Testimony of

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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 b v port

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.

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