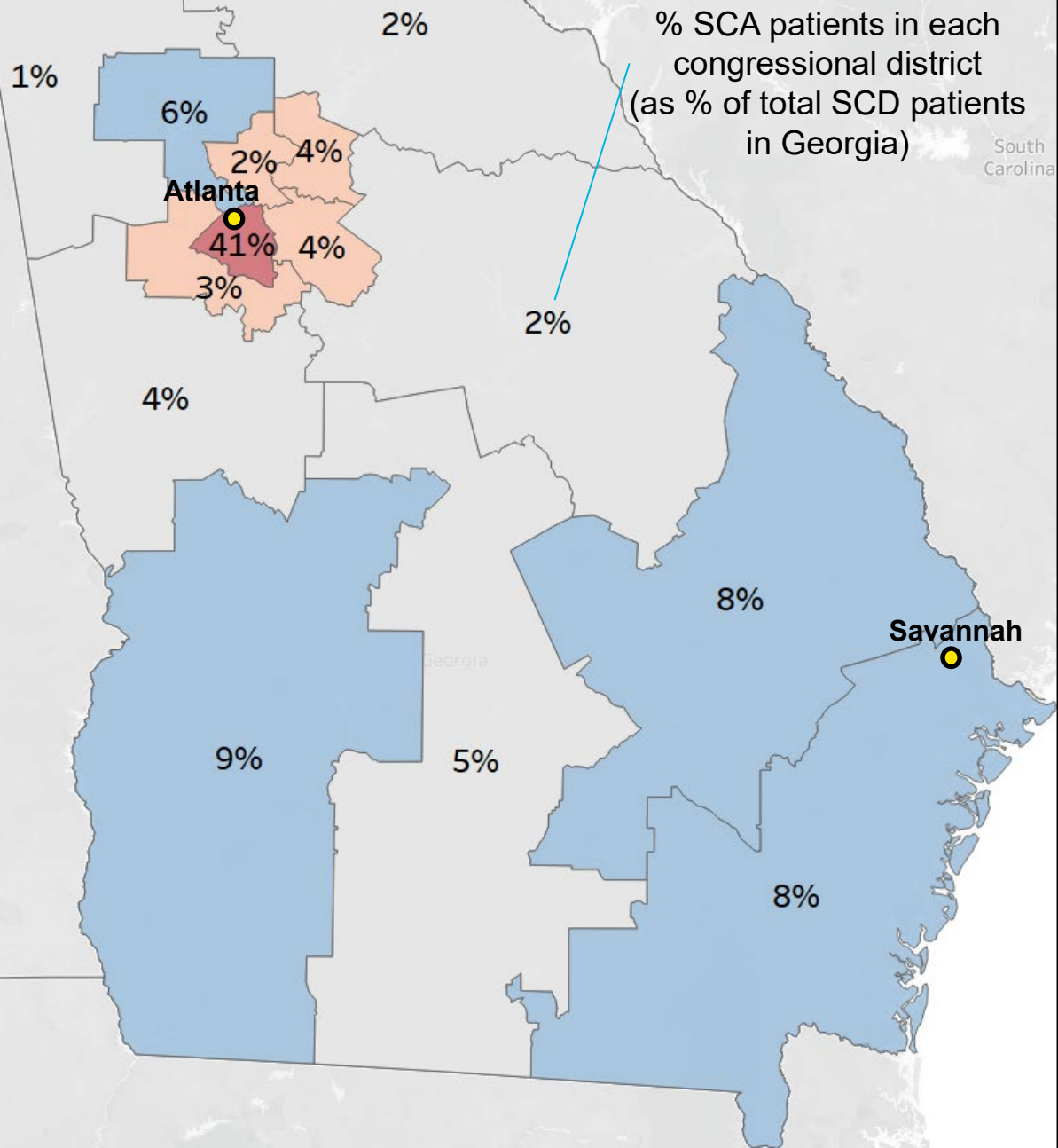
Top 20 Metro Areas with SCD Patients

1. New York-Northern New Jersey-Long Island, NY-NJ-PA MSA
2. Miami-Fort Lauderdale-Pompano Beach, FL MSA
3. Atlanta-Sandy Springs-Marietta, GA MSA
4. Washington-Arlington-Alexandria, DC-VA-MD-WV MSA
5. Philadelphia-Camden-Wilmington, PA-NJ-DE-MD MSA
6. Chicago-Naperville-Joliet, IL-IN-WI MSA
7. Houston-Sugar Land-Baytown, TX MSA
8. Dallas-Fort Worth-Arlington, TX MSA
9. Baltimore-Towson, MD MSA
10. Los Angeles-Long Beach-Santa Ana, CA MSA
11. Detroit-Warren-Livonia, MI MSA
12. Memphis, TN-AR-MS MSA
13. Orlando-Kissimmee, FL MSA
14. Tampa-St. Petersburg-Clearwater, FL
15. Virginia Beach-Norfolk-Newport News, VA-NC MSA
16. New Orleans-Metairie-Kenner, LA MSA
17. Phoenix-Mesa-Scottsdale, AZ MSA
18. Las Vegas-Paradise, NV MSA
19. Cleveland-Elyria-Mentor, OH MSA
20. Jacksonville, FL MSA



GEORGIA

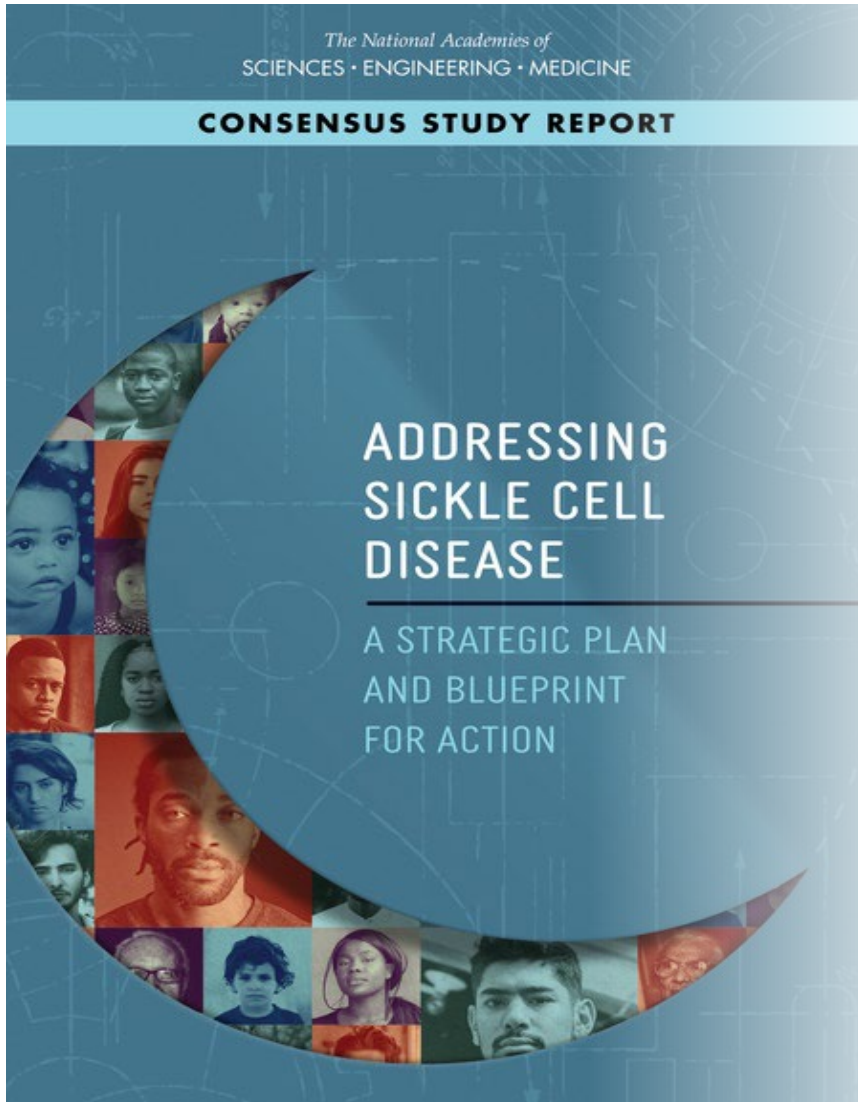
% SCA patients in each congressional district (as % of total SCD patients in Georgia)



**Total SCD Patients in Georgia = 3,812
(6% of total SCD Patients in Nation)**

Category (CDs in GA)	
	High Density – Urban (1 in GA)
	High Density – Rural (0 in GA)
	Medium Density – Urban (0 in GA)
	Medium Density – Rural (4 in GA)
	Low Density – Urban (4 in GA)
	Low Density – Rural (5 in GA)

NATIONAL AND STATE SICKLE CELL DISEASE MOMENTUM



**Increased
investment in
innovative
medicines**

**Growing focus
on health equity**

**Policy initiatives
to improve SCD
patient care**

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



COUNCIL FOR SCD HEALTHCARE EQUITY



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



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



Terry Jackson, PhD
Jaxsun Enterprises



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



Latasha Lee, PhD, MPH
Independent



Emma Andelson
Program Manager, Sick
Cells



Matt Powers
Managing Director, MMS
Health Management
Associates



Mattie Robinson
Micromattie Consulting



Donnell Ivy, MD
Vice-CMO SCDAA



Mary Brown
President & CEO
Sickle Cell Disease
Foundation



Charlotte Curtis
Founder, Sickle
Cycle



**Wanda Whitten-
Shurney, MD**
CEO, Michigan Chapter
SCDAA

Purpose

- + Create a unified advocacy voice at highest level to bring forth broad programs that impactfully improve the healthcare of people with sickle cell disease

Approach

- + Actionable objectives anchored to vision of NASEM report

Goals

- + Align with key stakeholders on high priority initiatives
- + Advance priorities to fruition using collective strength

Vision

- + U.S.-focused effort initially to create traction/precedent
- + Global expansion using momentum from U.S. effort

THE OUTCOME



The Sickle Cell Disease Treatment Centers Appropriations Act

To amend the Public Health Service Act to authorize grants to fund sickle cell disease and other heritable hemoglobinopathy treatment centers and reauthorize a sickle cell disease prevention and treatment demonstration program – and appropriate funds to support these programs accordingly.

Key Sponsors:

- + Congresswoman Barbara Lee (D-CA)
- + Congressman Michael Burgess (R-TX)



KEY ASPECTS OF PROPOSED LEGISLATION

Funds a nationwide network of 128 sickle cell disease treatment centers based on a hub-and-spoke framework to treat patients with SCD or other hemoglobinopathies

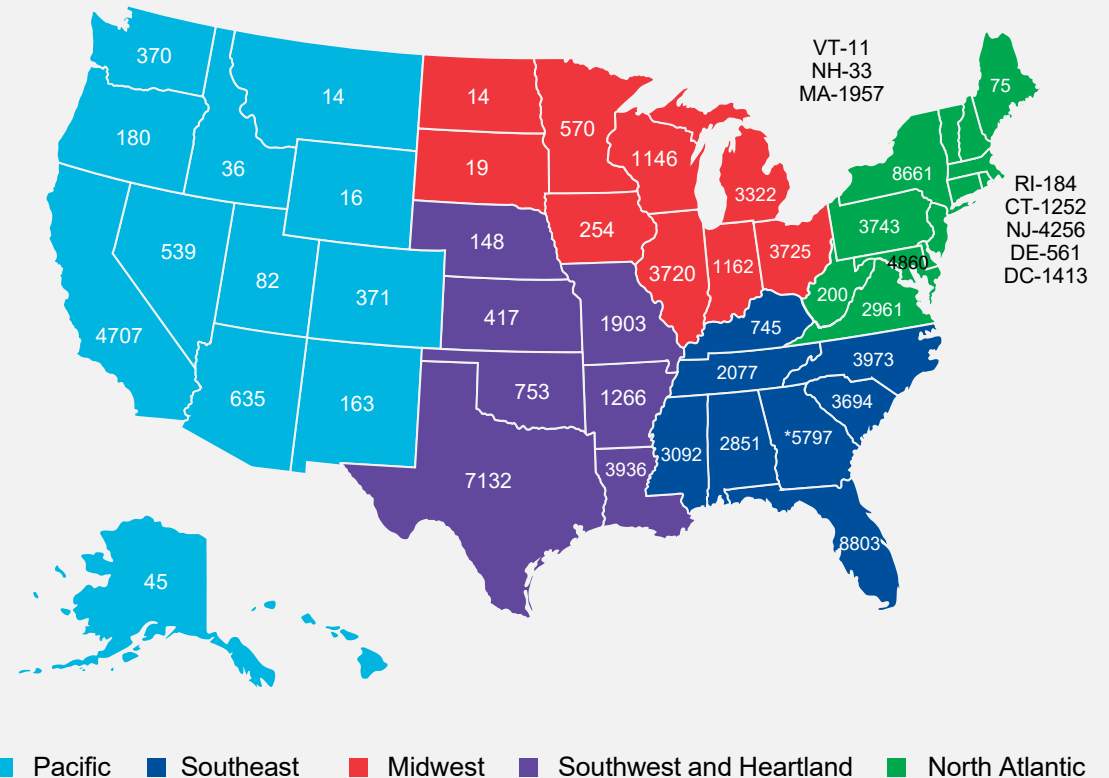
Funds 110 community-based organizations so that they can support patients, families, and communities to provide training and education to providers, patients, and families

Reauthorizes the HRSA Sickle Cell Disease Treatment Demonstration Project

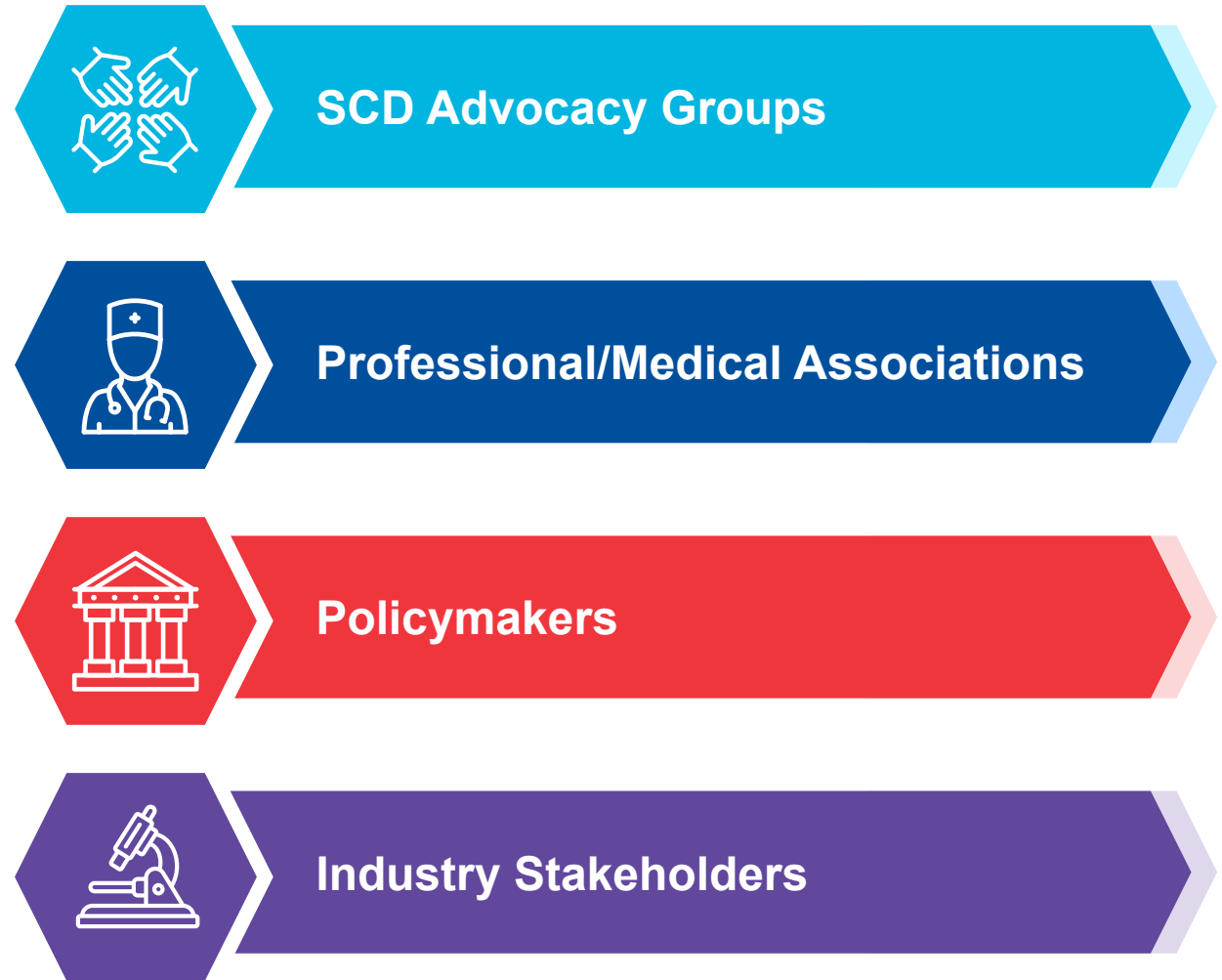
Establishes a National/Regional Coordinating Center(s) that will coordinate infrastructure and submit report to Congress

Expansion of the Centers for Disease Control and Prevention (CDC) data collection and surveillance

Sickle Cell Regional Structure



STAKEHOLDER ACTIVATION AND ENGAGEMENT

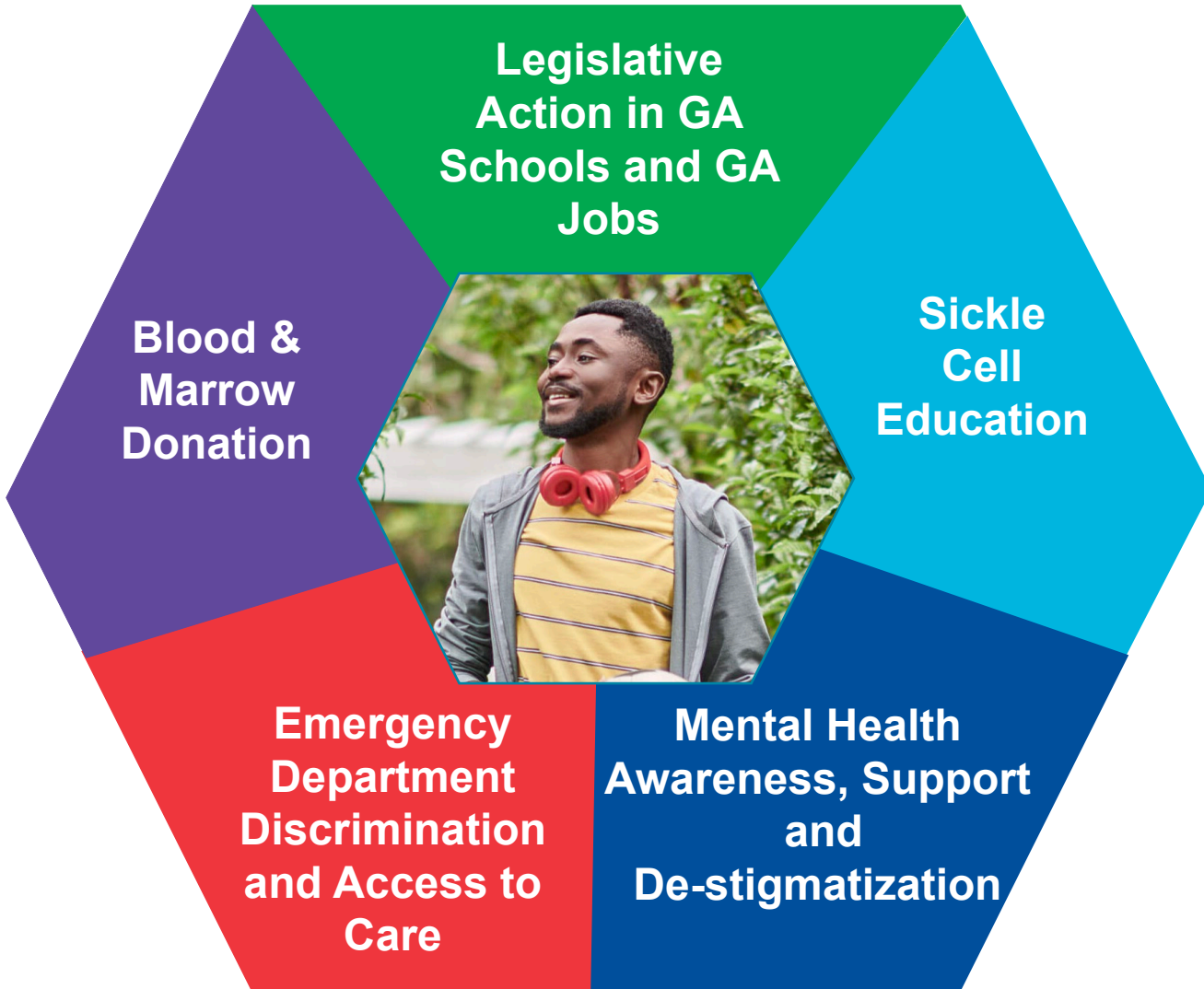




SCCC AGENDA FOR GEORGIA: POWER IN THE COLLECTIVE



sickle cell
communityconsortium



A CALL TO ACTION





Thank You

