

[Access]

1. Sickle Cell Disease Health Disparities

Abstract: Explores the challenges faced by individuals with sickle cell disease (SCD) in terms of health outcomes and access to healthcare resources

<https://www.cdcfoundation.org/sites/default/files/files/SickleCellDisease-HealthDisparities-FactSheet021618.pdf>

2. Reducing Health Care Disparities in Sickle Cell Disease

Abstract: Discusses the health inequities affecting persons with SCD, describes programs intended to improve their care, and identifies actions that could be taken to further reduce these inequities, improve care, control treatment costs, and ease the burden of disease

<https://pubmed.ncbi.nlm.nih.gov/31600481/>

3. Gene Therapies- Current State, Access, and Financial Factors

Abstract: American Academy of Actuaries committees provide comments on the request for information on access to gene therapies from a commercial marketplace perspective

<https://www.actuary.org/sites/default/files/2024-01/health-comment-gene-therapy-RFI.pdf>

4. FDA approves two groundbreaking new gene therapy treatments for sickle cell disease

Abstract: Melissa Creary, Assistant Professor, University of Michigan School of Public Health describes SCD and the gene therapies for treatment

<https://www.youtube.com/watch?v=3Fslj0YxaSc-Dr.>

[Financing]

5. Overview of Payment Models:

Abstract: Consolidation of 3 payment methods- amortization, risk spreading, and performance-based payment which consider both equitable patient access and payer reimbursement

<https://www.ajmc.com/view/high-cost-gene-therapies-present-reimbursement-access-challenges>

6. Florida Medicaid withholds a percent of the capitation rate to fund

Abstract: Florida Medicaid Managed Care Data Book-Milliman

<https://ahca.myflorida.com/content/download/20819/file/Presentation.pdf>

7. Confronting High Costs and Clinical Uncertainty: Innovative Payment Models for Gene Therapies (Behind pay wall)

Abstract: Discusses each payment model (installments, risk pools, reinsurance, price-volume agreements, expenditure caps, subscriptions, outcomes-based payments and rebates, warranties, population outcomes-based agreements, and coverage with evidence development), its advantages and challenges, and considerations for US payers

<https://www.healthaffairs.org/doi/10.1377/hlthaff.2023.00527>

8. ICER and NEWDIGS Release White Paper Analyzing the Challenges and Potential Policy Options for Paying for Gene Therapies

Abstract: Describes the white paper which outlines policy reforms and market actions to support innovation and access while managing uncertainty, affordability, and equity concerns

<https://icer.org/news-insights/press-releases/icer-and-newdigs-release-white-paper-analyzing-the-challenges-and-potential-policy-options-for-paying-for-gene-therapies/>

9. Managing the Challenges of Paying for Gene Therapy: Strategies for Market Action and Policy Reform

Abstract: Explores the range of emerging market approaches and possible policy reforms that have the potential to help the broader US health system achieve equitable and affordable access to gene therapies

https://icer.org/wp-content/uploads/2024/04/Managing-the-Challenges-of-Paying-for-Gene-Therapy--ICER-NEWDIGS-White-Paper-2024_final.pdf

10. Sources of Innovation in Gene Therapies - Approaches to Achieving Affordable Prices

Abstract: Suggests possible approaches could help reduce the extremely high prices charged for gene therapies aimed at academic and other research institutions and US Congress

<https://pubmed.ncbi.nlm.nih.gov/36688504/>

11. IDPH Public Report

<https://dph.illinois.gov/content/dam/soi/en/web/idph/publications/idph/topics-and-services/life-stages-populations/genomics/sickle-cell-disease-impact-surveillance-report-2024.pdf> -A

statewide data report aimed at developing an understanding of challenges people who live with SCD have with accessing needed health care

[Shared during July's Adv. Council Meeting Chat]

12. Networking California for Sickle Cell Care

Abstract: Outlines a regional network of adult SCD skilled subspecialty clinics in California aimed to increase access to Sickle Cell Disease individuals

<https://sicklecellcare-ca.com/clinical-network/>

13. SCDC Program

Abstract: CDC program which collects health information about people with SCD to study the long-term trends in diagnosis, treatment, and healthcare access for people with SCD in the United States

<https://www.cdc.gov/sickle-cell/scdc/index.html>