

VARIATION IN THE USE OF STEP THERAPY PROTOCOLS ACROSS MEDICAID PLANS FOR SICKLE CELL DISEASE

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Summary

This issue brief analyzes the use of step therapy protocols for sickle cell disease therapies across state Medicaid programs, and explores limited protections established by state legislation.

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)** criteria 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, nearly 75% 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

Methodology

To assess coverage and access restrictions in state Medicaid programs for therapies prescribed to individuals with sickle cell disease (SCD), Sick Cells contracted Artia Solutions to analyze coverage policies from 51 FFS programs (50 states and the District of Columbia) 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). Sick Cells identified five pharmacy benefit products (Droxia®, Endari™, Oxbryta®, Siklos®, and generic hydroxyurea) and one medical benefit product (Adakveo®) indicated to treat complications of SCD to include in the analysis. Using comprehensive formulary and medical policy data provided by Artia Solutions, augmented by Sick Cells research, we analyzed ST policies, including published PA criteria and step edits, as of February 2023. A companion [issue brief](#) provides an overview of prior authorization policies for SCD therapies with FFS programs. Plans without published PA requirements were excluded from this ST analysis. Hydroxyurea products including Droxia®, Siklos®, and generic hydroxyurea were excluded from the analysis in this issue brief due to inconsistent data available on step therapy across FFS and MCO programs.

Sick Cells also identified and audited state legislation with protections from ST by utilizing existing legislative databases.³ State legislation pertaining to beneficiary protections in Medicaid programs was further analyzed and coded by Sick Cells staff to provide additional context in this issue brief.

Findings

We find that in 2023:

MCOs **more frequently** require multiple steps of two or more SCD therapies when compared to state FFS programs, which only require one SCD therapy

Some MCO programs include step edits **not based on clinical practice guidelines** developed by SCD experts

There are **limited eligible justifications** that providers and patients can use to challenge the requirements of step therapy

State legislation impacting step therapy protocols **establishes limited protections** around appeal processes, response times, and condition exclusions

1. 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>

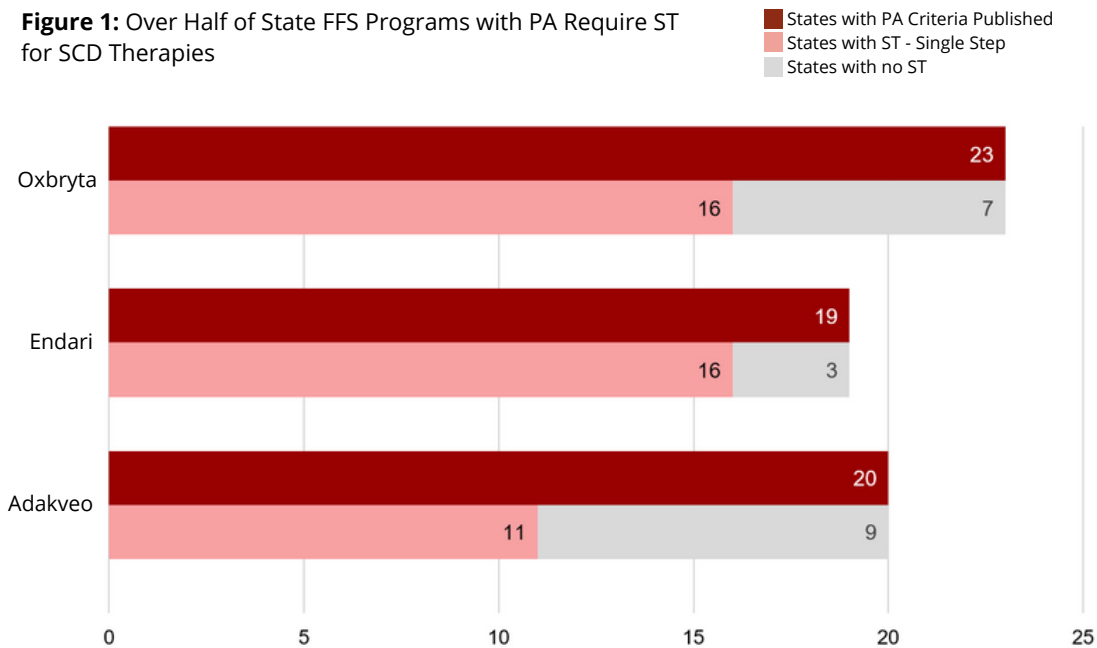
2. Stancil, John (Artia Solutions). “Coverage for Sickle Cell Disease Summit Presented by Sick Cells.” 2022 Coverage for Sickle Cell Disease Summit (Virtual). August 2022. <https://youtu.be/PzQf82CGH7E?t=9666>

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

Finding 1: MCOs more frequently require multiple steps of two or more SCD therapies when compared to state FFS programs, which only require one SCD therapy

Across FFS programs with published PA criteria for SCD therapies, over half of the programs require patients to fail first on hydroxyurea before accessing other SCD therapies [Oxbryta® (69%), Endari™ (84%), and Adakveo® (55%)] (**Figure 1**). Our analysis found that MCOs require a step through hydroxyurea even more frequently, with 88% requiring ST for Oxbryta®, 87% requiring ST for Endari™, and 75% requiring ST for Adakveo®.

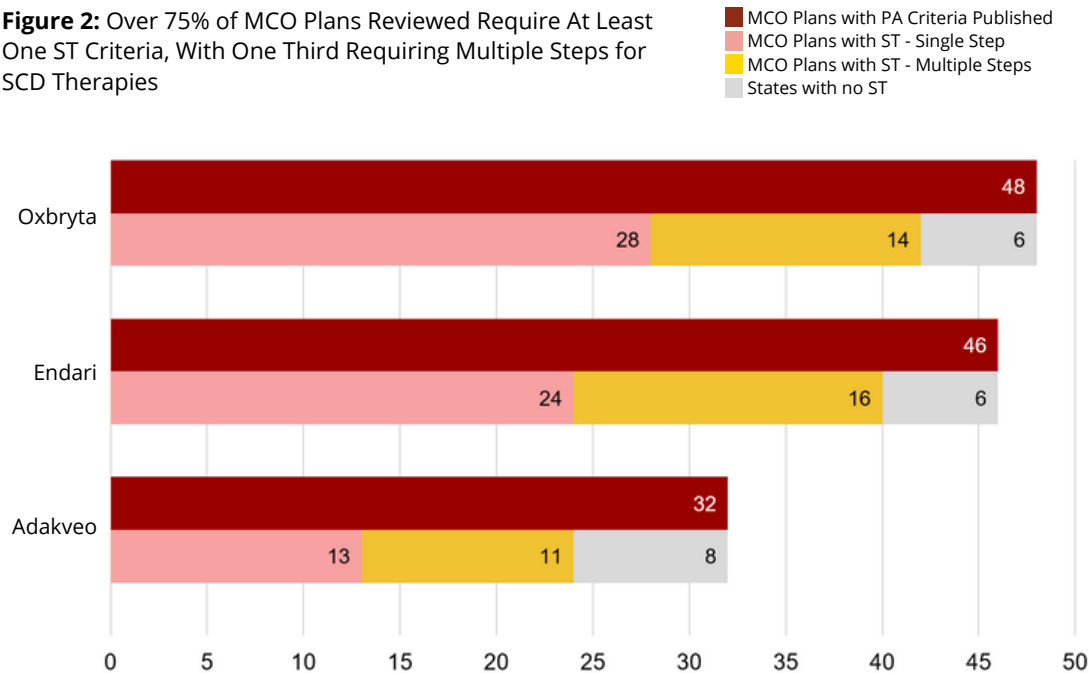
Figure 1: Over Half of State FFS Programs with PA Require ST for SCD Therapies



NOTE: A total of 51 FFS programs across 50 states and the 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.

While the extent and frequency of use vary, **hydroxyurea is a standard step requirement across all FFS and MCO plans requiring ST.** Two FFS programs (Maine and Alabama) require a step through a “preferred drug” in some ST criteria; however, generic hydroxyurea is the only preferred SCD therapy on the PDL for both states. MCOs are often more restrictive, with a third of the MCO plans requiring patients to fail on two or more SCD therapies before accessing their desired SCD therapy (**Figure 2**). One national MCO firm (Centene), which controls pharmacy benefits across 14 states, incorporates a three-step criteria, requiring patients to try and fail on hydroxyurea, Endari™, and Adakveo® before accessing Oxbryta®.

Figure 2: Over 75% of MCO Plans Reviewed Require At Least One ST Criteria, With One Third Requiring Multiple Steps for SCD Therapies



NOTE: A total of 50 MCO plans were reviewed, representing seven national MCO firms -- Centene, Anthem, UnitedHealthcare Community, Molina, Aetna Better Health, CareSource, 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.

Finding 2: Some MCO programs include step edits not based on clinical practice guidelines developed by SCD experts

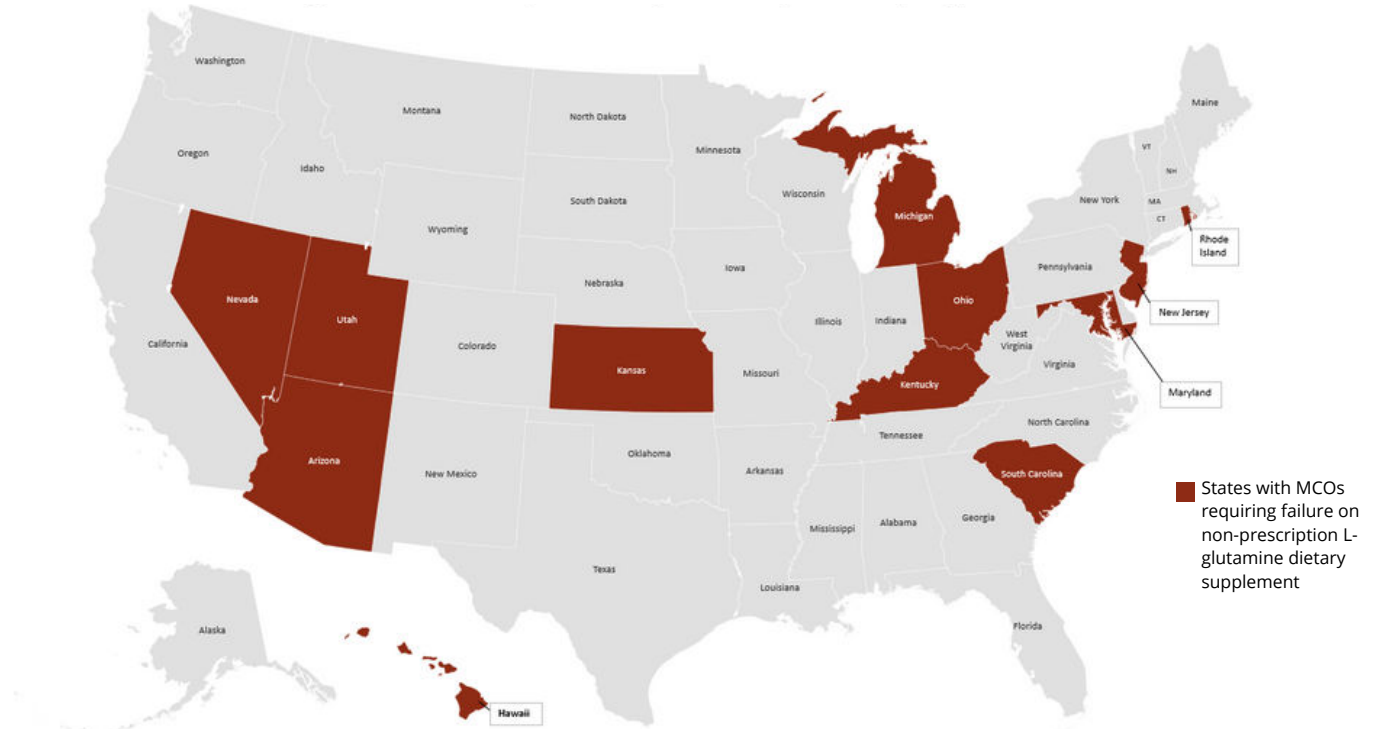
Step therapy protocols should be based on clinical practice and ensure the interests of the patient are front and center. Our analysis revealed some MCO plans require failure on a non-prescription or over-the-counter (OTC) dietary supplementation for L-glutamine before accessing a desired therapy.

Finding 2: Continued

Endari™ is the only FDA-approved pharmaceutical L-glutamine therapy for the treatment of SCD. There is no evidence to support the use of non-prescription L-glutamine dietary supplements for treating SCD or as a replacement for Endari™. Without clinical guidelines or comparative studies to measure the safety and efficacy of pharmaceutical L-glutamine (Endari™) and OTC L-glutamine dietary supplements, a ST policy requiring failure of the latter is inappropriate and not based on medical criteria.

16 MCO affiliate plans from **two national firms** (United Healthcare Community and Molina Healthcare) require the patient to try and fail OTC L-glutamine. These MCO plans control pharmacy benefit access to patients across 11 different states (**Figure 3**).

Figure 3: OTC L-glutamine is Used Inappropriately on ST Protocols for MCO Plans Operating Across 11 States

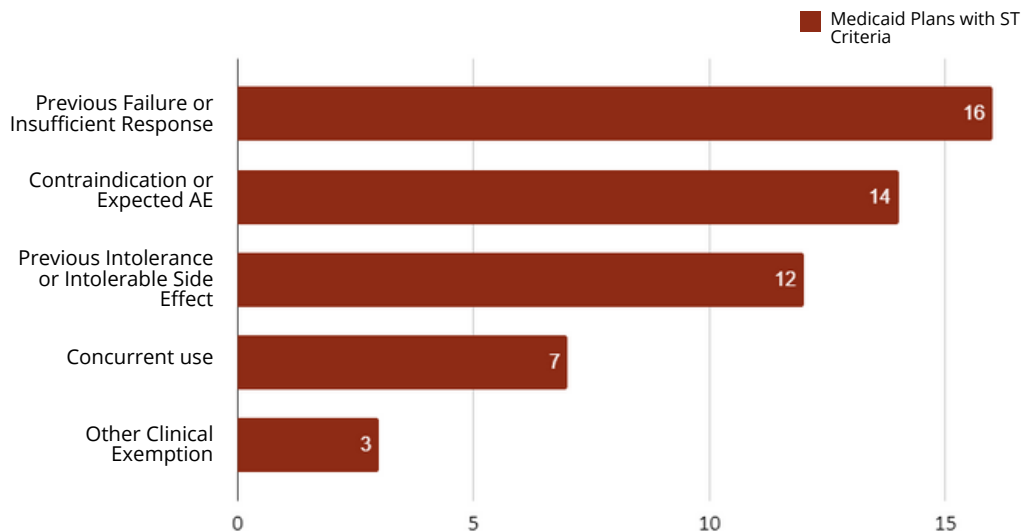


NOTE: Findings include a total of 16 MCO plans from two national firms (United Healthcare Community and Molina Healthcare) that control pharmacy benefits coverage for patients across 11 states. States that control drug coverage with a single PDL states and carve-out states were excluded from this analysis.

Finding 3: There are limited eligible justifications that providers and patients can use to challenge the requirements of step therapy

Establishing a framework for when it is most appropriate to remove a ST requirement or exempt patients from ST is critical to ensuring patients can obtain the therapies they need at the right time. Our review of PA criteria and other coverage policies revealed inconsistencies across Medicaid programs when determining the appropriate circumstances for ST approval or exceptions. **Figure 4** reviews ST criteria to obtain one SCD therapy (Adakveo®) across 11 FFS programs and five national MCO firms and notes the variation in the number and type of criteria across policies.

Figure 4: There is a Large Variance in the Number and Type of ST Criteria (Including Exemptions from ST) for Adakveo® Across 16 Medicaid Plans



Finding 3: Continued

We found that the most common circumstance included as a reason for exemption was if the required prescription drug is **contraindicated or will likely cause an adverse reaction** to the patient (included in **14 of the 16** plans). Only **three plans** included an exemption circumstance for other clinical exceptions, for example, a suspected potential drug interaction. None of the plans discussed exemption if the patient was already stable on Adakveo while on a previous health insurance plan or if the ST was not in the best interest of the patient. Patient preference, such as concerns for one type of medication over another based on side effects or other daily function requirements, was also not included in any of the policies. Without full inclusion of exceptions like these, the use of step therapy protocols is particularly concerning, as these requirements are more likely to interfere with appropriate care for patients.

Finding 4: State legislation impacting step therapy protocols establish limited protections around appeal processes, response times, and condition exclusions

Legislation on utilization management practices has become more popular in the last decade. While this analysis focuses on state legislation for ST, there are also examples of state legislation for PA and federal legislation for ST. As of July 2023, there were **36 states** with active ST legislation impacting private insurers, including eight also impacting Medicaid. This analysis highlights data collected on the **eight** states with ST legislation impacting Medicaid.⁴

While none of the state legislation prohibits the use of step therapy, they provide protections that can reduce burdens on patients and providers. The eight states included in this analysis share common themes across legislation:

- **7 states** require a “clear and convenient” appeal process for step therapy
- **7 states** stipulate allowable response times from the state for standard or urgent appeals (**Table 1**)
- **3 states** mention step therapy exclusions for certain conditions (Connecticut and Louisiana exclude stage IV metastatic cancer, and Kansas excludes multiple sclerosis)
- States outline eligible appeal requests based on: a contraindication (**8 states**), expected ineffectiveness (**7**), past trial and failure of the therapy (**7**), a determination that the therapy is not in the best interest of the patient (**6**), or proof that patient is currently stable using requested or other therapy (**6**)

Table 1: Effect of Appeal Request on Time Limits for Insurer Response in 8 States with Step Therapy Legislation Impacting Medicaid

Effect on Appeal If No Response Within Time Limit	Time Limit for Responses From Insurer: Standard Appeals	Time Limit for Responses From Insurer: Urgent Appeals	State(s)
Denied if no response within time limit	72 hours	24 hours	New York
Approved if no response within time limit	72 hours	72 hours	Kansas
	72 hours	24 hours	Louisiana New Mexico Oklahoma
	2 business days	24 hours	Tennessee
	10 calendar days	48 hours	Ohio
No information in legislation	N/A	N/A	Connecticut

Discussion

Step therapy protocols and protections for SCD therapies vary significantly across state Medicaid programs. Current differences across programs can create confusion and delays for individuals and their providers, particularly those who come on and off coverage, move out of state, or change insurance plans. It is critical for step therapy protocols to be based on clinical practice guidelines and created with input from patients and SCD experts to account for unique needs and preferences. This analysis highlights opportunities for future studies, including effectiveness and potential adverse consequences of ST, inconsistent definitions for failure on SCD therapies, and necessary circumstances for exceptions or appeals. This study also emphasizes advocacy opportunities for community experts to support payer decision-makers and recommend certain patient-centered protections that can be incorporated across state Medicaid programs.

This issue brief was made possible by our sponsors: [Vertex Pharmaceuticals](#), [Chiesi](#), [Agios](#), [Beam Therapeutics](#), and [Editas Medicine](#).

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