

The National Heart, Lung, and Blood Institute (NHLBI) launched the Cure Sickle Cell Initiative (CureSCi) in 2018 to accelerate the advancement of safe and promising gene therapies for sickle cell disease (SCD). As a collaborative, patient-focused research effort, CureSCi brings together the entire sickle cell community—including patients, advocates, caregivers, researchers, providers, industry, and others.

Importance of Patient Engagement

Patient engagement in research has been shown to positively impact clinical trial participation, which plays an integral role in advancing therapies for SCD. In fact, involving patients early in the process has helped to reduce mistrust and allows researchers to gain insight into important aspects of a disease from a patient perspective. Patient engagement and outreach are not only central to clinical trials recruitment and retention, but also in informing clinical trial design, patient visit schedule, and patient reported outcomes.¹

CureSCi Community and Patient Engagement

Well before the launch of CureSCi, it was recognized that no input is more essential than that of a person living with SCD. Identification of avenues to seize scientific opportunities, such as gene therapies, are impossible without patient participation in clinical research. Overcoming barriers to progress, including beliefs surrounding clinical trials participation, are key elements of the SCD community-wide conversations that can only happen with active involvement and engagement of those living with the disease.

Community and Patient Engagement Activities have included:

2017

LISTENING SESSIONS

- Led sessions with 4 SCD clinics and institutions.
- Explored barriers to treatment and participation in research.

FOCUS GROUPS

- Obtained feedback to help shape CureSCi vision, mission, brand, and direction.
- Conducted sessions in six cities (Baltimore Maryland; Atlanta, Georgia; Jackson, Mississippi; Chicago, Illinois; Los Angeles, California; and Portland, Oregon).

2018

COLLABORATIVE ROUNDTABLE

- Identified and discussed community engagement ideas and opportunities with patients and community-based organization leaders.

2018-2020

COMMUNITY OUTREACH VISITS

- Met with community organizations to introduce the Initiative and increase engagement.
- Included 9 visits with 39 provider interviews and 32 patient interviews.

2020 - PRESENT

COMMUNITY INPUT PANEL

- Launched panel to support two-way dialogue and engagement with the patient community.
- Membership included diverse representation of patients, advocates, and experts.

2020 - PRESENT

ONGOING COMMUNITY FEEDBACK

- Continued engagement with patients, caregivers, and advocates regarding potential and/or upcoming activities.

¹ <https://pubmed.ncbi.nlm.nih.gov/35093266/>

“The CIP ensured that the perspectives and needs of the SCD community were integrated into research and decision-making processes relating to curative therapies. The CIP fostered collaboration and trust between researchers, healthcare providers, and those affected by SCD.”

ADE ADEYOKUNNU
CIP Member

The SCD patient and caregiver community have helped to build the foundation of CureSCi, and their knowledge and expertise continues to guide its work. Those living with or impacted by SCD can be found at every level of the Initiative—including involvement in committees, subcommittees, working groups, and panels—and goes beyond having a seat at the table; their voices are integrated throughout the Initiative.

Initially, patients and caregivers helped to establish the CureSCi vision, mission, and brand. Today, their active engagement helps to support the realization of the goals of the Initiative.

Role of the CureSCi Community Input Panel

The CureSCi Community Input Panel (CIP) consisted of patients, advocates, and caregivers, and was formed in 2020. The CIP was established to engage in conversations with CureSCi leadership and Subcommittees to better understand challenges and help advance opportunities in gene therapies. The CIP bridged the gap between patients and researchers, and members provided guidance on the best ways to identify barriers and educate patients

and the community on gene therapies and clinical trials. CIP members provided input on clinical trial design, strategies, materials, progress, and overall direction of the Initiative. In addition, they have collaborated with the CureSCi Patient Readiness and Resilience Working Group to develop a white paper on psychosocial considerations for gene therapy readiness assessment (published in January 2024).

CureSCi Resources Generated through Engagement Activities

- Pre-gene Therapy Assessment of Patient Readiness and Resilience [White Paper](#)
- Identification of [Common Data Elements](#) for the collection of gene therapy data
- [Clinical and Economic Impact of Sickle Cell Disease](#) publication
- Webinars: [Mental Health and Psychosocial Considerations for Gene Therapies](#) and [Journeys in Mental Health](#)

Ongoing activities also included addressing community needs for educational materials on gene therapy conditioning, mental health, and other topics of importance. Specifically, this has included co-sponsored educational activities with national sickle cell organizations, including the Sickle Cell Disease Association of America, Sickle Cell Community Consortium, American Society of Hematology, as well as attendance and presentations at annual patient focused SCD conferences and conventions.

Future Patient Engagement Opportunities

CureSCi continues to partner with patients and community-based organizations to learn more about the needs of the sickle cell community and identify opportunities to work together to address those needs. For example, efforts are currently focused on addressing mental health and resiliency in the postgene therapy space, as well as discussing barriers and developing strategies for long-term follow up after SCD gene therapies, among others.