# The Promise of a Cure: An Analysis of the Social Construction of Gene Therapy for Sickle Cell Disease

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**Introduction**

Sickle Cell Disease or SCD is a disorder which causes red blood cells to be shaped incorrectly, in a sickle shape, rather than as a disc. These misshaped red blood cells are caused by a mutation in the gene associated with hemoglobin. Hemoglobin is a molecule that allows red blood cells to carry oxygen (NHLBI & NIH, 2025). Due to the mutated hemoglobin, sickle cell disease patients, or SCD patients, often experience intense episodes of severe pain, known as vaso-occlusive crises, and chronic anemia (Kidane Gebremeskel et al., 2025, p.2).

Although historically there have been numerous promises for a cure to SCD, there is currently no universal cure readily available. However, gene therapy has emerged as a potential option for long-term treatment of SCD. CASGEVY, a gene therapy treatment for SCD developed by Vertex Pharmaceuticals and CRISPR Therapeutics, was approved by the FDA for use by SCD patients 12 and older on December 8, 2023 (Olalla & Río, 2024). CASGEVY treatment requires collection of the patient's stem cells, which are cells that have the potential to differentiate into red blood cells (Mayo Clinic Staff, 2026). The patient's stem cells are then modified, using the CRISPR/Cas9 gene editing technology. These modified cells are reinfused back into the patient, who is then monitored at the hospital while the modified stem cells begin to engraft, or settle themselves, into the bone marrow. Patients then continue to attend follow up appointments for fifteen years (*CASGEVY (Exagamglogene Autotemcel) | Boston Children's Hospital*, n.d.). CASGEVY treatment is a complex process, involving numerous hospital visits, and fifteen years of follow up appointments.

The historical promise of a single, simple cure for sickle cell disease continues to influence the contradictory narrative of the SCD gene therapy CASGEVY. This contradictory narrative of CASGEVY embodies the uncertain, variable, and changing nature of the social

construction of science, and highlights the role of racial politics in the field of SCD research. The social construction of science is symbolized through the changing, variable, and uncertain nature of science, contradicting the current paradigm of the objectivity of science (Gandolfi, 2023, p.87). CASGEVY's contradictory nature is exemplified in its presentation as a breakthrough, milestone therapy, in opposition to the lack of uptake among SCD patients (Office of the Commissioner, 2024; McKenzie, 2026).

Giulia Gandolfi's paper (2023), titled "Social and Individual Features of Knowledge: The Non-Neutrality of Science in Personalized Medicine" utilizes a three pronged approach to demonstrate the social construction of science in personalized medicine. This three pronged approach includes analysis of the origins of science, truth validation, and the function of scientific practice (p. 84). Personalized medicine within Gandolfi's paper (2023) refers to use of genomic information to inform drug recommendations (p. 98). To analyze the social construction of gene therapy for SCD, this three pronged approach will be utilized. To begin, the analysis will examine the origins and history of treatment for SCD, how truth validation is performed in the field of SCD research, and the function of scientific practice as it relates to gene therapy.

**Origins of Treatment for SCD**

*"[We] may be able to devise a small innocuous molecule which will lock permanently on to the defective hemoglobin and prevent the abnormal molecule from misbehaving."*

- *George Gray, 1951 (as cited by Wailoo & Pemberton, 2006, p. 124)*

The origins of gene therapy of sickle cell disease finds its roots in the proposed etiologies of sickle cell disease, and the search for a one-time long-term cure for the disease. One of the most prominent themes through this history is a promise of a single, simple cure that has yet to manifest itself within the current field of scientific practice. While gene therapies like

CASGEVY are marketed as one-time treatments, the truth is much more complex, and ultimately does not fulfill the historical search for a single, simple cure.

Sickle cell disease was first identified by James B. Herrick in 1910, when he published a description about 20-year old Walter Clement Noel's sickling red blood cells (NHLBI & NIH, 2010). In 1917, Victor Emmel established a test to check for sickling of red blood cells, which was termed "Emmel's test" (Wailoo, 1999, p.142). While Emmel posited that his technique could detect a potential disease, it soon became the defining test for assessing sickling of red blood cells (Wailoo, 1999, p.142). This marked the shift from sickle cell disease as, "a *clinical* entity [to] a *technological* one" (Wailoo, 1999, p.142). As Emmel's test narrowed the focus of the etiology of SCD to a patient's blood, "Negro" blood began to emerge as a cause of SCD (Wailoo, 1999, p.144, 147). The disease itself became associated with the Black community, reinforced by prominent organizations in the field of medicine, including the Journal of the American Medical Association (JAMA) and the American Red Cross, a humanitarian organization (Wailoo, 1999, p. 148, 150). Racial identity became embodied within the etiology of the disease itself.

In 1949, Linus Pauling and his associates discovered that sickle cell disease is caused by an abnormal hemoglobin molecule (Feldman & Tauber, 1997, p.623). Sickle cell disease was thus named a "molecular disease", the first of its kind. (Feldman & Tauber, 1997, p.623). The reduction of sickle cell disease to an abnormal hemoglobin molecule informs the possibility of a single cure targeted to the abnormal hemoglobin molecule (Wailoo & Pemberton, 2006, p.123). A scientist, George Gray, claimed in 1951 that researchers may be able to create a, "small innocuous molecule," (Wailoo & Pemberton, 2006, p.124) to cure sickle cell disease. There is a false equivalency drawn in the history of sickle cell disease from this abnormal hemoglobin

molecule and a small molecule which will be used to cure SCD. Thus, the discovery of the etiology of sickle cell disease directly informed the search for a single, simple cure.

Once this false equivalency was established, several contenders emerged for the role of this single, simple cure. From the 1950s to the 1970s, sickle cell began to acquire additional attention from the media, new diagnostic tools were developed, and further information about the molecular basis of sickle cell disease was uncovered (NHLBI & NIH, 2010). However, no cure had been identified. During this time, the Civil Rights movement helped to define the vaso-occlusive, painful crises as an embodiment of the failure of the medical establishment to confront the issue of suffering in Black communities (Wailoo, 1999, p.154). In 1970, Dr. Robert Nalbandian, announced that he had treated two SCD patients with the molecule urea, which had successfully reinstated the sickle red blood cells to their normal shape (Wailoo & Pemberton, 2006, p.126). Urea quickly became marketed as a therapeutic breakthrough, but the severe side effects of urea were just as quick to knock it down (Wailoo & Pemberton, 2006, p.126-128). Sodium cyanate was additionally among the numerous de-sickling agents, "promoted, studied, and prematurely celebrated for their desickling qualities," (Wailoo & Pemberton, 2006, p.129). The false equivalency of the etiology of SCD and a cure continued to haunt the search and promise of a single, simple cure for sickle cell disease.

Current treatment options include blood transfusions, as well as a variety of drug options, such as hydroxyurea, voxelotor, crizanlizumab, and L-glutamine. Hydroxyurea is the standard treatment option, following blood transfusions (Kuriri, 2023, p.1).. Voxelotor, crizanlizumab, and L-glutamine are all recently approved medications for SCD treatment (Kuriri, 2023, p.1). The shift to characterizing SCD by the vaso-occlusive crises are a crucial element of approvals for therapeutic treatments (Kuriri, 2023, p. 2; Wailoo, 1999, p.154; Office of the Commissioner,

2024). For many Black Americans, this focus on vaso-occlusive crises helped to capture the, "true identity of this disease," (Wailoo, 1999, p.154). Conversations about race construct the identity of SCD beyond the molecular etiology, demonstrating how science, and specifically SCD treatment, relies upon social construction.

Beyond these treatments, curative potential lies within bone marrow transplantation, and gene therapy. Bone marrow transplantation, also referred to as hematopoietic stem cell transplantation, involves the replacement of a patient's diseased stem cells with healthy donor stem cells to cure the disease (Kuriri, 2023, p.5). Bone marrow transplantation began to become a viable option for treatment of SCD in the early 1990s, but was marred by questions of doubt given the history of the promise for a cure for SCD that never seemed to come (Wailoo & Pemberton, 2006, p.149-151). Additionally, bone marrow transplantation was marketed as offering a cure, but given the risks, could ultimately leave SCD patients with another disease (Wailoo & Pemberton, 2006, p.150). Specifically, one of the potential risks of bone marrow transplantation is the development of graft versus host disease, in which the patient rejects the transplantation, which may lead to death (Kuriri, 2023, p.5). Gene therapy comes with its own set of challenges, namely the uncertainty in outcomes and lack of information about potential long-term consequences (Kuriri, 2023, p.9; Romero et al., 2018, p.1133). However, emerging gene therapies such as CASGEVY offer a glimpse into the possibilities that gene therapy can provide in treatment of SCD (Olalla & Río, 2024). Ultimately, the hope for a cure for SCD remains powerful, permeating paradigms around bone marrow transplantation, and gene therapy. CASGEVY's potential to "cure" SCD is complicated by the limits of gene therapy, ethical concerns, and financial burdens (Munung et al., 2023; Romero et al., 2018). These social

concerns serve to highlight the ways in which the social construction of science intersects with the contradictory narrative around CASGEVY.

**Truth Validation**

*"Every piece of knowledge is socially conditioned, challenging the traditional notion that science rests solely on the subject-object dichotomy."*

- *Giulia Gandolfi (2023, p.87)*

Truth validation in the context of gene therapy of sickle cell disease is the methodology used to support scientific arguments made in the field. Gene therapy development uses a wide variety of methodologies to support scientific arguments, and all of these methodologies will not be explored in this section. To demonstrate the social construction of this science, and specifically truth validation mechanisms, this section will focus on the use of ethno-racial categories, and the uncertainty of long-term effects in gene therapy research for SCD. The uncertainty and variation in the use of ethno-racial categories highlights the role of racial politics in SCD research. Both the variation and uncertainty in use of ethno-racial categories, and lack of truth validation mechanisms for long-term effects assessment of SCD gene therapy demonstrate the process of the social construction of science.

In order to analyze the truth validation mechanisms of SCD research, a frame of analysis must be established. Gandolfi (2023) writes of a "thought collective", as established by Ludwig Fleck, a physician and biologist. A "thought collective" is defined as, "a community of individuals bound together by a shared style of thinking, encompassing common ideas, values, and perceptions of the world." (Gandolfi, 2023, p.86). For this analysis, biomedical researchers for SCD form a thought collective, bound together by a shared focus on sickle cell disease from a

biomedical perspective. Each individual researcher ultimately has their own complex views of the research they are performing, and its positionality within the field. However, in order to perform an analysis of truth validation mechanisms, the thought collective of SCD biomedical researchers will be utilized as a singular entity. Additionally, due to the limited availability of published papers discussing large-scale studies utilizing gene therapy mechanisms for SCD, other biomedical publications discussing SCD are used to represent the thought collective which encompasses SCD gene therapy researchers.

Biomedical researchers often use ethno-racial categories, or ERCs to categorize individuals based on their ethnic or racial identity (Kidane Gebremeskel et al., 2025, p. 1). Historically, organizations such as the FDA or NIH have pushed for information on safety and efficacy information stratified by categories such as race and ethnicity (Epstein, 2004, p.184). Dr. Steven Epstein, a professor of sociology, questions the meaning of implying that there are medical consequences of categories, such as race and ethnicity, and how these ideas are utilized in achieving various "social, political, economic, scientific, and medical agendas," (2004, p. 185). Social categories, such as race and ethnicity, may become falsely constructed as medical or genetic identities, if the medical consequences of these categories are not interrogated. SCD has a history of association with the Black community, from the proposed etiology of SCD arising form the presence of "Negro" blood, to the high incidence rates of SCD within the Black community in the United States. The use of ethno-racial categorization ("Negro" blood) in SCD research has historically had tangible effects on the dialogue surrounding race and racism in the United States (Wailoo, 1999; Kidane Gebremeskel et al., 2025). Therefore, it is important to interrogate the use and non-use of ethno-racial categories in this field of research.

Aida S. Kidane-Gebremeskel and their colleagues (2025) examined ERCs in SCD research through the lens of its role as a confounder within biomedical research. Their findings highlighted that there was a lack of standardized practice in the use of ERCs as a confounder in SCD research (p.1). While medical journals encourage explanations in methodological decisions for how race and ethnicity were determined and used, this study revealed that this encouragement did not translate into practice (p.7). Given the fluidity of ERCs and the non-congruence of these categories globally (p.2), it is increasingly important to clarify the use and meaning of these categories within SCD research. Use or non-use of ERCs as a confounder in the methodological design of a study underscores the variability in the truth validation mechanisms for SCD research. This use or non-use of ethno-racial categories in SCD biomedical research exemplifies the ways in which this science relies on social construction, and continues to carry the racial meanings uncovered in the origins of gene therapy for SCD.

Publications discussing gene therapy for SCD also have variation within the language use and non-use to address ERCs, and the social identity of SCD as a "Black disease". Many gene therapy publications only assessed one or two patients, which made it difficult to address how ERCs were utilized more broadly in the specific subfield of gene therapy research for SCD. In two papers which explored gene therapy treatments for patients with SCD (Frangoul et al., 2021; Ribeil et al., 2017), the racial and ethnic demographics of the patients were not listed. In review articles discussing gene therapy more broadly, race was mentioned at the beginning, typically listed alongside information regarding incidence rates (Romero et al., 2018; Anderson et al., 2023). It is important to note that the analysis conducted by Daniela Anderson (2023) and their colleagues makes specific mention that race as they are discussing the category has social implications, and not biological origins. This use of ERCs to frame SCD incidence rates is also

found on the National Heart, Lung, and Blood Institute's website explaining what SCD is (NHLBI & NIH, 2025), as well as in the book, *The Troubled Dream of Genetic Medicine* by Keith Wailoo & Stephen Pemberton which explores the search for a cure for SCD from the lens of genetic medicine (2006, p.133). The varied use and non-use of ethno-racial categories in SCD gene therapy research mirrors the variation in biomedical research for SCD as well, positioning SCD gene therapy research within the broader thought collective of biomedical research for SCD. This variety demonstrates the ways in which science is informed by social underpinnings, and reveals the uncertain role of race in SCD research in the future.

In addition to the use of ERCs as an uncertain form of truth validation in SCD gene therapy research, the uncertainty of the long-term effects of gene therapy also highlight the unresolved foundation of the truth validation mechanisms of this research. Various authors across review articles and gene therapy trials assert that the long-term effects of gene therapy are unknown (Munung et al., 2024; Ribeil et al., 2017; Romero et al., 2018). There are a lack of truth validation mechanisms to support the long-term safety and efficacy of gene therapy for SCD. This lack of mechanisms further demonstrates how gene therapy is not completely objective, but rather reliant upon a thought collective to create mechanisms for truth validation that are accepted by the scientific community.

**Function of Scientific Practice**

*"What makes you think that because [SCD treatment is] now open to all people . . . that the uptake would be higher?"*

- *Colleague of Courtney Rice (McKenzie, 2026)*

Giulia Gandolfi defines the function of scientific practice to be exploring, "the material and cultural environment in which scientific progress unfolds" (Gandolfi, 2023, p. 84). To

demonstrate the ways in which CASGEVY embodies the uncertain nature of the social construction of science, the focus of this section will remain on the cultural environment in which scientific progress unfolds, rather than the physical and material environment. This is to be demonstrated through the contradiction between the promise of CASGEVY, and the lack of widespread administration of CASGEVY to affected SCD patients.

The origins of the promise of a single, simple cure for SCD find their roots in the history of the disorder itself. Researchers and media were quick to market new molecules and agents as curative for SCD, but ultimately no single, simple cure has been found (Wailoo & Pemberton, 2006). CASGEVY follows a similar path, with grand promises and hope surrounding the gene therapy, to the lack of widespread use of CASGEVY among SCD patients. In 2024, Ajeet Singh and their colleagues asserted that CASGEVY, "has emerged as a beacon of hope for SCD patients and signifies a potential paradigm shift in SCD management," (p.4555). Similar grandiose language ("beacon of hope", "paradigm shift") is employed throughout the piece (2025, p.4), signaling to the larger promise of the curative potential of CASGEVY. News articles also hint at the curative potential of CASGEVY, returning to the historical promise of a cure for SCD (D'Amico, 2024). The cultural environment that CASGEVY enters into is built on a historical promise for a cure for SCD. This built cultural environment permeates into presentations of CASGEVY within both the scientific community and broader public through grandiose and cure-centered language.

The contradiction in the narrative of CASGEVY stems from the cultural environment surrounding its FDA approval, and the lack of uptake of this therapy among SCD patients. One article in particular by Heather McKenzie (2026) discusses how both CASGEVY and another approved gene therapy for SCD, LYFGENIA, both lack traction two years after their approval by

the FDA. McKenzie interviewed Courtney Rice, a principal at Acadia Strategy Partners, which is an advising company for biotech and gene therapy companies. Rice highlighted various barriers in accessing care that contradict the high hopes and promises for CASGEVY and LYFGENIA. In particular, Rice discussed the lengthy time of the procedure, which although marketed as a one-time treatment, involves several steps before the gene therapy treatment. This dichotomy is visible on Vertex Pharmaceuticals and Boston Children's Hospital's CASGEVY information pages. On both websites, the primary explanation for CASGEVY is a "one-time treatment" (although Boston Children's Hospital does clarify that it is a "one-time gene therapy treatment"). However, further down the page, four steps of the treatment are listed, which will ultimately span several months for the initial treatment, and includes fifteen years of follow up appointments (Boston Children's Hospital, n.d.; Vertex Pharmaceuticals, 2024). Additionally, one of the potential side effects of the initial conditioning treatment to prepare patients for gene therapy is infertility (Boston Children's Hospital, n.d.; Vertex Pharmaceuticals, 2024). Rice also emphasizes that likely those eligible for the treatment, "are probably in the working population and commercially insured," (McKenzie, 2026, p. 3), making the lengthy time commitment a key barrier to accessing treatment. The contradictory nature of CASGEVY as both a breakthrough therapy and a gene therapy lacking traction underscores the social construction involved in this gene therapy. Uncertainty and variation are hallmarks of social constructions, and CASGEVY embodies these traits. While CASGEVY does have curative potential, and this should not be taken lightly, addressing barriers to treatment needs to be prioritized in order to maximize the accessibility of CASGEVY and similar treatments.

Broadly, gene therapy researchers have previously identified potential barriers for patients to access gene therapy treatment for SCD. Similar to the devastating side effects of urea,

one of the desickling agents presented as a cure for SCD, gene therapy has serious risks which lack long-term information. Several of the risks include, “gene silencing, gene toxicity, phototoxicity, uncertainties (even if transient) around germline transmission of DNA, and viral shedding,” (Munung et al., 2024, p. 205). Additionally, as previously discussed, infertility is another potential side effect of the conditioning treatment for gene therapy. Presentation of gene therapy as a one-time treatment can lead to therapeutic misestimation, wherein patients and caregivers may be misinformed about the limits and benefits of gene therapy (Munung et al., 2024, p. 205). Overall, the changing, uncertain, and variable cultural environment surrounding both CASGEVY and SCD gene therapy more broadly serve as demonstration of how this science is embedded in social construction. The science of gene therapy is ever-changing, rejecting science’s supposed objectivity, and demonstrating the social conditioning of each piece of knowledge.

**The Limits to CASGEVY & Gene Therapy: How to Move Forward**

*“Without the intentionality of equity as a primary and central motivator, it’s of little surprise that the developed product is inaccessible and incongruent to how those living with SCD envision equity.”*

- Santiago J. Molina & Melissa Creary, 2025, p. 9

Santiago J. Molina & Melissa Creary (2025) demonstrate how the racial politics in SCD and associated health justice language help to prop up the promise for a cure for SCD, while reinforcing barriers to accessibility. In order to move forward and address patient barriers, equity should be centered within future gene therapy research (Molina & Creary, 2025, p.9). The social construction of science must be taken into consideration, and addressed in future gene therapy research to avoid future contradictory narratives such as that surrounding CASGEVY.

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