Alliance *for* Regenerative Medicine

Issue Brief: Medicaid Barriers to Accessing Cell & Gene Therapies

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ANALYSIS

Medicaid Cell and Gene Therapy Landscape

What barriers are precluding Medicaid beneficiaries' access to innovative, life-changing cell and gene therapies?

Federal law requires that U.S. Food and Drug Administration (FDA) approved drugs subject to a rebate agreement be covered by each state Medicaid program upon availability. Medicaid is an important payer for cell and gene therapies, including many approved therapies that treat rare genetic diseases affecting children and potential approvals for sickle cell disease and other severe conditions on the horizon. A recent analysis found several barriers to access at the state level for cell and gene therapies (CGT) for patients and providers given the lack of state resources and policies in this burgeoning area.

ADVI Health reviewed Medicaid access barriers in CGT and conducted interviews with Alliance for Regenerative Medicine (ARM) member organizations that have currently marketed products to assess the state Medicaid CGT landscape.

Key findings include

Cross-border credentialing, education and communication between states remain the highest concerns for CGT manufacturers as they seek to launch successfully across the US.

Payment is more of a concern than coverage for CGT in Medicaid due to lower reimbursement rates compared to Medicare and commercial settings. When access to CGT products is delayed it is often a result of the administrative burden of negotiating Medicaid payment.

States often do not have a **formal coverage policy** for CGT products upon availability in the state, delaying patient access while the manufacturer and the state adjudicate coverage.

The use of **value-based arrangements** and carve-outs leading to separate payment is still in its nascent stages for states, many of which would rather claim traditional supplemental rebates.

For outpatient therapies the biggest concerns have been whether coverage will be to **FDA label** or to trial inclusion/exclusion criteria at launch and whether the state has existing rules or regulations around payment to invoice, acquisition cost, or average sales **price**.

THE LANDSCAPE

Medicaid is a health insurance program covering nearly 87 million low-income people across the United States, including four in ten children, eight in ten children in poverty, one in six adults, and almost half of adults in poverty. Relative to White children and adults, Medicaid covers a higher proportion of Black, Hispanic, and American Indian American Native children and adults[1]. The Medicaid program is administered by states according to federal requirements and is jointly funded by both state and federal governments.

Under the Medicaid Drug Rebate Program (MDRP) (Section 1927 of the SSA), a state is required to cover all of a participating manufacturer's products that are subject to an MDRP national rebate agreement upon approval by the FDA and available for sale in the state. Each state follows a process prescribed by Section 1927 under which physicians, pharmacists, and other state-appointed individuals deliberate on, publish, and implement formal coverage criteria for such drugs. To facilitate this process, federal Medicaid rules require each state to have a comprehensive Drug Utilization Review (DUR) program that assesses the utilization, quality, medical appropriateness, and cost of prescribed medication.

States generally run DUR programs through DUR Boards (DURB) and/or Pharmacy and Therapeutics (P&T) Committees. DUR/P&T proceedings may lead states to place utilization restrictions on reviewed drugs, including prior authorization and step therapy. Although federal law allows states to employ these tools to manage the use of a particular drug, CMS has said in guidance that the effect of these limitations "should not result in the denial of access to effective, clinically appropriate, and medically necessary treatments.[2]" Despite the federal coverage requirement and CMS guidance, some states restrict coverage of a new drug before it undergoes DUR/P&T committee review, which in many states can take 6 months to a year, depending on the controlling state statute or regulation. As a result, such states are, in effect, delaying access to that drug for an unreasonable period beyond its availability in the state.

[1] https://www.kff.org/medicaid/issue-brief/10-things-to-know-about-medicaid/

[2] CMS MDRP Notice titled Assuring Medicaid Beneficiaries Access to Hepatitis C (HCV) Drugs (Release No. 172, Nov. 5, 2015).

To date, the FDA has approved nine gene therapies – eight for rare genetic diseases -- and six CAR-T cell therapies for various blood cancer indications. Recent approvals have been announced in Duchenne muscular dystrophy, Hemophilia A and B, and a cell therapy for type 1 diabetes, among others. Similarly, approvals for rare genetic pediatric indications have been announced in spinal muscular atrophy and cerebral adrenoleukodystrophy, as well as for pediatric acute lymphoblastic leukemia. These innovative therapies address high unmet medical needs, can be lifesaving, have the potential to reduce the need for burdensome and costly chronic care, and may help address CMS' health equity goals of closing gaps in care for underserved populations and eliminating racial health disparities.

The CGT pipeline for both rare and prevalent diseases is accelerating; notably, two gene therapies for sickle cell disease could be approved by the FDA in late 2023. As an example of the impact of the Medicaid program on these transformative therapies, Medicaid covered 66 percent of sickle cell disease hospitalizations in 2004 and 58 percent of emergency department visits for the disease between 1999 and 2007[3]. Medicaid pays for most of the acute care for sickle cell disease patients — who are overwhelmingly people of color[4].

[3] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8550393/.

[4] https://www.cdc.gov/ncbddd/sicklecell/data.html#:~:text=SCD%20affects%20approximately%20100%2C000%2 Americans,sickle%20cell%20trait%20(SCT).

BARRIERS TO ACCESS

ADVI interviewed CGT manufacturers with currently marketed products. Several issues related to coverage and reimbursement were identified as immediate barriers to access:

01 Nearly all manufacturers interviewed listed cross-border coverage and credentialing issues as a significant challenge. CGT manufacturers typically launch with a small number of qualified treatment centers and must balance the number of treatment sites with the number of patients they expect in each geography. In many cases, beneficiaries must travel to a neighboring state to receive a therapy when they lack a treatment center in their area. Additionally, providers seeking to treat out-of-state Medicaid beneficiaries must bill the patient's home state Medicaid program. To receive payment, the provider must enroll with and be credentialed by that patient's Medicaid program. Since these credentialing procedures are often time-consuming, complicated and costly, in many cases, they can delay or even prohibit the ability of a provider to treat out of state patients—many of whom may require immediate care due to severe and complex medical conditions.

02 Those interviewed also identified the lack of formal written coverage policies and the inability to seek coverage policies before FDA approval as a significant challenge. Many states do not write coverage criteria prior to identifying a potential patient who is typically initially denied coverage and then must appeal. An interviewee mentioned that they have patients who have waited over 2 years to receive a therapy while state administrators and Medicaid health plans continue to delay necessary treatment. For many patients with rapidly progressing or severe disease, this delay could lead to disease progression past the point of benefit from a CGT, or even death.

"We found that states often don't begin the coverage policy development process until a patient candidate is identified, and by then, it is often too late for certain patients with unmet medical needs."

– Marc Samuels, CEO, ADVI Health

03 State Medicaid leaders often have no experience with CGTs and would benefit from additional education on the science, the patient journey to receive these products, and the value that these products can provide. There is also a need for additional CMS or state guidance around issues such as value-based agreements, carve outs from inpatient bundled payment, the multiple best price rule, supplemental rebates, appropriate guidance under the Early Periodic Screening, Diagnosis and Treatment Act (EPSDT)[5] and cross-border coverage and credentialing processes.

04 Finally, states have been laser focused on reducing per-beneficiary expenditures, including for drugs and biologics. In the case of outpatient therapies, interviewees who have marketed gene therapies commented that most states are paying at invoice for the CGT product for the small number of beneficiaries and not paying what they do for biologics on an ASP+ basis.

A handful of states have instituted draconian cost measures. Under the guise of using the same tools as those of commercial payers they have instituted negotiation or cost boards that in effect create a closed formulary and chill access despite MDRP guarantees.

Massachusetts is one such state. Drug spending in the Massachusetts Medicaid program reached $2.2 billion over the last 5 years; about 40 percent of the state budget. The state has also noted that enrollment in MassHealth, the state Medicaid and CHIP program, has significantly increased. To manage rising drug spending, in 2019 Massachusetts instituted a drug price review process in the MassHealth program, described below.

11 states have proposed or enacted prescription drug affordability board (PDAB) legislation – independent boards at the state level that are tasked with assessing "high-cost" drugs. When identifying "high-cost" drugs, PDABs may consider drugs (including biologics) that meet certain price thresholds, or price increase thresholds; PDAB legislation may also include additional reporting requirements from entities such as plans, PBMs, manufacturers, and wholesalers[6] . In four states, PDABs are empowered to set upper payment limits (UPLs)[7] . UPLs are a maximum reimbursement rate above which purchasers/payers in a state may not pay for prescription drugs. A UPL does not set the price that a manufacturer can charge but creates a ceiling on what a payer can pay. The number of drugs subject to UPLs will vary by state (i.e., 12-25). In 2023, states with PDABs that set UPLs are just beginning the process of identifying drugs. The future impact of PDABs will be important to follow.

[5] https://www.macpac.gov/subtopic/epsdt-in-medicaid/

[6] https://nashp.org/qa-on-nashps-model-act-to-reduce-the-cost-of-prescription drugs-by-establishing-a-prescription-drug affordability-board/

[7] https://nashp.org/comparison-of-state-prescription-drug-affordability-review-initiatives/

Example of State Medicaid Programs Limiting Spending on Prescription Drugs

New York Medicaid	Massachusetts Medicaid
Has a global drug spending cap[8].	**Has a drug pricing review process.** • Applies to drugs that cost more than $25,000 per year per patient after rebates or total state spending exceeds $10 million per year after rebates.
Specific products that trigger the cap are referred to the New York DUR board which recommends supplemental rebate amounts. • If agreement with manufacturer is not reached, the state may implement PA requirements, require manufacturers to report cost transparency data, or remove products from PDLs	**Manufacturers may be subject to public price transparency or referral to the Health Policy Commission (HPC) for review to determine if a drug's price is "unreasonable or excessive in relation to HPC's proposed value for the drug[9]."**

[8] https://www.health.ny.gov/health_care/medicaid/regulations/global_cap/docs/general_faqs.pdf

[9] https://www.mass.gov/service-details/drug-pricing-review

Summary of "To Labeled Indication" Coverage Determinations for 16 States[10]

	Kymriah	Luxturna	Zolgensma
Arizona	N/A	N/A	N/A
Arkansas	N/A	N/A	N/A
California	To label	More restrictive[a,d]	More restrictive[b,d]
Colorado	To label	N/A	More restrictive[b,d]
Florida	To label	More restrictive[a,d]	More restrictive[a,b]
Georgia	N/A	N/A	N/A
Illinois	N/A	N/A	N/A
Indiana	N/A	More restrictive[a]	More restrictive[a,b,d]
Massachusetts	To label	To label	More restrictive[b]
Michigan	N/A	N/A	N/A
Mississippi	More restrictive[c,b]	More permissive[a,b]	More restrictive[a]
New York	To label	To label	More restrictive[a,b,d]
North Carolina	To label	More restrictive[a]	More restrictive[b,d]
Oklahoma	To label	More restrictive[a,b,c]	More restrictive[b]
Oregon	More restrictive[e]	More restrictive[b]	More restrictive[b,d]
Texas	More permissive	More restrictive[a,b,d]	More restrictive[b]
United Healthcare (MCO Policy)	More restrictive[c,d]	To label	More restrictive[b]
Anthem (MCO Policy)	More restrictive[b]	To label	More restrictive[b]
Centene (MCO Policy)	More restrictive[b]	More restrictive[a]	More restrictive[b]
Covered to, or beyond labeled indication	9	4	0
Total policies available	**13/19**	**13/19**	**14/19**

N/A no policy publicly available.

a Age limitations are narrower than the label (unless the payor does not cover such ages).

b Severity of condition thresholds (visual acuity, advanced disease, physical performance scores, expectation of outcomes).

c Limitations on use based on pregnancy or being of childbearing age, even if not recommended in pregnancy in section 8.1 of the FDA package insert.

d Limiting use in populations not included in the clinical trial, even if the lack of data from such populations is noted in the indications and usage section of the label.

e Allowing compendia diagnoses.

[10] Allen, et al. (2023). Medicaid coverage practices for approved gene and cell therapies: Existing barriers and proposed policy solutions. Molecular Therapy. 29, 513-521. https://doi.org/10.1016/j.omtm.2023.05.015

PAYMENT IS THE REAL ISSUE?

Separate from federal or state policy, the mechanics of how some CGTs that are used in the inpatient setting are paid for can impact access. Oftentimes, delays occur as treatment centers grapple with contracting and claims processing issues. In addition, Medicaid programs are often unsure how to process reimbursement at launch, thereby delaying treatment for the patient for administrative reasons.

"We've had some patients waiting to be treated for over 2 years. States and health plans continue to use the delay and deny tactic"

Inpatient therapies are paid for in a variety of ways in the Medicaid program and not under a unified approach like in Medicare. 29 states use All Patient Refined Diagnosis Related Group (APR-DRG) classifications as the basis of payment for hospital inpatient episodes of care[11]. Unlike the Medicare Severity Diagnosis Related Group (MS-DRG) classifications, however, AP-DRGs are not uniform across the country; each state can have a different base version of the APR-DRG system with different relative weights and classifications. Additionally, each state has a different hospital outlier payment policy which covers significantly less than the federal Medicare program and Medicaid programs do not provide New Technology Add on Payments (NTAP).

State Medicaid agencies, like commercial payers, often negotiate single-case agreements to allow for reimbursement when case rates do not exist, either in- network or out-of-network. Single case agreements can be useful when patients are being treated at an out-of-state treatment center. However, every intermediary in the system causes administrative delays. Manufacturers often need to deal with the patient's state Medicaid Agency, the treating state Medicaid Agency, the patient's Medicaid Managed Care plan, treatment centers, and referring providers. Access can be delayed by negotiations among health plans, states, and treatment centers about payment issues such as 340B discounts, supplemental rebate negotiations, and services included as part of a single case agreement.

Value based agreements (VBA) may be a tool to improve patient access to cell and gene therapies. Appropriately structured VBAs could address payer uncertainty regarding real-world efficacy that supports the durability and value of these cutting-edge therapies. Because of the focused nature of VBAs, their implementation involves more upfront costs and operational challenges for health plans to implement than traditional contracts. Additionally, VBAs present implementation challenges for manufacturers because of their impact on certain price reporting metrics.

[11] https://www.3m.com/3M/en_US/health-information-systems-us/drive-value-based-care/patient-classification-methodologies apr-drgs/#:~:text=3M%20APR%20DRGs%20have%20become,approximately%20a%20dozen%20commercial%20payers.

In an attempt to alleviate some of manufacturers' concerns about best price impacts of VBAs, CMS issued a "Multiple Best Price Rule" that took effect in mid-2022, allowing manufacturers to report different best prices for products that are part of a VBA. However, there are still implementation challenges. First, the National Association of Medicaid Directors (NAMD) noted that the "administrative burdens and demands on state staff resources to implement multiple best prices is significant" and many state Medicaid programs still lack the resources to adopt these innovative arrangements. Second, certain additional regulatory barriers remain, including the risk of liability under the Anti-kickback statute and the lack of VBA portability as patients change health plans. Finally, the inclusion of commercial sales under a VBA in the Medicaid Rebate formula dramatically stymies VBAs from the manufacturer perspective.

Interviewees reported that state Medicaid programs have inconsistent and unclear timelines and processes for manufacturers to engage with Medicaid policy staff. Most states do not have set coverage meetings with qualified clinical staff or matched peer review, which is why P&T and DUR boards are merging in many states. A 2023 study anecdotally cites one state that only allows a single 30-minute meeting per year for manufacturers to discuss all access issues and products in their pipeline[12]. Some states will only allow manufacturers to present their data once they have received FDA approval for their product, delaying time to coverage and impacting timely patient access.

"States have no guidelines for how quickly their [Medicaid] systems need to be updated to reflect pricing for new products and when coverage policies need to be updated."

These payment and process delays in Medicaid stand in contrast to the more expedient review determinations required under Medicare where Part D and Medicare Advantage plans also utilize P&T committees to develop formularies and make coverage decisions. However, Part D plans are required to make a reasonable effort to review new drugs within 90 days and make coverage decisions within 180 days of a drug's release into the market[13]. If a drug is within one of six designated protected classes, Part D plans are required to conduct an expedited review and render a coverage decision 90 days after it comes on the market[14].

[12]Allen, et al. (2023). Medicaid coverage practices for approved gene and cell therapies: Existing barriers and proposed policy solutions. Molecular Therapy. 29, 513-521. https://doi.org/10.1016/j.omtm.2023.05.015

[13]Medicare Prescription Drug Manual 30.1.5

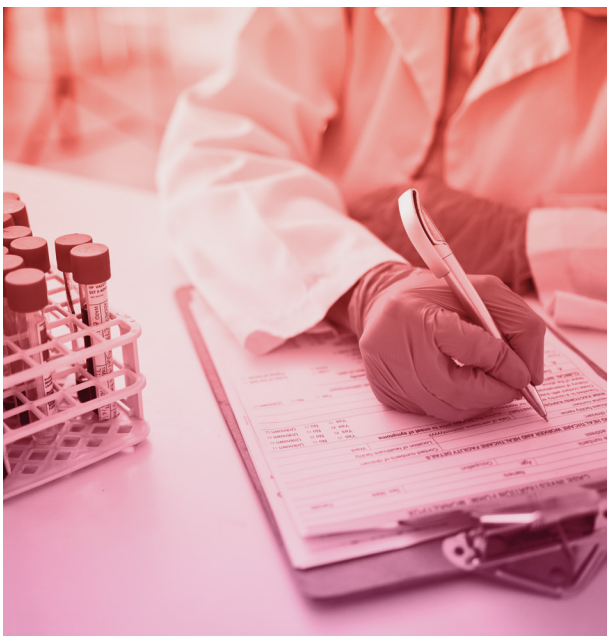
[14]Medicare Prescription Drug Manual 30.2.5

MEDICAID HEALTH PLANS

Medicaid health plans (MHP) often do not have specialists in oncology, hemophilia, and rare diseases to review CGTs currently approved by the FDA. They also do not often use matched peer review when making specific coverage decisions. MHPs are generally more product price sensitive as payment for new high-cost drugs come out of existing per member per month negotiated amounts with the state Medicaid Agency. In the past, products like Sovaldi and Spinraza, although not CGTs, have caused considerable conversation among MHPs when launched because they were high-cost products that were not accounted for in the budgeted capitated payments.

MHPs have limited recourse if a high-cost drug launches in the middle of an existing contract and thus are likely to implement strict utilization management criteria. If MHPs are aware of new products entering the market before a contract renewal year, they can seek additional funds from the state to account for new drugs or "surprises." Some states (e.g., Arizona) have created reinsurance programs to help MHPs absorb high costs. Interviewees reported that MHPs are more likely to have stricter prior authorization requirements and ask for supplemental rebates, value-based agreements, or other price concessions. At product costs above $1 million MHPs are likely to put up access barriers without regard to manufacturer concessions, and will look to specialist providers, key opinion leaders, academic centers, and centers of excellence to help define coverage criteria. Unlike in Medicare, Medicaid health plans are not required to cover CGTs if the state covers the product in their fee for service (FFS) population. Seven states have carved out the entire prescription drug benefit from their MHPs and maintain single state formularies, and thirteen states have specifically carved out CGTs from their MHP.

A July 2023 OIG Report raised concern regarding high rates of prior authorization denials by some plans and limited state oversight in Medicaid Managed Care. OIG found the MCOs included in the review denied one out of every eight requests for the prior authorization of services in 2019. 12 of the 115 MCOs included in the review had prior authorization denial rates greater than 25 percent—twice the overall rate. Unlike Medicare Managed Care's robust oversight of prior authorization denials, OIG found that most State Medicaid agencies reported they did not routinely review the appropriateness of a sample of MCO denials of prior authorization requests, and many agencies did not collect and monitor data on these decisions[15].



[12]https://oig.hhs.gov/oei/reports/OEI-09-19-00350.pdf

DRIVING CHANGE

The Alliance for Regenerative Medicine (ARM) supports policy solutions that expedite access for Medicaid patients seeking treatment, remove administrative barriers to care, and advance fair and adequate reimbursement.

Achieve shorter, streamlined drug coverage review processes.
Each state DUR Board or P&T Committee follows its own new drug review law or policy, which in many states can allow or even mandate extended review timelines. Such delays can result in unreasonable access restrictions on new drugs. Congress should work with CMS to require states to meet their legal obligations to provide access to FDA-approved drugs upon availability and cover to label.

Rapid Review Incentives.
Congress should consider offering incentives for states to conduct expedient coverage reviews of newly available cell and gene therapies e.g., by the next scheduled DUR Board meeting after availability and in no case later than 90 days.

Create universal credentialing standards for CGT providers treating out-of-state patients.
The intricacies of CGTs may limit the number of centers of excellence (COEs) offering these complex therapies. As a result, some Medicaid beneficiaries may be required to travel out of their home states for treatment. Each state generally develops and administers its own credentialing rules, and the credentialing process can be complex, time-consuming, and expensive for providers to complete. We urge Congress to work with CMS to develop a minimum national credentialing standard for providers seeking to administer CGTs to out-of-state beneficiaries of all ages.

Adoption of VBAs.
Congress should require CMS to inform states of their ability to implement separate payment methodologies for inpatient products and to adopt VBAs. To encourage the adoption of VBAs between manufacturers and payers, Congress should establish a statutory Anti-kickback Statute safe harbor for such VBAs.

WHAT DOES THE FUTURE HOLD?

Medicaid agencies continue to be a pain point for manufacturers launching CGTs and, even more importantly, an access barrier for patients in Medicaid programs. Medicaid health plans, unlike Medicare Advantage plans, do not have to cover CGTs even when state fee for service programs do.

01

Lack of clarity in coverage policy development, consistency in processes and extensive administrative burden in contracting with individual state Medicaid agencies, and cross-border issues creates unnecessary delays to potentially life-saving therapies.

02

Long treatment delays can permanently damper the impact of the therapies. In other cases, delays in access may cause a patient to age beyond the limits imposed by insurers for coverage, permanent damage due to the disease that cannot be reversed, or disease progression to the point of permanently eliminating their opportunity to benefit.

03

Patients – many of whom are children - suffering from such conditions do not have the luxury of time to wait for Medicaid to make a product available after extended delays.

Active communication with state Medicaid leaders is necessary if change is to occur. Future work to reform state Medicaid payment policies including continued reinforcement to states that they can pay separately and promulgate value-based arrangements is key to alleviating state costs without risking harm to state beneficiaries. Congress and states should act responsibly and make these changes now while many cell and gene therapies are still in pivotal trials. To wait until many more therapies launch is irresponsible given so many beneficiaries are those with unmet needs.

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