

Disease

Sickle cell anemia^{1, 2, 3}

Gene

HBB

Inheritance	AR
Current Treatments	glutamine, voxelotor, crizanlizumab, hydroxyurea, ,red cell transfusions, bone marrow transplantation (Hematopoietic Stem Cell Transplantation (HSCT)), gene therapy (BCH-BB694 BCL11A shmiR lentiviral vector - clinical trial and autologous CRISPR-Cas9-edited CD34+ hematopoietic stem and progenitor cells) - clinical trial)
Evidence for Treatment	guideline ⁴
Non-Molecular Testing Availability	yes
Non-Molecular Test	complete blood count, qualitative and quantitative hemoglobin analysis ¹
Disease Frequency (per 100,000)	28 ⁵
Genes Included in Disease Frequency	HBB
Experimental / Investigational Therapies	https://clinicaltrials.gov/ct2/results?cond=Sickle+Cell+Disease&term=&cntry=&state=&city=&dist=

References

- ¹ <https://www.ncbi.nlm.nih.gov/books/NBK1377/>
- ² <https://pubmed.ncbi.nlm.nih.gov/33283990/>
- ³ <https://pubmed.ncbi.nlm.nih.gov/33283989/>
- ⁴ <https://www.cdc.gov/ncbddd/sicklecell/recommendations.html>
- ⁵ <https://www.ncbi.nlm.nih.gov/pubmed/10192451>