



An Examination of the 2024 Medicaid Access and Landscape Review for Sickle Cell Disease:

**The Renewal of State Medicaid Programs
and the Advent of Emerging Gene
Therapies**

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Foreword

On December 8, 2023, a historic day for sickle cell disease occurred when the Food & Drug Administration (FDA) approved two cellular gene therapies, Casgevy[®] (exagamglogene autotemcel) and Lyfgenia[®] (lovotibeglogene autotemcel). Notably, Casgevy[®] is the first CRISPR-edited therapy to receive approval. Lyfgenia[®] uses a vector-based gene therapy technology. Together, Casgevy[®] and Lyfgenia[®] represent the first genetic curative therapies for sickle cell disease (SCD).

SCD is a genetic, chronic blood disorder that entails debilitating pain, irreversible organ and bone damage, and complications that affect one's quality of life. Since this chronic disease has been stigmatized for decades, SCD has not seen appropriate funding and research toward disease-modifying therapies. Consequently, many patients living with this disease and their families have been overwhelmed with hurdles to access therapies that can aid in alleviating some of these debilitating complications. Now, we are in a time where we can prevent these complications through gene-modifying technologies so that individuals can live to their utmost potential. However, as more than 60% of patients with SCD use Medicaid as their primary insurance, we continue to see barriers to access, gaps in coverage, and questioning of eligibility when one of the two gene therapies is considered. Sick Cells partnered with Artia Solutions to analyze and juxtapose 2023 access and coverage policies for SCD therapies against the current landscape for Casgevy[®] and Lyfgenia[®] across the United States.

As of October 2024, we conducted an environmental scan of state and managed care organizations under Medicaid to present coverage and access criteria for SCD gene therapies and to identify significant differences in policies related to SCD disease-modifying therapies we saw in 2023. This report outlines the challenges and opportunities for community advocacy to support changes in Medicaid policies. With curative therapies at the forefront of SCD treatment, Medicaid coverage is essential to the agenda of healthy living for the SCD community. The findings from this report can initiate essential discussions and decisions to motivate positive change and highlight SCD as a public health priority.

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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.



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	5
Introduction	6
Methodology	8
Examining Prior Authorizations for SCD Therapies	9
Variation in the Use of Step Therapy Protocols	12
Coverage of and Access to Casgevy [®] and Lyfgenia [®] for SCD	16
Recommendations for Stakeholders	21
Conclusion	23
Glossary	24
Acknowledgements	25
References	26

Executive Summary

Background

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

Landscape Assessment

To assess the barriers associated with accessing disease-modifying and gene therapies for SCD in Medicaid, Sick Cells and Artia Solutions analyzed the Medicaid coverage and access landscape for state Medicaid programs. We conducted an environmental scan to examine the current Medicaid coverage criteria for two products used to treat complications of SCD (Endari™ and Adakveo®) and two genetic curative therapies for SCD (Casgevy® and Lyfgenia®).

Key Findings

The environmental scan findings around the following three key topics:

1. Examining Prior Authorizations between 2023 and 2024

- a. The utilization of prior authorizations has increased for SCD therapies across states.
- b. The lack of standardization among prior authorization requirements remains consistent for SCD therapies.
- c. States have made limited efforts to adequately report prior authorization criteria and guidelines.
- d. Many states under fee-for-service (FFS) programs require two or more documented vaso-occlusive crises (VOCs) within a 12-month period.

2. Variation in the Use of Step Therapy Protocols between 2023 and 2024

- a. Managed care organization (MCO) plans continue to require multiple steps of SCD therapies compared to state FFS programs.
- b. Additional states under specific MCO plans include step edits not based on clinical practice guidelines in research or SCD subject matter experts (SMEs).
- c. Between 2023 and 2024, regional MCO plans have varied requirements for step therapy protocols.

3. Primary Analysis of Casgevy® (Exa-cel) and Lyfgenia® (Lovo-cel)

- a. MCO plans require more step therapy protocols compared to FFS programs.
- b. MCOs and FFS prior authorization criteria include extraneous genetic testing and medical documentation.
- c. In some cases, quality of life is considered a measure of impact and is listed as a prior authorization criterion.
- d. An example of a state that enforces restrictive criteria beyond Food and Drug Administration approved-labeling for both gene therapies is provided.

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 result in 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. It is unfortunate that, since SCD is predominantly among African Americans and Hispanics, the burden of managing a chronic illness while experiencing racial disparities affects access to care and health outcomes.¹ Individuals living with SCD often have considerable unmet health needs, and with the absence of a cohesive policy to address care gaps, health inequities persist.²

The field of SCD research has undergone a significant milestone, with two new cellular gene therapies now available for patients aged 12 and older. As these treatment options emerge, assessing the payer's role and coverage policies nationwide is crucial, focusing on their contribution to ensuring equitable access to SCD therapies. Patients with SCD and their caregivers frequently encounter obstacles that hinder timely access to care and lead to substantial out-of-pocket expenses. Nearly 50% of the approximately 100,000 individuals in the U.S. living with SCD rely on Medicaid.

In line with our dedication to enhancing access and promoting advocacy opportunities, Sick Cells strives to understand which factors decision-makers consider—and disregard—when setting access criteria for SCD therapies. This knowledge contributes to a comprehensive understanding of access issues and supports state initiatives to improve health equity for SCD patients.

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 available SCD therapies to manage SCD and gene therapies for a curative option?
- How are prior authorization protocols and step therapy requirements used across state Medicaid programs?
- Do Medicaid programs provide adequate clarity and transparency into coverage criteria and decision-making processes?

1. Power-Hays, A., Li, S., Mensah, A., & Sobota, A. (2020). Universal screening for social determinants of health in pediatric sickle cell disease: A quality-improvement initiative. *Pediatr Blood Cancer*, 67(1), e28006. <https://doi.org/10.1002/pbc.28006>

2. 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>

This full analysis includes an environmental scan of coverage criteria and utilization management under state fee-for-service programs 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
Endari™	L-glutamine	Pharmacy	July 2017
Casgevy®	exagamglogene autotemcel (exa-cel)	Medical*	December 2023
Lyfgenia®	lovotibeglogene autotemcel (lovo-cel)	Medical*	December 2023

***NOTE:** Gene therapy is restricted to medical benefit in some states. In the analysis, we included all plans with published criteria under medical or pharmacy for Casgevy® and Lyfgenia®.

Methodology

— Environmental Scan —

To assess coverage and access restrictions in state Medicaid programs for therapies prescribed to individuals with SCD, Sick Cells contracted with Artia Solutions to analyze 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 cellular gene therapies 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 October 2024 for state fee-for-service (FFS) programs and managed care organizations (MCOs) for all SCD therapies.

Sick Cells and Artia Solutions identified one pharmacy benefit product, Endari™, and one medical benefit product, Adakveo®, as disease-modifying treatments (DMTs) for SCD, as well as two medical benefit products, Casgevy® and Lyfgenia®, as cellular gene therapies for a curative treatment for SCD. All hydroxyurea products were excluded from this analysis as we found that at least one product is preferred in every state, and there have been no notable changes since 2023. Our analysis began in October 2024 due to the global recall of Oxbryta® (Volextor) for safety concerns and its subsequent removal from state Medicaid formularies. We analyzed preferred drug lists (PDLs), prior authorization criteria, and coverage policies using comprehensive formularies and medical policy data. Exploring the landscape of state Medicaid programs, Artia Solutions data included 83% of FFS-enrolled lives under pharmacy benefits (prescription drugs administered at home).

Artia Solutions provided data categories for utilization management under statewide criteria for PDLs, prior authorizations (PAs), and step therapy (ST) 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. The PDL and PA analysis also included MCOs in states with regional plans. ST 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) and seven regional state-specific MCO plans (New Jersey, New Mexico, Oregon, Maryland, Michigan, and Arizona). All data were compared to the 2023 analysis to quantify the magnitude of changes in utilization management of SCD disease-modifying therapies. (Note: Plans without published PA requirements were excluded from the ST analysis.)

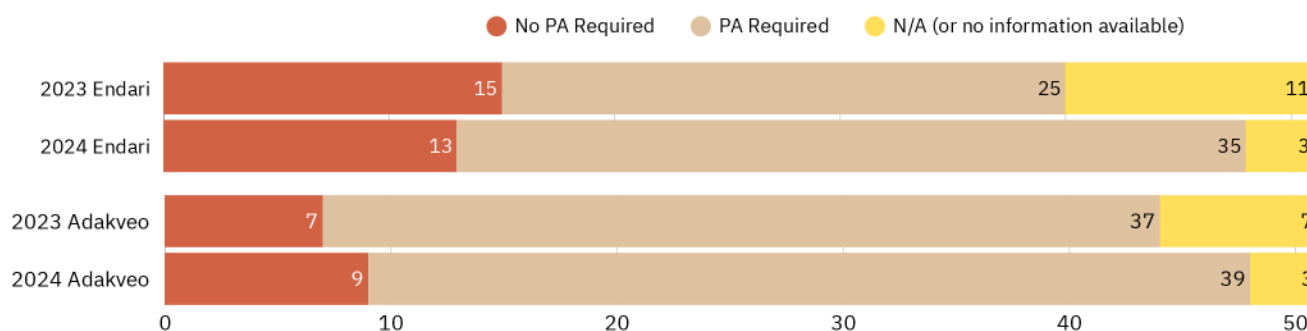
Sick Cells also utilized prevalence data from the Medicaid and CHIP Sickle Cell Disease Report, T-MSIS Analytic Files 2017. These data were used to highlight and analyze access in the top 11 states that report a high prevalence of Medicaid beneficiaries with SCD. However, these data present a limitation: they reflect trends in SCD in 2017 and may not account for changes from 2017 to 2023.

Examining Prior Authorizations for SCD Therapies

Primarily, prior authorization (PA) is used as a cost-saving mechanism for payers. Most often, 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 proper medications. The process to obtain PA varies by payer, and the plans themselves develop their own 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.

After analyzing the current landscape of prior authorization policies for SCD therapies and comparing the 2023 landscape under FFS programs, results have shown that the use of PAs continues to vary widely across states and therapies. Furthermore, the prevalence of utilization of PAs has increased. In 2023, we found that for the six SCD therapies, only four states do not require a PA: Alabama, Delaware, New Hampshire, and North Carolina. That has decreased in 2024 to three states (Alabama, New Hampshire, and North Carolina). As we saw an increase in the use of PAs for disease-modifying therapies (DMTs), there has been a marginal increase in states that do not require a PA for Adakveo[®], from seven states in 2023 to nine states in 2024 (Figure 1).

Figure 1: 1-Year Changes in Prior Authorization Prevalence for SCD Therapies Across State Medicaid Programs



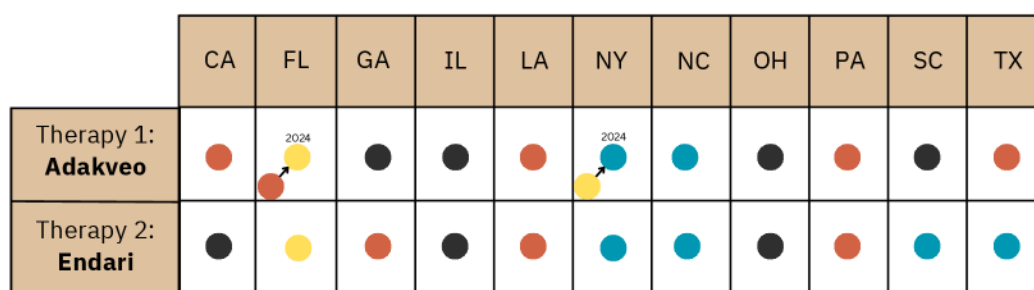
It is essential to highlight that, in 2023, only three states did not have published information available for Endari[™] and Adakveo[®]. As more information is available, this becomes less of a concern for the beneficiary and provider, as they can both be aware of their eligibility criteria.

An examination of prior authorization criteria reveals a growing need for greater standardization among the six SCD therapies. When the FDA approves a drug, it sets forth guidelines and eligibility criteria in the approved labeling to inform patient access to that treatment. However, once these criteria are implemented within state Medicaid programs, they may either remain unchanged or be modified by state-specific policies.

This analysis highlights the policy variations observed in selected states with larger populations of individuals with SCD concerning the newer DMTs for SCD. According to the Medicaid and CHIP Sickle Cell Disease Report, T-MSIS Analytic Files 2017, the 11 states with the highest number of Medicaid beneficiaries living with SCD are: California, Florida, Georgia, Illinois, Louisiana, New York, North Carolina, Ohio, Pennsylvania, South Carolina, and Texas.

Our previous report found six states have restrictive prior authorization criteria beyond the FDA label for one of the newer DMTs.³ Only one of those states has changed its criteria to more consistent guidelines, and one state has changed its policies to be more accessible for Adakveo® (Figure 2).

Figure 2: Changes in State Medicaid FFS Prior Authorization Policies in 11 High SCD Prevalent States



Variation in Prior Authorization Processes and Approved Criteria:

- No Prior Authorization
- Prior Authorization consistent with FDA label
- Prior Authorization beyond with FDA label (i.e., more restrictive)
- Prior Authorization required but policies are unavailable

In Florida and New York, the prior authorization criteria have been updated, extending the authorization period from six months to one year. Additionally, the requirement for documenting clinical response has been eliminated. Observing the positive adjustments being made to PA guidelines in Florida and New York, it remains essential for high-prevalence states to propose similar revisions for SCD therapies.

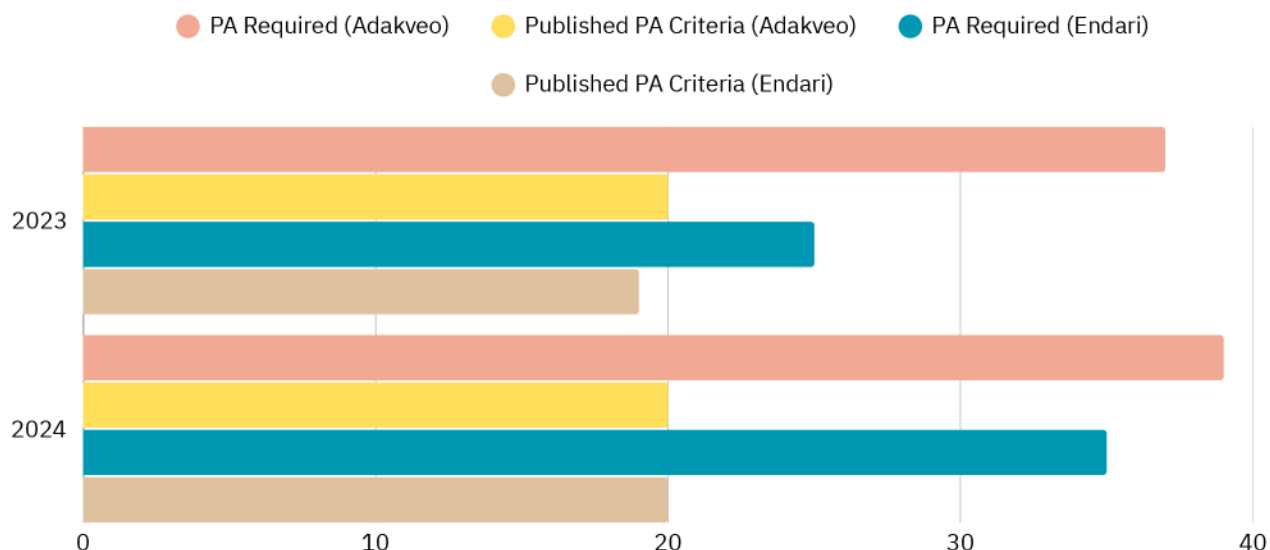
Once again, we observe five states exhibiting prior authorization criteria that are not publicly accessible, akin to Figure 1. The absence of transparency constitutes a recognized impediment and challenge within healthcare.

The lack of clarity in state policies and guidelines poses difficulties for patients and providers seeking therapy access. Our examination of FFS programs indicates that discrepancies regarding prior authorization transparency and availability persist into 2024.

3. Sick Cells. (2023). 2023 Medicaid Landscape and Access Review for Sickle Cell Disease. <https://sickcells.org/wp-content/uploads/2024/04/2023-Medicaid-Access-and-Landscape-Review-for-SCD.pdf>

While the number of states mandating prior authorization has grown, the percentage that has shared published criteria has decreased. Specifically, among the 25 states that require prior authorization for Endari™, only 57% have provided published criteria (Figure 3). Ideally, we envision these figures reaching 100%. Addressing the numerous challenges associated with prior authorization can drive improvements in transparency and access within state Medicaid programs.

Figure 3: Differences in State Medicaid FFS Prior Authorization Required and Published



As we discussed, examining the previously published prior authorization and step therapy protocols will further our conversation about potential obstacles. A common criterion in most prior authorizations is the requirement of two or more vaso-occlusive crises (VOCs) supported by substantial medical documentation. It is crucial to emphasize that VOCs are the primary complications in SCD that clinical trials most frequently target. VOCs are acute pain episodes characterized by intense and throbbing pain, often leaving individuals unwilling to eat and speak, or unable to walk.⁴ The intensity of these crises significantly impairs the quality of life and increases the risk of developing co-morbidities.^{5,6} Experiencing three or more crises within a year is strongly linked to acute chest syndrome and avascular necrosis.⁶ The higher the frequency of VOCs, the greater the impact on work and school attendance, as well as emotional health.⁵

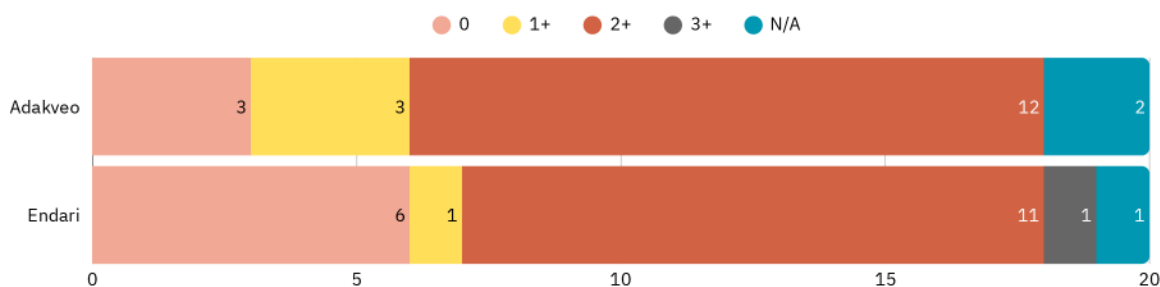
4. Ballas, S. K., Gupta, K., & Adams-Graves, P. (2012). Sick cell pain: a critical reappraisal. *Blood*, 120(18), 3647–3656. <https://doi.org/10.1182/blood-2012-04-383430>

5. Rizio, A. A., Bhor, M., Lin, X., McCausland, K. L., White, M. K., Paulose, J., Nandal, S., Halloway, R. I., & Bronté-Hall, L. (2020). The relationship between frequency and severity of vaso-occlusive crises and health-related quality of life and work productivity in adults with sickle cell disease. *Quality of Life Research: An International Journal of Quality of Life Aspects of Treatment, Care and Rehabilitation*, 29(6), 1533–1547 <https://doi.org/10.1007/s11136-019-02412-5>

6. Bailey, M., Abioye, A., Morgan, G., Burke, T., Disher, T., Brown, S., Bonner, A., Herquelot, E., Lamarsalle, L., & Raguideau, F. (2019). Relationship between vaso-occlusive crises and important complications in sickle cell disease patients. *Blood*, 134(Supplement_1), 2167. <https://doi.org/10.1182/blood-2019-131721>

Among the 20 state FFS programs that have published PA criteria, more than half mandate that patients provide documentation of two or more VOCs experienced in the past year to receive approval for Endari™ or Adakveo® (Figure 4). This documentation must include details of all VOCs treated in a medical facility and the administered doses of ketorolac, a widely used nonsteroidal anti-inflammatory drug (NSAID), and narcotics. One state (Arkansas) requires three or more VOCs to be documented in a 12-month lookback period. This is important as Sick Cells has found that many individuals living with SCD treat half of their VOCs at home within a 12-month period due to many reasons, including past medical trauma in emergency departments and stigmatization.⁷ This hinders the preparation of the required documentation. Further, in our analysis, the critical importance of considering patients' quality of life will be a pertinent discussion in proposing changes in policies guiding access to these therapies.

Figure 4: VOCs with Medical Documentation Required Under FFS Prior Authorization



Variation in the Use of Step Therapy Protocols

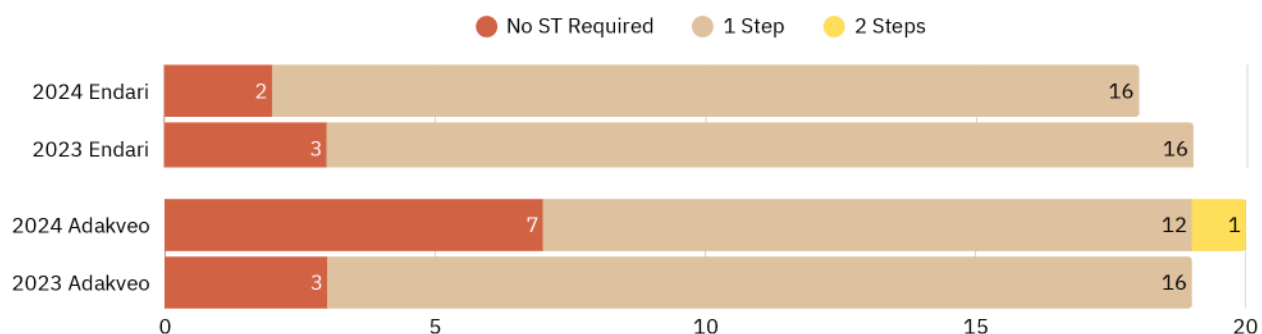
Step therapy (ST), often referred to as "fail first" therapy, mandates that a preferred treatment must be tried and failed for the individual patient before another option can receive insurance approval and coverage. Insurers commonly implement this policy as a PA requirement and as a strategy to reduce costs for payers. ST criteria typically dictate that patients try a less expensive or generic treatment before accessing a newer, pricier alternative. Nevertheless, time constraints, financial situations, and overall quality of life influence patients' healthcare decisions. Payers may have a different perspective on this issue, which has an impact on patient access to critical therapies which potentially leads to treatment delays, reduced adherence to prescribed regimens, and adverse clinical outcomes.⁸

7. Sick Cells. (2020) My life with sickle cell survey. <https://sickcells.org/wp-content/uploads/2022/08/SCDAA-Convention-2020-Presentation-Slides.pdf>

8. 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-therapy-can-lead-to-higher-oop-costs-for-crohns-disease-patients>

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. Hydroxyurea is a standard step requirement across all FFS and MCO plans requiring ST. A patient must first try hydroxyurea and that therapy must fail in a single step to access Endari™ and Adakveo®. This is illustrated in Figure 5, where within the states with published PA criteria, more than half require a single ST with hydroxyurea. There is one state as an outlier from this finding, requiring two steps (Hydroxyurea and L-glutamine) to be eligible for Adakveo®.

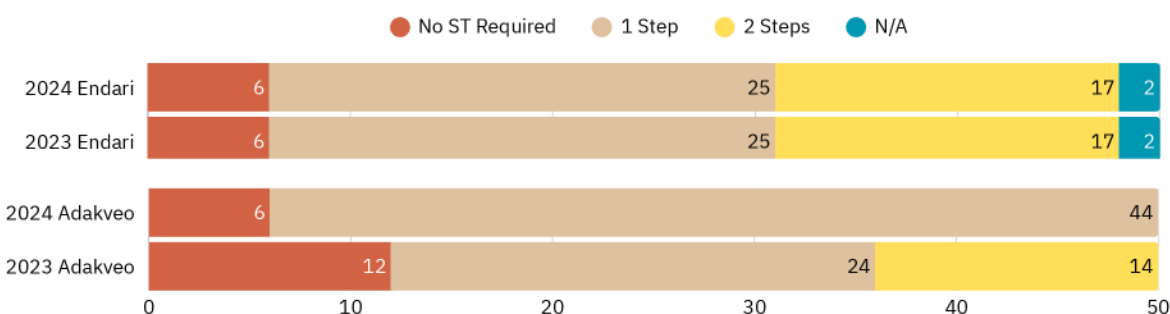
Figure 5: Differences in State FFS Programs with Prior Authorization Requiring Step Therapy for SCD Therapies



NOTE: Plans excluded from this analysis included no published criteria, PA is not required, Adakveo® is indicated as a medical benefit under 51 FFS programs across states and District of Columbia.

As previously found in 2023, MCO plans demonstrate more restrictive ST policies, as one-third of MCO plans require multiple steps for SCD DMTs. Most plans required two or more SCD therapies before a patient could access a preferred DMT. In comparison, 44 MCO plans require one step to access Adakevo® (Figure 6). With further analysis, Centene, a national MCO firm that controls pharmacy benefits across 14 states, has moved from a two-step to a one-step process by eliminating the requirement of having to “try and fail” hydroxyurea with L-glutamine to be eligible for Adakveo®.

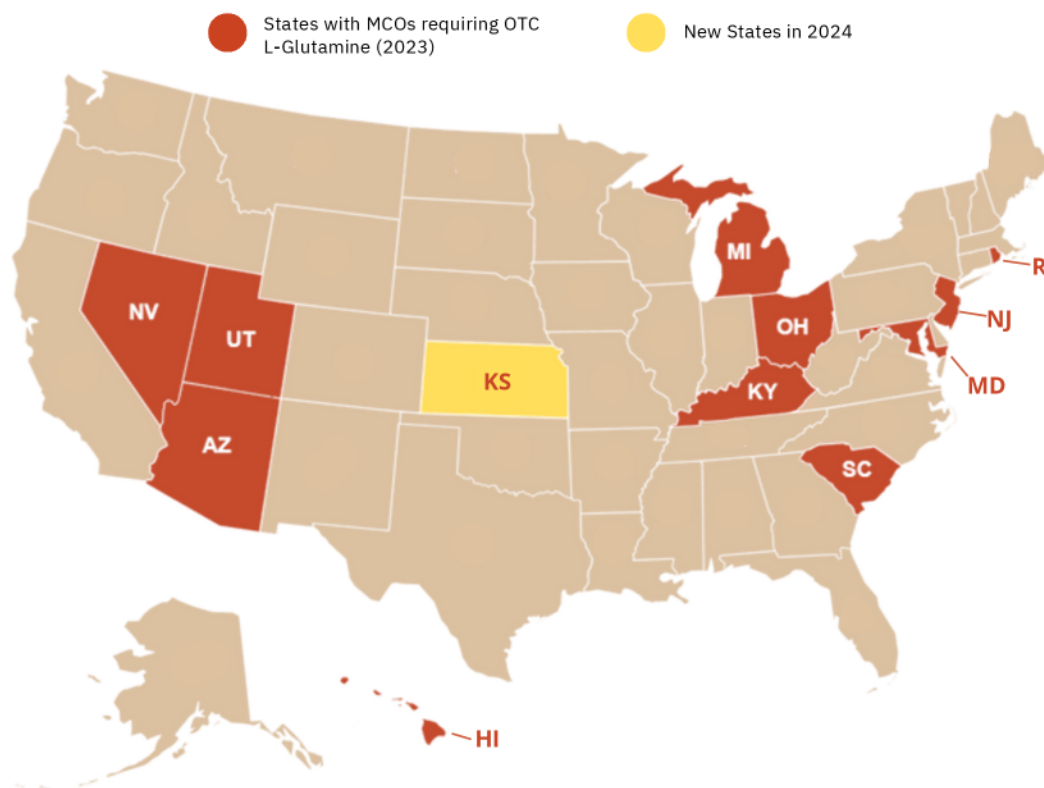
Figure 6: Differences in State MCO Programs with Prior Authorization Requiring Step Therapy for SCD Therapies



NOTE: Plans excluded from this analysis included no published criteria, PA is not required, or Adakveo® is indicated as a medical benefit under 50 MCO plans that represent 7 national MCO networks: Centene (including WellCare), Anthem (including Amerigroup), UnitedHealthcare Community, Molina Healthcare, Aetna Better health, CareSource, and AmeriHealth.

Sick Cells highlighted the inclusion of step edits not based on clinical practice guidelines required by a few MCO plans. ST protocols must be based on clinical evidence and prioritize the patients' best interests. 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™. This continued to be a dismaying finding in 2024. 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.⁹ In a real-world example, one bottle of OTC L-glutamine (500 mg) supplements will cost an average of \$10 per bottle with 120 capsules. Endari™ is administered in 5-gram packets, taken twice daily for 10 grams. To reach the equivalent of a 5-gram packet, a person would need to take 10 or 20 capsules per day. Therefore, requiring an ST policy that does not have the proper pharmaceutical composition, clinical guidelines, and economic standards causes many hurdles in accessing optimal care. Many other states have followed these guidelines under 17 MCO affiliate plans from two national firms (United Healthcare Community and Molina Healthcare) (Figure 7).

Figure 7: OTC L-glutamine Continues to be Used Inappropriately on ST Protocols for MCO Plans in 2024



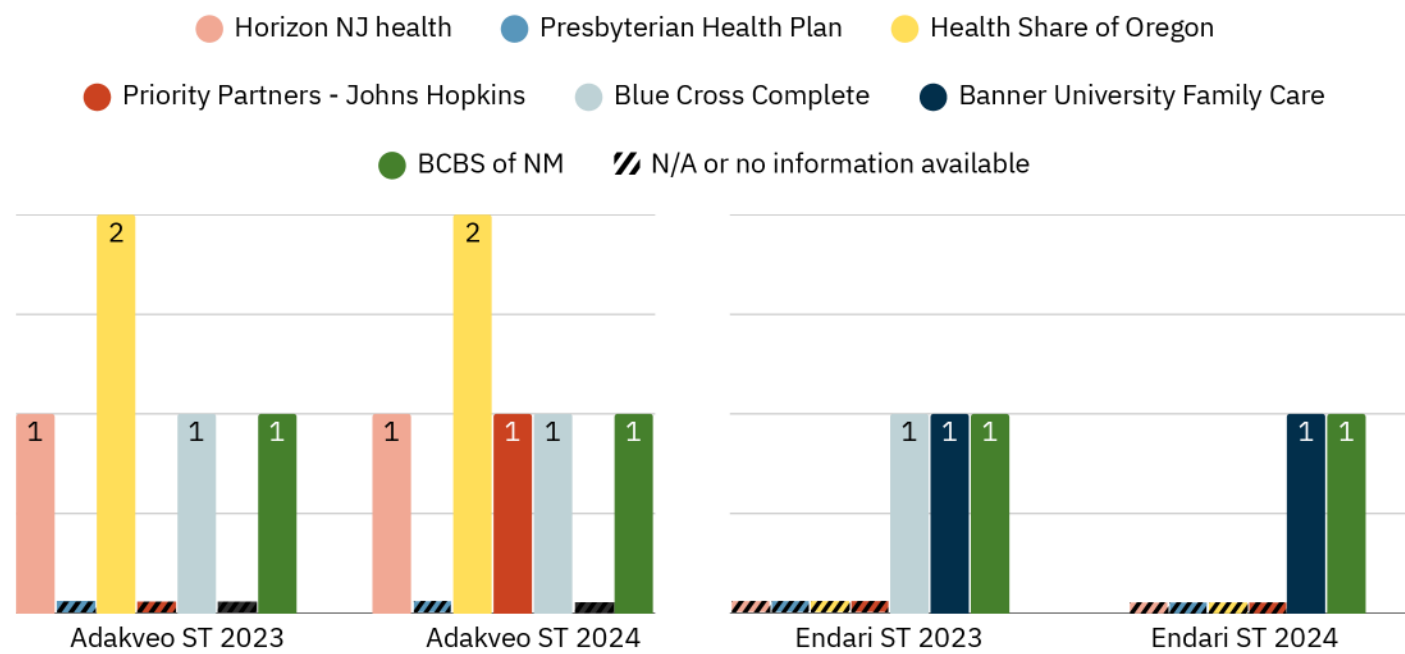
9. Molina Clinical Policy: Endari (L-glutamine Oral Powder). (2018, March).
<https://www.molinahealthcare.com/providers/wa/medicaid/resource/PDF/endari-glutamine-mcp305.pdf>

These ST protocols may not align with the patient's best interests. An essential aspect of advocating for one's health involves constructing a framework that facilitates the establishment of patients' preferences regarding removing an ST requirement or exemption. Patient preference is critical in achieving optimal treatment adherence and sustaining persistence in various chronic conditions.¹⁰

In this analysis for 2024, we conducted a thorough examination of regional MCO plans, which are state-specific programs that furnish coverage and services to beneficiaries within designated regions. Given the state-specific nature of these plans, they often address more tailored healthcare needs in response to community demands, such as dental care and maternal/perinatal health services. However, these plans may not suit individuals who must frequently travel outside the state. Furthermore, individuals may lose coverage and access to in-network providers when seeking treatment outside their state.¹¹

In 2024, access to Endari™ improved when Blue Cross Complete (Michigan) eliminated the requirement to “try and fail” hydroxyurea (Figure 8a). However, eligibility for Adakveo® under certain regional plans can be intricate. For instance, one plan (Health Share of Oregon) necessitates two steps for Adakveo® eligibility: (1) OTC L-glutamine (GlutaSolve®) OR Endari™ must be tried and failed, AND (2) hospitalizations due to SCD remain uncontrolled with hydroxyurea within a 12-month period. (Figure 8).

Figure 8: Regional MCO Plans Step Therapy Requirements from 2023 to 2024 for Adakveo® and for Endari™



10. Losi, Serena, et al. (2021, November 8). The role of patient preferences in adherence to treatment in chronic disease: A narrative review. Drug Target Insights, 15, 13-20. doi:10.33393/dti.2021.2342

11. State of New Jersey Department of Human Services. (2020). *NJ Medicaid & Managed Care*. Division of Medical Assistance and Health Services, New Jersey. <https://www.nj.gov/humanservices/dmahs/info/resources/care/>

Additionally, the lack of transparency in published prior authorization criteria can become more problematic when regional MCO plans do not share their criteria, as these plans aim to limit their reach to beneficiaries.

Our analysis indicates that ST has a significant negative impact on individuals with SCD due to multiple-step criteria and inadequate ST protocols. Physicians have highlighted how these ST guidelines influence their ability to care for patients. Previous studies reveal that doctors often hold negative views of ST and express a need for greater transparency in insurer policies to avoid confusion prior to approval and to address these policies with patients.¹²

Specialty physicians, particularly those treating patients with complex conditions, predominantly share these concerns, as such patients frequently require prescriptions limited by ST policies.

These delays and inconsistencies can obstruct the prompt care that physicians aim to provide. For patients living with SCD, these issues can disrupt comprehensive treatment plans and ultimately affect their quality of life.

Coverage of and Access to Casgevy® and Lyfgenia® for SCD

According to this report, individuals diagnosed with SCD as young as 12 are now eligible for curative gene therapies, Casgevy® (Exa-cel) and Lyfgenia® (Lovo-cel), following their 2023 FDA approvals. Both therapies utilize the patient's own stem cells but employ different technologies. Exa-cel is one of the first treatments approved that uses CRISPR/Cas9, a cutting-edge gene editing tool. This technology boosts fetal hemoglobin production, reducing the formation of sickled cells and helping to prevent vaso-occlusive crises (VOCs). Similarly, Lovo-cel also utilizes the patient's stem cells but delivers a functional gene called HbAT87Q through a vector that mimics normal hemoglobin. This enables it to bind oxygen like regular hemoglobin, thereby decreasing the production of sickled cells and alleviating VOCs.

As we discuss the coverage policies for these gene therapies in this report, it is helpful to consider their technologies.

As these are complicated and high-cost treatments, both gene therapies require prior authorization. While it was hypothesized there would not be many states that would have published criteria as states continue to review and decide on the appropriate criteria during Drug Utilization Review Boards (DURBs) and Pharmacy and Therapeutics (P&T) meetings, several

12. Fischer, M. A., Kesselheim, A. S., Lu, Z., Ross, K. M., Tessema, F. A., & Avorn, J. (2019). Physician perceptions of step therapy prescribing requirements. *Journal of Managed Care & Specialty Pharmacy*, 25(11), 1210–1224. <https://doi.org/10.18553/jmcp.2019.25.11.1210>

states have made their criteria public. Between MCO and FFS plans, over 75% of MCO plans with published criteria require step therapy to be eligible for either gene therapy of choice (Figures 9 and 10). Most commonly, the therapy to “try and fail” is a preferred hydroxyurea product. Furthermore, of the states that have published FFS criteria, only one state requires a two-step process for ST, compared to five states controlled by MCO plans.

Figure 9: MCO and FFS Step Therapy Protocols for Casgevy® (Exa-cel)

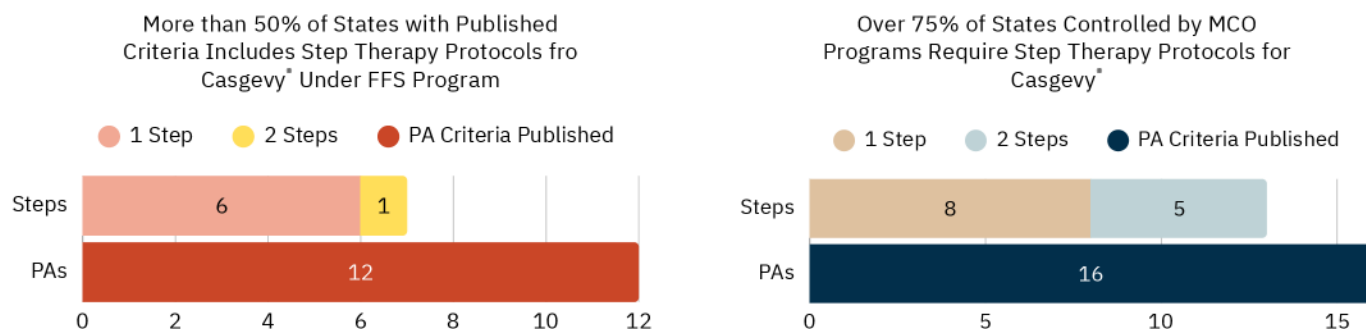
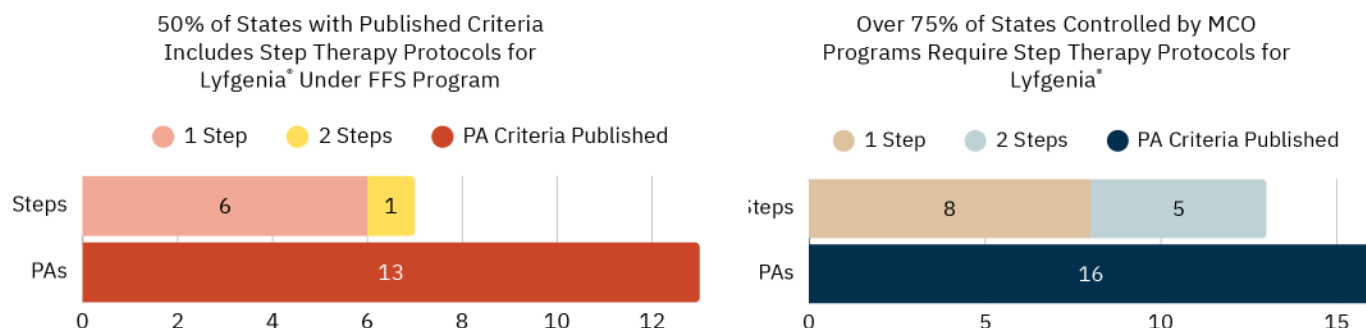


Figure 10: MCO and FFS Step Therapy Protocols for Lyfgenia® (Lovo-cel)



In further analysis of prior authorization requirements, additional criteria and documentation were identified. The analyses assessed if these guidelines are in line with clinical trials and what has been approved in FDA labeling. Between the two gene therapies, there is medical documentation that may require additional genetic testing or may be difficult to document. Casgevy® and Lyfgenia® prior authorizations under FFS and MCO plans require the documentation of the following: (1) ≥ 2 vaso-occlusive crises with medical documentation; (2) matched HLA donor not available for hematopoietic stem cell transplant (HSCT); and (3) reproductive health/fertility counseling. Documentation of psychiatric disorder is the fourth medical documentation requirement found specifically for Lyfgenia®. All of the MCO plans require medical documentation of two or more VOCs that were addressed in a medical facility with the administration of common pain management narcotics for SCD (Figures 11 and 12).

Figure 11: Genetic Testing and Medical Documentation in Prior Authorization Criteria under FFS and MCO programs for Casgevy® (Exa-cel)

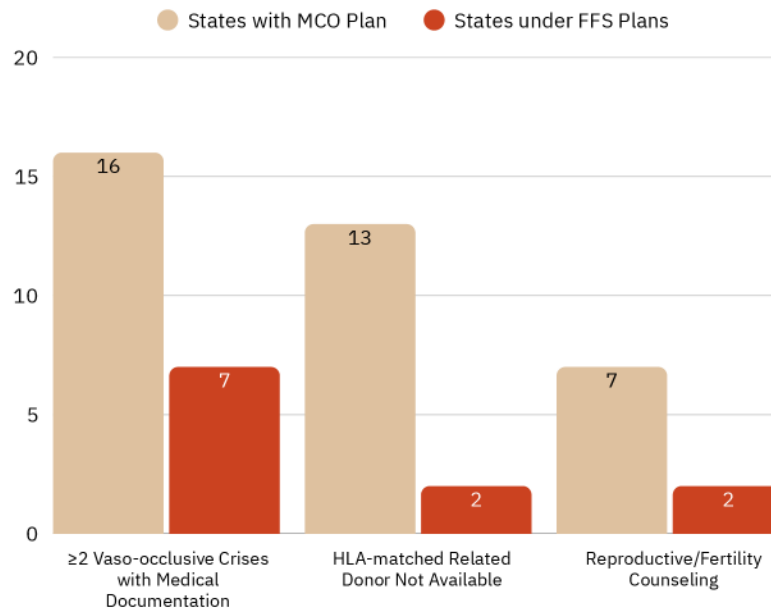
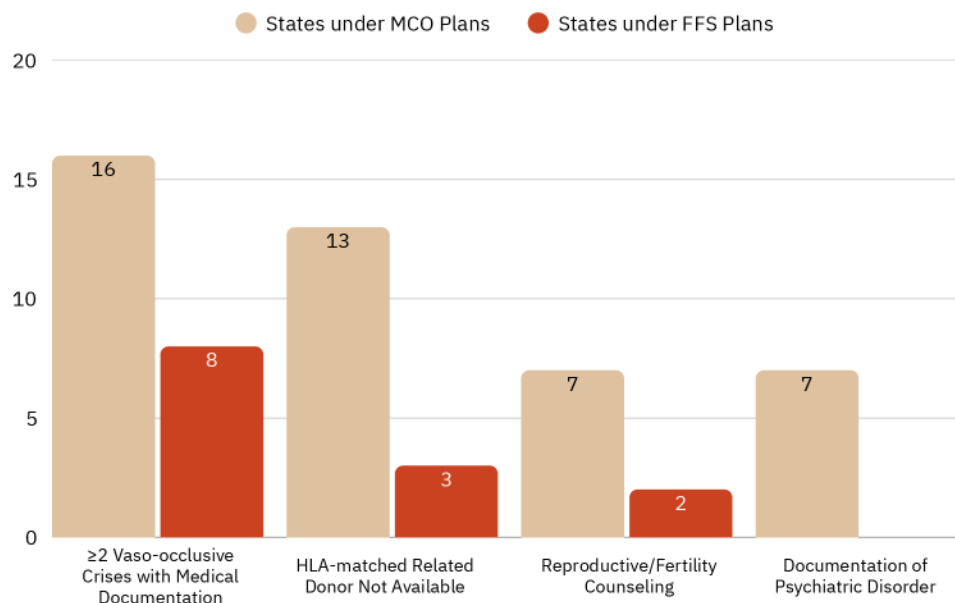


Figure 11: Genetic Testing and Medical Documentation in Prior Authorization Criteria under FFS and MCO programs for Lyfgenia® (Lovo-cel)



As we may recall, many individuals living with SCD address their VOCs at home⁷, making documentation challenging. Furthermore, the biology of these gene therapies is different from a hematopoietic stem cell transplant (HSCT). An HSCT or bone marrow transplant entails administering healthy stem cells to patients who have depleted bone marrow. There are many

13. Khaddour K, Hana CK, & Mewawalla P. (Updated 2023, May 6). Hematopoietic Stem Cell Transplantation. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing. Available from <https://www.ncbi.nlm.nih.gov/books/NBK536951/>

instances where the healthy stem cells are from a donated source, related, or bone marrow donation services. Unfortunately, the odds of finding an 8/8 HLA match for Black patients are only 29%, 48% for Hispanics, and 79% for Caucasians, which can take months or years.¹⁴ The conversation on reproductive and fertility counseling is essential and integral to these therapies. Patients would have to undergo chemotherapy as a conditioning process for the bone marrow to accept the edited stem cells. A risk of this chemotherapy is infertility.¹⁵ Two centers have shared that the youngest individual living with SCD who was exposed to any type of reproductive counseling was 14 years of age, and only four percent of their population had discussions about fertility and SCD.¹⁶ Lastly, under Lyfgenia[®] prior authorizations, the necessity for continued documentation of a psychiatric disorder is broad. Patients with SCD may have a low prevalence of being diagnosed and documented in medical records as having a psychiatric disorder despite an estimated 20-57% of patients with SCD live with depressive disorders, including social isolation, helplessness, and suicide ideation.¹⁷ Assessments of mental health disorders add additional time for the patient and the clinical team when determining an appropriate SCD therapy. Further, access to fertility counseling and documentation of mental disorders can be scarce in many SCD clinics, especially for adolescents and young adults.

Specific to Lyfgenia, this gene therapy has not been studied in patients with more than two α -globin gene deletions.¹⁸ Thus, the package insert that was FDA approved includes a limitation of use for patients with α -thalassemia trait, who may experience complications of chronic red blood cell transfusions. While some FFS and MCO plans require documentation of α -globin deletions (Table 2), it is unclear if the cost of the necessary genetic testing is covered by the insurer. For instance, BlueCross North Carolina states that alpha-thalassemia testing is covered under two conditions: (1) for individuals for whom one parent is a known carrier of alpha-thalassemia or (2) for individuals for whom other testing to diagnose the cause of microcytic anemia has been inconclusive.¹⁹ This is an area where decision-makers can provide transparency for beneficiaries to prepare themselves as they assess eligibility for this curative therapy.

14. BeTheMatch.org. (n.d.) *How does a patient's ethnic background affect matching. Why ethnicity matters when donating bone marrow*. Retrieved January 2025, from <https://bethematch.org/transplant-basics/how-blood-stem-cell-transplants-work/how-does-a-patients-ethnic-background-affect-matching/#:~:text=When%20it%20comes%20to%20matching,complex%20tissue%20types%20than%20others>.

15. National Human Genome Research Institute. (n.d.). *Sickle cell disease gene therapy FAQ*. [Fact sheet].

https://www.genome.gov/sites/default/files/media/files/2022-09/Sickle_cell_disease_gene_therapy_FAQ_0.pdf

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<https://doi.org/10.1016/j.jpog.2023.03.002>

17. Treadwell M. J. (2023). Mental health and psychological resilience in sickle cell disease. *The Lancet. Haematology*, 10(8), e569–e571. [https://doi.org/10.1016/S2352-3026\(23\)00166-7](https://doi.org/10.1016/S2352-3026(23)00166-7)

18. Tisdale JF, Pierciey FJ, Bonner M, et al. (2020). Safety and feasibility of hematopoietic progenitor stem cell collection by mobilization with plerixafor followed by apheresis vs bone marrow harvest in patients with sickle cell disease in the multi-center HGB-206 trial. *Am J Hematol*, E239–E242. <https://doi.org/10.1002/ajh.25867>.

19. Blue Cross NC. (n.d.). Genetic Testing for Alpha- and Beta-thalassemia AHS - M2131 | Providers. Retrieved January 2025, from <https://www.bluecrossnc.com/providers/policies-guidelines-codes/commercial/laboratory/updates/genetic-testing-for-alpha-and-beta-thalassemia>

Table 2: State FFS and MCO Plans that Require α -globin Genetic Testing for Lovo-cel

States under MCO plans	Arizona	Kentucky	Michigan	Nevada	Ohio	South Carolina	Utah
States under FFS plans	Arizona	North Dakota	New Hampshire	Oregon			

Either of the gene therapies can eliminate debilitating VOCs for an individual living with SCD, reduce the burden of disease on society, and, most importantly, improve the individual's quality of life.²⁰ One regional MCO plan highlights the importance of the beneficiary's quality of life. Health Share of Oregon PA criteria address the physical, mental, and social well-being that Casgevy® can bring for one living with SCD. The plan's criteria read as follows:

“Does the request meet the definition of medical necessity per Oregon Administrative Rules, defined as meeting one or more of the following: a. Prevention, diagnosis, or treatment of a disorder that results in health impairment or a disability; OR b. Improve the ability for the member to achieve age-appropriate growth and development; OR c. Establish the ability for a member to attain, maintain, or regain independence in self-care, ability to perform activities of daily living, or improve health status.”

The criteria set by Health Share of Oregon PA are a key model to reference in advocating that Medicaid programs see the importance of access to gene therapies, which can change one's life. In previous discussions with and research by Sick Cells, quality of life has been prioritized for its substantial impact on the patient and families. Casgevy® can ultimately make that significant difference in quality of life.

Overall, it is important to highlight the opportunity to heighten education and awareness for SCD as we analyzed state Medicaid formulary data. This is especially true when we see states that implement restrictive criteria that encompass themes in this report—criteria beyond the FDA label and clinically inappropriate steps.

For example, New Hampshire FFS criteria outline restrictive Casgevy® and Lyfgenia® criteria beyond the approved FDA label. The published criteria read as follows:

20. Raghuraman, A., Lawrence, R., Shetty, R., Avanthika, C., Jhaveri, S., Pichardo, B. V., & Mujakari, A. (2024). Role of gene therapy in sickle cell disease. *Disease-a-month: DM*, 70(7), 101689. <https://doi.org/10.1016/j.disamonth.2024.101689>

“Patient does not have a history of hypersensitivity to dimethyl sulfoxide (DMSO) or dextran 40; Patient is a candidate for autologous hematopoietic stem cell transplant (HSCT); AND Patient does not have a known 10/10 human leukocyte antigen matched related donor willing to participate in an allogeneic HSCT. Patient has symptomatic disease despite treatment with hydroxyurea and add-on therapy (e.g., crizanlizumab, voxelotor, etc.); Patient experienced two or more vaso-occlusive events/crises (VOE/VOC) in the previous year while adhering to the above therapy; Patient does NOT have disease with more than two α -globin gene deletions (per genetic testing for Lyfgenia®)”

Within these conditions, the beneficiary is asked to undergo HSCT eligibility testing to document being unable to find an HLA-matched donor and two DMTs must be tried and failed. Despite the relatively low prevalence of SCD in New Hampshire, these restrictive protocols create channels of advocacy and storytelling of the lived experience about how these criteria are creating multiple barriers for the patient and their families.

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 an administrative burden with challenges in determining if a medication requires a PA as well as understanding 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 a PA approval have led to hospitalization, and 33% reported that pending PA approvals have 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:

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

22. Salzbrenner, Stephen G et al. (2022). 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*, 28(10), 1121-1128. doi:10.18553/jmcp.2022.28.10.1121

23. “Medicare and Medicaid Programs. (2022, December 13). 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*. www.federalregister.gov/documents/2022/12/13/2022-26479/medicare-and-medicare-programs-patient-protection-and-affordable-care-act-advancing-interoperability.

24. American Medical Association. (2022). AMA prior authorization (PA) physician survey | AMA. www.ama-assn.org/system/files/prior-authorization-survey.pdf.

- Enhance transparency as a result of frequently changing policies, the lack of which may create challenges for providers and patients.
- Standardize the prior authorization request forms and criteria in order to alleviate the administrative burden.
- Mitigate the requirements for prior authorizations and decrease the frequency of policy modifications.
- Ensure that prior authorization policies are harmonized with the approvals and labeling provided by the FDA.

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 standard treatment protocols in accordance with appropriate medical criteria and research conducted by scientific experts.
- Ensure that exemptions are available for patients to contest standard treatment protocols, including the allowance for patients to exercise their rights regarding health decisions and treatment preferences.
- Implement expedited timelines for approvals and appeals in standard circumstances to mitigate delays in treatment or adverse side effects.

SCD has historically been poorly represented in the trajectory of biomedical research, especially with curative treatments. Two curative therapies are now available; yet, our analysis shows some challenges to access. Caregivers have published that many patients living with SCD do not have a sibling who is a full HLA-matched donor; therefore, these gene therapies change the curative landscape for SCD.²⁷ From an economic perspective, gene therapies can be an equitable therapeutic option for SCD patients, realizing the value of improving the length and quality of life.²⁸ These recommendations are to support and educate decision-makers on the utmost importance of the need for accessibility and access to gene therapies:

25. American Academy of Family Physicians. (2019, December 12). Prior authorization and step therapy (position paper). www.aafp.org/about/policies/all/prior-authorizations.html.

26. Snow, Jennifer, et al. (2018). *The impact of step-therapy policies on patients – XCENDA*. 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. Enakhumhe J, & Sheikh Y. (2025). Casgevy gene therapy could be life changing for people with sickle cell disease. *BMJ*, 389:r840. <https://doi.org/10.1136/bmj.r840>

28. Goshua, G., Calhoun, C., Ito, S., James, L. P., Luviano, A., Krishnamurti, L., & Pandya, A. (2023). Distributional cost-effectiveness of equity-enhancing gene therapy in sickle cell disease in the United States. *Annals of Internal Medicine*, 176(6), 779–787. <https://doi.org/10.7326/M22-3272>

- Develop standardized access protocols across all states to ensure equity in gene therapy availability and delivery for SCD.
- Provide comprehensive genetic counseling and mental health support as integral components of gene therapy for SCD.
- Promote targeted education campaigns for healthcare providers, patients, and families about gene therapy benefits, risks, and eligibility.
- Enhance transparency as a result of frequently changing policies, the lack of which may create challenges for providers and patients.
- Standardize the prior authorization request forms and criteria in order to alleviate the administrative burden.
- Mitigate the requirements for prior authorizations and decrease the frequency of policy modifications.

Conclusion

This report offers a comprehensive overview of the current access landscape under Medicaid state programs for patients with sickle cell disease. The analysis has underscored deficiencies in transparency and potential barriers that may result in coverage gaps. Numerous challenges affect physicians who dedicate extensive hours to caring for patients with SCD. Families are experiencing financial, physical, and emotional repercussions as a result of these decisions. Enhancements in prior authorization processes are essential to mitigate coverage gaps and ensure that patients receive adequate treatment. Substandard treatment can lead to adverse consequences, including unexpected side effects and decreased treatment adherence. Accordingly, step therapy protocols must prioritize the patient's best interests and take precedence in policy decision-making. With the extreme burden of SCD complications, a one-time gene therapy would be invaluable for a patient and family living with SCD. In addition, our analysis reveals significant limitations in accessing optimal pain management protocols, primarily due to policies that hinder opioid access and management. In summary, this study presents opportunities for advocacy among community leaders, patient advocates, and healthcare professionals to support payer decision-makers, such as Medicaid directors, and to advocate for patient-centered outcomes across all programs. While this study has illuminated the current state of access to SCD therapies and SCD gene therapies, it is essential to acknowledge that our work has certain limitations:

- This year, we did not include a survey of state Medicaid directors. These limitations include the current knowledge of SCD and coverage policies within states and outcomes from Pharmacy and Therapeutics (P&T) meetings or Drug Utilization Review Boards (DURBs).
- A few state data points did not have publicly available Medicaid data.

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.²⁸

Fee-for-Service (FFS): Health services delivered through a state-rendered program where the state pays direct reimbursement to the providers.³⁰

Hematopoietic stem cell transplant (HSCT): An HSCT or bone marrow transplant entails administering healthy stem cells to patients who have depleted bone marrow.¹³

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.

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.

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

29. Better Health Channel, Department of Health & Human Services. (2001, July 13). *Pain and Pain Management – Adults*.

30. Stancil, John. (2023, February). *Medicaid 101 primer*. Artia Solutions

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

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