CURE SICKLE CELL.

National Heart, Lung, and Blood Institute

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

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., Long-Boyle, J. R., & Shizuru, J. A. (2021). 5-Azacytidine depletes HSCs and synergizes with an anti-CD117 antibody to augment donor engraftment in immunocompetent mice. Blood Advances, 5(19), 3900-3912. https://doi.org/10.1182/bloodadvances.2020003841

COST ANALYSIS

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., Krishnamurti, L., Stites, E., Tormey, C., ... & Goshua, G. (2024). Decreasing alloimmunization-specific mortality in sickle cell disease in the United States: cost-effectiveness of a shared transfusion resource. American Journal of Hematology, 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., Jiao, B., Devine, B., Hankins, J. S., ... & Ramsey, S. D. (2024). Gene therapy versus common care for eligible individuals with sickle cell disease in the United States: a cost-effectiveness analysis. Annals of Internal Medicine, 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., & 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., Bender, M. A., Devine, B., & Basu, A. (2023). Long-Term Survival with Sickle Cell Disease: A Nationwide Cohort Study of Medicare and Medicaid Beneficiaries. Blood Advances. https://doi.org/10.1182/bloodadvances.2022009202

A Framework for a Health Economic Evaluation Model for Patients with Sickle Cell Disease

11 February 2023

Winn, A., Basu, A., & 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. PharmacoEconomics - 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., Bender, M., Devine, B., & Basu, A. (2023). Lifetime medical costs attributable to sickle cell disease among nonelderly individuals with commercial insurance. Blood Advances, 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., Johnson, K. M., Jiao, B., Devine, B., & Basu, A. (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., Barton, M., Bender, M., & Basu, A. (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

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26 April 2022

Baldwin, Z., Jiao, B., Basu, A., Roth, J. A., Bender, M., Elsisi, Z., Johnson, K., Cousin, E., Ramsey, S. D., & Devine, B. (2022). Medical and Non-medical Costs of Sickle Cell Disease and Treatments from a US Perspective: A Systematic Review and Landscape Analysis. PharmacoEconomics - 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., Bender, M. A., Hankins, J., Ramsey, S., & Devine, B. (2022). A landscape analysis and discussion of value of gene therapies for sickle cell disease. Expert review of pharmacoeconomics & outcomes research, 22(6), 891–911. https://doi.org/10.1080/14737167.2022.2060823

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

09 August 2021

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

01 April 2021

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DATA RESOURCES

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., Ives, C., Hendershot, T., Kroner, B., ... & Pan, H. (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., Hodges, B., Chopra, M., He, J., ... & DeBaun, M. R. (2023). Creating an automated contemporaneous cohort in sickle cell anemia to predict survival after disease-modifying therapy. Blood Advances, 7(15), 3775-3782. https://doi.org/10.1182/bloodadvances.2022008692

CureSCi Metadata Catalog –Making Sickle Cell Studies Findable

12 December 2022

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Understanding Sickle Cell Disease: Impact of Surveillance and Gaps in Knowledge

06 February 2020

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GENE EDITING AND GENE THERAPY

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

06 February 2025

Zeng, J., Nguyen, M. A., Liu, P., Da Silva, L. F., Levesque, S., Lin, L. Y., ... & Bauer, D. E. (2025). Gene editing without ex vivo culture evades genotoxicity in human hematopoietic stem cells. Cell Stem Cell, 32(2), 191-208. https://doi.org/10.1016/j.stem.2024.11.001

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

14 March 2024

Benz Jr, E. J., Silberstein, L. E., & 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

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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

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

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

03 November 2021

Rosanwo, T.O. and D.E. Bauer, Editing outside the body: Ex vivo gene-modification for beta-hemoglobinopathy cellular therapy. Mol Ther, 2021. 29(11): p. 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., & 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

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

08 August 2019

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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., Smith, C., Sinha, C., Veludhandi, A., Dutreuil, V., Bai, S., Meacham, L. R., Guilcher, G., Bhatia, M., Abraham, A., Kasow, K. A., Haight, A., El Rassi, F., Stenger, E., Lipscomb, J., & Krishnamurti, L. (2024). Long term quality of life after hematopoietic cell transplant for sickle cell disease in childhood: A STELLAR interim analysis. American journal of hematology, 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., Walters, M. C., St Martin, A., Jacobs, B. L., Antin, J. H., Bona, K., Chaudhury, S., Coleman-Cowger, V. H., DiFronzo, N. L., Esrick, E. B., Field, J. J., Fitzhugh, C. D., Kanter, J., Kapoor, N., Kohn, D. B., Krishnamurti, L., London, W. B., Pulsipher, M. A., ... Horowitz, M. M. (2023). Secondary Neoplasms After Hematopoietic Cell Transplant for Sickle Cell Disease. Journal of clinical oncology : official journal of the American Society of Clinical Oncology, 41(12), 2227–2237. https://doi.org/10.1200/JCO.22.01203

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06 July 2022

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15 March 2022

St Martin, A., Hebert, K. M., Serret-Larmande, A., Jouhet, V., Hughes, E., Stedman, J., DeSain, T., Pillion, D., Lyons, J. C., Steinert, P., Avillach, P., & Eapen, M. (2022). Long-term Survival after Hematopoietic Cell Transplant for Sickle Cell Disease Compared to the United States Population. Transplantation and cellular therapy, 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

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04 January 2021

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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., Sil, S., Valrie, C. R., Jonassaint, C. R., ... & Coleman-Cowger, V. H. (2024). Assessing Psychosocial Risk and Resilience to Support Readiness for Gene Therapy in Sickle Cell Disease: A Consensus Statement. JAMA network open, 7(8), e2429443-e2429443. https://doi.org/10.1001/jamanetworkopen.2024.29443

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., Domínguez-Medina, S., Man, Y., Bode, A., ... & Gurkan, U. A. (2023). Catch bonds in sickle cell disease: shear-enhanced adhesion of red blood cells to laminin. Biophysical Journal, 122(12), 2564-2576.

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11 May 2023

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15 February 2023

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01 November 2022

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13 July 2022

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15 February 2022

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29 November 2021

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Microfluidic Assessment of Red Blood Cell Mediated Microvascular Occlusion

19 May 2021

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Biophysical and Rheological Biomarkers of Red Blood Cell Physiology and Pathophysiology

01 May 2021

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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., Lezzar, D., Sheehan, V. A., & Shevkoplyas, S. S. (2021). Concurrent Assessment of Deformability and Adhesiveness of Sickle Red Blood Cells by Measuring Perfusion of an Adhesive Artificial Microvascular Network. Frontiers in Physiology, 12. https://doi.org/10.3389/fphys.2021.633080

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05 March 2021

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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

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

10 March 2020

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POST-GENE THERAPY, SAFETY

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

15 December 2022

Cancellieri, S., Zeng, J., Lin, L. Y., Tognon, M., Nguyen, M. A., Lin, J., ... & Pinello, L. (2023). Human genetic diversity alters off-target outcomes of therapeutic gene editing. Nature Genetics, 55(1), 34-43. https://doi.org/10.1038/s41588-022-01257-y

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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., Davis, T. H., Saxena, L., Deshmukh, H., ... & Bao, G. (2022). Comprehensive analysis and accurate quantification of unintended large gene modifications induced by CRISPR-Cas9 gene editing. Science Advances, 8(42), eabo7676. DOI: 10.1126/sciadv.abo767

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05 May 2022

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

07 December 2020

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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., Liu, K., Stenger, E., Arnold, S. D., ... & Meacham, L. R. (2024). Reproductive Health Assessment and Reports of Fertility Counseling in Pediatric and Adolescent Patients with Sickle Cell Disease After Hematopoietic Cell Transplantation. Transplantation and cellular therapy, 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., Pruett, M. C., Haight, A. E., Arnold, S. D., ... & Krishnamurti, L. (2023). Female reproductive health outcomes after hematopoietic cell transplantation for sickle cell disease: is reduced intensity better than myeloablative conditioning?. Transplantation and Cellular Therapy, 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., Ross, D., & Krishnamurti, L. (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. Pediatric 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., Bailey, L., Valdes, K. M., & Coleman-Cowger, V. H. (2022). Process and strategies for patient engagement and outreach in the Sickle Cell Disease (SCD) community to promote clinical trial participation. Journal of the National Medical Association, 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., Bazira, D., Brandon, E., Dee, L., ... & Dropulić, B. (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

Beckman JD, Zhang P, Nguyen J, Hebbel RP, Vercellotti GM and Belcher JD (2024) Missing the mark(ers): circulating endothelial cells and endothelial-derived extracellular vesicles are elevated in sickle cell disease plasma. Front. Immunol. 15:1493904.

Genetic Reversal of the Globin Switch Concurrently Modulates Both Fetal and Sickle Hemoglobin and Reduces Red Cell Sickling.

20 September 2023

De Souza, D. C., Hebert, N., Esrick, E. B., Ciuculescu, M. F., Archer, N. M., Armant, M., ... & Higgins, J. M. (2023). Genetic reversal of the globin switch concurrently modulates both fetal and sickle hemoglobin and reduces red cell sickling. Nature communications, 14(1), 5850. https://doi.org/10.1038/s41467-023-40923-5

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