

Public Health Implications of CRISPR-Based Gene Therapy for Sickle Cell Disease: Access, Equity, and Health System Readiness in the United Kingdom

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Prevalence

Sickle cell Disease [SCD] is an inherited blood condition that reduce quality and expectancy of life. It is mainly caused by mutation in a single gene that effect haemoglobin (Mutebi, 2025: Tariq et al., 2024) This mutation is occurred in β -globin gene (Butt et al., 2025). This disease is most common in people with African or Caribbean family background (NHS, 2017).

❑ In England it is now considered as one of the most common and serious genetic condition. Study conducted in 2016 highlight that around 14000 people which is equal to 1 in 4600 people are suffering from this problem (Dormandy et al., 2017).

❑ This number had reached to 15000 in 2021 (Williams and Maguire, 2021) while in 2024 the number of impacted persons had reached upto 17000 which is approximately 1 in 79 babies [The PDA, 2024]. Although the condition of SCD is worse in England but the patient with this issue is under supported because of the limited treatment options (Nwasike, 2025).

❑ The study presented by Hsu et al., (2022) highlight that patients with SCD are suffering from several inequalities of both 20 and 21 century.

The NHS Race and Health Observatory report presented in 2025 also mentioned that the patients with SCD are impacted by negative experience of care, lack of particular knowledge and under resourcing (NHS, 2025). One of the major reason of these inequalities is very limited availability of treatment. To overcome this UK has recently got for CRISPR-based gene therapy that offer a potential functional cure (NYSCF, 2023: SYNTHEGO, 2025). The evacuation of this tool have identified that treatment through this requires specialised centres, conditioning chemotherapy, and long-term follow-up, raising major public health challenges regarding equitable access across NHS regions. The study presented by Nih.gov (2024) further highlighted that patient eligibility criteria can also create limitations in implication of CRISPR.

References

Mutebi, N. (2025). *Sickle cell and beta thalassemia: what are the treatment options and challenges?* POST. <https://post.parliament.uk/research-briefings/post-pn-0754/#:~:text=Personalised%20medicines,-Recent%20advances%20in&text=These%20include%20advanced%20therapy%20medicinal,to%20receive%20the%20therapy%20initially.>

NHS (2017). *Sickle cell disease*. Nhs.uk. <https://www.nhs.uk/conditions/sickle-cell-disease/>

Dormandy, E., James, J., Inusa, B., & Rees, D. (2017). How many people have sickle cell disease in the UK? *Journal of Public Health*, 40(3), e291–e295. <https://pubmed.ncbi.nlm.nih.gov/29294057/>

PDA (2024). *Sickle Cell Disease in the UK: A Call to Action for Pharmacists*. The Pharmacists' Defence Association. <https://www.the-pda.org/sickle-cell-disease-in-the-uk-a-call-to-action-for-pharmacists/>

Williams, T., & Maguire, B. (2021). *A report for Sickle Cell Society Sickle Cell in South London - Research Report*. <https://www.sicklecellsociety.org/wp-content/uploads/2021/02/South-London-Research-FINAL-April-2021-SD-Hyperlink-Removed.pdf>

Butt, H., Mandava, M., & Jacobsohn, D. (2025). Advances in Gene Therapy for Sickle Cell Disease: From Preclinical Innovations to Clinical Implementation and Access Challenges. *The CRISPR Journal*, 8(3), 174–188. <https://doi.org/10.1089/crispr.2024.0101>

Tariq, H., Khurshid, F., Khan, M. H., Dilshad, A., Zain, A., Rasool, W., Jawaidd, A., Kunwar, D., Khanduja, S., & Akbar, A. (2024). CRISPR/Cas9 in the treatment of sickle cell disease (SCD) and its comparison with traditional treatment approaches: a review. *Annals of Medicine & Surgery*, 86(10), 5938–5946. <https://doi.org/10.1097/ms9.0000000000002478>

Nwasike, (2025). *The implicit bias of sickle cell disease*. The King's Fund. <https://www.kingsfund.org.uk/insight-and-analysis/blogs/implicit-bias-sickle-cell-disease>

Hsu, L. L., Hooper, W. C., & Schieve, L. A. (2022). Prioritizing Sickle Cell Disease. *Pediatrics*, 150(6). <https://doi.org/10.1542/peds.2022-059491>

NHS (2025). *NHS - Race and Health Observatory*. (2025, June 18). NHS – Race and Health Observatory. <https://nhsrhc.org/news/sickle-cell-patients-face-unequal-care-new-report-highlights/>

NYSCF (2023). *World's First CRISPR-Based Gene Therapy Approved by FDA - New York Stem Cell Foundation*. New York Stem Cell Foundation. <https://www.nyscf.org/resources/worlds-first-crispr-based-gene-therapy-approved-by-fda/>

SYNTHEGO (2025). *CRISPR Sickle Cell Gene Therapy: Approaches, Challenges, and Progress*. (2025). Synthego. <https://www.synthego.com/crispr-sickle-cell-disease/>

Nih.gov (2024). *CRISPR Technologies for In Vivo and Ex Vivo Gene Editing*.; Canadian Agency for Drugs and Technologies in Health. <https://www.ncbi.nlm.nih.gov/books/NBK609557/#:~:text=Ethical%20Considerations,-The%20literature%20identifies&text=Uncertainties%20also%20remain%20related%20to,unintended%20consequences%20of%20these%20therapies.&text=These%20uncertainties%20have%20implications%20for,human%20disease%20models%20and%20therapies.&text=Access%20to%20CRISPR%20technologies%20may,equitable%20access%20across%20population%20groups.>

Subica, A. M. (2023). CRISPR in Public Health: The Health Equity Implications and Role of Community in Gene-Editing Research and Applications. *American Journal of Public Health*, 113(8), 874–882. <https://doi.org/10.2105/ajph.2023.307315>

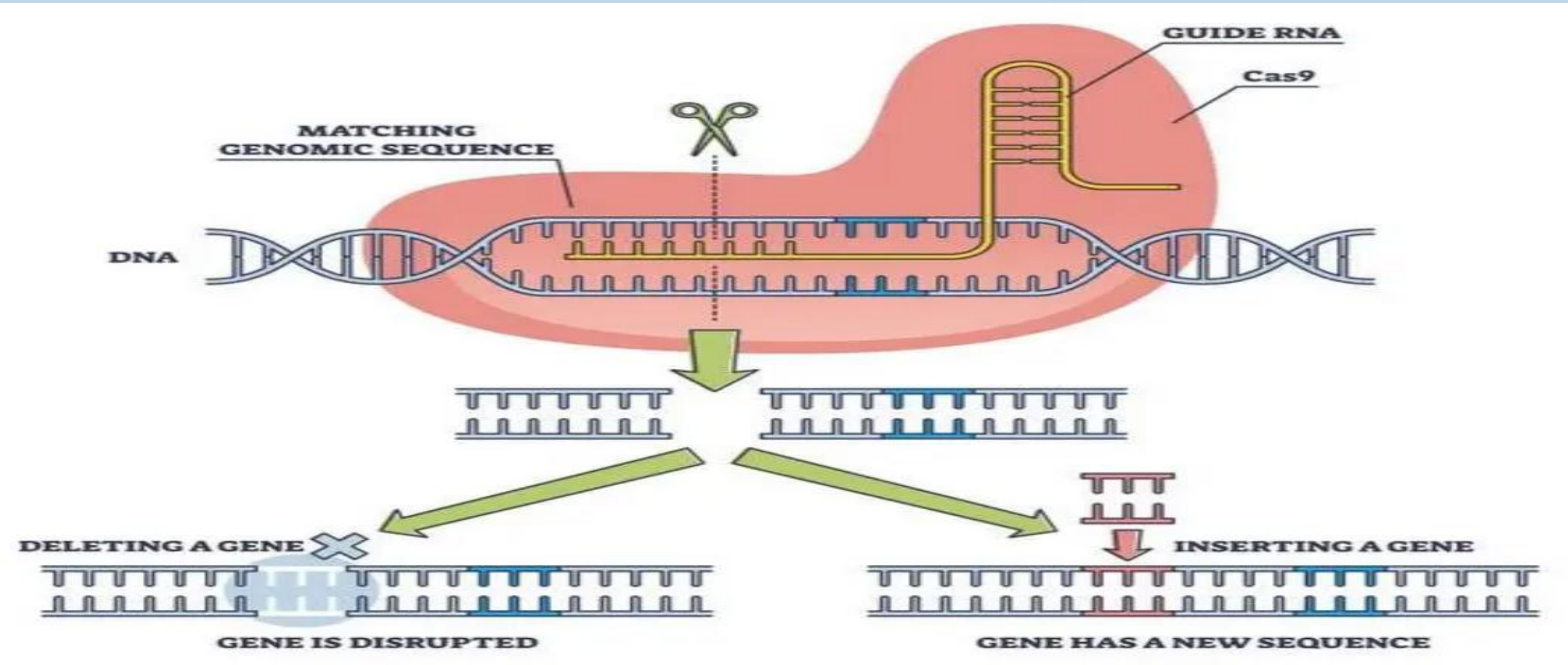
Strategy, E. (2024). *Equality, Diversity and Inclusion Strategy*. <https://centralsouthgenomics.nhs.uk/wp-content/uploads/2025/03/Central-and-South-EDI-Strategy.pdf>

Rationale of study

Sickle Cell Disease (SCD) disproportionately affects ethnically marginalised populations in the UK who already experience health inequalities. Although CRISPR gene therapy offers a potential cure (Subica, 2023), its high cost, specialised infrastructure requirements, and reliance on advanced genomic services risk widening disparities in access. Despite NHS commitments to advancing genomic medicine and reducing ethnic health inequalities (Strategy, 2024), there is limited evidence on system readiness to deliver CRISPR equitably. This study addresses this gap by evaluating health-system preparedness and identifying barriers to ensure fair and sustainable implementation within the NHS.

Research Impact

- This research will inform equitable implementation of advanced genomic therapies the major impacts of this study are:
- Support NHS planning for specialist gene-therapy service expansion
 - Identify structural barriers affecting ethnic minority populations with SCD
 - NICE guidance and NHS commissioning policies governing equitable access criteria
 - Contribute to UK strategies on reducing genomic health inequalities



Aim and Objectives

- The main aim of his study is to critically evaluate the public health implications of introducing CRISPR-based gene therapy for sickle cell disease in the UK, with emphasis on health system readiness, equitable access, and potential disparities in implementation.
- 1.To critically analyse the current policy, regulatory, and commissioning framework governing CRISPR-based gene therapy for sickle cell disease within the UK.
 - 2.To assess potential barriers and facilitators to equitable access, including socioeconomic status, ethnicity, geographic variation, and service capacity within NHS trusts.
 - 3.To evaluate health system readiness, including infrastructure requirements, funding mechanisms, and long-term monitoring capacity.
 - 4.To explore public health and ethical considerations, including distributive justice, community engagement, and implications for reducing or exacerbating health inequalities.

Research question

How prepared is the UK health system to equitably implement CRISPR-based gene therapy for sickle cell disease, and what public health factors influence access and equity?

