PUBLICATIONS LIST

The Cure Sickle Cell Initiative is a collaborative, patient-focused research effort dedicated to accelerating the development of treatments aimed at genetic-based cures. Funded by the National Heart, Lung, and Blood Institute (NHLBI), the Initiative complements NHLBI's investment in sickle cell disease research by helping to fill gaps that cannot be covered by traditional funding methods. We bring together the sickle cell disease (SCD) community—patients, advocates, caregivers, providers, researchers, industry, etc.—and consider non traditional ways to advance research. The following is a list of publications produced since the Initiative started in 2018.

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CELL COLLECTION AND CONDITIONING

Challenges and Limitations of Mobilization and Stem Cell Collection for Gene Therapy of Sickle Cell Disease

15 December 2025

Tanhehco, Y. C., Thibodeaux, S., Shi, P. A., et al. (2025). Challenges and limitations of mobilization and stem cell collection for gene therapy of sickle cell disease. Blood Adv, 9(24), 6524-6533. https://doi.org/10.1182/bloodadvances.2025016054

5-Azacytidine Depletes HSCs and Synergizes with an Anti-CD117 Antibody to Augment Donor Engraftment in Immunocompetent Mice

12 October 2021

Bankova, A. K., Pang, W. W., Velasco, B. J., et al. (2021). 5-Azacytidine depletes HSCs and synergizes with an anti-CD117 antibody to augment donor engraftment in immunocompetent mice. Blood Adv, 5(19), 3900-3912. https://doi.org/10.1182/bloodadvances.2020003841

Parameters Affecting Successful Stem Cell Collections for Genetic Therapies in Sickle Cell Disease

10 January 2021

Justus, D. G. and Manis, J. P. (2021). Parameters affecting successful stem cell collections for genetic therapies in sickle cell disease. Transfus Apher Sci, 60(1), 103059. https://doi.org/10.1016/j.transci.2021.103059

CLONAL HEMOATOPOIESIS

Characteristics of Donor and Recipient Clonal Hematopoiesis in Nonmyeloablative Transplant for Sickly Cell Disease

29 July 2029

Weeks LD, Neuberg DS, Limerick EM, Hsieh MM, Xu X, Kabore MD, Lawal RA, Leonard A, Wang Y, Phang O, Brooks J, Ali MAE, Lindsley RC, Fitzhugh CD. (2026). Characteristics of donor and recipient clonal hematopoiesis in nonmyeloablative transplant for sickle cell disease. Blood. https://doi.org/10.1182/blood.2026033476

COST ANALYSIS

Financial Impacts of Paying for Gene Therapy for Sickle Cell Disease Under Alternative Pricing and Financing Mechanisms

10 November 2024

Basu, A. (2024). Financial impacts of paying for gene therapy for sickle cell disease under alternative pricing and financing mechanisms. Value Health, 28(2), 233-240. https://doi.org/10.1016/j.jval.2024.10.3848

Decreasing Alloimmunization-Specific Mortality in Sickle Cell Disease in the United States: Cost-Effectiveness of a Shared Transfusion Resource

26 January 2024

Ito, S., Pandya, A., Hauser, R. G., et al. (2024). Decreasing alloimmunization-specific mortality in sickle cell disease in the United States: cost-effectiveness of a shared transfusion resource. Am J Hematol, 99(4), 570-576. https://doi.org/10.1002/ajh.27211

Gene Therapy versus Common Care for Eligible Individuals with Sickle Cell Disease in the United States: A Cost-Effectiveness Analysis

23 January 2024

Basu, A., Winn, A. N., Johnson, K. M., et al. (2024). Gene therapy versus common care for eligible individuals with sickle cell disease in the United States: a cost-effectiveness analysis. Ann Intern Med, 177(2), 155-164. https://doi.org/10.7326/M23-1520

Associating Health-Related Quality-of-Life Score with Time Uses to Inform Productivity Measures in Cost-Effectiveness Analysis

06 March 2023

Jiao, B., and Basu, A. (2023). Associating health-related quality-of-life score with time uses to inform productivity measures in cost-effectiveness analysis. Pharmacoeconomics, 41(9), 1065-1077. https://doi.org/10.1007/s40273-023-01246-x

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Long-Term Survival with Sickle Cell Disease: A Nationwide Cohort Study of Medicare and Medicaid Beneficiaries

03 July 2023

Jiao, B., Johnson, K. M., Ramsey, S. D., et al. (2023). Long-term survival with sickle cell disease: A nationwide cohort study of Medicare and Medicaid beneficiaries. Blood Adv. https://doi.org/10.1182/bloodadvances.2022009202

A Framework for a Health Economic Evaluation Model for Patients with Sickle Cell Disease to Estimate the Value of New Treatments in the United States of America

11 February 2023

Winn, A., Basu, A., and Ramsey, S. D. (2023). A framework for a health economic evaluation model for patients with sickle cell disease to estimate the value of new treatments in the United States of America. Pharmacoecon Open, 7(2), 313–320. https://doi.org/10.1007/s41669-023-00390-6

Lifetime Medical Costs Attributable to Sickle Cell Disease Among Nonelderly Individuals with Commercial Insurance

27 January 2023

Johnson, K., Jiao, B., Ramsey, S. D., et al. (2023). Lifetime medical costs attributable to sickle cell disease among nonelderly individuals with commercial insurance. Blood Adv, 7(3), 365–374. https://doi.org/10.1182/bloodadvances.2021006281

Prevalence of Comorbidities Associated with Sickle Cell Disease among Non-elderly Individuals with Commercial Insurance – A Retrospective Cohort Study

29 November 2022

Ramsey, S. D., Bender, M. A., Li, L., et al. (2022). Prevalence of comorbidities associated with sickle cell disease among non-elderly individuals with commercial insurance-A retrospective cohort study. PLoS One, 17(11), e0278137. https://doi.org/10.1371/journal.pone.0278137

Application of Validated Mapping Algorithms between Generic PedsQL Scores and Utility Values to Individuals with Sickle Cell Disease

17 June 2022

Jiao, B., Hankins, J. S., Devine, B., et al. (2022). Application of validated mapping algorithms between generic PedsQL scores and utility values to individuals with sickle cell disease. Quality of life research: an international journal of quality of life aspects of treatment, care and rehabilitation, 31(9), 2729–2738. https://doi.org/10.1007/s11136-022-03167-2

Development of a Conceptual Model for Evaluating New Non-Curative and Curative Therapies for Sickle Cell Disease

28 April 2022

Johnson, K., Jiao, B., Bender, M., et al. (2022). Development of a conceptual model for evaluating new non-curative and curative therapies for sickle cell disease. PLoS One, 17(4), e0267448. https://doi.org/10.1371/journal.pone.0267448

Medical and Non-medical Costs of Sickle Cell Disease and Treatments from a US Perspective: A Systematic Review and Landscape Analysis

26 April 2022

Baldwin, Z., Jiao, B., Basu, A., et al. (2022). Medical and non-medical costs of sickle cell disease and treatments from a US perspective: A systematic review and landscape analysis. Pharmacoecon Open, 6(4), 469–481. https://doi.org/10.1007/s41669-022-00330-w

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A Landscape Analysis and Discussion of Value of Gene Therapies for Sickle Cell Disease

18 April 2022

Quach, D., Jiao, B., Basu, A., et al. (2022). A landscape analysis and discussion of value of gene therapies for sickle cell disease. Expert Rev Pharmacoecon Outcomes Res, 22(6), 891–911. https://doi.org/10.1080/14737167.2022.2060823

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February 2022

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The Use of Cost-Effectiveness Analysis in Sickle Cell Disease: A Critical Review of the Literature

09 August 2021

Jiao, B., Basu, A., Roth, J. et al. The use of cost-effectiveness analysis in sickle cell disease: A critical review of the literature. Pharmacoecon, 39, 1225–1241 (2021). https://doi.org/10.1007/s40273-021-01072-z

Gaps in Medicaid Coverage and Financial Toxicity: A Health Disparity in Hematopoietic Cell Transplantation for Sickle Cell Disease

01 April 2021

Krishnamurti, L. (2021). Gaps in Medicaid coverage and financial toxicity: a health disparity in hematopoietic cell transplantation for sickle cell disease. Transplant and Cell Ther, 27(4), 284-285. https://doi.org/10.1016/j.jtct.2021.03.016

DATA RESOURCES

Cure Sickle Cell Initiative Recommendations on Common Data Elements for Sickle Cell Disease Gene Therapy Trials.

10 April 2026

Lanzkron, S., Coleman_Cowger, V. H., Thompson, A. A., et al. (2026). Cure Sickle Cell Initiative recommendations on common data elements for sickle cell disease gene therapy trials. Blood Adv, bloodadvances-2025019400. https://doi.org/10.1182/bloodadvances.2025019400

CureSCi Metadata Catalog—Finding and Harmonizing Studies for Secondary Analysis of Hydroxyurea Discontinuation for Sickle Cell Disease

23 April 2025

Wu, X., Stratford, J., Kesler, K., et al. (2025). CureSCi Metadata Catalog— Finding and harmonizing studies for secondary analysis of hydroxyurea discontinuation in sickle cell disease. PLoS One, 20(4), e0309572. https://doi.org/10.1101/2024.08.15.608203

Creating an Automated Contemporaneous Cohort in Sickle Cell Anemia to Predict Survival After Disease-Modifying Therapy

26 July 2023

Cronin, R. M., Wuichet, K., Ghafuri, D. L., et al. (2023). Creating an automated contemporaneous cohort in sickle cell anemia to predict survival after disease-modifying therapy. Blood Adv, 7(15), 3775-3782. https://doi.org/10.1182/bloodadvances.2022008692

CureSCi Metadata Catalog –Making Sickle Cell Studies Findable

12 December 2022

Pan, H., Ives, C., Mandal, M., et al. (2022). CureSCi Metadata Catalog-Making sickle cell studies findable. PLoS One, 17(12), e0256248. https://doi.org/10.1371/journal.pone.0256248

GENE EDITING AND GENE THERAPY

Development of CRISPR_SCD001, an Autologous Hematopoietic Stem Cell Gene Therapy for Sickle Cell Disease after CRISPR-Cas9 Mediated Correction

19 September 2026

DeWitt, M., Campo-Fernandez, B., Roy, S., et al. (2026). Development of CRISPR_SCD001, an autologous hematopoietic stem cell gene therapy for sickle cell disease. Mol Ther, S1525-0016(26)00790-2. https://doi.org/10.1016/j.ymthe.2026.09.020

Long-term stability of posttranscriptional genetic silencing of BCL11A using a shmiR vector in sickle cell disease

20 August 2026

Esrick, E. B., Lehmann, L., Federico, A., Vincon, H., Liu, B., et al. (2026). Long-term stability of posttranscriptional genetic silencing of BCL11A using a shmiR vector in sickle cell disease. Blood, 148(8), 957-966. https://doi.org/10.1182/blood.2026033871

Gene Editing without Ex Vivo Culture Evades Genotoxicity in Human Hematopoietic Stem Cells

06 February 2025

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Treatment with Curative Intent: The Emergence of Genetic Therapies for Sickle Cell Anemia

14 March 2024

Benz Jr, E. J., Silberstein, L. E., and Panepinto, J. (2024). "Treatment with curative intent": the emergence of genetic therapies for sickle cell anemia. Blood, 143(11), 967-970. https://doi.org/10.1182/blood.2023021598

Clonal Selection of Hematopoietic Stem Cells after Gene Therapy for Sickle Cell Disease

16 November 2023

Spencer Chapman, M., Cull, A. H., Ciuculescu, M. F., et al. (2023). Clonal selection of hematopoietic stem cells after gene therapy for sickle cell disease. Nat Med, 29(12), 3175-3183. https://doi.org/10.1038/s41591-023-02636-6

Molecular Basis and Genetic Modifiers of Thalassemia

10 March 2023

Tesio, N. and Bauer, D. E. (2023). Molecular basis and genetic modifiers of thalassemia. Hematol Oncol Clin North Am, 37(2), 273-299. https://doi.org/10.1016/j.hoc.2022.12.001

Base Editing of Human Hematopoietic Stem Cells

03 January 2023

Zeng, J., Casirati, G., Nguyen, M. A., et al. (2023). Base editing of human hematopoietic stem cells. In Base Editors: Methods and Protocols (pp. 43-62). New York, NY: Springer US. https://doi.org/10.1007/978-1-0716-2879-9_5

Development of a Double shmiR Lentivirus Effectively Targeting Both BCL11A and ZNF410 for Enhanced Induction of Fetal Hemoglobin to Treat β-Hemoglobinopathies

03 August 2022

Liu, B., Brendel, C., Vinjamur, D. S., et al. (2022). Development of a double shmiR lentivirus effectively targeting both BCL11A and ZNF410 for enhanced induction of fetal hemoglobin to treat β-hemoglobinopathies. Mol Ther, 30(8), 2693-2708. https://doi.org/10.1016/j.ymthe.2022.05.002

Optimization of Nuclear Localization Signal Composition Improves CRISPR-Cas12a Editing Rates in Human Primary Cells

14 June 2022

Luk, K., Liu, P., Zeng, J., et al. (2022). Optimization of nuclear localization signal composition improves CRISPR-Cas12a editing rates in human primary cells. GEN Biotechnol, 1(3), 271-284. https://doi.org/10.1089/genbio.2022.0003

Editing Outside the Body: Ex Vivo Gene-Modification for Beta-Hemoglobinopathy Cellular Therapy

03 November 2021

Rosanwo, T.O. and Bauer, D.E. (2021). Editing outside the body: Ex vivo gene-modification for beta-hemoglobinopathy cellular therapy. Mol Ther, 29(11), 3163-3178. https://doi.org/10.1016/j.ymthe.2021.10.002

Update on the Cure Sickle Cell Initiative

5 February 2021

Silberstein, L. E., and Telen, M. J. (2021). Update on the Cure Sickle Cell Initiative. The Hematologist, 18(2).

CRISPR/Cas9 Gene Editing for Curing Sickle Cell Disease

01 February 2021

Park, S.H. and Bao, G. (2021). CRISPR/Cas9 gene editing for curing sickle cell disease. Transfus Apher Sci, 60(1), 103060. https://doi.org/10.1016/j.transci.2021.103060

Magnetic Forces Enable Control of Biological Processes In Vivo

12 January 2021

Bao, G. (2021). Magnetic forces enable control of biological processes in vivo. J Appl Mech, 88(3), 030801. https://doi: 10.1115/1.4049331

Accelerating the Science of SCD Therapies—Is a Cure Possible?

08 August 2019

Benz, E. J., Mondoro, T. H., and Gibbons, G. H. (2019). Accelerating the science of SCD therapies—Is a cure possible? JAMA, 322(10), 921. https://doi.org/10.1001/jama.2019.11419

HEMATOPOIETIC CELL TRANSPLANT (HCT)

Long-Term Quality of Life after Hematopoietic Cell Transplant for Sickle Cell Disease in Childhood: A STELLAR Interim Analysis

06 August 2024

Arnold, S. D., Bakshi, N., Ross, D., et al. (2024). Long term quality of life after hematopoietic cell transplant for sickle cell disease in childhood: A STELLAR interim analysis. Am J Hematol, 99(10), 2037–2040. https://doi.org/10.1002/ajh.27436

Secondary Neoplasms After Hematopoietic Cell Transplant for Sickle Cell Disease

9 January 2023

Eapen, M., Brazauskas, R., Williams, D. A., et al. (2023). Secondary neoplasms after hematopoietic cell transplant for sickle cell disease. J Clin Oncol, 41(12), 2227–2237. https://doi.org/10.1200/JCO.22.01203

Sickle Cell Transplantation Evaluation of Long-term and Late Effects Registry (STELLAR) to Compare Long-term Outcomes After Hematopoietic Cell Transplantation to Those in Siblings Without Sickle Cell Disease and in Nontransplanted Individuals with Sickle Cell Disease: Design and Feasibility Study

06 July 2022

Krishnamurti, L., Arnold, S. D., Haight, A., et al. (2022). Sickle cell transplantation evaluation of long-term and late effects registry (STELLAR) to compare long-term outcomes after hematopoietic cell transplantation to those in siblings without sickle cell disease and in nontransplanted individuals with sickle cell disease: design and feasibility study. JMIR Res Protoc, 11(7), e36780. https://doi.org/10.2196/36780

Long-term Survival after Hematopoietic Cell Transplant for Sickle Cell Disease Compared to the United States Population

15 March 2022

St Martin, A., Hebert, K. M., Serret-Larmande, A., et al. (2022). Long-term survival after hematopoietic cell transplant for sickle cell disease compared to the United States population. Transplant Cell Ther, 28(6), 325.e1–325.e7. https://doi.org/10.1016/j.jtct.2022.03.014

A Decision Support Tool for Allogeneic Hematopoietic Stem Cell Transplantation for Children With Sickle Cell Disease: Acceptability and Usability Study

28 October 2021

Veludhandi, A., Ross, D., Sinha, C. B., et al. (2021). A decision support tool for allogeneic hematopoietic stem cell transplantation for children with sickle cell disease: Acceptability and usability study. JMIR Form Res, 5(10), e30093. https://doi.org/10.2196/30093

Hematopoietic Cell Transplantation for Sickle Cell Disease

04 January 2021

Krishnamurti, L. (2021). Hematopoietic cell transplantation for sickle cell disease. Front Pediatr, 8, 551170. https://doi.org/10.3389/fped.2020.551170

MENTAL HEALTH

Assessing Psychosocial Risk and Resilience to Support Readiness for Gene Therapy in Sickle Cell Disease: A Consensus Statement

21 August 2024

Hardy, S. J., Crosby, L. E., Porter, J. S., et al. (2024). Assessing psychosocial risk and resilience to support readiness for gene therapy in sickle cell disease: A consensus statement. JAMA Netw Open, 7(8), e2429443-e2429443. https://doi.org/10.1001/jamanetworkopen.2024.29443

Mental Health Challenges Associated with Sickle Cell Disease and Strategies to Address Them: Reflections from a Community Input Panel

25 March 2026

Adeyokunnu, A., Avant, H., Hardy, S., et al. (2026). Mental health challenges associated with sickle cell disease and strategies to address them: Reflections from a community input panel. J Natl Med Assoc, 118(2), 178-182. https://doi.org/10.1016/j.jnma.2025.12.002

MICROFLUIDICS

Catch Bonds in Sickle Cell Disease: Shear-Enhanced Adhesion of Red Blood Cells to Laminin

20 June 2023

Goreke, U., Iram, S., Singh, G., et al. (2023). Catch bonds in sickle cell disease: shear-enhanced adhesion of red blood cells to laminin. Biophys J, 122(12), 2564-2576. https://doi.org/10.1016/j.bpj.2023.05.010

Membrane Bending and Sphingomyelinase-Associated, Sulfatide-Dependent Hypoxic Adhesion of Sickle Mature Erythrocytes

11 May 2023

Goreke, U., Kucukal, E., Wang, F., et al. (2023). Membrane bending and sphingomyelinase-associated, sulfatide-dependent hypoxic adhesion of sickle mature erythrocytes. Blood Adv, 7(10), 2094-2104. https://doi.org/10.1182/bloodadvances.2022008392

A Microfluidic Device for Assessment of E-Selectin-Mediated Neutrophil Recruitment to Inflamed Endothelium and Prediction of Therapeutic Response in Sickle Cell Disease

15 February 2023

Man, Y., Kucukal, E., Liu, S., et al. (2023). A microfluidic device for assessment of E-selectin-mediated neutrophil recruitment to inflamed endothelium and prediction of therapeutic response in sickle cell disease. Biosens Bioelectron, 222, 114921. https://doi.org/10.1016/j.bios.2022.114921

Emerging Functional Microfluidic Assays for the Study of Thromboinflammation in Sickle Cell Disease

01 November 2022

An, R., and Gurkan, U. A. (2022). Emerging functional microfluidic assays for the study of thromboinflammation in sickle cell disease. Curr Opin Hematol, 29(6), 327-334. https://doi.org/10.1097/MOH.0000000000000731

Antithrombin-III Mitigates Thrombin-Mediated Endothelial Cell Contraction and Sickle Red Blood Cell Adhesion in Microscale Flow

13 July 2022

Wulftange, W. J., Kucukal, E., Man, Y., et al. (2022). Antithrombin–III mitigates thrombin-mediated endothelial cell contraction and sickle red blood cell adhesion in microscale flow. Br J Haematol, 198(5), 893-902. https://doi.org/10.1111/bjh.18328

Size and Density Measurements of Single Sickle Red Blood Cells Using Microfluidic Magnetic Levitation

15 February 2022

Goreke, U., Bode, A., Yaman, S., et al. (2022). Size and density measurements of single sickle red blood cells using microfluidic magnetic levitation. Lab Chip, 22(4), 683–696. https://doi.org/10.1039/d1lc00686j

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Integrating Deep Learning with Microfluidics for Biophysical Classification of Sickle Red Blood Cells Adhered to Laminin

29 November 2021

Praljak, N., Iram, S., Goreke, U., et al. (2021). Integrating deep learning with microfluidics for biophysical classification of sickle red blood cells adhered to laminin. PLoS Comput Biol, 17(11), e1008946. https://doi.org/10.1371/journal.pcbi.1008946

Microfluidic Assessment of Red Blood Cell Mediated Microvascular Occlusion

19 May 2021

Man, Y., Kucukal, E., An, R., et al. (2020). Microfluidic assessment of red blood cell mediated microvascular occlusion. Lab Chip, 20(12), 2086–2099. https://doi.org/10.1039/dolc00112k

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Biophysical and Rheological Biomarkers of Red Blood Cell Physiology and Pathophysiology

01 May 2021

Gurkan U. A. (2021). Biophysical and rheological biomarkers of red blood cell physiology and pathophysiology. Curr Opin Hematol, 28(3), 138–149. https://doi.org/10.1097/MOH.0000000000000639

Concurrent Assessment of Deformability and Adhesiveness of Sickle Red Blood Cells by Measuring Perfusion of an Adhesive Artificial Microvascular Network

28 April 2021

Lu, M., Kanne, C. K., Reddington, R. C., et al. (2021). Concurrent assessment of deformability and adhesiveness of sickle red blood cells by measuring perfusion of an adhesive artificial microvascular network. Front Physiol, 12. https://doi.org/10.3389/fphys.2021.633080

Microfluidic Electrical Impedance Assessment of Red Blood Cell-Mediated Microvascular Occlusion

05 March 2021

Man, Y., Maji, D., An, R., et al. (2021). Microfluidic electrical impedance assessment of red blood cell-mediated microvascular occlusion. Lab Chip, 21(6), 1036–1048. https://doi.org/10.1039/dolc01133a

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Standardized Microfluidic Assessment of Red Blood Cell-Mediated Microcapillary Occlusion: Association with Clinical Phenotype and Hydroxyurea Responsiveness in Sickle Cell Disease

09 January 2021

Man, Y., Kucukal, E., An, R., et al. (2021). Standardized microfluidic assessment of red blood cell–mediated microcapillary occlusion: Association with clinical phenotype and hydroxyurea responsiveness in sickle cell disease. Microcirculation, 28(2). https://doi.org/10.1111/micc.12662

Leukocyte Adhesion to P-Selectin and the Inhibitory Role of Crizanlizumab in Sickle Cell Disease: A Standardized Microfluidic Assessment

10 March 2020

Man, Y., Goreke, U., Kucukal, E., et al. (2020). Leukocyte adhesion to P-selectin and the inhibitory role of Crizanlizumab in sickle cell disease: A standardized microfluidic assessment. Blood Cells Mol Dis, 83, 102424. https://doi.org/10.1016/j.bcmd.2020.102424

POST-GENE THERAPY, SAFETY

Oligonucleotide Synthesis Errors Are a Source of Untoward Variation in HDR-Mediated Gene Editing

24 June 2026

Wyman, S.K., Romero, Z., Heo, S.-J., et al. (2026). Oligonucleotide synthesis errors are a source of untoward variation in HDR-mediated gene editing. Genes, 17, 729. https://doi.org/10.3390/genes17070729

Synthetic Oligonucleotides Contain Synthesis Errors That Are Propagated into the Genome by Editing Through Homology-directed Repair

03 November 2025

Wyman, S., Romero Garcia, Z., Heo, S.J., et al. (2025). Synthetic oligonucleotides contain synthesis errors that are propagated into the genome by editing through homology-directed repair. Blood, 146(1): 4321. https://doi.org/10.1182/blood-2025-4321

Human Genetic Diversity Alters Off-Target Outcomes of Therapeutic Gene Editing

15 December 2022

Cancellieri, S., Zeng, J., Lin, L. Y., et al. (2023). Human genetic diversity alters off-target outcomes of therapeutic gene editing. Nat Genet, 55(1), 34-43. https://doi.org/10.1038/s41588-022-01257-y

Comprehensive Analysis and Accurate Quantification of Unintended Large Gene Modifications Induced by CRISPR-Cas9 Gene Editing

21 October 2022

Park, S. H., Cao, M., Pan, Y., et al. (2022). Comprehensive analysis and accurate quantification of unintended large gene modifications induced by CRISPR-Cas9 gene editing. Sci Adv, 8(42), eabo7676. https://doi.org/10.1126/sciadv.abo7676

Identification and Validation of CRISPR/Cas9 Off-Target Activity in Hematopoietic Stem and Progenitor Cells

05 May 2022

Park, S. H., Lee, C. M., and Bao, G. (2022). Identification and validation of CRISPR/Cas9 off-target activity in hematopoietic stem and progenitor cells. In Stem Cell Assays: Methods and Protocols (pp. 281-306). New York, NY: Springer US. https://doi.org/10.1007/978-1-0716-1979-7_19

Tools for Experimental and Computational Analyses of Off-Target Editing by Programmable Nucleases

07 December 2020

Bao, X. R., Pan, Y., Lee, C. M., et al. (2021). Tools for experimental and computational analyses of off-target editing by programmable nucleases. Nat Protoc, 16(1), 10–26. https://doi.org/10.1038/s41596-020-00431-y

REPRODUCTIVE HEALTH

Reproductive Health Assessment and Reports of Fertility Counseling in Pediatric and Adolescent Patients with Sickle Cell Disease After Hematopoietic Cell Transplantation

05 July 2024

George, S. A., Veludhandi, A., Xiang, Y., et al. (2024). Reproductive health assessment and reports of fertility counseling in pediatric and adolescent patients with sickle cell disease after hematopoietic cell transplantation. Transplant Cell Ther, 30(9), 912-e1. https://doi.org/10.1016/j.jtct.2024.06.029

Female Reproductive Health Outcomes After Hematopoietic Cell Transplantation for Sickle Cell Disease: Is Reduced Intensity Better Than Myeloablative Conditioning?

09 May 2023

Meacham, L. R., George, S., Veludhandi, A., et al. (2023). Female reproductive health outcomes after hematopoietic cell transplantation for sickle cell disease: is reduced intensity better than myeloablative conditioning? Transplant Cell Ther, 29(8), 531-e1. https://doi.org/10.1016/j.jtct.2023.05.004

Parental Perspective on the Risk of Infertility and Fertility Preservation Options for Children and Adolescents with Sickle Cell Disease Considering Hematopoietic Stem Cell Transplantation

13 April 2023

Sinha, C. B., Meacham, L. R., Bakshi, N., et al. (2023). Parental perspective on the risk of infertility and fertility preservation options for children and adolescents with sickle cell disease considering hematopoietic stem cell transplantation. Pediatr Blood Cancer, 70(7), e30276. https://doi.org/10.1002/pbc.30276

SCD COMMUNITY OUTREACH

Process and Strategies for Patient Engagement and Outreach in the Sickle Cell Disease (SCD) Community to Promote Clinical Trial Participation

26 April 2022

Byrnes, C., Botello-Harbaum, M., Clemons, T., et al. (2022). Process and strategies for patient engagement and outreach in the sickle cell disease (SCD) community to promote clinical trial participation. J Natl Med Assoc, 114(2), 211-217. https://doi.org/10.1016/j.jnma.2022.01.003

Towards Access for All: 1st Working Group Report for the Global Gene Therapy Initiative (GGTI)

08 September 2021

Adair, J. E., Androski, L., Bayigga, L., et al. (2023). Towards access for all: 1st working group report for the global gene therapy initiative (GGTI). Gene Therapy, 30(3), 216-221. https://doi.org/10.1038/s41434-021-00284-4

SCD PATHOPHYSIOLOGY

Missing the Mark(ers): Circulating Endothelial Cells and Endothelial-Derived Microvesicles are Elevated in Sickle Cell Disease

23 December 2024

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