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Access to Transformative Therapies for Medicaid Enrollees

Current Barriers and Proposed Policy Solutions

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Executive Summary

Thanks to breakthroughs in precision medicine, leading scientists have developed a growing number of cell and gene therapies (CGTs) for serious and life-threatening diseases. By addressing the root causes of disease, these transformative therapies can slow, stop, or even reverse disease progression, achieving life-changing results for patients who previously had no hope of effective treatment.

Troublingly, these breakthroughs are not accessible to all. Nationwide, the low-income people covered by the Medicaid program have reduced access to CGTs compared to those covered by Medicare or commercial insurance.[1] On paper, federal law provides that all Medicaid enrollees should have timely access to Food and Drug Administration (FDA)-approved drugs and most specialty care. But in practice, patients and their caregivers can encounter barriers at each step in their journey from diagnosis to treatment.

Many of these barriers reflect certain key differences between transformative therapies and other, more familiar drugs and services. Moreover, Medicaid policies can vary significantly from state to state, and also across managed care organizations (MCOs) within a single state. In addition, although not the focus of this paper, states have identified issues concerning the high upfront cost of certain CGTs and challenges associated with managing the financial risk created by this growing class of treatments.

Transformative therapies are more like a heart transplant than a statin. These highly sophisticated therapies require specialized training to manufacture and administer. As a result, CGTs may only be available at a few "Center of Excellence" providers nationwide.

This paper identifies key barriers that can impede Medicaid patients' access to CGTs, as well as policy solutions to address those barriers at the state and federal levels. These barriers and solutions—which are summarized on the next page and presented in greater detail in the Appendices—were informed by Manatt Health's survey of relevant Medicaid policies in all 50 states and Washington, D.C., as well as a review of the literature and conversations with diverse stakeholders in the Medicaid ecosystem.

The federal government is currently implementing a Medicaid demonstration project aimed at promoting access to CGTs for Medicaid enrollees with sickle cell disease.[2] A broader conversation is needed, however. The FDA has approved more than 25 CGTs to date, and that number is growing rapidly. By 2030, it's estimated that CGTs could offer life-changing results to more than 100,000 patients every year. By implementing the reforms outlined in this paper, federal and state policymakers can ensure that Medicaid enrollees are fairly represented in that number.

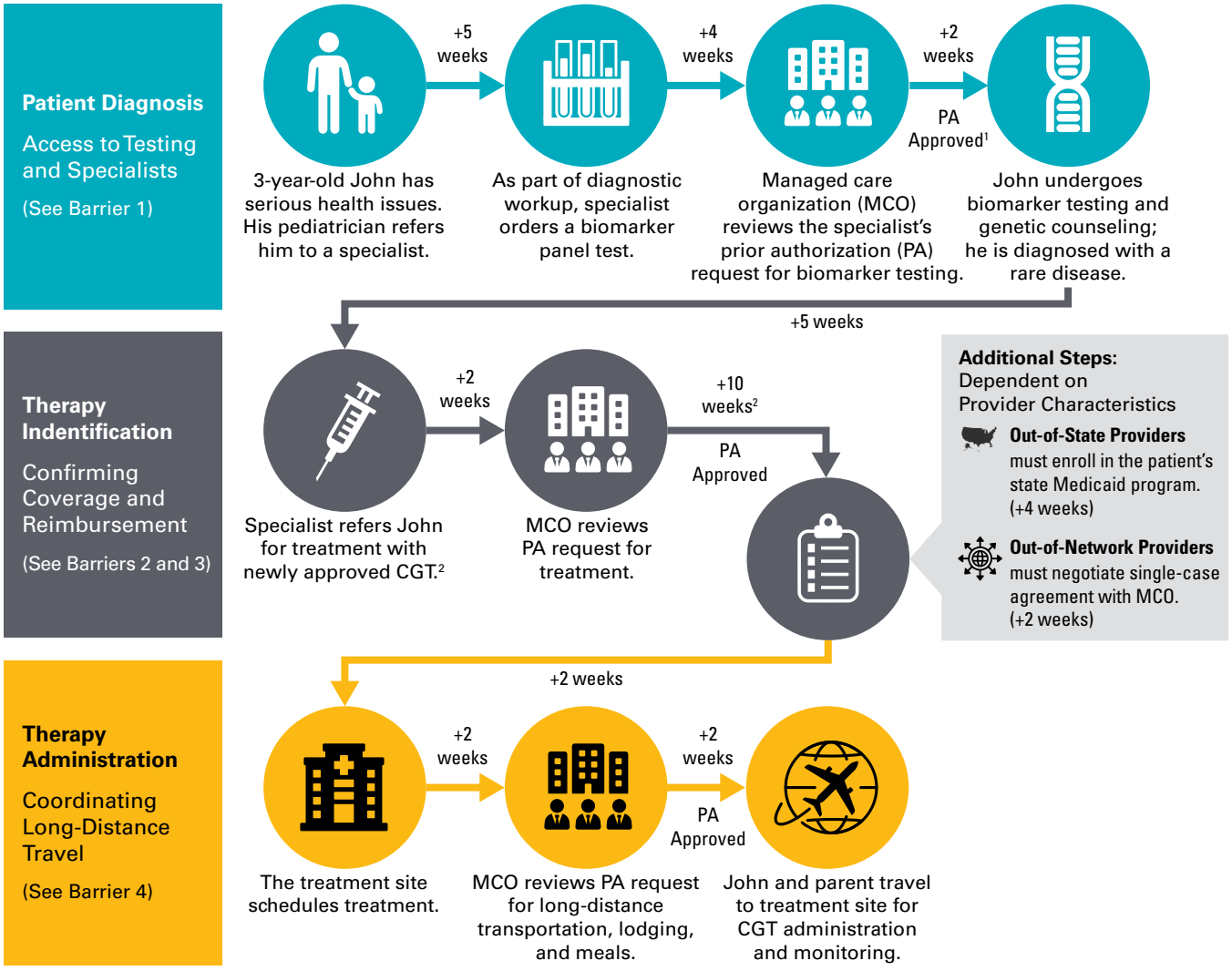
Access Barrier for Transformative Therapies	Policy Goals to Promote Access to Care
BARRIER 1. Inconsistent access to the testing and specialists needed to identify **and refer eligible patients.** Notably, genetic testing is nearly always required to determine whether a patient is eligible for a CGT.	**Goal A. Publish clear policies that ensure coverage for medically necessary biomarker tests**, as well as genetic counseling **Goal B. Ensure timely access to specialists for diagnosis and referral**
BARRIER 2. Unclear, untimely, or unduly restrictive coverage policies for new therapies. Providers are typically unable to take on the significant cost of acquiring, pretreatment processing, and administering a CGT until they receive confirmation that the therapy will be covered.	**Goal. Publish clear policies ensuring timely and comprehensive coverage of newly approved therapies**, including compliance with federal law
BARRIER 3. Administrative delays between referral and treatment as the provider navigates issues such as:	
• **Prior authorization** processes that can delay access and interrupt continuity of care	**Goal A.** Streamline p**rior authorization processes**, protect continuity of care, and ensure effective oversight
• **Reimbursement rates that fail to cover providers' costs**, especially when hospitals receive a single bundled payment for the therapy and all associated services	**Goal B. Offer a discrete payment for the transformative therapy** that is calculated to cover the hospital's acquisition costs
• **"Single-case agreements"** negotiated between MCOs and out-of-network providers (as is often the case for providers administering CGTs)	**Goal C. Carve transformative therapies out of managed care**, instead covering them through the fee-for-service (FFS) delivery system
• **Burdensome and duplicative enrollment processes** for providers located out of state (as is often the case for providers administering CGTs)	**Goal D. Streamline Medicaid enrollment** for out-of-state providers who present a low risk of fraud and abuse
BARRIER 4. Inadequate support for long-distance travel, without which a low-income patient may be unable to afford the trip to a Center of Excellence for care.	**Goal A. Ensure comprehensive coverage for transportation, lodging, and meals**, in accordance with federal law **Goal B. Minimize administrative and financial burdens on beneficiaries**

Exhibit 1. Patient Journey Map: Potential Delays in Medicaid Enrollees' Access to Transformative Therapies

Meet John, age 3, who has serious health issues and is enrolled in Medicaid. This diagram outlines John's hypothetical journey from diagnosis to treatment with a transformative therapy.

All told, John experiences more than ten months of administrative delays.

Different patients may experience longer or shorter delays depending on their state and the specific therapy they need, as well as clinical factors such as required pretreatment services (e.g., psychosocial screenings, fertility preservation services).



1. If a PA request is denied, John may file an appeal with the MCO, and then with the state. Even if John ultimately wins the appeal, this may result in a several weeks or months of additional delay.

2. In some states, Medicaid coverage for a newly approved product may not take effect until several months after FDA approval—sometimes even more than a year.

Background: Medicaid Enrollees Deserve Access to Transformative Therapies

Disparities in Access to Life-Changing Therapies

Being diagnosed with a serious disease can be overwhelming and life changing. However, advances in precision medicine offer new hope to patients and can make a significant difference in treatment outcomes. Over the last decade, leading scientists have developed a number of CGTs that address the root causes of genetic and chronic diseases.

CGTs go beyond alleviating the symptoms of a disease. **In some cases, these therapies may cure diseases once considered uncurable**, leading to recoveries for patients who were dying from treatment-resistant leukemia or suffering painfully from sickle cell anemia, for example. For other conditions, CGTs can halt or slow disease progression, preventing children from going blind due to inherited retinal diseases or helping infants with spinal muscular atrophy live longer lives free of ventilators and feeding tubes.

Troublingly, **these breakthroughs are not equally accessible to all**. Nationwide, the low-income people covered by the Medicaid program have reduced access to CGTs compared to those covered by Medicare or commercial insurance,[3] consistent with historical trends for other novel, highly specialized therapies.[4] There are also disparities **within** the Medicaid program: due to significant policy variations across state lines, Medicaid enrollees in some states have more reliable access than in others.[5]

Supporting access for Medicaid means supporting health outcomes for historically underserved groups, including:

- Low- and middle-income families. In households below 200% of the Federal Poverty Level ($62,400 for a family of four), Medicaid covers 4 out of 10 nonelderly adults and 7 out of 10 children.[6]
- **Communities of color**. As compared to non-Hispanic White people, Medicaid enrollment is 70 to 220% higher among people who are Black, Hispanic, or indigenous (i.e., American Indians, Alaska Natives, and Native Hawaiians and Pacific Islanders).[7]
- **Rural populations**. People in rural communities are more likely to be enrolled in Medicaid than those in urban centers.[8]
- **Children with special health care needs**. Nationwide, almost half of these children are covered by Medicaid across all income levels.[9]

Ensuring access to CGTs for Medicaid enrollees is thus a crucial component of the broader push to promote equity in precision medicine overall, as described in the sidebar.

Promoting Access to Precision Medicine

Precision medicine is an innovative clinical approach that leverages variation among individuals with respect to their genes, environments, and lifestyles to inform treatment and personalized care. As science advances, clinicians have ever-better tools to predict how each patient's disease will progress and which treatment will be the most effective. CGTs represent the pinnacle of targeted treatments tailored to patients' specific genetic variants and other clinical factors.[124]

Unfortunately, the benefits of precision medicine are not always equally distributed. Significant attention has been paid to inequities in the pipeline of targeted tests and treatments, including the underrepresentation of historically marginalized groups in genetic datasets and clinical trials.[125] But as emphasized by the Centers for Disease Control and Prevention (CDC), precision medicine "needs to go beyond basic and clinical research" to address issues of access and community engagement.[126] Medicaid is an essential avenue for promoting equitable access to precision medicine testing and treatment.

Diagnosing the Problem: Access Barriers at Each Step in the Patient's Journey

In policy, as in medicine, identifying the right treatment depends on accurately diagnosing the problem.

On paper, federal law provides that all Medicaid enrollees should have timely access to FDA-approved drugs and most specialty care. Moreover, for children and youth under the age of 21, states must cover **all** medically necessary diagnostic and treatment services—including those not covered for older adults—under the comprehensive benefit for Early and Periodic Diagnostic, Screening, and Treatment (EPSDT) services.[10]

States vary significantly in how they implement these federal requirements, however. And within a single state, certain policies may be implemented differently by each of the state's MCOs—private plans that contract with the state to administer coverage, similar to third-party administrators for employer health plans. (See the sidebar for additional discussion of Medicaid program structure and areas of variation.)

In practice, when Medicaid-enrolled patients seek transformative therapies for rare and serious diseases, they and their caregivers can encounter barriers at each step in their journey from diagnosis to treatment, particularly in the early years following FDA approval. with respect to new transformative therapies for rare and serious diseases. Many of these barriers reflect certain key differences between transformative therapies and other, more familiar drugs and services.

CGTs are more like a heart transplant than a statin. These are highly sophisticated therapies that require specialized training to manufacture and administer. Some CGTs must be manufactured specifically for each patient. As a result, CGTs are typically available at only a few Centers of Excellence nationwide, especially in the early years following approval. These therapies address serious and life-threatening conditions that can't be managed as effectively, or at all, with maintenance medications or other, more commonly available therapies. They also typically come with higher upfront costs than many other therapies, while offering a potential cure or permanent remission.

Medicaid Fundamentals and State-Level Variation

Each Medicaid Program is Unique. Each state operates its own Medicaid program subject to baseline requirements under federal law. Federal law requires Medicaid programs to cover certain core populations and benefits, including most types of hospital and physician services. States can choose to extend coverage for additional eligibility groups or services above the federal minimum. States also have the ability to impose various types of coverage restrictions and utilization management requirements, such as prior authorization. And each state establishes its own provider reimbursement rates and payment methodologies. The federal government pays for 50% to 90% of most qualifying Medicaid costs, depending on the state, the patient, and the type of health care service or other program activity.

Most States Contract with One or More Managed Care Organizations. Traditionally, Medicaid programs reimbursed on a FFS basis, paying each provider directly for each service rendered to a Medicaid patient based on a published fee schedule. Today, however, most Medicaid enrollees receive their coverage through MCOs.[127] Each MCO is generally free to establish its own provider network, negotiate its own rates with providers, and develop its own drug formulary and other utilization management requirements, subject to compliance with federal law and any standards established by the state.

This paper discusses four key barriers that can impede Medicaid enrollees' access to CGTs:

- Inconsistent access to the testing and specialists needed to confirm the patient's diagnosis and identify the appropriate treatment. Notably, genetic testing is nearly always required to determine whether a patient is eligible for a CGT.
- Unclear, untimely, or unduly restrictive coverage policies for new therapies. Providers are typically unable to take on the significant cost of acquiring, pretreatment processing, and administering a CGT until they receive confirmation that the therapy will be covered for a specific patient.
- Administrative delays between referral and treatment as the provider navigates issues such as prior authorization, reimbursement policies, and cross-state provider enrollment.
- Inadequate support for long-distance travel, without which a low-income patient may be unable to afford the trip to a Center of Excellence for care.

We identified these barriers based on a 50-state survey of Medicaid policies (see the sidebar for details), a review of the literature, and extensive anecdotal evidence from stakeholders including patient advocacy organizations and Centers of Excellence that offer CGTs.

Depending on the circumstances, these barriers may prevent a patient from accessing care, or may delay the start of treatment. But for many patients with serious and life-threatening diseases, time is of the essence. Any delays in diagnosis and treatment could allow a patient's cancer to progress beyond the point of treatment, or a child's field of vision to shrink even further.[11] In addition to causing additional suffering for the patient and their family, ongoing disease progression may reduce the likelihood of successful treatment and could even jeopardize the patient's eligibility to receive the treatment at all.[12]

Manatt Cell & Gene Therapy Research Collaborative: 50-State Survey Methodology

With the support of a multidisciplinary group of stakeholders, for all 50 states, plus Washington, DC (collectively referred to as "states"), Manatt evaluated Medicaid FFS and managed care policies pertaining to:

- Coverage and reimbursement for CGTs in hospitals and physician offices, including policies specific to out-of-state or out-of-network care.
- Coverage and reimbursement for genetic testing and genetic counseling (FFS only)
- Policies on Medicaid enrollment for out-of-state providers.
- Coverage for travel supports

This research was conducted between June 2023 and December 2024. Manatt consulted publicly available materials including each state's Medicaid State Plan, statutes, regulations, fee schedules, policy guidance, and MCO contracts. For any ambiguities, Manatt reached out to state officials for clarification. If you are interested in learning more about the Manatt Cell & Gene Therapy Research Collaborative, please email cgt@manatt.com.

This Is a Moment of Urgency and Opportunity

As scientific innovation accelerates, Medicaid policy needs to keep up or patients will be left behind. Since the first CGT was approved in 2017, policymakers and MCOs have adapted their drug coverage frameworks to accommodate these new therapies, often in a delayed, inconsistent, or ad hoc fashion. As of the end of 2024, the FDA approved more than 25 CGTs, almost half of them in the last two years, with hundreds more therapies in clinical development.[13]

These products offer the hope of new cures for dozens of cancers and other diseases—many of them rare conditions that currently lack effective treatments[14]—but only if patients have reliable and timely access. Acknowledging current access challenges, the Center for Medicare and Medicaid Innovation (CMMI) is currently implementing a Medicaid demonstration project with federally standardized terms for promoting access to CGTs for sickle cell disease.[15] The model's full details, extent of state participation, and ultimate impact remain to be seen, but in the meantime, this narrowly targeted demonstration does not support access for the many Medicaid enrollees with other rare and serious conditions.

At the same time, state Medicaid programs are confronting new budget pressures due to declining tax revenues and the expiration of temporary pandemic-era funding,[16] combined with the threat of significant cuts to federal Medicaid funding in the coming years.[17] States have previously expressed concerns about the high upfront cost of certain CGTs, particularly in light of state requirements for balanced budgets and federal requirements around rate setting for Medicaid MCOs. These budget constraints contribute to ongoing challenges to ensuring access for Medicaid enrollees.

Policymakers need a comprehensive playbook for CGT access. We can now list the barriers that most often prevent Medicaid enrollees from accessing the right treatment at the right time. That means we can also develop targeted strategies to address those barriers and promote equitable access.

For each of the access barriers discussed below, this paper describes opportunities for reforms by the following key stakeholders. Many of these solutions align with recommendations from other thought leaders.[18]

- Congress has broad authority to add or modify federal requirements for state Medicaid programs. This paper focuses on access barriers for patients, but we note that other stakeholders have identified opportunities for Congress to ease state financial impacts associated with transformative therapies for Medicaid enrollees.[19]
- The federal Centers for Medicare & Medicaid Services (CMS) oversee the Medicaid program, including issuing regulations or guidance to clarify federal requirements.
- State policymakers define and implement Medicaid policies within the bounds of federal law.
- MCO leaders choose how to administer coverage for their members, subject to the requirements of federal law and state contract requirements.

By 2030, CGTs could offer life-changing results to more than 100,000 patients annually.[20] **Policymakers can ensure that Medicaid enrollees are fairly represented in that number** by implementing reforms outlined in this paper.

See the Appendix for Compiled Lists of Access Barriers and Policy Solutions

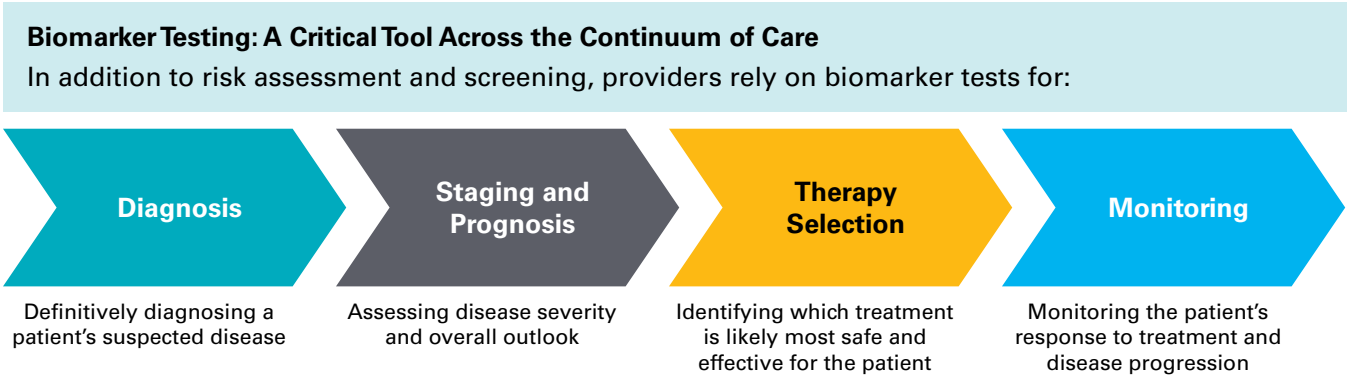
Appendix 1 is sorted by access barrier.

Appendix 2 is sorted by stakeholder group (CMS, Congress, and states and MCOs).

Barrier 1. Inconsistent Access to the Testing and Specialists Needed to Identify and Refer Eligible Patients

CGTs are targeted cures for patients with particular genetic variants or other specific biomarkers. For that reason, a genetic test (or other biomarker test) is typically needed to confirm whether a patient is likely to benefit from a CGT, as shown below. (See the Biomarker Testing and Genetic Counseling: Key Terms text box below for additional detail regarding biomarkers and biomarker testing.)

Exhibit 2. Biomarker Testing: A Critical Tool Across the Continuum of Care



As compared to patients with private insurance, patients enrolled in Medicaid are:

- Less likely to receive genetic testing;[21]
- Less likely to receive genetic counseling to support their decision making;[22] and
- Less likely to have timely access to specialists with the expertise to order the right test, interpret the results, and recommend the right treatment.[23]

These can be serious setbacks, especially for the patients with rare and serious diseases who have the most to gain from transformative therapies. Their disease will continue to progress during any delays in confirming their diagnosis, causing additional suffering for the patient and their family and potentially reducing the likelihood that the treatment will be a success.

Fortunately, there are steps policymakers and MCOs can take to address current access gaps for Medicaid enrollees, as described below.

Access Barrier 1	**Inconsistent Access to the Testing and Specialists Needed to Identify and Refer Eligible Patients**
Why This Matters	To identify the right treatment, patients need access to the right test and the right specialist at the right time.

Potential Access Barriers	Strategies to Address the Access Barriers
Variable coverage for: • **Biomarker testing, including genetic testing** • **Genetic counseling**	Federal/state policymakers and MCOs should **ensure comprehensive access to medically necessary biomarker testing**, including: • Publishing clear coverage conditions based on federal standards and evidence-based guidelines and consensus statements • Covering multigene panel testing ahead of approval for products in development that have been designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs) • Covering genetic counseling in connection with covered genetic tests • Limiting prior authorization requirements for biomarker testing and genetic counseling
Variable access to specialists who can confirm a diagnosis and make referrals to appropriate Centers of Excellence	Federal/state policymakers and MCOs should **ensure timely access to diagnostic specialty care**, including through: • Appropriate standards and oversight for adequate provider networks and timely access • Appropriate reimbursement for specialists • Supporting access to specialists via telehealth, including across state lines

Biomarker Testing and Genetic Counseling: Key Terms

Terms such as biomarker testing, genetic testing, and genomic testing are sometimes used in different ways by different stakeholders. This paper uses the terms as defined below. These definitions align with model legislative language developed by the American Cancer Society Cancer Action Network (ACS CAN) to require comprehensive coverage for biomarker testing by Medicaid programs and state-regulated private insurers.

- **Biomarkers** mean variations in a person's genes, proteins, or other molecules that may provide useful information for disease diagnosis or treatment.
- **Biomarker testing** includes:
  - Genetic tests that identify chromosomal abnormalities or inherited variants in a patient's DNA (e.g., genes associated with various congenital disorders, or BRCA gene variants that impact therapy selection).
  - Other types of genetic tests, such as somatic testing to identify the genetic makeup of a specific cancerous tumor (which may be different from other tumors in the same patient).
  - Testing other biomarkers, such as proteins or enzymes, to see how a patient's genes express themselves, how the patient's disease is progressing, or how a patient responded to treatment.

This section focuses on biomarker testing of symptomatic patients for purposes of confirming a diagnosis and identifying the most appropriate therapy. Different considerations may apply to screening asymptomatic individuals for biomarkers or genetic variants that carry an increased risk of developing a disease in the future.

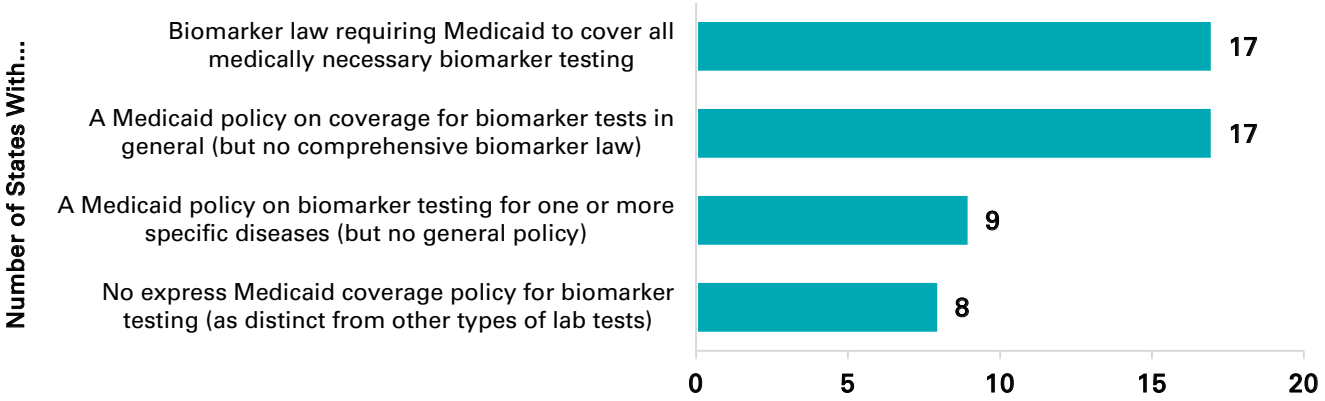
- **Genetic counselors** are professionals who specialize in helping patients make decisions about whether to seek a genetic test and how to interpret the findings, including advising patients about how inherited diseases and conditions might affect them or their families.[24]

Goal A. Coverage of Biomarker Testing and Genetic Counseling

The Problem

Most Medicaid programs have published coverage policies for specific types of genetic tests—prenatal and newborn screening, for example, as well as testing for certain inherited genes that carry an increased risk of cancer, such as BRCA gene variants. However, **only two out of three states have published generally applicable policies describing the circumstances under which they will cover genetic tests or associated genetic counseling**, as illustrated below in Exhibit 3. Moreover, in states with managed care, coverage determinations are often left to individual MCOs. This complexity can create uncertainty and delays for both patients and providers as they request prior authorization and, if necessary, appeal denials of coverage. (For additional discussion of prior authorization and appeals, see Barrier 3, below.)

Exhibit 3. State Medicaid FFS Coverage Policies for Biomarker Tests



Source: Manatt CGT Research Collaborative. Fifty-state survey conducted between April and August 2024.

A biomarker test is generally required to confirm a patient's eligibility to receive a CGT. Furthermore, patients with rare or serious diseases may need to assess multiple genes and other biomarkers before unlocking the right diagnosis and plan of care.[25] It may be necessary to sequence the entire genome or exome in some cases, but often, a patient can be diagnosed and treatment eligibility may be confirmed with a faster, cheaper multigene panel test—**if** that test is covered by their state or their MCO.[26]

As compared to a series of single-gene tests, a panel test can provide more information more rapidly, and is often more cost-effective thanks to next-generation sequencing technology—a rapidly evolving field that allows for testing more genes more quickly and more cheaply than ever before.[27] As the name suggests, panels assess a few dozen (or even a few hundred) biomarkers or genes that provide the most useful information regarding a particular disease, set of symptoms, or drug response.

A panel test may prevent the need for repeat patient visits to a lab, specialist's office, or genetic counselor, as well as repeat lab processing fees. Meanwhile, in many cases, labs do not charge significantly more for a panel test than for a more targeted test.[28] This may reflect the common practice among labs of running panel tests on all samples, but limiting their analysis and reported results to the specific biomarker or genes that

were ordered. As a result, for the lab, there is no difference in the cost of returning results for a single-gene assay or a broader multigene panel. Moreover, the advantages of panel tests will only improve over time due to rapid advances in next-generation sequencing technology.

Policy Solutions

Publish clear policies that ensure coverage for medically necessary biomarker tests, including multigene panel tests, as well as genetic counseling. The following strategies focus on biomarker tests for purposes of diagnosing, treating, or monitoring a patient's existing health condition; different policy considerations may apply to biomarker testing of asymptomatic individuals for purposes of risk assessment or screening.

States and **MCOs** should consider these solutions.

Congress could require these policies for all Medicaid programs nationwide.

- **Establish clear, generally applicable coverage conditions for biomarker tests** that support diagnosis, treatment, and monitoring of a patient's conditions. Policymakers and MCOs could look to the model language developed by the ACS CAN.[29] This model legislative language requires Medicaid programs and state-regulated private insurers to cover biomarker testing in accordance with federal standards and other authoritative sources, including:
  - All clinical indications approved by FDA, including:
    - The specific uses that FDA approved for each genetic test; and
    - Any tests required to establish clinical eligibility for a drug or to assess the risk of side effects, as described on the drug label.

    (This policy mirrors the existing federal requirement for states to cover all FDA-approved drugs for all clinical indications on the label. This requirement is described further below under Barrier 2.)
  - Medicare coverage policies established by CMS or one of its Medicare Administrative Contractors.
  - Nationally recognized clinical practice guidelines (such as those issued by the National Comprehensive Cancer Network) and other evidence-based consensus statements.

State Spotlight

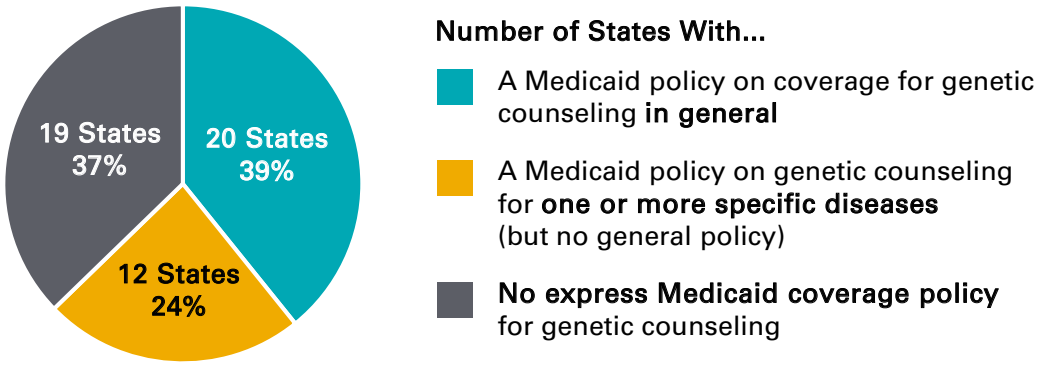
At least 17 states have adopted comprehensive biomarker laws that apply to Medicaid, leveraging ACS CAN's model language.[30]

- **Cover panel tests that could confirm a patient's eligibility for a forthcoming product that meets a serious unmet need for a rare, serious, or life-threatening disease**. FDA grants expedited processing for qualifying products designated as Orphan Drugs, Breakthrough Therapies, and Fast-Tracked Drugs, among others.[31] For any such product that is targeted to particular biomarkers, Medicaid should ensure that panel testing is covered for eligible patients as the product concludes its final phase of pre-approval testing. This way, eligible patients can seek access as soon as the product is approved. Moreover, if they're undergoing other tests in the interim, a panel test would avoid the time and expense of additional testing.

- **Cover genetic counseling before and after any genetic test that may reveal information about the patient's risk of, or treatment options for, a serious or life-threatening disease**. Research shows that after meeting with a genetic counselor, patients and caregivers have a deeper understanding of what their test results mean for them, their family members, and their treatment options.[32]

Opportunity for CMS Guidance. CMS should clarify that, for children and youth under 21, federal law already requires Medicaid programs and MCOs to cover biomarker tests under all these circumstances (at a minimum). Per federal EPSDT requirements, Medicaid programs must cover medically necessary diagnostic and treatment services. FDA-approved labels, CMS coverage policies, and nationally recognized guidelines all demonstrate medical necessity.

Exhibit 4. State Medicaid FFS Coverage Policies for Genetic Counseling



Source: Manatt CGT Research Collaborative. Fifty-state survey conducted between April and August 2024.

State Spotlight

Washington. Washington state requires genetic counseling as a condition of coverage for genetic testing. The state expressly covers genetic counseling for all FFS adults and children when performed by a physician or appropriately credentialed health care professional.[33]

- **Limit prior authorization for biomarker testing and genetic counseling regarding diagnosis, treatment, or monitoring**. If states and MCOs publish clear coverage policies for precision medicine tests, as described above, then they should be able to confirm coverage for many biomarker tests simultaneous with claim review by requiring providers to submit proof of medical necessity along with their claim for reimbursement, such as by identifying the FDA-approved indication or CMS coverage policy that matches the patient's profile.

Goal B. Timely Access to Specialists for Diagnosis and Referral

The Problem

For patients with rare, serious, and life-threatening diseases, it typically requires a specialist to confirm the patient's diagnosis and identify treatment options, particularly with respect to novel transformative therapies that may only be available at a few sites nationwide.

Under federal law, all Medicaid programs must cover medically necessary specialty care, for both children and adults. However, as compared to those with Medicare or commercial insurance, **patients enrolled in Medicaid typically have fewer choices among providers and face longer wait times for specialty and subspecialty care**.

This unfortunate outcome reflects a combination of factors, including provider limitations on accepting new Medicaid patients due to Medicaid's lower reimbursement rates, as well as narrow MCO provider networks that offer few options for local specialists.[34]

Policy Solutions

Ensure timely access to specialty care for Medicaid enrollees with rare and serious diseases, who typically need a specialist to confirm their diagnosis and make a referral for appropriate treatment.

Establish appropriate standards for network adequacy and timely access.[35]

- Establish maximum wait times for specialist visits.
  - CMS could require that MCO members have access to specialists within 30 days of a referral, monitored by secret shopper surveys. This would align with CMS's existing requirements for private plans on the Federally Facilitated Exchanges.[36] (CMS recently finalized new maximum wait times for MCOs, effective in 2027, for primary care, OB/GYN, and outpatient behavioral health visits, but opted not to define a standard for specialty care.[37])
  - States can establish maximum wait times for specialty care, for both their MCOs and their FFS programs.
- States and MCOs should ensure that MCO provider networks include the full spectrum of specialty care, leveraging telehealth as needed for underserved areas when clinically appropriate. They should further ensure (through contract requirements or secret shopper surveys) that the network includes specialists who are accepting new Medicaid patients. However, if at any time the MCO's network is unable to provide timely access to medically necessary services, existing federal law requires the MCO to cover care out of network—and, if necessary, out of state.

States and **MCOs** should consider these solutions.

CMS could establish these policies for all Medicaid programs.

Ensure reimbursement is sufficient to secure participation by specialists. Providers are dedicated to their patients, but they must also ensure sustainable funding for their continued operations. If providers lose money each time they treat a

States and **MCOs** should consider these solutions.

patient enrolled in Medicaid, that creates an incentive to limit the number of Medicaid enrollees they serve. To ensure their reimbursement rates are sufficient to promote access, states and MCOs should consider benchmarking against Medicare or commercial rates. Although CMS does not directly regulate provider reimbursement by states or MCOs, CMS has suggested appropriate payment levels by:

- Cautioning that access problems are likely to occur if Medicaid rates fall below a certain percentage of Medicare rates.[38]
- Encouraging states to consider matching Medicare rates, while confirming that states can also benchmark against average commercial rates if they wish.[39]

States should consider these solutions.

Expand access to specialists via telehealth, including across state lines. Specialists and subspecialists (including genetic counselors) are not evenly distributed across the United States. Rather, they tend to be concentrated in academic medical centers and urban areas.[40] For rural patients and others who lack nearby options, states can enhance access to specialists by ensuring coverage for telehealth consultations. In states with limited specialist capacity overall, members may benefit from increased access to telehealth consultations with out-of-state specialists. States could consider policies such as the following:

- States have broad flexibility to define telehealth coverage for their FFS and managed care programs.[41] Since the COVID-19 pandemic, a growing number of states have adopted "parity" standards under which telehealth is covered for any service that can safely and effectively be delivered via telehealth, as determined by the treating provider.[42] Some states also require MCOs to make telehealth options available when needed care cannot be provided locally in a timely manner, while also preserving the option for patients to travel for in-person services if they wish.
- Under state licensure laws, a provider furnishing telehealth services generally must be licensed to practice in the state where the patient resides. During the pandemic, federal and state officials temporarily relaxed requirements for in-state licensure requirements. Some states have now implemented permanent solutions such as:
  - Adopting a license exception or streamlined registration process for out-of-state providers that seek to provide telehealth (but not in-person services); or
  - Participating in interstate licensure compacts that expedite license approval for practitioners who are already licensed in another participating state.[43]

(In addition to licensure flexibilities, states can also take steps to streamline Medicaid enrollment for out-of-state providers, as discussed below under Barrier 3.)

Barrier 2. Unclear, Untimely, or Unduly Restrictive Coverage Policies for New Therapies

Under the federal Medicaid Drug Rebate Program, state Medicaid programs receive significant rebates from drug manufacturers. In return, **states are required to cover all "medically accepted indications" for most FDA-approved drugs**, as described in the sidebar below.[44] This requirement applies alongside the EPSDT mandate for states to cover all medically necessary services for children and youth under the age of 21, as noted above.

On the ground, however, **states vary significantly in how they operationalize these federal coverage requirements** with respect to transformative therapies, especially in the early years following FDA approval. For patients with rare and serious diseases, the day FDA approves a new therapy should be a day of hope and excitement. But in some cases, Medicaid-enrolled patients may be left waiting for months as their provider seeks confirmation that the therapy will be covered, even when the patient satisfies all FDA-approved clinical eligibility criteria.

Due to the high upfront cost of delivering a transformative therapy, providers are generally not able to deliver treatment to a given patient until they have confirmed both that the therapy will be covered and that the reimbursement rate will allow the provider to recoup its costs (or at least most of its costs).

To ensure that Medicaid beneficiaries receive timely access to the latest therapeutic advancements in accordance with federal requirements, policymakers and MCOs should consider publishing clear, timely, and comprehensive coverage policies, as described below.

Key Terms: Drug Coverage Policies and Processes

- **The Medicaid Drug Rebate Program** requires pharmaceutical manufacturers to pay significant rebates to state Medicaid programs. In exchange, states must cover most FDA-approved drugs for all "**medically accepted indications**."[128] In addition to the indications listed on the FDA-approved drug label, states must also cover evidence-based off-label uses listed in certain established compendiums of drug indications (the American Hospital Formulary Service Drug Information, the United States Pharmacopeia, and the DRUGDEX Information System).[129]
- **Formularies** (also known as **preferred drug lists**) identify which drugs are and are not subject to prior authorization or other utilization management requirements (discussed further under Barrier 3, below). However, the state or MCO must maintain an **"exceptions" process** through which patient and providers can request off-formulary coverage for any medically accepted indication. As compared to a standard prior authorization request, the exceptions process may require additional documentation from the provider, may take longer, and may involve additional levels of internal review for the state or MCO (e.g., case-by-case review by the **Pharmacy and Therapeutics (P&T)** committee).
- **A P&T Committee** is a stakeholder group (typically including physicians and pharmacists, at a minimum) that develops and updates the drug formulary. (For some states or MCOs, formulary development may include additional steps such as review by a Drug Utilization Review Board and/or approval by Medicaid agency leadership.)

Access Barrier 2	**Unclear, Untimely, or Unduly Restrictive Coverage Policies for New Therapies**
Why This Matters	Without clear coverage policies, patient access to a new therapy may be delayed or denied, despite the federal requirement to cover all FDA-approved drugs.

Potential Access Barriers	Strategies to Address the Access Barriers
• **Delayed coverage for newly approved drugs** • **Coverage restrictions narrower than the FDA-approved drug label** • **Incomplete or unclear policies that leave key questions unanswered**	• State policymakers and MCOs should **ensure timely access to newly approved therapies**, by: – Publishing a general policy describing the timeline and processes for ensuring access to newly approved drugs, in accordance with federal requirements. Among other details, confirm that: ▪ Coverage takes effect once a new drug appears on CMS' weekly list of "newly reported drugs" under the Medicaid Drug Rebate Program.[45] ▪ Drugs are covered for all medically accepted indications, as defined in federal law. – Publishing timely policy alerts (e.g., within 90 days) confirming coverage and reimbursement details for significant new therapies designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs). • CMS should **clarify and monitor compliance with federal coverage requirements for newly approved drugs** by: – Publishing guidance that defines minimum expectations and outlines best practices – Leveraging the CMMI CGT Access Model to ensure that states' general drug coverage policies meet federal requirements

Goal. Clear, Timely, and Comprehensive Coverage Policies

The Problem

Some states have published clear guidance outlining coverage criteria, timelines, and processes for newly approved drugs in accordance with federal law—but many states have not. Our 50-state survey, together with prior research and anecdotal reports, identified several common barriers in states' FFS coverage policies, as described in this section. Adding further complexity in states with managed care, each individual MCO may be able to establish its own drug coverage policies and timelines unless the state requires all MCOs to follow FFS drug coverage policies or some other standardized process (as discussed further under Barrier 3).

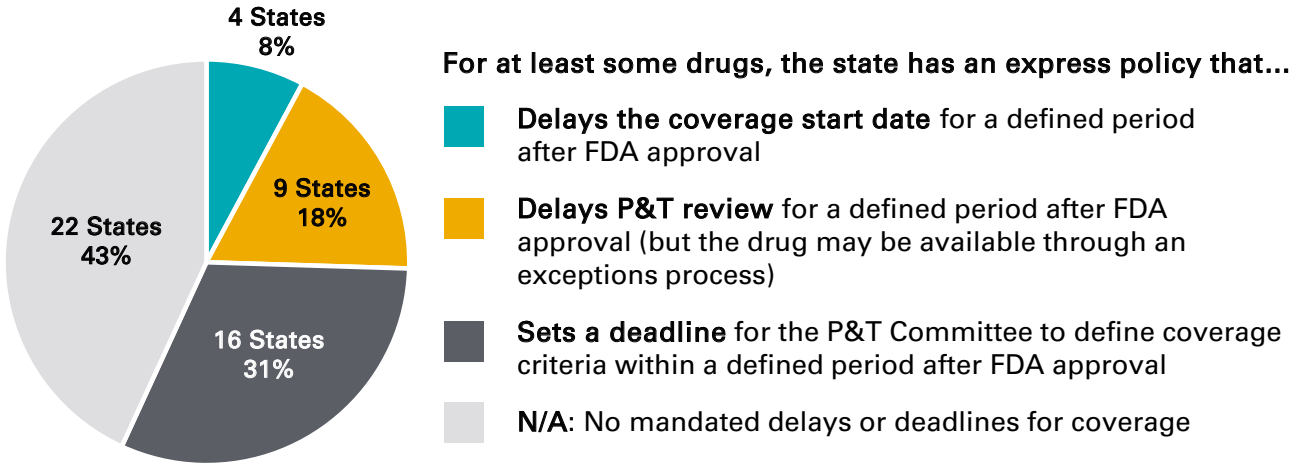
Coverage for new drugs may not take effect until many months after FDA approval. Each week, CMS publishes a list of "newly reported drugs" that have received FDA approval, are available for purchase on the market, and now qualify for mandatory coverage and rebates under the Medicaid Drug Rebate Program.[46]

However, it may take many months after FDA approval—and sometimes even more than a year—until a provider is able to confirm that the drug is covered for a particular patient in need.[47] These delays typically reflect one or more of the following policies:

- Delaying coverage until the drug is published on a list **other** than CMS's weekly list of newly reported drugs. For example:
  - Some states/MCOs may wait for CMS's next **quarterly** Medicaid drug product report.
  - Others may wait until the drug has been assigned its own unique billing code in the Healthcare Common Procedure Coding System (HCPCS), which can take six months or more.
- Delaying coverage until the drug has been reviewed by the state's P&T Committee or other state body, such as Medicaid agency leadership or (for drugs expected to have a significant fiscal impact) by the Governor's office or a legislative committee. Depending on the state, this internal process can take up to a year.

Absent a published coverage policy, patients and providers can use the exceptions process to request coverage before the drug is officially added to the formulary. For high-cost transformative therapies, however, anecdotal reports suggest that exceptions requests are sometimes left pending until the state or MCO has completed its internal process of confirming coverage criteria.

Exhibit 5. State Medicaid FFS Coverage Delays for Newly Approved Drugs



Source: Manatt CGT Research Collaborative. Fifty-state survey conducted between June 2023 and December 2024.

Some states and MCOs define restrictive coverage criteria. As noted above, federal law requires states to cover all "medically accepted indications" for a drug, including all indications approved by FDA on the drug label. In some cases, however, states and MCOs define coverage policies more narrowly than the FDA label, often based on the eligibility criteria for the drug's Phase 3 clinical trial. This presents an access barrier because Phase 3 trials are typically restricted to only a subset (sometimes a very narrow subset) of the patients who would benefit from the drug, and so they tend to define eligibility more narrowly than the final drug label as approved by FDA.[48] A recent study confirmed this barrier by examining several states' published coverage policies for three different CGTs, as described on the next page.

Even if a patient is ultimately able to obtain coverage by appealing a coverage denial or requesting off-formulary coverage through the exceptions process, that process would likely delay the start of treatment in addition to imposing additional administrative burdens on the patient, their family, and their provider.

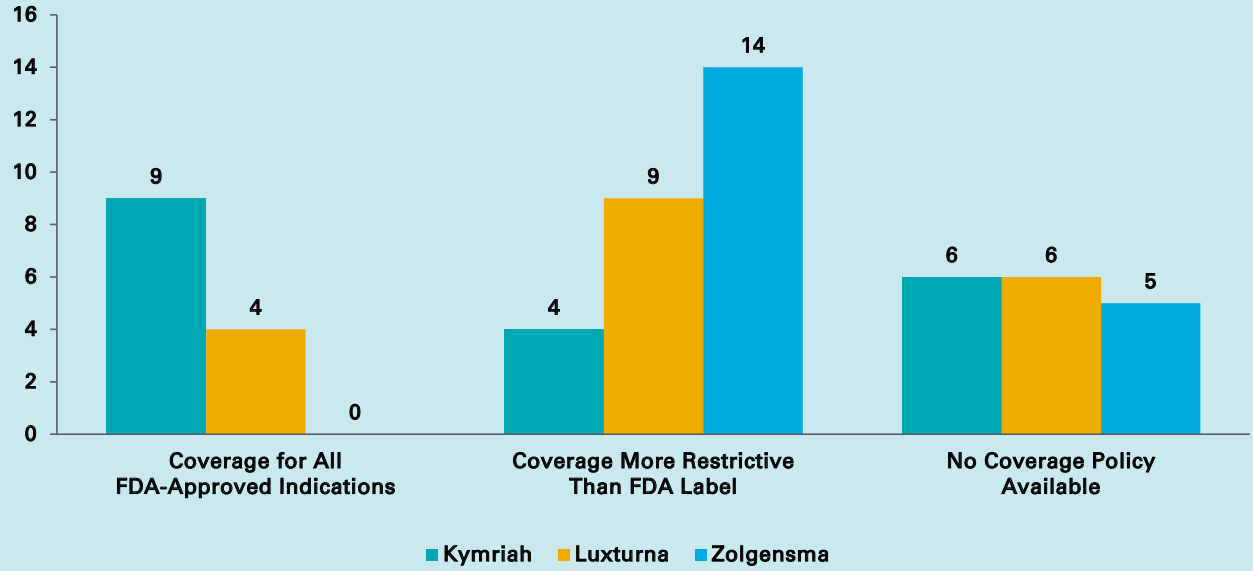
Recent Study Finds Medicaid Coverage Restrictions on CGTs

In a 2023 study,[49] researchers examined published Medicaid coverage policies in 16 states and three national MCOs for the following CGTs:

- Kymriah, which treats blood cancer;
- Luxturna, which prevents blindness from an inherited retinal disease; and
- Zolgensma, which treats babies with spinal muscular atrophy.

In multiple states and managed care plans, the researchers identified "additional exclusionary criteria and/or requests for additional clinical information or assessments" beyond the clinical criteria listed on the FDA-approved drug label, as shown in Exhibit 6. The researchers cautioned that "adverse impacts to patients' clinical outcomes due to treatment delays" could "end up costing Medicaid more money in the long run than if the therapy were promptly covered following FDA approval" as the patients' condition continues to deteriorate, potentially resulting in reduced treatment efficacy.

Exhibit 6. Assessing Whether Medicaid and CGT Coverage Policies Match FDA-Approved Labels in 16 State Programs and Three National MCOs



Source: American Society of Gene & Cell Therapy. "Medicaid Coverage Practices for Approved Gene and Cell Therapies." *Molecular Therapy – Methods & Clinical Development*, 8 June 2023, https://www.cell.com/molecular-therapy-family/methods/fulltext/S2329-0501(23)00077-3.

Drug coverage policies are sometimes incomplete or unclear, particularly for newly approved transformative therapies. Most states have published general policies describing their policies on coverage for newly approved drugs, but those policies often leave key questions unanswered, leaving patients and providers unsure about key details such as the state's timeline for developing coverage criteria for a newly approved product or the specific process for requesting a coverage exception.[50]

In recent years, some states have published product-specific coverage updates for certain transformative therapies on a case-by-case basis. Those updates provide additional certainty for those specific products, but they do not address the need for a framework for clear, consistent updates for new products.

Policy Solutions

Publish clear policies ensuring timely and comprehensive coverage of newly approved therapies, in accordance with federal law.[51]

States and MCOs should consider these solutions.

- **Publish a general policy defining coverage criteria, processes, and timelines for all newly approved drugs** in accordance with federal law and best practices, as enumerated in Exhibit 7.

Exhibit 7. State/MCO Coverage Policies for New Drugs: Federal Requirements and Best Practices

States and MCOs should publish a general policy to advise patients, providers, and other stakeholders about their processes for adding coverage of newly approved drugs, including transformative therapies. These general policies should achieve the following goals.[52]

- **Confirm baseline drug coverage requirements**, including the following:
  - Coverage is effective no later than the date a new drug is included in CMS's weekly list of newly reported drugs under the Medicaid Drug Rebate Program.[53]

State Spotlight: Pennsylvania. MCOs are required to "allow access to all new drugs … within 10 days from their availability in the marketplace," whether by adding them to the formulary or through an exceptions process.[54]

  - A drug is covered for all medically accepted indications, including—but not limited—to the indications listed on the FDA-approved drug label.[55]

State Spotlight: Ohio. If a newly approved drug does not fall within any existing classes in Ohio's preferred drug list, the drug "will be added to coverage with prior authorization criteria consistent with the product labeling approved by the [FDA]."[56]

  - Before the therapy has received its own HCPCS code, providers can bill using an appropriate "miscellaneous" code.

(continued on next page)

Exhibit 7. State/MCO Coverage Policies for New Drugs: Federal Requirements and Best Practices

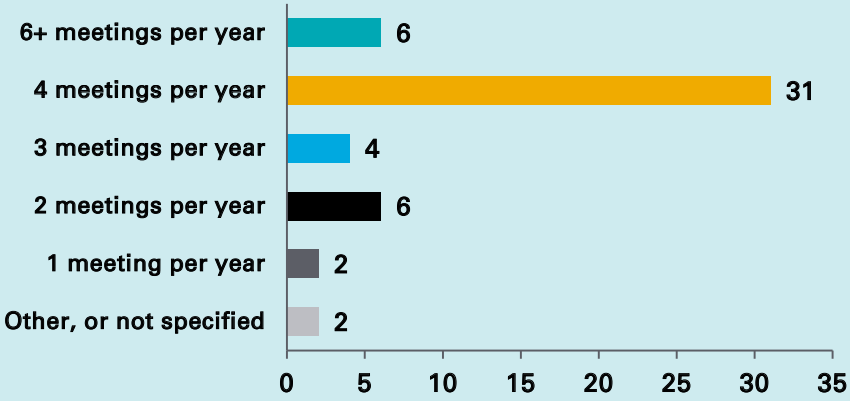
(continued from prior page)

State Spotlight: Indiana. For a newly covered drug that does not yet have its own billing code, "the provider should bill using an appropriate nonspecific CPT or HCPCS code, such as the following: J3490 – Unclassified drugs; J3590 – Unclassified biologics; 90749 – Unlisted vaccine/toxoid. … Providers must include a narrative that accurately describes the drug being administered or the drug's route of administration."[57]

  - Information about managed care carve-outs if relevant, as discussed under Barrier 3.

- **Describe the process and expected timeline for P&T committee review and formulary updates**. Consider best practices such as:
  - Holding quarterly P&T committee meetings to ensure timely formulary updates based on new approvals or updated evidence. Quarterly meetings are already the norm in three out of four states, as shown in Exhibit 7A.
  - Requiring expedited P&T committee review of newly approved products designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (i.e., orphan drugs, breakthrough therapies, and fast track drugs).

Exhibit 7A. State Medicaid P&T Committees: Minimum Meeting Frequency



Source: Manatt CGT Research Collaborative. Fifty-state survey conducted between June 2023 and December 2024.

State Spotlight: Maryland. The P&T Committee must (1) ensure that "any new products are reviewed at the next regularly scheduled meeting," and (2) "provide an expedited review process for newly approved drugs designated as priority by the FDA."[58]

- **Explain the exceptions process** through which patients and providers can request coverage for a drug before it is added to the formulary, or for a medically accepted indication not described in the formulary. Ensure that the exceptions requests are reviewed within the maximum timeframes defined under federal law, as described under Barrier 3, even if the product has not yet completed the P&T review process.
- **Summarize (or link to) the state or MCO's reimbursement methodologies**, both for drugs dispensed by a pharmacy and drugs administered in a provider setting (hospital inpatient, hospital outpatient, physician office, pharmacy, etc.).

- **Publish timely, product-specific policies confirming coverage criteria for significant new products** and other key details, outlined below. These product-specific coverage updates are all the more important for states that have not published a clear general policy on new drug coverage. This update would:
  - Confirm coverage criteria within 90 days of FDA approval to provide certainty and predictability for patients and providers, and to avoid delays associated with the formulary exceptions process. Time is of the essence for these patients.
  - Clarify other key details relevant for access by summarizing (or linking to) the state's policies on:
    - Managed care carve-outs, if applicable, as discussed under Barrier 3;
    - Reimbursement methodologies, as discussed under Barrier 3; and
    - Coverage for transportation, lodging, and meals in states where the significant new therapy is likely to require cross-state or long-distance travel, as discussed under Barrier 4.
  - When deciding which significant therapies merit product-specific coverage updates, states and MCOs should, at a minimum, include products designated by the FDA as addressing an unmet need for a rare, serious, or life-threatening disease (i.e., orphan drugs, breakthrough therapies, and fast track drugs).

Clarify federal coverage requirements for newly approved drugs and monitor compliance. CMS's recent guidance on Medicaid drug coverage has focused on high-level overviews of federal requirements, as well as flexibilities for state to develop new payment models.[59] Our research findings highlight the need for greater clarity and certainty around baseline coverage requirements for new drugs, especially transformative therapies.[60]

CMS could establish these policies for all Medicaid programs.

- **Publish federal guidance clarifying minimum expectations and defining best practices for coverage of new drugs, including transformative therapies**. This guidance should, for example:
  - Clarify minimum federal requirements concerning coverage criteria and timelines for newly approved drugs.
  - Identify specific examples of impermissible practices (e.g., delaying coverage until a new product receives a unique HCPCS code, leaving exceptions requests pending until the P&T committee completes its normal review process).
  - Describe best practices for state/MCO drug coverage policies, as outlined above, as well as MCO risk mitigation and provider reimbursement policies, as discussed under Barrier 3.
  - Describe CMS's approach for oversight of federal drug coverage requirements, including recommended actions for patients or providers who experience access barriers due to potentially noncompliant policies.
  - Include key details relevant for accessing significant new therapies, including long-distance travel supports as discussed under Barrier 4.
- **Leverage the CMMI CGT Access Model to Enhance Oversight of States' Drug Coverage Policies**. As CMMI updates and expands its CGT Access Model in the coming years, CMS could use the model as an opportunity to ensure that states are meeting their federal obligations regarding drug coverage in general.

For example, as a condition of participation for states, CMS could audit states' coverage policies or could require states to submit an attestation confirming compliance with specific federal requirements (as clarified in the new guidance recommended above). CMS could make these attestations publicly accessible, providing transparency and accountability to stakeholders while encouraging adherence to best practices in coverage policy implementation.

State Spotlight

Ohio. In 2022, the Ohio Department of Medicaid published the following coverage update for Zynteglo, which treats the blood disorder beta thalassemia:[61]

TO: Contracted Medicaid Managed Care Organizations

FROM: Jim Tassie, Deputy Director
Office of Managed Care

DATE: November 18, 2022

SUBJECT: Zynteglo Coverage Under Medicaid Hospital Benefit

The Ohio Department of Medicaid (ODM) will be adding coverage of Zynteglo under the Ohio Medicaid Fee-for-Service (FFS) hospital benefit. Zynteglo is a one-time gene therapy to treat beta thalassemia (also known as beta thalassemia major or Cooley's Anemia) in patients who require regular transfusions. More information about Zynteglo can be found here https://www.zynteglo.com/.

Claims guidance below explains how coverage of the drug will be handled in the managed care delivery system. Managed care organizations (MCOs) are required to cover, and provide payment for, all medically necessary inpatient or outpatient hospital claims associated with the treatment of these individuals. Regardless of the setting and the payer (FFS or Managed Care), Zynteglo must be prior authorized through FFS. The approved prior authorization will be shared with the appropriate MCO for care management purposes. A copy of the request form will be shared with the MCOs at a later date.

Outpatient Hospital Setting

- The hospital submits all services, except for Zynteglo, provided on the date of service on an outpatient claim to the MCO.
- The hospital submits a fee-for-service outpatient claim for Zynteglo and only bill for drug acquisition charges on revenue code 631 with C9399 and Zynteglo product specific NDC.

Inpatient Hospital Setting

- The hospital submits an inpatient claim for the admission, except for Zynteglo, to the MCO.
- The hospital submits a fee-for-service outpatient claim for Zynteglo and only bill for drug acquisition charges on revenue code 631 with C9399 and Zynteglo product specific NDC.

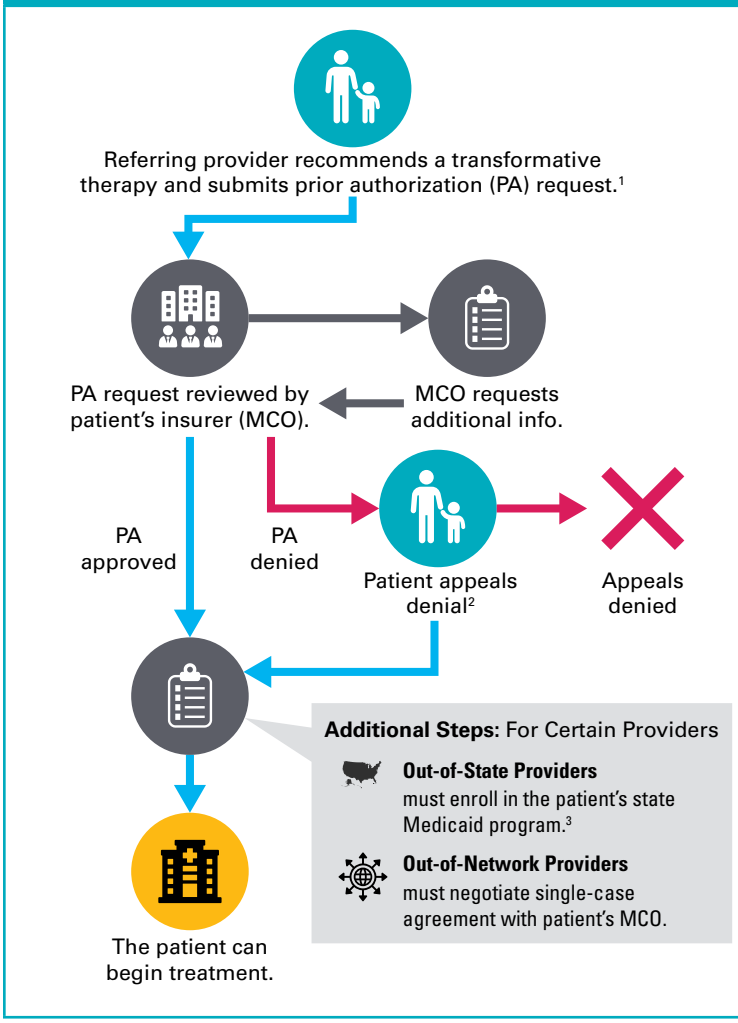
ODM coverage of Zynteglo is effective for dates of service on or after August 17, 2022 – the date of the Federal Drug Administration approval.

Barrier 3. Administrative Delays Between Referral and Treatment: Navigating Prior Authorization, Reimbursement, and Cross-State Provider Enrollment

For many patients, receiving a referral for a transformative therapy feels like the end of a journey—a journey that may have involved multiple rounds of testing and specialist consultations to confirm a diagnosis, identify the most promising therapy, and coordinate with a provider capable of delivering that therapy. But in reality, this referral is merely another milestone on the long road to treatment.

Patients and providers often encounter administrative barriers that create delays between the day a patient is referred for treatment and the day treatment actually begins. Even after confirming that the patient meets clinical eligibility criteria, the provider likely needs to seek prior authorization to confirm that the therapy will be covered, and may also need to negotiate reimbursement rates or coverage for additional services related to therapy administration or monitoring, as discussed below. These processes are particularly complex for providers that are outside the MCO's network or located in another state—both common scenarios for new transformative therapies that are available at only a limited number of Centers of Excellence nationwide. Moreover, an out-of-state provider typically must enroll in the patient's home state Medicaid program.

Exhibit 8. Administrative Delays Between Referral and Treatment: Infographic



1. Depending on the state or MCO, the patient and/or the out-of-state provider may play a role in the PA process.
2. The patient may have access to multiple levels of review with the MCO, the state agency, and the courts.
3. Some states require provider enrollment before the prior authorization process can begin.

All told, these administrative hurdles can delay care by weeks or months, all while the patient's condition continues to decline. Moreover, they increase burdens on patients, caregivers, and providers.

In a positive step, CMS recently finalized rules that will make some prior authorization processes more transparent and efficient. To continue that positive trend, state and federal policymakers should consider other high-impact opportunities to cut through unnecessary red tape. The policy strategies below would accelerate patients' access to the transformative therapies they need to survive and thrive without impairing the ability for states and MCOs to safeguard against fraud, waste, and abuse.

Access Barrier 3	**Administrative Delays Between Referral and Treatment**
Why This Matters	Patients who benefit from CGTs are likely to encounter delays in access related to prior authorization, reimbursement policies, and cross-state provider enrollment.

Potential Access Barriers	Strategies to Address the Access Barriers
• **Treatment delays and administrative burdens due to prior authorization processes**, including when authorization requests must be resubmitted due to minor errors, short expiration timelines, or changes in patient coverage • **Denied authorizations even for clinically appropriate services**	CMS, states, and MCOs should: • **Modernize drug prior authorizations** by making them subject to the same transparency, streamlining, and oversight requirements in CMS's recently finalized Interoperability and Prior Authorization rule, which currently applies only to non-drug services. • **Enhance reporting requirements** to pinpoint problem areas in service authorization denials and appeals. • **Enhance patient protections for continuity of care**. • **Strengthen state and federal oversight** of prior authorization timelines and standards.
Hospital reimbursement rates that fail to cover providers' costs of acquiring and administering transformative therapies	• For therapies administered in hospital settings **offer separate payments designed to (at a minimum) cover the hospital's cost of acquiring the drug product**. Under bundled payments, hospitals risk suffering a substantial financial loss for each patient they treat.

Potential Access Barriers	Strategies to Address the Access Barriers
Heightened administrative burdens and delays for managed care enrollees due to, e.g.: • MCO-specific prior authorization policies and procedures • The need for out-of-network providers to negotiate "single-case agreements" defining coverage and reimbursement	States should: • **Carve transformative therapies out of managed care**, such that the FFS program handles all prior authorizations and provider reimbursement; the MCO remains responsible for other services such as hospital stays and monitoring • **Standardize single-case agreements for transformative therapies** by defining a minimum episode of care and requiring that reimbursement be at or above Medicaid FFS rates
Burdensome and duplicative enrollment processes for out-of-state providers, which can delay care for patients and increase costs for providers	States should implement, and Congress should require, strategies to **streamline enrollment for out-of-state providers who present a low risk of fraud and abuse, and who are already enrolled in Medicare and their home state's Medicaid program**. These strategies include: • Waiving enrollment for out-of-state providers treating a single patient for a short episode of care (<180 days) • Waiving redundant screening requirements • Exempting these providers from any heightened state-specific screening requirements • Allowing proactive enrollment, before a patient is already at the provider's door • Applying the standard revalidation timeframe for out-of-state providers rather than automatically disenrolling them following the episode of care

Goal A. Streamlined Prior Authorization and Effective Oversight

The Problem

Like all health care payers, state Medicaid programs and MCOs will only pay for services that are medically necessary. For certain services—typically including transformative therapies—payers require providers to submit a request for prior authorization before the service is furnished. The request must explain why this particular patient needs this particular treatment, supported by clinical documentation as needed. If the provider furnishes services without the necessary prior authorization, the payer will typically deny the claim, even if the patient met all clinical eligibility criteria.

At its best, prior authorization helps avoid low-value care for patients and unexpected payment denials for providers. But too often, as currently practiced, **prior authorization creates delays for patients and providers**. Fortunately, policymakers and MCOs can take steps to improve the timeliness of prior authorization decisions for transformative therapies, both by clarifying the standards for drug prior authorization (as discussed in Barrier 2) and by enhancing the efficiency, transparency, and oversight of prior authorization processes (as described below).

Prior Authorization for Transformative Therapies

A patient seeking a transformative therapy will typically need to request prior authorization for:

- The transformative therapy itself;
- Other higher-cost services, such as a hospital stay associated with administering the transformative therapy (which may be separate from the authorization for the therapy itself);
- Most services furnished by out-of-network or out-of-state providers, even if those services wouldn't normally require prior authorization; and
- Long-distance travel supports, as discussed under Barrier 4.

Prior authorization is also often required for threshold services to confirm a diagnosis, such as biomarker testing or specialist consultations, as discussed under Barrier 1.

A 2024 report from the Medicaid and CHIP Payment and Access Commission (MACPAC)—a non-partisan legislative branch agency—concluded that "prior authorization may impose significant administrative burdens on patients and providers" and "can delay patient access to care," including life-saving therapies for patients with cancer and other rare and serious diseases.[62]

Even after securing payer authorization, patients may need to start the process all over again. The initial authorization may expire before the patient is able to receive the treatment, given the many other delays in the process. In others, a commercially insured patient may receive prior authorization shortly before losing coverage and enrolling in Medicaid—for example, because they or a family member lost their job—and the FFS program or MCO does not honor the prior plan's authorization decision.[63]

Within a given state, prior authorization policies may vary between the FFS program and each individual MCO, and may also vary for different types of services, including whether prior authorization requests must be submitted by fax, email, or an online submission portal.[64] These dynamics add complexity for providers—especially out-of-state and out-of-network providers not familiar with standard practices for a particular state or MCO—which can compound delays due to misunderstandings or minor paperwork errors.

Unfortunately, evidence also suggests that **some Medicaid MCOs deny a significant number of clinically appropriate prior authorization requests**. MACPAC recently concluded that "prior authorization can produce clinically inappropriate denials of care that may lead to adverse patient outcomes."[65] MACPAC further concluded that this trend is more acute for Medicaid managed care than other types of health coverage, including for novel transformative therapies.[66]

Exhibit 9. MCO Prior Authorization Denials and Appeals by the Numbers

One in eight prior authorization requests are **denied** by MCOs–a denial rate twice as high as Medicare Advantage organizations. For individual MCOs, the denial rate ranged as high as 40%.[67]

1/3 of the denials are **overturned on appeal**.[68] This high reversal rate suggests that structural reforms are needed.[69]

A 2024 federal rule will improve prior authorization processes and transparency requirements, but additional reforms are needed—especially with respect to drug products. CMS's Interoperability and Prior Authorization rule requires most payers—including Medicaid FFS and managed care, as well as Medicare Advantage and Marketplace plans—to publish standardized information on prior authorization requirements, create standardized tools for electronic authorization requests, and publish reports on prior authorization timeliness and denial rates.[70] Importantly, however, these requirements do not apply to most drugs, whether administered by a provider or dispensed by a pharmacy.

The final rule also shortens the maximum timeframes for processing prior authorization requests for non-drug services effective January 1, 2026, as shown in Exhibit 10 below. For drugs—including most provider-administered drugs—federal law already defined a 24-hour maximum for prior authorizations under the Medicaid Drug Rebate Program.[71]

Exhibit 10. Maximum Prior Authorization Timelines for Medicaid MCOs and FFS

		Services Other Than Drugs		
Type of Prior Authorization Request	**Drugs MCOs & FFS**	**Effective 2026: MCOs and FFS**	**Until 2026: MCOs**	**Until 2026: FFS**
Standard Requests	24 hours	7 days	14 days	Not specified
Expedited Requests based on medical need	24 hours	72 hours	72 hours	Not specified

These timeframes appear strong on paper, but as with so many requirements, the reality is more complicated. These timelines can be extended under certain circumstances, including if the payer requests additional information—as payers often do with respect to novel, higher-cost therapies. And as noted under Barrier 2, providers report that requests for newly approved transformative therapies are sometimes left pending until the state or MCO has completed its months-long P&T committee review process.

Policy Solutions

Include drugs in CMS's new standards for prior authorization transparency and oversight. Enhance reporting requirements to pinpoint problem areas. CMS excluded drugs from its 2024 Interoperability and Prior Authorization final rule, but acknowledged "overwhelming" public opposition to the exclusion due to drugs representing "the majority of all prior authorizations."[72] Although the Medicaid Drug Rebate Program already includes maximum authorization timelines, patients and providers would benefit tremendously if CMS's new transparency and oversight standard were extended to transformative therapies and other drugs. In particular, CMS could require FFS programs and MCOs to do the following (and absent a federal requirement, states and MCOs should choose to):

States and MCOs should consider these solutions.

CMS could establish these policies for all Medicaid programs.

- Allow patients and providers, through third-party applications, to:[73]
  - View standardized information on prior authorization requirements for each drug; and
  - Submit electronic prior authorization requests for drugs and receive electronic responses.
  - Provide a "specific reason" when denying drug authorizations. MACPAC reports that denial notices "can be unclear and difficult to understand," which makes it challenging for patients and providers to assess whether the claim was denied based on an administrative error (e.g., failing to include a required form) as opposed to a substantive determination that the patient does not meet clinical eligibility requirements.[74]
  - Publicly report aggregated metrics about prior authorization, including denial rates, the proportion of denials that were reversed on appeal, and average resolution timelines. These metrics should be reported separately for drugs and other services due to the differences in required timelines.
- Building on CMS's final rule, FFS programs and MCOs could also be required to:
  - Report the proportion of authorization decisions that exceeded maximum timelines under federal and state law.
  - Identify trends in the types of services or providers most likely to face authorization delays or denials. This would help to pinpoint common challenges experienced by, for example, out-of-network or out-of-state providers.[75]

Strengthen state and federal oversight of prior authorization timelines and standards. The policies in this section align with recent recommendations issued by MACPAC, the Office of the Inspector General (OIG) for the U.S. Department of Health & Human Services (HHS), and the Government Accountability Office, each of which undertook a thorough study of Medicaid prior authorization.[76]

States and MCOs should consider these solutions.

CMS could establish these policies for all Medicaid programs.

- State and federal officials should review prior authorization reports (as described above) and initiate corrective action upon identifying noncompliance. As new prior authorization reports become available, CMS could update the template for states' Managed Care Annual Report[77] to specifically collect data on prior authorization denials and appeals for each plan.
- To minimize the risk of plans denying medically necessary services:
  - MCO denials should be subject to automatic external medical review if, on appeal, the MCO upholds an initial denial based on lack of medical necessity. A similar requirement could be applied to FFS hearings as well. Medicare Advantage plans are already subject to a similar requirement, causing OIG to express concerns about fairness and "access to care for Medicaid managed care enrollees."[78]
  - States should conduct routine clinical audits of MCOs' prior authorization denials to confirm that MCOs are not inappropriately denying legitimate authorization requests.
- In the forthcoming MCO Quality Rating Systems, which states must implement by 2028 pursuant to a 2024 CMS rulemaking, states should publish MCO-specific data on denials and appeals data.[79] Patients would then have this key information at their fingertips when assessing their options for MCO enrollment.

Enhance patient protections for continuity of care. Consistent with existing requirements for Medicare Advantage plans,[80] MCOs and FFS programs should be required to:

States and **MCOs** should consider these solutions.

CMS could establish these policies for all Medicaid programs.

- Issue prior authorizations that remain valid for as long as medically necessary to avoid disruptions in care, in accordance with applicable coverage criteria, the individual patient's medical history, and the treating provider's recommendation.[81] This includes a prohibition on automatically resetting all prior authorizations at the end of the plan year.
- Honor prior authorizations issued by a prior payer for at least 90 days if a patient switches coverage after beginning a course of treatment, including for services furnished by an out-of-network or out-of-state provider.

Goal B. Adequate Reimbursement for Transformative Therapies

The Problem

For drugs dispensed by a pharmacy or administered in a physician's office, Medicaid reimbursement is typically based on the provider's actual cost of acquiring the therapy or an objective pricing benchmark designed to cover that cost, such as the average sales price (ASP)—the same benchmark used in Medicare Part B.

By contrast, **for transformative therapies that must be administered in hospitals, Medicaid payments sometimes fail to cover providers' costs**. If a state effectively requires providers to suffer a significant financial loss with every patient they treat with a transformative therapy, the state may struggle ensure access for its enrollees. Even if a provider is ultimately able to negotiate a fair rate, that process can delay treatment for the patient.

Many state Medicaid programs rely on bundled payment for some or all hospital services, meaning the hospital receives a lump-sum payment designed to capture all items and services furnished to a particular patient. For inpatient services, for example, many states pay a daily rate based on the patient's clinical acuity. Many states also make bundled payments for defined episodes of care such as delivering a baby or performing a knee replacement surgery (from the preoperative visit through 90 days of recovery).

Bundled payments avoid the hassle of line-item billing for routine products such as anesthesia drugs for a surgery or common IV drips for inpatients. However, for transformative therapies with a higher upfront cost, these bundled payments are typically insufficient to cover the provider's acquisition cost for the therapy alone, never mind the many other items and services associated with administering the therapy and monitoring the patient for potential complications.

Recognizing these risks, a number of states make separate payments for certain hospital-administered therapies, meaning a discrete payment designed to capture the cost of the drug product, over and above the bundled rate for hospital services. Recognizing the potential benefits of separate payment for patient access, CMS will require participating states to make separate payments for sickle-cell CGTs under the Cell and Gene Therapy Access Model.

In some states, it can be challenging to identify which therapies are eligible for separate payment or what reimbursement methodology the state applies. Moreover, in the months following FDA approval of a new product, states themselves may be unsure how they will approach reimbursement, compounding uncertainty and delays for patients and providers.[82]

These separate payments are most common for therapies administered in the outpatient setting, as described in the sidebar. In the inpatient setting, meanwhile, all states have "outlier" payment policies that offer enhanced reimbursement for patients that generate unusually high costs. However, these outlier payments may be limited to only certain types of patients and are often designed to capture only part of the hospital's excess spending beyond the bundled payment. Those limitations may unduly restrict reimbursement for a CGT for which the provider incurs fixed and easily documented costs.

Focus on FFS: Separate Payments and Outlier Policies

This discussion focuses on FFS payment policies because managed care rates are generally not publicly available unless the state requires its MCOs to follow specific payment rules, referred to as "state directed payments" (SDPs). Of the 41 states with managed care, our survey identified 36 states with SDPs for in-network hospital outpatient services and 17 for inpatient services. In many cases, however, Medicaid enrollees must go out of network for transformative therapies, meaning their providers would not benefit from those payment policies.

Policy Solutions

Offer separate payments designed to (at a minimum) cover the provider's cost of acquiring the drug product for transformative therapies administered in hospital settings. Separate payments allow states to promote access to transformative therapies without needing to revise their existing methodologies for bundled payments or cost outliers.[83]

States should consider these solutions.

- For separate payments linked to external pricing benchmarks (such as ASP), states should keep their rates updated so their payments don't lag behind market trends. For example, some states update their fee schedules only once a year, or even less often.
- In addition to covering the provider's acquisition cost, states should consider pricing benchmarks that allow for provider margin (such as Medicare Part B's reimbursement at 106% of ASP) to recognize the many unreimbursed administrative costs borne by providers.
- To avoid confusion for providers, states should publish clear policies identifying which hospital therapies are eligible for separate payment, including in product-specific updates for newly approved therapies, as described under Barrier 2. To avoid uncertainty and confusion, the state could define automatic triggers for separate payment, such as any hospital-administered drug with a list price above a certain threshold.

For managed care, this paper recommends that states carve transformative therapies out of MCO contracts so they're reimbursed solely under the FFS program, as described below. If a state declines to adopt carve-outs, however, the state should consider establishing minimum reimbursement requirements for MCOs, including for out-of-network providers.

State Spotlights

California. For CGTs administered in the hospital setting—inpatient or outpatient—the FFS Medicaid program makes a separate payment at the Medicare Part B rate (ASP + 6%).[84]

Vermont. The state reimburses providers at acquisition cost for certain CGTs administered in the inpatient setting.[85]

Goal C. Ensuring Access for Managed Care Enrollees

The Problem

As compared to members enrolled in FFS, **providers report that MCO enrollees often face greater procedural delays in accessing transformative therapies**. As discussed above, each MCO may define slightly different prior authorization criteria, and the evidence shows that some MCOs impose more restrictive prior authorization criteria or deny claims at a higher rate than their state's FFS program. But for providers outside the MCO's network—as is often the case for Centers of Excellence administering transformative therapies—prior authorization is only the first step.

The out-of-network provider must negotiate a "single-case agreement" with the MCO which can take a week or more.[86] This agreement defines which services the MCO will cover, and at what reimbursement rate. Centers of Excellence who treat a significant number of out-of-state and out-of-network patients may need to negotiate hundreds of single-case agreements every year.[87] By contrast, many MCOs will have minimal experience with any given transformative therapy, especially for treatments that were only recently approved or that treat rare conditions. Single-case agreement negotiations tend to be particularly lengthy in these circumstances: the MCO has a strong incentive to minimize spending, but lacks a playbook for defining an episode of care or setting reasonable reimbursement rates.[88]

Centers of Excellence have expressed frustration about narrowly drafted single-case agreements, which may require patients to go back and forth between in- and out-of-network providers for different services, and which may need to be renegotiated in response to minor changes in the patient's treatment plan.[89]

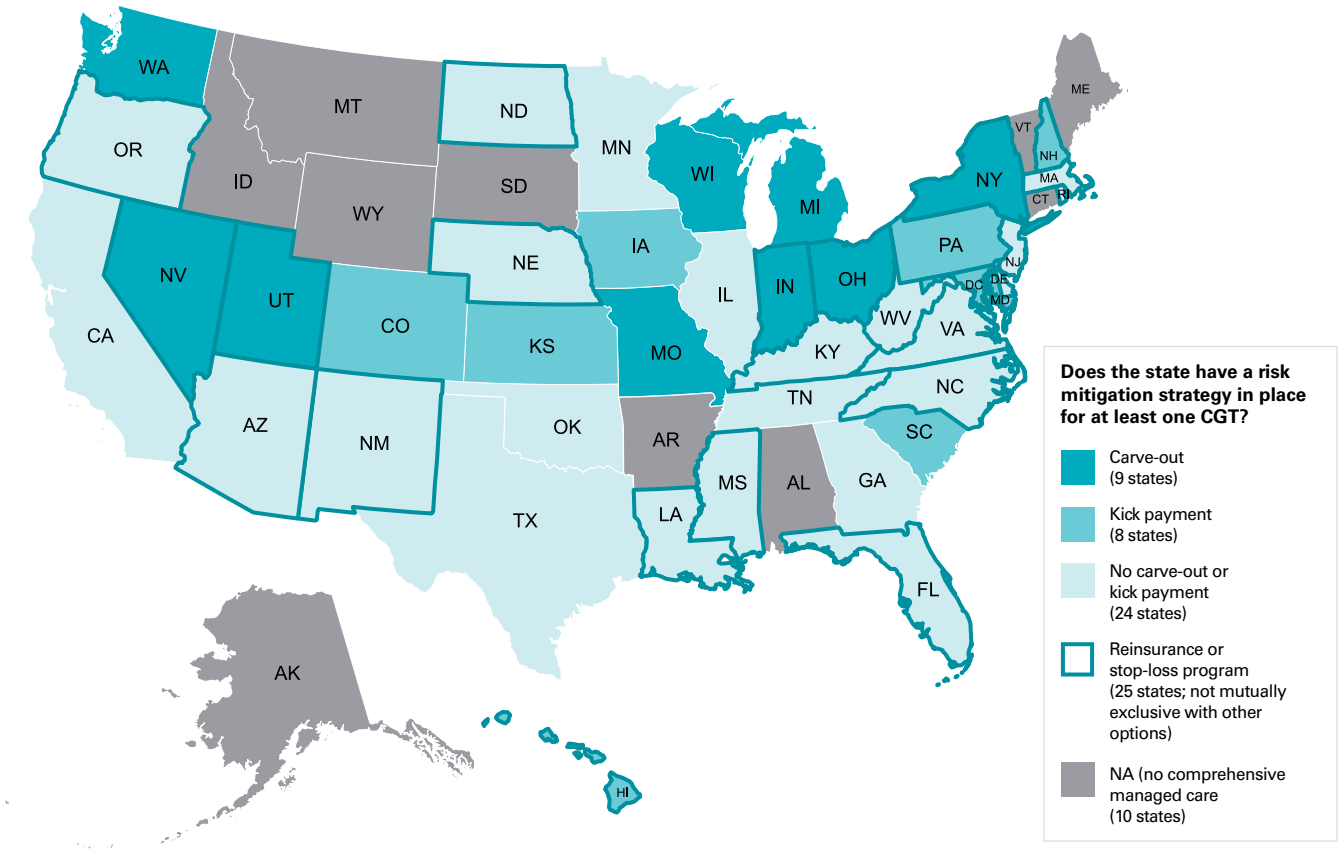
Policy Solutions

States should consider these solutions.

Carve transformative therapies out of managed care. FFS program should process prior authorizations and provider reimbursement, while the MCO remains responsible for other services related to therapy administration and monitoring (services for which the MCO has significantly more experience). For low-volume therapies with high upfront costs but significant long-term benefits, research shows that carve-outs promote patient access to care.[90] Our study identified 9 states that carve at least one CGT out of their managed care contracts, as shown in Exhibit 11. By centralizing authorization and payment processes under FFS, the state would achieve the following benefits:

- **Standardization, transparency, and simplicity for patients, providers, and MCOs.**
  - It is much simpler for a Center of Excellence to confirm a state's FFS policies than to try and identify coverage policies and key contacts for each individual MCO—especially if the state's coverage policies include the best practices described in Barrier 2, above.
  - States can design MCO contracts that allow the state to adjust the list of carved-out products without the need for a contract amendment, such as automatically applying the carve out to any newly approved product above a specified price threshold and/or maintaining a list of carved-out products that can be updated through sub-regulatory guidance. In this way, when FDA approves a new transformative therapy, the state can quickly and clearly confirm that the product is carved out.

Exhibit 11. State Managed Care Risk Mitigation Strategies for CGTs



Source: Manatt CGT Research Collaborative. 50-state survey conducted between June 2023 and December 2024. For states that do not have a published policy, we have solicited feedback from the state to confirm their current practice.

State Spotlights

Utah. The MCO contract carves out all drugs reimbursed at $1 million per dose or more, as listed in the state's Preferred Drug List.[91]

Missouri. All drugs administered on an outpatient basis are carved out of managed care, including transformative therapies administered in physician offices and hospital outpatient departments.[92]

- **Appropriately aligning financial incentives**.
  - MCOs typically receive a fixed payment per member, per month to administer Medicaid coverage. However, this "capitation" structure can be a poor fit for high-cost, low-volume services,[93] especially for:
    - Novel products approved in the middle of a plan year, after capitation rates are already locked in;
    - New and rare services that lack years of historical spending patterns;
    - Smaller MCOs that are unable to spread risk across an extremely large patient population; and
    - MCOs with policies that promote access to care for rare and serious diseases (e.g., broader specialty provider networks or lower rates of improper prior authorization denials), with the result that their members receive transformative therapies at a higher rate than under other MCOs.
  - Among states' tools for mitigating MCO risk (as enumerated in the text box on the next page), a carve-out is the cleanest and clearest way both to avoid any perverse incentive for an MCO to delay or deny access to care, and also to streamline processes for patients and providers, as described above. The state has a concrete interest in maximizing the long-term health of every resident, including when a higher-cost service today can avoid costs down the road by curing or treating a disabling condition.
- **Supporting the state's negotiations with pharmaceutical manufacturers**. If the state consolidates coverage for transformative therapies in the FFS program, the state could leverage that volume when negotiating supplemental rebates with manufacturers.

Mitigating MCO Financial Risk for High-Cost Patients: State Policy Options

Federal law requires states to compensate their MCOs in an "actuarially sound" manner to minimize the risk of MCO insolvency. States have a number of policy options to mitigate the financial impact of a small number of patients with unusually high service utilization and costs. See Exhibit 11 for current state practices.

- With a **carve-out**, coverage responsibilities shift from the MCO to the state FFS program, as described above. Providers submit prior authorization requests and claims directly to the state. In addition to targeted carve-outs for specific items or services (such as CGTs), some states define broader carve-outs (such as a few states that carve out their entire prescription drug benefit).
- Under a **kick payment**, the MCO remains responsible for administering coverage, but the state takes on financial responsibility by making targeted payments to the MCO for specific services, over and above the per-member, per-month capitation fee. States commonly make kick payments for maternity-related services, for example.
- Some states require their MCOs to purchase a **reinsurance or stop-loss** policy, which reimburses the MCO in certain scenarios of unusually high spending (e.g., a patient whose health care costs exceed $500,000 per year). Some states operate their own reinsurance programs, which may be mandatory or optional for their MCOs.[94]
- A **risk corridor program** (also known as a "risk pool") spreads risk across all MCOs in the state by defining a range (or "corridor") of acceptable spending for defined classes of patients or services. If an MCO's costs exceed the corridor's upper limit, the MCO receives an additional payment. Conversely, MCOs with spending below the lower limit must **make** an additional payment.

States should consider these solutions.

Streamline single-case agreement processes for transformative therapies. Carving transformative therapies out of managed care would go a long way toward simplifying negotiations over single-case agreements, as providers and MCOs would then be negotiating coverage and reimbursement solely for familiar services such as inpatient stays, outpatient visits, and patient monitoring through imaging or bloodwork. To streamline this process still further, states should consider the following policies, which could be targeted specifically to transformative therapies (whether or not they are carved out of the managed care contract):

- **Promoting continuity of care by requiring coverage for the full episode of care, when medically necessary.**
  - As an example, states could look to existing federal laws governing clinical trials. Across all major payers (Medicaid, Medicare, and commercial insurance), when a patient enrolls in a clinical trial, the payer must cover all "routine patient costs" associated with that trial.[95] That includes any services necessary for administering the investigational therapy, as well as any other trial-related services related that satisfy the payer's standard coverage criteria (but not, for example, diagnostic tests done solely for research purposes rather than for monitoring the patient's wellbeing).
  - States could apply a similar standard to single-case agreements for transformative therapies: once a patient has received prior authorization to receive a particular therapy at a particular Center of Excellence provider, then the MCO's single-case agreement must allow the Center of Excellence to furnish all routine patient services associated with administering that therapy and monitoring the patient's condition, where medically necessary and appropriate for continuity of care.[96]
- **Establishing reimbursement baselines for providers administering transformative therapies**. As noted above, MCOs are generally free to negotiate provider reimbursement unless the state establishes specific payment requirements. A state could require that, for any provider that receives prior authorization for a transformative therapy (including out-of-network providers), the MCO must pay a baseline amount, at a minimum ensuring that costs are covered. This could accelerate single-case agreement negotiations for any provider willing to accept this baseline, while still preserving the flexibility for providers to negotiate higher rates as they see fit.

Goal D. Streamlined Medicaid Enrollment for Out-of-State Providers

The Problem

Under federal law, providers are generally required to enroll with a state's Medicaid program before they may receive Medicaid reimbursement, including when treating patients enrolled in managed care plans.[97] **The enrollment process may take multiple weeks to months**, depending on the state and the provider type.[98]

When patients travel across state lines for care, the treating provider must enroll with the patient's home state Medicaid program. As a result, **Centers of Excellence that attract patients nationwide may need to enroll with dozens of states**, including separate enrollment processes for the hospital, the treating physician(s), and

often other providers as well, such as physician assistants and nurse practitioners. If the patient is expected to require inpatient care, that may require enrollments for multiple clinicians across multiple inpatient shifts, in addition to the clinicians who actually administer the transformative therapy.

States can waive duplicative screenings, but few do so. Under federal law, all Medicaid providers must undergo certain screenings and background checks—for example, to prevent reenrollment by a provider who was excluded from participation after committing Medicaid fraud.[99] However, if an out-of-state provider is already enrolled in Medicare or another state's Medicaid program, the state is permitted to waive duplicative screenings, or even exempt the provider from enrollment entirely if (1) the provider is only treating one patient from that state and (2) the episode of care will last less than 180 days.[100]

Our study identified only five states that promise expedited enrollment or enrollment waivers for out-of-state providers (setting aside targeted policies for "border providers" located only a short distance outside state lines).

State Spotlights

Mississippi. For out-of-state providers, the Medicaid agency may rely on screening results from Medicare or the provider's home state Medicaid program.[101]

Moreover, **several states create additional enrollment burdens for out-of-state providers**. In addition to delaying treatment, these policies increase the administrative burdens on providers. Centers of Excellence may be deterred from treating patients from states with particularly burdensome processes,[102] such as the following:

- Some states define state-specific screening requirements beyond the federal baseline. Under federal law, hospitals and physicians are considered low-risk providers that require minimal screening.[103] However, states may choose to impose heightened screening requirements that typically apply only to high-risk providers, such as requesting the Social Security Numbers for every single one of a hospital's board members or requiring the hospital CEO to be fingerprinted for a criminal background check.
- Three states prohibit an out-of-state provider from starting the enrollment process until they've already received prior authorization to treat a patient from that state. This makes it impossible for a Center of Excellence to minimize delays by initiating the enrollment process as soon as they receive the patient referral, or by preemptively enrolling in all states in their catchment area to avoid delays down the road. (Meanwhile, certain other states require out-of-state providers to enroll before they can initiate a prior authorization request.)
- One state has expressly advised out-of-state providers that the enrollment process may take **longer** for them as compared to in-state providers, even though out-of-state providers have typically already been screened and enrolled in at least one other state.

- Even after an out-of-state provider has enrolled, in 15 states, their enrollment will be automatically terminated if they don't regularly bill that state's Medicaid program (e.g., at least once every 12 months). Under federal law, states may allow a provider to remain enrolled for five years before revalidation, and may allow an out-of-state provider to revalidate for another five years just like an in-state provider. For a Center of Excellence that intermittently treats patients from many other states, these auto-termination provisions may require the provider to restart the enrollment process each time they serve a new patient.

Congress is considering a partial legislative solution, but further efforts are needed. The Accelerating Kids' Access to Care Act (AKACA) passed the House in September 2024 but never came to a vote in the Senate.[104] This bipartisan bill would streamline enrollment in several respects for certain providers treating children and youth under the age of 21. If enacted, this bill would be a major step forward. It would not solve the issue, however, due to its focus only on children. Similar language was proposed in an omnibus continuing resolution in December 2024, but was removed from the final, stripped-down version of the bill.

Policy Solutions

States should consider these solutions.

Congress could require these policies for all Medicaid programs nationwide.

Streamline Medicaid enrollment for out-of-state providers who present a low risk of fraud and abuse. As CMS has explained, federal screening and enrollment requirements exist to "reduce the amount of improper payments in Medicaid by minimizing the risk of allowing unscrupulous providers to bill the Medicaid program."[105] CMS considers hospitals and physicians to be low-risk providers as a default, and all the more so for providers—like Centers of Excellence—who are already enrolled in good standing in Medicare and one or more state Medicaid programs. Requiring these providers to undergo duplicative screenings contributes little to program integrity, but increases delays for patients and raises costs for providers.[106]

- States should consider adopting all cross-state enrollment flexibilities under existing federal law,[107] at least for low-risk, out-of-state providers that are enrolled in good standing in both Medicare and their home state's Medicaid program.
- Congress should require these policies nationwide, acknowledging the reality that life changing treatments are available for a growing number of rare and serious diseases, but those treatments often require patients to travel to a Center of Excellence.

These flexibilities include:

- Waiving enrollment requirements for out-of-state providers treating a single patient for an episode of care less than 180 days.
- Waiving redundant screening requirements.
- Exempting these providers from any heightened state-specific screening requirements.
- Allowing proactive enrollment, rather than prohibiting enrollment by out-of-state providers until they're already trying to treat a specific patient.
- Applying the standard revalidation timeframe for out-of-state providers rather than automatically disenrolling them if they do not regularly bill the program.

Barrier 4. Inadequate Support for Long-Distance Travel

The journey to accessing CGTs is marked by multiple milestones: confirming a diagnosis, finding the right provider, and confirming coverage for the right therapy. For all that work to pay off, **the patient must be able to physically travel to the provider site where they will receive treatment**.

For many CGTs, a patient's journey will require travel over long distances, often across state lines. For some treatments, a patient may need to return multiple times, or may need to stay at or near the site of care for multiple days or even weeks for their therapy's preparation, administration, and post-treatment monitoring. Although Medicaid programs must cover so-called "non-emergency medical transportation" (NEMT), some Medicaid enrollees are unable to obtain coverage for necessary costs associated with their long-distance, overnight trip. In other cases, a low-income patient may be required to pay upfront for covered travel costs, then follow the state's administrative processes to obtain reimbursement after the fact.

Transportation is a well-documented barrier to accessing health care services, particularly for complex treatments that are geographically inaccessible to many patients. According to MACPAC, 2.1 million Medicaid enrollees under age 65 reported that they had delayed care because of lack of transportation. Almost two-thirds (65.7%) of those reporting a transportation barrier had income below 100% of the federal poverty level.

It is critical to ensure comprehensive coverage for all supports needed to get a patient to treatment, including compliance with minimum standards under federal law as well as best practices to minimize administrative and financial barriers for patients.

Access Barrier 4	**Inadequate Support for Long-Distance Travel**
Why This Matters	To access transformative therapies, patients and their caregivers often need to travel long distances, sometimes across state lines, and stay nearby to the treatment centers throughout the duration of the treatment.

Potential Access Barriers	Strategies to Address the Access Barriers
Variable and non-transparent state policies regarding long-distance or out-of-state travel supports	• State policymakers should ensure compliance with **federal NEMT coverage requirements**, per CMS's 2023 guidance.[108] • CMS should: – **Remind states and MCOs about federal NEMT coverage requirements**, with an emphasis on scenarios involving long-distance/out-of-state travel and overnight stays – **Audit states' NEMT policies for compliance with federal coverage requirements** – **Urge OIG to enable providers and manufacturers to support patient access**, consistent with the Anti-Kickback Statute
Requirements for beneficiaries to pay upfront for certain travel supports, then seek reimbursement after the fact	Federal/state policymakers and MCOs should: • Ensure that **beneficiaries are never asked to pay upfront for lodging or transportation by common carrier**, which can be unaffordable for low-income patients • Seek opportunities to **minimize the burden on beneficiaries for meals** (e.g., preloaded credit card with minimum per diem; beneficiary may seek reimbursement for excess costs up to maximum per diem)
Unreasonably low per diems for lodging or meals	• Federal/state policymakers and MCOs should **ensure that per diems are both appropriate and updated over time**

Goal A. Comprehensive Coverage for Travel Supports

The Problem

Inadequate coverage for travel support presents a significant barrier for Medicaid beneficiaries, particularly those requiring long-distance travel for highly specialized care. Transportation is a well-documented barrier to accessing health care services, particularly for complex treatments that are geographically inaccessible to many patients.[109] According to MACPAC, 2.1 million Medicaid enrollees under age 65 reported that they had delayed care because of lack of transportation.[110] Almost two-thirds (65.7%) of those reporting a transportation barrier had income below 100% of the federal poverty level.[111]

Federal law requires comprehensive coverage of travel supports for both children and adults under the NEMT benefit. In 2023 guidance, CMS expressly confirmed that states are required to cover cross-state transportation, lodging, and meals, as well as an attendant—such as a parent or other caregiver—to accompany the patient when medically necessary.[112]

On the ground, however, **coverage policies for travel supports varies considerably from state to** state **and MCO to MCO**, especially with respect to lodging and meals for overnight trips, as well as an attendant to accompany the patient when medically necessary. As shown below in Exhibit 12, some states restrict coverage to children only, or decline to cover these travel supports at all.

Exhibit 12. Coverage Gaps for Lodging, Meals, and Attendants in Medicaid FFS

Many Medicaid enrollees will need to travel long distances, including across state lines, to access CGTs. All state Medicaid programs cover transportation, but several states have coverage gaps for lodging, meals, or attendants. The following data focus on FFS Medicaid policies. In states that direct MCOs to administer the NEMT benefit, coverage must be at least as comprehensive as under FFS.

Type of Coverage	Lodging	Meals	Attendant
Covered for adults + children	39 states (76%)	39 states (76%)	41 states (80%)
Covered for children only	2 states (4%)	2 states (4%)	5 states (10%)
Not covered	5 states (10%)	4 states (8%)	1 state (2%)
No published policy	5 states (10%)	6 states (12%)	4 states (8%)

Policies in the yellow and red categories appear to be out of compliance with CMS's 2023 NEMT coverage guidance.[113] **In some cases**, the state's written policies may be out of date and no longer reflect current state practices.

Source: Manatt CGT Research Collaborative. 50-state survey conducted between June 2023 and December 2024.

As an additional complicating factor, some state coverage policies focus solely on local transportation, without addressing the types of long-distance or overnight travel so often required for patients receiving transformative therapies at a Center of Excellence. This lack of transparency and standardized policies creates confusion for both patients and health care providers, leading to delays in care and significant administrative burdens.

State Spotlights

Colorado. State policy confirms that lodging and meals are covered in connection with all authorized out-of-state services, and also for in-state treatments if a one-day round trip is not feasible.[114]

Policy Solutions

Ensure compliance with federal NEMT coverage requirements for Medicaid beneficiaries' long-distance transportation, meals and lodging.[115]

States and MCOs should review their policies to ensure compliance.

CMS should consider the following oversight strategies.

- **Remind states and MCOs about federal NEMT coverage requirements**, with an emphasis on scenarios involving long-distance, out-of-state travel and overnight stays.
- **Audit states' NEMT policies for compliance with federal coverage requirements**. These audits could be useful to CMS as a tool to evaluate states for various programs and demonstrations, like CMMI's CGT Access Model.
- **Urge OIG to enable providers and manufacturers to support patient access to transformative therapies**, consistent with the Anti-Kickback Statute. Certain advisory opinions have approved manufacturer support of transportation expenses for Medicaid beneficiaries, but the issuance of an Anti-Kickback Statute safe harbor would allow manufacturers to provide such assistance more quickly, since they would no longer need to go through the lengthy advisory opinion process.

States Should Ensure They Are Capturing Available Federal Funds for Travel Supports

States have the option of claiming for NEMT as an administrative cost (for which the federal government pays 50%) or as a covered Medicaid service. **Covering NEMT as a Medicaid service allows the state to claim a higher federal Medicaid match rate**.[116]

Goal B. Minimizing Administrative and Financial Burdens for Patients

The Problem

According to the Federal Reserve, almost 40% of Americans don't have enough savings to cover a $400 emergency expense.[117] By definition, people enrolled in Medicaid fall on the lower end of the income scale.[118] They often live paycheck to paycheck, making them especially vulnerable to financial hardship.

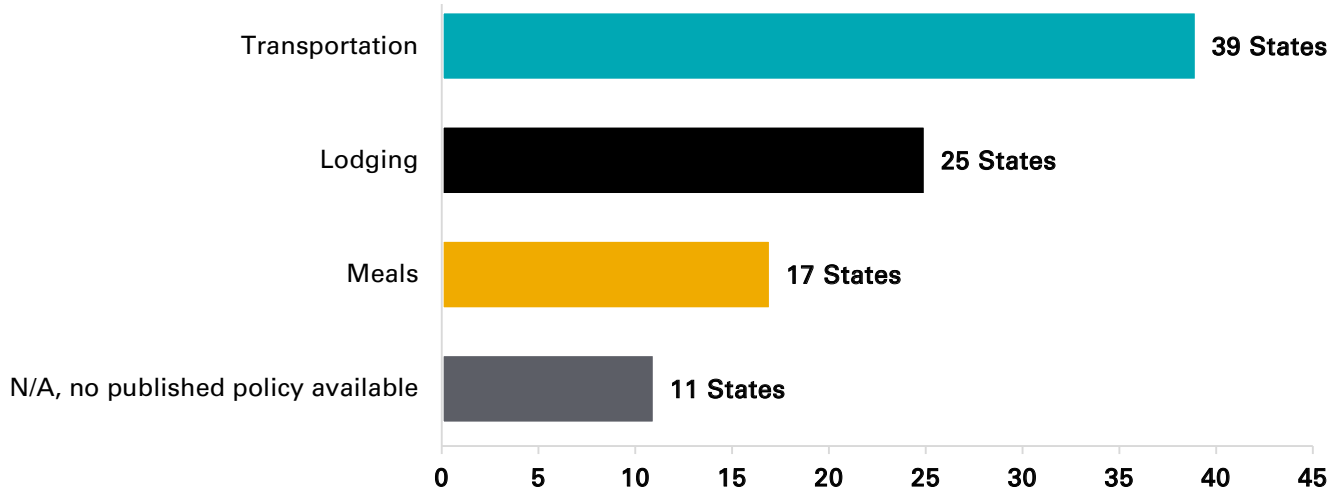
Yet in some states, **Medicaid beneficiaries are required to pay for travel costs upfront and seek reimbursement afterward from the Medicaid program**. Even with the expectation of reimbursement, low-income patients may be unable to pay out of pocket for flights or hotels.

As shown in Exhibit 13, most state Medicaid programs will pay directly for at least some transportation, but less than half commit to doing so for lodging, and less than a third do so for meals. Notably, only 16 states provide direct payment for all three types of travel supports. Even where direct payments are available, they may be limited to specific scenarios, such as only certain modes of transportation, or only specific hotels in neighboring states that have signed agreements with the patient's home state).

Furthermore, the process of seeking reimbursement can be complex and time consuming, requiring beneficiaries to navigate bureaucratic procedures, submit detailed receipts, and potentially wait weeks or months for repayment. Such requirements place a heavy burden on patients already dealing with serious health conditions and can deter or delay necessary treatment.

Along similar lines, **in some locations, the state's Medicaid per diem may be insufficient to cover the costs of lodging and meals**. Some states set per diem allowances with in-state travel in mind. But for a patient from a low-cost, rural state who travels to a Center of Excellence in a major city, those local per diem rates may fall far short of actual costs. Similarly, some states set fixed maximum per diem rates that stay constant for years, even as costs climb. Exacerbating the issue, many states prohibit enrollees from using their per diem to purchase groceries—a lower cost option than restaurants, especially for longer term stays, such as a parent who stays nearby during their child's two-week hospitalization.

Exhibit 13. State Medicaid FFS Programs with Confirmed Policies on Direct Payment for Covered Transportation, Lodging, or Meals



Source: Manatt CGT Research Collaborative. Fifty-state survey conducted between June 2023 and December 2024.

Policy Solutions

Seek opportunities to minimize the burden on beneficiaries for transportation, lodging, and meals.

States and **MCOs** should consider these solutions.

CMS could establish these policies for all Medicaid programs.

- **Ensure that beneficiaries are never asked to pay upfront for lodging or transportation** by common carrier, which can be unaffordable for low-income patients.[119] States and MCOs should pay directly for long-distance transportation and lodging, without requiring the patient to pay upfront and seek reimbursement.
- **Seek opportunities to minimize the burden on beneficiaries for meals**. Although direct payment may be less feasible for meals than for transportation or lodging, states and MCOs could consider alternatives to enrollees paying out of pocket and then seeking reimbursement after the fact. For example, providing enrollees with a preloaded credit card with a minimum per diem, while preserving the ability to seek reimbursement for any meal costs exceeding the provided per diem up to a specified maximum limit.
- **Ensure that per diems are both appropriate and updated over time.**
  - States should ensure their per diem rates reflect current economic realities and regional cost differences. This approach could involve benchmarking per diems allowances against the rates established for state or federal employees.
  - Policymakers should also consider exceptions to standard per diem rates in cases where beneficiaries demonstrate that the allocated amount is insufficient to access necessary health care services.

State Spotlights

Maine. The state's uses the U.S. Government Service Administration's per diem rates for lodging and meals, which are updated regularly to account for variations in prevailing costs across regions and over time. In addition, Maine will make exceptions to those maximum per diems in circumstances where adherence to the maximum limits would prohibit a Medicaid enrollee from being able to access needed covered services.

Conclusion

We live in an exciting era of medical innovation. Transformative therapies offer new hope to patients with rare and serious diseases. This paper diagnoses some of the barriers that Medicaid enrollees commonly encounter when attempting to access those life-changing therapies, backed by robust 50-state survey data. While there is tremendous variability across states, it is clear that there are consistent opportunities to improve patient access. We recommend policy solutions to ensure coverage for the tests needed to accurately identify the correct treatment for a patient at the very start of their journey, to eliminating barriers to completing their treatment plan with qualified providers. These solutions include strategies for robust implementation of federal coverage requirements, best practices to enhance the transparency and efficiency of state and MCO processes, and other opportunities to strengthen coverage and promote timely access to services. We hope this paper can contribute to the important ongoing discussions on this issue. Implementing these solutions will help ensure that these groundbreaking treatments are not just scientific milestones, but accessible lifelines for all who need them.

If you are interested in learning more about the Manatt Cell & Gene Therapy Research Collaborative, please email cgt@manatt.com.

Appendix 1. Policy Solutions, Organized by Access Barrier

Barrier 1. Inconsistent Access to the Testing and Specialists Needed to Identify and Refer Eligible Patients

Why This Matters	To identify the right treatment, patients need access to the right test and the right specialist at the right time.

Potential Access Barriers	Strategies to Address the Access Barriers
Variable coverage for: • **Biomarker testing, including genetic testing** • **Genetic counseling**	Federal/state policymakers and MCOs should **ensure comprehensive access to medically necessary biomarker testing**, including: • Publishing clear coverage conditions based on federal standards and evidence-based guidelines and consensus statements • Covering multigene panel testing ahead of approval for products in development that have been designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs) • Covering genetic counseling in connection with covered genetic tests • Limiting prior authorization requirements for biomarker testing and genetic counseling
Variable access to specialists who can confirm a diagnosis and make referrals to appropriate Centers of Excellence	Federal/state policymakers and MCOs should **ensure timely access to diagnostic specialty care**, including through: • Appropriate standards and oversight for adequate provider networks and timely access • Appropriate reimbursement for specialists • Supporting access to specialists via telehealth, including across state lines

Barrier 2. Unclear, Untimely, or Unduly Restrictive Coverage Policies for New Therapies

Why This Matters	Without clear coverage policies, patient access to a new therapy may be delayed or denied, despite the federal requirement to cover all FDA-approved drugs.

Potential Access Barriers	Strategies to Address the Access Barriers
• **Delayed coverage for newly approved drugs** • **Coverage restrictions narrower than the FDA-approved drug label** • **Incomplete or unclear policies that leave key questions unanswered**	• State policymakers and MCOs should **ensure timely access to newly approved therapies**, by: – Publishing a general policy describing the timeline and processes for ensuring access to newly approved drugs, in accordance with federal requirements. Among other details, confirm that: ▪ Coverage takes effect once a new drug appears on CMS' weekly list of "newly reported drugs" under the Medicaid Drug Rebate Program[120] ▪ Drugs are covered for all medically accepted indications, as defined in federal law – Publishing timely policy alerts (e.g., within 90 days) confirming coverage and reimbursement details for significant new therapies designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs) • CMS should **clarify and monitor compliance with federal coverage requirements for newly approved drugs** by: – Publishing guidance that defines minimum expectations and outlines best practices – Leveraging the CMMI CGT Access Model to ensure that states' general drug coverage policies meet federal requirements

Barrier 3. Administrative Delays Between Referral and Treatment

Why This Matters	Patients who benefit from CGTs are likely to encounter delays in access related to prior authorization, reimbursement policies and cross-state provider enrollment.

Potential Access Barriers	Strategies to Address the Access Barriers
• **Treatment delays and administrative burdens due to prior authorization processes**, including when authorization requests must be resubmitted due to minor errors, short expiration timelines or changes in patient coverage • Denied authorizations even for clinically appropriate services	CMS, states and MCOs should: • **Modernize drug prior authorizations** by making them subject to the same transparency, streamlining, and oversight requirements in CMS's recently finalized Interoperability and Prior Authorization rule, which currently applies only to non-drug services • **Enhance reporting requirements** to pinpoint problem areas in service authorization denials and appeals • **Enhance patient protections for continuity of care** • **Strengthen state and federal oversight** of prior authorization timelines and standards
Hospital reimbursement rates that fail to cover providers' costs of acquiring and administering transformative therapies	For therapies administered in hospital settings, **offer separate payments designed to (at a minimum) cover the hospital's cost of acquiring the drug product**. Under bundled payments, hospitals risk suffering a substantial financial loss for each patient they treat.
Heightened administrative burdens and delays for managed care enrollees due to, e.g.: • MCO-specific prior authorization policies and procedures • The need for out-of-network providers to negotiate "single-case agreements" defining coverage and reimbursement	States should: • **Carve transformative therapies out of managed care**, such that the FFS program handles all prior authorizations and provider reimbursement; the MCO remains responsible for other services such as hospital stays and monitoring • **Standardize single-case agreements for transformative therapies** by defining a minimum episode of care and requiring that reimbursement be at or above Medicaid FFS rates

Potential Access Barriers	Strategies to Address the Access Barriers
Burdensome and duplicative enrollment processes for out-of-state providers, which can delay care for patients and increase costs for providers	States should implement, and Congress should require, strategies to **streamline enrollment for out-of-state providers who present a low risk of fraud and abuse, and who are already enrolled in Medicare and their home state's Medicaid program**. These strategies include: • Waiving enrollment for out-of-state providers treating a single patient for a short episode of care (<180 days) • Waiving redundant screening requirements • Exempting these providers from any heightened state-specific screening requirements • Allowing proactive enrollment, before a patient is already at the provider's door • Applying the standard revalidation timeframe for out-of-state providers rather than automatically disenrolling them following the episode of care

Barrier 4. Inadequate Support for Long-Distance Travel

Why This Matters	To access transformative therapies, patients and their caregivers often need to travel long distances, sometimes across state lines, and stay nearby to the treatment centers throughout the duration of the treatment.

Potential Access Barriers	Strategies to Address the Access Barriers
Variable and non-transparent state policies regarding long-distance or out-of-state travel supports	• State policymakers should ensure compliance with **federal NEMT coverage requirements**, per CMS's 2023 guidance[121] • CMS should: – **Remind states and MCOs about federal NEMT coverage requirements**, with an emphasis on scenarios involving long-distance/out-of-state travel and overnight stays – **Audit states' NEMT policies for compliance with federal coverage requirements** – **Urge OIG to enable providers and manufacturers to support patient access**, consistent with the Anti-Kickback Statute
Requirements for beneficiaries to pay upfront for certain travel supports, then seek reimbursement after the fact	Federal/state policymakers and MCOs should: • Ensure that **beneficiaries are never asked to pay upfront for lodging or transportation by common carrier**, which can be unaffordable for low-income patients • Seek opportunities to **minimize the burden on beneficiaries for meals** (e.g., preloaded credit card with minimum per diem; beneficiary may seek reimbursement for excess costs up to maximum per diem)
Unreasonably low per diems for lodging or meals	• Federal/state policymakers and MCOs should **ensure that per diems are both appropriate and updated over time**

Appendix 2. Policy Solutions, Organized by Policymaker

The Centers for Medicare and Medicaid Services (CMS)

CMS likely has the authority to implement the following strategies through rulemaking or guidance, without the need for new legislation enacted by Congress.

Potential Access Barriers	Opportunity for CMS Guidance to Clarify Existing Requirements
BARRIER 1. Inconsistent Access to the Testing and Specialists Needed to Identify and Refer Eligible Patients	
Variable coverage for: • **Biomarker testing**, including genetic testing • **Genetic counseling**	To ensure comprehensive access to medically necessary biomarker testing for children and youth under the age of 21, **issue guidance clarifying that the EPSDT standard requires states and MCOs to cover** the following: • Biomarker testing based on federal standards and evidence-based guidelines and consensus statements, consistent with ACS CAN's model language • Genetic counseling in connection with covered genetic tests
Variable access to specialists who can confirm a diagnosis and make referrals to appropriate Centers of Excellence	To ensure timely access to diagnostic specialty care, **issue regulations establishing maximum wait times for specialist visits**, consistent with existing requirements for qualified health plans on the Federally Facilitated Exchanges

Potential Access Barriers	Opportunity for CMS Guidance to Clarify Existing Requirements
BARRIER 2. Unclear, Untimely, or Unduly Restrictive Coverage Policies for New Therapies	
• **Delayed coverage** for newly approved drugs • **Coverage restrictions** narrower than the FDA-approved drug label • **Incomplete or unclear policies** that leave key questions unanswered	**Issue guidance clarifying minimum expectations and defining best practices for coverage of new drugs**, including transformative therapies. This guidance should, for example: • Clarify minimum federal requirements concerning coverage criteria and timelines for newly approved drugs, as well as the issues discussed below under Barriers 3 and 4 • Identify specific examples of impermissible practices • Describe CMS's approach for oversight of federal drug coverage requirements • Describe best practices for state/MCO drug coverage policies, as well as the issues discussed under Barrier 3 • Include key details relevant for accessing significant new therapies, including long-distance travel supports **Leverage the CMMI CGT Access Model** to ensure that states' general drug coverage policies meet federal requirements (not just for the products covered under the CMMI model)

Potential Access Barriers	Opportunity for CMS Guidance to Clarify Existing Requirements
BARRIER 3. Administrative Delays Between Referral and Treatment	
• **Treatment delays and administrative burdens due to prior authorization processes**, including when authorization requests must be resubmitted due to minor errors, short expiration timelines, or changes in patient coverage • **Denied authorizations even for clinically appropriate services**	• **Issue regulations** that: – **Modernize drug prior authorizations** by making them subject to the same transparency, streamlining, and oversight requirements in CMS's recently finalized Interoperability and Prior Authorization rule, which currently applies only to non-drug services – **Enhance reporting requirements** to pinpoint problem areas in service authorization denials and appeals – Enhance patient protections for continuity of care • **Strengthen federal oversight** of prior authorization timelines and standards • **Issue guidance clarifying minimum expectations and defining best practices for drug prior authorizations**; this could be included in the drug coverage guidance recommended above under Barrier 2

Potential Access Barriers	Opportunity for CMS Guidance to Clarify Existing Requirements
Hospital reimbursement rates that fail to cover providers' costs of acquiring and administering transformative therapies	**Issue guidance clarifying minimum expectations and defining best practices** for: • Reimbursing providers for transformative therapies • MCO risk mitigation and single-case agreements • Out-of-state provider enrollment (expanding upon the strategies discussed in CMS's 2021 guidance on coordinating out-of-state care for children with medically complex conditions) These points could be included in the drug coverage guidance recommended above under Barrier 2
Heightened administrative burdens and delays for managed care enrollees due to, e.g.: • MCO-specific prior authorization policies and procedures • The need for out-of-network providers to negotiate "single-case agreements" defining coverage and reimbursement	
Burdensome and duplicative enrollment processes for out-of-state providers, which can delay care for patients and increase costs for providers	

Potential Access Barriers	Opportunity for CMS Guidance to Clarify Existing Requirements
BARRIER 4. Inadequate Support for Long-Distance Travel	
Variable and non-transparent state policies regarding long-distance or out-of-state travel supports	• **Issue guidance reminding states and MCOs about minimum expectations and best practices for NEMT**, building on CMS's 2023 NEMT guidance, including: – Emphasizing coverage for long-distance and out-of-state travel supports – Ensuring that beneficiaries are never asked to pay upfront for lodging or transportation by common carrier – Ensuring that per diems are both appropriate and updated over time This could be included in the drug coverage guidance recommended above under Barrier 2. • **Urge OIG to enable providers and manufacturers to support patient access**, consistent with the Anti-Kickback Statute
Requirements for beneficiaries to pay upfront for certain travel supports, then seek reimbursement after the fact	
Unreasonably low per diems for lodging or meals	

Congress

Congress could enact legislation to implement any of the policies recommended above for CMS. By contrast, the following policy strategies likely **require** legislation to implement:

Potential Access Barriers	Opportunity for Congress to Address the Access Barrier
BARRIER 1. Inconsistent Access to the Testing and Specialists Needed to Identify and Refer Eligible Patients	**Require comprehensive coverage for to medically necessary biomarker testing** by: • Defining clear coverage conditions for biomarker testing based on federal standards and evidence-based guidelines and consensus statements • Requiring coverage of multigene panel testing ahead of approval for products in development that have been designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs) • Requiring coverage for genetic counseling in connection with covered genetic tests • Limiting prior authorization requirements for biomarker testing and genetic counseling
BARRIER 2. Unclear, Untimely, or Unduly Restrictive Coverage Policies for New Therapies	This paper does not discuss high-priority legislative strategies for this barrier. Federal law already requires timely and comprehensive coverage for new drugs, although patients still experience access barriers in practice.
BARRIER 3. Administrative Delays Between Referral and Treatment	**Require states to streamline enrollment for out-of-state providers who present a low risk of fraud and abuse, and who are already enrolled in Medicare and their home state's Medicaid program**. These strategies include: • Waiving enrollment for out-of-state providers treating a single patient for a short episode of care (<180 days) • Waiving redundant screening requirements • Exempting these providers from any heightened state-specific screening requirements • Allowing proactive enrollment, before a patient is already at the provider's door • Applying the standard revalidation timeframe for out-of-state providers rather than automatically disenrolling them following the episode of care
BARRIER 4. Inadequate Support for Long-Distance Travel	This paper does not discuss high-priority legislative strategies for this barrier. Federal law already requires timely and comprehensive coverage for new drugs, although patients still experience access barriers in practice.

States and Managed Care Organizations (MCOs)

The following strategies are available to state policymakers. Except as otherwise noted, these strategies are also available to MCOs unless the state has required an alternative policy.

Potential Access Barriers	Opportunity for States and MCOs to Address the Access Barrier
BARRIER 1. Inconsistent Access to the Testing and Specialists Needed to Identify and Refer Eligible Patients	
Variable coverage for: • **Biomarker testing**, including genetic testing • **Genetic counseling**	**Require comprehensive coverage for to medically necessary biomarker testing** by: • Defining clear coverage conditions for biomarker testing based on federal standards and evidence-based guidelines and consensus statements • Requiring coverage of multigene panel testing ahead of approval for products in development that have been designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs) • Requiring coverage for genetic counseling in connection with covered genetic tests • Limiting prior authorization requirements for biomarker testing and genetic counseling
Variable access to specialists who can confirm a diagnosis and make referrals to appropriate Centers of Excellence	**Ensure timely access to diagnostic specialty care**, including through: • Appropriate standards and oversight for adequate provider networks and timely access • Appropriate reimbursement for specialists • Supporting access to specialists via telehealth, including across state lines [Note: MCOs cannot displace state licensure requirements for out-of-state telehealth practitioners]

Potential Access Barriers	Opportunity for States and MCOs to Address the Access Barrier
BARRIER 2. Unclear, Untimely, or Unduly Restrictive Coverage Policies for New Therapies	
• **Delayed coverage** for newly approved drugs • **Coverage restrictions** narrower than the FDA-approved drug label • **Incomplete or unclear policies** that leave key questions unanswered	**Ensure timely access to newly approved therapies**, by: • Publishing a general policy describing the timeline and processes for ensuring access to newly approved drugs, in accordance with federal requirements. Among other details, confirm that: – Coverage takes effect once a new drug appears on CMS' weekly list of "newly reported drugs" under the Medicaid Drug Rebate Program[122] – Drugs are covered for all medically accepted indications, as defined in federal law • Publishing timely policy alerts (e.g., within 90 days) confirming coverage and reimbursement details for significant new therapies designated by FDA as addressing an unmet need for a rare, serious, or life-threatening disease (orphan drugs, breakthrough therapies, and fast track drugs)
BARRIER 3. Administrative Delays Between Referral and Treatment	
• **Treatment delays and administrative burdens due to prior authorization processes**, including when authorization requests must be resubmitted due to minor errors, short expiration timelines, or changes in patient coverage • **Denied authorizations even for clinically appropriate services**	• **Modernize drug prior authorizations** by making them subject to the same transparency, streamlining, and oversight requirements in CMS's recently finalized Interoperability and Prior Authorization rule, which currently applies only to non-drug services • **Enhance reporting requirements** to pinpoint problem areas in service authorization denials and appeals • Enhance patient protections for continuity of care • **Strengthen state and federal oversight** of prior authorization timelines and standards
Hospital reimbursement rates that fail to cover providers' costs of acquiring and administering transformative therapies	For therapies administered in hospital settings, **offer separate payments designed to (at a minimum) cover the hospital's cost of acquiring the drug product**. Under bundled payments, hospitals risk suffering a substantial financial loss for each patient they treat.

Potential Access Barriers	Opportunity for States and MCOs to Address the Access Barrier
Heightened administrative burdens and delays for managed care enrollees due to, e.g.: • MCO-specific prior authorization policies and procedures • The need for out-of-network providers to negotiate "single-case agreements" defining coverage and reimbursement	**States** should: • **Carve transformative therapies out of managed care**, such that the FFS program handles all prior authorizations and provider reimbursement; the MCO remains responsible for other services such as hospital stays and monitoring • **Standardize single-case agreements for transformative therapies** by defining a minimum episode of care and requiring that reimbursement be at or above Medicaid FFS rates [Note: Although strategies focus on state action, MCOs can examine their processes for single-case agreements to promote timely access to transformative therapies]
Burdensome and duplicative enrollment processes for out-of-state providers, which can delay care for patients and increase costs for providers	**States** should implement strategies to **streamline enrollment for out-of-state providers who present a low risk of fraud and abuse, and who are already enrolled in Medicare and their home state's Medicaid program**. These strategies include: • Waiving enrollment for out-of-state providers treating a single patient for a short episode of care (<180 days) • Waiving redundant screening requirements • Exempting these providers from any heightened state-specific screening requirements • Allowing proactive enrollment, before a patient is already at the provider's door • Applying the standard revalidation timeframe for out-of-state providers rather than automatically disenrolling them following the episode of care [Note: MCOs lack authority to modify state policies on out-of-state provider enrollment]

Potential Access Barriers	Opportunity for States and MCOs to Address the Access Barrier
BARRIER 4. Inadequate Support For Long-Distance Travel	
Variable and non-transparent state policies regarding long-distance or out-of-state travel supports	**Ensure compliance with federal NEMT coverage requirements**, per CMS's 2023 guidance[123]
Requirements for beneficiaries to pay upfront for certain travel supports, then seek reimbursement after the fact	• Ensure that **beneficiaries are never asked to pay upfront for lodging or transportation by common carrier**, which can be unaffordable for low-income patients • Seek opportunities to **minimize the burden on beneficiaries for meals** (e.g., preloaded credit card with minimum per diem; beneficiary may seek reimbursement for excess costs up to maximum per diem)
Unreasonably low per diems for lodging or meals	**Ensure that per diems are both appropriate and updated over time**

Endnotes

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68. When patients challenge MCO denials on appeal, at least one out of every three denials is reversed—whether by the MCO itself, by an external medical reviewer (if the state offers this option), or by the state (after an unsuccessful initial appeal to the MCO). U.S. Department of Health and Human Services Office of Inspector General. High Rates of Prior Authorization Denials by Some Plans and Limited State Oversight Raise Concerns About Access to Care in Medicaid Managed Care. July 2023.

69. The vast majority of patients do not appeal service authorization denials, meaning that the initial denial—even if unjustified—is effectively a final denial. MACPAC recognized that the appeals process is often "challenging and burdensome to navigate." However, anecdotal reports suggest that appeal rates for life-changing transformative therapies are higher than for other types of services. Medicaid and CHIP Payment and Access Commission. Report to Congress: Chapter 2 Denials and Appeals in Medicaid Managed Care. March 2024.; U.S. Department of Health and Human Services Office of Inspector General. High Rates of Prior Authorization Denials by Some Plans and Limited State Oversight Raise Concerns About Access to Care in Medicaid Managed Care. July 2023.

70. Centers for Medicare & Medicaid Services. Advancing Interoperability and Improving Prior Authorization Processes, 89 Fed. Reg. 8758. February 2024.; Centers for Medicare & Medicaid Services. CMS Interoperability and Prior Authorization Final Rule (CMS-0057-F). September 2024.

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72. Centers for Medicare & Medicaid Services. Advancing Interoperability and Improving Prior Authorization Processes, 89 Fed. Reg. 8758. February 2024.

73. Specifically the Interoperability and Prior Authorization rule requires payers to develop application programming interfaces (APIs) that make prior authorization data and processes available in a standardized fashion. That way, patients and providers can use a third-party application of their choosing to engage directly with the payer's systems.

74. Medicaid and CHIP Payment and Access Commission. Report to Congress: Chapter 2 Denials and Appeals in Medicaid Managed Care. March 2024.

75. State of Illinois Department of Healthcare and Family Services. Recommendations Report by the Illinois Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment. December 2024.

76. Medicaid and CHIP Payment and Access Commission. Report to Congress: Chapter 2 Denials and Appeals in Medicaid Managed Care. March 2024.; U.S. Department of Health and Human Services Office of Inspector General. High Rates of Prior Authorization Denials by Some Plans and Limited State Oversight Raise Concerns About Access to Care in Medicaid Managed Care. July 2023.; U.S. Government Accountability Office. MEDICAID: Managed Care Plans' Prior Authorization Decisions for Children Need Additional Oversight. April 2024.

77. Medicaid. Medicaid and CHIP Managed Care Reporting.

78. U.S. Department of Health and Human Services Office of Inspector General. High Rates of Prior Authorization Denials by Some Plans and Limited State Oversight Raise Concerns About Access to Care in Medicaid Managed Care. July 2023.

79. The forthcoming Quality Ratings Systems are required under CMS's 2024 rulemaking on Medicaid and CHIP Managed Care Access, Finance, and Quality, 89 Fed. Reg. 41002 (May 10, 2024).

80. 42 CFR § 422.112(b)(8).

81. State of Illinois Department of Healthcare and Family Services. Recommendations Report by the Illinois Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment. December 2024. Leukemia & Lymphoma Society. Trials and Tribulations: How to Remove Barriers Blocking Cancer Patients from Clinical Trials and Advance the Next Generation of Treatment. December 2023.

82. Alliance for Regenerative Medicine. Issue Brief: Medicaid Barriers to Accessing Cell & Gene Therapies. November 2023.

83. In a 2024 rulemaking for the Medicaid Drug Rebate Program, CMS clarified that states may claim federal rebates for drugs included in bundled payments **if** the payment "includes an amount directly attributable to the drug" based on a CMS-approved drug payment methodology—effectively, a separate payment that is factored into the bundled payment. 42 CFR § 447.502 (as amended by CMS, Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program, 89 Fed. Reg. 79020 (Sept. 26, 2024)).

84. Cal. Code Regs. Tit. 22, § 51509(g). Cal. Code Regs. Tit. 22, § 51503. State Plan, Attachment 4.19-B. Medi-Cal Notes to Rates.

85. Vermont Medicaid General Billing Forms Manual – Section 4.17; GCR 22-022.

86. Leukemia & Lymphoma Society. Trials and Tribulations: How to Remove Barriers Blocking Cancer Patients from Clinical Trials and Advance the Next Generation of Treatment.

87. Leukemia & Lymphoma Society. Trials and Tribulations: How to Remove Barriers Blocking Cancer Patients from Clinical Trials and Advance the Next Generation of Treatment.

88. State of Illinois Department of Healthcare and Family Services. Recommendations Report by the Illinois Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment. December 2024.

89. Leukemia & Lymphoma Society. Trials and Tribulations: How to Remove Barriers Blocking Cancer Patients from Clinical Trials and Advance the Next Generation of Treatment.

90. Auty SG et al., Association of Medicaid Managed Care Drug Carve Outs With Hepatitis C Virus Prescription Use. August 2021.

91. UT DHHS. Pharmacy Services Manual. Section IV.A.5.; Health Choice MCO Contract. Attachment C.1.1.2.(A).(21).; UT DHHS. Preferred Drug List & Pharmacy Coverage Resources. Effective January 1, 2025. Ultra High Cost Drugs, p. 113.

92. MO HealthNet, Managed Care Contract – Section 2.11.6 Pharmacy Services.

93. Alliance for Regenerative Medicine. Issue Brief: Medicaid Barriers to Accessing Cell & Gene Therapies. November 2023.

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95. Center for Medicare & Medicaid Services, National Coverage Determination for Routine Costs in Clinical Trials (310.1). July 9, 2007.; 42 U.S.C. §§ 300gg–8, 1396d(a)(30), (gg).

96. Leukemia & Lymphoma Society. Trials and Tribulations: How to Remove Barriers Blocking Cancer Patients from Clinical Trials and Advance the Next Generation of Treatment. December 2023.

97. Social Security Act section 1902(a)(78); 42 C.F.R. § 455.410(b).

98. House Energy and Commerce Health Subcommittee Hearing, "Legislative Proposals to Support Patients with Rare Diseases," February 2024.

99. 42 C.F.R. Part 455.

100. CMS has summarized these and other enrollment flexibilities in Informational Bulletin: Guidance on Coordinating Care Provided by Out-of-State Providers for Children with Medically Complex Conditions (October 2021) and the Medicaid Provider Enrollment Compendium (MPEC) (July 2018).

101. MS Admin Code. Title 23. Part 200. Rule 4.2: Conditions of Participation.

102. Alliance for Regenerative Medicine. Issue Brief: Medicaid Barriers to Accessing Cell & Gene Therapies. November 2023.

103. Centers for Medicare and Medicaid Services. Fact Sheet: Medicaid Provider Enrollment Requirements. August 2016.

104. 118th Congress (2023-2024). H.R.4758: Accelerating Kids' Access to Care Act. July 2024.

105. Centers for Medicare and Medicaid Services. Fact Sheet: Medicaid Provider Enrollment Requirements. August 2016.

106. Alliance for Regenerative Medicine. Issue Brief: Medicaid Barriers to Accessing Cell & Gene Therapies. November 2023.; State of Illinois Department of Healthcare and Family Services. Recommendations Report by the Illinois Advisory Council on Financing and Access to Sickle Cell Disease Treatment and Other High-Cost Drugs and Treatment. December 2024. Leukemia & Lymphoma Society. Trials and Tribulations: How to Remove Barriers Blocking Cancer Patients from Clinical Trials and Advance the Next Generation of Treatment. December 2023.

107. See CMS's Informational Bulletin: Guidance on Coordinating Care Provided by Out-of-State Providers for Children with Medically Complex Conditions (October 2021) and the Medicaid Provider Enrollment Compendium (MPEC) (July 2018).

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113. Centers for Medicare & Medicaid Services. SMD#23-006 - Assurance of Transportation: A Medicaid Transportation Coverage Guide. September 2023.

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116. Medicaid and CHIP Payment and Access Commission. Federal Medical Assistance Percentages (FMAPs) and Enhanced FMAPs (E-FMAPS) by State. September 2024.

117. Federal Reserve. Report on the Economic Well-Being of U.S. Households in 2022 - May 2023. May 2023.

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119. This paper recommends that beneficiaries are never asked to pay upfront for lodging or transportation, as described above. However, if a beneficiary is required to make an upfront payment, CMS should issue guidance to ensure states/MCOs do not require upfront payments in excess of federal cost-sharing limits. When the current cost-sharing limit for an inpatient stay is $75, it is unreasonable to require beneficiaries to front hundreds of dollars for flights and hotel fees.

120. Centers for Medicare & Medicaid Services, Product Data for Newly Reported Drugs in the Medicaid Drug Rebate Program.

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122. Centers for Medicare & Medicaid Services, Product Data for Newly Reported Drugs in the Medicaid Drug Rebate Program.

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124. For more information on precision medicine and related federal initiatives, see The National Institutes of Health. The Promise of Precision Medicine; The White House. Bold Goals for U.S. Biotechnology and Biomanufacturing: Harnessing Research and Development to Further Societal Goals. March 2023.

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126. Khoury, M et al., Health equity in the implementation of genomics and precision medicine: A public health imperative. August 2022.

127. Kaiser Family Foundation. Medicaid Managed Care Tracker.

128. This coverage requirement applies only to "covered outpatient drugs," a term of art that also includes inpatient drugs for which the state identifies a specific payment amount separate from, or factored into, a bundled hospital payment, (Social Security Act § 1927(k)(2); 42 CFR § 447.502). However, states are **not** required to cover certain classes of drugs, including drugs used for weight loss, fertility, hair growth, or erectile dysfunction, as well as most over-the-counter drugs, (Social Security Act § 1927(d)(2)).

129. Social Security Act § 1927(d).

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