July 07, 2025

THE CONTINUATION OF VARIATIONS IN THE STEP THERAPY PROTOCOLS AND CLINICAL REQUIREMENTS FOR 2024 UNDER STATE MEDICAID PROGRAMS FOR SICKLE CELL DISEASE

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Summary

This issue brief compares step therapy protocols for sickle cell disease (SCD) modifiers from 2023 to 2024 across state Medicaid programs. We will also explore the clinical presentation of SCD and how this can predict access to these therapies among those living with SCD.

Background

Step therapy (ST), often called a "fail first" policy, is a process that requires an individual to try and fail a different therapy before another one will be covered by their insurance. Typically, the insurer uses step therapy as a **prior authorization (PA)** criterion to require patients to try a lower-cost or generic therapy before "stepping up" to a more expensive or non-preferred therapy. These protocols are used to control spending for health insurance plans; however, they can also impact patient access to life-saving therapies and may cause delayed treatment, reduced patient adherence, or a negative clinical outcome.[1]

States can manage Medicaid coverage through a **fee-for-service (FFS)** model or an external contract with a **managed care organization (MCO)**. Under a FFS model, the state pays directly for each covered service a Medicaid beneficiary receives. Under a managed care model, the state pays a fee to a managed care plan for each person enrolled. Although MCOs provide comprehensive services to beneficiaries, states may **"carve out"** specific services from MCO contracts to FFS systems, such as **pharmacy benefits** (i.e., prescription drugs or therapy administered at home). Additionally, some states use a statewide **preferred drug list (PDL)** to control coverage policies and criteria for pharmacy benefits. With the increasing prevalence of these types of Medicaid models, over 80% of Medicaid beneficiaries across the country have pharmacy benefits managed exclusively by FFS, regardless of whether medical care is managed by MCO or FFS[2], regardless of whether an MCO or FFS model manages medical care.

Methodology

This research aims to assess changes in access and coverage of SCD therapies in state Medicaid programs from 2023 to 2024. Sick Cells in contract with Artia Solutions analyzed coverage policies in the 50 states and D.C. We reviewed policies for two pharmacy benefit products, Endari™ and Adakveo®, indicated as disease-modifying therapies for SCD. Coverage policies from 51 FFS programs (50 states and the District of Columbia), 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) were analyzed as of September 2024. This analysis included a comprehensive comparison of ST policies and published PA criteria. A companion paper called *Prior Authorization Refresh for Sickle Cell Disease Therapies in State Medicaid Programs* provides an overview of prior authorization policies for SCD therapies within FFS programs. Hydroxyurea products were excluded from this analysis as at least one product is preferred in every state Medicaid formulary, and there have been no notable changes in coverage since 2023. In September 2024, Oxbryta® was globally recalled for safety concerns. Thus, Oxbryta® has been removed from state Medicaid formularies. Plans without published PA requirements were excluded from this ST analysis.

Key Findings

We found that in 2024:

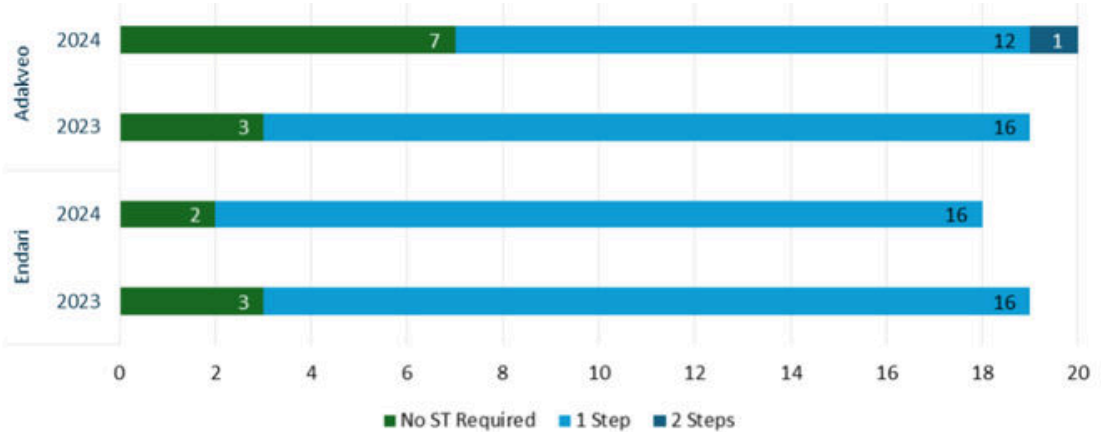
- MCO plans **continue to require multiple steps** of SCD therapies compared to state FFS programs
- **Additional** states under specific MCO plans include step edits not based on clinical practice guidelines in research or SCD subject matter experts (SMEs)
- Between 2023 and 2024, regional MCO plans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

1. Avalere. (2020). Step therapy can lead to higher OOP costs for Crohn's disease patients [Issue Brief]. https://avalere.com/insights/step-therapy-can-lead-to-higher-oop-costs-for-crohns-disease-patients#:~:text=Avalere's%20analysis%20shows%20that%20after,K%20compared%20to%20%24136K.
2. Bissell, C., & Andress, A. (2024, September). Sick Cells Refresh September 2024 [Presentation]. Artia Solutions, Tallahassee, FL, United States.

Finding 1: Compared to FFS programs, the frequency of MCO plans requiring two or more steps of SCD therapies continues in 2024

In 2023, we observed the published PA criteria under FFS programs: Over 50% require failure of hydroxyurea first to access other SCD therapies. These results continued to be observed in 2024. In the 20 states with published criteria, more than half describe utilizing hydroxyurea as the preferred product (**Figure 1**). Therefore, hydroxyurea remains the standard step requirement across all FFS and MCO plans requiring ST. One state (Utah) deviates from this finding, as we report that two disease-modifying therapies (generic Hydroxyurea and L-glutamine) must be tried and have failed before accessing Adakveo®.

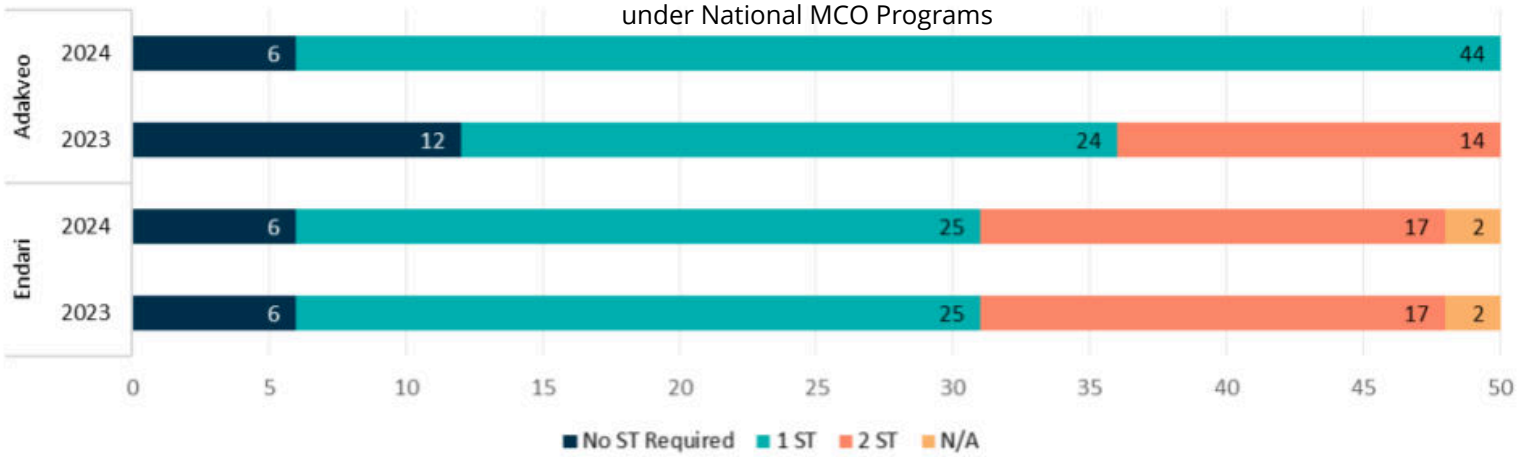
Figure 1: Differences in Step Therapy Requirement from 2023 to 2024 in State FFS Medicaid Programs



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 the District of Columbia.

Access to Endari™ in 2024 is similar to that in 2023; however, for Adakveo®, all MCO plans only require one step (**Figure 2**). This is a positive finding, as we found Centene (the plan that controls the pharmacy benefit in 14 states) **moved from two steps to one step by eliminating the requirement of having to "try and fail" generic hydroxyurea plus L-glutamine** before one could be eligible for Adakveo®.

Figure 2: Step Therapy Requirement for SCD Therapies from 2023 to 2024 under National MCO Programs



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

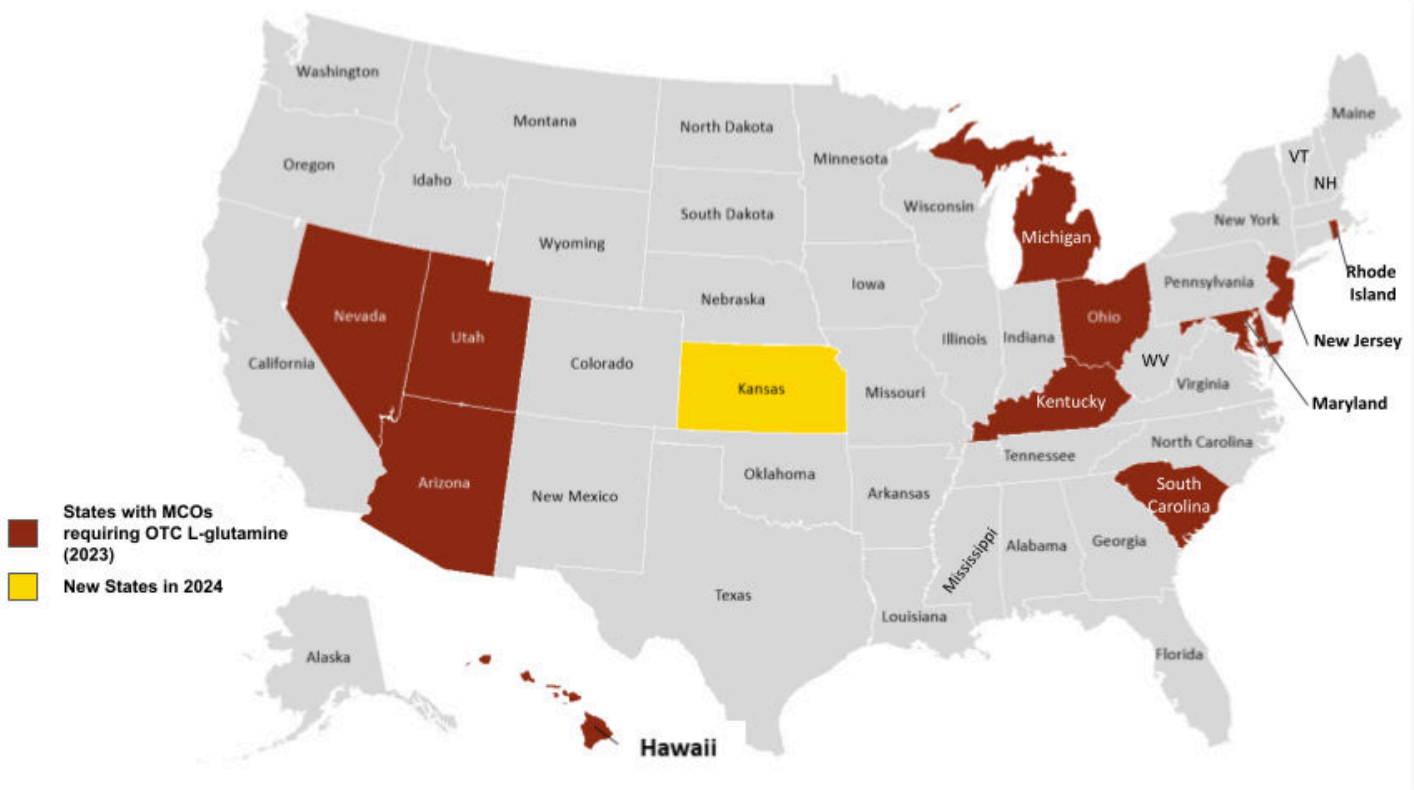
Finding 2: MCO plans requiring step edits that are not clinically based on scientific literature and research have incorporated additional states to their network

In 2023, Sick Cells highlighted an **alarming** ST requirement that potentially creates economic burdens and barriers to access to care. A few MCO plans required beneficiaries to "try and fail" over-the-counter (OTC) L-glutamine dietary supplements to be eligible to access Endari™. Endari™ is the only FDA-approved pharmaceutical L-glutamine therapy for the treatment of SCD. This policy would also require the patient to pay out-of-pocket for the OTC therapy. 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 capsules or 20 capsules per day. Requiring an ST policy that does not have the proper pharmaceutical composition is inconsistent with clinical guidelines and raises economic standards, causing many hurdles in accessing optimal care.

Finding 2: Continued

Seventeen MCO affiliate plans from two national networks (UnitedHealthcare Community and Molina Healthcare) require the patient to "try and fail" OTC L-glutamine. Kansas is the new state (highlighted in yellow) added to the UnitedHealthcare Community network in 2024. These MCO plans control pharmacy benefit access to patients across 12 states (**Figure 3**).

Figure 3: OTC L-glutamine Continues to be Used Inappropriately on ST Protocols for MCO Plans in 2024



Finding 3: Large variability exists in step therapy protocols among regional MCO plans for SCD therapies from 2023 to 2024

In 2023 and in this issue brief, we discussed the variability of ST protocols among state FFS programs and national MCO networks. We took a deeper dive into the regional MCO plans to highlight the specific ST protocols for 2024 and to determine if there are noticeable changes from 2023. Regional MCO plans are state-specific programs that provide coverage and services to beneficiaries in particular regions. Due to the state specificity, some of these plans have more tailored healthcare needs due to community demands, such as dental and maternal/perinatal health. These plans may not be ideal if one travels outside the state frequently. In addition, coverage may be lost along with in-network providers once access to treatment is sought out of state.[3]

More than half of the regional MCO plans require one step to access SCD therapies. In 2024, access to Endari™ showed positive changes as Blue Cross Complete (Michigan) removed the requirement of trying and failing hydroxyurea (**Figure 4a**).

Accessing Adakveo® under some of the regional plans can be complex, as one plan (Health Share of Oregon) requires two steps to be eligible for Adakveo® (**Figure 4b**) by the following criteria:

- OTC L-glutamine (GlutaSolve®) OR Endari™ must be tried and failed, AND
- Hospitalizations due to SCD remain uncontrolled with hydroxyurea within a 12-month period

Reflecting on our 2023 issue brief on prior authorizations, the lack of transparency and unavailability of published criteria raise concerns for providers and beneficiaries.[4] These concerns can be exacerbated when state-specific MCO plans do not have published criteria available when those plans aim to provide a narrower reach to beneficiaries (**Figure 4a and 4b**).

3. 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/

4. Jalowsky, M., Andelson, E., & Scott, M. (2023, June 12). Examining prior authorization for sickle cell disease therapies in state Medicaid programs. Sick Cells. https://sickcells.org/wp-content/uploads/2024/01/Medicaid-Issue-Brief-1.pdf

Finding 3: Continued

Figure 4a: Regional MCO Plans Step Therapy Requirements from 2023 to 2024 for Endari™

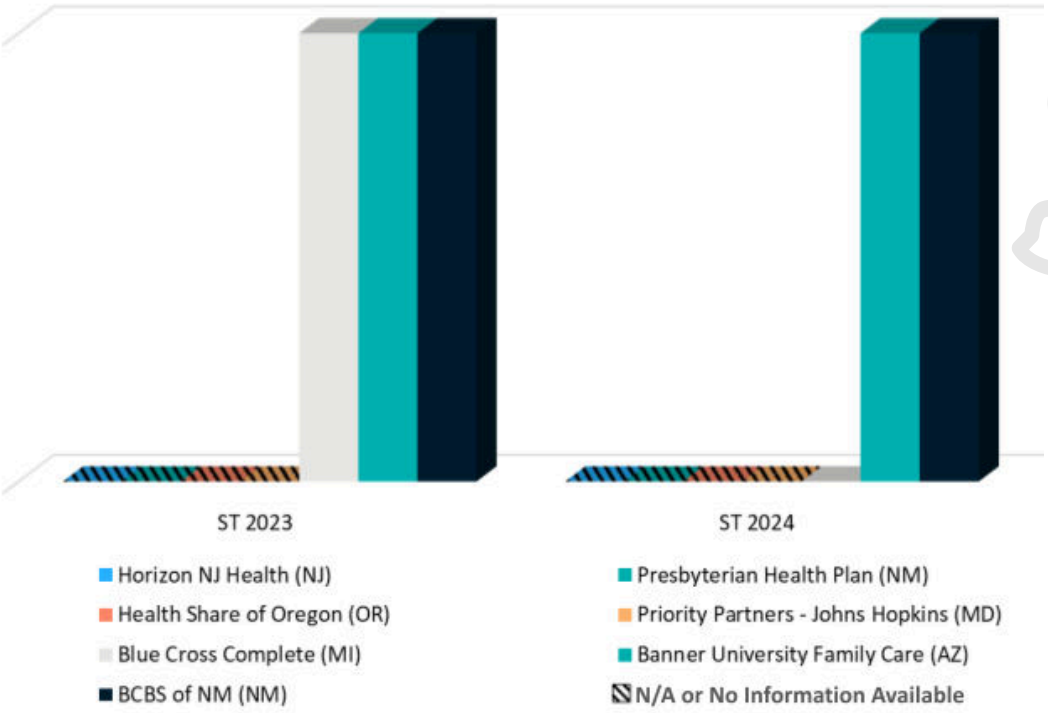
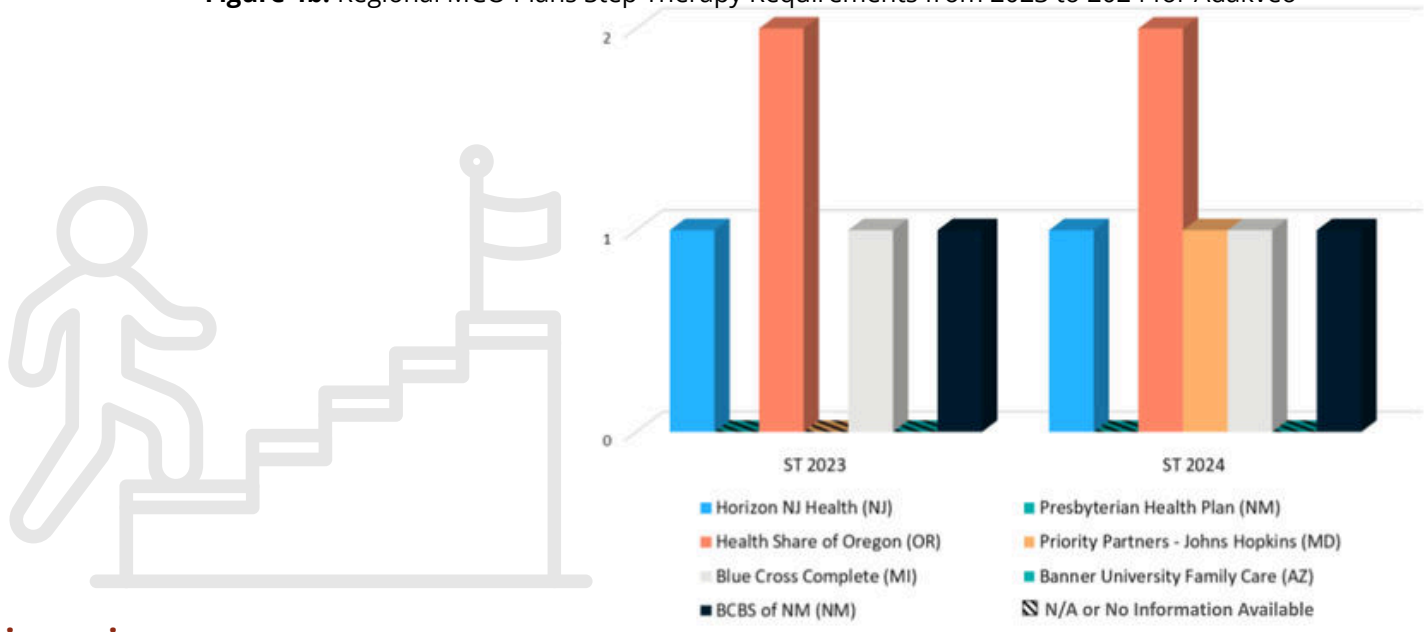


Figure 4b: Regional MCO Plans Step Therapy Requirements from 2023 to 2024 for Adakveo



Discussion

In 2024, we witnessed that step therapy protocols continue to be a potential barrier for Medicaid beneficiaries living with SCD nationally and in individual states. The variations among ST guidelines can introduce more delays and uncertainty for providers and patients. This study has also shown the movement of states toward adopting clinically inappropriate ST guidelines. Opportunities exist for advocates to impress upon payer decision-makers the crucial need for ST protocols that are clinically accurate and accessible. In their advocacy, individuals can successfully highlight states with Medicaid programs that are accessible and employ consistent definitions as prime examples of medically appropriate access and coverage for SCD.

This issue brief was made possible by our sponsors: Chiesi, Agios, Pfizer, and Editas Medicine.