

The Cell and Gene Therapy (CGT) Access Model: Evaluation of CMS-Led Development of Outcomes-Based Agreements



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Glossary of Terms and Acronyms

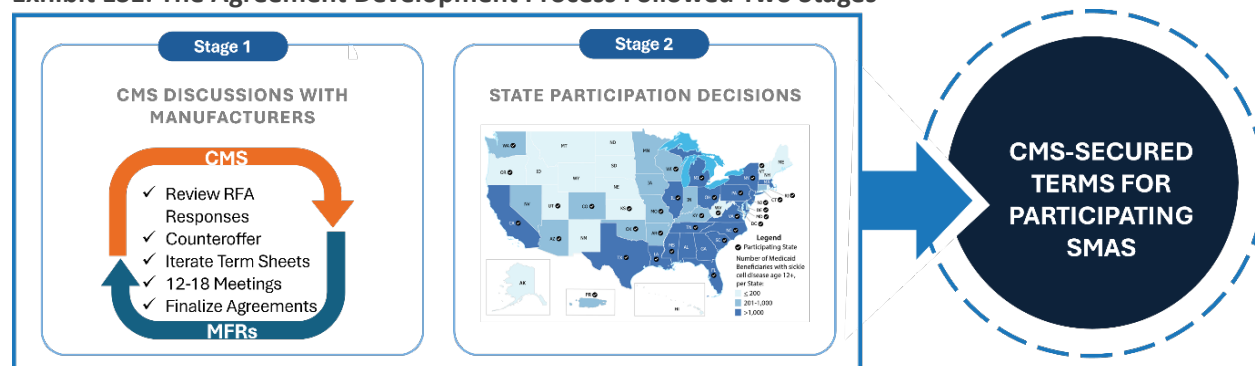
Acronym	Term	Definition
CGT	Cell and gene therapy	Using cells and genes to treat genetic diseases.
CHIP	Children's Health Insurance Program	Federal program that provides health coverage to eligible children. Can be administered as part of the Medicaid program or separately.
CMS	Centers for Medicare & Medicaid Services	Federal agency that runs Medicare, Medicaid, the Children's Health Insurance Program and the federal health insurance marketplace.
FDA	Food and Drug Administration	Federal agency that monitors the safety, efficacy, and security of food, drugs, medical devices, vaccines and other health-related products.
MCOs	Managed care organizations	Healthcare entities that manage the delivery, cost and quality of medical services for members.
MDRP	Medicaid Drug Rebate Program	Federal program that helps to offset the Federal and state costs of outpatient prescription drugs dispensed to Medicaid patients.
OBA	Outcomes-based agreement	Contract where payment to a provider or manufacturer is contingent on achieving agreed-upon, measurable results rather than paying for volume.
RFA	Request for Applications	Formal, competitive solicitation issued by government agencies, foundations, or organizations to invite proposals for funding for contracts.
SCD	Sickle Cell Disease	An inherited blood disease characterized by abnormal hemoglobin that causes red blood cells to become rigid and crescent-shaped, leading to anemia, pain and organ complications due to impaired blood flow.
SMA	State Medicaid agency	The state agency with authority to administer a state's Medicaid State Plan.
SRA	Supplemental rebate agreement	Contract between a state Medicaid agency and a pharmaceutical manufacturer to secure additional rebates beyond the mandatory federal rebate.
T-MSIS	Transformed Medicaid Statistical Information System	Comprehensive database managed by the Centers for Medicare & Medicaid Services that collects data from state Medicaid and the Children's Health Insurance Program. Data cover enrollment, demographics, claims, service use and expenditures.
VBC	Value-based contract	Healthcare payment model that ties reimbursement to the quality, effectiveness and cost-efficiency of care.
VBP SRA	Value-based purchasing supplemental rebate agreement	Contract between a state Medicaid program and a drug manufacturer that ties the cost of a prescription drug to its clinical outcomes or effectiveness, rather than just the volume dispensed.

1. Executive Summary

The Centers for Medicare & Medicaid Services (CMS) launched the Cell and Gene Therapy (CGT) Access Model in 2025 to test whether CMS-led support for agreements between states and manufacturers of high-cost, potentially curative therapies for Medicaid beneficiaries can expand access to these treatments, reduce health care costs, reduce burden to state Medicaid programs, and make it easier for states to pay for cell and gene therapies¹. The first application of this model focuses on cell and gene therapies for sickle cell disease (SCD), a rare inherited blood disorder affecting approximately 100,000 Americans, half of whom are covered by Medicaid or the Children’s Health Insurance Program (CHIP).

This evaluation assesses the CMS-led agreement development guided by the question, *Is it efficient and effective for CMS to negotiate with manufacturers on behalf of multiple states?* The evaluation draws on a framework adapted from prior literature to systematically examine activities, decision points, and key informant perspectives. Using CMS documentation, publicly available materials, and interviews with CMS, manufacturers, and state Medicaid agencies (SMAs) in ten states, the evaluation examines CMS’ discussions with manufacturers and SMA participation decisions (See **Exhibit ES1**). At each stage of the agreement development process, the evaluation identifies criteria for effectiveness, then examines how the two stages contributed to the overall effectiveness of the CGT Access Model in establishing key terms. Within each stage, the evaluation assessed efficiency – defined as administrative burden, timeliness, and clarity – as a necessary component of effectiveness. The findings demonstrate the effectiveness of CMS-led agreement development to secure rebates for high-cost therapies.

Exhibit ES1: The Agreement Development Process Followed Two Stages



Key: CMS - Centers for Medicare & Medicaid Services; MFR - drug manufacturer; RFA - Request for Applications; SMA – State Medicaid Agency

Note: A larger, more detailed map is shown in Exhibit 6.

Stage 1: Discussions with Manufacturers

For about nine months, CMS met with manufacturers of the two Food and Drug Administration-approved gene therapies for SCD, Casgevy (manufactured by Vertex Pharmaceuticals) and Lyfgenia (manufactured by Genetix Biotherapeutics), to establish mutually agreeable terms that would then be offered to states. Manufacturers expressed strong interest in joining the model because it allowed them to reach a larger share of Medicaid beneficiaries with SCD without negotiating separate agreements

with each state. They adjusted internal processes to meet CMS documentation and auditing requirements but did not view these changes as barriers to participation. Manufacturers felt that the process of coming to an agreement was less efficient than they had expected based on their experiences with other public and private-sector negotiations, but still more efficient than negotiating individually with each state. CMS is also satisfied with the terms agreed to with manufacturers. Stage 1 of the agreement development process met the criteria for effectiveness: CMS and manufacturers reached agreement on a set of key terms — rebates, fertility preservation services, access policies, and coverage criteria; discussions concluded on schedule to allow states to start model implementation by January 1, 2026; and CMS released the finalized terms to SMAs in December 2024.

Stage 2: State Medicaid Agency Participation in the CGT Access Model

After reviewing the CMS-secured key terms, 32 states, the District of Columbia, and Puerto Rico¹—representing roughly 84% of Medicaid beneficiaries with SCD—opted to participate in the CGT Access Model. In interviews, SMAs reported that they considered:

- High cost of CGTs for SCD
- State Medicaid SCD population size
- Ability to independently negotiate with manufacturers
- Perceptions of the costs and benefits of model participation
- State legal considerations
- Signaling support for CMS-led discussions with manufacturers

For states with large Medicaid populations with SCD, population size shaped several considerations, including the expected cost of CGTs, the state’s ability to negotiate independently, and the perceived costs and benefits of joining the model. However, states balanced multiple factors in deciding whether to participate, and no single factor predominated across state decision-making.

SMAs invested administrative resources into assessing the model key terms and making participation decisions. Both manufacturers and SMAs noted that reaching agreement on the outcomes measurement requirements in the model’s value-based payment supplemental rebate agreements (VBP SRAs) was more complex than a typical supplemental rebate agreement (SRA) negotiated by state purchasing pools. Finally, some SMAs expressed surprise at the level of documentation required by CMS. Despite some challenges with burden, timeliness, and clarity, 34 states signed state participation agreements with CMS and executed VBP SRAs with manufacturers, making this final stage of the negotiation process effective.

¹ Unless otherwise noted, references to ‘states’ throughout this report include the District of Columbia and Puerto Rico.

Assessment of the CMS-Led Agreement Development Process

States, manufacturers, and CMS each brought competing interests and priorities to the negotiation process. The different parties identified several insights from the novel approach of CMS developing a set of outcomes-based agreement (OBA) key terms that states can choose to apply in agreements with manufacturers.

- CMS gained substantial leverage in discussions by pooling beneficiaries across states.
- CMS brought negotiation capacity and resources that are often limited at the state level, particularly for SMAs with fewer resources or less experience with value-based payment arrangements.
- SMAs appreciated CMS sharing the administrative burden of collecting outcomes data for rebates and managing dispute resolution for rebate determinations. CMS noted that this centralized approach could also benefit manufacturers by consolidating rebate calculations.
- Both manufacturers and SMAs found the federal authority to allow manufacturer-paid fertility preservation services under the model appealing.
- Manufacturers liked that the model offers assurances of timely reimbursement to qualified treatment centers that provide CGTs.
- CMS, manufacturers, and SMAs described the CMS-led agreements as complex. Manufacturers and SMAs viewed the agreements as more detailed and administratively intensive than their typical value-based payment contracts, though still more efficient than a series of individual negotiations.
- The model's voluntary participation structure allowed SMAs to independently negotiate with manufacturers, preserving this option for interested states.

Key informants verified that **CMS-led agreement development can be an efficient alternative to state-by-state negotiations for high-cost CGTs**. At the same time, key informants identified structural tensions that created efficiency challenges. Manufacturers indicated that negotiations were more protracted than expected. They cited CMS' limited prior experience with negotiations and disagreements between the parties around rebate expectations which extended the time required to reach agreement. CMS agreed that starting with tighter central parameters could have been more efficient, but noted that overall the process was completed within pre-established timelines. State Medicaid agencies, while broadly supportive of the model, reported that the administrative requirements for joining the model were more complex and burdensome than anticipated, particularly for agencies with limited resources and experience with value-based contracts. These efficiency challenges do not undermine the overall finding of the effectiveness of the CMS-led negotiation process, but they identify specific areas — negotiation preparation, clarity of expectations, and state onboarding support — where efficiency could be improved.

Overall, CMS successfully navigated the two stages of the agreement development process, meeting milestones that demonstrated how **CMS-led discussions with manufacturers were efficient and effective**:

- Stage 1: CMS led discussions with manufacturers that **concluded on schedule** with **agreement on model terms** and **released those model terms to SMAs** for their consideration.
- Stage2: Roughly two-thirds of **states signed agreements with CMS** to participate in the model, **established VBP SRAs with manufacturers**, and **submitted required onboarding documentation**. Collectively, **these states cover 84% of Medicaid patients with SCD**.

CMS secured terms that aligned with manufacturer priorities, addressed SMA needs, and promoted patient access to potentially curative treatments. SMAs and manufacturers expressed interest in CMS leading future deliberations for other drugs, building on the successes and lessons learned in this process. The CGT Access Model discussions with manufacturers and the resulting agreements between manufacturers and SMAs, coupled with interest from all parties in future discussions, suggest future CMS-led agreement development processes for other high-cost therapies may also be successful.

2. Introduction and Background

The Centers for Medicare & Medicaid Services (CMS) introduced the Cell and Gene Therapy (CGT) Access Model with the aim of increasing access to potentially curative treatments for people living with rare and severe diseases, beginning with cell and gene therapy for sickle cell disease (SCD).ⁱⁱ Sickle cell disease is an inherited blood disease affecting approximately 100,000 Americans — primarily those of Black/African American and, to a lesser extent, Hispanic/Latino descentⁱⁱⁱ — and causes misshapen red blood cells that restrict blood flow, leading to anemia, tissue and organ damage and stroke, and severe pain crises known as vaso-occlusive events.^{iv,v}

Gene therapies for SCD offer a new, potentially curative treatment by altering a person's genetic code to prevent production of the abnormal protein that causes red blood cells to become misshapen.^{vi} The two CGTs for SCD cost about \$2 million to \$3 million per patient and require complex care that can be delivered only at manufacturer-certified treatment centers. The CGT Access Model tests whether CMS-secured rebates increase Medicaid beneficiaries' access to these therapies, improve health outcomes, and reduce costs and administrative burden for state Medicaid agencies (SMAs).^{v,2}

As part of the CGT Access Model, in 2024, CMS secured key terms with CGT manufacturers on behalf of SMAs. This was the first time CMS served in this capacity. The initial focus of the CGT model was SCD because it has a relatively high prevalence in the Medicaid population,^{3,vii} existing treatment options had limited effectiveness, and new treatment options were becoming available.

CMS and manufacturers of the two newly approved CGTs for SCD agreed on key terms,^{4,viii} including upfront rebates and outcomes-based rebates for SMAs. Because CGT administration involves chemotherapy that may cause infertility, CMS also secured manufacturer-paid fertility preservation services for Medicaid beneficiaries receiving CGT through the model.⁵

In 2025, CMS announced that 34 SMAs had signed state agreements with CMS, the precursor to finalizing value-based purchasing supplemental rebate agreements (VBP SRAs) between SMAs and manufacturers.⁶

Exhibit 1 illustrates key milestones in the CGT Access Model, from Food and Drug Administration (FDA) approval of CGTs for SCD in 2023 through model implementation with participating SMAs planned

² The CGT Access Model includes both Medicaid and Medicaid Expansion CHIP beneficiaries, referred to collectively in this report as "Medicaid beneficiaries."

³ The eligible SCD population is estimated to be approximately 52,524 (2021).

⁴ Key terms, as defined by CMS in the RFA to manufacturers, "means the central parameters of the agreement negotiated with CMS... that will form the basis for individual SRAs between the Manufacturer and participating States." (CMS, Request for Applications from Applicable Manufacturers)

⁵ The Manufacturer Request for Applications (RFA) required that manufacturers pay for fertility preservation services encompassing patient education, egg harvesting, sperm collection, testicular tissue extraction and tissue cryopreservation. Manufacturers may not offer in vitro fertilization services.

⁶ Variation in key terms is permitted when it is necessary to comply with state laws and regulations but must be approved by CMS.

through 2035. This first report focuses on the 2024–2025 period and examines early model development and participation activities rather than long-term implementation outcomes.

Exhibit 1. CGT Access Model Timeline



Key: **CGT** - cell and gene therapy; **CMS** - Centers for Medicare & Medicaid Services; **FDA** - Food and Drug Administration; **RFA** - Request for Applications; **SCD** - sickle cell disease; **SMA** - state Medicaid agency

As shown in **Exhibit 1**, CMS developed the CGT Access Model to be ready shortly after FDA approval of Casgevy (manufactured by Vertex Pharmaceuticals) and Lyfgenia (manufactured by Genetix Biotherapeutics) in December 2023. As part of model development, CMS gathered input from multiple stakeholders to inform model design, negotiation strategy, and to gauge SMA interest. Early engagement focused on building CMS understanding of SCD, emerging gene therapies, and value-based payment approaches. It also addressed SMA data reporting and rebate processing capacity and prior experience with high-cost therapies. CMS used input from manufacturers, SMAs, clinicians, patient and community representatives, and other federal partners to identify priorities, risks, and implementation considerations. CMS had limited quantitative data at that time to validate assumptions regarding CGT uptake, budget impacts, or financial risks that could inform subsequent discussions.

SCD Care Options and Costs

Several standard care options are available for individuals with SCD. While these treatments can reduce symptoms and complications, they do not cure the disease or fully prevent pain episodes for most patients. In contrast, new CGTs present potentially curative treatments. Standard care options include:

- Supportive care, such as pain management and infection prevention
- Blood transfusions to reduce complications
- Hydroxyurea, an oral chemotherapy medication that keeps red blood cells round
- Crizanlizumab, a monoclonal antibody that improves blood flow

Approximately half of individuals with SCD are enrolled in Medicaid or Children’s Health Insurance Program (CHIP),^{ix} and ongoing health care costs for their treatment are high. Between 2010 and 2018, average annual costs for Medicaid beneficiaries with SCD ranged from \$47,404 for beneficiaries younger than 18 years old to \$134,264 for Medicaid beneficiaries ages 18-30 and \$90,574 for beneficiaries ages 31 and older.^x

Medicaid Payment Options for CGT for SCD

CGTs for SCD build on technological breakthroughs and offer potentially curative treatment. However, several cost-related factors create challenges for payers and can limit patient access, including:

- High upfront treatment costs, including clinical evaluation and diagnostic testing before CGT administration
- A small patient population, limiting ability to spread financial risk
- Uncertainty about long-term durability of treatment outcomes
- Ineligibility for 340B pricing,^{7,xi} because CGT for SCD is administered in inpatient settings

Together, these factors require payers to balance access to potentially curative therapies with budgetary constraints and uncertainty about the treatment’s long-term effectiveness.

Because of the high cost of CGTs for SCD, many SMAs have chosen to carve out payment for CGT from inpatient and outpatient hospital reimbursement methodologies. SMAs that carve out payment have four options to determine the price they pay for CGTs for SCD: 1) pay for CGTs as a separate line item; 2) accept the standard Medicaid Drug Rebate Program (MDRP) rebate; 3) negotiate supplemental rebate agreements (SRAs) independently with one or both CGT manufacturers for rebates in addition to the MDRP rebate, or 4) join the CGT Access Model and accept its CMS-secured rebates in addition to the MDRP rebate.

⁷ The 340B Drug Pricing Program, established by Section 1927 of the Social Security Act, requires manufacturers participating in Medicaid to provide discounted prices on outpatient drugs to eligible covered entities and prohibits states from claiming Medicaid rebates on drugs already purchased at a 340B-discounted price.

Medicaid Drug Rebate Program

Section 1927 of the Social Security Act established the MDRP, which requires drug manufacturers to pay statutorily defined quarterly rebates to state Medicaid programs for their covered outpatient drugs in exchange for having those covered outpatient drugs reimbursed by Medicaid.^{xii} However, MDRP rebates alone may be insufficient to alleviate the strain on state Medicaid budgets from high-cost therapies like CGTs.

Supplemental Rebate Agreements

To secure rebates in addition to the MDRP rebate, SMAs may enter voluntary SRAs with manufacturers, who usually provide supplemental rebates in exchange for placement on the Medicaid preferred drug list, also sometimes referred to as the Medicaid drug formulary.^{xiii} With CMS authorization, SMAs have the ability to independently pursue SRAs with manufacturers to meet state needs.

One form of SRAs is value-based purchasing (VBP) arrangements which link reimbursement to improved outcomes, care quality, and cost control, rather than paying for volume.^{xiv} Outcomes-based agreements (OBAs), a type of VBP arrangement, tie payment for a drug to its real-world performance over time, requiring manufacturers to provide refunds, discounts, or other financial adjustments if expected health outcomes are not achieved.^{xv}

How Outcome Based Agreements Work in the CGT Access Model

The CGT Access Model outcomes-based agreements are contracts between an SMA and a manufacturer that tie the ultimate cost of CGT to defined outcome measures (for example, clinical values or utilization of care) over a specified measurement period. If outcome measures are not met, manufacturers pay rebates to SMAs. CMS is helping to coordinate tracking outcomes and payments.

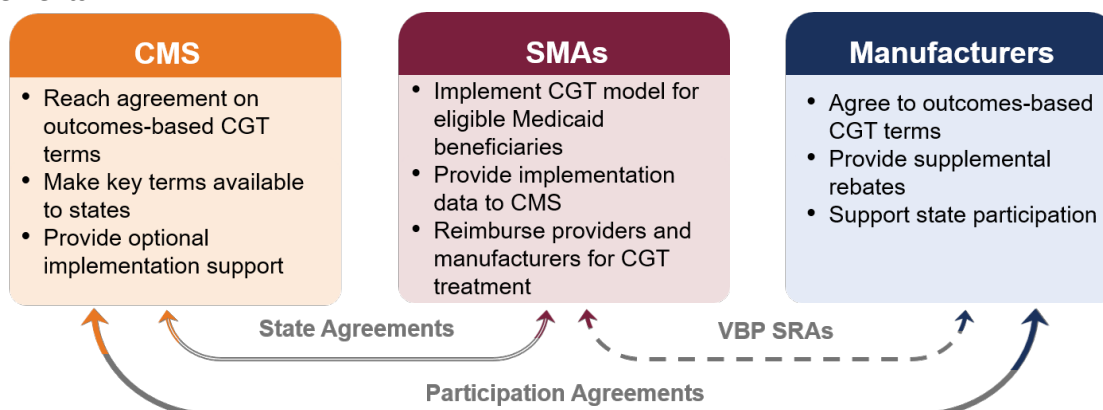
Source: Centers for Medicare & Medicaid Services. (2024). Cell and Gene Therapy Access Model: Request for Applications for States <https://www.cms.gov/files/document/cgt-model-rfa.pdf>

CGT Access Model

The CGT Access Model takes a new approach to high-cost CGTs, testing whether CMS-led agreements with manufacturers can reduce burden to state Medicaid programs, make it easier for states to pay for CGT for SCD and consequently expand Medicaid beneficiary access to this treatment, improve health outcomes, and reduce long-term health care spending. CMS-led agreements include guaranteed rebates and outcome-based rebates in addition to the MDRP rebate. The agreed-upon rebates are only available to SMAs that choose to participate in the Model.

CMS, CGT manufacturers, and SMAs have unique two-way relationships under the model as shown in **Exhibit 2**.

Exhibit 2: Roles and Responsibilities in the CGT Access Model are Formalized Through Written Agreements



Key: CGT - cell and gene therapy; **CMS** - Centers for Medicare & Medicaid Services; **SMA** - state Medicaid agency; **VBP SRA** - value-based payment supplemental rebate agreement

CMS supports SMAs to operationalize the model by providing technical assistance, specifying data collection requirements, and monitoring outcomes. CMS also provided optional funding through a Notice of Funding Opportunity to support SMAs with implementation activities.

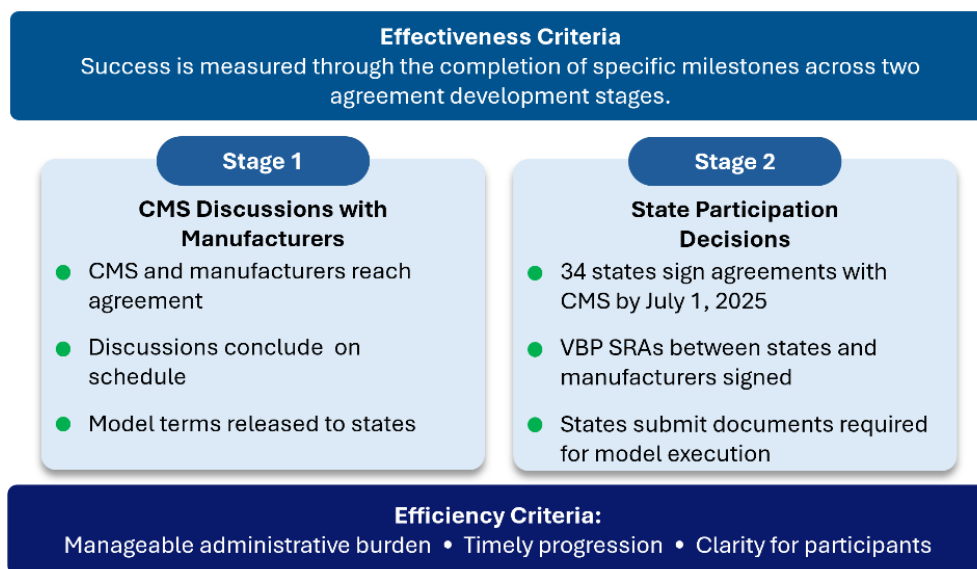
Evaluation Overview

This first evaluation report describes how the CMS-led process to establish agreed-upon terms functioned and assesses whether CMS discussions with manufacturers are an efficient and effective path to OBAs between manufacturers and multiple states. This report includes the perspectives of CMS, manufacturers, and SMAs. Future evaluation reports will explore model implementation and model impacts on CGT access, health outcomes, and state finances.

Approach to Evaluating the Agreement-Development Process

To conduct this evaluation, Abt reviewed CMS materials describing the model and interviewed CMS, manufacturers, and SMAs after model-established terms were set, and states had made participation decisions. The evaluation team coded interview transcripts using NVivo® qualitative analysis software using a set of predetermined codes based on the interview questions. The evaluation team identified emerging themes, refined and agreed on secondary codes, and applied thematic queries to assess patterns across responses.

Based on this analysis of stakeholder interviews, the evaluation team conceptualized the negotiation process as two sequential stages with specific effectiveness criteria: discussions with manufacturers and state participation decisions (see **Exhibit 3**). The evaluation defines efficiency as a component of effectiveness within each stage. To be efficient, a negotiation process stage must be executed with manageable administrative burden, timely progression through the stage, and reasonable clarity for participants.

Exhibit 3: The Evaluation Assesses Efficiency and Effectiveness

Key: CMS - Centers for Medicare & Medicaid Services; VBP SRA - value-based payment supplemental rebate agreement

Stage 1: CMS discussions with manufacturers assesses how CMS balanced competing priorities among stakeholders, maintained momentum, and produced key terms that define rebate amounts and other central parameters that form the basis for SRAs between manufacturers and SMAs.

Stage 2: State participation decisions examines how states viewed model-established terms and weighed their options for model participation.

To assess the effectiveness of the CMS-led process to establish key terms that apply to all participating SMAs, the evaluation considers efficiency at each stage, determines whether stage-specific criteria for effectiveness were met, and then examines how the two stages together contributed to overall effectiveness. The evaluation explores the factors that enabled or hindered the CMS-led agreement development process in each stage and explores the sometimes competing aims that participants in each stage brought to the process.

Limitations

This evaluation has several limitations. Findings reflect the perspectives of a limited number of interviewed SMAs (10 SMAs represented by 18 officials) and are not generalizable to all SMAs. Interview findings may also be affected by variation in how candid key informants—from CMS, manufacturers, and SMAs—were willing to be with the evaluation team. In addition, respecting the proprietary nature of the model-established terms, the evaluation team limited document review to redacted request for applications (RFA) responses provided by CMS and publicly available information. Appendix A details key informant characteristics and provides detail on data collection and analysis methods.

3. Stage 1: CMS Discussions with Manufacturers of CGT for SCD

Section summary: This chapter examines the first stage of the agreement development process—discussions with manufacturers and how CMS balanced competing priorities to arrive at mutually agreeable terms. The chapter describes manufacturer impressions of the discussions and how CMS and manufacturers negotiated key terms. Manufacturers participated because they wanted to reach all SCD patients, including those with Medicaid coverage, and negotiating individually with each SMA would have required more resources. Manufacturers needed to modify their standard contracting processes to participate in the model, but they did not find these changes to be barriers to participation. Key terms in the participation agreements CMS discussed with both manufacturers included rebates, fertility preservation services, an access policy, and coverage criteria.

How to read this section: Key findings are written in bold text. The text under each key finding provides details that explain or describe the key finding. **Exhibit 5** defines the key terms that were eligible for discussion in developing the participation agreements with manufacturers.

CMS Developed an Appealing Manufacturer RFA

CMS engaged a diverse set of stakeholders to refine the CGT Access Model design and identify potential challenges prior to and during discussions with manufacturers. CMS sought input from manufacturers, state Medicaid officials, clinicians, and SCD patient and community representatives to inform the RFA and discussion parameters.

In March 2024, CMS issued a manufacturer RFA to formally request manufacturer participation in the model and establish a framework for collaborative discussion, rather than fixed agreement terms. In the RFA, CMS defined core elements of the model — including the overall payment approach and access expectations — while intentionally leaving specific terms to be finalized through collaborative discussion. This approach enabled CMS to align model launch with market entry of the therapies, while allowing flexibility to resolve key issues through subsequent discussions with manufacturers. Two manufacturers responded to the RFA by the May 2024 deadline.

Effectiveness of Discussions with Manufacturers

In this stage of the agreement development process, CMS engaged each of the two manufacturers of FDA-approved CGT drugs for SCD in discussions of key terms. Success at this stage is achieved when CMS is satisfied with the terms that manufacturers agree to include in participation agreements with CMS to join the CGT Access Model. The evaluation applied three criteria to determine effectiveness at this stage:

1. CMS and manufacturers reach agreement on model terms.
2. Discussions conclude on schedule.
3. CMS releases the model terms for SMA consideration.

Based on these three criteria, **Stage 1: Discussions with Manufacturers was effective**. CMS and both manufacturers signed participation agreements in late 2024, following approximately nine months of discussions. The discussions concluded on schedule. In December 2024, CMS released the model key terms to SMAs for their consideration. The sections below provide details on manufacturers' experience of the discussions and the key terms.

CMS and Manufacturers Agreed on Key Terms

CMS and manufacturers established key terms by which SMAs pay for CGTs for SCD. The CMS-secured rebates will be applied in addition to existing statutory rebates required under the MDRP. Top CMS priorities in developing outcomes-based agreements with manufacturers were to:

- 1. Reduce CGT costs borne by SMAs:** Prior to the agreement development process, state Medicaid officials communicated their concerns to CMS regarding the cost of CGTs. These officials expressed the expectation that the federal government would be able to secure more favorable terms for CGTs than SMAs might obtain independently. The arranged guaranteed rebates, combined with the MDRP rebate, reduce the CGT net unit price for participating SMAs regardless of potential additional outcomes-based rebates.
- 2. Enhance patient access and maintain patient choice:** The two manufacturers provide products with significantly different initial pricing. CMS strove to minimize price variation between CGTs that could encourage SMAs to favor the less expensive CGT.

Exhibit 4 defines the types of key terms that were eligible for discussion, followed by a review of how CMS and manufacturers negotiated the key terms.

Exhibit 4. CMS Asked Manufacturers to Consider Different Types of Key Terms in their Response to the Request for Applications

Key Terms	Definition
Guaranteed rebates	Flat rebates paid to the SMA at the time of CGT administration, regardless of number of patients or treatment outcomes. These rebates are in addition to rebates required under the MDRP.
Outcomes-based rebates	Rebates paid to the SMA by the manufacturer if a model CGT fails to provide pre-determined clinical benefits to the patient.
Volume-based rebates	Rebates tied to volume targets. Manufacturers offer higher rebates if the proportion of eligible beneficiaries administered a drug meet or exceed certain thresholds.
Rebate reporting and calculations	Data sources and calculations used to track and validate when an outcomes-based rebate is triggered and is refunded to the SMA.
Fertility preservation services	A series of procedures to save and protect reproductive material before undergoing treatments, such as chemotherapy, which can pose a risk of infertility.

Key Terms	Definition
Access policy	An SMA's coverage policy for a model CGT, including utilization management approaches, such as prior authorization. The model requires access policies to use standardized elements, such as coverage criteria, across participating SMAs, though modifications are allowed to align with state laws.
Coverage criteria	Clinical criteria and demographic characteristics which define a beneficiary's eligibility to receive a model CGT.

Key: MDRP - Medicaid Drug Rebate Program; SMA - state Medicaid agency

CMS evaluated the interplay among rebate types to secure the most advantageous terms for SMAs. CMS observed that initial manufacturer rebate proposals did not align with their expectations. Both CMS and manufacturer representatives acknowledged that extensive discussion and compromise were required to finalize rebate agreements. CMS ultimately succeeded in securing participation agreements that encompassed rebates, fertility preservation services, access policies, and coverage criteria as essential components.

Participation Opportunity and Experience Generally Met Manufacturer Expectations

Manufacturers offered multiple reasons for their willingness to participate in discussions with CMS.

Conceptually, representatives from both manufacturers described their commitment to reaching as many SCD patients as possible with their new therapies and the opportunity to increase access to Medicaid beneficiaries as a clear way to meet that commitment. Manufacturer representatives also appreciated CMS' provision of a safe harbor for fertility preservation, as it enabled them to effectively address the specific needs of patients with SCD.

Practically, manufacturers were unsure whether individual SMAs would have the capacity to negotiate OBAs and expected that direct negotiation with individual SMAs would require a higher level of effort. In addition, manufacturers noted that reimbursements to qualified treatment centers, the transplant centers certified by manufacturers to administer their CGTs, have historically not been timely or adequately reimbursed for high-cost treatments. According to manufacturers, this has resulted in some centers not accepting Medicaid beneficiaries for certain treatments. Manufacturers viewed the model as ensuring that qualified treatment centers would receive prompt payment for the cost of CGT, which they believed removes a barrier for Medicaid patients to access CGT. Additionally, manufacturers viewed the guarantee of out-of-state treatment for states without a qualified treatment center as a positive element of the model.

Though manufacturers found discussions generally collegial, they expressed some concerns with the agreement development process. In negotiations with other parties, manufacturers typically know the size of the patient population that will be served. In discussions with CMS, manufacturers were initially skeptical of CMS estimates regarding the number of SMAs expected to join. Manufacturers would have preferred to know which SMAs would participate in the model to have a better estimate of the total number of beneficiaries receiving CGT through the model. In contrast, SMAs wanted to assess the key

terms of the agreements before deciding whether to join the model. Manufacturer uncertainty regarding which states would ultimately participate in the model introduced inefficiencies to the agreement development process by complicating financial forecasting. This uncertainty reflects a structural tension inherent in CMS-led discussions: manufacturers sought early commitment signals, while states preferred to evaluate finalized terms before committing. Ultimately, manufacturers were pleasantly surprised by the high state participation rate.

Manufacturers felt that CMS placed excessive emphasis on rebates during discussions, and deprioritized other issues. From the manufacturers' perspective, outcome-based rebate triggers and documentation requirements were just as important as rebates. However, CMS' priority was to reduce administrative burden on SMAs, including streamlined documentation requirements. This difference in priorities prolonged negotiations from the perspective of manufacturers but ultimately the negotiations proceeded along the original timeline. The discussion timeline remained in line with CMS goals, reflecting reasonable management of any misalignment experienced during the process.

Manufacturers adjusted their usual contracting processes to join the CGT Access Model but did not see these changes as obstacles. Manufacturers followed CMS rules for audits, retention, and documentation. When SMAs required customized VBP SRAs, manufacturers sometimes had to complete separate data use agreements or use state-specific contract templates, which was time-consuming. Manufacturers also ended all direct negotiations and existing agreements with SMAs that opted into the model, allowing the model's key terms to supersede any other agreements.

Rebates

CMS noted that manufacturers' initial rebate proposals did not align with CMS expectations.

Negotiating the rebates outlined in the model participation agreements entailed considerable time and required multiple compromises. Manufacturers indicated that discussions may have been more efficient had CMS articulated clearer parameters regarding rebate ranges and CMS priorities earlier in the process. CMS agreed that starting with tighter central parameters could have been more efficient but noted that setting parameters earlier might have changed the discussion and the rebates ultimately achieved. CMS conducted thorough reviews and modelling of various possible combinations of rebate components to reach a consensus with manufacturers on outcomes-based rebates that they believe offer more favorable terms than SMAs could individually negotiate with manufacturers.

Fertility Preservation Services

Both CMS and manufacturers agreed that access to fertility preservation services is important for patients receiving CGT. Including manufacturer payment for fertility preservation services made CGT a more practical option for patients concerned about fertility risks and high out-of-pocket costs. The safe harbor provision offered by CMS to allow this payment strongly motivated manufacturers to participate. While both parties were aligned on the need for fertility preservation early in discussions, finalizing the details required effort during the agreement development process.

Access Policy

CMS required all participating SMAs to adopt a standard access policy to ensure consistent access to CGT. The access policy guarantees Medicaid

beneficiaries fair access to CGT treatment regardless of where they live, whether they are enrolled in Medicaid fee-for-service or managed care, and whether their state has a qualified treatment center. CMS and manufacturers reported few tradeoffs in agreeing to access policy terms, and manufacturers were satisfied with this aspect of the model.

Coverage Criteria

CMS based coverage criteria on clinical trial data and FDA labels. These criteria directly impact the size of the eligible patient population for CGT, shaping both the market opportunity for manufacturers and potential expenditures for CMS and SMAs. As permitted, several SMAs further defined coverage criteria in their VBP SRAs with manufacturers, in line with clinical standards. Manufacturers allowed these state clarifications if they did not unnecessarily restrict patient eligibility.

Stage 1 Assessment Summary

Effectiveness in Stage 1 is judged by whether CMS and manufacturers reached agreement, on schedule, so that model terms can be released to SMAs. In Stage 1, CMS successfully reached agreements with manufacturers within a pre-determined timeline, allowing the release of model terms to SMAs. **The agreement development discussions thus met the criteria for effectiveness at this stage.**

Safe Harbor Provision: Waiver of the Anti-kickback Statute

CGT manufacturers routinely provide payment to commercially insured patients for certain fertility preservation services not already covered by their health care coverage payor. However, federal anti-kickback statutes prohibit offering anything of value to Medicaid patients in exchange for their use of services. CMS sponsored a safe harbor provision for CGT manufacturers and SMAs that participate in the model. The safe harbor provision protects them from liability for violations of federal anti-kickback laws. The safe harbor does not extend to states that chose not to participate in the model.

Source: Centers for Medicare & Medicaid Services. (2024). Cell and Gene Therapy Access Model: Request for Applications for States
<https://www.cms.gov/files/document/cgt-model-rfa.pdf>

4. Stage 2: State Medicaid Agency Participation in the CGT Access Model

Section summary: This chapter outlines the second stage in developing the CGT Access Model – key factors that shaped SMA participation decisions, SMA views on how well model-established key terms met state needs, and changes SMAs made to implement the model. Most SMAs – representing 34 states^{xvi} – chose to participate in the model. Collectively, they cover 84% of Medicaid patients with SCD. SMAs considered multiple factors in deciding whether to join the model, including the high cost of CGTs, their ability to negotiate with manufacturers independently, the value of model agreements relative to state needs, their perception of costs, benefits, and barriers to model participation, and their interest in supporting CMS-led discussions with manufacturers. SMAs that joined the model generally found the model-established terms aligned with their needs, while noting opportunities for refinement. Implementing the model required targeted policy and operational adjustments at the state level.

How to read this section: Key findings are written in bold text. The text under each key finding provides details, examples, and quotations that explain or describe the key finding. **Exhibit 5** shows characteristics of states participating in the model, e.g., the size of the state’s Medicaid population with SCD and infrastructure for CGT treatment. **Exhibit 6** depicts state participation, SCD patient density and qualified treatment center locations on a map of the United States.

Effectiveness of Securing State Medicaid Agency Participation

The second stage of the CMS-led agreement development consists of SMA decisions about whether to join the model. Success in this stage is defined by a reasonable number of states and territories deciding to join the CGT Access Model. The evaluation applied three criteria to determine effectiveness at this stage:

1. SMAs sign a state agreement with CMS by July 1, 2025.
2. SMAs establish a VBP SRA with at least one CGT manufacturer.
3. SMAs submit required documentation to CMS to start model implementation by January 1, 2026.

After reviewing the key terms that CMS agreed on with manufacturers, 34 SMAs joined the model. This high uptake demonstrates that CMS-led discussions with manufacturers produced sufficiently attractive key terms for SMAs. Participating SMAs went on to establish VBP SRAs with manufacturers and submit needed documentation to CMS. Both manufacturers and SMAs noted this process was more complex and required more time and effort than for a typical SRA. SMAs then executed their VBP SRAs by the end of 2025 and began participation in the CGT Model at a date of their choosing, no later than January 1, 2026.⁸ **Based on these three criteria, SMA participation in the CGT Access Model was effective.**

⁸ CMS approved later model start dates for a small number of states with extenuating circumstances.

The sections below describe which SMAs are participating in the CGT Access Model, the process and factors states used in deciding whether to participate in the model, how SMAs assessed model-established terms, and early implementation steps taken by participating SMAs.

Most Medicaid Agencies Chose to Participate in the CGT Access Model

The rising cost of drugs — especially high-cost specialty treatments like CGTs — puts significant pressure on state Medicaid budgets. State Medicaid agencies deliberated among four options that determine the price they pay for CGTs for SCD: 1) pay for CGTs as a separate line item; 2) accept only the standard MDRP rebate; 3) negotiate SRAs directly with one or both CGT manufacturers, or 4) participate in the CGT Access Model and accept its CMS-secured rebates in addition to the MDRP rebate.

After considering their options, 34 SMAs chose to participate in the CGT Access Model. Collectively, these SMAs cover roughly 84% of Medicaid beneficiaries with SCD nationwide. **Exhibit 5** explores how participation in the CGT Access Model differs between states in relation to the number of Medicaid beneficiaries with SCD living in the state, the percent of the nationwide Medicaid population affected by SCD in each state, and the presence, absence, and quantity of centers in the state that are qualified by the Casgevy and Lyfgenia manufacturers to administer these treatments.

Exhibit 5. Select Characteristics of Participating and Non-Participating States

State/Territory Characteristics	Total Number of Eligible SMAs	Number of Participating SMAs	Number of non-Participating SMAs
Number of Medicaid beneficiaries with SCD			
≤ 200	17	6	11
201 – 1,000	16	12	4
> 1,000	19	16	3
Share of national Medicaid population with SCD			
< 1%	28	14	14
1% – 2.99%	10	7	3
3% – 4.99%	10	10	0
≥ 5%	4	3	1
Qualified treatment center presence			
No CGT qualified treatment centers	25	11	14
1 CGT qualified treatment center	10	9	1
2 or more CGT qualified treatment centers	17	14	3
CGT Access Model Totals			
States and territories eligible to participate (Medicaid population with SCD)	52 (100%)	34 (84%)	18 (16%)

Key: CGT - cell and gene therapy; SCD - sickle cell disease; SMA - state Medicaid agency

Data Sources: Abt analysis of claims for Medicaid beneficiaries 12 years old and older with SCD. Data from CMS, Transformed Medicaid Statistical Information System (T-MSIS) Analytic Files, 2019-2023, retrieved June 18, 2025. Abt review of Qualified Treatment Centers authorized to provide treatment with Casgevy and/or Lyfgenia, as listed on manufacturer websites, retrieved May 2025.

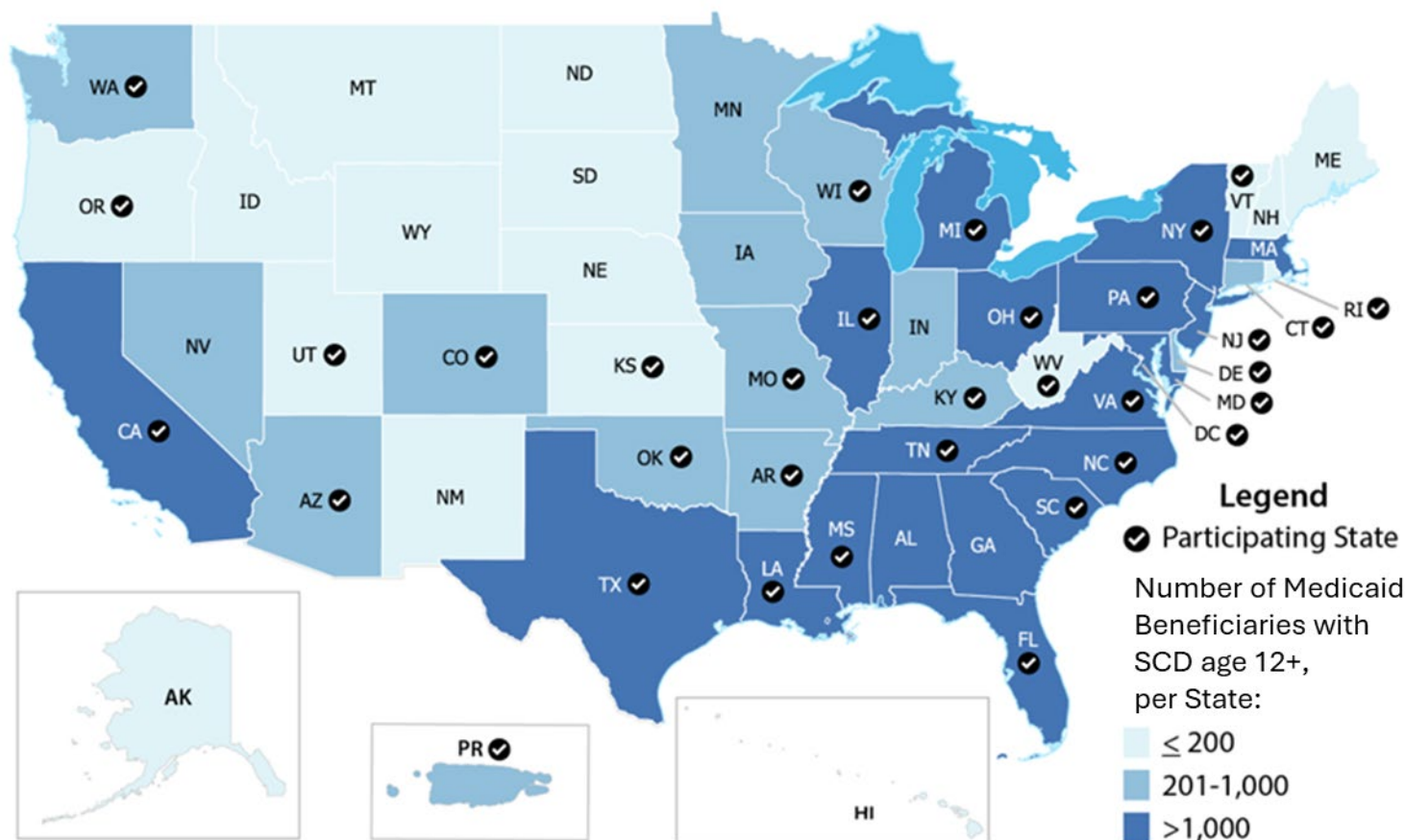
Note: Qualified treatment center counts are fluid, and data presented here reflect a point-in-time count of the centers around the time of CMS-manufacturer discussions and state decisions to participate in the CGT Access Model. Counts are subject to change as centers gain or lose their qualifying status with drug manufacturers.

State participation in the CGT Access Model is associated with the level of disease burden and treatment readiness. States with larger Medicaid population with SCD make up the largest proportion of participating states. Of SMAs serving more than 1,000 individuals with SCD, 84% joined the model (16 of 19 SMAs). In contrast, only 35% of SMAs with fewer than 200 individuals with SCD opted to join (6 of 17 SMAs). Participation is also higher among states representing a larger share of the national Medicaid population with SCD. Among SMAs accounting for 3% or more of the national Medicaid SCD population, 93% enrolled in the model (13 of 14 SMAs). In contrast, only half of SMAs accounting for less than 1% of the national Medicaid SCD population enrolled (14 of 28 SMAs).

Treatment infrastructure is also associated with state participation. Among states with at least one CGT qualified treatment center, 85% joined the model (23 of 27 SMAs), compared to only 44% participation among states without a qualified treatment center (11 of 25 SMAs).

The geographic distribution of state participation further reinforces these patterns. **Exhibit 6** shows that model participation is concentrated in areas with the larger Medicaid SCD populations (more than 1,000 individuals per state). In contrast, many non-participating states are in lower-density areas, including the Mountain West and Great Plains, where beneficiary counts are smaller and access to CGT qualified treatment centers is more limited.

Exhibit 6. State Medicaid Agency Participation and SCD Patient Density



Key: SCD – sickle cell disease

Notes: a. Map is not to scale. b. Geographic coordinate system = North American Datum (NAD) 1983.

Data Sources: Abt analysis of claims of Medicaid beneficiaries 12 years old and older with SCD. Data from CMS, Transformed Medicaid Statistical Information System (T-MSIS) Analytic Files, 2019-2023, retrieved June 18, 2025. Geographic boundary files (shapefiles): States from U.S. Census 2024 TIGER/Line Shapefiles: States (and equivalent), retrieved May 28, 2025.

State Medicaid Agencies Used Staff Expertise, Data Analysis, and Terms Review to Inform Model Participation

Given the CGT Access Model introduces a new payment approach for high-cost drugs, SMAs requested detailed information to understand its advantages and rebate arrangements before deciding whether to participate. SMAs drew on internal expertise, data analysis, and systematic reviews of model-established terms to guide their decisions.

Use of Agency Staff Expertise

Technical experts at SMAs informed participation decisions. While decisions were ultimately made by top-level Medicaid agency staff (such as Medicaid Directors), participating and non-participating SMAs consulted with Medicaid staff within their agencies as they reviewed the CMS-secured terms. These consultations included pharmacy and legal department staff, staff involved with rebates and prior authorizations, and clinicians.

No SMA interviewed for the evaluation sought input from external groups or the public when deciding whether to participate. One state noted that state legislative priorities and a sister agency's SCD initiatives influenced their decision, but they did not engage the public directly. Another SMA simply informed the public about their participation through press releases. As one Medicaid official explained: *"This was a yes or no decision, so we did not talk with [external] stakeholders."*

Analysis of State Medicaid Data

Half of the interviewed SMAs reviewed their own Medicaid data to inform participation decisions. Both participating and non-participating SMAs reported that they analyzed their own Medicaid claims data to assess the financial impact of SCD care with and without the CGT Access Model. Some SMAs worked with external vendors or academic partners to conduct these analyses, and some did this analysis internally.

Comprehensive Review of Model-Established Terms

Some states conducted detailed reviews of the model-established terms as part of their decision-making process. To assess whether to participate and whether to sign agreements with one or both CGT manufacturers, several SMAs reported comparing the two model-established agreements with one another. In addition, SMAs that had themselves initiated or completed negotiations with the manufacturers of CGTs for SCD compared the CMS-led agreements against their own state-negotiated terms. Finally, some SMAs used advanced tools, including predictive models, to assess the overall value and risk protection offered by each CMS-led agreement.

State Medicaid Agencies Considered Multiple Factors in Deciding Whether to Join the CGT Access Model

State Medicaid agencies weighed several factors when evaluating whether to join the model, including the high cost of CGTs for SCD, state Medicaid SCD population size, state's ability to independently

negotiate with manufacturers and potentially secure better terms, the expected costs and benefits of model participation, perceived legal barriers to model participation, and their interest in signaling support for future CMS-led agreements with manufacturers. Ultimately, states weighed multiple factors rather than making decisions based on a single overriding consideration and reported that they made decisions based on what was best for their state.

High Cost of CGTs for SCD

Most states acknowledged concern over the high cost of CGTs for SCD. High cost was a particularly important factor for states with large SCD populations. These states anticipated that the number of beneficiaries receiving CGTs could significantly affect overall Medicaid spending.

Some SMAs expressed interest in outcomes-based rebates as a way to protect their budgets if the treatment failed. Although they did not expect significant savings because they anticipated CGTs would perform as demonstrated in clinical trials, they still sought financial protection against the risk of failure. As one Medicaid official explained, outcomes-based rebates functioned like an insurance policy:

“Value-based contracting is never going to be the thing that takes your spend from this trajectory to that trajectory. It's protection. It's what allows us ... to sleep at night and to say, we're going to pay that, and if it works, we're good. And if it doesn't, I'm going to get my state's money back. That's why you sign value-based contracts. They're never going to be this huge moneymaker.”

- Non-participating state Medicaid official

State Medicaid SCD Population Size

The size of a state's Medicaid population with SCD was an important consideration in participation decisions. Both SMA interview and state participation data as shown in **Exhibit 5** suggest the state Medicaid population with SCD was associated with state participation decisions. First, states with larger SCD populations faced greater potential spending on CGTs and therefore had more at stake financially. Second, larger Medicaid populations with SCD gave states greater leverage to negotiate directly with CGT manufacturers. Finally, states with larger SCD populations were more likely to view the administrative effort required for model participation as worthwhile given the potential savings and financial protections secured through the model-established terms. In contrast, states with smaller SCD populations faced lower financial exposure. One interviewed state with small SCD population explicitly stated that the administrative effort required for participation outweighed the expected benefits.

Ability to Independently Negotiate with Manufacturers

State Medicaid agencies could choose to negotiate directly with either or both CGT manufacturers.

Four interviewed SMAs determined that CMS-led agreements offered higher rebates than those achievable through independent negotiations. Of these, two SMAs had received manufacturer offers during independent negotiations but had not reached final agreements.

Two interviewed SMAs declined to join the CMS model after securing independent agreements that they believe provided higher rebates or better protection against failed outcomes. SMAs noted these

deals were reached faster and allowed more flexibility, as they could be renegotiated or terminated at any time.

An SMA's skills and resources as well as their interest in independently negotiating with manufacturers appears to be driven by three elements: (1) the agency's prior experience securing value-based contracts, (2) the size of the state Medicaid population with SCD and (3) the resources available to the agency to negotiate a value-based contract (VBC).

State Medicaid agencies' prior experience with VBCs shaped their willingness to negotiate with CGT manufacturers. Several SMAs with prior value-based contracts for drugs other than CGT pursued independent negotiations. Some of them reached agreements. For others, positive past experiences with VBCs supported their decision to join the model. For a third group, prior VBC negotiations and implementation had been administratively burdensome, leading them to prefer participating in the CGT Access Model to avoid repeating that workload.

Larger SCD populations gave SMAs leverage to negotiate directly with manufacturers. Five of six interviewed SMAs that negotiated independently had at least 500 Medicaid beneficiaries with SCD in their states. As one non-participating state noted: *"We had enough population that for this conversation we had real negotiating strength."*

Still, not all SMAs with large SCD populations reached agreements with manufacturers on their own. Officials acknowledged that CMS had an even greater population size leverage by pooling Medicaid beneficiaries with SCD across many states.

"The fact that CMS was going to ... leverage all the states, it really felt like they would come out with something far, far better than we would have been able to do on our own."

- Participating state Medicaid official

Negotiating VBCs requires significant resources. States described needing staff time, expertise and resources to analyze their SCD population's health care utilization and understand how potential health outcomes would present in claims data, compare different scenarios under negotiation, conduct negotiations, and perform a legal review of draft contracts. Some states believed they did not have the same level of negotiating resources as the CGT manufacturers.

State's Perception of Costs and Benefits of Model Participation

Some interviewed SMAs weighed the anticipated benefits of model participation against the administrative effort required to operationalize the model.

Operationalizing an OBA requires significantly more administrative effort by the SMAs than other payment arrangements. Although CMS assumes some of the reporting and dispute resolution activities, SMAs must collect and monitor outcomes data to manage outcomes-based rebates, and they must ensure they have a dispute resolution process in place to handle disagreements when outcomes are not met.

The size of a state's SCD population and its administrative capacity affect how it weighs the costs and benefits of joining the model. States with larger SCD populations can gain more from CMS sharing the responsibilities related to rebate tracking, reporting, and dispute resolution. Therefore, interviewed states with significant SCD populations decided that participating in the model was beneficial. Conversely, one interviewed state with a small SCD population (fewer than 500 beneficiaries with SCD) and limited staff determined that managing outcomes tracking, reporting, and finances required more effort than the expected savings over standard MDRP rebates. As **Exhibit 5** shows, six states with the smallest SCD populations (fewer than 200 beneficiaries with SCD) did join the model, which suggests that state participation decisions reflecting SCD population size were not universal.

State Legal Considerations

Two states did not join the model due to perceived statutory barriers and other legal concerns. One interviewed SMA interested in model participation erroneously thought that the model required carving out CGTs from managed care contracts. This SMA lacked statutory authority for a carve out and so did not join the model. Another state considered signing onto the model for one of the CGTs and executing its own, independently negotiated agreement for the other CGT. Without access to the safe harbor available under the CMS-led agreement, the state-negotiated agreement does not provide patient access to manufacturer-funded fertility preservation services. The state's concern about potential legal risk posed by differential access to fertility preservation services led to the state's decision not to join the model.

Signaling Support for CMS-Led Discussions with Manufacturers

Most states wanted to signal support for CMS leading discussions with manufacturers. SMAs noted that states face growing pharmaceutical expenditures for high-cost, high-demand drugs in general, and viewed CMS-led pricing discussions as a way to address these pressures. They supported CMS testing both the CMS-led agreement development and administration of an OBA through the CGT Access Model. Participating states noted that CMS leverage in discussions with manufacturers would increase as more SMAs participate in the model and indicated that they joined the model not only for the benefit of their state, but also to help other states. They also hoped their participation would encourage CMS to pursue contracts for other drugs, therapies, and health conditions on behalf of all states.

"So, we knew that participating in the model would send a message to CMS to encourage CMS to pursue these types of models, the second phase of the model in the future and to negotiate contracts on behalf of states for future cell and gene therapies."

- Participating state Medicaid official

States viewed the CGT Access Model as having the potential to *"become a new standard"* for securing VBCs with drug manufacturers. One state expressed concern that if too few states joined the model, CMS would discontinue discussions with manufacturers to secure rebates for SMAs.

CMS-Led Agreements Met Most State Needs, but Medicaid Agencies Outlined Concerns

States offered insights on key model terms and how well those aligned with state needs:

The model-established terms met the needs of most SMAs. Most participating SMAs interviewed for the evaluation reported satisfaction with the model and indicated that the CMS-led agreements met their state's needs. A few SMAs canceled independently negotiated agreements in order to join the model, indicating that CMS-led agreements better met the state's needs than agreements those SMAs secured on their own. In addition, CMS reported that very few states requested formal variances to the model-established terms to comport with state laws, further indicating that the terms met state needs.

A few SMAs identified shortcomings of OBAs. States generally valued the OBA as a mechanism to protect against financial risk in the event of treatment failure but did not expect it to substantially lower CGT treatment costs. Instead, states placed greater value on other types of rebates that addressed the immediate financial burden of high-cost therapies like CGTs.

States cared most about agreement terms related to cost. Satisfaction with the model-established terms was shaped by individual agency preferences for rebate types. States shared concerns about the administrative and staff burden of reporting, calculating, and documenting outcomes-based rebates.

Many states were pleased with the fertility preservation services and saw them as a value add for patients. Both participating and non-participating SMAs praised the CMS-sponsored model safe harbor provision for these services and noted this was an attractive feature of the model.

State Medicaid Agencies Made Changes to VBP SRAs and Operations to Participate in the CGT Access Model

To participate in the model, SMAs needed to revise agreements and make operational changes, which presented some challenges.

To participate in the CGT Access Model, SMAs either opted to use their state-specific VBP SRA template or the model template. Some SMAs chose to use a state-specific contract template approved by CMS as the basis for their agreement with manufacturers instead of the VBP SRA template created by CMS. This choice helped SMAs align with state laws and policies but required additional effort for both SMAs and manufacturers to ensure agreements aligned with model-established key terms. SMAs worked with manufacturers to modify VBP SRAs, including amending prior authorization and coverage criteria, without making agreements more restrictive than the model-established terms.

State Medicaid agencies made operational changes to participate in the model. Managed care organizations (MCOs) are key partners in delivering CGT and related care to SCD patients. SMAs amended MCO contracts as needed to join the model. A few states mentioned that the burden of model launch increases with the number of MCOs operating in the state, since each MCO's contract must be renegotiated. Topics that SMAs discussed with MCOs included how to deliver treatment, care coordination for people with SCD and restrictions on 340B pricing for CGT. Many states also had to submit a state plan amendment to change how they reimburse the drugs in order to collect rebates

States reported this was resource-intensive and challenging for meeting model timelines. Some SMAs carved CGTs out from standard care bundles for separate reimbursement and obtained a state plan amendment to support participation in value-based purchasing agreements. One participating state needed to amend a state statute to take advantage of the fertility preservation services offered to patients through the model.

Some SMAs found the complexity of setting up an OBA challenging, especially given their experience with simpler SRAs. State expectations of the burden involved with the model were grounded in their prior SRA experience or a direct agreement with the manufacturer, which have fewer requirements. States reported that setting up an OBA was more complicated than anticipated. CMS and manufacturers also acknowledged the complexity of this OBA.

SMAs with prior VBCs reported facing fewer start-up requirements compared to agencies that did not have prior VBCs. States with prior VBCs stated that they already had some needed policies, systems and processes set up, which made it easier for them to integrate the model requirements. Some had experience carving drugs out from MCO contracts or had other payment mechanisms in place that allowed them to participate without establishing new processes and would thus be able to pay for CGTs for SCD without establishing new processes. One SMA had previously established reimbursement procedures with their MCOs for several CGTs, including the two CGT drugs for SCD. As one participating state official put it, *“Because we had had that [VBC] experience before, it wasn’t overwhelming for us to participate in this model.”*

Some SMAs found the timeline and some CMS-required documentation to enter the model administratively burdensome and unnecessarily complex. States described challenges submitting required documentation due to their capacity and time limitations. Some SMAs expressed frustration with duplicative paperwork, such as submitting a contract template that CMS previously approved. When operational changes required time to process (e.g., obtaining a state plan amendment or completing legislative processes) the deadlines set by CMS became more challenging for SMAs to meet.

States appreciated CMS flexibility and support during model onboarding. For example, they acknowledged the value of CMS accepting draft documentation and working with SMAs within their existing policy infrastructure, which reduced the administrative burden of aligning with model requirements. States and manufacturers anticipated that future models would require less effort thanks to the groundwork they established to participate in this model.

Stage 2 Assessment Summary

The evaluation judged efficiency by administrative burden, timeliness, and clarity for all participants. SMAs invested administrative resources into assessing the model key terms and making the decision about whether to participate in the model. Both manufacturers and SMAs noted that reaching agreement on VBP SRAs was more complex than a typical SRA. Finally, some SMAs expressed surprise at the level of documentation required by CMS.

Effectiveness in Stage 2 is judged by whether SMAs sign participation agreements with CMS and VBP SRAs with manufacturers, then submit required documentation for model participation to CMS. Despite

some challenges with burden, timeliness, and clarity, 34 SMAs signed state participation agreements with CMS and executed VBP SRAs with manufacturers. These SMAs also began submitting required documents to CMS to prepare for model implementation. State participation decisions in Stage 2 and subsequent adoption of the model by 34 SMAs – that collectively cover about 84% of Medicaid beneficiaries with SCD nationwide – are revealing indicators of the effectiveness of CMS-led agreement development. SMAs found the CMS-secured agreements terms sufficiently valuable, implementable and administratively manageable across diverse state contexts.

At this stage, SMAs identified areas for efficiency improvements, but CMS-led agreement development discussions met the evaluation criteria for effectiveness.

5. Assessment of the CMS-Led Agreement Development Process for the CGT Access Model

Section summary: This chapter synthesizes findings across both stages of the CMS-led agreement development process to describe the efficiency and effectiveness of the CMS-led approach to developing OBAs with drug manufacturers in the CGT Access Model. Stakeholders with competing interests and priorities offered insights into this CMS-led process. The participation of both manufacturers and a majority of SMAs demonstrates that the CMS-led process was efficient and effective.

How to read this section: Key findings are written in bold text. The text under each key finding provides details that explain the key findings. **Exhibit 7** summarizes the assessment of the two-stage process.

CMS successfully interacted with both manufacturers and states to achieve OBAs that appealed to manufacturers and the majority of SMAs. Throughout the process, key insights emerged about the benefits and potential challenges of CMS-led discussions with manufacturers to benefit SMAs. Ultimately, an assessment of the CGT Access Model finds CMS-led agreement development can be both efficient and effective. The model demonstrated that CMS-led agreement development can be an efficient alternative to state-by-state negotiations for high-cost CGTs with sufficient attention to reducing administrative burden, timely advancement through stages, and clear communication.

Insights from CMS-Led Discussions with Drug Manufacturers

Participating and non-participating SMAs, manufacturers, and CMS brought competing interests and priorities to the entire model agreement development process. In developing the key terms with manufacturers, CMS accommodated state preferences to the extent possible. The different parties identified several insights from the novel approach of CMS leading discussions to secure rebates available to SMAs.

Pooling beneficiaries across states gave CMS leverage. SMAs, regardless of whether they participated in the model, widely recognized that CMS had significantly more influence in discussions with drug manufacturers compared to individual states, since CMS represented more beneficiaries than any single state.

For manufacturers, uncertainty about which states would ultimately join the model made it challenging to forecast financial outcomes given the variation in the size of the SCD population across states. Manufacturers indicated that having SMAs commit to participation prior to manufacturer discussions with CMS would help mitigate this risk. In contrast, SMAs wanted to know the terms of the agreements between CMS and manufacturers before deciding whether to join the model. Some SMAs suggested that CMS and SMAs could have agreed on parameters for key terms that would broadly meet state needs before CMS initiated discussions with manufacturers.

CMS brought negotiation capabilities, capacity, and resources that are often limited at the state level. Several participating and non-participating SMAs with varying levels of resource capacity emphasized

the high effort and level of resources involved in independently negotiating an OBA. In addition, some SMAs cited agency challenges including limited negotiation experience and skills, inadequate staffing, and lack of resources as barriers to negotiating directly with manufacturers.

CMS shares the administrative burden of collecting outcomes data for rebates and making rebate determinations in the model. Some SMAs appreciated CMS taking responsibility for aggregating data for outcomes-based rebates, rather than tasking this responsibility to SMAs. In addition, one SMA felt reassured by their interpretation that CMS could act as a third-party intermediary in a failed outcome dispute between a SMA and manufacturer, reducing pressure on the SMA to resolve potentially contentious issues around the access policy, outcomes, or documentation. CMS noted that this centralized approach could also benefit manufacturers by consolidating rebate calculations across states.

The model offers fertility preservation services that require federal authority. One participating SMA underlined the advantage that CMS has in sponsoring a fertility preservation safe harbor.^{xvii} Without the model, manufacturers generally lack the authority to pay for these services, and patients may be less likely to pursue CGT without access to a fertility preservation option. CMS noted that creating the CMS-sponsored model safe harbor^{9,xviii} to require manufacturers to pay for fertility preservation services was also a significant motivator for manufacturers.

The model offers reimbursement assurances to the qualified treatment centers that provide CGT. Qualified treatment centers incur significant upfront costs when providing CGT and are often concerned about lengthy reimbursement timelines from SMAs. When patients travel out of state, qualified treatment centers must navigate the patient's home state Medicaid credentialing and billing processes, further slowing reimbursement. The model's access policy standardizes reimbursement procedures, which manufacturers noted gives qualified treatment centers greater assurance of timely Medicaid reimbursement, particularly for services provided to out-of-state patients.

CMS, manufacturers, and SMAs characterized the CMS-led agreements as complex. Manufacturers observed that both the manufacturer RFA and the participation agreements were lengthy and highly detailed. Several SMAs indicated that the VBP SRAs, particularly those involving outcome measurement requirements, were more complex than SRAs commonly negotiated by state purchasing pools. These SMAs expressed a preference for agreements that are straightforward and easier to implement. CMS also recognized the challenges SMAs encountered when developing direct reimbursement methodologies and coordinating with MCOs due to this complexity.

The model's voluntary participation structure allows SMAs to independently negotiate with manufacturers. CMS-led agreement on model key terms eliminated the burden of conducting negotiations for SMAs that choose to accept the CMS-led agreements, while preserving flexibility for SMAs to negotiate independently. SMAs who reported that they negotiated better overall agreements or terms better tailored to their SCD population chose not to join the model.

⁹ The federal rules and regulations on fertility kickbacks are primarily governed by the Anti-Kickback Statute and the Beneficiary Inducement Statute. These statutes prohibit pharmaceutical companies from offering or paying, directly or indirectly, any remuneration to induce federal healthcare programs to purchase their drugs.

Effectiveness of the CMS-Led Agreement Development Process

The CGT Access Model is a novel approach through which CMS successfully secured OBAs with both manufacturers of high-cost CGTs for SCD that SMAs can choose to adopt.

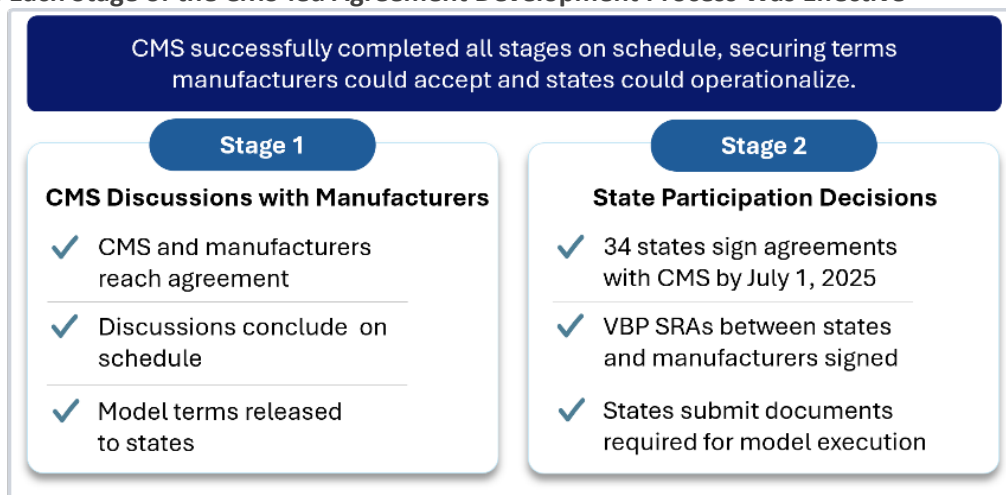
Effectiveness was evaluated across the two negotiation stages, with each stage contributing to the overall effectiveness of the model. As illustrated in **Exhibit 7**, the CMS-led process met milestones for success at each stage:

- Stage 1: CMS established key terms with manufacturers and released those model terms to SMAs for their consideration on schedule.
- Stage 2: Roughly two-thirds of states signed agreements with CMS to participate in the model, established VBP SRAs with manufacturers, and submitted required onboarding documentation.

Efficiency was assessed by reviewing the administrative burden on participants, timely progression of the negotiations process, and clarity of expectations and processes for participants in each state of the negotiations process. Manufacturers suggested negotiations were **less efficient** than they could have been if CMS had been more explicit about their expectations for rebates and other terms, but still **more efficient** than negotiating individually with each state. While some SMAs believed they could have negotiated simpler agreements directly with manufacturers, most found it **more efficient** for CMS to negotiate key terms on their behalf.

Ultimately, the Model-established key terms appealed sufficiently to both manufacturers and to 34 SMAs – covering 84% of Medicaid SCD patients – for them to participate in the CGT Access Model. This outcome demonstrates that CMS-led agreement development for high-cost CGTs was **effective**.

Exhibit 7: Each Stage of the CMS-led Agreement Development Process Was Effective



Key: CMS - Centers for Medicare & Medicaid Services; VBP SRA - value-based purchasing supplemental rebate agreement

Both manufacturers and SMAs expressed an openness to engage in CMS models for high-cost drugs in the future. Manufacturer and state interest in CMS-secured rebates is not limited to CGTs that could be part of the CGT Access Model. Instead, they offered a range of drug classes and diseases as candidates for CMS-led discussions with manufacturers that result in key terms available to SMAs, such as GLP-1s,

oncology drugs, and diabetes drugs. The CGT Access Model discussions with manufacturers and the resulting agreements between manufacturers and SMAs, coupled with interest from all parties in future CMS-led agreements, suggests future CMS-led establishment of key terms to pay for high-cost, potentially curative therapies may also be successful.

Appendix A. Methods

To produce this first CGT Access Model evaluation report, focused on discussions between CMS and manufacturers to agree upon key terms, the evaluation team used documents provided by CMS and qualitative data collected through listening sessions and key informant interviews. Portions of this evaluation report were drafted with assistance from Microsoft Copilot. All content was reviewed, edited, and approved by the report authors. This appendix lists research questions and describes data collection and analysis methods.

Research Questions and Framework

The overarching research question addressed by this evaluation report is, *‘Is it efficient and effective to have CMS negotiate with manufacturers on behalf of multiple states?’* To answer this overarching question, the team designed an evaluation framework grounded in a review of the literature on negotiations^{xix} to conceptualize the negotiation process and to systematically identify activities, decisions, and key informant perspectives. Key informants included CMS staff who participated in discussions with manufacturers, CGT manufacturer representatives, officials from SMAs that joined the CGT Access Model, and officials from SMAs that did not join the model. **Exhibit A1** displays the research questions specific to each key informant type as well as additional cross-cutting questions and the relevant report sections that address each group of questions.

Exhibit A1. Research Questions Addressed in the Negotiation Report

Key Informant Type	Research Sub-Questions	Relevant Report Sections
CMS Perspectives	1.1.1. What changes did CMS make to accomplish the negotiations? 1.1.2. What were the opportunity costs for CMS to undertake this role? 1.1.3. Were the appropriate CMS staff involved with the negotiations?	4.1, 4.2, 5.2
Manufacturer Perspectives	1.2.1. How did manufacturers’ expectations and prior experience factor into their willingness to participate? 1.2.2. What changes did manufacturers make to participate in or implement the OBA? 1.2.3. Would the manufacturers be interested in negotiating similar types of agreements in the future?	5.1, 7.1
State Perspectives	1.3.1. What factors did SMAs consider when deciding whether to participate in the Model? 1.3.2. What aspects of the Model did SMAs find beneficial, and what did they have concerns about? 1.3.3. To what extent did the SMAs perceive the OBAs met their needs, and did they have any concerns about the terms? 1.3.4. How did SMA expectations and prior experience factor into their willingness to participate? 1.3.5. Based on this experience, are SMAs interested in similar Models in the future?	6.1, 6.2, 6.3, 6.4, 6.5, 7.1, 7.2

Key Informant Type	Research Sub-Questions	Relevant Report Sections
	1.3.6. Among the states that did not participate in the Model, what factored into the decision?	
Cross-Cutting Questions	1.4.1. Did CMS-led negotiations result in a lower net unit price for CGT, including statutory and OBA-related rebates? 1.4.2. What elements were key to negotiations? 1.4.3. What aspects of the negotiation process could be improved? 1.4.4. Is leading negotiations best retained as a CMS function?	3, 5.2, 7.2, 8

Key: CMS - Centers for Medicare and Medicaid Services; OBA - outcomes-based agreement; SMA - state Medicaid agency

Note: The evaluation team does not have access to proprietary information about prices or rebate amounts.

Data Sources

The evaluation used both secondary and primary data sources. None of the data sources available to the evaluation team included proprietary details about rebate amounts.

Secondary Data Sources

The evaluation team reviewed CMS websites and documents describing the CGT Access Model and used Transformed Medicaid Statistical Information System (T-MSIS) data extracted June 18, 2025 to estimate the number of Medicaid beneficiaries with SCD in each state.

Document Review

The evaluation team reviewed model documents to capture substantive and contextual information relevant to the negotiation phase of the CGT Access Model (**Exhibit A2**). The team downloaded documents that are publicly available. In addition, CMS securely transferred copies of non-public documents to the evaluation team for review. CMS redacted all proprietary information before providing copies to the evaluation team. The team used the documents to inform primary data collection through listening session questions and key informant interview protocols.

Exhibit A2. Documents reviewed by the evaluation team

Documents Reviewed
• SMA letters of intent • RFA for manufacturers • Manufacturers' initial and final responses to RFA (redacted) • RFA for SMAs • SMAs' responses to RFA • NOFO for SMAs • Documents SMAs are required to submit for model participation

Key: SMA - state Medicaid agency; RFA - Request for Applications; NOFO - Notice of Funding Opportunity

Primary Data Sources

The evaluation team collected data through listening sessions and key informant interviews.

CMS Listening Sessions

The evaluation team facilitated a series of five informal listening sessions with the CMS Model Team Lead in June and July 2025. Through the listening sessions, the evaluation team learned about the negotiation process and critical model development activities.

Each listening session focused on a specific topic:

- **Session 1:** Understanding the negotiation process
- **Session 2:** External engagement process
- **Session 3:** Participation agreement evolution/state RFA development and review
- **Session 4:** Model background and development
- **Session 5:** Savings estimate, monitoring, and rebates

The evaluation team facilitated the listening sessions and took notes, then used the notes to inform development of key informant interview protocols.

Key Informant Interviews

The evaluation team conducted interviews with four types of key informants from August to October 2025: 1) CMS staff who participated in the negotiations; 2) manufacturer representatives; 3) officials from SMAs that joined the CGT Access Model; 4) officials from SMAs that did not join the model.

Sampling and Key Informant Selection

This section describes how the evaluation team selected key informants.

CMS Staff

The evaluation team conducted virtual, semi-structured individual and small group interviews with CMS staff, representing the Innovation Center and the Center for Medicaid and CHIP Services (CMCS), each of whom conducted, supported or approved the outcomes of discussions between CMS and manufacturers and/or assessed state RFA responses. The evaluation team selected key informants from a list provided by the CMS Model Team Lead and in consultation with the CMS Evaluation Lead. Individual staff participation in interviews was voluntary.

Manufacturer Representatives

The evaluation team conducted virtual, semi-structured small group interviews with representatives of manufacturers with FDA-approved CGT for SCD to gain their perspectives on the discussions held with CMS. The team identified a point of contact at each manufacturer in consultation with the CMS Evaluation Lead. The team then asked points of contact to recommend the most relevant staff at each manufacturer to participate in interviews. Manufacturers' participation in interviews was voluntary.

State Medicaid Officials

The evaluation team conducted virtual, semi-structured small-group interviews with representatives from ten SMAs. The team first selected states, then reached out to key decisionmakers in each state, using contact information provided by CMS or publicly available online.

The team used a purposive sampling approach for states called ‘maximum variation.’ This approach entails articulation of key factors that vary across states and may influence respondent perspectives, followed by intentional selection of states that vary across those key factors. Shared patterns that emerge across the resulting heterogeneous sample of states may therefore be considered more significant because they appear across multiple contexts or conditions.^{xx}

Given the primary research question and focus on the model-established terms, the team used two main criteria for sampling, augmented by additional factors to ensure variation among selected states, as shown in **Exhibit A3**.

Exhibit A3: Sampling Criteria

Main Criteria	Additional Factors to Ensure Maximum Variation
- Model participation/non-participation - Prior experience with VBCs (yes/no)^a	- Size of Medicaid-enrolled SCD population - Regional representation - Medicaid expansion status - Presence/absence of CGT qualified treatment site - Submission of a letter of intent to join the CGT Access Model followed by decision not to participate in the model

Key: SCD - sickle cell disease; CGT - cell and gene therapy; VBC - value-based contract

Note: ^a The evaluation team used state RFA responses and an environmental scan to identify this information. Not all information about negotiation experience is publicly available, and thus scan findings may be incomplete.

The evaluation team applied these criteria to initially select ten states comprising five participating and five non-participating states. The team also identified alternative participating and non-participating states in case of refusal or non-response from any of the ten selected states. Based on responses to invitations to participate in the evaluation, the team substituted one initially selected participating state and one initially selected non-participating state from the list of alternatives and interviewed an additional participating state in lieu of a non-participating state. The final list of SMA key informants includes six participating states and four non-participating states.

The evaluation team conducted online searches and requested referrals from CMS to identify state Medicaid officials in the selected states that could serve as points of contact for the interview invitations. The evaluation team invited Pharmacy Directors, Medical Directors, state Medicaid Directors, and any other staff who made substantial contributions to the decision-making process around participation in the model to participate in interviews. Evaluators conducted either individual interviews or small group interviews with up to four respondents, depending on the availability of Medicaid agency staff.

Interview Protocol Development

The evaluation team developed semi-structured interview protocols for each key informant type. To develop each protocol, evaluators reviewed the research questions and limited interview questions to new areas of inquiry not already addressed through the secondary data sources described in the previous section. **Exhibit A4** displays the topics asked about for each key informant type.

Exhibit A4: Topics included in interview protocols for each key informant type

Topic	CMS Staff	Manufacturer Representatives	Participating State Medicaid Officials	Non-participating State Medicaid Officials
Prior experience with negotiations	✓	✓	✓	✓
External engagement	✓		✓	✓
Key terms in participation agreements	✓	✓	✓	✓
Negotiation effectiveness and lessons for the future	✓	✓	✓	✓
Level of effort and preparation for negotiations	✓		✓	✓
Decision to participate in the model		✓	✓	✓
Overview of the process and reactions to RFA		✓		
Satisfaction	✓	✓	✓	✓

Key: CMS - Centers for Medicare and Medicaid Services; RFA - Request for Applications

The Abt Global Institutional Review Board (IRB) conducted a courtesy review of all study materials, including the interview protocols, data security plan, consent form, Frequently Asked Questions (FAQ) sheet, and outreach emails, to ensure compliance with Abt's ethics standards and maximum protection for interview respondents. The Abt Global IRB determined this evaluation to be exempt from IRB approval as it comprises research to evaluate a public benefit or service program.

Key Informant Interview Recruitment and Interview Scheduling

The evaluation team recruited key informants and conducted interviews from July to October 2025. The team sent initial outreach emails to each contact describing the evaluation and the purpose of the interview and included an FAQ attachment. In some cases, the evaluation team asked for interviews directly with the points of contact; in other cases, evaluators asked those points of contact to identify the best individual(s) to serve as key informants based on their involvement in the negotiation or decision to join the model. The team made up to three total attempts, by email or phone, to reach each identified key informant. Evaluators sent an informed consent document to all individuals who agreed to schedule an interview.

Interviewer Assignments and Training

Three-person teams comprised of a lead interviewer, a secondary interviewer, and a notetaker, conducted the key informant interviews. Senior qualitative researchers served as lead and secondary interviewers, while early career researchers took notes. Whenever possible, the researcher who developed the protocol for each key informant type served as the lead interviewer.

Interviewers and notetakers participated in training sessions to review interview protocols, interviewing best practices and to gain hands-on experience through mock interviews that simulated realistic respondent scenarios. To streamline operations and support efficiency throughout the data collection period, interviewers and notetakers received training on data collection roles, responsibilities, and processes.

Data Collection and Analysis

The evaluation team facilitated all key informant interviews virtually. The team recorded interviews with participants' permission and transcribed interview recordings. The team assigned attributes to each transcript to identify key informant type which facilitated later analysis and triangulation of findings.

The evaluation team then coded transcripts using NVivo® qualitative analysis software. Analysts coded each transcript using a list of predetermined codes based on the interview questions. The evaluation team iteratively discussed emerging themes, assessed the need for secondary codes based on key informant responses, and agreed on secondary code definitions. Analysts then applied secondary thematic codes and applied queries to assess patterns of responses. The evaluation team regularly reviewed the application of codes to text excerpts for quality assurance and inter-rater reliability.

Limitations

The evaluation of the negotiation process is subject to several limitations. These limitations include:

- The findings described in this report are limited to only the ten SMAs who were interviewed and cannot be generalized to reflect the views of all SMAs.
- Due to the proprietary nature of the negotiated agreements, the evaluation team reviewed redacted RFA responses and all publicly available materials.
- Key informants may have been unwilling to be fully transparent in their answers to interview questions. To encourage candor, informed consent statements explained the steps the team took to maintain privacy and confidentiality. Evaluators reassured key informants that proprietary information will not be revealed in the evaluation reports.
- To minimize the burden on SMAs, the evaluation team conducted only a small number of interviews with state Medicaid officials. Officials from non-participating SMAs were not always available, leading the team to contact alternates.

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