COMMONWEALTH of VIRGINIA

Department of Medical Assistance Services

CHERYL ROBERTS
DIRECTOR

SUITE 1300
600 EAST BROAD STREET
RICHMOND, VA 23219
804/786-7933
804/343-0634 (TDD)

November 15, 2025

MEMORANDUM

TO: The Honorable Mark D. Sickles
Chair, House Health and Human Services Committee

The Honorable Ghazala F. Hashmi
Chair, Senate Education and Health Committee

The Honorable Rodney T. Willett
Chair, Joint Commission on Health Care

FROM: Cheryl J. Roberts
Director, Virginia Department of Medical Assistance Services

SUBJECT: Annual Review of Medications, Services, and Forms of Treatment for Sickle Cell Disease Report

This report is submitted in compliance with Section 32.1-331.06. of the Code of Virginia which states:

C. By November 15 of each year, the Department of Medical Assistance Services shall prepare and submit a report that details the Department's findings from the annual review required by this section, as well as any recommendations to the General Assembly based upon those findings, to the Chairmen of the House Committee on Health and Human Services and the Senate Committee on Education and Health and to the Joint Commission on Health Care. The report shall be submitted for publication as a report document as provided in the procedures of the Division of Legislative Automated Systems for the processing of legislative documents and reports and shall be posted on the General Assembly's website and the Department's website.

Should you have any questions or need additional information, please feel free to contact me at (804) 664-2660.

CJR/wrf

Enclosure

Pc: The Honorable Janet V. Kelly, Secretary of Health and Human Resources

Annual Review of Medications, Services and Forms of Treatment for Sickle Cell Disease Report

November 2025

Report Mandate:

Section 32.1-331.06. of the Code of Virginia states:

A. The Department of Medical Assistance Services shall conduct an annual review of all medications, services, and forms of treatment for sickle cell disease available to individuals with a diagnosis of sickle cell disease who are eligible for coverage under the state plan for medical assistance services. The purpose of the annual review is to determine (i) if the available covered medications, treatments, and services are adequate to meet the needs of enrollees with a diagnosis of sickle cell disease and (ii) whether the Department of Medical Assistance Services should seek to expand coverage for additional medications, treatments, or services.

B. When conducting the annual review required by this section, the Department of Medical Assistance Services shall solicit and consider input from the general public, with specific emphasis on input from persons or groups with knowledge and experience in the area of sickle cell disease treatment.

C. By November 15 of each year, the Department of Medical Assistance Services shall prepare and submit a report that details the Department's findings from the annual review required by this section, as well as any recommendations to the General Assembly based upon those findings, to the Chairmen of the House Committee on Health and Human Services and the Senate Committee on Education and Health and to the Joint Commission on Health Care. The report shall be submitted for publication as a report document as provided in the procedures of the Division of Legislative Automated Systems for the processing of legislative documents and reports and shall be posted on the General Assembly's website and the Department's website.

Annual Review of Medications, Services and Forms of Treatment for Sickle Cell Disease Report for 2025

In compliance with §32.1-331.06, DMAS conducted a review of medications, services and forms of treatment for Sickle Cell Disease (SCD).

For the Fiscal Year (FY) 2025 report, DMAS utilized claims and enrollment data, information provided by the MCOs, and input from providers, advocates, and Medicaid members.

Sickle Cell Disease

Sickle Cell Disease (SCD) is a group of inherited red blood cell disorders. People with SCD have abnormal hemoglobin, called hemoglobin S or sickle hemoglobin, in their red blood cells. This abnormal hemoglobin causes the red blood cells to become rigid, sticky, and shaped like crescent moons or sickles. These sickle cells can block blood flow, leading to pain and other serious complications such as infections, acute chest syndrome, and stroke.

Individuals with sickle cell trait (SCT) have a mutation that causes them to make hemoglobin S instead of beta-globin. These people inherit one sickle cell gene and one normal gene. Although many individuals with SCT are asymptomatic, they can pass the trait on to their children. If both parents

have SCT, there is a 50% chance that any child of theirs also will have SCT if the child inherits the sickle cell gene from one of the parents. If both parents have SCT, there is a 25% chance that any child of theirs will have SCD. There is the same 25% chance that the child will not have SCD or SCT. According to the American Society of Hematology, approximately one in 12 African Americans has SCT. Those of Hispanic, Middle Eastern, Asian, Indian and Mediterranean descent are also at risk for SCD and SCT.

Evidence-Based Treatment Guidelines

The American Society of Hematology (ASH) has developed comprehensive guidelines for the management of SCD, focusing on various aspects of the disease:[1]

1. **Pain Management**:
    - Use of nonsteroidal anti-inflammatory drugs (NSAIDs) and opioids for managing acute pain episodes.
    - Chronic pain management may include physical therapy, cognitive behavioral therapy, and medications.
    - Other integrative approaches such as acupuncture or
    - massage therapy.
2. **Hydroxyurea Therapy**:
    - Recommended for children and adults with frequent pain crises to reduce the frequency of these episodes and the need for blood transfusions.
    - Hydroxyurea can also help prevent acute chest syndrome and improve overall quality of life.
3. **Blood Transfusions**:
    - Regular blood transfusions are recommended for preventing stroke in children with abnormal transcranial Doppler ultrasound results.
    - Transfusions are also used to manage severe anemia and other complications.
4. **Stem Cell Transplantation**:
    - The only potential cure for SCD is a hematopoietic stem cell transplant (HSCT), typically from a matched sibling donor.
    - This option is considered for patients with severe complications who have a suitable donor.
5. **Infection Prevention**:
    - Prophylactic penicillin is recommended for children until at least five years of age to prevent pneumococcal infections.
    - Vaccinations, including pneumococcal, meningococcal, and annual influenza vaccines, are crucial.
6. **Stroke Prevention**:
    - Adults and children should be screened via brain scans to assess their risk for silent stroke.

[1] Source: American Society of Hematology 2019-2021 Clinical Practice Guidelines on Sickle Cell Disease.

- Hydroxyurea and blood transfusions are used for primary and secondary stroke prevention.

7. **Management of Acute Chest Syndrome**:
    - Prompt treatment with antibiotics, oxygen therapy, and blood transfusions.
    - Pain management and incentive spirometry to prevent recurrence.

These guidelines are designed to improve the quality of life and outcomes for individuals with SCD by providing evidence-based recommendations for the prevention and management of complications.

Demographics[2]

During SFY2025, there were 3,418 Medicaid and CHIP enrollees identified with a SCD diagnosis. Eight thousand nine hundred and thirty-two Medicaid and CHIP enrollees were identified with a SCT diagnosis.

- 62% of the population is female, 38% male.
- 86% identified as African American, 8% Caucasian, 2% Hispanic, Other race, Asian or Native American, and less than 1% Native Hawaiian or Pacific Islander or Bi-/Multi-racial.
- 30% are less than 19 years old, 49% aged 19-44, 17% aged 45-64, and 4% aged 65 and over.
- 4% have limited Medicaid benefits, 3% are in pregnant woman categories and 3% have full Medicare coverage.
- 39% reside in Tidewater, 31% in Central, 16% in Northern Virginia/Winchester, 8% in Charlottesville/Western, 5% in Roanoke/Alleghany, and less than 1% in Southwest.

Treatment[3]

Management of sickle cell anemia is typically aimed at preventing pain episodes, relieving symptoms and avoiding complications. The management of SCD relies on a multi-modal approach consisting of a combination of pharmacological interventions, supportive care, and monitoring of the diseases and impacts on other functions. Globally, Hydroxyurea remains the cornerstone of first-line therapy.

Pharmacological Interventions

- Hydroxyurea (Droxia, Hydrea, Xromi[4])- Reduces the frequency of pain crises and possibly reduces the need for blood transfusions and hospital stays
- L-glutamine oral powder (Endari)- Helps in reducing the frequency of pain crises
- Crizanlizumab (Adakveo)- Helps to reduce the frequency of pain crises in adults and in children older than 16 years
- Voxelotor (Oxbryta)- Decreases the risk of anemia and improves blood flow throughout

[2] Figures are based on updated methodology which includes a three-year lookback to include individuals who had a sickle cell disease or sickle cell trait diagnosis who were still enrolled in Medicaid during FY2025 but may not have had an SCD/SCT diagnosis within FY2025.
[3] Source: Mayo Clinic, Sickle Cell Anemia Diagnosis and Treatments.
[4] Xromi will be a preferred hydroxyurea product as of 1/1/26. It is currently listed as non-preferred but can be approved with a service authorization.

the body. Removed from the market September 2024 due to safety concerns.
- Pain-relieving medicines- Helps to relieve pain during sickle cell pain crises

Hydroxyurea remains the first-line treatment for SCD. It has been proven to reduce the frequency of VOCs. It also increases the level of fetal hemoglobin[5] as well as overall hemoglobin levels. It can decrease the rate of VOCs and blood transfusions in adults by 50% and has proven to be instrumental in decreasing the rate of acute chest syndrome episodes. Hydroxyurea continues to be the only therapy approved for children diagnosed with SCD aged 9 months to 4 years old.

During SFY2025, 25% of eligible Virginia Medicaid members diagnosed with SCD filled at least one prescription for hydroxyurea. Members aged less than 19 had the highest adherence to hydroxyurea treatment with 40% of the population receiving treatment and 23% adhering to daily treatment lasting for at least 6 months.

Medication Utilization in Eligible Medicaid and CHIP Enrollees with SCD			
Medication	**Number of Enrollees with Filled Prescription**	**Number Eligible for Medication**	**% Eligible Population w/Filled Prescription**
Hydroxyurea	795	3,138	25%
L-glutamine	43	2,915	1%
Voxelotor*	104	2,968	4%
Crizanlizumab	44	2,339	2%

* *Removed from the market Sep 2024*

Narcotic analgesics were utilized by 39% of the Virginia Medicaid population diagnosed with SCD to decrease chronic pain. During SFY2025, there was a noticeable increase in the usage of alternate pain management therapies such as physical therapy, acupuncture and heat- and/or cryo-therapy- 12% of individuals diagnosed with SCD received alternate pain management therapies.

Investigational Drugs in Clinical Trials

- Rilzabrutinib- Developed to target inflammation and reduce vaso-occlusive crises (also known as VOCs or pain crises)
- Etavopivat- Developed to improve hemoglobin levels and reducing VOCs

Drugs in late-stage or long-term trials

- Inclacumab- Developed to reduce inflammation and lessen the frequency of vaso-occlusive (VOCs or pain) crises. In August 2025, Pfizer announced that the drug did not meet its primary endpoint of significant reduction in the rate of VOCs in participants receiving inclacumab versus placebo every 12 weeks
- Benserazide- Developed to increase fetal hemoglobin; one clinical trial is expected to be completed in late 2024, but preliminary results were limited in a patient population without SCD
- Mitapivat- A Phase 2/3 trial is in progress to determine the dose and efficacy of this

[5] Fetal hemoglobin is important in treating SCD because it does not sickle and can prevent or reduce sickling of red blood cells.

agent.

Emerging therapeutic strategies

- There is increased interest in repurposing existing drugs for sickle cell disease, which can expedite the development timeline
- Clinical trials using gene-editing technology are ongoing, with a strong focus on both early-stage safety trials and later-stage efficacy trials
- A Phase 1/2 trial in combination with other agents is being expanded into non-oncological hematology indications, including sickle cell disease

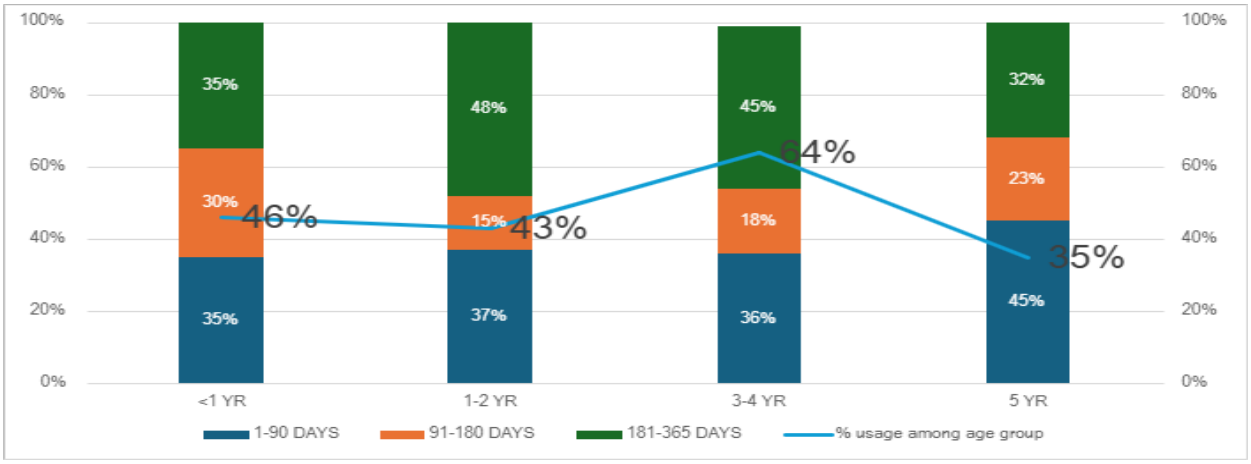
Infection Prevention

Children with sickle cell anemia should follow a daily regimen of prophylactic antibiotic treatment from about 2 months old to 5 years old to help prevent infections.

Infections are life-threatening to individuals living with sickle cell disease due to blockages of the spleen caused by sickling blood cells. This greatly impairs the spleen's ability to defend against bacteria. Children with SCD are especially vulnerable to severe infections, especially pneumococcal pneumonia, which can lead to rapid and life-threatening conditions such as acute chest syndrome. Infections can aggravate symptoms by increasing dehydration and stress- both of which can contribute to a sickle cell crisis.

During SFY2025, 46% of eligible Virginia Medicaid members diagnosed with SCD under the age of 6 received prophylactic antibiotics. By age group, children aged 3-4 years old were most likely to receive prophylactic antibiotics and those aged over 4 years old were least likely. Among children who adhered to an antibiotic regimen, 40% of the population met between 181 and 365 days of a daily dose of prophylactic antibiotics.

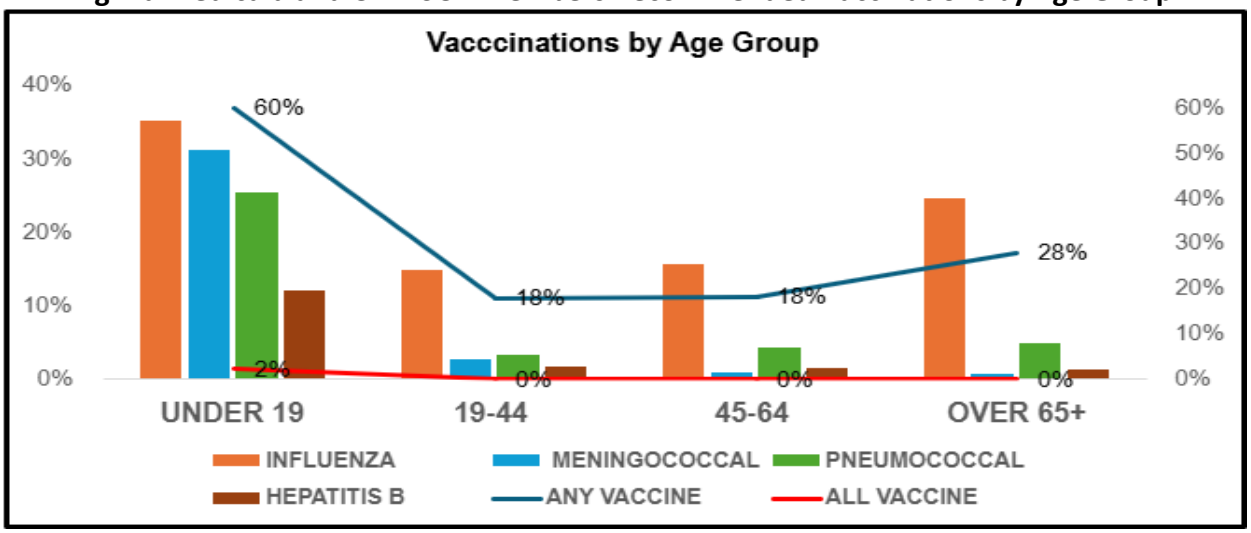
Utilization of Prophylactic Antibiotics- Medicaid and CHIP Members Under 6



Individuals living with SCD have a compromised immune system and are at a higher risk for serious infections like pneumococcal, meningococcal, and other bacterial and viral illnesses. Vaccines help protect them from life-threatening infections that can lead to complications or even death. Vaccinations are critical for individuals with SCD and should include COVID-19, pneumonia, meningitis, and hepatitis B in addition to an annual flu shot. Staying current on vaccinations is also important for adults with SCD. Individuals with SCD may require a specialized vaccination schedule to ensure they have adequate protection.

During SFY2025, Virginia Medicaid members diagnosed with SCD under 19 years of age received the highest percentage of vaccinations with 60% of the population receiving at least one recommended vaccine and 2% receiving all recommended vaccines. All ages are more likely to receive flu shots. Those aged 21-44 were least likely to receive recommended vaccinations with those aged 19-21 showing higher incidence of receiving vaccines than the 19-44 population average.

Virginia Medicaid and CHIP SCD Members Recommended Vaccinations by Age Group



Stroke Prevention

Regular transcranial doppler (TCD) ultrasound screening is used to identify stroke risk and is accompanied by preventative treatments like monthly blood transfusions and hydroxyurea for those at high risk. TCD is a standard screening tool for children aged 2-6 with SCD to identify those at high risk of stroke. During SFY2025, 21% of children aged less than 19 received a TCD screening.

Other Treatments

- Blood transfusions- Blood transfusions are used to treat and prevent complications, such as stroke, in people with SCD. Transfusion increases the number of non-sickling blood cells and can help reduce the symptoms and complications of SCD. Eight percent

of Virginia Medicaid members diagnosed with SCD received blood transfusions during SFY2025.

- Stem cell transplant- Also referred to as a bone marrow transplant, this procedure involves replacing bone marrow affected by SCD with bone marrow from a matched donor, such as a sibling, who doesn't have SCD. Stem cell transplant is only recommended for individuals with significant symptoms and complications of SCD since the risks associated with the procedure are high and include death. Less than 1% of Virginia Medicaid members diagnosed with SCD received stem cell transplant treatment during SFY2025.
- Incentive spirometry- Incentive spirometry is used in SCD treatment to prevent acute chest syndrome (ACS), which is a leading cause of morbidity and mortality in patients. The test encourages deep breathing, which helps prevent lung complications like lung collapse and hypoventilation, and maintains lung expansion. The ASH guidelines recommend that patients with SCD who are hospitalized perform incentive spirometry regularly while awake to help prevent ACS. Five percent of Virginia Medicaid members diagnosed with SCD received incentive spirometry therapy- the majority of which received therapy following an ACS incident.

Cell Gene Therapy

Sickle cell gene therapy modifies a patient's own blood stem cells to produce healthy red blood cells, addressing the genetic cause of the disease. This treatment involves collecting stem cells, genetically editing them outside the body to fix the faulty gene (gene editing) or adding a new gene (gene addition), and then returning them to the patient after a chemotherapy regimen has eliminated the original, sickle-causing cells. Both therapies involve modifying a patient's own blood stem cells and reinfusing them to produce healthy, non-sickling red blood cells, potentially offering a cure for the disease. Gene therapy can improve both overall health and quality of life for individuals living with SCD by reducing the frequency and severity of VOCs and preventing complications of SCD.

During SFY2025, four Virginia Medicaid members diagnosed with SCD were authorized for stem cell addition gene therapy treatment with Lyfgenia. One Medicaid member diagnosed with SCD is currently approved for stem cell addition gene therapy treatment in SFY2026.

- Stem cell gene addition therapy- The individual's own stem cells are removed and a gene to produce typical hemoglobin is injected. The stem cells are then given back to the person in a process known as autologous transplant. This option may provide a cure for people with SCD without a well-matched donor.
- Gene editing therapy- Stem cells are removed from the body, and the sickling gene is changed, or edited, to help restore the cells' ability to make healthy red blood cells. The treated stem cells are then returned (infused) to the body through the blood.

DMAS has successfully applied and been selected for the CMS Center for Medicare and Medicaid Innovation (CMMI) Cell and Gene Therapy (CGT) Access Model with a tentative start date of 7/1/2026. While DMAS is a part of the CMS CMMI CGT Access Model, this model requires a reimbursement change, which DMAS currently lacks the authority to implement.

State-Certified Cell Gene Therapy Centers

Name of Provider	State	Certified	Lyfgenia/Casgevey
Children's Hospital VCU	Richmond, VA	✓	Lyfgenia
VCUHS Massey Comprehensive Cancer Center	Richmond, VA	✓	Lyfgenia
Children's National Medical Center	Washington, DC	✓	Lyfgenia and Casgevy
Duke University Health	Durham, NC	✓	Lyfgenia and Casgevy
University of North Carolina	Chapel Hill, NC	✓	Lyfgenia and Casgevy
Atrium Levine Cancer Center	Charlotte, NC	✓	Casgevy
University of Maryland Medical Center	Baltimore, MD	✓	Lyfgenia
Nemours Children's Hospital	Wilmington, DE	✓	Lyfgenia
Tristar Centennial Medical Center	Nashville, TN	✓	Lyfgenia and Casgevy
Vanderbilt University Medical Center	Nashville, TN	✓	Lyfgenia and Casgevy
Thomas Jefferson University Hospital	Philadelphia, PA	✓	Casgevy
Children's Hospital of Philadelphia	Philadelphia, PA	✓	Lyfgenia and Casgevy

Virginia Medicaid Covered Services

Ninety-three percent of the Virginia Medicaid population diagnosed with SCD have full coverage and access to all evidence-based treatment services recommended by the American Society of Hematology as well as the current treatments listed above. Virginia Medicaid members diagnosed with SCD also have access to:

- medical benefits
- pharmacy benefits
- inpatient/outpatient hospital
- behavioral health services
- mental health residential treatment services
- addiction and recovery treatment services
- coverage in connection with clinical trials
- long-term care services and supports
- emergency transportation
- preventive services
- non-emergency transportation
- maternal and infant health services
- telehealth and telemedicine

Virginia Medicaid offers adult eye exams and treatment for the detection and prevention of SCD-related vision complications such as retinopathy. Virginia Medicaid managed care plans offer enhanced benefits which provide coverage for glasses, frames, or contacts.

Virginia Medicaid also offers routine dental care for children and adults through the Cardinal Care Smiles program offered through DentaQuest. Medicaid members living with SCD have access to dental services such as routine cleaning, exams, x-rays and fillings as well as gum related treatments, dentures, and root canals.

Virginia Medicaid covers audiology services that are provided as an inpatient, outpatient hospital service, outpatient rehabilitation agencies, or home health service to detect and treat hearing loss associated with SCD.

Utilization of Covered Services

Service category	% Medicaid Population diagnosed with SCD
Primary Care Visit	84%
Hematologist Visit	38%
Emergency Room Visit (any)	69%
Emergency Room Visit (SCD-related)	42%
Inpatient Hospital Visit (any)	33%
Inpatient Hospital Visit (SCD-related)	14%
Pharmacy Services	77%
Behavioral Health Services	29%
Vision Services	21%
Audiology Services	3%

Early and Periodic Screening, Diagnostic, and Treatment

The Early and Periodic Screening, Diagnostic and Treatment program (EPSDT) provides global benefit global coverage for Medicaid members under the age of 21 who are living with SCD. EPSDT includes periodic screening, vision, dental and hearing services. EPSDT also includes a federal requirement which compels state Medicaid agencies to cover services, products, or procedures for children if those items are determined to be medically necessary to correct or ameliorate a defect, physical or mental illness, or health problem identified through routine medical screening or examination, regardless of whether coverage for the same service/support is available to adults under the state plan.

Addressing Social Determinants of Health

The World Health Organization identifies social determinants of health (SdoH) as a key factor in determining the health status of individuals with chronic complications. Research indicates that SDoH, such as inadequate housing, food insecurity, and limited access to affordable healthcare,

shape the health behavior of patients with SCD[6]. Eighty-nine percent of Virginia Medicaid's population diagnosed with SCD is enrolled in managed care. Virginia Medicaid's managed care plans offer enhanced benefits which can help to address the social needs of Medicaid enrollees diagnosed with SCD such as providing resources for housing and food security as well as identifying community supports.

Summary

DMAS provides coverage for all SCD evidence-based treatments under Fee-for-Service (FFS) and managed care. Virginia Medicaid data shows gaps in recommended care for Medicaid and CHIP members diagnosed with SCD, specifically in rates of transcranial Doppler ultrasound screening, pneumococcal vaccination for children, and hydroxyurea use among children and adults. DMAS provides coverage for SCD support services such as behavioral health, vision, and hearing services under FFS and managed care. Analysis of Medicaid data indicates that these services are often under-utilized by the SCD population.

Transitioning from pediatric to adult SCD care can create barriers to treatment due to gaps in insurance coverage. Changes in Medicaid eligibility rules (e.g., extending Medicaid coverage to former foster care youth (2014), Medicaid Expansion coverage of childless adults (2019), and 12-month postpartum coverage extension for pregnant women (2021)) have had a positive impact on decreasing gaps in insurance coverage for Medicaid enrollees diagnosed with SCD who are transitioning from pediatric to adult care.

Barriers to treatment also exist due to a limited number of treatment centers. As of January 2023, the Virginia Department of Health (VDH) noted 9 Virginia Sickle Cell/ Hemoglobinopathy Treatment Centers. There are limited treatment centers for pediatric or adult SCD care in the far southwest. In the fall of 2024, Sentara Norfolk General Hospital and Eastern Virginia Medical School opened the Sentara-EVMS Comprehensive Sickle Cell Program which brings specialists from general internal medicine, hospital medicine, pain management, and psychiatry together in a single location to treat adults with SCD.

Area	Pediatric	Adult
Charlottesville	University of Virginia Medical Center	UVA Health Emily Couric Clinical Cancer Center
Fairfax	Pediatric Specialists of Virginia	INOVA Schar Cancer Institute
Norfolk	Children's Hospital of the King's Daughters	Eastern Virginia Medical School Comprehensive Sickle Cell Program
Richmond	Children's Hospital of Richmond at VCU	VCU Health Adult Outpatient Pavillion
Roanoke	Carilion Clinic Children's Hospital	Carilion Roanoke Memorial Hospital

Recommendations

- Utilize forums such as the Medicaid Member Advisory Committee and the Medicaid Physician and Managed Care Liaison Committee to create

[6] Source: Sickle Cell Disease and Social Determinants of Health- A Scoping Review

opportunities for input from the SCD community (patients, families, community-based organizations, and providers) within DMAS forums
- Increase opportunities for DMAS and managed care partners to collaborate on messaging to increase awareness of services
- Promote and augment managed care partners' role in providing resources and tools relevant to disease education, medication adherence monitoring, and care coordination for healthcare providers who treat individuals living with SCD
- Highlight partnership with Virginia Health Information (VHI) to increase awareness of SmartChart and the utilization of Care Insights to create individualized plans of care for individuals living with SCD
- Foster data sharing agreements between VDH and DMAS concerning screening results and SCD/SCT status

About DMAS and Medicaid

The mission of the Virginia Medicaid agency is to improve the health and well-being of Virginians through access to high-quality health care coverage. The Department of Medical Assistance Services (DMAS) administers Virginia's Medicaid and CHIP programs for approximately two million Virginians. Members have access to primary and specialty health services, inpatient care, dental, behavioral health as well as addiction and recovery treatment services. In addition, Medicaid long-term services and supports enable thousands of Virginians to remain in their homes or to access residential and nursing home care.

Medicaid members historically have included children, pregnant women, parents and caretakers, older adults, and individuals with disabilities. In 2019, Virginia expanded the Medicaid eligibility rules to make health care coverage available to more than 600,000 newly eligible, low-income adults.

Medicaid and CHIP (known in Virginia as Family Access to Medical Insurance Security, or FAMIS) are jointly funded by Virginia and the federal government under Title XIX and Title XXI of the Social Security Act. Virginia generally receives an approximate dollar-for-dollar federal spending match in the Medicaid program. Medicaid expansion qualifies the Commonwealth for a federal funding match of no less than 90% for newly eligible adults, generating cost savings that benefit the overall state budget.