Testimony of

Alexis Thompson, MD, MPH
Chief, Division of Hematology
Elias Schwartz MD Endowed Chair in Hematology
Children's Hospital of Philadelphia

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on

"Examining Proposals That Provide Access To Care For Patients and Support Research For Rare Diseases"

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Chairman Guthrie, Ranking Member Eshoo, and distinguished members of the Committee:

Thank you for the opportunity to participate in this hearing to discuss H.R. 3884, *the Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention, and Treatment Act of 2023* and the importance of this reauthorization to federal efforts to improve the lives of the approximately 100,000 Americans living with sickle cell disease (SCD). This legislation is critical to support access to care for patients with SCD and other hemoglobin diseases. With early diagnosis achieved through universal newborn screening for most individuals, effective evidence-based interventions can save lives and reduce suffering. Yet for far too long, many individuals with SCD have lacked consistent access to high-quality, comprehensive care and treatment. Data collection and research efforts for SCD have also been inadequate, compared to other rare diseases which further compromises measurable progress that could be made to give these individuals the quality and length of life that we all deserve.

My name is Dr. Alexis Thompson. I am Chief of the Division of Hematology and the Elias Schwartz MD Endowed Chair in Hematology at the Children's Hospital of Philadelphia, and Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine. In these roles, I treat children and young adults with sickle cell disease, educate future clinicians about how to deliver comprehensive care to children with SCD and other blood diseases and I lead a research team engaged in innovations for sickle cell and other blood disorders such as gene therapy as potential cures. Over the course of my career, I have also had the privilege of serving as an advisor to governmental agencies such as the National Institutes of Health and the Advisory Committee on Heritable Disorders of Newborns and Children. I also have served as president of the American Society of Hematology (ASH), which is the world's largest professional society, serving both clinicians and scientists who are working to conquer blood diseases.

SCD, the most common inherited blood disorder in the United States, affects the red blood cells which are critical for delivery of oxygen to organs throughout our bodies. The genetic mutation that causes SCD results in the patients' red blood cells becoming rigid and sickle-shaped causing them to block blood and oxygen flow to tissues, causing excruciating pain as well as damage to vital organs such as the brain, the heart, the lungs, and the kidneys. New approaches in managing the disease have improved early detection of sickle cell complications and intervention with effective treatments, which have extended the lifespan of many individuals living with SCD. Unfortunately, without access to knowledgeable providers and appropriate care, these patients

still face significant and sometimes devastating complications such as severe pain, stroke, acute chest syndrome, and kidney disease which can be debilitating and, in some cases, leads to premature death.

Since the initial authorization of the Sickle Cell Disease Treatment Demonstration Program (SCDTDP), the Health Resources and Services Administration (HRSA) has provided resources for education and training of clinicians to improve access to quality care for patients living with SCD and sickle cell trait. This program addresses an important recommendation of Strategy D in the National Academies of Science, Engineering, and Medicine report, *Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action*: To increase the number of qualified health professionals providing SCD care.

The federal government's support has been critical to aiding children and adults living with SCD. Despite this investment, there are still not enough knowledgeable providers to treat this complicated patient population. H.R. 3884 will reauthorize the HRSA demonstration program through fiscal year (FY) 2028 and will allow the agency to build upon its efforts and investments to date. The SCDTDP is a grant program that (1) increases the number of clinicians knowledgeable about SCD care; (2) improves the quality of care provided to individuals with SCD; (3) improves care coordination with other providers; and (4) develops best practices for coordination of services during the pediatric to adult care transition. This regional grant program advances these goals by building the provider workforce with mentoring, education, and training.

To increase provider confidence in treating individuals with SCD, the program grantees use the Project ECHO® (Extension for Community Healthcare Outcomes) model of telementoring and training. The program's objective is to connect providers with SCD experts so that they can increase their knowledge about best practices in managing individuals with this complex disease. By establishing a regional SCD infrastructure, the program partners with states to develop and support comprehensive SCD care teams to deliver care across the lifespan; implements telehealth technologies for health care delivery, education, and health information and services; increases access to evidence-based care and the latest treatment options; and increases collaboration and care coordination within each region. Deploying ECHOs in various regions has helped reach hundreds of clinicians across the country and has been particularly helpful to support the care delivered at smaller institutions in each region.

The TDP covers the entire country and utilizes a regional hub and spoke model of care. The demonstration sites work with local community-based organizations (CBOs), which were funded in FY 2021 through the Sickle Cell Disease Newborn Screening Follow-up Program. The regional aspect of the grant program has been particularly successful in this current funding cycle in promoting collaboration amongst grantees to extend the geographic reach in order to serve greater numbers of patients, including those who live some distance from more experienced academic centers.

Progress in SCD can be accelerated with continued support as outlined in this legislation but also through engagement with non-governmental organizations. The American Society of Hematology (ASH) has supported multifaceted SCD efforts including, convening multidisciplinary partners and collaborators, promoting access to high quality care, global issues, policy, research, and leveraging data to accomplish these goals. ASH, which has education and training as a core component of its mission, has also partnered with HRSA grantees in the ASH SCD Adult Care Centers Workshop that supports the establishment of more capable and comprehensive clinics throughout the country. ASH also provides opportunities for doctors in training to spend elective time at the most experienced sickle cell centers in the country to further strengthen the capacity of our workforce to meet the needs of these complex patients.

As this Committee considers H.R. 3884, I also urge you to consider how to improve the program. While the program has improved clinical interventions, it is vital to improve the metrics used to evaluate the care being delivered to patients. Measurement is critical to continue to quantify increases in screening for stroke, use of pediatric to adult care transition plans, expanding use of appropriate therapeutics such as hydroxyurea, etc. Additionally, the demonstration sites should continue to expand their focus on identifying unaffiliated patients. To accomplish these goals, more resources are required for the demonstration sites to collect the required data and deliver care.

The SCDTDP has focused on getting more primary care physicians and mid-level providers such as nurse practitioners involved in SCD care, while also focusing on improving access to SCD specialists who remain best equipped to treat SCD patients at this point. I recommend that the Committee encourage HRSA to work with its grantees to pilot new approaches to comprehensive care delivery. For example, a program for goal setting and coaching adolescents, or a pilot to help people with addressing mental health issues would be particularly impactful for patients with SCD.

Again, I urge you to reauthorize this program before the start of FY 2024 when the current authorization expires to ensure its continuity. However, the SCDTDP is just one component of improving SCD care. Congress also has invested in the Centers for Disease Control and Preventions' (CDC) SCD Data Collection program, which awards grants to states, academic institutions, and non-profit organizations to gather information on the prevalence of SCD and health outcomes, complications, and treatment that people with SCD experience. Currently, 11 states participate in the program; however, these 11 states only account for an estimated 35 percent of the population of Americans living with SCD. Continued congressional support for this program is vital because it complements the HRSA program to ensure those living with SCD have access to care. Additionally, I encourage this Committee to support H.R. 1672, the Sickle Cell Disease Comprehensive Care Act, which would direct the Centers for Medicare & Medicaid Services (CMS) to create a demonstration program in up to 10 states to improve access to comprehensive, high-quality outpatient care for individuals with SCD enrolled in Medicaid. The CDC program and CMS demonstration, alongside the HRSA program, are key federal investments for improving the health of individuals with SCD.

The sickle cell community is deeply appreciative of this Committee and Congress' commitment to address SCD through these programs. Again, I urge this Committee to act now and reauthorize H.R. 3884. Thank you for the opportunity to testify before you today.