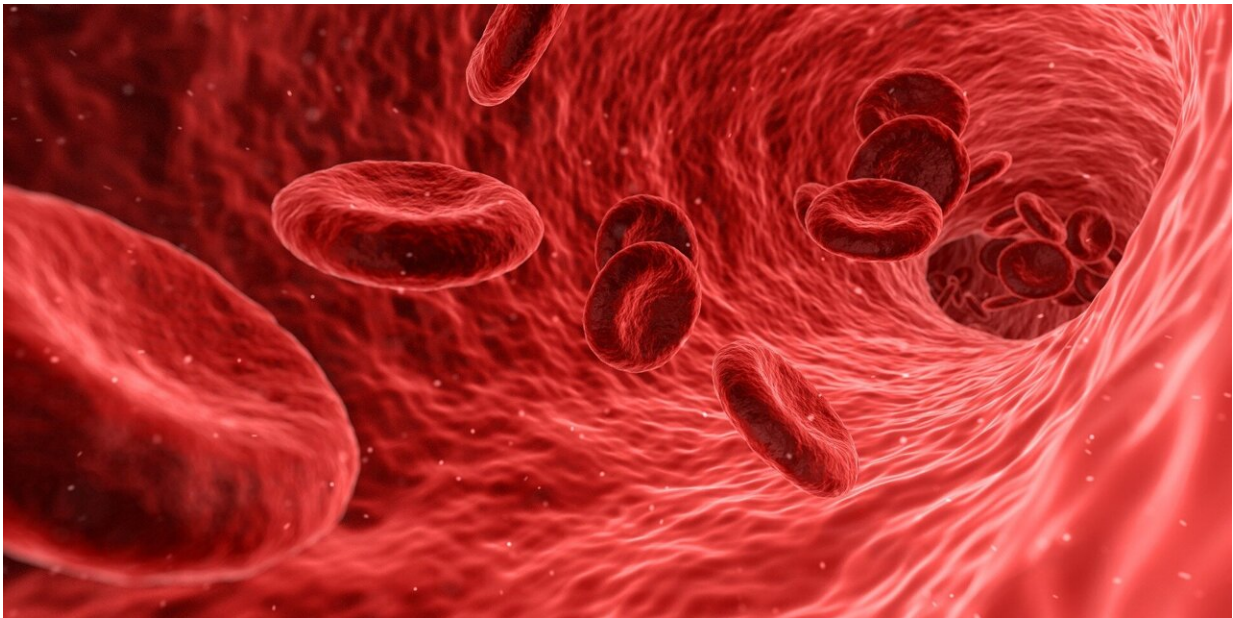


Project creates new educational materials for sickle cell disease community

September 28 2022



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New educational materials to help the sickle cell disease community learn about gene therapies for the disease are now available from researchers at the National Human Genome Research Institute (NHGRI), part of the National Institutes of Health.

The Democratizing Education for Sickle Cell Disease Gene Therapy Project, led by Vence Bonham Jr., J.D., NHGRI acting deputy director

and associate investigator in the Social and Behavioral Research Branch, developed the materials for individuals living with [sickle cell disease](#) and their support networks to learn about the gene therapy clinical trial process, its benefits and risks, mental health considerations and the science behind different treatment options.

"These materials offer the sickle cell disease community information about what participating in a gene therapy clinical trial entails," said Kiana Amini, a postbaccalaureate research fellow in Bonham's group who co-led the project. "These resources reflect our response to the need for more accessible educational materials to help patients and their families learn about the gene therapy process."

The project seeks both to help the sickle cell disease community understand these new therapies and to facilitate engagement between this community and those conducting relevant [clinical trials](#).

Bonham and his group led a series of four community engagement dialogues that brought together more than 50 scientists, physicians, patients and other experts to develop content targeted specifically for people with sickle cell disease and their support networks.

"We were intentional in including the individual perspectives of diverse stakeholders within the sickle cell disease community in this research project," Bonham said. "Specifically, we involved researchers who conduct gene therapy trials, health communication experts, [government agencies](#), industry representatives, physicians and colleagues at the National Institutes of Health, particularly the National Heart, Lung, and Blood Institute."

"Most importantly, the project included people living with sickle cell disease, caregivers, patient advocacy groups and individuals who have participated in gene therapy clinical trials. We brought together different

voices and views to create the new patient education materials," said Bonham.

Participants determined what critical information people would need to properly weigh the costs and benefits of participating in a gene therapy clinical trial for sickle cell disease.

The name of the disease comes from the "sickle" shape of the [red blood cells](#) that form in patients with the disorder. Red blood cells contain the protein hemoglobin, which carries oxygen in the blood. People with sickle cell disease have a [mutated gene](#) that codes for abnormal hemoglobin, which affects [blood flow](#) and causes pain, fatigue and other symptoms.

Patients can either take medication to reduce symptoms and prevent complications or receive blood transfusions, but these treatments offer short-term relief. Currently, the only treatment to cure sickle cell disease is a [bone marrow transplant](#), but this is not possible for everyone as many people do not have a matched donor.

Researchers are exploring new gene therapy treatments, which aim to modify the burden of the disease and to be curative at the DNA level by altering the genes that play a role in sickle cell disease.

"The number of clinical trials for sickle cell disease gene therapy has increased significantly in the last several years and will continue to increase as researchers refine different approaches in terms of efficacy and safety," Bonham said. "Making these resources available for the sickle cell disease community is important to support people who are considering whether to participate in a [gene therapy](#) clinical trial."

More information: [Sickle Cell Disease Gene Therapy Project](#)

Provided by NIH/National Human Genome Research Institute

Citation: Project creates new educational materials for sickle cell disease community (2022, September 28) retrieved 27 April 2024 from <https://medicalxpress.com/news/2022-09-materials-sickle-cell-disease.html>

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