State of Illinois
Department of Healthcare and Family Services

December 2024

Recommendations REPORT

By the Illinois Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment

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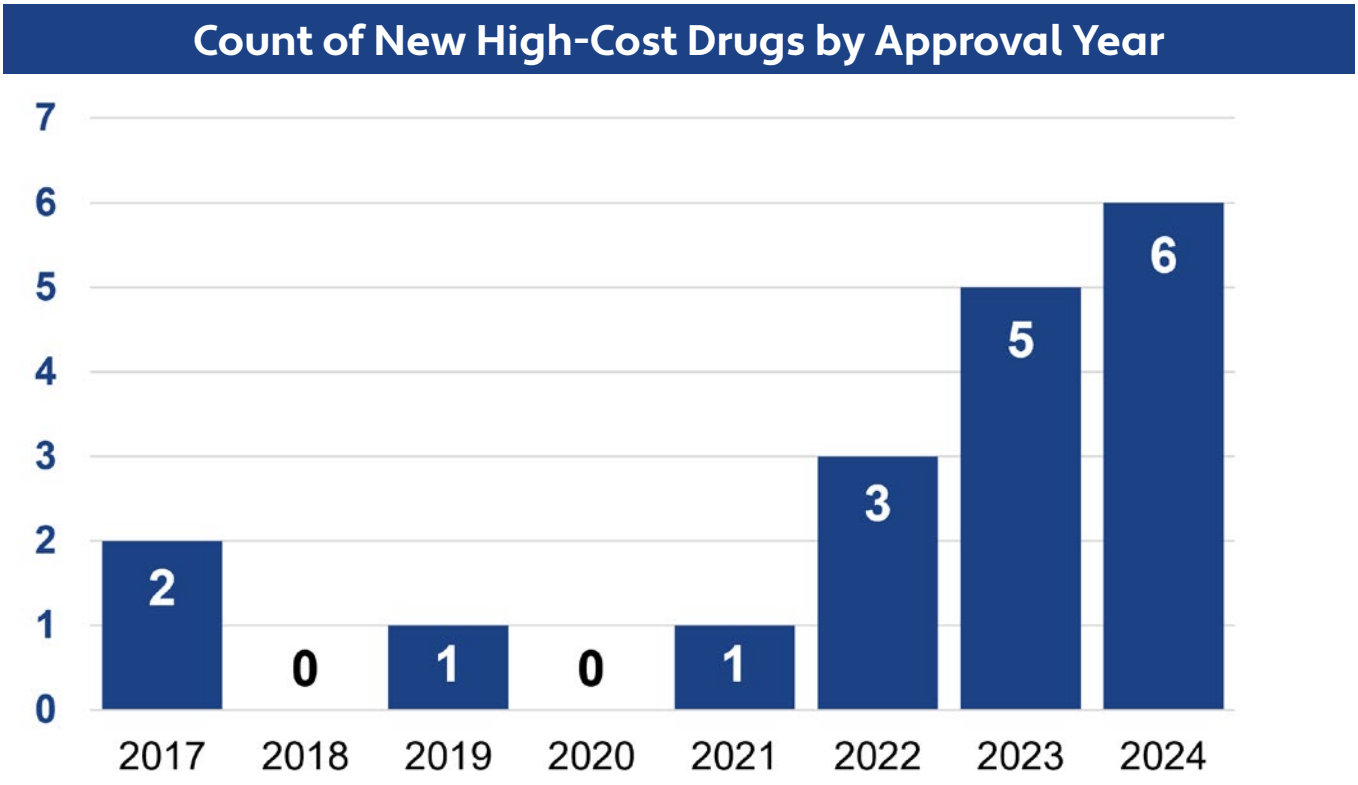
This report serves to memorialize the discussions and recommendations of the Illinois Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment. The contents of this report reflect the deliberations and consensus points raised during the council's meetings. While the recommendations represent the views of the majority of council members, they may not reflect unanimous agreement on each recommendation. Feasibility and implementation of the recommendations contained herein will be determined by the Illinois Department of Healthcare and Family Services (HFS) in consultation with relevant experts and stakeholders.

1. Introduction

The healthcare landscape is rapidly evolving with the increasing availability of advanced treatments for serious and often life-threatening diseases. These new therapeutics span a broad spectrum, including biologics for autoimmune disorders,[1] enzyme replacement therapies for rare genetic conditions,[2] and targeted cancer drugs.[3] Cell and gene therapies (CGTs) have emerged as a particularly complex category of high-cost drugs due to their transformative potential, high prices, and unique clinical demands. In the next decade many new CGTs are expected to become approved and available for use. Many existing and expected CGTs will target children and young adults, who are disproportionately insured by state Medicaid programs.

These therapeutics have high prices. As of November 2024, the United States Food and Drugs Administration (FDA) has approved 39* CGTs,[4] with an estimated 185 therapies looking to enter the U.S. market in the next three to five years.[5] The cost of CGTs can often exceed $1 million per patient.[6] For example, the approved CGTs for sickle cell disease (SCD), Lyfgenia and Casgevy, are listed at $3.1 million and $2.2 million, respectively.[7]



The counts reference one-time drugs with an estimated cost greater than $500,000. Estimated annual treatment costs range from $457,000 to $4.25 million for each drug. Estimated member counts for each one-time gene therapies range from 0 to 4 and 5 to 10 depending on the therapy indication.

* This count includes some CGTs, such as cord blood products, skin graft therapies, and treatments for certain cancers like bladder cancer and melanoma, which differ in scope and application from CGTs designed to treat rare genetic diseases like SCD or spinal muscular atrophy.

Recent FDA Approved Drugs 2023-2024

Sickle cell disease, transfusion-dependent β-thalassemia (TDT) (Casgevy)

Duchene muscular dystrophy, ages 4 and older (Elevidys)

Metachromatic leukodystrophy (MLD) (Lenmeldy)

Sickle cell disease (Lyfgenia)

Hemophilia A (Roctavian)

Hemophilia B (Beqvez)

Aromatic L-Amino Acid Decarboxylase Deficiency (Kebilidi)

These high prices present a significant challenge for states like Illinois, which are required to both maintain balanced budgets and cover all FDA-approved drugs. A balanced budget means that the state cannot spend more than it earns in revenue, necessitating careful consideration of spending decisions. Illinois must, therefore, assess how covering new therapies affects broader budgetary priorities and constraints.

With prescription drugs already accounting for an increasing share of the Medicaid budget, the high costs of CGTs and other advanced therapies—particularly those targeting pediatric populations—are poised to further intensify this financial strain.[8] For state Medicaid programs, these therapies present significant policy challenges, as they must now determine how to ensure access to these transformative treatments—offering curative potential or substantial quality-of-life improvements—while managing the significant financial burdens on state budgets these therapies impose.[9]

Adding complexity to the decision-making process for HFS and other insurers is the fact that some of the CGTs and high-cost drugs currently on the market have been approved through FDA's "accelerated approval pathway." This pathway allows drugs for serious conditions to be approved faster based on surrogate endpoints (indirect measures, such as biomarkers, that are likely to predict clinical benefit), rather than waiting for evidence that directly establishes long-term clinical benefits.[10] This pathway requires post-approval studies to confirm long-term efficacy and safety. The federal requirement to cover all FDA-approved drugs with a federal rebate agreement applies irrespective of the pathway under which they were approved. State Medicaid agencies must therefore balance the need to provide equitable and timely access to these potentially transformative therapies with the significant financial risk posed by treatments whose long-term value is not yet confirmed.

State Medicaid programs face financial risk concerns related to the unpredictability and magnitude of costs associated with covering high-cost therapies like CGTs. Their small target

populations make utilization patterns unpredictable and challenging to forecast while their significant, upfront expenditures can disrupt operational cashflows.

The absence of national or state registries comprehensively tracking the prevalence and incidence of all rare diseases targeted by CGTs and other high-cost therapies further compounds this forecasting challenge. However, Illinois does screen newborns for nearly all conditions included on the federally Recommended Uniform Screening Panel (RUSP), which includes a few rare diseases that have approved CGTs, such as SCD, spinal muscular atrophy (SMA), and certain forms of Severe Combined Immune Deficiency.[11] Illinois is currently in prescreening preparations to add new disorders with recently approved CGTs, like metachromatic leukodystrophy (MLD).

While budgetary limitations and forecasting challenges are central to policy considerations, there are also several critical access issues that affect the real-world impact of these therapies on patients' lives. Today, many patients face substantial challenges to accessing the existing standard of care for rare diseases, such as shortages of specialized providers, lack of geographically proximate care, and associated financial and logistical barriers.[12] These disparities are further exacerbated for patients seeking CGTs and high-cost drugs, which often require more advanced facilities, skilled practitioners with specialized training, and complex care coordination, along with extensive pre-treatment care that may require multiple appointments and significant time off of work or school for both patients and caregivers.

CGTs and high-cost drugs, particularly those requiring genetic manipulation or stem cell modification, often must be administered in hospitals equipped with advanced laboratory and treatment facilities.[13] In Illinois, these hospitals are located in metropolitan areas, creating geographic access barriers for patients in rural or underserved areas who have to travel significant distances to access care. This places substantial financial and logistical burdens on patients and their caregivers. Moreover, manufacturing and clinically administering CGTs and other high-cost drugs requires a specialized workforce, which can further limit the availability of these drugs.[14]

Complicating access further is the issue of continuity of care for patients, particularly children, who experience shifts among different payers—either between different health plans, between public and private insurance, or, in some cases, losing insurance coverage entirely and becoming uninsured. Changes in insurance can disrupt treatment and delay approvals for high-cost therapies, as it takes time to navigate through prior authorization and other requirements with a new payer. This disruption is particularly concerning for CGTs, which require stable, ongoing monitoring and coordination to ensure safety and efficacy.

Additionally, even if administered as a one-time treatment, receiving CGTs and other high-cost drugs can be a lengthy, complex, and physically demanding process that may deter some patients from pursuing them.[15] Despite these challenges, one councilmember and SCD patient advocate emphasized the importance of expanding access to the full range of therapies, including CGTs and other high-cost drugs, in order to provide patients with more opportunities for personalized care and improved quality of life. The challenges described above underscore

the need for flexible, patient-centered approaches to streamline access, reduce logistical burdens, and ensure continuity of care for patients undergoing these complex treatments.

Recognizing these critical issues, Illinois Governor JB Pritzker convened an advisory council to provide expert advice and a policy framework to help HFS address some of these financial and access challenges. The following section details the council's mandate, objectives, and composition.

The Advisory Council – Mandate, Principles, Composition, and Scope

On March 18, 2024, Illinois Governor JB Pritzker issued Executive Order 2024-01,[16] establishing the Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment (the council). The council was tasked with advising HFS on creating payment models and financing structures that support equitable access to treatments for SCD and other costly, life-changing therapies for Illinois Medicaid patients.

Over a series of meetings, the council engaged with healthcare experts, stakeholders, and policy advisors to evaluate value-based and outcome-driven financing strategies with a focus on ensuring sustainable and equitable access. Their findings provide a policy framework for HFS as it develops strategies to address the unique financial and access challenges these therapies pose. This document represents the council's final report.

Council's Vision Statement:

Provide access to emerging and transformative cell and gene therapies and other high-cost drugs and treatments to all Medicaid customers who need them.

Council's Guiding Principles:

- Ensuring patient access to evidence-based care and therapies
- Improving health outcomes for populations receiving care
- Prioritizing equity in the distribution and dissemination of care as a means to decrease disparities for impacted populations
- Maximizing the use of public resources in a financially responsible and ethically driven manner
- Developing appropriate reimbursement models
- Considering a public health perspective which leverages the state's purchasing power

Council Membership:

- **Chair**: Lizzy Whitehorn – Director, Illinois Department of Healthcare and Family Services
- **Members:**
    - Joan Ehrhardt, Chief, Health Assessment and Screening Section, Illinois Department of Public Health
    - Adam Flores, Senior Insurance Advisor, Illinois Department of Insurance

- Theodore (Ted) Tapas, Budget Analyst II, Governor's Office of Management and Budget
- Ifeanyi Beverly Chukwudozie, Director, Cancer Research Training and Education Coordination (CRTEC), University of Illinois Cancer Center- Person with lived experience as a person with sickle cell disease
- Alexandra Carpenter - Persons with lived experience as a person with a condition requiring access to new, innovative drugs or treatment
- Dr. James LaBelle, Associate Professor of Pediatrics, UChicago Medicine - Providers treating sickle cell disease patients
- TaLana Hughes, Executive Director, Sickle Cell Disease Association of Illinois
- Nathan Schaefer, Senior Vice President, National Hemophilia Foundation - Advocacy organization for a condition(s) requiring access to new, innovative drugs and treatment
- Steve Sproat, Pharmacy Director, Aetna Better Health – Health Insurance Industry Representative
- Brian Smolich, Vice President of Quality and Managed Care Operations, Health Alliance Medical Plans - Health Insurance Industry Representative
- George Kitchens, Co-CEO, Artia Solutions - Expert in prescription drug rebate negotiations and outcomes-based agreements
- Susan Stuard, Director, State Technical Assistance, Oregon Health & Science University - Expert in prescription drug rebate negotiations and outcomes-based agreements
- Dr. Melissa Creary, Assistant Professor, University of Michigan - Expert with published research in financing new, innovative drugs and treatments within public health insurance programs
- Rachel Sachs, Professor of Law, Washington University School of Law - Experts with published research in financing new, innovative drugs and treatments within public health insurance programs
- Dr. Anirban Basu, Stergachis Family Endowed Director & Professor, Pharmaceutical Outcomes Research and Policy Program, University of Washington - Expert with health economist or actuarial background
- Dr. Rena Conti, Associate Professor, Boston University - Expert with health economist or actuarial background
- Dr. Radhika Peddinti, Associate Professor of Pediatrics, UChicago – At-Large Member, who may or may not meet the qualification requirements for the other appointees
- Mark Trusheim, NEWDIGS Strategic Director, Tufts Medical Center – At-Large Member, who may or may not meet the qualification requirements for the other appointees
- Dr. Ruchika Goel, Professor, Hematology/Oncology, SIU School of Medicine - Providers treating patients with another condition requiring access to new, innovative drugs or treatment

Council Meetings and Scope of Discussions

The council convened five times between June and September 2024. Each meeting was designed to address different aspects of the council's mandate and build toward a comprehensive set of recommendations:

1. **June 25, 2024:** The first meeting included an overview by HFS introducing the issue and a discussion of the council's vision statement and guiding principles. The council engaged in an open brainstorming session on payment and financing models available to HFS.
2. **July 23, 2024:** Councilmembers heard directly from patients and caregivers with lived experience, followed by an open brainstorming session on access and equity challenges.
3. **August 27, 2024:** Councilmembers discussed proposed guidelines and recommendations on payment and financing models for high-cost therapies.
4. **September 17, 2024:** Further refinement of the proposed guidelines and recommendations on payment and financing models for high-cost therapies.
5. **September 24, 2024:** Councilmembers discussed proposed guidelines and recommendations to ensure equitable access to high-cost therapies.

Throughout these meetings, discussions primarily centered on financing and access issues related to CGTs for rare diseases, such as SCD, specifically for the state's Medicaid population. While certain recommendations discussed in this report may also apply to (1) other high-cost drugs that are not CGTs or (2) CGTs targeting more common conditions, these therapies were not the primary focus of the council's discussions.

A Focus on Sickle Cell Disease

Many high-cost drugs, and CGTs in particular, tend to target rare diseases. In the United States, the Orphan Drug Act defines a "rare disease" as a condition that affects fewer than 200,000 people. The Act provides financial incentives for drug development targeting rare diseases; this investment has encouraged research and the development of innovative treatments. While CGTs target several types of rare diseases, SCD stands out among other rare diseases with CGTs currently available in a few key ways.

First, SCD is one of the most prevalent rare diseases, affecting an estimated 100,000 individuals in the country.[17] For comparison, spinal muscular atrophy, another rare disease with an approved CGT, affects about 10,000 to 25,000 individuals in the country.[18] Illinois has a higher-than-average prevalence of SCD among its Medicaid customers compared to the national average prevalence.[19] Medicaid claims data suggest that at least 6,000 Medicaid customers in Illinois have SCD, likely accounting for approximately half of the state's total population with the disease.[20]

Second, in the U.S., SCD predominantly affects African Americans, who are more likely to experience systemic healthcare barriers, including limited access to specialized care, financial constraints, and disparities in care quality.[21] While the disease is rare overall, it is far more common within the African American community, affecting 1 in 365 births.[22] Addressing equity for SCD patients is critical, as they experience unique challenges not only due to the rarity and complexity of the disease but also due to intersecting racial and socioeconomic disparities.

While the council listened to patients, advocates, and providers affected by various rare diseases, discussions and recommendations often focused on equitable access and financing strategies for CGTs and other high-cost drugs that could benefit SCD patients. The council's emphasis on equity aims to ensure that Medicaid patients with SCD have access to transformative therapies, overcoming barriers that have historically limited treatment options and outcomes for this population.

List of Recommendations by the Advisory Council

The recommendations reflect the council's deliberations, which addressed three distinct but interconnected areas of focus: (1) strategies for addressing the unique financial challenges posed by CGTs and other high-cost drugs, including innovative payment models and negotiating strategies; (2) specific measures HFS can take to improve equitable access and care coordination for Medicaid customers with SCD and other rare diseases; and (3) broader systemic and federal-level advocacy efforts to support HFS's ability to provide equitable and sustainable access to CGTs and other high-cost therapies to Medicaid customers.

Recommendations for HFS on Financing CGT and Other High-Cost Therapies

1. *Ensure CGT and other high-cost drugs are considered "covered outpatient drugs" to entitle the state to rebates and discounts on their use.*
2. *Consider ways to estimate manufacturer production supply capacity and use HFS data and analysis to estimate eligible patient populations and their treatment adoption rates to inform drug negotiation strategies and budget impact analysis.*
3. *Consider using a third-party assessment to benchmark drug value.*
4. *Evaluate the federal CGT access model and determine whether participation would provide cost and access benefits.*
5. *Consider strategies to secure larger, volume-based rebates or discounts.*
6. *Continue to use the uniform PDL as leverage in negotiating prices and, where feasible, leverage the presence of alternative therapies in price negotiations.*
7. *Explore all available discounting and rebating strategies on a case-by-case basis for each CGT or high-cost drug. Supplemental discounts and rebates are likely to be easier to implement, but other strategies, including Value-Based Agreements (VBAs), might be appropriate in certain specific circumstances.*

Recommendations for HFS on Ensuring Equitable Access to CGT and Other High-Cost Therapies

8. *Consider establishing uniform utilization management (UM) criteria across MCOs to ensure equitable access to CGTs and high-cost therapies for all Medicaid customers.*
9. *Consider developing UM criteria that ensure access encompasses not just the CGT or high-cost drug itself but also the full continuum of pre- and post-therapy services, such as preparation, follow-up monitoring, and long-term patient care, which are essential for delivery and patient outcomes.*
10. *Collaborate with expert clinicians to create initial UM guidelines for CGTs and high-cost drugs that distinguish between populations for whom FDA approval was granted and those included in clinical trials, ensuring clinical inclusion criteria are aligned with evidence and include provider assessments and shared decision-making with patients.*
11. *Use a combination of policy levers—including the model MCO contract, standardized single-case agreements, and HFS policies and procedures—to implement and adapt these UM criteria effectively. Collaborate with state-based experts who have specific expertise in the state's insured population and their multidimensional needs.*
12. *Consider stratifying managed care customers with SCD and other rare diseases as high-risk early in diagnosis to enable timely access to specialized care coordination.*
13. *Consider expanding tailored care management services for rare disease patients.*
14. *Consider developing and implementing disease-specific performance metrics to monitor and enhance the quality of MCO care coordination for rare disease patients, ensuring timely access, high-quality care, and comprehensive support.*
15. *Consider expanding Section 1115 waiver health-related social needs services to cover more patients with rare diseases and a broader range of wrap-around services for Medicaid customers undergoing CGTs and other high-cost therapies, including essential supports like temporary housing near a qualified treatment center, long-distance transportation, parking reimbursement, and childcare, to ensure equitable access to advanced therapies for patients with rare diseases.*
16. *Consider ensuring that both in-state and out-of-state health centers in contiguous states administering CGTs and other high-cost drugs are included in Medicaid networks to ensure equitable patient access throughout Illinois. Also consider incentivizing a robust network of specialized providers, including hematologists, across the state and ensuring that provider networks are accurate.*
17. *Explore the use of financial and nonfinancial incentives and supports to encourage specialist availability and patient access for rural populations.*
18. *Consider balancing access to CGTs and other high-cost drugs with support for comprehensive care for all patients with SCD and other rare diseases, ensuring that resources are allocated to maintain high-quality preventive and routine services for those who may not qualify for or require these high-cost therapies.*

Additional Considerations that Can Impact Financing of and Access to CGTs

19. *Consider expanding HFS staff to include clinical and pharmaceutical experts and seek consulting support to implement the above recommendations, including to support enhanced tracking, reporting, and negotiations for CGTs and other high-cost drugs.*
20. *Strive to promote improved access to care at the provider-level for patients with rare diseases seeking complex therapies by supporting the expansion of complex care coordination and health IT infrastructure.*
21. *Consider evaluating available financial risk mitigation strategies, such as reinsurance, annuity models, and multi-payer risk pools, and monitor the implementation of financial risk mitigation strategies in other state Medicaid programs.*
22. *Consider reviewing state regulatory options and advocating for federal regulatory changes to ensure access to CGTs and other high-cost drugs in ACA and employer-sponsored plans.*
23. *Consider advocating for federal policies that advance equitable access to advanced therapies, deter other payers from shifting patients to Medicaid, and enhance state Medicaid agencies' capacity to pay for CGTs and other high-cost drugs.*

2. Background

A. Understanding High-Cost Drugs and CGTs

High-cost drugs are typically defined based on their price per unit or the total cost of the entire course of treatment, with no single universally accepted threshold. While some high-cost drugs are one-time treatments, others require continuous use that can result in high cumulative costs.

Specialty drugs are usually defined as a type of high-cost drug requiring complex manufacturing and specialized handling or administration.[23] They are often administered via infusions, sometimes in clinical settings, and require ongoing patient management, including close monitoring of side effects, response, and adherence.

CGTs, a subset of high-cost specialty drugs, are advanced medical treatments designed to address the root causes of diseases at a genetic or cellular level. Unlike conventional therapies that often manage symptoms, CGTs seek to modify genetic material or utilize engineered cells to restore normal function.[24] This approach holds the promise of long-lasting, transformative, and potentially curative effects for serious or life-threatening conditions, offering hope to patients seeking alternatives to lifelong treatment regimens or options for diseases previously considered untreatable.

However, the cost per treatment can exceed $1 million. For example, the cost of the two recently approved CGTs for SCD are $2.2 million and $3.1 million. Additionally, there are often significant associated medical costs, including pre-treatment preparation, hospital stays, and follow-up care. Receiving CGT is often a demanding, multi-step process requiring repeated travel to specialized health centers equipped to administer these therapies.[25] Patients and their families may need to take extended time off of work or school, arrange for childcare, and manage the logistical challenges of extended hospital stays. Further, CGTs can expose patients to significant side effects. For example, patients with SCD who undergo CGT may face an increased risk of infertility, especially if chemotherapy-based conditioning is required, as well as a potential cancer risk, depending on the specific gene therapy approach used.[26]

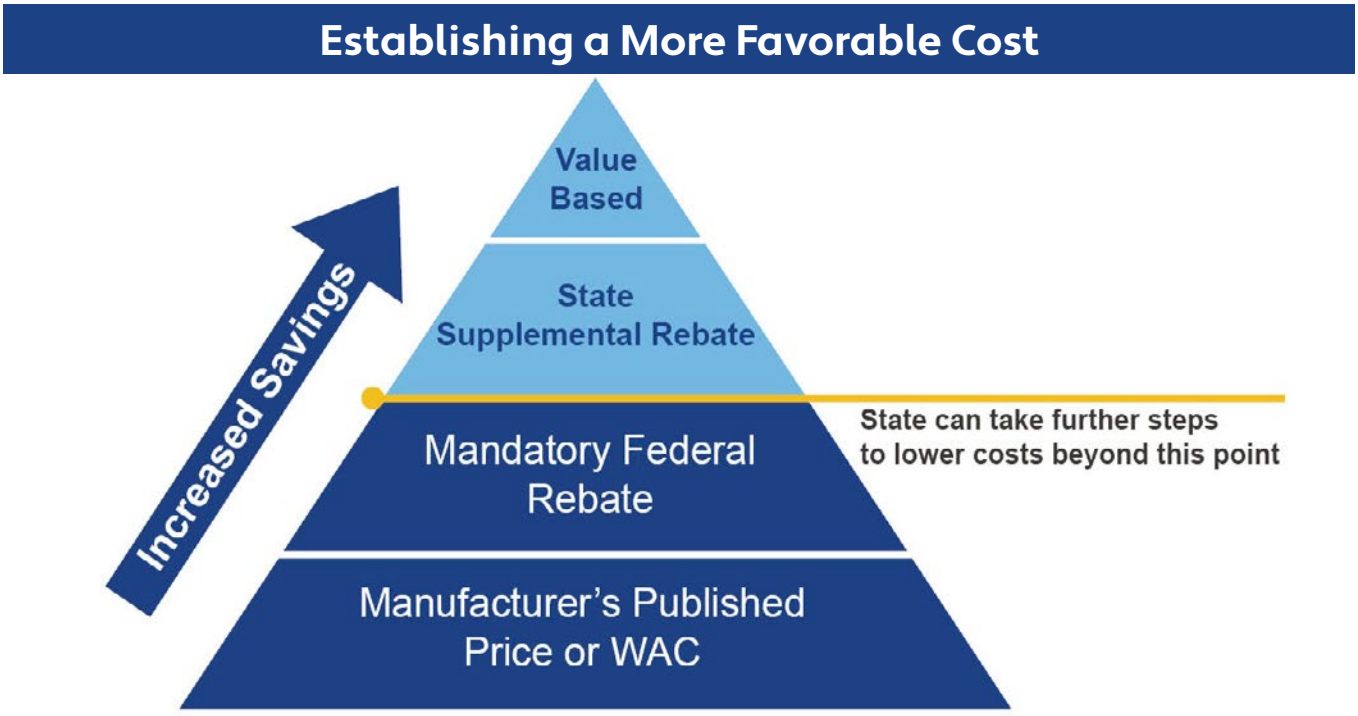
Advocates also noted that it is essential to ensure that CGTs are made available alongside the current standard of care. For example, for SCD, the standard of care focuses on managing symptoms, preventing complications, and improving patients' quality of life. While it often involves significant costs and repeated hospital visits throughout a patient's life, it remains a critical option for maintaining stability and preventing life-threatening complications. Many patients may still prefer the standard of care due to the risks, intensive demands, or uncertain long-term outcomes associated with CGTs.

However, accessing high-quality standard-of-care services remains a significant challenge.[27] A survey of SCD healthcare providers in Illinois conducted by the Illinois Department of Public Health identified transportation challenges, a lack of specialists, and insurance complications as the top barriers. These challenges apply across all types of insurance coverage and payers, not just Medicaid.

B. Federal Medicaid Coverage Requirements and Drug Rebates

Created in 1990, the Medicaid Prescription Drug Rebate Program (MDRP) requires a drug manufacturer who wants Medicaid to cover its drugs to enter into a rebate agreement with the Secretary of Health and Human Services.[28] However, under federal law only "covered outpatient drugs" are eligible for the rebates. The term "covered outpatient drugs" encompasses any drug that is paid for separately and not through bundled payments (fixed payments that cover all services provided during a single episode of care, such as a hospital stay). A state could require a drug administered in an inpatient setting to be billed as a separate line item on a claim. This would result in the drug being deemed a "covered outpatient drug," thereby making it eligible for rebates.[29] While CGT drugs are administered in both inpatient and outpatient settings, as a matter of billing policy, a state could require separate billing for CGT drugs irrespective of the setting in which they are administered.

Under the MDRP, state Medicaid agencies, including HFS, are required to cover almost all FDA-approved drugs, including CGTs.[30] This applies to drugs approved through the traditional pathway as well as drugs approved through the accelerated pathway.[31] In exchange for this mandatory coverage, manufacturers must provide discounts to offset the cost of these drugs for Medicaid customers. Federally mandated rebates are shared between the state Medicaid agencies and the federal government.



Almost all states have additional supplemental rebate agreements in place with manufacturers to extract additional discounts. States often offer inclusion on their preferred drug list (PDL) as an incentive for manufacturers to provide these additional discounts.[32] States can also structure these supplemental rebates in the form of outcomes- or value-based agreements (OBAs or

VBAs) to mitigate financial risk by tying payments to patient outcomes. These agreements link payment to patient outcomes, helping offset the financial risk for Medicaid programs while ensuring access to new therapies. While the federally mandated rebates are substantial, the additional supplemental rebates negotiated by the states can be more modest. For example, in 2021, the federal rebates reduced Medicaid prescription drug costs nationally by $39.7 billion, while total supplemental rebates negotiated by states amounted to $2.8 billion.[33] Out of this $2.8 billion, Illinois received $160 million (5.7%).

The federal government receives a portion of the supplemental rebates determined by the Federal Medical Assistance Percentage (FMAP), which represents the portion of each state's Medicaid costs paid for by the federal government. For Illinois, the FMAP percentage is 51.38% for those traditionally eligible for Medicaid, 65.97% for CHIP enrollees, and 90% for those who are eligible because of the Affordable Care Act's (ACA) Medicaid expansion is 90%.

HFS currently negotiates state supplemental rebates with drug manufacturers. HFS received federal approval to enter Outcomes-Based Agreements (OBAs) in March 2024 through a State Plan Amendment (SPA); HFS has not implemented an OBA yet. HFS is pursuing a federal SPA that would allow it to carve out CGTs from bundled Diagnostic Related Groups (DRG) payments to make them eligible for rebates.

However, while these state-level efforts are vital for addressing immediate fiscal pressures, a more sustainable approach may require additional assistance or support from the federal government. This could include long-term federal strategies such as enhanced funding mechanisms or expanded rebate programs to support affordability across the country. These strategies are discussed in more detail in Sections 5D and 5E of this report.

C. Federal CGT Access Model

In 2024, the Centers for Medicare & Medicaid Services Innovation Center (CMS) introduced the CGT Access Model, a new initiative designed to expand Medicaid patients' access to CGTs for serious, rare diseases. The program's initial focus is on therapies for SCD, and CMS plans to operate the model for 11 years. Participation is voluntary for both states and manufacturers, and states can begin participating between January 2025 and January 2026.

This model is a "CMS-led approach to developing and administering outcomes-based agreements (OBAs)" specifically for CGTs. An OBA is an agreement between payers and drug manufacturers that ties payment to a therapy's real-world effectiveness, often basing reimbursement on specific patient health outcomes. The goal is to enhance treatment access, improve patient health outcomes, and alleviate the financial burden on state Medicaid budgets.

In 2024, CMS announced that it had reached an agreement with the two manufacturers of the CGTs for SCD on pricing and outcome criteria.[34] CMS will now work with states to help them decide whether to participate in the model. Participating states can adopt the CMS-negotiated agreement as the basis for their agreements with each manufacturer. CMS will also provide

technical assistance and funding to support participating states in data collection, model implementation, and OBA enforcement. For Medicaid customers participating in the model, the program may extend coverage beyond the costs of the CGT itself to include supportive services, such as fertility preservation, travel assistance, case management, and behavioral healthcare.

HFS has submitted a letter of interest to CMMI, indicating potential interest in participating in the model. Illinois is currently evaluating whether to enter the model.

D. Understanding the Illinois Landscape

Structure of Illinois Medicaid Program

Illinois Medicaid customers are either in the state-run fee-for-service (FFS) program or in a health plan operated by one of the state's Medicaid Managed Care Organizations (MCOs). About 80% of the state's Medicaid enrollees are enrolled in MCOs. Illinois Medicaid does not impose premiums or cost-sharing for customers. HFS enters a contract with each MCO establishing standards for, among many things, approving medical benefits. Current MCO contracts in the state generally require any utilization management or prior authorization criteria to be clinically sound, but, beyond that, the state does not have uniform prior authorization criteria that the MCOs must follow.

For prescription drugs, MCOs must establish a drug utilization review board (DUR board) to review and monitor the prescribing, dispensing, and use of medications within their network.. Generally speaking, MCOs can set their own clinically appropriate criteria. The Illinois FFS program has its own DUR board. However, Illinois uses a uniform preferred drug list (PDL), meaning that when the state adds a drug to the PDL, MCOs must also designate the drug as "preferred." Of the drugs on Illinois' PDL, 61% do not allow for any prior authorization.

Limited Sites of Care Offering CGTs and other High-Cost Drugs

Due to the complexity of administering CGTs and other high-cost drugs, a limited number of facilities are equipped to provide treatments. For example, only four healthcare facilities in Illinois† are equipped to provide CGT treatments, and all of them are in Chicago (referred to as "qualified treatment center" or QTC in this report). Further, not every QTC provides every CGT currently available on the market, further narrowing the pool of potential sites of care available for these services. The limited number of QTCs offering CGTs is due to several factors: these therapies require specialized infrastructure, such as advanced laboratory facilities, to handle cellular modifications safely. Additionally, CGTs often involve intricate and novel medical procedures, necessitating a highly trained workforce of specialists, including hematologists, geneticists, and transplant experts.

† University of Chicago Medical Center; University of Illinois Hospital & Health Sciences System; Northwestern Memorial Hospital; and Ann & Robert H. Lurie Children's Hospital of Chicago.

Even when a QTC offers a particular CGT, it can only treat a small number of patients simultaneously. As demand for CGTs grow, Illinois Medicaid customers might have to compete for these limited treatment spots not just with commercially insured patients but likely patients from neighboring states without sufficient access to hospitals that can administer CGTs.

Healthcare providers offering high-cost advanced therapies often establish single-case agreements with MCOs for the CGT, even if the provider is in the MCOs provider network.[35] These agreements are individualized contracts between an MCO or state Medicaid agency and a healthcare provider to cover a specific, often high-cost, service or treatment for a particular patient. Single-case agreements typically outline specific reimbursement terms negotiated on a case-by-case basis, reducing financial uncertainty for providers who face high upfront costs for advanced therapies, like CGTs.

3. Recommendations for Financing CGTs and Other High-Cost Therapies

A. Excluding CGTs and other High-Cost Drugs from hospital Diagnostic Related Groups (DRGs)

One of the council's primary recommendations for financing CGTs in Illinois is to carve CGTs and other high-cost drugs out of hospital Diagnostic Related Groups (DRGs). This approach would make these therapies eligible for rebates and offer financial flexibility, helping to mitigate the significant financial burden CGTs impose on state Medicaid budgets.

> ***Recommendation 1:*** *HFS should ensure CGT and other high-cost drugs are considered "covered outpatient drugs" to entitle the state to rebates and discounts on their use.*

Understanding DRGs and Their Role in Hospital Reimbursement

Diagnostic Related Groups (DRGs) are a classification system developed by the Centers for Medicare & Medicaid Services (CMS) to facilitate bundled hospital reimbursement based on the complexity of patient cases. Most state Medicaid programs use a DRG system to reimburse hospitals for inpatient care, providing a single, bundled payment that covers both the services rendered and any drugs given to the patient. CMS assigns each bundle, or DRG, a specific weight, representing the resources necessary to treat a particular condition. This weight is used alongside hospital-specific data to determine reimbursement rates.

Limitations of Bundled Payments for CGTs and other High-Cost Drugs, and Ensuring Eligibility for Rebates

The DRG bundled payment structure may discourage hospitals from providing these therapies because the costs of CGTs for hospitals can far exceed the standard DRG reimbursement. To address this, some state Medicaid agencies have established separate payment policies, carving CGTs out of bundled payments to ensure adequate hospital reimbursement and maintain patient access.

While CGT drugs can be administered in either inpatient or outpatient settings, only "covered outpatient drugs" are eligible for rebates. Covered outpatient drugs are generally defined as drugs billed as separate line items and not included in a bundled payment structure, such as a DRG. Most inpatient drugs, unless explicitly carved out of the DRG, fall under bundled payment structures and are thus ineligible for federal and state supplemental rebates. If Illinois were to mandate that all CGTs and high-cost drugs administered in an inpatient setting be carved out and billed as separate line items, these drugs would also become eligible for federal and state rebates.

In light of these policy considerations, Illinois is currently pursuing a State Plan Amendment (SPA) to enable the state to carve-out these drugs from DRGs. This would allow Illinois to establish a separate payment model for CGTs and high-cost drugs, better aligning reimbursement with the actual cost of these treatments and ensuring that the state captures federal rebate savings and allow for state negotiated rebates.

Interplay between Medicaid Drug Rebates and the 340B Drug Discount Program

Under the federal 340B drug discount program, drug manufacturers must provide significant discounts on outpatient drugs to eligible facilities that serve low-income and uninsured populations.

The federal government prohibits "duplicate discounts," and under this rule, a single drug claim cannot receive both a 340B discount and a Medicaid rebate under the MDRP.[36] In practice, 340B-covered entities, which include all the qualified treatment centers offering CGTs, must decide whether to "carve in" or "carve out" Medicaid patients from the 340B program.

When they "carve in" Medicaid patients, these providers use 340B-purchased drugs for Medicaid claims, meaning they forgo Medicaid rebates for those claims to comply with the duplicate discount prohibition. If they choose to "carve out" Medicaid patients, they do not use 340B-purchased drugs for Medicaid claims, allowing the Medicaid program to seek rebates through MDRP instead.

The council considered whether the 340B discounts could secure cost reductions for CGTs but ultimately did not make a recommendation on 340B drug discounts.

While 340B discounts are available for physician-administered drugs, including infusion therapies, they only apply when administered in an outpatient setting. Since some CGTs require inpatient administration, this restriction can limit the applicability of 340B discounts for these therapies.

Although the 340B program provides considerable savings to eligible hospitals, there is no guarantee that these savings would directly benefit the state Medicaid agency as the Medicaid reimbursement rate remains the same.

B. Preparing to Negotiate with Drug Manufacturers

As Illinois prepares to negotiate pricing for CGTs and other high-cost drugs, it will need to lay the groundwork for these negotiations by (1) estimating the demand and supply for CGTs and other high-cost drugs, (2) establishing a benchmark for each drug, and (3) deciding whether to pool its purchasing power.

Estimating Supply and Demand for CGTs

Estimating supply (the availability of the drugs themselves and the capacity of the treatment centers administering the drug) and demand (the eligible patient population who are able and

willing to undergo the treatment) for CGTs for the Medicaid beneficiary population is crucial not only for understanding the potential impact on the Medicaid budget but also for framing discussions with manufacturers, where anticipated treatment volume can influence pricing strategies.

To improve negotiation outcomes, HFS should consider ways to use HFS data and analysis to estimate eligible patient populations and their treatment adoption rates to inform drug negotiation strategies and budget impact analysis. Beyond this, Illinois can consider leveraging collaborative approaches with manufacturers and third-party resources to obtain reliable data on the eligible patient population and the projected demand for specific CGTs.

> ***Recommendation 2:*** *HFS should consider ways to estimate manufacturer production supply capacity and use HFS data and analysis to estimate eligible patient populations and their treatment adoption rates to inform drug negotiation strategies and budget impact analysis.*

Estimating supply availability, which includes both manufacturers' ability to produce the drugs and hospitals' capacity to administer them, helps the state anticipate potential bottlenecks. This insight can guide negotiations on pricing and supply guarantees.

1. **Provider Capacity**: As discussed above, provider capacity for administering CGTs in Illinois is limited and primarily located in larger urban centers. Further, the number of patients that each site of care can accommodate is limited, and Medicaid customers might have to compete for these spots with the commercially insured populations as well as patients from bordering states, creating a potential supply bottleneck. All these barriers can limit the number of Medicaid customers who are practically able to access treatment.
2. **Drug Supply Constraints**: CGTs and other high-cost drugs are often challenging to produce, requiring specialized manufacturing processes and significant time and resources. These production constraints can limit the availability of CGTs, impacting how many Medicaid customers can realistically be treated within a given timeframe. Having knowledge of supply limitations can influence how the state approaches negotiations. For example, if production is highly constrained, the state might seek guarantees on supply availability or prioritize access for specific populations, and if the supply is scalable, the state could pursue volume-based discounts. Further, understanding the challenges and costs associated with producing CGTs can also help the state gain insights into the manufacturers' production costs, allowing the state to evaluate whether the price being negotiated is reasonable.

Estimating Demand: Understanding the eligible patient population size within a Medicaid program allows the state to project the budgetary impact of the therapy and determine its potential value. High demand could justify a need for volume-based discounts, while limited demand might highlight the need for flexible pricing structures or risk-sharing agreements.

1. **Connection to Standard of Care***:* A critical factor in estimating demand for the novel CGTs is determining how many patients in the Medicaid program are already connected to the current standard of care for their condition. This assessment would help identify gaps in care and understand which patients may be most likely to seek CGTs. Additionally, evaluating how patients access (or struggle to access) the standard of care is critical to this analysis.
2. **Screening and Treatment Capacity***:* Given that only a small percentage of rare disease patients are likely clinically eligible for CGTs, identifying eligible patients and enrolling them in treatment in a timely manner can be challenging. Estimating demand will require insight into how quickly patients can be screened and initiated into treatment upon approval. Currently, a patchwork of disease-specific registries exists, maintained by government agencies, research institutions, or patient advocacy organizations. For example, the Centers for Disease Control and Prevention (CDC) created the Sickle Cell Data Collection (SCDC) Program as a multistate collaborative. SCDC collects data from a variety of sources to track the prevalence and incidence of SCD at the state level. However, an expansion of this program beyond the current 16 state participants, which does not include Illinois, is dependent on additional federal resources.

   New efforts and resources beyond those like newborn screening are needed to adequately serve individuals eligible for CGTs, because newborn screening may not identify every newborn with SMA or other rare diseases included on the newborn screening panel with CGTs. Building robust data collection and monitoring infrastructure will be essential for improving screening and enrollment for CGTs, as well as for accurately estimating the demand for these therapies.
3. **Patient Awareness and Preferences***:* Understanding patient interest in CGTs and potential barriers to acceptance is crucial in accurately forecasting demand. This includes assessing the level of patient awareness, willingness to undergo treatment, and preferences regarding innovative but high-risk therapies like CGTs.

Estimating Drug Value

Determining the value of a CGT is essential for negotiating a fair price, and third-party assessments can offer valuable reference points. Several reputable institutions specialize in health economics and value assessments for high-cost therapies. These organizations produce valuations and comparative studies that can provide reference points for estimating drug value, which can be used during negotiations with drug manufacturers.

Complexities in Valuing High-Cost Therapies: Establishing the value of CGTs is challenging due to the wide range of data inputs and the transformative yet uncertain nature of these treatments. Beyond clinical and financial benefits, it is important to consider whether the price of these therapies ought to reflect factors such as the therapy's potential to reduce caregiver strain, alleviate long-term economic hardship, and improve educational or employment outcomes for patients and their families.[37] These factors can add substantial value that traditional pricing metrics may overlook.[38] Studies from diverse sources often yield

varied estimates, but comprehensive, evidence-based valuations can help the state more fully understand these multifaceted benefits, enabling more informed and equitable price discussions.

> ***Recommendation 3:*** *HFS should consider using a third-party assessment to benchmark drug value.*

Pooling Purchasing Power

Illinois should carefully assess the potential benefits of participating in the federal CGT access model as it prepares for CGT negotiations. This model offers states an opportunity to pool purchasing power under federally negotiated terms, which could alleviate administrative burdens such as collecting and analyzing outcome measures. By joining the model, Illinois could benefit from the federal government's purchasing leverage and technical expertise, which may lead to more cost-effective agreements and improved patient access to CGTs. Alternatively, Illinois could also use the federal framework as a benchmark to inform its independent negotiations, maintaining flexibility while leveraging the model's insights and negotiated terms.

Within the state, some councilmembers recommended that HFS explore collaborating with the State Employee Health Plan as another avenue to expand the state's purchasing leverage and secure more favorable terms in CGT negotiations.

Some states participate in multi-state purchasing pools, which allow them to collectively negotiate drug prices and rebates, thereby leveraging greater purchasing power to reduce the financial impact of high-cost drugs. As of 2023, three pools focus on Medicaid purchasers: the National Medicaid Pooling Initiative, the Top Dollar Program, and the Sovereign States Drug Consortium. These pools leverage participating states' preferred drug lists to generate savings. According to one estimate, states participating in these pools generally save between 3 and 5% on prescription drug prices, but these amounts can vary depending on whether a state negotiates as a single purchaser or in collaboration with others.[39] Some evidence finds that purchasing pools might not be very effective at cost containment, but they can potentially help increase access to gene therapies for Medicaid customers.[40] By aggregating demand and standardizing access criteria across states, purchasing pools can make it easier for Medicaid beneficiaries, particularly in smaller states, to access therapies that might otherwise be unavailable due to limited negotiating power or logistical barriers.

HFS does not currently participate in any multi-state purchasing pools. As a large state, Illinois has been able to leverage its sizable Medicaid population to secure favorable pricing and rebate terms directly with manufacturers. Additionally, Illinois has its own Preferred Drug List (PDL) and a well-established process for negotiating supplemental rebates.

Councilmembers expressed differing views on whether Illinois should join a larger group of states to negotiate CGTs. Some councilmembers who have participated in CGT drug negotiations have found that manufacturers sometimes prefer to negotiate directly with

larger states due to the administrative simplicity of a single, high-volume contract compared to coordinating with a multi-state group. These councilmembers emphasized that Illinois's independent approach could yield comparable or even better terms without the constraints of multi-state coordination.

However, other councilmembers argued that irrespective of its size advantage, Illinois could still benefit from the increased negotiating leverage of a larger purchasing pool. They pointed out that these purchasing pools could especially be helpful in situations where the state does not have a sufficient patient population.

Given these differing perspectives, the council reached no recommendation on whether Illinois should join a multi-state purchasing pool.

> ***Recommendation 4:*** *HFS should evaluate the federal CGT access model and determine whether participation would provide cost and access benefits.*

C. State Options During the Drug Price Negotiation Process

Negotiating with drug manufacturers over CGTs poses significant challenges for state Medicaid programs. Because state Medicaid programs are required to cover all FDA-approved drugs under the MDRP, the negotiation process inherently favors manufacturers. This creates an asymmetric dynamic where Medicaid agencies must employ thoughtful strategies to balance this power imbalance, ensuring fair pricing and expanding access to CGTs for Medicaid customers. Illinois could consider a few different approaches during drug price negotiations, from improving patient access to using alternative therapies as leverage.

Improving Access to CGTs

Expanding patient access to CGTs could simultaneously support equity goals and increase Illinois's negotiation power by promising manufacturers a larger number of potential recipients. The state can consider a few different strategies to improve access to CGTs.

1. **Improved Screening of Medicaid Patients:** Implementing comprehensive screening protocols can identify more patients eligible for CGTs, increasing the potential treatment population.
2. **Outreach to Disadvantaged Populations:** Conducting targeted outreach about rare diseases and all available therapies can ensure that underserved communities are diagnosed and have timely and equitable access to necessary care, including cutting-edge treatments such as CGTs.
3. **Investing in Infrastructure at Qualified Treatment Centers:** Enhancing facilities and resources at specialized treatment centers can increase treatment capacity, making the state a more attractive partner for manufacturers.

The state could use these strategies to negotiate different types of volume-based discounts, as discussed in Section 3D below.

However, several councilmembers noted that expanding access to CGTs should be accompanied by improvements in access to the current standard of care. A system that favors high-cost therapies over longstanding, widely available options could inadvertently create structural biases in favor of expensive treatments. This issue is particularly complex when defining the standard of care for SCD. While therapies like hydroxyurea and blood transfusions are considered the baseline, newer, non-CGT disease-modifying therapies, such as crizanlizumab (Adakveo), have emerged over the years.[41] These newer treatments are also costly, and councilmembers expressed differing views on their value and whether they should be considered the new standard of care. Illinois must prioritize what is best for patients, ensuring equitable access to both standard-of-care options and novel treatments.

> ***Recommendation 5:*** *HFS should consider strategies to secure larger, volume-based rebates or discounts.*

Using Alternative Therapies as Leverage

Alternative therapies can offer another form of leverage, though using them in negotiations in relation to CGTs and other high-cost drugs is rarely practical. In cases where multiple treatment options exist for the same condition, Illinois Medicaid could potentially leverage its PDL to incentivize manufacturers to offer better terms. For instance, the state might negotiate to make a product preferred on the PDL over competing therapies if the manufacturer agrees to favorable pricing or rebate terms.

However, CGTs are not always interchangeable; each therapy may offer unique benefits, side effects, and eligibility criteria that make direct substitution challenging. Additionally, differences in effectiveness, cost, and opinions among providers and patients on what constitutes standard care can complicate these comparisons, limiting the state's ability to rely solely on alternatives as leverage.

> ***Recommendation 6:*** *HFS should continue to use the uniform PDL as leverage in negotiating prices and, where feasible, HFS should leverage the presence of alternative therapies in price negotiations.*

D. Exploring Different Discounting and Payment Strategies for CGTs

In managing the financial burden of high-cost therapies like CGTs, states can use innovative payment models such as value-based agreements (VBAs), outcome-based agreements (OBAs), subscription, and annuity payment models.[42] Supplemental rebates, negotiated by state Medicaid agencies in addition to the federal statutory rebate, can take various forms, including price reductions, volume discounts, or agreements tied to therapeutic outcomes or value.

Supplemental rebates structured as straightforward price reductions are often the simplest and quickest to negotiate and implement. By contrast, supplemental rebates structured as VBAs and OBAs, which align payments with the drug's real-world performance, can be more challenging to negotiate and administer. However, they offer the potential to mitigate financial risk if a therapy underperforms.

Subscription and annuity models address cost management through payment structuring rather than direct price reductions. Subscription models can achieve savings by stabilizing per-patient costs, but are most effective when there is a high patient volume with predictable demand.[43] Annuity models, better suited to high-cost, one-time therapies with lower patient counts, spread payments over several years to ease immediate financial pressures.[44] When paired with outcome measures, annuity models can also link payments to therapeutic results, adding a layer of cost control. Each approach offers a unique tool to help states manage budgetary demands while ensuring broader access to high-quality treatments.

Using the Uncertainty about the Long-Term Effectiveness of CGTs and other High-Cost Drugs

State Medicaid offices face unique challenges in negotiating for CGTs and other high-cost drugs approved through the accelerated approval pathway, particularly due to the uncertainty surrounding their long-term effectiveness.[45] The accelerated approval pathway allows drugs to enter the market based on preliminary evidence, which may later require confirmation through additional studies. The number of drugs receiving approval through this pathway has been increasing.[46] Research indicates that a substantial portion of Medicaid spending on drugs approved through this pathway has been allocated to therapies without confirmed clinical benefits, underscoring the importance of post-approval studies to ensure long-term effectiveness.[47]

Further, FDA approvals can sometimes extend beyond the population narrowly studied in clinical trials to a broader group of patients who may benefit from the drug based on its mechanism of action. Although broad labeling occurs in both traditional and accelerated pathways, it is more pronounced in the accelerated approval pathway, as drugs often reach the market based on surrogate endpoints or early-stage evidence, heightening the uncertainty about how the drug will perform across diverse patient groups. A councilmember highlighted that while the accelerated approval program poses a particularly acute concern in this area, depending on FDA approval standards, concerns about long-term effectiveness may persist even in CGTs and other high-cost drugs approved through the traditional approval pathway.

Illinois can leverage this uncertainty in price negotiations. One approach discussed by councilmembers was implementing a tiered pricing model, where patients who align with the clinical trial criteria pay a higher price, while a lower price is assigned to those outside that cohort, reflecting the less certain benefit. However, implementing this approach presents practical challenges, as Medicaid is required to cover drugs as specified by their FDA label, which may limit Illinois' negotiating power.

Moreover, a tiered pricing structure could discourage physician participation due to added administrative demands, and implementing such a mechanism would require a robust system to track and categorize patients based on eligibility criteria. Given these considerations, Illinois must weigh the potential benefits of negotiating a tiered pricing structure against the logistical complexities and the potential for limited manufacturer cooperation.

Deciding Whether to Pursue Value- or Outcome-Based Agreements

Councilmembers discussed the pros and cons of pursuing performance-based agreements and the circumstances under which Illinois should consider them instead of straight supplemental rebate agreements.

> **A note on terminology -** VBAs broadly link the payment for a drug to its assessed value, which may include cost-based considerations (e.g., price reductions tied to expected savings), clinical value (e.g., improved patient health outcomes or reduced disease burden), and societal value (e.g., enhanced quality of life or workforce participation). OBAs represent a specific type of VBA that ties payment directly to measurable health outcomes, such as biomarker improvements or reductions in hospitalizations. In their discussions, councilmembers generally grouped VBAs and OBAs together, recognizing the overlap in benefits and challenges associated with these agreement structures. The considerations discussed in this section apply to both types of agreements.

The Complexity of Implementing VBAs and OBAs: While these agreements offer the potential for aligning costs with therapeutic effectiveness, implementing them can be complex. Challenges include agreeing upon appropriate outcome measures, establishing reliable data collection methods, and managing administrative costs and burdens. Additionally, some health plans have encountered difficulties in enforcing these agreements, as manufacturers may include exclusions that render these agreements unenforceable in practice. For example, one health plan representative spoke of a manufacturer refusing to issue rebates when a patient has delayed or missed a dose of the medication. Given the complexity of the administration of CGTs, payers entering these agreements must ensure that they build sufficient flexibility to account for patient compliance issues.

Another major issue is that the time required to observe measurable outcomes can span multiple years, delaying the assessment of the agreement's effectiveness. Given that patients can switch Medicaid plans or switch out of Medicaid altogether, the state might find it challenging to track the patient’s progress over the measurement period. Further, one councilmember noted that because VBAs often expire after a set amount of time, the state will have to engage in ongoing negotiations to maintain access and discounts beyond the initial VBA period.

VBAs and OBAs also impose significant administrative responsibilities, which, depending on how the agreement is structured, can fall on the state, providers, health plans, and/or manufacturers. Placing significant burdens on providers might dissuade them from offering these therapies.

Additionally, the state would need to clarify the allocation of financial risk in these agreements. For instance, Illinois could pay the entire cost upfront and expect rebates when agreed-upon outcomes are not achieved or adopt a periodic payment structure linked to specific performance milestones. Establishing clear compliance obligations and assigning responsibilities among stakeholders will be critical to effective implementation.

Given all the challenges described above, implementing these agreements can be a costly process, and any potential cost savings generated through the discounts must outweigh the significant expenses associated with their implementation.

Despite these challenges, VBAs might be particularly suitable in specific scenarios. For example, given their ability to mitigate the financial risk related to uncertainty, VBAs could be a practical option for therapies where efficacy has not been fully established, such as those approved through the FDA's accelerated approval pathway. By tying payments to measurable outcomes, VBAs can help ensure that the state pays for value rather than unproven efficacy. Additionally, VBAs may be more appropriate for conditions affecting large populations, where the broader assessment of impact justifies the administrative costs of implementing these agreements. In such cases, the potential benefits of cost savings and performance-based payments might outweigh the implementation challenges

One councilmember pointed out that successful implementation of other council recommendations—such as strengthening access to standard-of-care services, investing in care coordination, and increasing staffing capacity—could also alleviate some of the logistical challenges associated with implementing VBAs and OBAs. Strengthened clinical infrastructure and better data collection systems could make implementing these agreements more manageable in the future.

To help the state decide whether to pursue VBAs, councilmembers suggested the following guiding principles:

- *Given their ability to mitigate the financial risk related to uncertainty, VBAs might be more suitable to situations where the efficacy of the drug has not been fully established, such as when a therapy is approved through the accelerated pathway.*
- *VBAs may be more appropriate for conditions affecting large populations, where the impact of the therapy can be more broadly assessed, and the administrative costs of implementing VBAs can be justified by the potential benefits.*
- *The feasibility of VBAs is contingent on the ability to establish a drug's effectiveness through robust signals in claims data or medical records. If such data are lacking or difficult to collect, supplemental rebates may be a more practical approach.*

According to a 2024 evaluation of 12 state Medicaid agencies' coverage and financing policies for gene therapies, at least three states—Arizona, Massachusetts, and Michigan—have entered into OBAs with manufacturers.[48] Another evaluation finds that Colorado has also entered into an OBA with the manufacturer of Zolgensma (a CGT to treat spinal muscular atrophy).[49] These are relatively new developments and it is too soon to assess their effectiveness.

> ***Recommendation 7:*** *HFS should explore all available discounting and rebating strategies on a case-by-case basis for each CGT or high-cost drug. Supplemental discounts and rebates are likely to be easier to implement, but other strategies, including Value-Based Agreements (VBAs), might be appropriate in certain specific circumstances.*

Subscription and Annuity Payment Models

Subscription models involve a fixed payment over a specified period, granting access to unlimited treatments for all eligible patients. This approach has proven particularly effective for high-prevalence diseases with curative therapies or treatments with predictable, ongoing demand. For example, Louisiana and Washington implemented subscription models for Hepatitis C antiviral therapies using a two-part pricing strategy. Initially, the states pay a reduced price per prescription through supplemental rebates up to a predetermined spending threshold. After reaching the threshold, the per-prescription price drops to near zero through additional supplemental rebates.[50] This model has enabled states to work toward removing per-patient cost barriers, providing broad access to these curative treatments while ensuring predictable budgeting.

According to a councilmember, subscription models have also been used to pay for CGTs like Zolgensma and Luxturna, which target very small patient populations.

While traditional ***annuity models*** do not lower drug unit costs, they spread the cost of one-time, high-cost therapies over multiple years rather than requiring full upfront payment. This approach can alleviate the immediate financial burden on state Medicaid budgets. However, payers can pair annuity models with performance measures to create a form of outcome-based annuity model that can potentially lower drug unit costs. Under this structure, annuity payments would be contingent on the therapy achieving specified clinical benchmarks over time. Such outcome-based annuity models may require mechanisms to ensure payment continuity if Medicaid patients lose coverage or switch insurers during the payment period.

4. Recommendations for Ensuring Equitable Access to CGT and High-Cost Drugs and Therapies

To ensure equitable access to CGT and other high-cost drugs and therapies, Illinois will need to identify and address critical access pressure points, including coverage gaps, provider limitations, and complex patient needs. Patients and caregivers, as key stakeholders with lived experience, offer valuable perspectives on the barriers to care and the resources needed to address them. Ongoing collaboration with organizations like the Sickle Cell Disease Association of Illinois, the Illinois Rare Disease Commission, and other rare disease advocacy groups, as well as direct collaboration with patients and caregivers, will be essential for understanding access barriers and shaping responsive policies.

The recommendations in this section reflect the council's discussions on addressing significant gaps in access and care coordination for patients with rare diseases, including SCD. While it is true that the long-term effectiveness of these investment strategies must be carefully evaluated, the council's recommendations are grounded in observed barriers and immediate needs identified by stakeholders and healthcare providers.

A. Understanding Access Pressure Points

Councilmembers representing rare disease communities, along with providers serving these populations, shared their lived experiences with the council. These discussions inform the following sections, which outline key access concerns.

Health Insurance Coverage

Coverage gaps can pose significant barriers to consistent and equitable access to CGTs. Within Medicaid, variability in prior authorization and utilization management (UM) criteria across different MCOs—such as differences in therapy approval criteria or required documentation to establish medical necessity—can result in inequities, with patients covered by some MCOs facing stricter requirements than others. These disparities hinder equitable access to CGTs for eligible patients.

Additionally, councilmembers expressed concerns that Medicaid customers with rare diseases might face coverage denials and delays in accessing necessary, often high-cost treatments during transitions between health plans and types of insurance coverage. Disruptions in continuity of care may occur when patients switch between different health plans or transition between Medicaid, ACA marketplace plans, and employer-sponsored insurance. While CGTs are typically one-time treatments, changes in insurance coverage can complicate access to essential pre-treatment services, such as diagnostics or preparation, as well as critical post-treatment care, including ongoing monitoring and management of potential side effects. Even with continuity of care requirements, differences in coverage policies between insurers can create delays or gaps in these services, further impacting patient outcomes.

Continuity of care issues can be further exacerbated by supply shocks caused by the complex production processes for these drugs. If patients' access to the drug is delayed after they have begun pre-treatment services, disruption in insurance coverage could have adverse effects on the overall treatment regimen. To address this, improving forecasting of drug availability and establishing robust continuity-of-care protocols will be crucial for ensuring timely and reliable access for these vulnerable patient populations.

Provider Capacity

The demand for CGTs requires a specialized workforce and adequate infrastructure. Unfortunately, many areas lack providers trained in administering CGTs and managing long-term follow-up care, with particular shortages in rural regions. The treatment process for CGTs involves regular appointments with hematology specialists, follow-up care, and monitoring, which may or may not occur in the same locations where CGTs are administered. These geographic, workforce, and infrastructure barriers can make CGTs inaccessible to many Medicaid customers.

Councilmembers also spoke about the lack of access to culturally competent care for patients with rare diseases. This gap in care is particularly concerning considering the unique needs of the SCD population, which is predominantly composed of African American and Hispanic individuals who face systemic barriers, including provider bias and limited expertise in SCD treatment among emergency room staff. Without culturally competent care, these patients may struggle to find providers who understand the social, economic, and cultural factors that shape their experiences with the healthcare system, leading to lower trust, reduced treatment adherence, and poorer outcomes.[51]

For all patients undergoing CGTs, the need for culturally competent care is especially pressing due to the complex and intensive nature of these treatments. CGTs require regular follow-up care, monitoring, and a high level of patient-provider communication. Patients must feel comfortable sharing concerns and reporting side effects, which is challenging if they feel misunderstood or marginalized. The current lack of culturally competent providers can further exacerbate disparities in access to these high-cost, transformative therapies.

Patient Needs

Medicaid customers with rare diseases face a range of complex challenges that hinder their ability to access comprehensive and continuous care.

Councilmembers almost uniformly highlighted the need for specialized care coordination, as many patients with rare diseases have to manage multiple appointments and see various specialists across different life stages. Patients and their families also need access to comprehensive education about their conditions and genetic counseling to better understand the range of treatment options available to them. Effective coordination among providers, case managers, and insurers is essential to ensure that patients receive timely, comprehensive care. Without a designated coordinator, patients often face fragmented care and suboptimal outcomes.

In addition to coordination challenges, Medicaid customers with rare diseases also encounter significant logistical barriers. Many patients and their caregivers, especially those who live in rural and underserved areas, travel long distances to reach specialized providers and qualified treatment centers, incurring costs for lodging, transportation, childcare, and parking.

The nature of rare disease treatments, particularly those involving CGTs, adds further complexity. These treatments are often prolonged and require intensive follow-up care, making adherence difficult for patients who cannot take time off work or manage other responsibilities.

Councilmembers also raised concerns about access gaps in essential ancillary services that are critical to meeting the needs of this patient population. Consistent access to primary care is essential for managing complex and evolving health needs, while behavioral health services are crucial to address the psychological impact of living with a rare disease. Chronic pain management services are also necessary to alleviate the ongoing pain experienced by many patients with rare conditions. For patients considering CGTs, access to fertility counseling and preservation services is important, as these therapies can impact fertility. Additionally, immunocompromised patients often require specialized dental health services as part of their overall care, which might not always be easily accessible.

Addressing access pressure points by enhancing care coordination, reducing logistical barriers, and improving access to essential ancillary services is vital for Medicaid to equitably serve rare disease patients, ensuring high-quality care that evolves to meet their needs across all stages of life.

B. Establishing Uniform Utilization Management Criteria Across MCOs

To ensure equitable and consistent access to CGTs and other high-cost therapies for all Illinois Medicaid customers, councilmembers emphasized the importance of establishing uniform utilization management (UM) criteria across the Illinois Medicaid program. While HFS requires MCOs to cover all prescription drugs listed in the state's Preferred Drug List, councilmembers noted that approval for CGTs varies significantly among MCOs, as well as Medicaid fee-for-service, due to differences in substantive UM criteria (such as criteria for therapy approval) and procedural UM criteria (such as necessary documentation to establish medical necessity.

In developing uniform UM criteria, Illinois must consider several key components. First, access to CGTs and other high-cost therapies should extend beyond the drug itself to encompass the continuum of services that support the delivery of the therapy. Uniform criteria should also cover essential post-therapy services, which may be required for many years or even throughout the patient's lifetime. These follow-up services include monitoring for side effects and effectiveness, as well as ongoing care for any pre-existing conditions that were present before the therapy.

The criteria should ensure that Medicaid customers are not subject to unnecessary limitations based on disease severity while acknowledging that some drugs may have severity specifications in their labels or clinical trial guidelines. Wherever possible, eligibility for access to CGTs should be based on the recommendation and assessment of a qualified provider and

involve shared decision-making with the patient while aligning with the scope of FDA approvals and the clinical trial populations studied.

HFS presented the council with three potential mechanisms for enforcing uniform UM criteria across MCOs:

- **Model MCO Contract**: HFS's model MCO contract serves as the foundation for all state MCO contracts. Amending the contract to include uniform UM criteria could ensure consistency across plans, although the negotiation process with MCOs may be time-consuming, and contract amendments may lack flexibility for frequent updates.
- **Standardized Single-Case Agreements**: Many providers offering CGTs currently operate under single-case agreements, which negotiate coverage and reimbursement for individual patients. HFS has proposed creating standardized single-case agreements that would set clear parameters for CGT coverage, improving consistency across providers and MCOs.
- **HFS Policies and Procedures**: HFS's policies and procedures documents offer guidance to providers and MCOs. As sub-regulatory documents, they afford HFS greater flexibility for periodic updates, making them well-suited to adapting to evolving needs and therapy options.

Councilmembers advised that HFS consider using a combination of these policy levers to maintain flexibility in managing CGT access. They also recommended engaging expert clinicians in the development of these criteria to ensure that guidelines are clinically sound and tailored to meet the unique needs of CGT patients.

Recommendation 8: *HFS should consider establishing uniform utilization management (UM) criteria across MCOs to ensure equitable access to CGTs and high-cost therapies for all Medicaid customers.*

Recommendation 9: *HFS should consider developing UM criteria that ensure access encompasses not just the CGT or high-cost drug itself but also the full continuum of pre- and post-therapy services, such as preparation, follow-up monitoring, and long-term patient care, which are essential for delivery and patient outcomes.*

Recommendation 10: *HFS should collaborate with expert clinicians to create initial UM guidelines for CGTs and high-cost drugs that distinguish between populations for whom FDA approval was granted and those included in clinical trials, ensuring clinical inclusion criteria are aligned with evidence and include provider assessments and shared decision-making with patients.*

Recommendation 11: *HFS should use a combination of policy levers—including the model MCO contract, standardized single-case agreements, and HFS policies and procedures—to implement and adapt these UM criteria effectively. Collaborate with state-based experts who have specific expertise in the state's insured population and their multidimensional needs.*

C. Improving Care Coordination for Patients with SCD and Other Rare Diseases

Effective care coordination is vital for Medicaid customers with complex conditions, especially those eligible for costly and innovative treatments such as CGTs. Illinois does not provide care coordination for the 20% of Medicaid customers enrolled in the FFS program, which typically includes individuals for whom Medicaid serves as their secondary health insurance, certain customers dually eligible for Medicare and Medicaid who have opted out of managed care, and those enrolled in partial benefit programs.

For the 80% of Medicaid customers enrolled in MCOs, HFS mandates care coordination for members identified as moderate or high-risk. High-risk members must receive intensive care management, and HFS requires MCOs to report disease management activities within their quality assurance plans and submit care management and disease management reports to HFS. However, these requirements apply broadly across all MCO enrollees and do not address the unique needs of individuals with rare diseases like SCD. Recognizing the distinct challenges and needs of this population, HFS can take several steps to improve care coordination for rare disease patients.

Stratifying Patients as High-Risk Early in Diagnosis

To address the care needs of patients with SCD and other rare diseases, Illinois should implement high-risk stratification for these individuals as early as possible—ideally at the point of diagnosis. Early stratification allows eligible patients to access specialized care coordination services promptly, regardless of disease progression.

Rare diseases like SCD can be life-threatening, even in the absence of severe symptoms initially. Furthermore, the eligibility and efficacy of high-cost treatments, such as CGTs, often depend on the patient's overall health and stability at the time of intervention. Early intervention with care coordination could improve patient outcomes by ensuring that patients are in the best possible condition when they become eligible for advanced therapies.

Importantly, access to care coordination should not be conditioned upon strict adherence to treatment plans, as such requirements could limit patients' ability to receive the full benefits of CGTs and related therapies. Care coordination services should continue to support patients even after receiving CGTs, ensuring they can maintain optimal health and manage any long-term care needs.

Enhancing Care Management Services and Training

To ensure comprehensive care for patients with rare diseases, Illinois should consider providing additional care management services tailored to the needs of these populations. This could include education on pain management, training for caregivers, and access to specialized, complex care coordinators who understand the intricacies of rare diseases and CGTs. Pain management is particularly crucial for sickle cell patients, who frequently experience chronic and debilitating pain. Caregiver training can equip family members or other caregivers with the

skills to support patients effectively, which can be especially beneficial in managing the long-term impact of rare diseases and high-cost therapies.

Performance Measurement and Quality Assurance

To ensure high-quality care coordination for patients with SCD and other rare diseases, HFS should consider developing specific performance metrics for these high-risk populations. Currently, MCOs report general disease management activities in quality assurance reports, but there are no targeted metrics that assess care coordination specifically for rare disease patients. Introducing disease-specific metrics would allow HFS to more accurately monitor MCO performance, focusing on aspects like access, timeliness, and patient outcomes for individuals with complex care needs.

In addition to these care coordination metrics, HFS should establish broader quality-of-care standards for both CGTs and traditional therapies. By implementing quality metrics that cover the full spectrum of treatment, including follow-up care and monitoring, HFS can consistently evaluate treatment effectiveness and patient outcomes. These quality measures will enable the state to assess the impact of various therapies, ensuring that patients with rare diseases receive a high-standard healthcare experience across the continuum of care.

> ***Recommendation 12:*** *HFS should consider stratifying managed care customers with SCD and other rare diseases as high-risk early in diagnosis to enable timely access to specialized care coordination.*
>
> ***Recommendation 13:*** *HFS should consider expanding tailored care management services for rare disease patients.*
>
> ***Recommendation 14:*** *HFS should consider developing and implementing disease-specific performance metrics to monitor and enhance the quality of MCO care coordination for rare disease patients, ensuring timely access, high-quality care, and comprehensive support.*

D. Financing Wrap-Around Services

Addressing the comprehensive needs of patients undergoing CGTs and other high-cost treatments requires financing wrap-around services that extend beyond traditional medical care. As discussed above, patients often face challenges such as securing temporary housing near a qualified treatment center, arranging transportation over long distances, managing childcare, and covering parking fees—expenses not typically covered by Medicaid. While Medicaid's Non-Emergency Medical Transportation benefit offers some assistance, it is generally limited in scope and radius of service and may not fully support patients traveling significant distances for specialized care.

In 2022, the Biden Administration expanded the scope of health-related social needs (HRSN) services that states could provide through Medicaid, allowing for coverage of up to six months

of rent and the delivery of meals or groceries to qualifying populations.[52] In 2024, Illinois received approval for a broad Section 1115 waiver aimed at expanding Medicaid's scope to include various wrap-around services for Medicaid customers experiencing both a social risk factor (homelessness or food insecurity) and a clinical risk factor (a complex physical health condition such as SCD). A person with SCD would also have to have a social risk factor in order to access these benefits. This waiver represents a significant advancement in supporting patients requiring specialized treatments. However, there are certain gaps in the types of support approved through 1115 HRSN waivers to date. For example, no state's waiver currently includes childcare, which council members identified as a key barrier to accessing care.

Illinois can build on these recent advancements by exploring additional avenues to comprehensively support patients with rare diseases who require CGTs and other high-cost therapies. This could involve monitoring similar 1115 waiver activities in other states and identifying best practices for expanding coverage of essential, non-traditional services for more patients with SCD and other rare diseases. By doing so, Illinois can continue to enhance access to high-quality, holistic care, ensuring that Medicaid customers with rare and complex conditions have the resources they need to undergo advanced therapies successfully.

> ***Recommendation 15:*** *HFS should consider expanding its Section 1115 waiver health-related social needs services to cover more patients with rare diseases and a broader range of wrap-around services for Medicaid customers undergoing CGTs and other high-cost therapies, including essential supports like temporary housing near a qualified treatment center, long-distance transportation, parking reimbursement, and childcare, to ensure equitable access to advanced therapies for patients with rare diseases.*

E. Expanding Access to Providers

Improving access to providers in all areas of Illinois is critical to ensuring equitable care for Medicaid patients with rare diseases, such as SCD, who may require CGTs. Access to these therapies remains challenging in regions that lack specialized providers and qualified treatment centers. Illinois can explore several policy options to expand access for these populations while balancing resources for comprehensive care across the state.

Ensuring that both in-state and out-of-state health centers in contiguous states administering CGTs are included in Medicaid networks is essential for enabling patients in all areas of the state to access specialized care without incurring out-of-network costs. Additionally, it is important to have a robust network of providers, including hematologists and other specialized professionals, who can support the unique needs of patients requiring complex therapies. HFS should also keep provider network information current so patients and their primary care providers can easily locate nearby experts and facilities equipped to deliver high-quality care for rare diseases and advanced therapies.

Illinois can also explore financial incentives for providers in rural areas who are willing to treat rare disease patients, specifically those with SCD. These incentives could help alleviate the economic barriers that may prevent rural providers from investing in the specialized infrastructure and training necessary to administer complex therapies like CGTs. Furthermore, by supporting the use of telemedicine and innovative models such as the Extension for Community Healthcare Outcomes (ECHO), the state can provide ongoing support and expertise to rural providers managing complex cases remotely. The ECHO model connects rural providers with specialists in larger medical centers, fostering knowledge sharing and enabling effective patient management without necessitating travel.[53]

Another critical element for expanding access areas across the state is to support provider infrastructure, though this must be balanced against the state's limited resources. Directing funding toward specialized centers could reduce resources available for the broader population of sickle cell patients who may not pursue or require CGTs.

> ***Recommendation 16:*** *HFS should consider ensuring that both in-state and out-of-state health centers in contiguous states administering CGTs and other high-cost drugs are included in Medicaid networks to ensure equitable patient access throughout Illinois. HFS should also consider incentivizing a robust network of specialized providers, including hematologists, across the state and ensuring that provider networks are accurate.*
>
> ***Recommendation 17:*** *HFS should explore the use of financial and nonfinancial incentives and supports to encourage specialist availability and patient access for rural populations.*

When exploring ways to expand access to CGTs and other high-cost therapies, the state must carefully weigh the inherent limitations and trade-offs. For the SCD population specifically, a councilmember highlighted that CGT is a selective treatment, often unsuitable or unnecessary for many individuals who are well-managed on traditional therapies, such as hydroxyurea. With limited state resources, prioritizing CGT access should not detract from the broader need for comprehensive care for the majority of SCD patients, many of whom lack regular preventive services. In balancing these priorities, Illinois should ensure that efforts to improve CGT access complement and reinforce the availability of high-quality, accessible care for all SCD patients.

> ***Recommendation 18:*** *HFS should consider balancing access to CGTs and other high-cost drugs with support for comprehensive care for all patients with SCD and other rare diseases, ensuring that resources are allocated to maintain high-quality preventive and routine services for those who may not qualify for or require these high-cost therapies.*

5. Additional Considerations Impacting Access and Financing

Councilmembers also discussed additional considerations that can impact CGT access and financing. Implementing the recommendations discussed above will require Illinois to make targeted investments and explore broader policy options, in some cases that extend beyond Medicaid's scope, while addressing the needs of residents with private insurance or no insurance.

A. Strengthening HFS Capacity

To effectively implement these recommendations, HFS will require an expansion of its staff, along with the addition of specialized skill sets and consulting expertise. This will include hiring additional professionals with clinical and pharmaceutical expertise as well as those who can support the complex tracking, reporting, and outcome measurement for CGTs and other high-cost therapies. Consulting support may also be necessary to guide the state in navigating federal requirements, developing uniform standards, and negotiating innovative payment agreements.

> ***Recommendation 19:*** *HFS should consider expanding its staff to include clinical and pharmaceutical experts and seek consulting support to implement the above recommendations, including to support enhanced tracking, reporting, and negotiations for CGTs and other high-cost drugs.*

B. Building System Capacity and Enhancing Provider Infrastructure

To effectively expand access to CGTs, Illinois should strive to support more access to care for rare diseases and complex therapies across the state. The state could potentially collaborate with neighboring states or enter a public-private partnership. Further, Illinois could strengthen provider infrastructure to support complex care coordination at the provider level. This could involve investments in care coordinators and health information technology that facilitate seamless data sharing and communication among providers.

> ***Recommendation 20:*** *The state should strive to promote improved access to care at the provider-level for patients with rare diseases seeking complex therapies by supporting the expansion of complex care coordination and health IT infrastructure.*

C. Expanding the Healthcare Workforce

Workforce shortages in rare disease specialties, particularly hematology, represent a significant challenge that requires a comprehensive approach beyond the scope of this report. Implementing strategies such as loan forgiveness programs for specialists in rural or underserved areas and fields related to CGTs and rare diseases requires a broader, sustained effort. Other Illinois government agencies are currently engaged in initiatives to improve the recruitment and retention of healthcare providers across the state. Additionally, given the critical importance of culturally competent care, Illinois could further support these efforts by integrating cultural competence training into license renewal requirements and advancing policies that promote diversity within the healthcare workforce.

D. Financial Risk Mitigation Strategies

As discussed above, state Medicaid programs face financial risk concerns related to the unpredictability and magnitude of costs associated with covering high-cost therapies like CGTs. Their small target populations make utilization patterns unpredictable and challenging to forecast while their significant, upfront expenditures can disrupt operational cashflows.

Some fiscal mitigation strategies aim at dispersing the fiscal risk of these drugs more equitably across participating MCOs, like the high-cost drug risk pool that HFS will implement in 2025. Others are incorporated into discounting and payment strategies, like subscription and annuity models, which aim to smooth the up-front costs to state budgets of utilization through periodic payments, however they are not the appropriate tool for negotiating the best price for all high-cost drugs.

One councilmember recommended that the state explore a reinsurance model as a separate means to reduce financial risk. Reinsurance is a financial mechanism in which an insurer (referred to as the “ceding insurer”) transfers a portion of its risk to another insurance entity (the “reinsurer”). This arrangement allows the ceding insurer to protect itself against exceptionally high costs or claims, stabilizing its finances and ensuring solvency. States may sponsor the Medicaid reinsurance program directly or approve Medicaid MCOs to purchase private reinsurance[54] to ensure that expenditures above a set cost threshold are reimbursed, helping to stabilize budgets while maintaining patient access.

Another councilmember recommended that the state consider annuity models as an option for mitigating financial risk. As mentioned above, annuity models spread the cost of one-time, high-cost therapies over multiple years rather than requiring full upfront payment. This approach can alleviate the immediate financial burden on state Medicaid budgets. The councilmember considered funding this through a state bond.

Finally, one councilmember recommended pursuing a broader multi-payer risk pool beyond just Medicaid, which would distribute costs across multiple payers and prevent any single program from bearing a disproportionate financial burden or disrupting its operational cashflow used to pay claims. Illinois can monitor financial risk mitigation strategies in other state Medicaid

programs to identify best practices for stabilizing budgets while maintaining patient access to high-cost therapies.

> ***Recommendation 21:*** *HFS should evaluate available financial risk mitigation strategies, such as reinsurance, annuity models, and multi-payer risk pools, and monitor the implementation of financial risk mitigation strategies in other state Medicaid programs.*

E. Addressing Regulatory Gaps for Non-Medicaid Populations

Illinois will also need to address coverage issues for residents with rare diseases who are enrolled in private insurance plans, such as ACA marketplace plans and employer-sponsored insurance, as well as those who are uninsured. These populations often face inconsistent access to CGTs and other high-cost therapies due to variations in coverage policies and the potentially significant out-of-pocket expenses associated with these treatments.

For residents covered through **ACA plans and state-regulated employer plans**, Illinois could explore policies that would require insurers to include CGTs within their formularies under reasonable cost-sharing terms or consider state-supported subsidies to alleviate financial burdens.

Employer-based self-insured plans, which are governed by the Employee Retirement Income Security Act (ERISA), present unique challenges when it comes to expanding coverage for CGTs. Under ERISA, these plans are regulated at the federal level and are exempt from state insurance mandates, giving employers significant flexibility in designing their benefit plans. As a result, employers may exclude CGTs from coverage or apply inconsistent coverage standards, creating barriers for patients seeking these transformative therapies.

Advocacy at the federal level could either (1) push for a federal requirement mandating that ERISA-governed plans cover CGTs that are FDA-approved and deemed medically necessary or (2) aim to reform ERISA itself to allow states to require self-insured plans to cover specific benefits, including CGTs and essential related services such as pre- and post-therapy care coordination.

> ***Recommendation 22:*** *The state should consider reviewing state regulatory options and advocate for federal regulatory changes to ensure access to CGTs and other high-cost drugs in ACA and employer-sponsored plans.*

Finally, Illinois should consider advocating for several federal policy solutions that could alleviate the financial pressures of high-cost therapies. These could include advocating for a new national drug benefit program to support CGT and other high-cost therapies outside of Medicaid (a policy option considered by MACPAC in 2021),[55] which would reduce the burden on state budgets. Illinois could also advocate for increased Federal Medical Assistance

Percentage (FMAP) specifically for high-cost therapies, as well as for the related inpatient and outpatient services needed to administer these therapies.

To improve affordability in the long term, Illinois might benefit from federal policies such as drug cost caps at the time of approval,‡ expanded rebates for accelerated approval drugs pending confirmatory trials (a policy option recommended by MACPAC in 2021),[56] and ERISA reforms to improve access and affordability for those enrolled in employer-sponsored health plans.[57]

Additionally, Illinois could pursue federal capacity-building grants to bolster the state's readiness to manage CGT access. This could include the Notice of Funding Opportunity associated with the federal CGT Access Model or other future federal opportunities.[58]

While solutions at the state level outlined in this report may help Illinois cope with the immediate budget impact, a sustained federal-state framework could offer a more sustainable pathway for ensuring the affordability of high-cost therapies across the country.

> ***Recommendation 23:*** *The state should consider advocating for federal policies that advance equitable access to advanced therapies, deter other payers from shifting patients to Medicaid, and enhance state Medicaid agencies' capacity to pay for CGTs and other high-cost drugs.*

‡ One councilmember instead recommended that Illinois first maximize discounts using the payment mechanisms discussed in this report, such as supplemental rebates and VBAs, before pursuing federal cost caps, to not reduce innovation in the rare disease area.

6. Conclusion

Cell and gene therapies and other high-cost drugs represent a new frontier in medical treatment, offering the potential to transform the lives of patients with serious or life-threatening conditions. However, realizing their promise comes with significant challenges, particularly in ensuring equitable access and sustainable financing. Accessing CGTs often requires patients to navigate geographic, logistical, and financial hurdles, such as travel to specialized centers, extensive pre- and post-treatment needs, and the complexities of intensive care protocols. For HFS, the growing pipeline of CGTs—many targeting pediatric populations that are more likely to be covered via Medicaid—poses additional pressures on the state budget, necessitating innovative strategies to balance affordability with the need to provide transformative care.

The council's recommendations outlined in this report offer a comprehensive policy framework to address these challenges. They focus on improving affordability, streamlining access, and supporting patients and providers in navigating the complexities of CGTs.

Illinois will need to build on these recommendations, working with other states, federal policymakers, and stakeholders to refine its approach as the CGT landscape evolves. Further, exploring public-private partnerships could help Illinois leverage shared resources and expertise. A collaborative and adaptive response will be essential to establishing a sustainable framework that balances innovation with fiscal responsibility, ensuring Illinois remains at the forefront of equitable and effective healthcare delivery.

Endnotes

1. Rosman Z, Shoenfeld Y, Zandman-Goddard G, "Biologic Therapy for Autoimmune Diseases: An Update," *BMC Med*, 2013 Apr 4;11:88, doi: 10.1186/1741-7015-11-88, PMID: 23557513; PMCID: PMC3616818.
2. Safary A, Akbarzadeh Khiavi M, Mousavi R, Barar J, Rafi MA, "Enzyme Replacement Therapies: What is the Best Option?" *Bioimpacts*, 2018;8(3):153-157, doi: 10.15171/bi.2018.17, Epub 2018 Jul 7, PMID: 30211074; PMCID: PMC6128977.
3. Tran G, Zafar SY, "Financial Toxicity and Implications for Cancer Care in the Era of Molecular and Immune Therapies," *Ann Transl Med*, 2018 May;6(9):166, doi: 10.21037/atm.2018.03.28, PMID: 29911114; PMCID: PMC5985271.
4. U.S. Food and Drug Administration, "Approved Cellular and Gene Therapy Products," 2024 November 8, retrieved from https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products.
5. Cordell B, "Complexities in Forecasting Eligible Cases and Associated Costs of Cell and Gene Therapy," *Milliman*, 2024 Sep 18, retrieved from https://www.milliman.com/-/media/milliman/pdfs/2024-articles/9-18-24_complexities-forecasting-eligible-cases-costs-cell-gene-therapy.ashx.
6. Buntz B, "With Prices Topping $4 million, High Stakes Define Cell and Gene Therapy Landscape," *Drug Discov Dev.*,2024 Apr 26, retrieved from https://www.drugdiscoverytrends.com/how-price-safety-and-efficacy-shape-the-cell-and-gene-therapy-landscape/.
7. Id.
8. Medicaid and CHIP Payment and Access Commission (MACPAC), *Addressing High-Cost Specialty Drugs*, Chapter 1, June 2021, available at: https://www.macpac.gov/wp-content/uploads/2021/06/Chapter-1-Addressing-High-Cost-Specialty-Drugs.pdf.
9. McEvoy K, "CGT Excitement and Reality." *Natl Assoc Medicaid Dir*, 2024 Feb 26, retrieved from https://medicaiddirectors.org/resource/cgt-excitement-and-reality.
10. U.S. Food and Drug Administration, "Accelerated Approval Program," 2023 May 5, retrieved from https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approval-program.
11. Illinois Department of Public Health, "Newborn Screening" 2024, Retrieved from https://dph.illinois.gov/topics-services/life-stages-populations/newborn-screening.html.
12. Phillips S, Chen Y, Masese R, Noisette L, Jordan K, Jacobs S, Hsu LL, Melvin CL, Treadwell M, Shah N, Tanabe P, "Perspectives of Individuals with Sickle Cell Disease on Barriers to Care," *PloS one*, 2022 Mar 23;17(3):e026534.
13. Trilliant Health, "The Cell and Gene Patient Journey is Determined by Access to Treatment Sites. 2023, retrieved from https://www.trillianthealth.com/market-research/studies/the-cell-and-gene-patient-journey-is-determined-by-access-to-treatment-sites.
14. U.S. Government Accountability Office, "Regenerative Medicine and Advanced Therapies: Information on Workforce and Education," GAO-23-106030, 2023 Mar 23, Retrieved from https://www.gao.gov/assets/gao-23-106030.pdf.
15. Cordell B, "Complexities in Forecasting Eligible Cases and Associated Costs of Cell and Gene Therapy, *Milliman*, 2024 Sep 18, retrieved from https://www.milliman.com/-/media/milliman/pdfs/2024-articles/9-18-24_complexities-forecasting-eligible-cases-costs-cell-gene-therapy.ashx#page=19.
16. Office of the Governor, State of Illinois, "Executive Order 2024-01: To Advance Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment in Illinois," 2024 Mar 18, retrieved from https://www.illinois.gov/government/executive-orders/executive-order.executive-order-number-01.2024.html.
17. Bazell C, Dieguez G, Ferro C, Mirchandani H, "A Claims-Based Analysis of Sickle Cell Disease: Prevalence, Disease Complications, and Costs, *Milliman Research Report*, 2019 Oct, retrieved from https://assets.milliman.com/ektron/A_claims-based_analysis_of_sickle_cell_disease_Prevalence_disease_complications_and_costs.pdf.
18. Spinal Muscular Atrophy Foundation, "About SMA," November 11, 2024, retrieved from https://smafoundation.org/about-sma/.
19. Illinois Department of Public Health, "2024 Illinois Sickle Cell Statewide Impact and Surveillance Program Report, 2024, retrieved from https://dph.illinois.gov/content/dam/soi/en/web/idph/publications/idph/topics-and-services/life-stages-populations/genomics/sickle-cell-impact-surveillance-report-2024.pdf.
20. Illinois Department of Public Health, "2024 Illinois Sickle Cell Statewide Impact and Surveillance Program Report. 2024," retrieved from https://dph.illinois.gov/content/dam/soi/en/web/idph/publications/idph/topics-and-services/life-stages-populations/genomics/sickle-cell-disease-impact-surveillance-report-2024.pdf.
21. Centers for Disease Control and Prevention (CDC) Foundation. "Sickle Cell Disease: Health Disparities Fact Sheet," February 16, 2018, retrieved from https://www.cdcfoundation.org/sites/default/files/files/SickleCellDisease-HealthDisparities-FactSheet021618.pdf; Lee L, Smith-Whitley K, Banks S, Puckrein G, "Reducing Health Care Disparities in Sickle Cell Disease: A Review," Public Health Rep, 2019;134(6):599-607, doi:10.1177/0033354919881438.

22. Centers for Disease Control and Prevention (CDC), “Sickle Cell Data Collection,” June 29, 2022, retrieved from https://www.cdc.gov/sickle-cell/data/index.html.
23. National Association of Specialty Pharmacy, “NASP Specialty Pharmacy Patient Care and Dispensing Model Guidelines,” 2017 Feb 16, retrieved from https://naspnet.org/wp-content/uploads/2017/02/NASP-Defintions-final-2.16.pdf.
24. 2Mass General Brigham, “What Is Gene and Cell Therapy?” 2022, retrieved from https://www.massgeneralbrigham.org/en/about/newsroom/articles/what-is-gene-cell-therapy.
25. Gustafson K, Jackson J, West M, “Cell and Gene Therapy in Oncology, Part I,” *Avalere Health*, 2021 May 26, retrieved from https://avalere.com/interviews/cell-and-gene-therapy-in-oncology-part-i-2.
26. National Human Genome Research Institute. Sickle Cell Disease Gene Therapy: Frequently Asked Questions. 2022. Retrieved from https://www.genome.gov/sites/default/files/media/files/2022-09/Sickle_cell_disease_gene_therapy_FAQ_0.pdf.
27. Oyedeji C, Strouse JJ. Improving the Quality of Care for Adolescents and Adults with Sickle Cell Disease—It’s a Long Road. *JAMA Netw Open.* 2020;3(5):e206377. doi:10.1001/jamanetworkopen.2020.6377. Bazell C, Dieguez G, Ferro C, Mirchandani H. A claims-based analysis of sickle cell disease: Prevalence, disease complications, and costs. Milliman Research Report. 2019 Oct. Retrieved from https://assets.milliman.com/ektron/A_claims-based_analysis_of_sickle_cell_disease_Prevalence_disease_complications_and_costs.pdf.
28. Dolan R, “Understanding the Medicaid Prescription Drug Rebate Program,” *KFF*, November 12, 2019. retrieved from https://www.kff.org/medicaid/issue-brief/understanding-the-medicaid-prescription-drug-rebate-program/.
29. Medicaid and CHIP Payment and Access Commission. “Themes from Expert Roundtable on Physician-administered Drugs (PAD),” 2024 Mar, retrieved from https://www.macpac.gov/wp-content/uploads/2024/03/05_March-Slides_Themes-from-Expert-Roundtable-on-Physician-administered-Drugs-PAD-2.pdf#page=8.
30. 42 U.S.C. § 1396r-8(d).
31. Centers for Medicare & Medicaid Services, “State Medicaid Coverage of Drugs Approved by the FDA under Accelerated Approval Pathway,” *Medicaid Drug Rebate Program: State Release No. 185. 2021*, retrieved from https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf.
32. Medicaid and CHIP Payment and Access Commission, “Themes from Expert Roundtable on Physician-administered Drugs (PAD),” 2024 Mar,retrieved from https://www.macpac.gov/wp-content/uploads/2024/03/05_March-Slides_Themes-from-Expert-Roundtable-on-Physician-administered-Drugs-PAD-2.pdf#page=6.
33. Medicaid and CHIP Payment and Access Commission, “Trends in Medicaid Drug Spending and Rebates,” 2022 Oct, retrieved from https://www.macpac.gov/wp-content/uploads/2022/10/07_Trends-in-Medicaid-Drug-Spending-and-Rebates-Chris.pdf#page=23.
34. Centers for Medicare & Medicaid Services (CMS), “Biden-Harris Administration Takes Next Steps to Increase Access to Sickle Cell Disease Treatments, November 15, 2024, retrieved from https://www.cms.gov/newsroom/press-releases/biden-harris-administration-takes-next-steps-increase-access-sickle-cell-disease-treatments.
35. Alliance for Regenerative Medicine, *Medicaid Access Barriers to Regenerative and Advanced Therapies: Issue Brief*, 2023, November 6, retrieved from https://alliancerm.org/wp-content/uploads/2023/11/20231106-ARM_Medicaid-Access-Barriers-Issue_WEB.pdf.
36. Health Resources and Services Administration (HRSA), “Duplicate Discount Prohibition,” accessed November 18, 2024,retrieved from https://www.hrsa.gov/opa/program-requirements/medicaid-exclusion.
37. Douglas MP, Severin T, Andry C, Angiolillo AL, Austin CP, Bertier G, Biesecker LG, et al, “Gene-Targeted Therapies: Early Experiences, Challenges, and Strategies for Success, *American Journal of Medical Genetics Part C: Seminars in Medical Genetics*, 2022;190(1):38-47, doi:10.1002/ajmg.c.32037.
38. Hampson G, Towse A, Pearson SD, Dreitlein WB, Henshall C, “Gene Therapy: Evidence, Value and Affordability in the US Health Care System,” *Journal of Comparative Effectiveness Research*, 2018;7(1):15-28, doi:10.2217/cer-2017-0068.
39. National Conference of State Legislatures, “Bulk Purchasing of Prescription Drugs,” 2023, retrieved from https://www.ncsl.org/health/bulk-purchasing-of-prescription-drugs.
40. Jaffe S, Dolan R, Bach A, Sachs R, Dusetzina S, “Policy Approaches to Address the High Prices of Cell and Gene Therapies in Medicaid,” *Health Aff Scholar*, 2023;2(5), retrieved from https://academic.oup.com/healthaffairsscholar/article/2/5/qxae051/7658582.
41. National Heart, Lung, and Blood Institute (NHLBI), “Sickle Cell Disease: Treatment,” retrieved from https://www.nhlbi.nih.gov/health/sickle-cell-disease/treatment.
42. Horrow C, Kesselheim AS, “Confronting High Costs and Clinical Uncertainty: Innovative Payment Models for Gene Therapies,”. *Health Affairs*, 2023;42(11):1681-1688, doi:10.1377/hlthaff.2023.00527.

43. Liu, H. H., Mulcahy, A. W., & Rose, A. J.,*Subscription Models for Prescription Drugs: The Motivation, Potential, and Limitations of a New Payment Model*, RAND Corporation, 2020, retrieved from https://www.rand.org/pubs/perspectives/PEA289-1.html.

44. Colasante, W, "What Risks Do Annuity Pricing Models Present to Cell & Gene Therapy Developers?" *Cell and Gene*, 2019, May 19, retrieved from https://www.cellandgene.com/doc/what-risks-do-annuity-pricing-models-present-to-cell-gene-therapy-developers-0001.

45. Gaffney A, Lexchin J, Campbell J, "Reforming Reimbursement for the US Food and Drug Administration's Accelerated Approval Program to Support State Medicaid Programs," *JAMA Health Forum*, 2022;3(11) . doi:10.1001/jamahealthforum.2022.4610.

46. MACPAC, "June 2021 Report to Congress on Medicaid and CHIP. Medicaid and CHIP Payment and Access Commission." 2021 Jun, retrieved from https://www.macpac.gov/wp-content/uploads/2021/06/June-2021-Report-to-Congress-on-Medicaid-and-CHIP.pdf#page=33.

47. Sachs RE, Jazowski SA, Gavulic KA, Donohue JM, Dusetzina SB, "Medicaid and Accelerated Approval: Spending on Drugs with and without Proven Clinical Benefits," *J Health Polit Policy Law*, 2022 Dec 1;47(6):673-690, doi: 10.1215/03616878-10041107, PMID: 35867545; PMCID: PMC9789165.

48. Avalere Health, "Examining Variation in Gene Therapy Access Across States," 2024, August 27, retrieved from https://avalere.com/insights/examining-variation-in-gene-therapy-access-across-states.

49. Council for Affordable Health Coveragec "Outcomes-Based Arrangements: Sustainable Financing for Transformative Therapies and a Review of State Activity," April 2024, retrieved from https://cahc.net/wp-content/uploads/2024/05/CTT-VBA-White-Paper-vApril2024FIN.pdf.

50. Auty SG, Shafer PR, Griffith KN, "Medicaid Subscription-Based Payment Models and Implications for Access to Hepatitis C Medications," *JAMA Health Forum*, 2021;2(8), doi:10.1001/jamahealthforum.2021.2291.

51. Martinez, R. M., Osei-Anto, H. A., & McCormick, M. (Eds.),"Addressing Sickle Cell Disease: A Strategic Plan and Blueprint for Action. 2020, retrieved from https://www.ncbi.nlm.nih.gov/books/NBK566467/.

52. Gardner, A., Mondestin, T., Kaneb, N., & St Goar, J, (). "State Use of Section 1115 Demonstrations to Support the Health-Related Social Needs of Pregnant and Postpartum Women, Infants, and Young Children. Center for Children and Families." 2024, June 13.

53. Agency for Healthcare Research and Quality (AHRQ), "Project ECHO: Improving Patient Care Through Innovative Medical Education,". September 2021, retrieved from https://www.ahrq.gov/patient-safety/settings/multiple/project-echo/index.html.

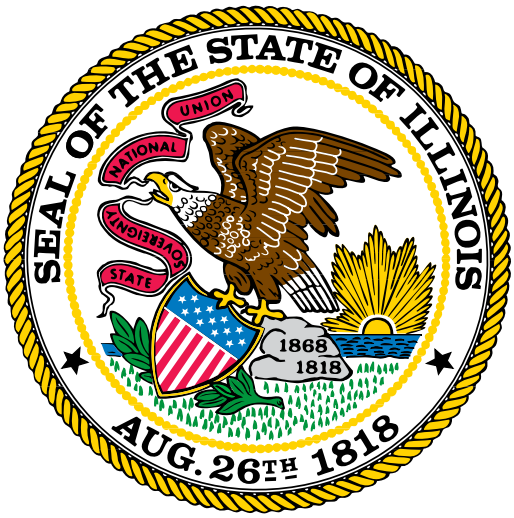
54. MACPAC. *Managed Care Capitation Issue Brief.* Published March 2022. Retrieved from https://www.macpac.gov/wp-content/uploads/2022/03/Managed-care-capitation-issue-brief.pdf.

55. Medicaid and CHIP Payment and Access Commission. "June 2021 Report to Congress on Medicaid and CHIP. Medicaid and CHIP Payment and Access Commission," 2021 June, retrieved from https://www.macpac.gov/wp-content/uploads/2021/06/June-2021-Report-to-Congress-on-Medicaid-and-CHIP.pdf#page=37.

56. Medicaid and CHIP Payment and Access Commission, "June 2021 Report to Congress on Medicaid and CHIP. Medicaid and CHIP Payment and Access Commission," 2021 June, retrieved from https://www.macpac.gov/wp-content/uploads/2021/06/June-2021-Report-to-Congress-on-Medicaid-and-CHIP.pdf#page=33.

57. Institute for Clinical and Economic Review & NEWDIGS at Tufts Medical Center. *Managing the Challenges of Paying for Gene Therapy: Strategies for Market Action and Policy Reform*, 2024, retrieved from https://icer.org/wp-content/uploads/2024/04/Managing-the-Challenges-of-Paying-for-Gene-Therapy-_-ICER-NEWDIGS-White-Paper-2024_final.pdf#page=29.

58. Centers for Medicare & Medicaid Services (CMS), "Cell and Gene Therapy Access Model Notice of Funding Opportunity Fact Sheet," 2024, retrieved from https://www.cms.gov/priorities/innovation/files/cgt-model-nofo-fs.pdf.



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