Florida Medicaid: Prevalence and Treatment of Sickle Cell Disease

House Health Care Regulation Subcommittee

December 6, 2023

Florida Medicaid Report to the Legislature: February 2023

- In 2022, the Florida Legislature directed the Agency for Health Care Administration (Agency), in consultation with the Florida Medical School Quality Network (FMSQN), to conduct a review and provide a report regarding Medicaid enrollees diagnosed with sickle cell disease.

- The report was completed and submitted on February 1, 2023. Subsequently, it was posted to the Agency's publicly accessible website.

- The report documented the impact of sickle cell disease in Florida Medicaid.

- In 2023, the Florida Legislature directed the Agency in SB 1352 to review sickle cell disease medications, treatments, and services for Medicaid recipients and develop a written report every 2 years beginning November 1, 2024.

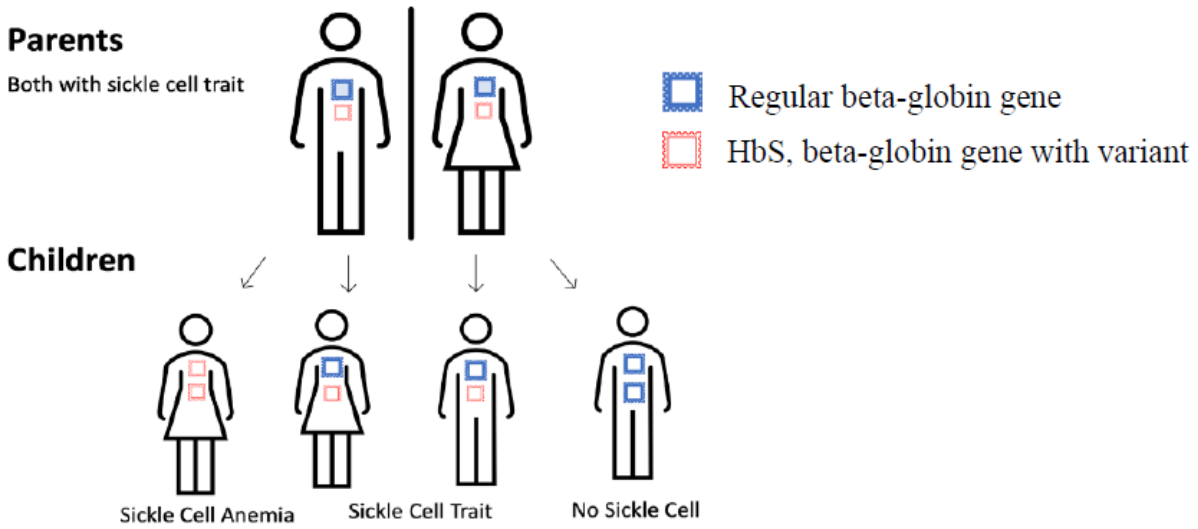
Florida Medicaid Report to the Legislature: February 2023

- In general, the report contains information on sickle cell disease including:
  - Background
  - Prevalence
  - Demographics and utilization
  - Geographic distribution
  - Treatment
- The report analyzed Medicaid program data from 2018-2021.
- The report cohort included individuals who:
  - were continuously enrolled in Medicaid for at least 12 of 12 months; and
  - had at least 2 health care encounters associated with sickle cell disease during the study period.
- This approach identified 9,206 Medicaid recipients for inclusion in the study.

What is Sickle Cell Disease?

- Sickle cell disease, sometimes called sickle cell anemia, is a blood disorder that affects hemoglobin, the protein that carries oxygen through the body.
- Sickle cell disease is an inherited disease – if both parents carry a gene for sickle cell, their children have a 25% chance of having sickle cell disease.

Figure 1. Sickle Cell Trait and Sickle Cell Disease Inheritance.



Sickle Cell Disease - Continued

Sickle cell disease (SCD) causes misshaped red blood cells to occlude blood vessels and chronically infarct multiple organs, including bones, brain, spleen, eyes, and kidneys.

Repeated crises suffocate downstream tissues, such as bones, spleen, brain, eyes, and kidneys, causing wracking bone pain attacks, unguarded bacterial infections, stroke, blindness, and kidney failure among many other organ failures.

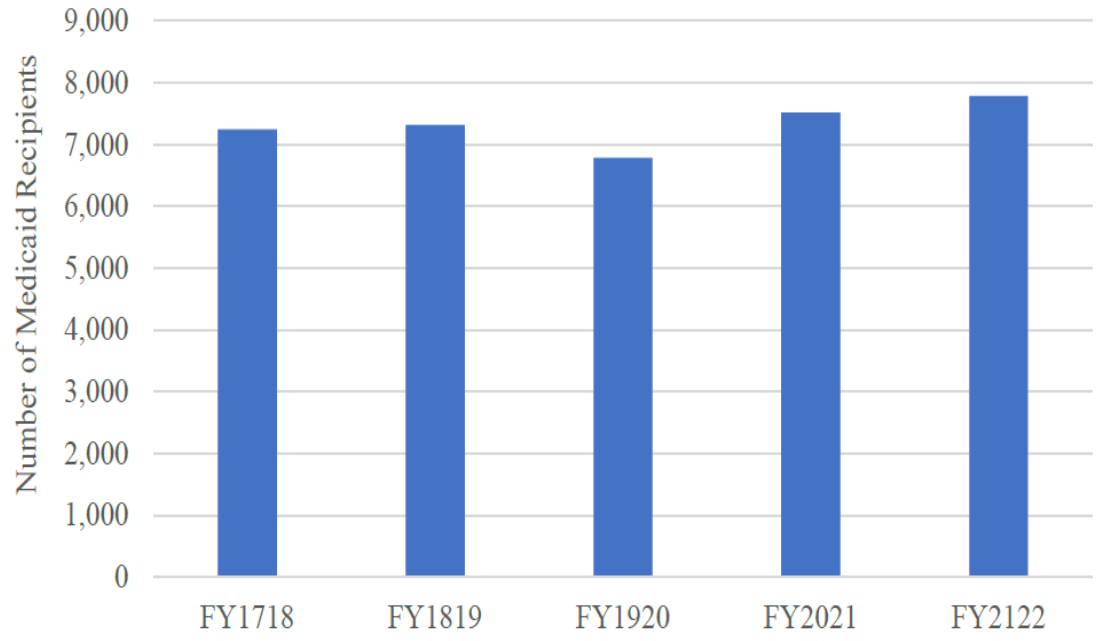
Currently, the life expectancy of a person with sickle cell disease is approximately 20 years less than the general population at 54 years of age compared to 76 years for people without sickle cell disease.

Florida Medicaid: Prevalence of Sickle Cell Disease

In Florida Medicaid:

- The prevalence rate of sickle cell disease is twice as high as the national average and Florida Medicaid has one of the highest numbers of sickle cell disease patients in the U.S., indicating a disproportionate impact from a national perspective.
- Since 2017, Florida and New York have the highest Medicaid cases of sickle cell disease in the United States.
- The yearly average of recipients with sickle cell disease is **7,328**.

Figure 2. Sickle Cell Disease in Florida Medicaid. The number of unique Medicaid recipients with sickle cell disease are plotted with respect to fiscal year (FY).



Florida Medicaid: Demographics for Recipients with Sickle Cell Disease

- The Florida Medicaid sickle cell disease (SCD) population was predominately female, young and black:

Recipient Demographic Breakdown:
58% Female, 42% Male

Majority of SCD cases are in recipients under 21, Median recipient age is 18 years

63% of Florida Medicaid recipients with SCD identified as black

Florida Medicaid: Service Utilization and Experience of Recipients with Sickle Cell Disease

Of those in the study cohort, nearly all were evaluated by a Florida physician at least once in the 4-year study period.

Care and treatment is received in both inpatient and outpatient settings.

~85% recipients were evaluated or treated in an outpatient clinic setting at least once.

52% recipients were admitted to an inpatient hospital.

Majority of recipients experience crisis events with trips to the Emergency Room (ER).

61% of the recipients were evaluated and treated in an ER at least once.

39% did not seek ER care.

Children between ages 2-16 years, should have an annual screening for stroke risk through a Transcranial Doppler Ultrasound (TCD).

Of the 2,906 children, 1,068 (41%) had at least one Transcranial Doppler (TCD) during the four-year study.

77% of recipients had a pharmacy expenditure for a sickle cell disease-relevant medication, such as disease-modifying treatments, prophylactic antibiotics, opioids, or iron chelating agents.

23% had no pharmacy expenditures for sickle cell disease-relevant medication.

Florida Medicaid: Treatment of Sickle Cell Disease

Medication	Number of Eligible Patients	Percent of Eligible Population Taking Medication
Oral Penicillin	2,054	58%
Hydroxyurea (Generic, Droxia, Siklos)	7,613	22%
L-glutamine	6,840	2%
Voxelotor	5,629	0.1%
Crizanlizumab	4,956	0.08%

The most commonly prescribed medications among the cohort were:

- Oral penicillin, which is recommended for children under 5 to reduce infection risk and mitigate damage to the spleen; and
- Hydroxyurea, to reduce pain episodes, stroke risk, and prevent organ damage.

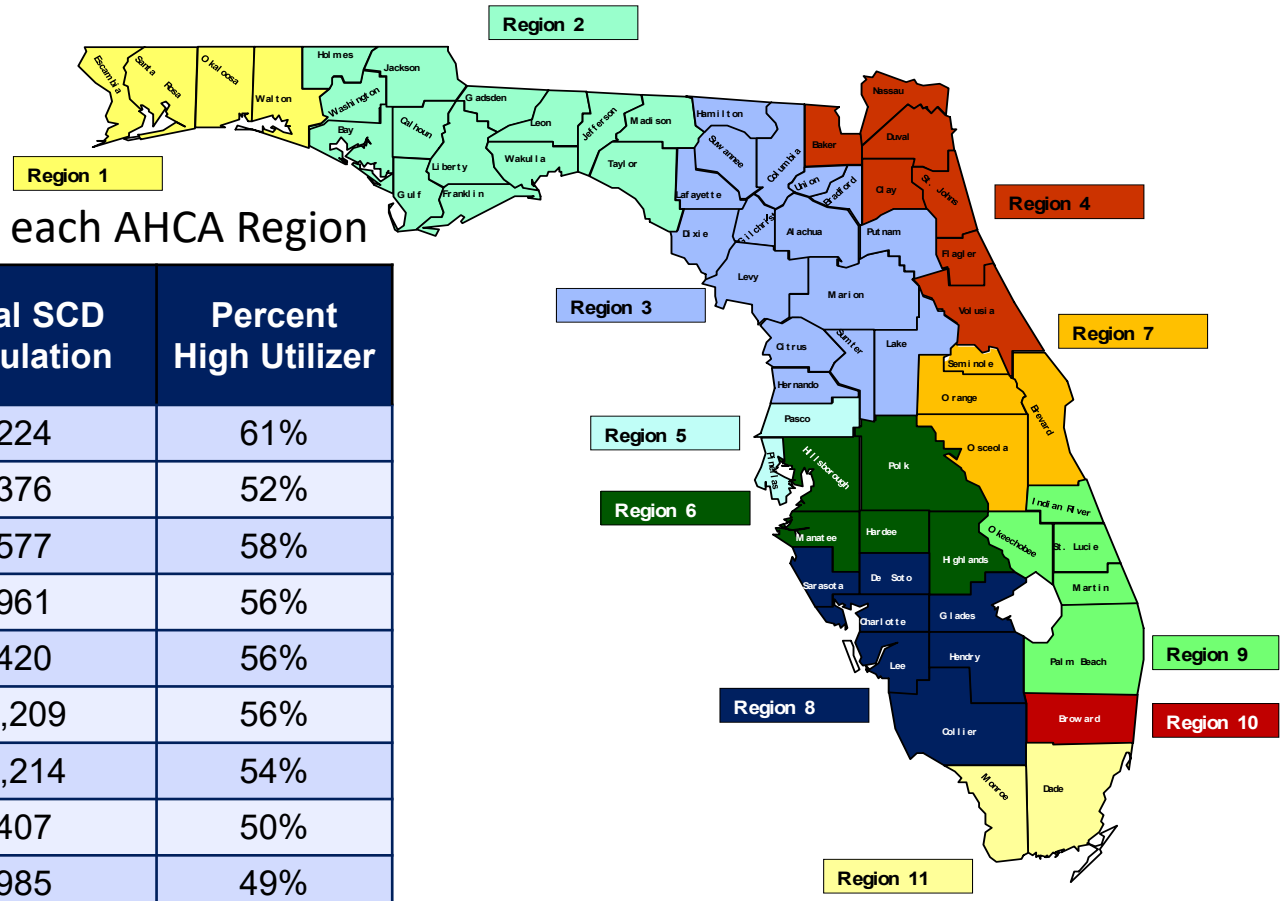
- In the four-year study period, recipients' total pharmacy expenditures per year ranged from $4,378.26 to $6,232.30 and increased incrementally by year.

Florida Medicaid: Recipients with SCD who are High Utilizers

High utilizer was defined as two or more emergency room visits or two or more inpatient admissions in a 12-month period.

Percent of High Utilizers in each AHCA Region

AHCA Region	SCD High Utilizer Population	Total SCD Population	Percent High Utilizer
1	137	224	61%
2	196	376	52%
3	336	577	58%
4	541	961	56%
5	261	420	56%
6	678	1,209	56%
7	656	1,214	54%
8	202	407	50%
9	486	985	49%
10	739	1,352	55%
11	708	1,388	51%
Overall Number of High Utilizers in Florida **4,940**			



Florida Medicaid: Clinical Treatment Programs for Sickle Cell Disease

Clinical treatment programs were identified by the FMSQN as available and contracted with managed care plans for the care of sickle cell recipients.

= Recognized as a treatment center of excellence by The National Alliance of Sickle Cell Centers

Hospital Systems

- AdventHealth System Orlando
- Broward Health Sickle Cell Care
- Memorial Health Care System
- Orlando Health's Arnold Palmer Hospital

Children's Hospitals

- Johns Hopkins All Children's
- Nemours Children's Center for Cancer and Blood Disorders
- Nicklaus Children's Hospital
- Wolfson Children's Hospital

University Teaching Hospitals and Systems

- University of Florida Shands Hospital
- University of Miami Sylvester Comprehensive Cancer Center
- University of South Florida Comprehensive Sickle Cell Center

AGENCY FOR HEALTH CARE ADMINISTRATION STATE OF FLORIDA

Emerging Treatments for Sickle Cell Disease

- Based on publicly available information, there are several new treatments for sickle cell disease currently going through the FDA approval process.

- Next month, the Food and Drug Administration will decide on the regulatory approval of two gene therapies for sickle cell disease. Exa-cel and lovo-cel are potentially one-time treatment options for sickle cell patient.

How will Medicaid cover these newly FDA approved treatments?

Florida Medicaid: Coverage of Outpatient Prescription Drugs

The State requires outpatient drugs under the Prescribed Drug Services Rule (59G-4.250) to:

1. Be approved by the Food and Drug Administration (FDA)
2. Have a federal rebate

Food and Drug Administration (FDA) Approval

- For new drugs to be covered by State Medicaid Programs, they first must have FDA approval.

- To receive FDA approval the FDA evaluates:

Federal Drug Rebate

Second they need a federal rebate –

- The Medicaid Drug Rebate Program (MDRP) establishes rebate rates that are applied to all state Medicaid programs.
- In order for branded drugs to be covered by Medicaid, drug manufacturers must pay a rebate of at least 23.1%

Florida Medicaid: Process Once Federally Approved = Clinical Appropriateness

After FDA approval has been met, the Agency develops coverage criteria based on:

1. Determine clinical appropriateness
2. Is the drug preferred?
3. Payment Methodology

Key Considerations	
Indication(s) of the drug	Drug manufacturer prescribing information
Medical standards	Treatment algorithms
Number of Medicaid recipients impacted	Preferred drug alternatives

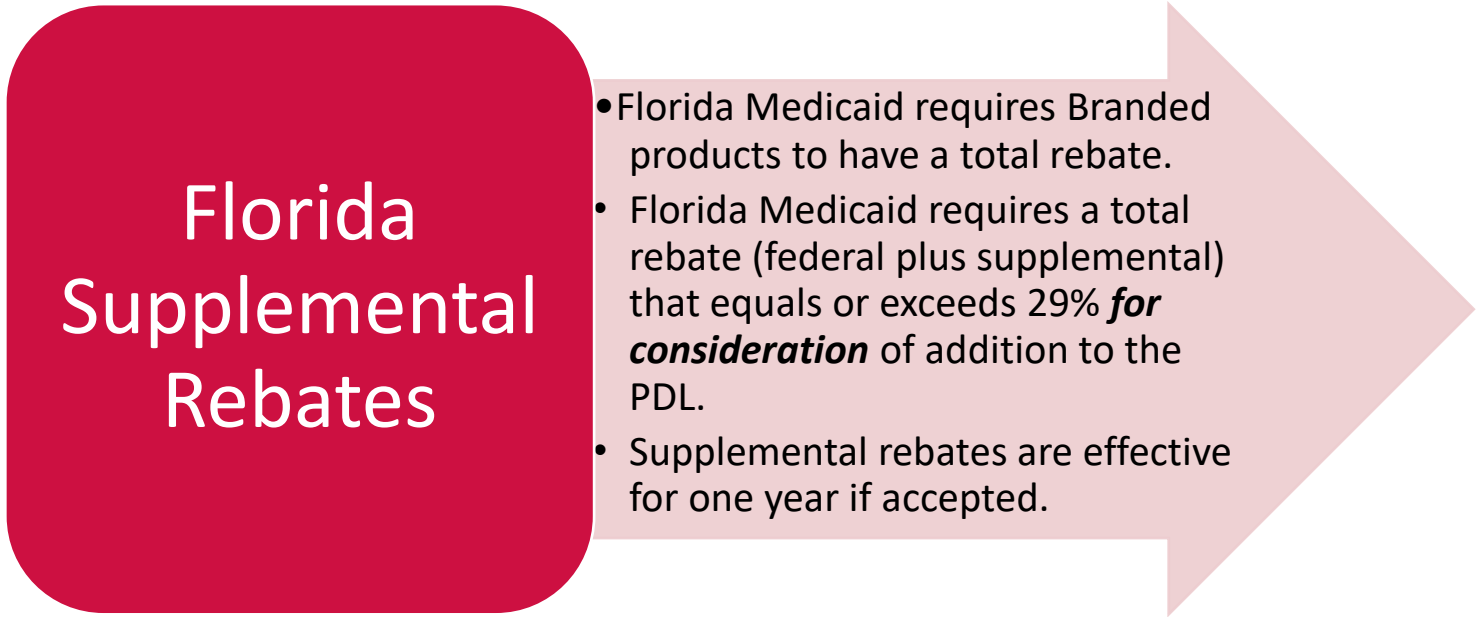
Florida Medicaid: Process Once Federally Approved = Is the Drug Preferred?

- Florida Medicaid has a single Preferred Drug List (PDL) that is established through a joint process with the Agency and the Medicaid Pharmacy and Therapeutics (P&T) Committee.
- Managed Care Organizations (MCOs) and Fee-For-Service (FFS) providers must follow the single PDL.
- Florida Medicaid utilizes the P&T Committee to review new drugs being considered for addition to the PDL.
- Drugs must be on the market and available for at least 90 days prior to review by the P&T Committee.

Drugs may be classified as:

Preferred	Non-Preferred
No clinical criteria	Requires additional steps for access compared to preferred
With clinical criteria (may be computer automated)	Requires prior authorization

Florida Medicaid: Supplemental Rebates



- States can get CMS approval to enter into value-based agreements (VBA) for rebates.
- VBAs are utilized in health systems and generally involve linking payment to value or specific outcomes achieved.

Payment Options to Address Additional Costs for Reimbursement

There are four different payment options considered for payment of the newly approved drugs.

1. Risk Pool
2. Risk Adjustment
3. Kick Payment

Option 1: High Pharmacy Cost Risk Pool

AHCA could implement a high-risk pool for members with high drug costs during the year. This option would cover both new and existing drugs with high costs per treatment.

Pros	Cons

• Directs more funding to capitated plans with higher total costs of drugs.
• Monthly capitation rates can be paid on the usual schedule with risk pool payments occurring on a regular schedule after pharmacy costs are known.
• Overall funding for pharmacy services in the capitation rates is consistent with recent observed costs and access to services.
	• The risk pool needs to be fully funded.
	• Does not mitigate capitated plan risk tied to uncertainty during the rate year.
	• Additional administrative burden associated with the risk pool:
	  ○ Defining the cost threshold for recipient eligibility.
	  ○ Creating withholds on the capitation rates
	  ○ Calculating, paying, and reviewing appropriate funds to each plan based on their portion of the pool.

Option 2: Risk Adjustment

Monthly capitation rates include a component for new pharmacy drug costs which would vary in the payments made to plans based on relative risk scores developed by Milliman.

Pros	Cons
• No additional administrative burden. • Overall funding for pharmacy services in the capitation rates is consistent with recent observed costs and access to services. • Payments will be directed to the specific capitated plans that incur new high pharmacy costs in the adjusted rate cells.	• Milliman will need to develop an approach to include explicit adjustment for high pharmacy costs which may delay completion of risk weights. • Additional payments will not necessarily be directed to the specific plans whose recipients have higher pharmacy costs and will not distribute payments to specific plans with recipients using high pharmacy cost drugs in rate cells that are not risk adjusted. • Presents timing concerns due to not knowing if someone is using these drugs until data is submitted. • Does not mitigate capitated plan risk tied to uncertainty surrounding the number of total utilizers, or average cost per utilizer of the high-cost drugs in a rate year.

Option 3: Kick Payment

AHCA could implement a kick payment to be paid once a month for each MMA capitated plan recipient who utilizes the high-cost drugs during that month.

Pros	Cons
• Mitigates capitated plan risk tied to uncertainty by paying plans a fixed amount per unique utilizer per month. • Payments are directed to the specific plans that cover individuals utilizing the new drugs.	• Kick payment cannot be paid until after the claims have been reported. • Increased administrative complexity. • If the cost per utilizer changes significantly, the kick payment may be either too high or too low. • Multiple kick payments would be needed for multiple drugs. • Potential for increased cost and not budget neutral.