

BACKGROUND



- On December 8, 2023, the U.S. Food & Drug Administration (FDA) approved two novel gene therapies (GT), each of which aim to cure sickle cell disease (SCD), a debilitating and historically marginalized condition.
- One of these GT marks the first clinical approval of a CRISPR-based therapy.

Image: Victoria Gray, the first recipient of GT for SCD, sitting in a hospital bed to receive long-term follow-up care.

OBJECTIVES

- Explore and synthesize diverse stakeholders' perspectives around the clinical implementation of GT for SCD in the United States (U.S.).
- Identify urgent issues complicating this pivotal milestone in SCD care & genomic medicine.
- Recommend interventions that address the underlying issues identified and ultimately promote restorative justice through the delivery of SCD care & GT.

METHODS

- Conducted a targeted literature review organized around 8 key stakeholder groups...

- PEOPLE LIVING WITH SCD & CAREGIVERS
- PATIENT ADVOCACY ORGANIZATIONS
- PROVIDERS & HEALTH CARE ORGANIZATIONS
- PUBLIC HEALTH & SOCIAL SERVICES ORGANIZATIONS
- FEDERAL & STATE GOVERNING BODIES
- RESEARCH & DEVELOPMENT INDUSTRY
- INSURANCE CARRIERS
- CIVIL SOCIETY



- Consulted with thought leaders
- Participated in relevant community meetings & listening sessions

ACKNOWLEDGEMENTS

- Thank you to the following individuals for their feedback and support in developing this capstone project and framework:
- Adele Jasperse, JD, MBE - Capstone Faculty Advisor, Harvard Medical School (HMS)
 - Bridget Kervin, BS, Palmer Montalbano, BA, & Taya Wallis, BSN, RN - Capstone Seminar Members, HMS
 - Vence Bonham, Jr., JD - Acting Deputy Director, National Human Genome Research Institute (NHGRI)
 - Melissa Creary, PhD, MPH - Assistant Professor, University of Michigan School of Public Health
 - SCD Community

KEY FINDINGS

UNDERLYING ISSUES	ETHICAL PRIORITIES	RECOMMENDATIONS
<ul style="list-style-type: none"> GT outcomes vary by person & are not always "curative" GT is novel & still experimental; long-term outcomes are unknown Severe risks & side effects are associated with GT & the chemotherapy conditioning required 	<p>Safety & Efficacy</p> <p>Benevolence, Nonmaleficence, & Utilitarianism</p>	<ul style="list-style-type: none"> Identify key age range to consider GT Prioritize patient-reported outcomes in evaluating long-term safety & efficacy Compensate for longitudinal research Create a long-term SCD survivorship program Develop SCD GT clinical guidelines Continue research & development for new and improved SCD treatments
<ul style="list-style-type: none"> Burdensome treatment process requiring significant time, resources, and support systems Minority of SCD population is eligible for GT Disparate institutional investments across SCD treatments, which risks inequities within the SCD population 	<p>Accessibility & Equity</p> <p>Liberal Egalitarianism & Well-being Theory of Justice</p>	<ul style="list-style-type: none"> Insure key expenses & wraparound services Collaborate with community groups to promote bidirectional learning & community-based, outpatient care support Ensure social securities & safety nets Standardize screening process for GT & social determinants of health Research if current eligibility standards are appropriate or overly stringent Create parity in coverage across SCD treatments & the population
<ul style="list-style-type: none"> Informed consent to pursue GT is undermined due to misinformation, limited GT education, & disparate investments across SCD treatments Poor baseline in trust & shared decision-making for SCD due to racism & medical paternalism 	<p>Shared Decision-Making</p> <p>Respect for Autonomy, Care Ethics, & Feminist Ethics</p>	<ul style="list-style-type: none"> Communicate about GT transparently & objectively in both the media & clinic Implement a standardized, interdisciplinary practice of genetic counseling for SCD & GT that engages various learning styles Create parity in access, investment, and counseling for all SCD treatments Train providers in relationship-building & shared decision-making in SCD care
<ul style="list-style-type: none"> Paucity in critical needs for delivery of any SCD treatment, such as trained health care providers, comprehensive treatment centers, & national data collection programs High upfront costs to prepare health care systems, manufacture GT, & deliver necessary procedures 	<p>Feasibility</p> <p>Liberal Egalitarianism & Capability Theory of Justice</p>	<ul style="list-style-type: none"> Promote SCD education & training in medical schools & hospitals Invest in the establishment of SCD comprehensive care centers Strengthen SCD data collection & sharing Insure & deliver basic SCD standards of care recommended in clinical guidelines Mandate anti-racism training Advance other quality improvement initiatives

SUMMARY

- Complex and multi-faceted issues complicate GT's integration into the SCD clinic.
- Stakeholders must collaboratively and comprehensively address these issues to ensure the clinical integration of GT for SCD promotes restorative justice throughout the U.S. SCD population.

LIMITATIONS

- This research was limited to the U.S., despite the majority of the global SCD population living abroad.
- The resulting framework is conceptual and should be validated through primary data collection with stakeholders.

FUTURE DIRECTIONS

- Disseminate initial framework outlining these findings & specific opportunities to take action (see "Framework" QR code below).
- Solicit feedback & advocate for action from key stakeholders.

FRAMEWORK, CITATIONS & CONTACT

Access the "Framework" for more detailed findings:

bit.ly/GTSCDFramework



Citations: bit.ly/GTSCDCitations

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