2023 MEDICAID ACCESS AND LANDSCAPE REVIEW FOR SICKLE CELL DISEASE

Findings from the Assessment of Access Barriers in State Medicaid Programs

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Foreword

Sickle cell disease (SCD) is a chronic blood disorder that entails debilitating pain, severe complications, and irreversible organ and bone damage. This chronic disease has been stigmatized for decades due to its significant prevalence in marginalized black and brown communities. Subsequently, many patients living with this disease and their families have been overwhelmed with barriers to accessing novel SCD therapies and pain management requirements. As more than 50% of patients with SCD use Medicaid as their primary insurance, these barriers to access and gaps in coverage occur continually. Sick Cells partnered with Artia Solutions to analyze and present the landscape of access and coverage among SCD therapies and opioid policies across the United States.

As of February 2023, we conducted an environmental scan of state and managed care organizations under Medicaid to present coverage and access criteria for SCD disease-modifying therapies and opioid management. This report highlights the challenges and opportunities for community advocacy to support change in Medicaid policies. As more curative therapies are on the horizon, coverage under Medicaid has been a priority topic for the SCD community. This report's results will illuminate crucial decisions for policymakers to implement change to significantly improve access and patient-reported outcomes for patients and their families

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About

Sick Cells is a national sickle cell patient advocacy organization founded in 2017. Sick Cells' mission is to elevate the community's voice and stories of resilience. By highlighting the grave disparities in the sickle cell community, Sick Cells aims to influence decision-makers and propel change.

Links2Equity is a patient advocacy and health policy firm, founded with the power of unity as the driving force behind patient advocacy efforts. They employ innovative strategic methodologies that promote health equity and diversity among all groups and sectors within the healthcare ecosystem to build healthier communities.

Artia Solutions provides drug manufacturers with the resources, guidance, and support to launch or manage their medical or pharmacy benefit products with the Medicaid and Medicaid Managed Care marketplaces through faith, innovation, and collaboration.

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Table of Contents

Executive Summary

Background

Sickle cell disease (SCD) is a genetic condition caused by unhealthy and abnormal red blood cells. These harmful cells clog up blood vessels and reduce adequate oxygen flow, creating severe complications of debilitating pain, acute chest syndrome, various infections, strokes, and organ damage. More than half of individuals with SCD in the United States rely on Medicaid as their primary insurance.

Landscape Assessment

To assess the barriers associated with accessing disease-modifying therapies for SCD in Medicaid, Sick Cells and Artia Solutions analyzed the Medicaid coverage and access landscape of state Medicaid programs. We conducted an environmental scan to examine the current Medicaid coverage criteria for six products used to treat complications of SCD (Siklos®, Droxia®, Endari™, Adakveo®, and Oxbryta®, generic Hydroxyurea) and opioid management to treat the acute and chronic pain of SCD.

Key Findings

The environmental scan findings around the following three key topics:

1. Examining Prior Authorizations

a. The use of prior authorization for SCD therapies varies widely across states and therapies.
b. There are no set standards for prior authorization requirements for SCD therapies.
c. Several states have inadequate reporting of prior authorization criteria and guidelines.

2. Variation in the Use of Step Therapy Protocols

a. MCOs frequently require multiple steps of two or more SCD therapies compared to state FFS programs, which only require one SCD therapy.
b. Some MCO programs include step edits not based on clinical practice guidelines developed by SCD experts.
c. There are limited eligible justifications that providers and patients can use to challenge step therapy requirements.

3. Policy Overview of Opioid Management for Sickle Cell Disease

a. There are limited specific prior authorization criteria for SCD for opioid management across states.
b. Several states have few exceptions for opioid policies for SCD.
c. How one state demonstrates the most restrictive access to opioid management for individuals with SCD.

Introduction

Sickle cell disease (SCD) is a group of inherited blood disorders caused by the presence of an abnormal form of hemoglobin known as Hemoglobin S. Individuals with SCD experience acute and chronic complications, including vaso-occlusive crises (VOCs), which may require multiple hospitalizations and organ damage. According to the Centers for Disease Control and Prevention, an estimated 100,000 Americans live with SCD, which occurs in about 1 out of every 365 Black or African American births and about 1 in every 16,300 Hispanic American births. For years, the SCD population has been plagued by racism, bias, and stigma within and outside the healthcare system that has negatively affected access to care, treatment, health, and outcomes. Individuals living with SCD often have considerable unmet health needs, and with the absence of cohesive policy to address care gaps, health inequities persist.[1]

SCD is experiencing a historic moment where new therapies are becoming available for patients. With newfound treatment options available, it is essential to evaluate the role of the payer and coverage policies across the U.S. and understand their role in supporting fair access to SCD therapies. SCD patients and caregivers often report challenges that impact their ability to have timely access to care or result in a high burden of out-of-pocket costs. Almost 50% of individuals of the estimated 100,000 individuals living with SCD in the U.S. are covered by Medicaid.[2] There is an emerging debate about whether insurance coverage provides fair access to therapies for SCD.

As part of our commitment to expanding access and increasing advocacy opportunities, Sick Cells is working to gain insight into what factors decision-makers do—and do not—consider when determining access criteria for SCD therapies. This information helps inform the broader picture of access and advocate for state efforts to improve health equity for SCD patients.

[1] National Academies of Sciences, Engineering, and Medicine. 2020. Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action. Washington, DC: The National Academies Press. https://doi.org/10.17226/25632

[2] Bazell et al. "A claims-based analysis of sickle cell disease: Prevalence, disease, complications and costs, Considerations for commercial and managed Medicaid payers." October 2019. https://www.milliman.com/-/media/milliman/pdfs/articles/a_claims_based_analysis_of_sickle_cell_disease_prevalence_disease_complications_and_costs.ashx?la=en&hash=E8361366C9BFED00A66001CCC646B42F

To provide context to these debates, Sick Cells conducts an annual Medicaid Access and Landscape Review to explore state and managed care documents that outline coverage criteria and answer three key questions:

- What is the current coverage status in Medicaid programs for common therapies and opioid management used to manage sickle cell disease?
- How are prior authorization protocols and step therapy requirements used across state Medicaid programs?
- Do Medicaid programs provide adequate transparency into coverage criteria and decision-making processes?

An environmental scan of coverage criteria under state and managed care organizations. These results can serve as a tool for assessment and as the starting point for dialogue and action to achieve improved access to therapies.

Table 1: SCD Therapies Included in this Analysis

Trade Name	Product Name	Medical or Pharmacy Benefit	FDA Approval Date
Adakveo®	Crizanlizumab-tmca	Medical	November 2019
Droxia®	Hydroxyurea	Pharmacy	February 1998
Endari™	L-glutamine	Pharmacy	July 2017
Oxbryta®	Voxelotor	Pharmacy	November 2019 (Accelerated approval)
Siklos®	Hydroxyurea	Pharmacy	December 2017
Hydrea®	Generic Hydroxyurea	Pharmacy	October 1998

Methodology

Environmental Scan

To assess coverage and access restrictions in state Medicaid programs for therapies prescribed to individuals with SCD, Sick Cells contracted to conduct an analysis of coverage policies in the 50 states and the District of Columbia. Sick Cells identified commonly used pharmacy and medical benefit products indicated to treat complications of SCD and current opioid policies to treat acute and chronic pain from SCD to include in the analysis. Using comprehensive formulary and medical policy data provided by Artia Solutions, augmented by Sick Cells research, we analyzed coverage policies as of February 2023 for state (FFS) programs and managed care organizations (MCOs) for SCD therapies.

Sick Cells and Artia Solutions identified five pharmacy benefit products (Droxia®, Endari™, Oxbryta®, Siklos®, and generic hydroxyurea) and one medical benefit product (Adakveo®) as disease-modifying treatments (DMTs) of SCD. By using comprehensive formularies and medical policy data, we analyzed PDLs, prior authorization criteria, opioid policies, and coverage policies. Exploring the landscape of state Medicaid, Artia Solutions data resulted in 78% of FFS enrolled lives under pharmacy benefits (prescription drugs administered at home) and 64% under medical benefits for SCD.

Artia Solutions provided data categories for utilization management under statewide criteria for preferred drug lists (PDLs), prior authorizations (PAs), step therapy (ST), and specific opioid policies for SCD. Each product fell under categories as 1) unrestricted (i.e., the payer does not require prior authorization or step therapy for the product), 2) requiring prior authorization only, 3) requiring step therapy only, 4) requiring both prior authorization and step therapy, or 5) information is unavailable. (Note: MCOs in states were excluded from the PDL, PA, and opioid management analysis) Step Therapy under state Medicaid programs was analyzed from comprehensive formulary and medical policy data augmented by Sick Cells to explore FFS programs and seven national MCO firms operating affiliate plans in multiple states with broad geographic reach (Centene, Anthem, UnitedHealthcare Community, Molina, Aetna Better Health, CareSource, and AmeriHealth). (Note: Plans without published PA requirements and Hydroxyurea products, including Droxia®, Siklos®, and generic hydroxyurea, were excluded from the ST analysis due to inconsistent data available on step therapy across FFS and MCO programs.)

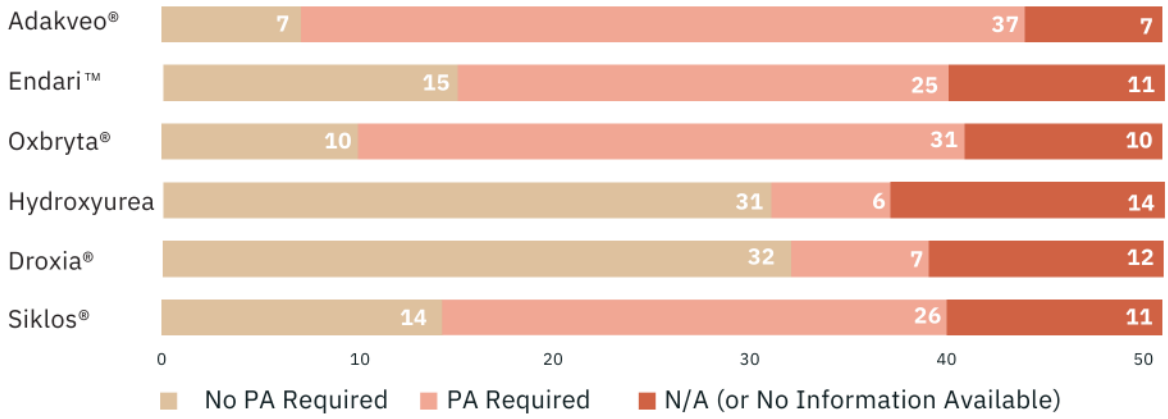
Sick Cells also utilized prevalence data from the Medicaid and CHIP Sickle Cell Disease Report, T-MSIS Analytic Files 2017. This data was used to highlight and analyze access in the top 11 states that report a high prevalence of Medicaid beneficiaries with SCD. These data present a limitation: data reflect trends in Sickle Cell Disease in 2017 and may not account for changes from 2017-2022.

Examining Prior Authorizations for SCD Therapies

The primary function of prior authorization (PA) is to be used as a cost-saving mechanism for payers. Most often, the more expensive drugs have PAs to limit access and are used as a tool for utilization management. PAs are also used as a patient safety tool to verify appropriate access to the appropriate medications. The process to obtain PA varies by payer, and the plans themselves develop their guidelines, such as approval criteria. As past studies have shown that over half of individuals living with SCD use Medicaid as their primary payer, many patients and providers have expressed concerns about PA requirements and processes that can create barriers and delays to treatment. ii

After analyzing the current landscape of prior authorization policies for SCD policies under FFS programs, results have shown that the use of PAs varies widely across states and therapies. For the six SCD therapies, only four states do not require a PA: Alabama, Delaware, New Hampshire, and North Carolina. Among the variability of the requirement of prior authorization, many states require a PA for the newly approved DMTs such as Endari™, Oxbryta®, and Adakveo® (Figure 1).

Figure 1: Prevalence of Prior Authorization for SCD Therapies Across State Medicaid Programs



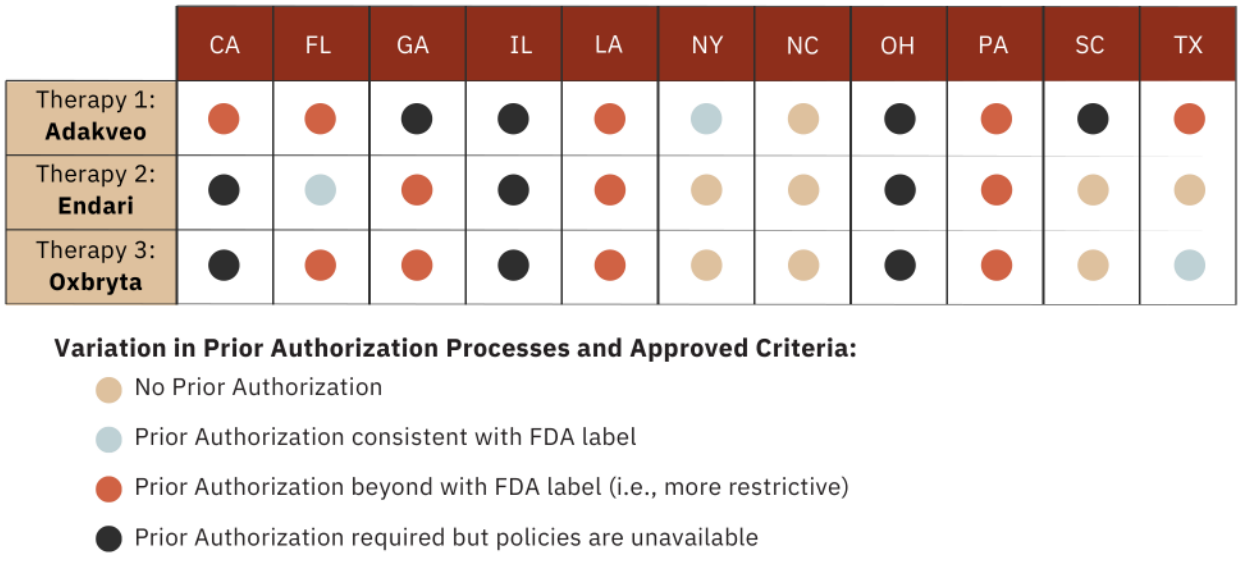
[3] Brandow AM, Panepinto JA. Monitoring toxicity, impact, and adherence of hydroxyurea in children with sickle cell disease. Am J Hematol. 2011;86(9):804–806. doi: 10.1002/ajh.22101

[4] Charache S, Terrin ML, Moore RD, et al.; Investigators of the Multicenter Study of Hydroxyurea in Sickle Cell Anemia. Effect of hydroxyurea on the frequency of painful crises in sickle cell anemia. N Engl J Med. 1995;332(20):1317–1322. doi:10.1056/NEJM199505183322001

The older therapies, which include generic hydroxyurea and Droxia®, show the least barriers to access. In contrast, 26 states require prior authorization for Siklos® despite this being a hydroxyurea-based therapy. Anecdotally, Siklos® has been used widely for the adolescent population unable to swallow pills or by preference. Among youths with SCD, hydroxyurea is associated with lower rates of recurrent pain crises, dactylitis, and acute chest syndrome.[3,4] In conclusion, this can be identified as one of many potential barriers to access to treatment for adolescents and families under Medicaid.

Further into identifying prior authorization criteria, there is a precedent of a need for more standardization across the six SCD therapies. A drug approval by the Food and Drug Administration (FDA) includes guidelines and criteria on eligibility for an individual to access a drug or treatment. However, once these moves in state Medicaid programs, the approval criteria can be the same or changed under state policies. This analysis demonstrated this in selected states for the newer DMTs for SCD. According to the Medicaid and CHIP Sickle Cell Disease Report, T-MSIS Analytic Files 2017, the 11 states that have the highest prevalence of Medicaid beneficiaries with SCD are as follows: California, Florida, Georgia, Illinois, Louisiana, New York, North Carolina, Ohio, Pennsylvania, South Carolina, and Texas. Six of these states have restrictive prior authorization criteria beyond the FDA label for one of the newer DMTs (Figure 2).

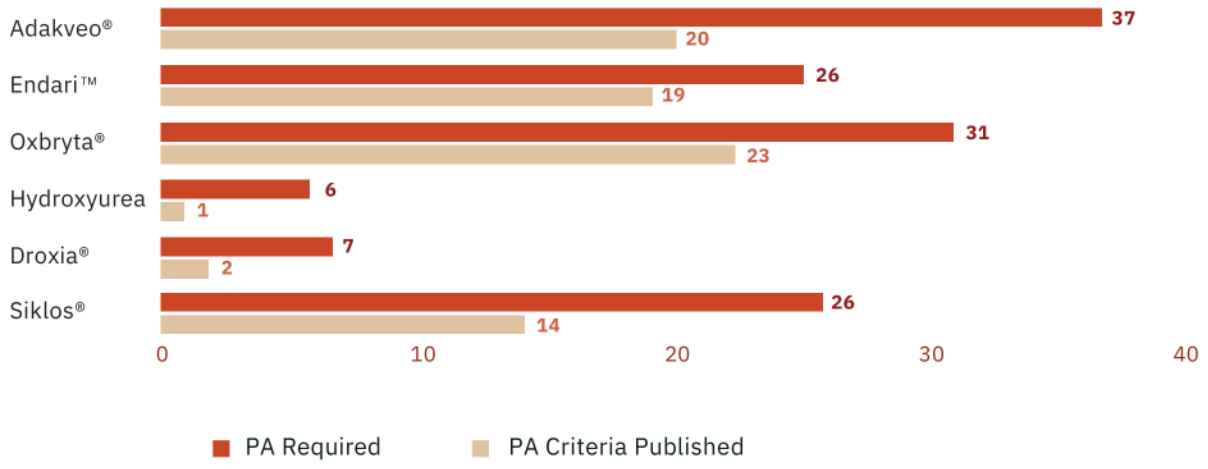
Figure 2: State Medicaid FFS Prior Authorization Policies and Approval Criteria for Three SCD Therapies in 11 Selected States



Many of the restrictive criteria include (1) authorization from a hematologist, (2) step therapy or "fail first" through hydroxyurea, (3) a minimum of medical treatment within 12 months, (4) older age criteria, and (5) documentation of continued clinical benefits to be reauthorized. For example, the requirement of a minimum of medically treated vaso-occlusive crises within 12 months can be problematic as individuals with SCD, on average, report treating six pain crises a year at home.[5] In addition, patients and healthcare providers find having prior authorization information unavailable problematic as healthcare providers are inundated with pages of paperwork and completing these for patients in a timely manner. Five states have prior authorization criteria that are unavailable yet but are required for the newer DMTs. The lack of transparency is a known barrier and provocation in healthcare.

The lack of transparency in state policies and criteria can create challenges for patients and providers to access therapies. As a continuum, insurance policies change year to year, which may bring changes to PDL placement or providers' acceptance of insurance plans. These unforeseen changes can ultimately create barriers to continuity of care. Our analysis across FFS programs revealed discrepancies in prior authorization transparency and availability. Less than half of the states that require PA for generic hydroxyurea have published PA criteria (Figure 3). The many challenges identified through prior authorization can help increase transparency and improve access to state Medicaid programs.

Figure 3: Number of States with Prior Authorization Criteria Published (by Therapy)



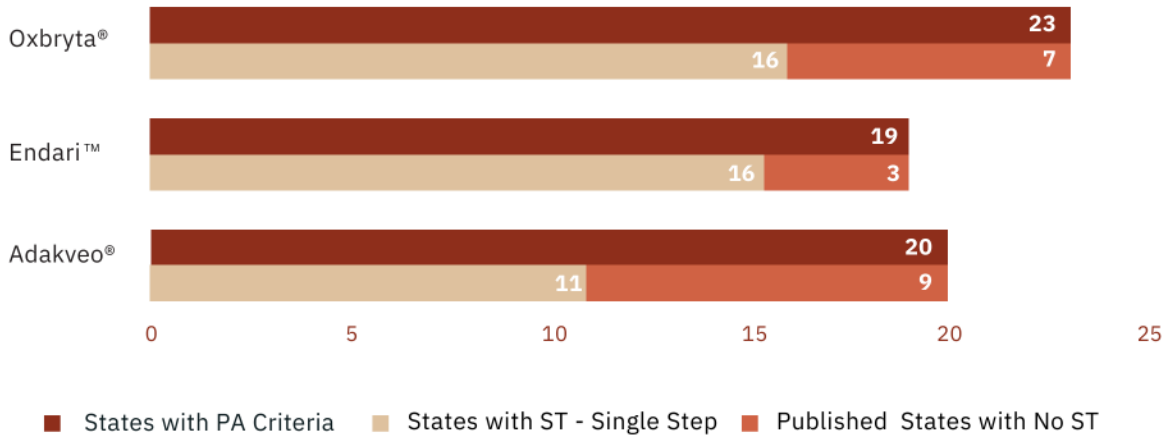
[5] "My Life with Sickle Cell Survey." Sick Cells. 2020. https://sickcells.org/wp-content/uploads/2022/08/SCDAA-Convention-2020_Presentation-Slides.pdf

Variation in the Use of Step Therapy Protocols

Step therapy (ST) as a "fail first" therapy requires a patient to try and fail a preferred therapy before another can be approved and covered by insurance. Insurers widely use this policy as PA criteria and a cost-saving mechanism to control spending for payers. Criteria under ST require the patient to try a lower-cost or generic therapy before trying a newer, more expensive one. However, time, finances, and quality of life are factors in the patients' reasons to choose what is best for their healthcare. Payers may see this differently; therefore, this impacts patient access to life-saving therapies and may cause a delay in treatment, reduce patient adherence, and adverse clinical outcomes.[6]

The current ST protocols under FFS and MCO programs demonstrate many variations across states and address the uncertainty of policy transparency for patients and providers. To begin with SCD therapies, hydroxyurea is a standard step requirement across all FFS and MCO plans requiring ST. A patient must first try hydroxyurea and fail in a single step to access Endari™, Oxbryta®, and Adakveo®. This is illustrated in Figure 4, where within the states with published PA criteria, more than half require a single ST with hydroxyurea.

Figure 4: Over Half of State FFS Programs with Prior Authorization (PA) Require Step Therapy (ST) for SCD Therapies

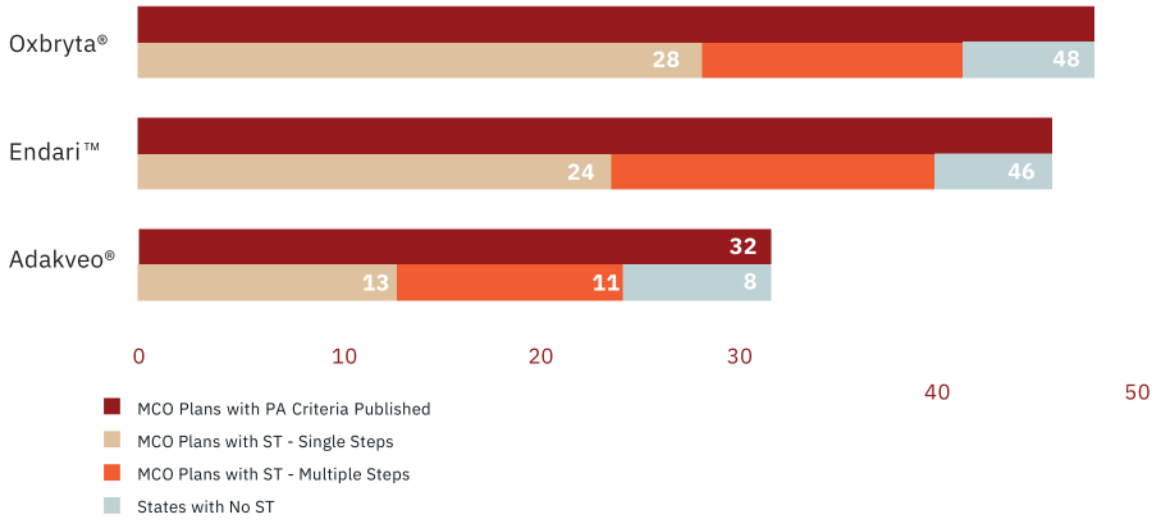


NOTE: A total of 51 FFS programs across 50 states and District of Columbia were reviewed. Plans excluded from this analysis included those without PA or published criteria, those with Adakveo listed as a medical benefit, or those without control of pharmacy benefits.

[6]Sullivan, M., et al. "Step Therapy Can Lead to Higher OOP Costs for Crohn's Disease Patients." Avalere. Retrieved August 15, 2023, from https://avalere.com/insights/step-therapycan-lead-to-higher-oop-costs-for-crohns-disease-patients

MCO plans demonstrate more restrictive ST policies, as one-third of MCO plans require multiple steps for SCD DMTs (Figure 5). Most of these plans require two or more SCD therapies before a patient can access a preferred DMT. With further analysis, Centene, a national MCO firm, controls pharmacy benefits across 14 states. This MCO plan includes three-step criteria for patients to try and fail on hydroxyurea, EndariTM, and Adakveo® before a patient can access Oxbryta®.

Figure 5: Over 75% of MCO Plans Reviewed Require At Least One Step Therapy (ST) Criteria, with One-Third or more Requiring Multiple Steps for SCD Therapies



NOTE: A total of 50 MCO plans were reviewed, representing seven national MCO firms - Centene, Anthem, Molina, UnitedHealthcare Community, Aetna, Better Health, Care Source, and AmeriHealth - with control of pharmacy coverage across 19 states. Plans excluded from the analysis included those without PA or published criteria or those with Adakveo listed as a medical benefit.

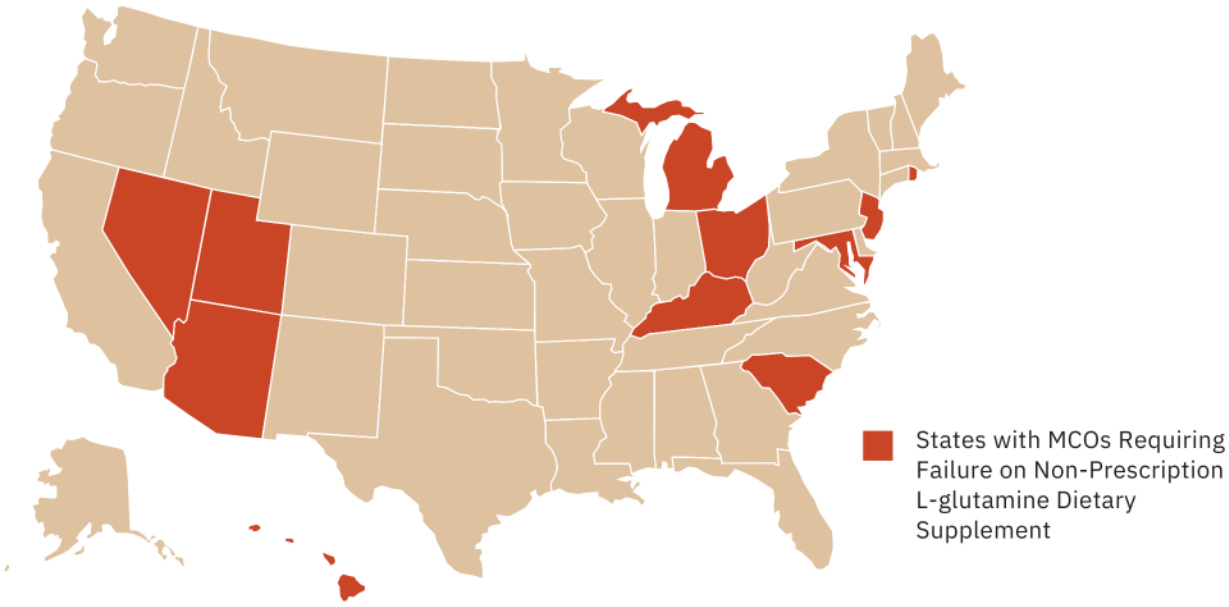
A deeper dive into MCO plans revealed the inclusion of step edits not based on clinical practice guidelines. ST protocols must be based on clinical evidence and prioritize the patients' best interests.[7] However, from our analysis, some MCO plans required a patient to try and fail on an over-the-counter (OTC) dietary supplement of L-glutamine before one can access Endari™. Moline Healthcare cites that Endari™ is not covered due to the lack of clinical effectiveness or superiority over standard L-glutamine dietary supplements, which come in significantly smaller doses than the recommended FDA approval for SCD.[8]

[7] Karmarkar T, Dubois RW, Graff JS. Stakeholders find that step therapy should be evidence-based, flexible, and transparent: assessing appropriateness using a consensus approach. J Manag Care Spec Pharm. 2021;27(2):268–275. doi:10.18553/jmcp.2021.27.2.268

[8] Molina Clinical Policy: Endari (L-glutamine Oral Powder). March 2018. https://www.molinahealthcare.com/providers/wa/medicaid/resource/PDF/endari-glutamine-mcp305.pdf

Due to the insufficient safety and efficacy evidence in OTC dietary supplements for SCD, it is highly inappropriate to request that a patient pay out-of-pocket and try a dietary supplement before accessing an FDA DMT. Many other states have followed these guidelines under 16 MCO affiliate plans from two national firms (United Healthcare Community and Moline Healthcare) (Figure 6).

Figure 6: OTC L-glutamine is Used Inappropriately on ST Protocols for MCO Plans Operating Across 11 States



NOTE: Findings include a total of 16 MCO plans from two national firms (United Healthcare Community and Molina Healthcare) that control pharmacy benefits coverage for patients across 11 states. States that control drug coverage with a single PDL states and carve-out states were excluded from the analysis.

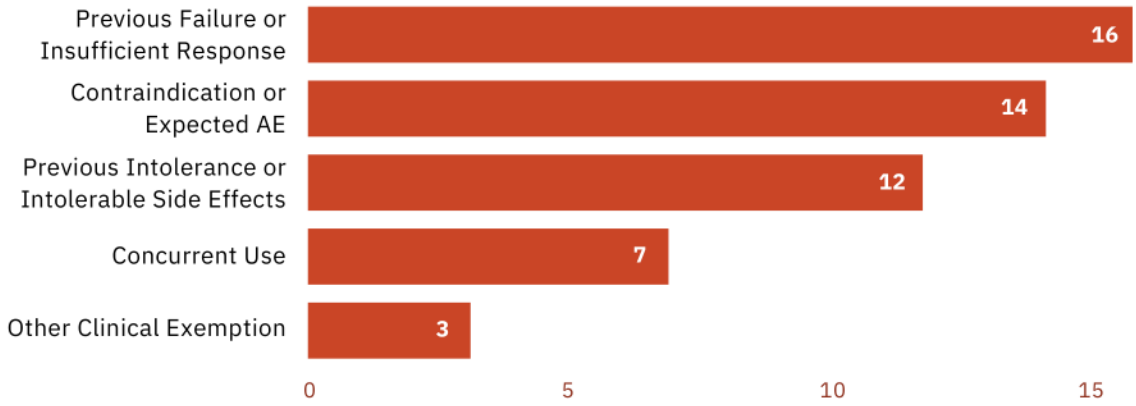
These ST protocols may not be in the best interest of the patient. Part of advocating for your health is building a framework for establishing the patients' preference to remove an ST requirement or exemption. Patient preference is crucial to achieving good treatment adherence and persistence in many chronic diseases.[9] Our analysis showed that the most common ST exemption for Adakveo® under 16 plans was a previous failure or insufficient response (Figure 7). During this analysis, we did not find exemptions if the patient has been stable on Adakveo® under a prior health plan or if the ST was not in the patient's best interest.

[9] Losi, Serena et al. "The role of patient preferences in adherence to treatment in chronic disease: a narrative review." Drug target insights vol. 15 13–20. 8 Nov. 2021, doi:10.33393/dti.2021.2342

Legislation toward ST in Medicaid has been evolving within the past few years. The inappropriateness of ST protocols can be applied to SCD and other chronic diseases. We augmented our analysis with ST legislation that affects Medicaid.[10] Eight states outlined ST legislation that impacts Medicaid policies (CT, KS, LA, NM, NY, OH, OK, TN). Within those eight states, seven states include a determination that the therapy is not in the patient's best interest as an eligible appeal request.

In our analysis, ST protocols heavily affect individuals with SCD as we have multiple-step criteria and inappropriate ST protocols; physicians have reported how these guidelines impact their practice and care for their patients. Past studies have shown that physicians have negative opinions of ST and need to see an increase in the transparency of policies from insurers to prevent confusion before approval and address these policies with patients.[11] These opinions are seen primarily by specialty physicians who see patients with complex conditions who more often require prescriptions restricted from ST policies.[12] These delays and discrepancies can hinder the timely care a physician provides for their patients. For caring for patients living with SCD, this can impede comprehensive treatment plans and, ultimately, quality of life.

Figure 7: Large Variance in the Number and Type of ST Exemptions for Adakveo® Across 16 Medicaid Plans



[10] National Psoriasis Foundation (n.d.). Step Therapy Legislation by State. Retrieved August 15, 2023, from https://steptherapy.com/step-therapy-legislation-by-state/

[11] Fischer, Michael A et al. "Physician Perceptions of Step Therapy Prescribing Requirements." Journal of managed care & specialty pharmacy vol. 25,11 (2019): 1210–1224. doi:10.18553/jmcp.2019.25.11.1210

[12] Lyles CR, Seligman HK, Parker MM, et al., financial strain and medication adherence among diabetes patients in an integrated health care delivery system: the Diabetes Study of Northern California (DISTANCE). Health Serv Res. 2016;51(2):610–24.

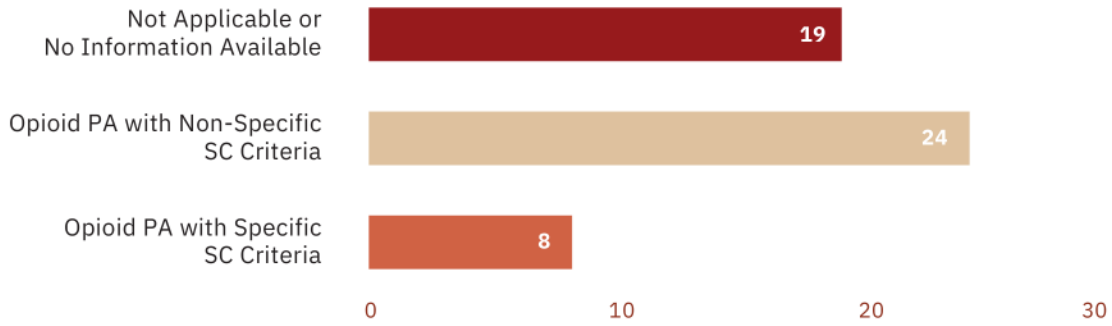
Medicaid Policy Overview of Opioid Management for SCD

As mentioned in this report, states commonly use prior authorization as a cost-saving and patient safety tool. This applies to opioid management as well. SCD is marked by debilitating pain-causing VOCs that may require hospitalization. In addition to these acute events, an estimated 50% of individuals living with SCD experience chronic daily pain that must be treated with long and short-acting opioids.[13,14,15] As we are in a growing opioid crisis, many states have implemented legislative restrictions in limiting prescribing opioids, including controlled substance agreements for patients, urine drug screenings, and prescription drug monitoring programs.[16] As a consequence of the opioid crisis, providers have found the opioid epidemic as a justification to minimize prescribing opioids for sickle cell pain.[17] In our analysis, we examine which states may or may not made efforts to eradicate these limitations for individuals living with SCD that will not disrupt their pain management protocols and quality of life.

Our analysis has shown how states have limited transparency with prior authorization and criteria. Once more, we have seen significant variations in policies for opioid management for SCD. Only eight states have prior authorizations specific for SCD for opioid policies: Arizona, Connecticut, Florida, Georgia, Indiana, Kansas, Kentucky, and Maryland. However, PA approval duration is 365 days compared to six months, applicable to long and short-acting opioids. Over 35% of states have unavailable information on opioid policies, giving rise to limited transparency for physicians and patients (Figure 8).

[13] Bunn HF. Pathogenesis and treatment of sickle cell disease. N Engl J Med.1997;337(11):762–769.
[14] Smith WR, Penberthy LT, Bovbjerg VE, et al. Daily assessment of pain in adults with sickle cell disease. Ann InternMed.2008;148:94–101.
[15] Ballas SK, Lusardi M. Hospital readmission for adult acute sickle cell painful episodes: frequency, etiology, HEMATOLOGY861 and prognostic significance. Am J Hematol.2005;79:17–25.
[16] Dart RC, Severtson SG, Bucher–Bartelson B. Trends in opioid analgesic abuse and mortality in the United States. N Engl J Med. 2015;372(16):1573–1574.
[17] Ballas S. K. (2021). Opioids and Sickle Cell Disease: From Opium to the Opioid Epidemic. Journal of clinical medicine, 10(3), 438. https://doi.org/10.3390/jcm10030438

Figure 8: Prevalence of Opioid Prior Authorization for SCD Across State Medicaid Programs



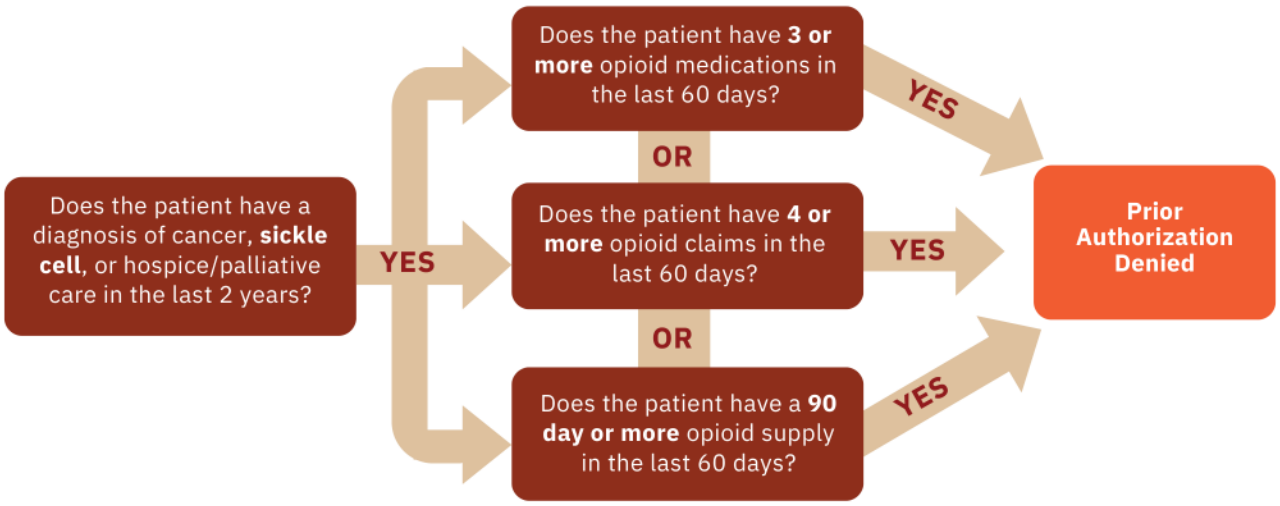
It would be valuable for the states that have a high prevalence of beneficiaries with SCD on Medicaid and CHIP to make accommodations in the opioid policies. The findings reveal the states with few to no opioid exceptions specifically for SCD (Figure 9). Illinois, Ohio, and Texas do not have any exemptions for opioid policies for individuals living with SCD. Louisiana has the most exemptions, including Maximum Morphine Equivalent (MME) exemptions and a total number of prescriptions per month or quantity limits. The absence of exemptions within these states proposes multiple barriers to adequate care and treating chronic pain promptly.

Figure 9: State Medicaid FFS Opioid Exemption Policies in 11 Selected States

	CA	FL	GA	IL	LA	NY	NC	OH	PA	SC	TX
Exemption from Maximum Morphine Equivalent (MME)		●			●					●	
Exemption from Chronic Opioid Treatment Plan and/or Urine Drug Screening			●								
Exemption in Total Number of Prescriptions Per Month or Quantity Limits	●				●	●					
Exemption from Letter of Medical Necessity Required							●				
SCD Diagnosis Required for Approval of Long-Acting Opioids									●		

As one of the states that are absent of opioid exemption policies for SCD, Texas has many restrictive limitations based on the quantity, duration, and type of opioid. Beginning with the PA, SCD is listed as one of the diagnoses. As a patient with SCD, they are limited by three scenarios: (1) does the patient have three or more opioid medications within the last 60 days?; (2) does the patient have four or more opioid claims in the last 60 days?; or (3) does the patient have 90 days or more opioid supply in the last 60 days? If the answer is yes to any of those scenarios, then the PA is denied (Figure 10). For the PA to be approved, the patient should not have any of the three scenarios listed above and, should not have three or more prescribers of opiates in the last 60 days and should not have three or more opiate dispensing pharmacies in the last 60 days.[18] While researching if there is legislation to advocate for individuals with SCD living in Texas for opioids, short-acting opioids should be prioritized over long-acting opioids.[19] This can be problematic for many patients as the combination of long-acting and short-acting opioids is the optimal approach in home-based management.[20] These vacancies in exemptions and sickle cell-specific opioid policies create a platform for advocacy for patients and families to have the ideal pain management plan.

Figure 10: Clinical Flow Diagram for Texas Opioid Prior Authorization Approval Restrictive for SCD



[18] "TX PA Opiate Clin Edit Criteria v17 – Paxpress.Txpa.Hidinc.Com." Texas Opiate Overutilization Clinical Criteria, paxpress.txpa.hidinc.com/opiate.pdf. Accessed 18 Jan. 2024.
[19] "Opioid Legislation and Guidelines." Opioid Legislation and Guidelines, www.exparel.com/hcp/enhanced-recovery/opioid-legislation-map. Accessed 17 Jan. 2024.
[20] Brandow, Amanda M, and Michael R DeBaun. "Key Components of Pain Management for Children and Adults with Sickle Cell Disease." Hematology/oncology clinics of North America vol. 32,3 (2018): 535–550. doi:10.1016/j.hoc.2018.01.014

Recommendations for Stakeholders

The findings from this analysis provide a landscape of many opportunities for improvement and action items for Medicaid directors and stakeholders. The prior authorization process can cause administrative burden with challenges in difficulty determining if a medication requires PA and the payer-specific requirements for review and approval.[21,22] The Centers for Medicaid and Medicare Services (CMS) supports the prior approval process by proposing that payers respond within 72 hours for urgent requests.[23] According to a study from the American Medical Association (AMA), 25% of physicians reported that long wait periods for PA approval have led to hospitalization, and 33% reported that pending PA approvals led to a severe adverse event for a patient.[24] Therefore, the following recommendations can create substantial improvements in healthcare for patients and providers of SCD:

- Increase transparency due to policies changing frequently, which may create difficulty in locating providers.
- Standardize PA request forms and criteria to relieve administrative burden.
- Reduce the requirements for PAs and the frequency of policy changes.
- Align PA policies with FDA approvals and labeling.

[21] American Medical Association. 2021. AMA prior authorization (PA) physician survey. American Medical Association. Accessed January 17, 2024. https://www. ama-assn.org/system/files/2021-04/prior-authorization-survey.pdf

[22] Salzbrenner, Stephen G et al. "Perceptions of prior authorization by use of electronic prior authorization software: A survey of providers in the United States." Journal of managed care & specialty pharmacy vol. 28,10 (2022): 1121–1128. doi:10.18553/jmcp.2022.28.10.1121

[23] "Medicare and Medicaid Programs; Patient Protection and Affordable Care Act; Advancing Interoperability and Improving Prior Authorization Processes for Medicare Advantage Organizations, Medicaid Managed Care Plans, State Medicaid Agencies, Children's Health Insurance Program (CHIP) Agencies and CHIP Managed Care Entities, Issuers of Qualified Health Plans on the Federally-Facilitated Exchanges, Merit-Based Incentive Payment System (MIPS) Eligible Clinicians, and Eligible Hospitals and Critical Access Hospitals in the Medicare Promoting Interoperability Program." Federal Register, 13 Dec. 2022, www.federalregister.gov/documents/2022/12/13/2022-26479/medicare-and-medicaid-programs-patient-protection-and-affordable-care-act-advancing-interoperability.

[24] "AMA Prior Authorization (PA) Physician Survey | AMA." American Medical Association, 2022, www.ama-assn.org/system/files/prior-authorization-survey.pdf.

In the PA process, step therapy is a requirement that generally has been a challenge for SCD and many other chronic diseases such as cancer and arthritis. The American Academy of Family Physicians (AAFP) believes that ST protocols should not be mandatory for patients already on a course of treatment as this can risk side effects and hinder adherence.[25] In a recent survey of chronically ill patients who experienced ST, 29% of patients stopped taking medicines due to cost, and 27% of patients stopped taking medication due to insufficient coverage from insurance.[26] These recommendations can create more safety guidelines to improve access and confidence in healthcare:

- Implement ST protocols based on appropriate medical criteria and research from scientific experts.
- Ensure accessible exemptions for patients to challenge ST protocols, including patient preference to exercise their right to health decisions.
- Implement expedited timelines for approvals and appeals in a standard situation to avoid delays in treatment or adverse side effects.

Pain is a hallmark complication of SCD and must be appropriately managed at home and in medical settings. However, from our analysis, the lack of opioid policies for SCD and exemptions can pose many challenges. The percentage of SCD patients treated with opioids in the U.S. far exceeds that of any other country.[27] Unfortunately, individuals living with SCD have been affected by the opioid epidemic and the strict legislation created to manage prescription opioids. Despite the epidemic, the prevalence of opioid misuse and opioid use disorder is not higher in adults with SCD compared to other pain disorders.[28] These recommendations are to support access to opioid management to improve daily chronic pain caused by SCD to improve lives:

- Create accessible exemptions across states in opioid policies due to the uncertainty of chronic pain requiring additional treatment and avoiding hospitalizations.
- Ensure opioid policies are equal to long-acting and short-acting opioids as they are seen in combination for pain management protocols.
- Reduce the amount of requirements to access opioids, including drug monitoring programs and urine screenings, as there is much to fulfill of PA requirements for DMTs.

[25] "Prior Authorization and Step Therapy (Position Paper)." AAFP, 12 Dec. 2019, www.aafp.org/about/policies/all/prior-authorizations.html.
[26] Snow, Jennifer et al. The Impact of Step-Therapy Policies on Patients – XCENDA, 2018, www.xcenda.com/-/media/assets/xcenda/english/content-assets/white-papers-issue-briefs-studies-pdf/impact-of-step-therapy-on-patients_final_1019.pdf.
[27] Makani J, Ofori-Acquah SF, Nnodu O, Wonkam A, Ohene-Frempong K. Sickle cell disease: new opportunities and challenges in Africa. Scientific World Journal. 2013;2013:193252.
[28] Solomon LR. Treatment and prevention of pain due to vaso-occlusive crises in adults with sickle cell disease: an educational void. Blood. 2008;111(3):997-1003.

Conclusion

This report provides an overview of the current access landscape under Medicaid state programs for patients with SCD. This analysis highlighted limits in transparency and potential barriers that can cause gaps in coverage. Many of these challenges affect physicians who spend endless hours caring for patients with SCD. Families are being affected financially, physically, and emotionally by these decisions. Improvements in PAs are needed to reduce the occurrence of gaps in coverage and prevent hindering a patient from receiving adequate treatment. ST can cause adverse consequences, including unforeseen side effects or a decline in adherence. At most, ST protocols must be in the patient's best interest and at the forefront of policy decisions. As chronic pain can affect a patient with SCD at school, work, home, and everyday living, managing pain is key to living a normal life for some. However, our analysis shows the limitations to accessing the best pain management protocols due to policies impeding opioid management. Overall, this study provides opportunities for advocacy for community leaders, patient advocates, and healthcare professionals to support payer decision-makers, such as Medicaid directors, and recommend patient-centered outcomes across all programs. While this study amplified the current state of access to SCD therapies and opioid management, our work has a few limitations:

- This year, we did not include a survey for Medicaid directors. Omitting the survey adds a possible limitation of not including important decision-makers current knowledge of SCD, the associated coverage policies within states for SCD therapies, and outcomes from Pharmacy and Therapeutics (P&T) or Drug Utilization Review Boards (DURBs) that may or may not affect their state sickle cell population.
- A few data points did not have publicly available Medicaid data, such as unavailable prior authorization data (Figure 1), specific prior authorization criteria (Figure 2), and sickle-specific opioid prior authorization within states (Figure 8).

Glossary

Acute pain: The body's response to a medical condition that starts suddenly and is short-lived.[29]

Chronic pain: Pain that continues beyond the time expected and lasts longer than three months. xxviii [28]

Fee-for-Service (FFS): Delivers health services through a state-rendered program where the state pays direct reimbursement to the providers.[30]

Managed Care Organization (MCO): Entity that delivers health benefits and additional services to Medicaid beneficiaries through contracted arrangements with state Medicaid programs.[31]

Pain management: Strategies to effectively cope with pain and improve quality of life. These include pain medication, physical therapy, and mind and body techniques.[28]

Preferred Drug List (PDL): A list of drugs available with no or little utilization management in each state that designates drugs as "preferred" or "non-preferred" under the Medicaid FFS program.

Prior Authorization (PA): A process requiring physicians and other healthcare providers to obtain advance approval from a payer before a service or product is provided to a patient to qualify for coverage. [32]

Step Therapy (ST): A requirement that patients try specific therapies before they can access others (e.g., the patient is required to try the most preferred drug first and may only progress to other therapies if necessary).[33]

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[29] Pain and Pain Management – Adults." Better Health Channel, Department of Health & Human Services, 13 July 2001, www.betterhealth.vic.gov.au/health/conditionsandtreatments/pain-and-pain-management-adults.
[30] Stancil, John. "Medicaid 101 Primer." Artia Solutions. February 2023.
[31] Centers for Medicare & Medicaid Services. 2022. "Managed Care." https://www.medicaid.gov/medicaid/managedcare/index.html
[32] American Medical Association. 2022. "Prior Authorization Practice Resources." https://www.ama-assn.org/practicemanagement/sustainability/prior-authorization-practice-resources.
[33] Centers for Medicare & Medicaid Services. 2018. "Medicare Advantage Prior Authorization and Step Therapy for Part B Drugs." https://www.cms.gov/newsroom/fact-sheets/medicare-advantage-prior-authorization-and-step-therapypart-b-drugs.

Utilization Management: Techniques such as prior authorization and step therapy used by payers to manage costs and ensure appropriate patient care.[34]

Vaso-occlusive Crisis (VOCs): Patients with SCD present with high-intensity pain caused by the polymerization of red blood cells not carrying enough oxygen. This pain can begin from any part of the body but frequently affects the extremities, back, and chest areas.[35]

[34] Buck JA, Silverman HA. Use of utilization management methods in State Medicaid programs. Health Care Financ Rev. 1996 Summer;17(4):77–86

[35] Borhade MB, Kondamudi NP. Sickle Cell Crisis. [Updated 2022 Aug 29]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2023 Jan–. Available from: https://www.ncbi.nlm.nih.gov/books/NBK526064/

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