GENE THERAPY

Nothing about us without us: Advocacy and engagement in genetic medicine

Olabimpe Olayiwola[1], Adam Castillejo[2], Michael Louella[3], Moses Supercharger[4], Evelyn Harlow[5], Lynda Dee[6], Khadidja Abdallah[7], Cissy Kityo-Mutuluuza[5], Jennifer E. Adair[8], Rimas Orentas[9], Karine Dubé[10,11]*

The development of new genetic medicines to treat sickle cell disease highlights the need for greater collaboration between researchers and people with lived experiences. Drawing on the adage "Nothing about us, without us," we call for increased investments in community advocacy and engagement.

INTRODUCTION

We are in the midst of a highly innovative era in medicine. Basic concepts of genetic engineering, repair, and editing are now being translated into new therapeutic modalities. Along with these exciting developments have come concerns from patients, regulators, and ethicists. New genetic therapies have a price tag in the millions, even with undefined long-term outcomes. These high costs restrict access to the therapies themselves, as well as to the infrastructure required to produce them. The infrastructure that supports the research and development of these genetic medicines benefits solely the nations and corporations in the nations where these therapies were developed. Thus, the developing world is locked out of access by cost and deprived of the development of infrastructure that can support innovation and entrepreneurship—although the intended populations for many of these gene therapy products exist primarily in low- and middle-income countries (LMICs). In many ways, these concerns echo those of patient groups and international health advocates heard in the earliest days of the HIV epidemic. There is a clear need for the intended patients of these gene therapies to be considered the major stakeholders and included as co-creators at every step in the development of new genetic medicines. Here, gene therapy primarily refers to ex vivo engineering of hematopoietic stem cells or peripheral blood-derived T cells, because these are the most common approaches with the highest curative impact. The need for patient-directed research, advocacy, and engagement in translational medicine is captured by the phrase, "Nothing about us, without us," an adage with roots in the AIDS empowerment movement of the early 1980s and embodied in the Denver principles (*1*).

The passage of time has not addressed these disparities. The first genetic disease to be described at the molecular level, sickle cell disease (SCD), has received little attention with regard to curative approaches until very recently. SCD is a genetic disorder caused by a single point mutation in the *HBB* gene and affects almost 8 million people globally. The single point mutation results in a valine–for–glutamic acid amino acid substitution in the β-globin chain of hemoglobin. SCD is characterized by the production of abnormal hemoglobin, which leads to the formation of sickle-shaped red blood cells. This abnormal shape causes the cells to become stiff and sticky and increases clumping, which leads to blockages in blood vessels, thus reducing oxygen delivery to tissues and causing excruciating pain for people with SCD. SCD is associated with a range of complications, including acute pain crises, anemia, organ damage, acute chest syndrome, avascular necrosis, and increased susceptibility to infections. Current treatment options for SCD mainly focus on managing symptoms and preventing complications. This includes pain management with analgesic medications; hydroxyurea therapy that increases fetal hemoglobin levels and reduces the frequency of pain crises; blood transfusions to alleviate severe anemia, improve oxygen delivery, and boost hemoglobin levels; and antibiotics to prevent infections. Additionally, supportive care measures, such as intravenous hydration, adequate nutrition, a healthy diet, and vaccinations, are essential for managing and preventing SCD complications. To move from symptom management to cure requires genetic engineering of autologous hematopoietic stem cells that give rise to red blood cells or bone marrow transplantation from a matched allogeneic donor.

The experience of people from racial and ethnically diverse groups around the world during the COVID-19 pandemic brought into sharper focus already existing disparities in health outcomes driven by racial, social, and economic determinants. Before the COVID-19 pandemic, the US National Institutes of Health (NIH) prioritized SCD research through the Cure Sickle Cell Initiative (https://curesickle.org/) to address these disparities. The hope is that focusing on non-communicable diseases like SCD, which can be addressed by genetic therapies, will call attention to the efforts of the research community to reverse decades of apathy and inactivity. The Cure Sickle Cell Initiative, started in 2018, was an important first step toward letting individuals know they matter, that their health matters, and that researchers not only care but want a better future for each patient with SCD. In an ideal world, genetic therapies would be a one-time treatment that could benefit millions (*2*). However, new advances cannot be so shortsighted as to ignore the inevitable challenges between clinical success in a small number of clinical trial participants and accessibility for millions globally.

INCREASING ACCESS TO SCD TREATMENT AND CURE CLINICAL TRIALS

Inclusivity in clinical trials is essential. We cannot assume that a therapy tested

[1]Roivant Social Ventures, New York City, NY, USA. [2]London Patient, London, UK. [3]University of Washington, Seattle, WA, USA. [4]Joint Adherent Brothers and Sisters Against AIDS (JABASA), Kampala, Uganda. [5]Joint Clinical Research Center (JCRC), Kampala, Uganda. [6]AIDS Action Baltimore, Baltimore, MD, USA. [7]University of Leuven, Leuven, Belgium. [8]Fred Hutchinson Cancer Center, Seattle, WA, USA. [9]Caring Cross, Gaithersburg, MD, USA. [10]University of California San Diego (UCSD), San Diego, CA, USA. [11]University of North Carolina Gillings School of Global Public Health, Chapel Hill, NC, USA.
*Corresponding author. Email: kdube@health.ucsd.edu

solely in the United States is applicable in resource-limited settings. Researchers may not be aware of the specific challenges raised in these settings until these therapies are tested in locations around the globe where SCD is most prominent (*3*). There is a lack of widespread access for early screening and diagnosis of SCD in affected countries. Inclusivity is further affected by a myriad of factors beyond geography and cost, such as poor general health, lack of experience with clinical procedures required for gene therapy to be initiated in its current form, apparent and hidden costs of participation, educational and language barriers, stigma, and implicit bias.

Furthermore, SCD in Africa is a completely different patient experience than that in the United States or Europe. For example, in the United States, having access to care and the availability of insurance are very different questions. Even insurance does not guarantee adequate treatment. For example, in a small study at Howard University's Sickle Cell Disease Clinic, 20% of patients were found to have no form of health insurance coverage (*4*). Data from the National Heart, Lung and Blood Institute (NHLBI) indicated that living with SCD resulted in a fourfold increase in out-of-pocket costs in the United States (*5*). In the same study, researchers found that only one-third of patients with SCD carried commercial health insurance (*5*). Although gene and cell therapy for SCD in the United States should be available, the reality is that most academic centers have administered these interventions to very few patients, and the current age restrictions require patients to be over 12 years old. Additionally, the Centers for Medicare and Medicaid have outlined a model for access to SCD gene and cell therapy, but it will be up to individual states and manufacturers to implement it (*6*).

In contrast, availability of SCD treatment in Africa is directly related to the gross domestic product (GDP) of the nation. Worldwide, no bone marrow transplants or hematopoietic stem cell transplantations (HSCTs) are carried out in nations with fewer than 300,000 people or with a gross national income of less than $1261 per person (*7*). In 2019, only one academic medical center in South Africa routinely carried out HSCT, reporting 48 transplants, with a cumulative national total of 357 transplants (*8*, *9*). Nigeria began HSCT in 2011 and has transplanted a total of 22 people with SCD. To date, across a handful of centers in India, hundreds of patients have received HSCT for SCD, and most of those patients have come from Africa. In all of these cases, patients pay out of pocket between $25,000 and $50,000 for HSCT depending on whether the transplant is from a matched sibling or a haploidentical donor, respectively. Other examples include Uganda, where HSCT is currently being implemented, with the first transplant center launched in late 2023, and Tanzania, which opened its first transplant center in Dodoma in November 2023, with eight transplants for SCD reported thus far. Concurrent with the need to have access to gene and cell therapy is the medical infrastructure required to support these therapies for SCD. We therefore encourage advocates and health systems to develop centers of excellence for HSCT that would ensure adequate supportive care for patients undergoing HSCT or gene therapies.

REDUCING THE COST OF SCD GENE AND CELL THERAPY

Although genetic therapies have the potential to alter people's lives for the better, the cost of current therapies and the high potential cost of those still in development will severely limit people's access and thus any hope of benefiting from these durable treatments and potential cures. The least expensive cell therapy product approved by the US Food and Drug Administration (FDA), Yescarta (a CAR T cell therapy for non-Hodgkin's lymphoma), costs approximately $373,000 per treatment. Newly approved gene therapies for factor IX hemophilia (hemophilia B) exceed $3 million, whereas the cost for a gene or cell therapy product that could treat or potentially cure SCD is $2.8 million. Considering that the global majority of patients with SCD do not have health care insurance, the vast majority of patients who would benefit will be left on the outside looking in on the wealthy few.

In addition to therapy in and of itself, diagnostic and supportive care must also be developed. There is an urgent need for newborn screening especially in LMICs, with an emphasis for optimal care and management for patients with SCD. HSCT has become an essential part of the treatment approach for leukemia and lymphoma in high-income countries. Yet, 65 years later, HSCT is still not routinely available in regions of the world where SCD incidence is highest. An effective human leukocyte antigen (HLA) typing and HSCT infrastructure would make curative low-risk HSCT a reality for some. HSCT is far from optimal, and post-transplant care represents a substantial struggle for those trying to meet the challenges of both the damage left by the disease and the sequelae of the transplant itself. In Uganda, about 20,000 babies are born with SCD each year; unfortunately, approximately 80% die within the first 5 years of life (*10*). Inclusion, focused on early curative approaches, will bring much-needed hope and a much-needed change in these statistics. SCD was first identified in 1910 and remains the most frequently inherited disease in the world, with increasing numbers of births each year. Yet, these facts have not inspired the investments needed to support comprehensive care. Diagnosis of SCD is not a cure, and hydroxyurea therapy is not a cure. Allogeneic bone marrow transplantation that can cure SCD is complicated and intensive and requires a matched, healthy donor. Despite being available for more than 40 years in the clinic, only approximately 2000 patients with SCD worldwide have received an allogeneic transplant (*11*). We believe that access to comprehensive care has suffered because there is not a cure for SCD available with scalability potential. Patients, governments, and populations need hope. Gene therapy, a more scalable cure for SCD, is on the horizon. Thus, leaders must invest in developing comprehensive care programs for early diagnosis of SCD and expanding the use of hydroxyurea therapy as research toward finding affordable and scalable gene therapies intensifies worldwide (Table 1).

In addition to early identification of SCD enabled by broader screening capacity, technological innovation will play a critical role in improving gene therapy accessibility and impact for SCD. The average cost for CAR T cell therapy was recently estimated to be $618,000, with the cost for 6% of patients exceeding $1,000,000 (*12*). From a financial aspect, drug companies are winning, patients are losing, and both public and private health care systems will not be able to sustain such costs for widespread conditions. Multiple vectors are being investigated for in vivo delivery of a therapeutic gene, but these are likely a decade or more away from being authorized in clinical trials (*13*). Direct in vivo injection of gene therapy vectors, such as lipid nanoparticles, could abrogate high prices to a degree by significantly lowering cost, but this gene therapy delivery approach has yet to be proven clinically effective and safe. In the United States,

Table 1. Key actions needed to ensure global access to gene therapies.

Challenges
• Critical challenges exist to making gene and cell therapy interventions accessible to the millions of people who need them worldwide.
Actions
• People with SCD should be considered major stakeholders and included as co-creators in the development of gene and cell therapies.
• Research teams need to invest in meaningful community involvement and engagement and ensure sustained funding for these efforts.
• CABs composed of patients, their caregivers, physicians, and other key stakeholders should advise on research efforts.
• Research teams should endeavor to create well-resourced CABs.
• The cost of gene and cell therapies should be reduced to enable access and the infrastructure required to produce them in LMICs.
• Value-based systems for the pricing of gene and cell therapies should be actively explored, and community input in setting pricing standards should be sought.
• Research sites in resource-limited settings should be included in clinical trials, and research capacity should be expanded in those settings.
• Investments are needed to develop comprehensive care programs for early diagnosis of SCD and to expand the use of hydroxyurea treatment as efforts toward affordable and scalable gene and cell therapies continue.
• Centers of excellence for bone marrow transplantation should be launched in resource-limited settings to ensure adequate supportive care for patients undergoing bone marrow transplant or gene/cell therapy.

the United Kingdom, and Europe, however, the cost of the drug product is divorced from the manufacturing cost, whereas in other countries such as India and most African countries, health care costs are paid out of pocket and thus manufacturing costs are directly related to drug prices (*14*). Furthermore, alternative pricing models will need to be tested to reduce drug costs related to manufacturing in the United States.

In late February 2023, Graphite Bio announced the discontinuation of development of their SCD gene therapy product (*15*). Graphite Bio's SCD gene therapy trial was on hold for review of one serious adverse event; however, protocol stopping criteria were not met. Shortly thereafter, Sangamo-Bioverativ issued official letters to clinical research sites participating in their SCD clinical trial that they would discontinue development of their gene therapy product, mainly because of market/business realities (*16*). Novartis similarly announced that it would discontinue its ex vivo gene therapy product development with Intellia Therapeutics (*17*). These announcements are devastating to the SCD community and represent critical moments for the genetic medicine field that require careful and intentional advocacy. These announcements also came at the same time as the US Centers for Medicare and Medicaid Services approved a cell and gene therapy access model giving Medicaid beneficiaries access to cell and gene therapies for cancer and SCD (*6*). Thus, the only patients who will likely have access to approved SCD gene therapies are those in the United States.

Numerous value-based systems used in other areas of medicine have the potential to mitigate the costs associated with gene and cell therapies (*18*) (Table 1). Bundled payment approaches (*19*) and a lack of compromise between pharmaceutical companies and the needs of the average patient have led to a rapid increase in the price tag for gene and cell therapies. This exacerbates enormous disparities regarding access to therapies. Mortgage-pricing and value-based systems may be key components of a multipronged solution wherein costly therapies could be paid off over time, and drugs could be priced based on the true value they provide for patients (*18*).

Patient input and feedback should be an invaluable resource for informing organizations like the Institute for Clinical and Economic Review (ICER) that set pricing standards to determine the impact of a drug, because they take into consideration the real-world effect on quality of life for patients (*19*). From a global perspective, wealthy countries should subsidize the financial burden associated with the development of cell and gene therapies in LMICs. These solutions place an emphasis not on what one person or company loses, but on what communities and societies around the world gain: a robust future with healthier citizens. Whereas most of the world has moved to some degree of national health care and the United Nations has declared access to health care to be an essential human right, civil society has allowed very narrow market considerations to govern how cell and gene therapy products are developed and then delivered. The economic considerations appear solely geared to maximize short-term profit in markets that can afford high-priced products, while ignoring the Majority World where these interventions are needed. Rather than allowing multiple products and approaches to be developed and to compete, the investors and boards of companies large and small alike use a financial risk-benefit analysis alone. This explains in part why the R&D environment for SCD cavitated when two leading contenders appeared to be close to gaining FDA approval. How can the community gain a voice?

NOTHING ABOUT US, WITHOUT US

The creation and requirement of a Community Advisory Board (CAB) by the organizations who make decisions (e.g., companies, research institutions, etc.) regarding gene and cell therapy research for SCD would allow patients to be at the table when options are being discussed and commitments are being made (Table 1). CAB members should include patients diagnosed with SCD, their caregivers, physicians who treat patients with SCD along with their clinical and research teams, and government officials in line with best practices outlined in 2011 by the US Centers for Disease Control and Prevention (*20*). Lack of diversity at the decision-making level is a disservice to patients. Patients must be empowered in the creation and maintenance of CABs for real change to occur. They must be empowered

to communicate what they want, whereas companies must be transparent about limitations. CAB members know that the ultimate stakeholders are always the patients. Researchers whose work benefits from our participation should encourage insight from a diverse group of patients who experience the disease daily. Although ascribed to a single genetic mutation, no two patients' experience with SCD is the same. SCD is not one size fits all, because our genetic diversity makes each of us unique and contributes to our experiential differences with this disease. Yet, outside of the groups organized by large pharma for clinical trials, the SCD community remains in need of greater resources to effectively organize and speak with a unified voice. Disease-specific foundations and patient rights and advocacy groups are excellent resources. However, many patient advocacy groups in the United States have very limited resources and are most concerned about direct challenges for patients, such as receiving appropriate pain relief and care during acute crises. Creation of well-resourced CABs should be the responsibility of research teams, with financial support from government and foundations for publicly funded research and from commercial entities when sponsored clinical research is conducted. Patient advocacy groups should lobby for this inclusion with the support of researchers. Government policy should be enacted to protect the rights of patients to participate in and benefit from research to improve their health through CABs.

The Global Gene Therapy Initiative (GGTI) was created in 2020 to tackle barriers to LMIC inclusion in gene and cell therapy development, including curative approaches for SCD and HIV. The GGTI brings together researchers, clinicians, regulators, funders, and other diverse stakeholders from various sectors in dialogue with patient advocates from the SCD and HIV communities to establish a sustainable pathway for implementing gene and cell therapies in LMICs. As community advocates for the GGTI, we strongly support synergies between the fields of SCD and HIV gene therapy research to meaningfully engage communities about the promise of gene therapy in resource-limited settings. For example, in Uganda, the community-based organization Joint Adherent Brothers and Sisters against AIDS (JABASA) has a mission to bridge gaps in the response to HIV/AIDS and is now involved in SCD community engagement and capacity-building efforts. JABASA was created to empower people with HIV about the importance of their medications and the dangers of not adhering to treatment as prescribed by a professional health worker. JABASA empowers the general community by educating them about the importance of new research advances, e.g., in gene and cell therapy. There are several parallels between the fields of HIV and SCD. For example, people with SCD and HIV need to adhere to current medication regimens as research toward curative options continues. We need to continue to bridge the mutual literacy gap between researchers and communities in both the SCD and HIV fields. In our opinion, institutional support—such as that we have witnessed at the Joint Clinical Research Center in Uganda—is critical because it helps connect researchers with affected communities. The Sickle in Africa Consortium (SickleInAfrica.org) has united patient, clinical, and research programs through its data coordinating center and clinical centers in Tanzania, Ghana, Nigeria, Mali, Zimbabwe, and Uganda. These programs are working hard to make a research and clinical impact and yet struggle to gain the level of funding that would be required for gene and cell therapy approaches to be implemented. They have set the example of how affected populations can begin to engage the global world of medical research.

This level of engagement should be required of researchers in developed countries. There must be evidence to regulators and patient care providers that the community has been a true partner and has been included in the decision-making process. If a system does not develop where meaningful input from community members occurs at every step of research and clinical studies—including aspects such as how to build community trust, affordability, risk tolerance, clinical trial design, appropriate participant incentives, retention, and long-term follow-up strategies—we will be left repeating the same mistakes of the previous five decades. Advanced health care and research capacity that supports gene and cell therapy cannot remain the province of the richest countries and populations. Local development and local manufacturing processes must become the norm. Gene and cell therapy trials should be conducted where most people with SCD and HIV live. We should invest in meaningful community engagement and ensure sustained funding for those efforts. We also need to invest in sociobehavioral sciences to make sure gene and cell therapy product development is aligned with patient/participant preferences. As we scale up gene and cell therapy globally, ethics should also be embedded as part of the research process. Given the long-term follow-up needed for people who participate in gene and cell therapy clinical trials, there should also be comprehensive support programs for clinical trial participants to support their mental and emotional health and understand their experiences with trial participation, including treatment pauses (*21*).

We therefore urge researchers to empower affected communities by speaking up and sharing information with patients. Most importantly, researchers, clinicians, regulators, and funders must become familiar with and informed by the lived experiences of their patients. Creation of a roadmap toward community advocacy and engagement should be done in collaboration with key stakeholders. We encourage those who are involved with research on SCD, HIV, and other illnesses to become knowledgeable not only in science, but also in learning about the human faces of these conditions. Moreover, we challenge health care providers to listen to their patients with understanding and empathy, invite open discussion, ask for their input, and include them in decisions. Together, we need to make sure that new advances are rolled out in the setting of CABs and in collaboration with patient advocates. We cannot miss this opportunity to build trust among physicians, scientists, and, most importantly, the communities that will benefit the most from advances in gene and cell therapies.

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Olabimpe Olayiwola, Adam Castillejo, Michael Louella, Moses Supercharger, Evelyn Harlow, Lynda Dee, Khadidja Abdallah, Cissy Kityo-Mutuluuza, Jennifer E. Adair, Rimas Orentas, and Karine Dubé

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