

# 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.

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

<https://doi.org/10.3389/fimmu.2024.1493904>

**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.

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

<https://doi.org/10.1001/jamanetworkopen.2024.29443>

**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.

### **CureSci Metadata Catalog—Finding and Harmonizing Studies for Secondary Analysis of Hydroxyurea Use for Sickle Cell Disease**

<https://doi.org/10.1101/2024.08.15.608203>

**19 August 2024 - PREPRINT**

Wu, X., Stratford, J., Kesler, K., Ives, C., Hendershot, T., Kroner, B., ... & Pan, H. (2024). CureSci Metadata Catalog—finding and harmonizing studies for secondary analysis of hydroxyurea use for sickle cell disease. *bioRxiv*.

## **“Treatment with Curative Intent”: The Emergence of Genetic Therapies for Sickle Cell Anemia**

<https://doi.org/10.1182/blood.2023021598>

**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.

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

<https://doi.org/10.7326/M23-1520>

**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.

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

<https://ashpublications.org/bloodadvances/article/doi/10.1182/bloodadvances.2022009202/494890/Long-Term-Survival-with-Sickle-Cell-Disease-A>

**16 March 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>

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

<https://pubmed.ncbi.nlm.nih.gov/36773220/>

**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>

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

<https://curesickle.org/system/files/bloodadvances.2021006281.pdf>

**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>

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## **Secondary Neoplasms After Hematopoietic Cell Transplant for Sickle Cell Disease**

<https://pubmed.ncbi.nlm.nih.gov/36623245/>

**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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## **CureSci Metadata Catalog –Making Sickle Cell Studies Findable**

<https://pubmed.ncbi.nlm.nih.gov/36508412/>

**12 December 2022**

Pan, H., Ives, C., Mandal, M., Qin, Y., Hendershot, T., Popovic, J., Brambilla, D., Stratford, J., Treadwell, M., Wu, X., & Kroner, B. (2022). CureSci Metadata Catalog-Making sickle cell studies findable. *PLoS one*, 17(12), e0256248. <https://doi.org/10.1371/journal.pone.0256248>

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

<https://pubmed.ncbi.nlm.nih.gov/36445914/>

**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>

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

<https://ashpublications.org/bloodadvances/article/doi/10.1182/bloodadvances.2022008692/487040/Creating-an-Automated-Contemporaneous-Cohort-in>

**10 November 2022**

Cronin, R. M., Wuichet, K., Ghafuri, D. L., Hodges, B., Chopra, M., He, J., Niu, X., Kassim, A., Wilkerson, K., Rodeghier, M., & DeBaun, M. R. (2022). Creating an Automated Contemporaneous Cohort in Sickle Cell Anemia to Predict Survival After Disease-Modifying Therapy. *Blood advances*, bloodadvances.2022008692. Advance online publication. <https://doi.org/10.1182/bloodadvances.2022008692>

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## **Application of validated mapping algorithms between generic PedsQL scores and utility values to individuals with sickle cell disease**

<https://pubmed.ncbi.nlm.nih.gov/35715626/>

**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>

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

[https://curesickle.org/system/files/Johnson.pone\\_.2022.pdf](https://curesickle.org/system/files/Johnson.pone_.2022.pdf)

**28 April 2022**

Johnson, K., Jiao, B., Bender, M., Ramsey, S. D., Devine, B., & Basu, A. (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>

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

[https://curesickle.org/system/files/Baldwin2022\\_Article\\_MedicalAndNon-medicalCostsOfSi\\_1.pdf](https://curesickle.org/system/files/Baldwin2022_Article_MedicalAndNon-medicalCostsOfSi_1.pdf)

**26 April 2022**

Baldwin, Z., Jiao, B., Basu, A., Roth, J. A., Bender, M., Elsis, 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**

<https://pubmed.ncbi.nlm.nih.gov/35363602/>

**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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## **Process and strategies for patient engagement and outreach in the Sickle Cell Disease (SCD) community to promote clinical trial participation**

<https://pubmed.ncbi.nlm.nih.gov/35093266/>

**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>

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

<https://pubmed.ncbi.nlm.nih.gov/35302009/>

**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>

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## **Size and density measurements of single sickle red blood cells using microfluidic magnetic levitation**

<https://pubmed.ncbi.nlm.nih.gov/35094036/>

**15 February 2022**

Goreke, U., Bode, A., Yaman, S., Gurkan, U. A., & Durmus, N. G. (2022). Size and density measurements of single sickle red blood cells using microfluidic magnetic levitation. *Lab on a chip*, 22(4), 683–696. <https://doi.org/10.1039/d1lc00686j>

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## **Health State Utilities for Sickle Cell Disease: A Catalog Prepared From a Systematic Review**

<https://curesickle.org/system/files/Jiao%20VALUE%20HEALTH.%202022%3B%2025%282%29276%E2%80%93287.pdf>

**February 2022**

Jiao, B., Basu, A., Ramsey, S. D., Roth, J. A., Bender, M., Quach, D., & Devine, B. (2022). Health State Utilities for Sickle Cell Disease: A Catalog Prepared From a Systematic Review. *Value in Health*. <https://doi.org/10.1016/j.jval.2021.08.002>

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

<https://ashpublications.org/bloodadvances/article/doi/10.1182/bloodadvances.2020003841/476701/5-Azacytidine-depletes-hematopoietic-stem-cells>

**12 October 2021**

Bankova, A. K., Pang, W. W., Velasco, B. J., Long-Boyle, J., & Shizuru, J. A. (2021). 5-Azacytidine depletes hematopoietic stem cells and synergizes with an anti-CD117 antibody to augment donor engraftment in immunocompetent mice. *Blood Advances*. <https://doi.org/10.1182/bloodadvances.2020003841>

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

[https://curesickle.org/system/files/Jiao2021\\_Article\\_TheUseOfCost-EffectivenessAnal.pdf](https://curesickle.org/system/files/Jiao2021_Article_TheUseOfCost-EffectivenessAnal.pdf)

**9 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. *PharmacoEconomics*, 39, 1225–1241 (2021). <https://doi.org/10.1007/s40273-021-01072-z>

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## **Microfluidic Assessment of Red Blood Cell Mediated Microvascular Occlusion**

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7473457/>

**19 May 2021**

Man, Y., Kucukal, E., An, R., Watson, Q. D., Bosch, J., Zimmerman, P. A., Little, J. A., & Gurkan, U. A. (2020). Microfluidic assessment of red blood cell mediated microvascular occlusion. *Lab on a Chip*, 20(12), 2086–2099. <https://doi.org/10.1039/d0lc00112k>

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## **Biophysical and Rheological Biomarkers of Red Blood Cell Physiology and Pathophysiology**

<https://pubmed.ncbi.nlm.nih.gov/33631785/>

**1 May 2021**

Gurkan, U. A. (2021). Biophysical and rheological biomarkers of red blood cell physiology and pathophysiology. *Current Opinion in Hematology*, 28(3), 138–149. <https://doi.org/10.1097/moh.0000000000000639>

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

<https://curesickle.org/system/files/fphys-Sergey%20%28002%29.pdf>

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

<https://pubmed.ncbi.nlm.nih.gov/33666615/>

**5 March 2021**

Man, Y., Maji, D., An, R., Ahuja, S. P., Little, J. A., Suster, M. A., Mohseni, P., & Gurkan, U. A. (2021). Microfluidic electrical impedance assessment of red blood cell-mediated microvascular occlusion. *Lab on a Chip*, 21(6), 1036–1048. <https://doi.org/10.1039/d0lc01133a>

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## **Update on the Cure Sickle Cell Initiative**

<https://ashpublications.org/thehematologist/article/475190/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).

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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**

<https://pubmed.ncbi.nlm.nih.gov/33025653/>

**9 January 2021**

Man, Y., Kucukal, E., An, R., Bode, A., Little, J. A., & Gurkan, U. A. (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>

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

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8049448/>

**7 December 2020**

Bao, X. R., Pan, Y., Lee, C. M., Davis, T. M. E., & Bao, G. (2021). Tools for experimental and computational analyses of off-target editing by programmable nucleases. *Nature Protocols*, 16(1), 10–26. <https://doi.org/10.1038/s41596-020-00431-y>

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## **Red Blood Cell Adhesion to ICAM-1 is Mediated by Fibrinogen and is Associated with Right-to-Left Shunts in Sickle Cell Disease**

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7422136/>

**10 August 2020**

Kucukal, E., Man, Y., Quinn, E., Tewari, N., An, R., Ilich, A., Key, N. S., Little, J. A., & Gurkan, U. A. (2020). Red blood cell adhesion to ICAM-1 is mediated by fibrinogen and is associated with right-to-left shunts in sickle cell disease. *Blood Advances*, 4(15), 3688–3698. <https://doi.org/10.1182/bloodadvances.2020001656>

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## **Whole Blood Viscosity and Red Blood Cell Adhesion: Potential Biomarkers for Targeted and Curative Therapies in Sickle Cell Disease**

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7689825/>

**10 August 2020**

Kucukal, E., Man, Y., Hill, A., Liu, S., Bode, A., An, R., Kadambi, J. R., Little, J. A., & Gurkan, U. A. (2020). Whole blood viscosity and red blood cell adhesion: Potential biomarkers for targeted and curative therapies in sickle cell disease. *American Journal of Hematology*, 95(11), 1246–1256. <https://doi.org/10.1002/ajh.25933>

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## **Paper-Based Microchip Electrophoresis for Point-of-Care Hemoglobin Testing**

<https://pubs.rsc.org/en/content/articlelanding/2020/AN/C9AN02250C#!divAbstract>

**7 April 2020**

Hasan, M., Fraiwan, A., An, R., Alapan, Y., Ung, R., Akkus, A., Xu, J. Z., Rezac, A. J., Kocmich, N., Creary, M. S., Oginni, T., Olanipekun, G., Hassan-Hanga, F., Jibir, B. W., Gambo, S., Verma, A. K., Bharti, P. K., Riolueang, S., Ngimhung, T., . . . Gurkan, U. A. (2020). Paper-based microchip electrophoresis for point-of-care hemoglobin testing. *Analyst*, 145(7), 2525–2542. <https://doi.org/10.1039/c9an02250c>

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

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7246173/>

**10 March 2020**

Man, Y., Goreke, U., Kucukal, E., Hill, A., An, R., Liu, S., Bode, A., Solis-Fuentes, A., Nayak, L., Little, J. A., & Gurkan, U. A. (2020). Leukocyte adhesion to P-selectin and the inhibitory role of Crizanlizumab in sickle cell disease: A standardized microfluidic assessment. *Blood Cells Molecules and Diseases*, 83, 102424. <https://doi.org/10.1016/j.bcmed.2020.102424>

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

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7013264/>

**6 February 2020**

Kaur, M., Brown, M. B., Love, T. W., Thompson, A. A., Treadwell, M., & Smith-Whitley, K. (2020). Understanding sickle cell disease: impact of surveillance and gaps in knowledge. *Blood Advances*, 4(3), 496–498. <https://doi.org/10.1182/bloodadvances.2019001000>

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

<https://jamanetwork.com/journals/jama/article-abstract/2748180>

**8 August 2019**

Benz, E. J., Mondoro, T. H., & 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>

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