

MEDICAID ACCESS & LANDSCAPE REVIEW

FOR PRESCRIPTION
DRUGS TREATING
SICKLE CELL DISEASE

OPPORTUNITIES TO
IMPROVE ACCESS FOR
SICKLE CELL DISEASE THERAPIES

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Foreword

Despite the need to improve care for sickle cell disease (SCD), SCD continues to be deprioritized within the US healthcare system. As the nation strives to innovate and improve care delivery for all Americans, opportunities remain to address patient barriers to needed SCD therapies. This is particularly important in the Medicaid program given the number of Medicaid beneficiaries with SCD. To raise awareness of these opportunities and help state Medicaid programs and their managed care partners act to improve care for SCD, Sick Cells and Avalere Health partnered to build upon their Roadmap for Advancing Care for Sickle Cell Disease (2021) and produce this report on the state of SCD therapy access within Medicaid.

In April 2022, we conducted a survey of individuals with current or recent influence on decisions related to management of SCD therapies in Medicaid, including 15 individuals currently employed at state Medicaid programs. The survey's primary objective was to gain insight into what factors decision makers do—and do not—consider when determining access criteria for SCD therapies. It also sought to inform the broader picture of access to SCD-related services and state efforts to improve health equity for those with SCD. Survey findings were supplemented with an environmental scan of state and managed care documents that outline coverage criteria for SCD therapies. We considered these analyses jointly to illuminate the state of access to SCD therapies in Medicaid—where there are challenges today and how access to therapies could be improved. We hope to start a conversation about key decision points where a change in course could dramatically impact access to therapies and propose recommendations to move in that direction.

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

[Avalere Health](#), a member of Fishawack Health, brings innovative, data-driven solutions to complex healthcare challenges. Avalere is a healthcare consulting firm that operates at the intersection of policy, access, and transformation. We rely on expansive, proprietary data to derive insights and imagine what does not yet exist.

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Executive Summary

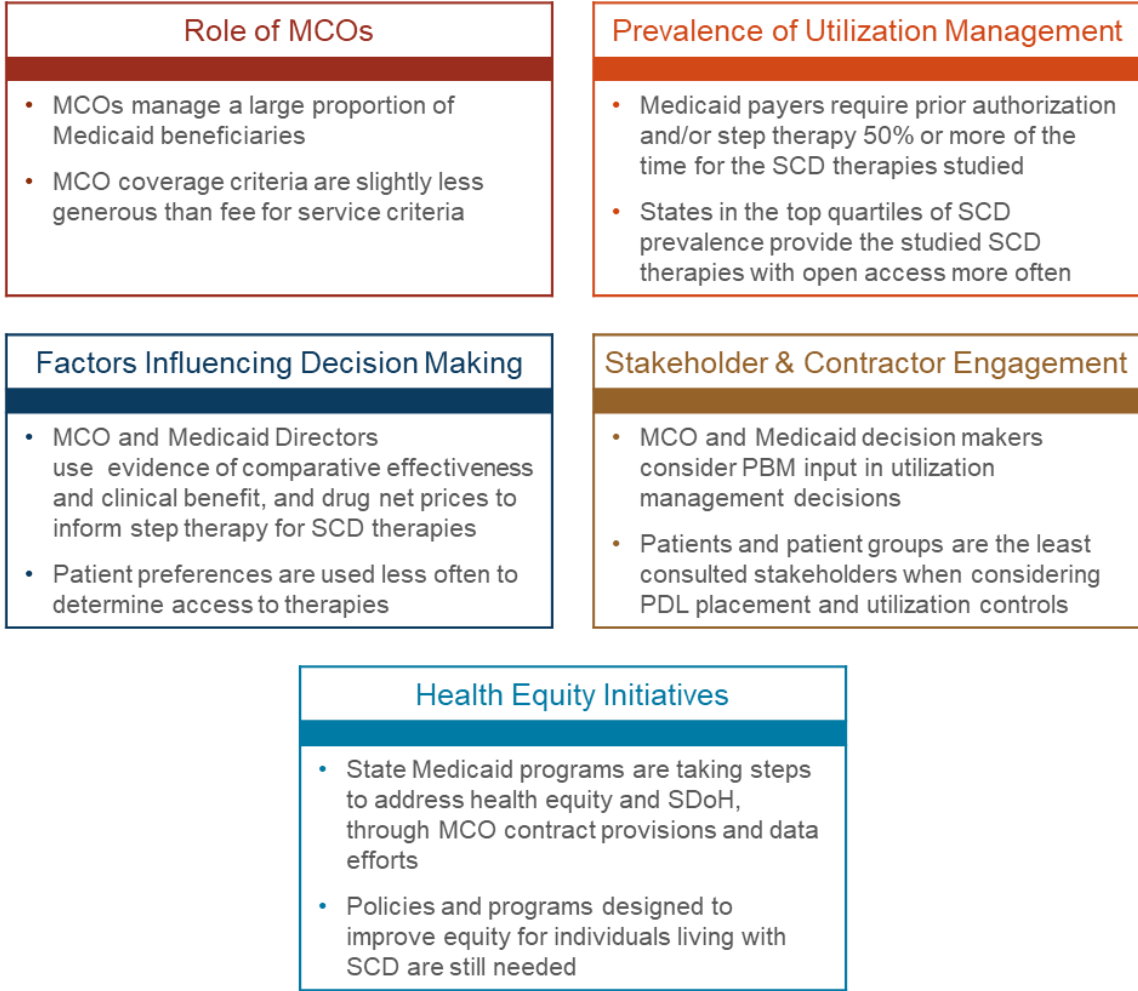
Background

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, acute chest syndrome, various infections, strokes, and organ damage. More than half of the individuals with SCD in the United States rely on Medicaid as their primary insurance.

Landscape Assessment and Decision Maker Survey

To assess the barriers associated with accessing treatments for SCD in Medicaid, Sick Cells and Avalere analyzed the Medicaid coverage and access landscape. We conducted an environmental scan using PlanScape® to examine Medicaid coverage criteria for 5 products that treat complications of SCD (Siklos®, Droxia®, Endari, Adakveo®, and Oxbryta®) and surveyed individuals from state Medicaid programs and Medicaid managed care organizations (MCOs) with recent drug management experience.

Figure 1 – Factors Influencing Decision Making



Key Findings

Across the environmental scan and survey, findings coalesced around 5 key topics:

1. The Role of MCOs

- Many Medicaid beneficiaries nationwide have their care managed by an MCO. Reflected in the survey, 35 of 40 survey respondents reported some or all Medicaid beneficiaries with SCD in their state(s) of purview are enrolled in managed care.
- Aggregated nationally, MCO coverage criteria were found to be slightly less generous than state fee-for-service (FFS) criteria when measured by unrestricted access. However, MCOs list their criteria more often and require the use of both prior authorization and step therapy for a single SCD prescription less frequently.
- Some states with MCOs manage drugs through full drug carve outs or uniform preferred drug lists (PDLs), reducing the influence of their MCOs.
- States are increasingly using their MCO contracts to collect data related to health disparities and to implement reforms addressing social determinants of health (SDoH).

2. Prevalence of Utilization Management

- Findings highlight general practices in the use of utilization management and how it varies across states/MCOs and by therapy:
 - Overall, Medicaid payers require prior authorization and/or step therapy at least 50% of the time for Adakveo[®], Endari, Oxbryta[®], and Siklos[®].
 - Droxia[®] has the least utilization management of the 5 SCD therapies studied; however, it still has restricted access (or unlisted criteria) 46% of the time in FFS Medicaid and 22% of the time in managed Medicaid.
 - Like how Droxia[®] management varies across FFS and MCOs, with MCOs using more open access, Siklos[®] also has more open access in managed Medicaid than state Medicaid (33% in MCOs vs 27% in state FFS). Oxbryta[®] and Endari have lower rates of open access (8-27%) and, conversely, have more open access in FFS Medicaid (17-27%) than MCOs (8-16%).
- Considering prevalence of SCD within states' Medicaid programs, states in the top quartiles of SCD prevalence provide SCD therapies with open access more often than states with lower SCD prevalence.
- When states or MCOs require clinical prior authorization for the SCD therapies studied, reauthorization is typically required every 6-12 months. However, some survey respondents reported more frequent reauthorization.

3. Factors Influencing Decision Making

- Most survey respondents reported using evidence of comparative clinical effectiveness and established clinical benefits in deciding whether to apply step therapy to SCD therapies.

- Drugs' net prices were also often cited as a factor in setting step therapy criteria. Medicaid net prices are complex and not available to the public.
- Preferences of patients and statements from patient advocacy groups are considered much less frequently than these other factors.

4. Stakeholder and Contractor Engagement

- Survey respondents indicate that many decision makers consider pharmacy benefit manager (PBM) input in utilization management decisions. PBMs—some of which interact with many states and MCOs—play a key role alongside states and MCOs in determining access.
- However, stakeholders considered vary across FFS and MCOs. MCOs look most frequently to input from PBMs, whereas states look most frequently to providers and clinical support vendors.
- When decision makers are considering PDL placement and utilization controls, patients and patient groups are the least consulted stakeholders.

5. Health Equity Initiatives

- State Medicaid programs are taking steps to address health equity and SDoH, primarily through MCO contract provisions and data efforts.
 - Screening for SDoH, data collection, and data reporting are important activities to address health equity and are among those included in some MCO contracts.
- Specific policies and programs designed to improve equity for individuals with SCD are still needed. Referrals to community organizations could be an important area for improved partnerships between community-based organizations (CBOs) and Medicaid.

Introduction

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, acute chest syndrome, various infections, strokes, 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.ⁱ Individuals living with SCD often have considerable unmet health needs, and with the absence of cohesive policy to address care gaps, health inequities persist.

The economic burden of SCD is substantial, and impacts payers, patients, and caregivers. Individuals with SCD have more medical appointments, more urgent care and emergency visits, more prescriptions, and higher out-of-pocket costs than those without the disease.ⁱⁱ Privately insured individuals with SCD accrue approximately \$1.7 million on disease-related medical expenses over their lifetime.ⁱⁱⁱ In 2017, 49% of publicly insured individuals with SCD had at least 1 inpatient hospital stay, compared to only 6% of beneficiaries without SCD. Economic burden goes beyond healthcare costs, affecting employment, education, and productivity.^{iv}

National estimates indicate Medicaid covers approximately 50 – 60% of all individuals with SCD, which puts Medicaid in a critical role for mitigating poor health outcomes and cost burden for patients.^v Unfortunately, evidence suggests that access to care is worst for Medicaid patients when compared to commercial payers and Medicare, warranting attention and action from Medicaid.^{vi}

SCD treatments have traditionally included blood transfusions or risky options like bone marrow transplants or stem cell transplants, with little in the way of prescription drug therapies. Since the Food and Drug Administration’s (FDA) approval of hydroxyurea for SCD in 1998, the drug treatment landscape has experienced limited growth. The FDA has granted approval for 4 disease-modifying therapies to treat SCD, including 2 different brands of hydroxyurea and 1 drug that was approved through the accelerated approval process (Oxbryta[®]). These new treatments have expanded options for individuals with SCD—but their value is dependent on access.

Table 1 – SCD Treatments Included in This Analysis

Trade Name	Product Name	Medical or Pharmacy Benefit	FDA Approval Date	Brand vs. Generic Status
Adakveo [®]	crizanlizumab	Medical	November 2019	Brand
Droxia [®]	hydroxyurea	Pharmacy	February 1998	Brand
Endari	L-glutamine	Pharmacy	July 2017	Brand
Oxbryta [®]	voxelotor	Pharmacy	November 2019 (Accelerated approval)	Brand
Siklos [®]	hydroxyurea	Pharmacy	December 2017	Brand

Prescription drugs are a covered benefit in Medicaid, and states must cover the outpatient products of all manufacturers participating in the Medicaid Drug Rebate Program. Drug manufacturers are also required to pay mandatory rebates to Medicaid. While states and their MCOs must cover eligible drugs, they may still use utilization management tools to control drug access and costs. Utilization management can involve a combination of several different approaches including:

- **PDLs**, i.e., lists of drugs in each state that designate drugs as “preferred” or “non-preferred” under the Medicaid program.
- **Prior Authorization**, which requires providers to submit clinical evidence or other documentation before a prescription is filled
- **Step Therapy**, which requires patients try certain therapies before they can access others
- **Quantity Limits**, where prescriptions may only be filled for a specified period and dose
- **Generic Substitution**, where generic drugs must be substituted for brands when available

To assess coverage and access restrictions in Medicaid for therapies prescribed to individuals with SCD, Avalere and Sick Cells conducted an analysis of coverage policies in the 50 states and the District of Columbia and a survey of individuals with recent drug management experience in FFS Medicaid programs or MCOs.

Methodology

Environmental Scan

To examine drug access criteria, Avalere and Sick Cells identified 4 pharmacy benefit products (Droxia[®], Endari, Oxbryta[®], and Siklos[®]) and 1 medical benefit product (Adakveo[®]) indicated to treat complications of SCD. Using comprehensive formulary and medical policy data provided by Managed Markets Insight & Technology, LLC (MMIT), augmented by Avalere research, we then analyzed PDLs and coverage policies as of June 2022 for state FFS programs and MCOs. MMIT captures generic hydroxyurea separately from the brand products Droxia[®] and Siklos[®]; generic hydroxyurea is not analyzed in this report.

Looking across insurance markets, MMIT's data include formularies used for 98% of enrolled lives. For this work, Avalere and Sick Cells analyzed MMIT's data from the Medicaid channel, which include Medicaid FFS programs and MCOs. MMIT includes some payers that are not purely Medicaid in its "Medicaid" categorization; to keep these from obscuring the analysis, Avalere excluded Aids Drug Assistance Programs (ADAPs), Special Needs Plans (SNPs), and Children's Health Insurance Program (CHIP)-only plans in instances where names indicated they fell into these categories.

Results are enrollment weighted, with weighting based on the number of Medicaid beneficiaries in each state or MCO. Weighting is not specific to the number of beneficiaries with SCD. When analyzing combinations of payer coverage information and drugs, results use "percent of the time" to express the enrollment-weighted share of total possible payer and drug combinations. Our approach uses enrollment weighting and "percent of time" to consider which PDLs and coverage policies represent higher enrollment and indicate the percent of the Medicaid population that would be subject to a specific level of utilization management for a particular drug.

MMIT compiles its utilization management designations from payer documents into several status categories and sets standard policies by payer for how drugs are labeled when they are not listed. To simplify these designations, we adapted MMIT's status categories to designate each product as 1) unrestricted (i.e., 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) not listed. Prior authorization and step therapy designations are determined from information found on PDLs but also other documents, such as SCD prior authorization criteria summaries linked to PDLs or listed independently on payer websites. Products in these groupings may show up on PDLs with incomplete detail or may not be listed on PDLs but have other coverage documents available. The not listed category includes products not listed on the state or plan's PDL for which MMIT did not capture any UM criteria elsewhere and products designated by MMIT as Non-Formulary/Exclusion (note: given Medicaid coverage rules under the MDRP, we assume all products are covered).

Step therapy policies were examined to identify the specific products a beneficiary must step through to gain access to another drug. Because MMIT includes steps that are not purely trial and failure on prescription drugs, we adapted MMIT's status categories to designate criteria as "general step therapy criteria" or "brand step therapy criteria" (Table 2).

Table 2 – Examples of Step Therapy Criteria Observed Across SCD Treatments

Examples of General Step Therapy Criteria	Examples of Brand Step Therapy Criteria
<ul style="list-style-type: none"> • Failure on generic medication, such as generic hydroxyurea • Failure on non-prescription L-glutamine supplement¹ • History of red blood cell transfusion • Inability to swallow capsules 	<ul style="list-style-type: none"> • Failure on brand medication, such as Adakveo[®], Droxia[®], or Endari

Information for pharmacy benefit products is primarily from MMIT’s formulary data, whereas information for Adakveo[®] is from a combination of formulary data and MMIT’s Patient Access Restrictions (PAR) data. Step therapy information for all products is from the PAR data. To give a comprehensive understanding of Adakveo[®] coverage, we calculated a composite coverage category reflecting the least restrictive coverage offered by an MCO or state Medicaid program between medical and pharmacy benefits for Adakveo[®]. The benefit type with greater access was then used as the composite coverage for that payer.

Avalere and Sick Cells also collected prevalence data from the Medicaid and CHIP Sickle Cell Disease Report, T-MSIS Analytic Files 2017. To analyze access by prevalence, we grouped states into quartile categories based on SCD prevalence in relation to total state Medicaid enrollment and separately for total national SCD prevalence counts. These data present a few limitations: data reflect trends in Sickle Cell Disease in 2017 and may not account for changes from 2017-2022; Maryland prevalence data was not reported due to concerns of data quality in the 2017 TAF files, excluding Maryland from prevalence analyses; and data is not segmented by managed Medicaid/state Medicaid and therefore inferences at the payer type level compared to overall Medicaid prevalence may differ.

Survey

To gain additional insights into decision making on the coverage and access landscape for SCD therapies, 2 surveys were designed and administered: 1 to current state Medicaid directors and another to a broader group of individuals including current or former Medicaid decision makers. For the first survey, Sick Cells conducted outreach to state Medicaid directors via email, yielding 15 respondents. For the partner survey, the Gerson Lehman Group (GLG) conducted outreach to its network and targeted relevant individuals at MCOs and individuals with prior state Medicaid experience, yielding 25 respondents. Both surveys were nearly identical and sought responses from individuals with current Medicaid drug decision-making experience. Some state Medicaid directors delegated survey response to a member of their staff. Responses were collected from

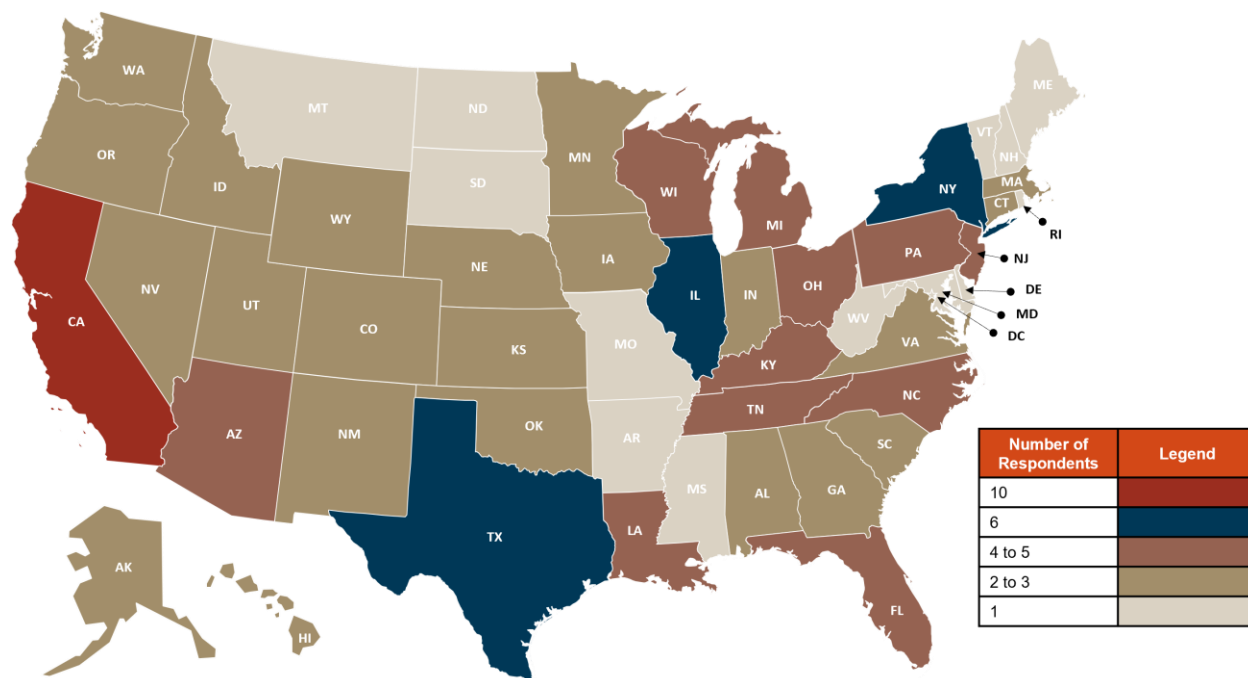
¹ Some coverage policies name failure on L-glutamine as a step but do not name the brand Endari. Endari is the only medicine-grade version of L-glutamine that is approved by the FDA for SCD treatment; it has no generic available. It is assumed that these policies are considering the use of the non-prescription L-glutamine dietary supplement as step therapy criteria.

individuals who held a relevant position at some point since 2019. See Appendix A for a full list of survey questions. Survey questions focused on the following domains:

- Coverage and care management for individuals with SCD
- Prescription drug management and access
- Health equity
- Stakeholder engagement

A combined total of 40 respondents represent experience in all 50 states, the District of Columbia, and Puerto Rico. Some respondents from GLG—particularly those with MCO experience—reported multiple states within their purview. While all states and the District of Columbia are represented, not all results could be mapped to a specific state(s). Of the full group of respondents, the number of respondents by state are as follows:

Figure 2 – Survey Respondents by State



**25 respondents reported results for more than 1 state; 15 respondents represented specific states²*

Of 40 respondents, 21 either held or currently hold positions in state Medicaid agencies (either as state Medicaid directors, pharmacy directors, or medical directors), and 11 held or currently hold positions in MCOs (either as pharmacy directors or medical directors). Eight respondents reported other titles. More than half (24) reported acting as a decision maker for prescription drugs and 15 reported their role as informing others who were decision makers for prescription drugs. All respondents reported familiarity with how beneficiaries with SCD are managed across the Medicaid program.

² Respondents responding for specific states represented Connecticut, Kentucky, Kansas, Illinois, Iowa, Louisiana, Massachusetts, Missouri, New York, Pennsylvania, Tennessee, Texas, Virginia, Wyoming, and the District of Columbia

The Role of MCOs

Most Medicaid beneficiaries are enrolled in MCOs. CMS' most recent data on Medicaid enrollment show that 72% of beneficiaries were in comprehensive MCOs in 2020. This balance was reflected in our survey pool, specifically for the SCD population, with 35 of 40 respondents reporting that some or all Medicaid beneficiaries with SCD in their state(s) of purview are enrolled in MCOs. Though some MCOs have Medicaid business in just 1 state, many are regional or national plans that have a broad reach; the survey respondents included many individuals who had worked with MCOs across multiple states. This speaks to the power and influence MCOs can have on the Medicaid population.

Role of MCOs

- MCOs manage a large proportion of Medicaid beneficiaries
- MCO coverage criteria are slightly less generous than state fee for service criteria

States decide who to enroll in managed care, and some use specialized MCOs for beneficiaries with SCD. Of states that use MCOs, some make MCO enrollment voluntary; others require enrollment of all beneficiaries or groups (e.g., by geography). Survey respondents frequently noted that their states either require MCO enrollment as mandatory for all beneficiaries (11 respondents) or that they determine enrollment by eligibility category (11 respondents). The survey also revealed the use of specialized MCOs for some beneficiaries with SCD. The use of specialized MCOs was less common among other specialized benefits reported for beneficiaries with SCD (chronic disease coordination being the most popular, with 23 responses, but 11 respondents cited the use of specialized MCOs).

Overall, Medicaid MCO coverage criteria for the SCD therapies analyzed were found to be slightly less generous than state FFS criteria—though MCOs list their criteria more often and require less step therapy. Aggregated nationally, Medicaid MCO coverage is slightly less generous than FFS Medicaid coverage for the 5 products in our analysis. This is particularly true for Droxia® (unrestricted 78% of the time for MCOs versus 54% for state Medicaid) and Siklos® (33% versus 27%). For Adakveo®, however, state Medicaid programs more frequently have unrestricted access (42%) than Medicaid MCOs (26%). Looking across states, MCOs generally list SCD therapies with prior authorization more often than FFS programs do; however, MCOs generally apply less step therapy. Moreover, a greater proportion of MCOs list access criteria for SCD therapies, with not listed rates ranging from just 7 – 9% of the time for MCOs but up to 25% of the time for FFS Medicaid. Having access criteria documented on a PDL or other document is important to ensure beneficiaries and providers understand how therapies can be accessed.

Some states with MCOs manage drugs through full drug carve outs or uniform PDLs, reducing the influence of their MCOs. States that enroll Medicaid beneficiaries in managed care may choose to manage the drug benefit wholly or in part at the state level. Five states use full drug “carve-outs” where the states exclude prescription drug provisions from MCO contracts, manage the drug benefit, and bear financial responsibility for drug costs. Fifteen states use uniform PDLs, where they establish a unified drug list across all Medicaid health plans and the fee-for-service (FFS) programs in the state but require their MCOs to process drug claims and

bear risk for drug costs.^{vii} Some states with the higher SCD prevalence, including Texas, use uniform PDLs, while several states with the lower SCD prevalence including Iowa and Kansas also use uniform PDLs.^{viii} Other states are planning to adopt these approaches soon (e.g., North Dakota will establish a uniform PDL for 1 or more classes). States with carve outs or uniform PDLs may still allow MCOs to control medical benefit drugs (e.g., Adakveo[®]) or to reduce utilization management below state levels. So, while these shifts reduce the control of MCOs, MCOs remain important in ensuring access to SCD therapies.

States are leveraging MCO contracts to address health equity. States are increasingly using MCO contracts as vehicles to require data collection related to health disparities and to implement reforms addressing SDoH. 25 of 40 survey respondents noted that their state(s) of purview are addressing equity using MCO contract provisions, mirroring broader findings beyond this survey (e.g., another study found that in state fiscal year 2021, 33 states were using MCO contract provisions to address SDoH in some capacity).^{ix} It is promising that states can leverage MCOs' broad reach to focus on health equity and SDoH but also important to note that these efforts are not always focused on beneficiaries with SCD and improving their care.

Prevalence of Utilization Management

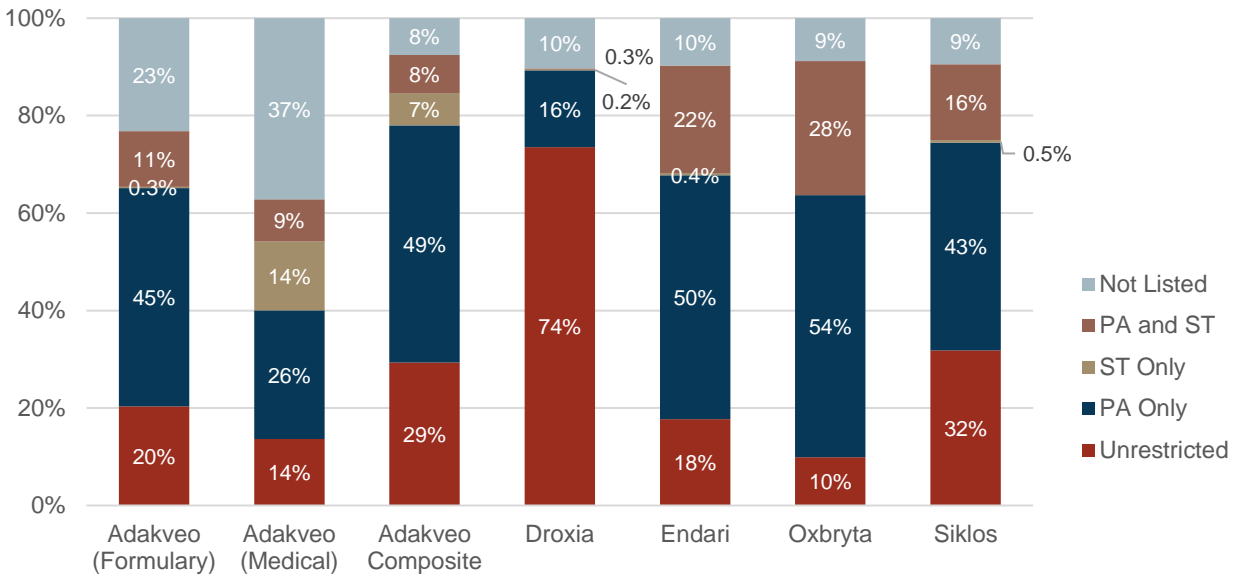
State Medicaid programs and Medicaid MCOs use utilization management techniques to control drug costs and manage beneficiary access to SCD therapies. These techniques may include prior authorization, step therapy, quantity limits, and generic substitution (of these, our environmental scan analyzed just prior authorization and step therapy). States and MCOs use utilization management techniques both to ensure beneficiaries are given clinically appropriate treatments and as a cost-saving strategy. Cost savings can accrue in 2 ways—by limiting the use of products and by negotiating down products' net cost with supplemental drug rebates in exchange for preferential coverage. Utilization management can also create burdens for beneficiaries with SCD, their caregivers, and healthcare providers, limiting timely access to SCD therapies.

Prevalence of Utilization Management

- Medicaid payers require prior authorization and/or step therapy 50% or more of the time for the SCD therapies studied
- States in the top quartiles of SCD prevalence provide the studied SCD therapies with open access more often

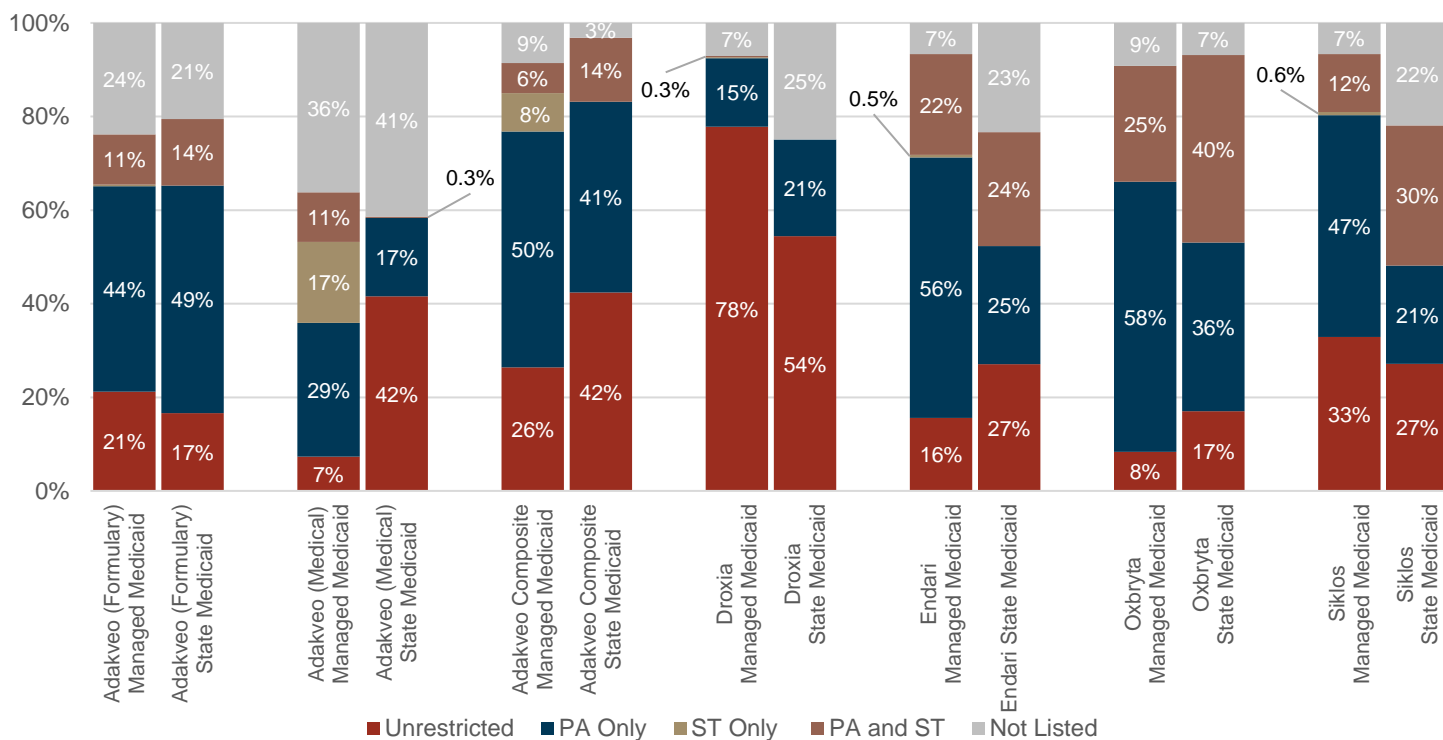
Utilization management varies across the SCD therapies included in our analysis, but the prevalence of prior authorization and step therapy is a key theme. Looking across all state FFS programs and MCOs, Medicaid payers require prior authorization and/or step therapy at least 50% of the time for Adakveo[®], Endari, Oxbryta[®], and Siklos[®]. Droxia[®] has the least utilization management of the 5 SCD therapies analyzed, but it is still subject to restrictions 27% of the time (46% of the time in state Medicaid and 22% of the time in managed Medicaid). Endari and Oxbryta[®] have the highest rates of prior authorization and step therapy.

Figure 3 – Utilization Management Techniques for SCD Therapies, All Medicaid



Aggregated nationally, Medicaid MCO coverage criteria are generally less generous than state Medicaid programs, with a slightly lower rate of unrestricted access across these 5 drugs (32% unrestricted for MCOs vs. 34% unrestricted for FFS). This is particularly true for Adakveo® (unrestricted 42% of the time for FFS versus 26% for MCOs) and Endari® (27% of FFS versus 16% of MCOs). As discussed previously, MCO coverage is not more restricted for every SCD therapy or every plan.

Figure 4 – Utilization Management for SCD Therapies, Managed and State Medicaid

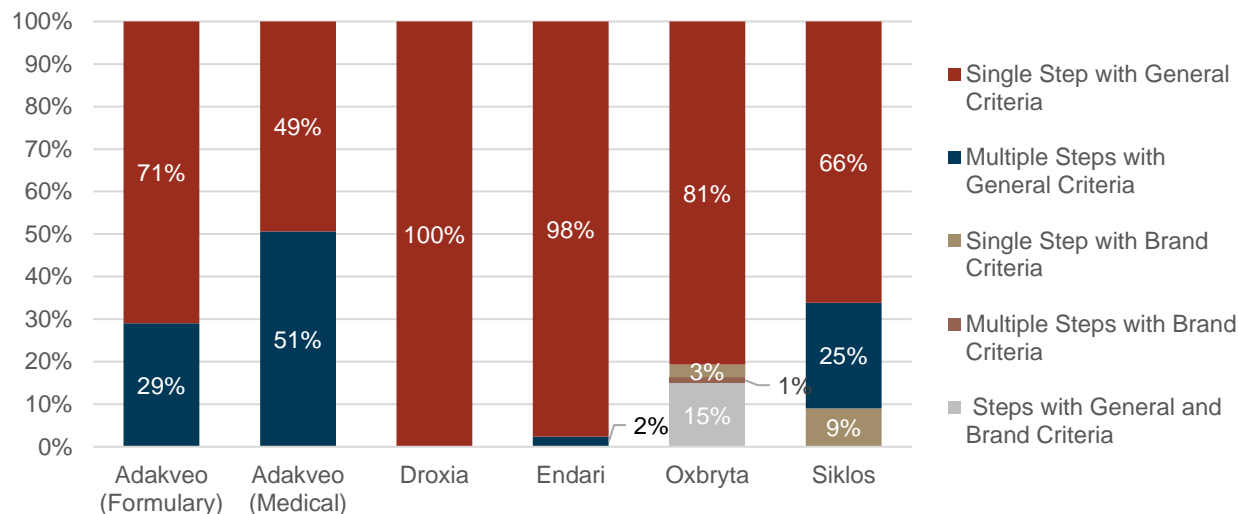


Of the 5 SCD therapies analyzed, Adakveo® is the only medical benefit product; it often has pharmacy benefit or medical benefit coverage criteria but not both. Accordingly, to gain a full picture of Adakveo® coverage required analyzing both pharmacy-benefit documents (e.g., PDLs) and medical benefit coverage criteria. Analysis of just pharmacy or medical coverage criteria results in a high rate of unlisted criteria (23% for pharmacy benefit and 37% for medical benefit criteria). However, examination of both criteria jointly provides a more complete picture, with coverage criteria not listed just 8% of the time. Adakveo® is most widely covered without restrictions under state Medicaid medical benefit policies (42%). It is encouraging that most Medicaid payers have some form of coverage criteria available for Adakveo®; however, beneficiaries with SCD and their providers may struggle to understand criteria listed across various parts of the Medicaid benefit.

When states and Medicaid MCOs apply step therapy to the selected SCD therapies, they most commonly require a single general step.³ Though MCOs use step therapy less widely than FFS, MCOs are the only Medicaid payers with policies requiring multiple steps.

³ Refer to Table 2 for examples of general step therapy criteria.

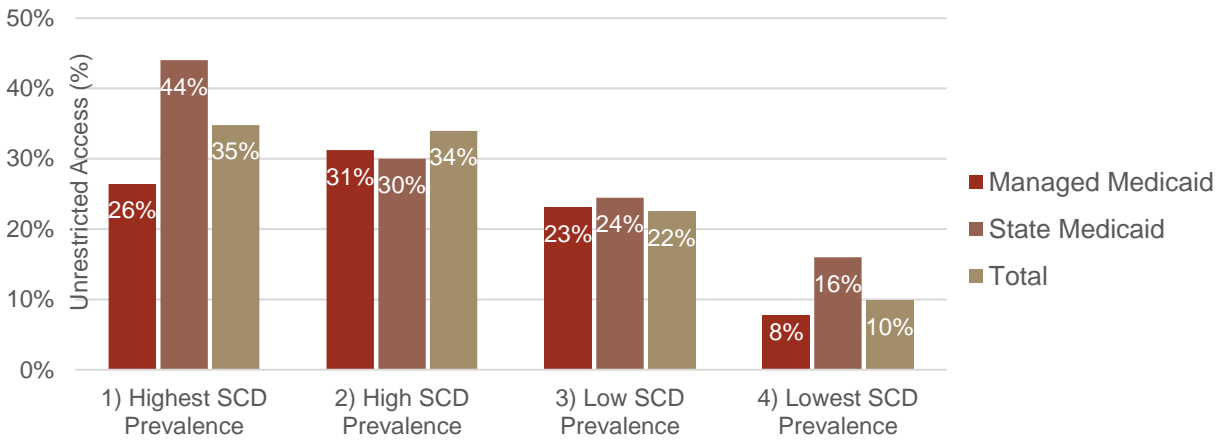
Figure 5 – Step Therapy Counts for SCD Therapies with Step Therapy, All Medicaid



Homing in on the exact step therapy criteria (i.e., which specific products a beneficiary must step through to gain access to another drug), the most common step for most products is a single step through hydroxyurea, which is a commonly prescribed therapy for individuals with SCD. However, for Adakveo®, the second-most common step is through hydroxyurea or L-glutamine (possibly the non-prescription supplement). MCOs include L-glutamine in step therapy criteria more commonly than FFS; just Maine, Nevada, and Vermont’s state Medicaid programs include references to L-glutamine in step criteria. Whether a Medicaid payer requires single or multiple steps, the requirements may be formulated without consideration to the intended drug’s mechanism of action compared to the step or the appropriateness for the individual’s presentation of SCD.

States in the top half of SCD prevalence list the analyzed SCD therapies with open access more often than states with lower SCD prevalence. However, this varies by state and product. An evaluation by prevalence group (with states broken into quartiles based on SCD prevalence in Medicaid) found that states with high SCD prevalence list these therapies with open access more than states with lower SCD prevalence. Drug-specific breakouts by prevalence quartile reveal more complexity—Droxia® has higher rates of open access across all prevalence quartiles, and Endari and Oxbryta® have almost no open access in the lowest prevalence quartiles, especially in managed Medicaid—however, each individual drug shows its lowest open access rates in the states with lowest SCD prevalence. Ensuring access to SCD therapies is important in states where it is most prevalent. Alabama allows for unrestricted access to Adakveo® 100% of the time, Louisiana allows unrestricted access to Droxia® 100% of the time, and Florida allows for unrestricted access to Endari and Siklos® 98.5% of the time. However, low open access rates in lower-prevalence states may impact patient access, given that an individual with SCD has the same need for treatment regardless of their location. Moreover, in states with the lowest prevalence, there is a lower likelihood that beneficiaries with SCD have access to an SCD specialist, and available providers may be least equipped to provide appropriate care or help access treatment; utilization management may pose an additional hurdle.

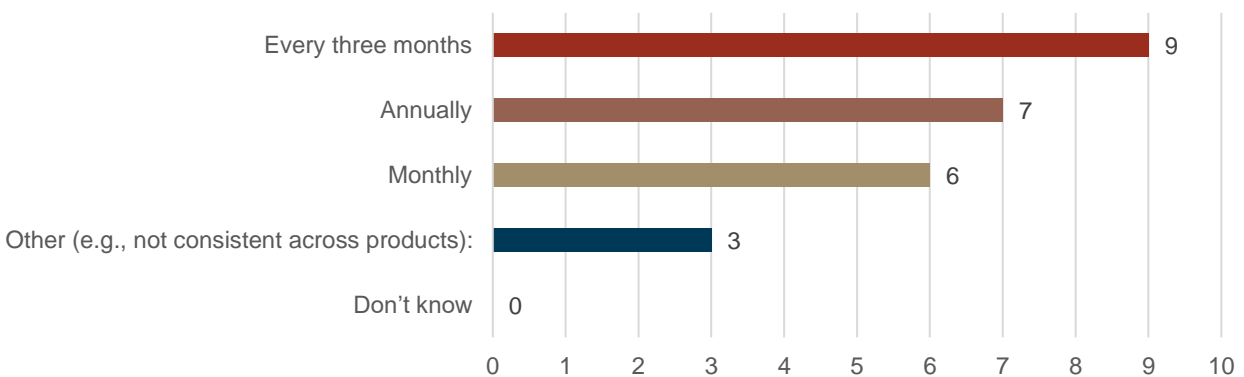
Figure 6 – Unrestricted Access by Prevalence Group



When states or their MCOs require prior authorization for prescription products that treat SCD, reauthorization is most often required every 6-12 months. A plurality of survey respondents reported reauthorization every 12 months (7 respondents). Other respondents reported prior authorization frequencies for SCD as often as every 3 months (9), or monthly (6). Shorter reauthorization periods may increase the number of administrative steps that individuals with SCD and their providers must take to obtain therapies. Administrative steps for individuals with SCD and their providers associated with prior authorization (and the increased frequency of those requirements) adds to administrative burdens. Future analyses should consider the states of high prevalence of SCD and evaluate prior authorization criteria in those states.

Figure 7 – Reauthorization Frequency, Survey Counts

When a product that treats SCD has clinical prior authorization criteria, how frequently must a beneficiary's prescription be reauthorized?



Factors Influencing Decision Making

Across Medicaid FFS programs and MCOs, pharmacy and therapeutics (P&T) committees or drug utilization review boards (DURBs) typically set coverage criteria for prescription drugs, including SCD therapies. Decision makers take a range of inputs into account, considering information about the therapies and stakeholder input.

Factors Influencing Decision Making

- MCO and Medicaid Directors use evidence of comparative effectiveness and clinical benefit, and drug net prices to inform step therapy for SCD therapies
- Patient preferences are used less often to determine access to therapies

Most survey respondents reported using evidence of comparative clinical effectiveness and established clinical benefits when making decisions about whether SCD therapies would be subject to step therapy. Out of 40 respondents, 34 noted that they consider clinical appropriateness (e.g., patient eligibility), 32 consider established clinical benefits, and 32 consider evidence of comparative clinical effectiveness. One respondent from a state Medicaid program specifically commented that they considered assessments from the Institute for Clinical and Economic Review (ICER) when determining PDL placement and/or utilization management techniques. However, several SCD advocates (including Sick Cells) note that when ICER evaluated SCD therapies in 2019 (i.e., Adakveo[®], Oxbryta[®], and Endari), it used a definition of value that did not account adequately for nonmedical and indirect costs, lacked appropriate comparators, and left out the patient perspective in its value-based price metric.

The next most-considered factor in determining whether SCD therapies will be subject to step therapy is a drug's net price. 25 of 40 respondents indicated considering the drug's net price (i.e., after rebates); just 10 noted a consideration of list price. This distinction is important, because Medicaid net price is opaque—though a drug's list price is a key component, mandatory Medicaid drug rebates and optional supplemental rebates can significantly affect the net price. SCD therapies' net prices are known to Medicaid programs and drug manufacturers but are not available to the public.

Patient preferences and statements from patient advocacy groups were the least commonly cited factors in prescription drug decision making among survey respondents. Only 10 respondents noted that patient preference was a factor that prescription drug decision makers incorporated. Similarly, only 7 respondents (including those from KS, KY, MA, MO, TX, and TN) noted that statements from patient advocacy groups were factors in determining step therapy management. These results draw attention to the need for Medicaid programs and MCOs to make a concerted effort to build and utilize relationships with beneficiaries with SCD, patient advocacy groups, and other expert groups in their state.

States are beginning to address the role of cell and gene therapies (CGTs) to inform future decision making. The treatment landscape for SCD could change significantly in the coming years with the expected approval of CGTs treating SCD. CGTs cultivate or modify immune cells or genetic material outside a patient's body before being injected into a patient, which can affect cell expression. Though these therapies only make up a small portion of the SCD community will be eligible for these therapies, it will still be important for states and MCOs to ensure access by

defining criteria and considering how they will finance these therapies. Our survey found that state Medicaid programs and MCOs are addressing CGTs by discussing, creating, or considering coverage policies and/or precertification criteria (28 respondents), value-based contracts (20 respondents), or specific drug carve-outs from MCO contracts (15 respondents). No reported CGT activities were specific to SCD. This makes sense, as states cannot firmly set criteria or implement value-based contracts for products that are not yet available.

Stakeholder and Contractor Engagement

State Medicaid programs and their MCO partners engage with stakeholders and contractors in a variety of capacities that affect access to care for individuals with SCD. Among others, these capacities include drug decision making and provision of additional services and supports.

When determining PDL placement or utilization controls for SCD therapies, the primary stakeholders that Medicaid and MCO prescription drug decision makers rely on for input vary.

States and MCOs both often work with subcontractors, typically PBMs, to conduct administrative functions of their pharmacy benefits. Moreover, 21 of 40 survey respondents reported that PBMs' input is often considered in utilization management decisions. This demonstrates how PBMs—some of which work with many states and MCOs—play a key role in determining access to SCD therapies. Though states and MCOs both rely on PBMs, our survey shows that MCOs primarily seek out PBMs' input for utilization management decisions (e.g., Kentucky, which is one of the most restrictive states for coverage of SCD therapies, uses a single PBM model across pharmacy benefit coverage), whereas survey respondents from state Medicaid programs most frequently reported seeking input from general providers (non-SCD specialists) and clinical information vendors.

When decision makers are considering PDL placement and utilization controls, patients and patient groups are the least consulted stakeholders, with 8 of 40 survey respondents seeking patient input and 11 seeking input from patient groups. Though many entities across healthcare express a desire to provide “patient-centric” services, this may not be happening in determinations of PDL placement and utilization management decision making given the lack of patient input. To ensure individuals with SCD have their voices heard, SCD stakeholders can engage with state Medicaid programs and Medicaid MCOs to influence PDL placement and utilization management techniques. For example, in 2021, Sick Cells worked with 2 advocates in Wisconsin who encouraged the committee to add all SCD therapies as preferred on the state's PDL without restrictions by highlighting how therapies work differently for each individual.^x

To improve outcomes for individuals with SCD, it is important for Medicaid to establish relationships with external stakeholders and CBOs. CBOs can help facilitate patients' access to SCD therapies, medical services, and wraparound services needed to address social determinants of health and help Medicaid programs understand the most pressing needs felt by the community. However, CBOs focusing on providing care for the SCD community are often

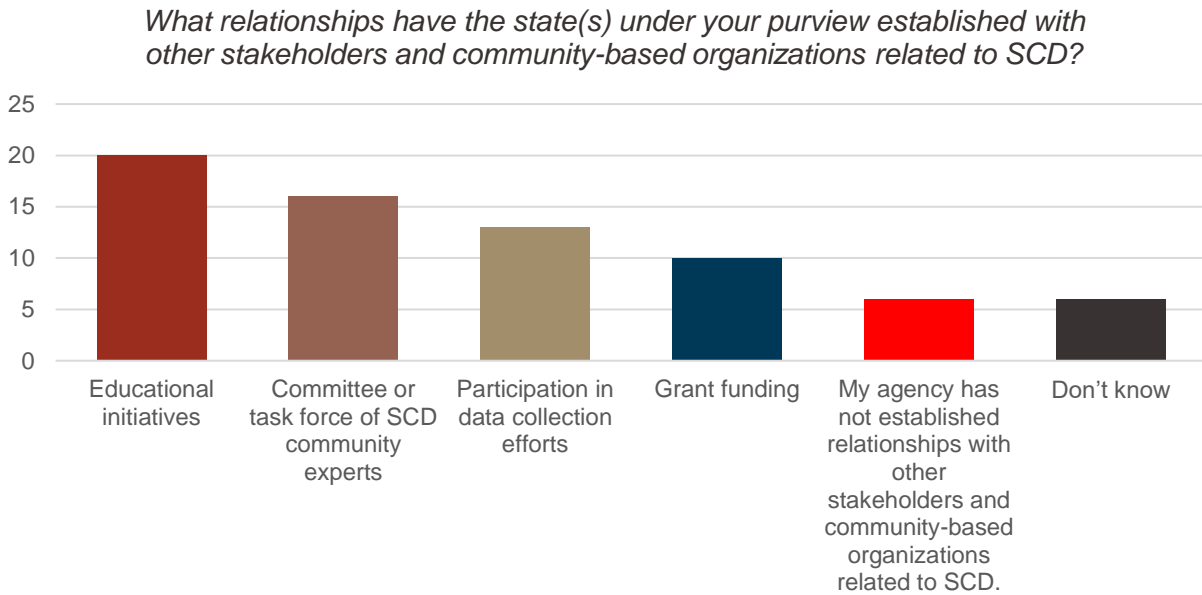
Stakeholder & Contractor Engagement

- MCO and Medicaid decision makers consider PBM input in utilization management decisions
- Patients and patient groups are the least consulted stakeholders when considering PDL placement and utilization controls

operating on limited, grant-based budgets, restricting their ability to expand and provide the necessary services for the entire state.^{xi}

When asked what relationships their state agency established with CBOs, **the most reported were for educational initiatives and committees or task forces of SCD community experts**, followed by data collection efforts and grant funding. Yet nearly a third of respondents (12) reported they did not know the level of external engagement, or their agency did not have any established relationships with external stakeholders.

Figure 8 – Stakeholder Relationships, Survey Counts



**N=40. Respondents could select more than 1 option.*

Respondents were also asked how their state(s)' agency was engaging with patient or caregiver organizations to ensure access to SCD therapies or medical services. Most respondents (28) reported their agency engaged with these organizations to coordinate care services. Others reported that their agency develops SCD care management guidelines for providers (17), or support provider referrals (17). Although somewhat less common, caregiver support (13) and coverage of genetic counseling (12) were also reported.

Health Equity Initiatives

Improving the health of individuals with SCD requires addressing health disparities (e.g., SDoH) and health inequities (i.e., systematic differences in health status and outcomes across populations) to achieve a universal standard of health and wellness. As individuals with SCD often experience adverse SDOH^{xii} and poorer health outcomes compared to other populations, addressing biases within the healthcare system can help to ensure that individuals with SCD have access to not only relevant therapies but also social services, nutritious food, and other supports.

Health Equity Initiatives

- State Medicaid programs are taking steps to address health equity and SDoH, through MCO contract provisions and data efforts
- Policies and programs designed to improve equity for individuals living with SCD are still needed

State Medicaid programs are taking steps to address health equity and SDoH, but those are primarily through MCO contract provisions and data efforts. Most survey respondents reported that Medicaid is acting to address health equity in their state(s) of purview. Highest reported responses were for Medicaid managed care contract provisions (25), data collection (22), and data reporting (21). Just under a third of respondents reported the use of payment incentives and structured systems for referring beneficiaries to other agencies or community organizations. Least reported was financial assistance to help beneficiaries afford medication or services; this is expected given that federal statute limits cost-sharing amounts for Medicaid beneficiaries. Six (6) survey respondents reported having no current programs or initiatives to address equity.

Some survey respondents provided details on specific state initiatives, including:

- A respondent from **New York** reported plans to submit a Section 1115 waiver⁴ amendment request related to health equity for Health Equity Regional Organizations and Social Determinants of Health Networks. The respondent also reported that MCO quality measures will be stratified by race/ethnicity starting in 2022 — 2023.
- A respondent from **Kentucky** reported a modification of MCO contracts focusing on health equity and development of data collection and reporting to identify specific inequities.
- A respondent from **Texas** reported the Texas Health and Human Services Commission collects race, gender, and Clinical Risk Group information on its Healthcare Effectiveness Data and Information Set measures. Also, the Texas External Quality Review Organization analyzes equity-focused data in their reports to the state.
- A respondent from **Louisiana** reported they have a Review, Advise and Inform Board of community members who advise their bureau on health equity and community activities.
- Respondents from **Kentucky** and **Ohio** reported their states were surveying providers and members to understand barriers to care access, including social and financial challenges.

⁴ Section 1115 of the Social Security Act permits the Secretary of Health and Human Services to approve pilot, demonstration, or experimental projects to assist the Medicaid program's goals. Medicaid.Gov "About Section 1115 Demonstrations." <https://www.medicaid.gov/medicaid/section-1115-demonstrations/about-section-1115-demonstrations/index.html>.

- Respondents from **California** and **New Mexico** responded their state was using a combination of provider incentives and in-depth reporting without further details.

Specific policies and programs designed to improve equity for individuals with SCD are still needed. When asked if their state(s) was considering any such policies, nearly half of respondents reported their state was either not considering (14) or were unaware of (5) any specific policies or programs for individuals with SCD. Other respondents reported their states were considering data collection efforts, such as a reporting system in California and examining prior authorization processes for SCD therapies in Kentucky. A New York respondent reported SCD was identified in New York Medicaid as a population needing special support and that New York has implemented support for care transitions from pediatricians to adult providers.

Despite the reported efforts of many states, the science of addressing health equity is still emerging. Many initiatives remain in the realm of data collection or reporting, which is a critical first step to understanding the scale and extent of disparities. But doing so does not showcase active attempts to re-structure programs to provide accessible support for beneficiaries with SCD. Without widespread commitment to innovative access and coverage for SCD, Medicaid programs and MCOs may underserve the SCD community and inadvertently restrict access to drugs and treatments.

Recommendations for Stakeholders

Findings from these analyses point toward actionable steps that Sick Cells recommends for state Medicaid programs, Medicaid MCOs, and other entities involved in drug decision making (i.e., PBMs, pharmaceutical manufacturers). This list of Sick Cells' recommendations⁵ should be seen as a starting point for continued improvement and collaboration.

Advancing Health Equity

Our analyses found that Medicaid programs and Medicaid MCOs are addressing health equity primarily through Medicaid managed care contract provisions and data reporting/ collection. These initiatives are positive; however, given the high needs of many individuals with SCD and the deep inequities associated with the condition, this focus should be more robust.

- Take a more active role in screening patients for SDoH-related needs.
- Provide preferential access or other considerations to ensure individuals with SCD can access disease-modifying therapies and prioritize those most affected by health inequities, given the Biden Administration's focus on equity and the demographics of the SCD population.
- Provide bias and discrimination trainings to relevant decision makers (i.e., DURB committee members, pharmacy directors, PBMs) and examine the impact of bias in prescription drug coverage decisions.

Refining Comparative Cost Effectiveness Data

Many survey respondents reported using evidence of clinical appropriateness, comparative clinical effectiveness, and established clinical benefits when determining whether SCD therapies should be subject to utilization management.

- Consider current limitations to available comparative clinical effectiveness data, such as ICER's value assessment of SCD treatments, which do not meaningfully reflect the patient perspective and lack data on the economic burdens associated with SCD.
- Partner with CBOs and other SCD stakeholders to collect real-world data and patient-reported quality of life information to use as evidence in coverage decision making.
- Evolve and improve approaches for estimating cost effectiveness to adequately account for patient perspective and nonmedical and indirect costs associated with SCD.

Prioritizing Patient Access to Avoid Treatment Delays

Our survey found that while many Medicaid programs and MCOs require annual clinical prior authorization for SCD therapies, some require reauthorization more frequently even though SCD is a chronic condition. This can create significant delays in individuals receiving their medications, severely impacting their health. Moreover, our analysis found that some form of utilization management is required 50% of the time for the SCD therapies studied. The use of step therapy

⁵ Recommendations are derived from research Sick Cells conducted jointly with Avalere Health; however, recommendations are solely from Sick Cells.

for SCD therapies presents concerns, as SCD therapies have different mechanisms of action and varying eligibility requirements. Furthermore, some treatment plans require the use of combination therapies and steps are sometimes not aligned with FDA labels.

As drug manufacturers prepare to launch CGTs intended to treat SCD, Medicaid programs and MCOs must work hard to understand the potential of these new treatments but must also continue working to create appropriate eligibility requirements for existing SCD therapies and treatments. While disease-modifying treatments for SCD have the potential to improve the lives of individuals with SCD CGTs will not be accessible options for all individuals living with SCD, nor an appropriate option for all, given the individuality of the disease.

- Develop consistent prior authorization criteria for existing SCD therapies, as well as new CGTs intended to treat SCD.
- Lengthen the prior authorization timeframe to a once-annual reauthorization to promote adherence and lessen patient and provider burden.
- Ensure that prior authorization criteria align with FDA labels.
- Educate decision makers on available and emerging therapies to ensure utilization management decisions are based in an understanding of SCD therapies' differing mechanisms of action.

Improving Patient-Centric Decision Making

Our analyses indicate that Medicaid prescription drug decision makers deprioritize patient perspectives compared to other types of input when setting access criteria for SCD therapies. For example, when determining PDL placement and/or utilization management for SCD therapies, state Medicaid programs and Medicaid MCOs consult providers, pharmacy benefit managers, and drug manufacturers more frequently than individuals with SCD or SCD patient groups. Without input from those with personal or professional expertise in SCD, it is difficult for state Medicaid programs and MCOs to develop a nuanced understanding of the impacts of their decisions on the sickle cell disease community.

- Connect with local CBOs or individuals living with SCD to gather insights on patient perspectives. Especially in states where providers specializing in SCD are not available for input, CBOs and community members are often the only local experts with experience in managing care.
- Incorporate patient-centric evidence into decision-making processes by consulting SCD task forces and rare disease advisory councils.
- Improve opportunities for public advocates to engage with decision makers by conducting outreach before relevant drug class reviews.

Increasing Transparency

The complexity of Medicaid results in limited transparency into access criteria for SCD therapies. Some states use MCOs and/or PBMs and other entities to influence prescription drug decision making, making it more difficult for interested stakeholders to easily understand coverage and

access criteria in their state. Additionally, differing PDLs across state Medicaid programs and MCOs make it challenging to draw conclusions regarding access and coverage in the state.

- Conduct an annual review of all medications and SCD therapies and review utilization management to understand how individuals with SCD access disease-modifying therapies.
- Share information on which factors are used when setting access criteria for SCD therapies (including the net price of drugs), the stakeholders and consultants solicited for input, and status of access to SCD therapies across Medicaid beneficiaries.
- Incorporate the use of uniform PDLs across state Medicaid and MCOs.
- Ensure PDL information regarding utilization management matches the criteria outlined in additional coverage documents.

Conclusion

This report aims to provide an overview of the state of access to SCD therapies in Medicaid, highlight current challenges, and discuss how access to therapies can be bolstered. Our analyses found that many Medicaid beneficiaries with SCD are enrolled in managed care, which has slightly less generous coverage criteria for SCD therapies. We also found that Medicaid payers require prior authorization and/or step therapy at least 50% of the time for SCD therapies and that states in the top quartiles of SCD prevalence provide open access to SCD therapies more often than states with lower SCD prevalence. Our analyses also found that state Medicaid programs and Medicaid MCOs considered preferences of patients and statements from patient advocacy groups less frequently than other factors in prescription drug decision making and could better address health equity for individuals with SCD.

While our analyses shed light on the current state of access to SCD therapies in Medicaid, challenges individuals with SCD face, and how to increase access to SCD therapies, our work had several limitations. These imitations include:

- A somewhat limited sample of current state Medicaid employees in our survey pool;
- Few data points on state-specific drug management practices; and
- Few publicly available Medicaid coverage policies.

Potential future analyses could further examine the role of prescription drug decision makers and their impact on individuals with SCD and highlight opportunities for engagement from SCD stakeholders by:

- Examining the impact of state Medicaid carve-out and uniform PDL policies;
- Analyzing the role of DURBs and P&T committees; and
- Analyzing prior authorization and step therapy policies found among other payers (e.g., commercial insurance, Medicare Part D, etc.).

Glossary

Brand Drug: A drug sold under a specific name or trademark that is patented.^{xiii}

Drug Carve Out: Arrangement wherein a state excludes prescription drug provisions from Medicaid MCO contracts so that the state manages the drug benefit and bears financial responsibility for drug costs.

Generic Drug: A drug that has the same ingredient formula as a brand drug.^{xiv}

Managed Care Organization: Entity that delivers health benefits and other services to Medicaid beneficiaries through contracted arrangements with state Medicaid programs.^{xv} MCOs referenced in this paper are typically under risk-bearing contracts and receive per-member-per-month payments from states.

Pharmacy Benefit Manager: Company that manages prescription drug benefits for a payer.

Preferred Drug List: 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: 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.^{xvi}

Social Determinants of Health: Conditions where people are born, live, and work that affect health, functioning, health risks, and quality-of-life outcomes. Additionally, social determinants of health include factors such as access to healthcare, socioeconomic status, education, physical environments, and employment.^{xvii}

Step Therapy: A requirement that patients try certain therapies before they can access others (e.g., patient is required to try the most preferred drug first and may only progress to other therapies if necessary).^{xviii}

Uniform Preferred Drug List: State-generated drug list outlining preferred status and/or utilization management criteria that Medicaid MCOs must follow for their Medicaid beneficiaries. A uniform PDL may include all drugs covered by Medicaid or a subset. Under this approach, MCOs still administer the drug benefit and bear risk for drug costs.

Utilization Management: Techniques such as prior authorization and step therapy used by payers to manage costs and ensure appropriate patient care.^{xix}

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Appendix: Survey Questions

S0. Are you willing to participate in the study?

- Yes
- No

S1. [Survey research firm participants] Please select the area of healthcare where you have the most experience.

- State Medicaid /Medicaid managed care organization
- Pharmaceuticals/Biotechnology
- Clinical healthcare provider
- Physician office management
- Group purchasing organization (GPO)/pediatric buying group (PBG)
- Other

S2. [Survey research firm participants] Please select the book of business in which you primarily focus on.

- State Medicaid/Medicaid managed care organization
- Medicare/Medicare Advantage
- Commercial
- Other

S3. [Survey research firm participants] Please select the title that best describes your current or most recent role impacting the Medicaid program:

- State Medicaid Director
- State Medicaid Pharmacy Director
- State Medicaid Medical Director
- MCO Pharmacy Director
- MCO Medical Director
- Other state Medicaid or Medicaid managed care employee, please describe:

S3x1. [Sick Cells/state Medicaid Directors] Please select the title that best describes your current role:

- State Medicaid Director
- State Medicaid Pharmacy Director
- State Medicaid Medical Director
- Other, please describe: _____

S4. Which years were you in this role? Check all that apply.

- Prior to 2019
- 2019
- 2020
- 2021
- 2022

S5. Please select the statement that best describes your role in prescription drug decision-making during that tenure:

- Acted as a decision-maker for prescription drugs
- Informed others who were decision-makers for prescription drugs
- Neither made nor informed decisions for prescription drugs

S6. How familiar are you with how patients with SCD are managed across the Medicaid program?

- Very familiar

- Somewhat familiar
- Not familiar at all

S7. Please select all states that fall (or previously fell) under your Medicaid decision-making purview:

- | | | |
|---|---|---|
| <input type="checkbox"/> Alaska | <input type="checkbox"/> Kentucky | <input type="checkbox"/> Ohio |
| <input type="checkbox"/> Alabama | <input type="checkbox"/> Louisiana | <input type="checkbox"/> Oklahoma |
| <input type="checkbox"/> Arkansas | <input type="checkbox"/> Massachusetts | <input type="checkbox"/> Oregon |
| <input type="checkbox"/> Arizona | <input type="checkbox"/> Maryland | <input type="checkbox"/> Pennsylvania |
| <input type="checkbox"/> California | <input type="checkbox"/> Maine | <input type="checkbox"/> Rhode Island |
| <input type="checkbox"/> Colorado | <input type="checkbox"/> Michigan | <input type="checkbox"/> South Carolina |
| <input type="checkbox"/> Connecticut | <input type="checkbox"/> Minnesota | <input type="checkbox"/> South Dakota |
| <input type="checkbox"/> District of Columbia | <input type="checkbox"/> Missouri | <input type="checkbox"/> Tennessee |
| <input type="checkbox"/> Delaware | <input type="checkbox"/> Mississippi | <input type="checkbox"/> Texas |
| <input type="checkbox"/> Florida | <input type="checkbox"/> Montana | <input type="checkbox"/> Utah |
| <input type="checkbox"/> Georgia | <input type="checkbox"/> North Carolina | <input type="checkbox"/> Virginia |
| <input type="checkbox"/> Hawaii | <input type="checkbox"/> North Dakota | <input type="checkbox"/> Vermont |
| <input type="checkbox"/> Iowa | <input type="checkbox"/> Nebraska | <input type="checkbox"/> Washington |
| <input type="checkbox"/> Idaho | <input type="checkbox"/> New Hampshire | <input type="checkbox"/> Wisconsin |
| <input type="checkbox"/> Illinois | <input type="checkbox"/> New Jersey | <input type="checkbox"/> West Virginia |
| <input type="checkbox"/> Indiana | <input type="checkbox"/> New Mexico | <input type="checkbox"/> Wyoming |
| <input type="checkbox"/> Kansas | <input type="checkbox"/> Nevada | |
| | <input type="checkbox"/> New York | |

Q1. [Survey research firm] How are SCD patients currently managed in Medicaid across your state(s) of purview?

[Sick Cells/state Medicaid Directors] How are SCD patients currently managed across your state's Medicaid program?

- Fee-for-service
- Managed care organization
- Both fee-for-service and by managed care organization
- Depends, please describe: _____

Q2. How is enrollment conducted for beneficiaries who obtain some or all benefits through managed care organizations?

- Enrollment in managed care organization is mandatory for all Medicaid beneficiaries
- Enrollment in managed care organization is voluntary
- Enrollment in managed care organization depends on geographic area
- Enrollment in managed care organization depends on eligibility category
- Other, please describe: _____

Q3. [Survey research firm] Does Medicaid or managed care in the states you work (or worked) for offer special protections, supports, or waivers for beneficiaries with SCD? Check all that apply.

[Sick Cells/state Medicaid Directors] Does Medicaid in your state offer special protections, supports, waivers for beneficiaries with SCD? Check all that apply.

- Enrollment of beneficiaries with SCD in a specialized MCO
- Health Homes (for chronic disease support in general or SCD support specifically) that provide services to individuals with SCD
- Other SCD-specific care coordination (e.g., care management programs run via MCOs, care coordinators for individuals with SCD)
- Broader chronic disease care coordination that includes support for individuals with SCD
- Mental health services
- Services during transition from pediatric care to adult care
- Peer support services
- Coverage of non-prescription supplements (e.g., vitamins, minerals)
- Waiver of any required cost sharing

- Waiver of prior authorization for SCD-related services or treatments
- N/A
- Other: _____

Q4. When determining whether beneficiaries will be subject to step therapy to access a prescription drug that treats SCD, what factors do decision-makers take into consideration? Check all that apply.

- Drug list price
- Drug net price (i.e., after rebates)
- Clinical appropriateness (i.e., patient eligibility)
- Established clinical benefit
- Evidence of comparative clinical effectiveness
- FDA approval pathway (e.g., accelerated approval)
- Adverse side effects or risk to patients
- Preferences of patients
- Statements from patient advocacy representatives
- Other: _____

Q5. When determining preferred drug list placement and/or utilization controls for prescription drugs treating SCD, who are the key stakeholders or outside experts that are consulted? Select all that apply.

- Drug manufacturers
- Pharmacy Benefit Managers
- Clinical Information Service/Vendor
- Provider (general or non-SCD specialist)
- Hematologist with expertise in SCD
- Patients
- Patient groups
- Don't know
- My agency does not consult stakeholders or outside experts when determining preferred drug list placement or utilization controls for prescription drugs treating SCD.
- Other: _____

Q6. When a product that treats SCD has clinical prior authorization criteria, how frequently must a beneficiary's prescription be reauthorized? Choose one.

- Monthly
- Every three months
- Annually
- Don't know
- Other (e.g., not consistent across products): _____

Q7. What clinical material or forms do healthcare providers need to present to receive reimbursement for SCD treatments? Check all that apply.

SCD Prescription Drugs	Provider Requirements
Adakveo (crizanlizumab)	<ul style="list-style-type: none"> <input type="checkbox"/> Lab results <input type="checkbox"/> Evidence of failed prior treatments <input type="checkbox"/> Other: _____ <input type="checkbox"/> No documentation is needed to prescribe this medication <input type="checkbox"/> Don't know

Droxia (hydroxyurea)	<input type="checkbox"/> Lab results <input type="checkbox"/> Evidence of failed prior treatments <input type="checkbox"/> Other: _____ <input type="checkbox"/> No documentation is needed to prescribe this medication <input type="checkbox"/> Don't know
Endari (L-glutamine)	<input type="checkbox"/> Lab results <input type="checkbox"/> Evidence of failed prior treatments <input type="checkbox"/> Other: _____ <input type="checkbox"/> No documentation is needed to prescribe this medication <input type="checkbox"/> Don't know
Oxbryta (voxelotor)	<input type="checkbox"/> Lab results <input type="checkbox"/> Evidence of failed prior treatments <input type="checkbox"/> Other: _____ <input type="checkbox"/> No documentation is needed to prescribe this medication <input type="checkbox"/> Don't know
Siklos (hydroxyurea)	<input type="checkbox"/> Lab results <input type="checkbox"/> Evidence of failed prior treatments <input type="checkbox"/> Other: _____ <input type="checkbox"/> No documentation is needed to prescribe this medication <input type="checkbox"/> Don't know

Q8. [Survey research firm] How is Medicaid addressing health equity in the state(s) that were under your purview? Check all that apply.

[Sick Cells/state Medicaid Directors] How is Medicaid addressing health equity in your state? Check all that apply.

- Payment incentives (e.g., increased provider payment for specific provider types, services, or regions)
- Medicaid managed care contract provisions (e.g., requiring that MCOs provide wraparound supports or referrals to enrolled beneficiaries)
- Structured system of referrals to other agencies or community organizations (e.g., to address food insecurity, housing instability)
- Financial assistance to help patients afford medications or services
- Data collection related to health equity (e.g., assessing information collected, response categories to ensure equity can be measured)
- Data reporting (e.g., analysis and reporting of equity-focused Medicaid data)
- My agency does not currently have any health equity-related programs or initiatives
- Other: _____

Q9. Please describe the specific initiatives related to your responses above.

Q10. [Survey research firm] Are the state(s) under your purview considering specific policies or programs to improve equity for beneficiaries with SCD? If so, please describe.

[Sick Cells/state Medicaid Directors] Is your state considering specific policies or programs to improve equity for beneficiaries with SCD? If so, please describe.

Q11. [Survey research firm] What relationships have the state(s) under your purview established with other stakeholders and community-based organizations related to SCD?

[Sick Cells/state Medicaid Directors] What relationships has your state agency established with other stakeholders and community-based organizations related to SCD?

- Committee or task force of SCD community experts
- Grant funding
- Participation in data collection efforts

- Educational initiatives
- Don't know
- My agency has not established relationships with other stakeholders and community-based organizations related to SCD.
- Other: _____

Q12. [Survey research firm] How do the state(s) under your purview engage with patients or caregiver focused organizations in the community to ensure access to SCD medications or medical services? Check all that apply.

[Sick Cells/state Medicaid Directors] How does your agency engage with patients or caregiver focused organizations in the community to ensure access to SCD medications or medical services? Check all that apply.

- Development of SCD care management guidelines for providers
- Coverage of genetic counseling
- Care coordination services
- Provider referrals
- Caregiver support
- Don't know
- My agency does not engage with community-based organizations to ensure access for SCD medications or services
- Other: _____

Q13. [Survey research firm] Are the state(s) under your purview doing any work to prepare for coverage of emerging gene and cell therapies? Select all that apply.

[Sick Cells/state Medicaid Directors] Is your Medicaid agency doing any work to prepare for coverage of emerging gene and cell therapies? Select all that apply.

- Discussing/developing/considering coverage policies and/or precertification criteria
- Discussing/developing/considering specific drug carve-outs from MCO contracts
- Discussing/developing/considering value-based arrangements
- Discussing/developing/considering reinsurance programs
- My agency is not actively preparing for coverage of emerging gene and cell therapies
- Don't know
- Other: _____

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