Centers for Medicare & Medicaid Services

Center for Medicare and Medicaid Innovation

Seamless Care Models Group (SCMG)

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Cell and Gene Therapy (CGT) Access Model

Request for Applications from Applicable Manufacturers

Contents

1. Background and General Information

1.1 Model Scope

The Centers for Medicare & Medicaid Services (CMS) is seeking applications for a voluntary Model (the Cell and Gene Therapy Access Model, or "the Model") that tests whether a CMS-led approach to developing and administering outcomes-based agreements (OBAs) for cell and gene therapies (CGTs) improves Medicaid beneficiary access to innovative treatment, improves health outcomes for Medicaid beneficiaries, and reduces health care expenditures. While the Model is focused at this time on participation by state Medicaid programs, depending on state and manufacturer interest, the Model could also include beneficiaries in separate Children's Health Insurance Programs (CHIP).[1]

This request for applications (RFA) is for pharmaceutical manufacturers that participate in the Medicaid Drug Rebate Program (MDRP)[2] and market U.S. Food & Drug Administration (FDA)-approved gene therapies for the treatment of sickle cell disease (SCD) (hereinafter, "Manufacturers")[3] and outlines Model design elements, Model eligibility criteria, and additional Model details. Manufacturers who submit a timely and complete response to this RFA will be eligible to participate in negotiation under this Model with CMS, and may, upon conclusion of negotiation, be eligible to become a Model participant.

CMS anticipates issuing an RFA for interested states in the summer of 2024. At this time, interested states can learn more about the Model by accessing general information at the Model website and contained in this RFA.[4] CMS looks forward to engaging with interested states in this process.

The Model was selected by the Secretary of Health and Human Services (HHS) for testing by the CMS Center for Medicare and Medicaid Innovation (the Innovation Center) in response to Executive Order 14087, "Lowering Prescription Drug Prices for Americans."[5] The Model was announced by the Innovation Center on January 30, 2024. The Innovation Center is conducting the Model under section 1115A of the Social Security Act.

1.1.1 General Approach

The Innovation Center is testing the impact of a voluntary Model wherein CMS facilitates the development and implementation of OBAs between States[6] and Manufacturers. Within this Model, CMS

[1] Inclusion of the separate CHIP population would be under a value-based purchasing arrangement.
[2] Under section 1927 of the Social Security Act.
[3] "Manufacturer" means an entity that holds the New Drug Application(s) (NDA(s)) / Biologics License Application(s) (BLA(s)) of a gene therapy with an FDA approved indication for the treatment of sickle cell disease.
[4] Additional information regarding the Model may be found on the Model website, available here.
[5] Becerra, X. "A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans." Department of Health and Human Services, 2023, available here.
[6] "State" means any state, the District of Columbia, and any U.S. territory that participates in the Medicaid Drug Rebate Program (MDRP).

will negotiate standard Key Terms[7] directly with the Manufacturer. These OBAs may include outcomes-based rebates, volume-based rebates, and guaranteed rebate components.

Upon agreement regarding the standard Key Terms between CMS and the Manufacturer, the Manufacturer will enter into a Participation Agreement (PA) with CMS, and formally become a participant in the Model. CMS will then communicate the agreed-upon standardized Key Terms to all States, who may, at their option, execute a State Agreement (SA) with CMS, thus also becoming participants in the Model. Participating States will adopt the Key Terms through a supplemental rebate agreement (SRA) with a participating Manufacturer.[8] (See Section 2.4 for a description of the legal relationships between CMS, Manufacturers, and States. See Section 2.3.1 for additional details regarding required variation in the optional participation of separate CHIPs.)

CMS will support implementation of the Model through responsibilities such as implementing, monitoring, reconciling, and evaluating the financial and clinical outcomes specified in the Key Terms. In addition, the Innovation Center will conduct a robust model evaluation by an independent contractor. CMS will conduct monitoring activities to ensure compliance with all aspects of the Model by States, Manufacturers, and other relevant entities. These activities will include a focus on the quality of services provided, beneficiary experience, and appropriate access to care. CMS retains the right to modify any Model policy or parameter on an annual basis, or more frequently, in accordance with procedures to be agreed upon in the applicable agreement with the Model participant (as described in Section 2.6). CMS may modify the terms of the Model or cancel it entirely. The terms set forth in this RFA may differ from the terms set forth in the finalized PAs for the Model test.

The Innovation Center is testing this Model for 11 performance years, beginning on January 1, 2025. The Model, at this time, is limited to manufacturers of gene therapies approved or licensed by the FDA for the treatment of SCD that are covered outpatient drugs under the MDRP (hereinafter, "Model Drugs"). Additional information regarding the Model timeline is set forth in Section 5.4.

1.2 Statutory Authority

The authority for the Model is section 1115A of the Social Security Act (the Act) (42 U.S.C. § 1315a, added by section 3021 of the Patient Protection and Affordable Care Act). Section 1115A of the Act authorizes CMS to test innovative healthcare payment and service delivery models that have the potential to lower Medicare, Medicaid, and CHIP spending while maintaining or improving the quality of beneficiaries' care.

The Innovation Center evaluates quality of care (including patient-level outcomes, patient satisfaction, and other patient-centeredness criteria) and changes in federal spending in each model. The Secretary of HHS is authorized to expand the scope and duration of successful models, through rulemaking, that

[7] "Key Terms" means the central parameters of the agreement negotiated with CMS, including rebate calculation and amounts, the duration of the agreement, data sharing arrangements, and any options or variations, that will form the basis for individual Supplemental Rebate Agreements between the Manufacturer and participating States.

[8] The State-specific contracts will comport with applicable laws and regulations.

reduce spending without reducing quality of care, or that improve the quality of patient care without increasing spending.[9]

1.3 Waiver Authority

Under section 1115A(d)(1) of the Act, the Secretary of HHS may waive such requirements of Titles XI and XVIII and of sections 1902(a)(1), 1902(a)(13), and 1903(m)(2)(A)(iii), and 1934 (other than subsections (b)(1)(A) and (c)(5) of such section) as may be necessary solely for purposes of testing models. In general, CMS believes such waivers are not necessary to test the Model for the Medicaid population. However, to the extent that Manufacturers and States execute value-based purchasing arrangements under the Model that include Model Drugs that are administered to beneficiaries under separate CHIPs, the Model could affect Manufacturers' calculations of average sales price (ASP) for Model Drugs in a manner that could disincentivize Manufacturer participation with respect to the separate CHIP population and impede the Innovation Center's ability to observe the impacts of the Model. To help avoid such disincentivizing effects and ensure the Innovation Center may observe and measure Model impacts, under the authority in section 1115A(d)(1) of the Act, CMS will issue a Model-specific waiver of requirements of 1847A(c) to the extent necessary to exclude from the calculation of the Manufacturers' ASP units of Model Drugs administered to participating separate CHIP beneficiaries, thereby avoiding impacts on a Manufacturer's calculation of ASP for a Model Drug. Consistent with section 1847A(c)(5) of the Act, CMS will issue program instructions to further describe how the waiver will impact a Manufacturer's calculation of ASP for a Model Drug. For example, CMS envisions that Manufacturers will take reasonable steps and make reasonable assumptions to exclude applicable units from this calculation. Notwithstanding such a waiver, Manufacturers must continue to comply with all other applicable ASP reporting requirements. For example, Manufacturers who misrepresent or fail to report Model Drug ASP data would remain subject to civil monetary penalties, as applicable and described in sections 1847A and 1927(b) of the Act and codified in regulations at 42 CFR § 414.806.

1.4 CMS-Sponsored Model Safe Harbor

Manufacturers will be required to financially support a defined scope of fertility preservation services at no cost to beneficiaries who receive treatment within the Model or other payers. In doing so, CMMI seeks to test whether manufacturer payment, rather than beneficiary or Medicaid payment, for fertility preservation services (defined further in Section 2.5.4) would improve health outcomes, by reducing long-term health care utilization for patients with SCD, and produce savings for the federal government and states. Specifically, Manufacturer payment for fertility preservation services may yield learning that could inform state Medicaid agencies' future decision-making regarding coverage for fertility preservation services in connection with gene therapy and the potential for contracting arrangements with Manufacturers to fund the cost of treating adverse outcomes related to use of the manufacturer's therapy.

To be eligible to qualify for protection under the "CMS-sponsored model" safe harbor at 42 CFR § 1001.952(*ii*), Manufacturers must meet program requirements, as outlined in Section 2.5.4, as well as

[9] Social Security Act § 1115A [42 U.S.C. § 1315a], "Center for Medicare and Medicaid Innovation."

the regulatory requirements of 42 CFR § 1001.952(*ii*). The CMS-Model safe harbors allow for certain remuneration to be provided in connection with a CMS-sponsored model, and in this case, eliminates the need for a separate and distinct fraud and abuse waiver.[10] CMS may detail additional safeguards and reporting requirements regarding these activities in the Model PA. Notwithstanding any other provisions of this RFA, all individuals and entities must comply with all applicable laws and regulations.

Please note that any safe harbor protections for activities in this Model apply solely to the Cell & Gene Therapy Access Model and could differ in scope or design from waivers and safe harbor protections in other situations, including other programs or models.

2. Description of Model

2.1 Purpose and Concept

Gene therapies are a rapidly growing class of transformative, potentially one-time, medicines designed to treat previously intractable diseases.[11,12] Through these novel technologies, it may be possible to correct the underlying causes of a disease, restore functionality, or halt the progression of devastating illnesses, such as SCD. However, the combination of a) the relative novelty of these products; b) the rare indications on which most gene therapies focus; and c) limited utilization and outcomes data to date, means the long-term curative potential of these therapies remains uncertain. In addition, the typically high cost of these therapies may present affordability challenges for state Medicaid programs and other payers, despite potential downstream savings that may result from avoided progression of these diseases and ongoing treatment costs. In response to these pressures, some payers, including state Medicaid agencies, are using cost containment and utilization management strategies, as legally permissible, that can have the effect of limiting access to gene therapies.[13]

One way to capture the positive potential of novel therapies, while addressing the uncertainty regarding their clinical outcomes, is by using an OBA, wherein a payer's spending for a gene therapy varies based on whether a pre-specified clinical outcome(s) is achieved over a defined period of time. There are a number of possible structures for an OBA, but in its simplest form, a payer and a manufacturer enter into a contract that defines Outcome Measures[14] (clinical values, patient-reported outcome (PRO) measures, or utilization of care measures) and a Measurement Period.[15] Over the course of the Measurement Period, an entity (such as the payer) tracks the agreed-upon Outcome Measures

[10] OIG, HHS Office of Inspector General Fact Sheet, Final Rule: Revisions to the Safe Harbors Under the Anti-Kickback Statute and Civil Monetary Penalty Rules Regarding Beneficiary Inducements (November 2020), available here.

[11] "Advancing Health Equity." Centers for Disease Control and Prevention, 2022, available here; Nowogrodzki, A. "No adult left behind: bridge the health-care gap for sickle-cell disease." Nature, 2021, available here.

[12] Cell and gene therapy represent overlapping fields of biomedical research with similar therapeutic goals, which target DNA or RNA inside or outside the body. Gene therapy involves making changes to a patient's genetic material, or DNA, whereas cell therapy involves the infusion or transplantation of whole cells into a patient.

[13] "Chapter 1: Addressing High-Cost Specialty Drugs." June 2021 Report to Congress on Medicaid and CHIP. Medicaid and CHIP Payment and Access Commission, 2021, available here.

[14] "Outcome Measures" mean the agreed-upon clinical or utilization-based factors that are linked to rebates.

[15] "Measurement Period" means the time period following administration of the drug during which Outcome Measures will be monitored.

applicable to an individual beneficiary or population. If pre-defined thresholds for the Outcome Measures are not met, then rebates may be due to the payer, from the manufacturer, at agreed-upon intervals. A retrospective analysis and reconciliation of rebates (i.e., final settlement of the rebate amounts owed and paid) occurs following the conclusion of the Measurement Period.

State Medicaid agencies today can, and do, independently negotiate rebates through SRAs permitted under CMS-authorized State Plan Amendments (SPAs). Specifically, states may enter into SRAs as long as the agreements result in rebates equal to or greater than the federal statutory rebate states receive from the MDRP.[16] A number of states have received authorization from CMS to enter specifically into Value-Based Purchasing SRAs (VBP SRAs), which allow them to operate or enter into OBAs. However, States have reported that their ability to pursue OBAs for gene therapies is curtailed by the complexity in negotiating endpoints and thresholds with manufacturers, the states' lack of leverage stemming from the lack of alternative treatments and statutory coverage obligations, as well as the burden of data collection and continuous level of effort for evaluation over multiple years.[17]

Through the Model, the Innovation Center will test whether a partnership among CMS, Manufacturers and States related to gene therapies could offer better and more equitable access to treatment for beneficiaries with rare and severe diseases, including those in underserved communities, and how that access may translate into improved quality and health outcomes.

For State participants, the Model aims to reduce the burden of negotiating and implementing VBP SRAs for gene therapies and potentially facilitate the adoption of OBAs in more states. This Model could also facilitate savings to, and improve stability for, States due to predictability, greater rebates, and long-term reductions in health expenditures.

For Manufacturers, participation in the Model may provide numerous advantages. Through a standardized policy across participating States, this Model also may ease burdens on beneficiaries and providers by improving efficiency in navigating utilization management in the patient's care journey.

In addition, the Model is expected to expand access to critical supportive services that are likely to increase beneficiary uptake of the Model Drug in order to improve health outcomes. This includes, but is not limited to, care coordination, access to SCD specialists, access to behavioral health providers and fertility preservation services. Finally, CMS will take a central role in data collection and monitoring to facilitate the adoption and implementation of OBAs and related monitoring, helping to relieve participants, both Manufacturers and States, of some of that burden (see Section 2.5.6 for additional information on CMS responsibilities). Overall, the Model aims to reduce the burden to States and Manufacturers of operating an OBA, while maximizing access to novel and transformative therapies for beneficiaries.

The purpose of this RFA is to outline the elements that must be included in a Manufacturer's application to join the Model. The application template is attached to this RFA as Appendix A. Eligible respondents

[16] 42 CFR § 447.502 (defining "CMS-authorized supplemental rebate agreement").

[17] Becerra, X. "A Report in Response to the Executive Order on Lowering Prescription Drug Costs for Americans." Department of Health and Human Services, 2023, available here.

to this RFA will be invited by CMS to participate in the Model pre-implementation period (outlined below), including negotiation of Key Terms. While this RFA may result in subsequent negotiation, a response to this RFA constitutes a formal offer to CMS regarding all aspects of the Model described herein. Responding to this RFA does not obligate the Manufacturer to become a Model participant.

At this time, the Model is exclusively soliciting applications from Manufacturers of gene therapies for SCD. In future years, this Model may solicit applications from pharmaceutical manufacturers of CGTs indicated to treat other conditions.

2.2 Model Participation

The Cell and Gene Therapy Access Model is voluntary to all participants. While this RFA only applies to Manufacturers, information regarding State participation is included within this document for the reference of Manufacturers and to aid in responses to this RFA. The legal agreements described throughout this Section are outlined in Section 2.4.

2.2.1 Manufacturer Participation

To be eligible to submit an application in response to this RFA, an entity must fulfill two requirements:

1. Hold the NDA(s) / BLA(s) of a gene therapy with an FDA approved indication for the treatment of SCD.
2. Participate in the MDRP.

Manufacturers that satisfy the above requirements and submit a timely and complete application in response to this RFA will be eligible to participate in the Model pre-implementation period.

The Model pre-implementation period begins May 1, 2024 and ends November 29, 2024. During the Model pre-implementation period, CMS and Manufacturers will negotiate the standard Key Terms. If an agreement between parties is reached, then the Manufacturer must execute a PA with CMS before the conclusion of the pre-implementation period. See Section 5 for more details about the Model timeline, including the dates for submission of applications in response to this RFA.

A Manufacturer that participates in the Model pre-implementation period and signs a PA by November 29, 2024 with CMS is considered a Model participant. **Manufacturer requirements for participation in the Model are as follows:**

1) Participated in negotiations with CMS during the Model pre-implementation period;
2) Entered into a PA with CMS before conclusion of the pre-implementation period; and
3) Maintains compliance with the PA.

Participating Manufacturers must offer the standard negotiated Key Terms to all States. If a State accepts the Key Terms, the Participating Manufacturer must enter into a VBP SRA incorporating the Key Terms with that State. Variation in Key Terms will only be permitted as necessary to comport with State laws and regulations and must be approved by CMS. A process for disclosure of variation in Key Terms by states. and approval, as necessary will be specified in the State RFA. A Participating Manufacturer may not exclude any States that elect to participate.

2.2.2 State Participation

Model participation is open to all states, the District of Columbia, and all U.S. territories that participate in the MDRP. If a territory does not participate in the MDRP as of February 28, 2025, but joins the MDRP during the course of the Model, CMS may open a new application cycle to allow the newly eligible territory to participate in the Model with any participating Manufacturer.

States will apply to participate in the Model after the Key Terms have been negotiated and at least one Manufacturer becomes a Model participant (i.e., executes a PA prior to November 29, 2024). CMS has requested that states that intend to participate in the Model submit preliminary non-binding Letters of Intent to CMS by April 1, 2024. States will participate in the Model by responding to a State Request for Applications (expected to be released in Summer 2024) by no later than February 28, 2025, and executing a SA with CMS. Additional information regarding State obligations in the SA are described in Section 2.4. States will be required to adopt the Key Terms for at least one Model Drug on a date of their choosing from January 1, 2025, to January 1, 2026. States must execute VBP SRAs with Manufacturer(s) that reflect the negotiated Key Terms for the State's selected Model Drug(s)) and may change their selected Model Drug(s) at annual renewals of the VBP SRA (see Section 2.6). States that do not join the Model by January 1, 2026, will not be allowed to participate in the Model except at CMS discretion. CMS will inform the participating Manufacturer upon acceptance of a new State participant. States that participate in this Model may not alter or make additions to the Key Terms except as necessary to comport with State laws and regulations and as approved by CMS.

States may also choose to apply for optional Model funding from CMS through a separate Notice of Funding Opportunity (NOFO) that is expected to be released in Summer 2024. States may receive funding for activities related to Model implementation and performance milestones by responding to the NOFO and executing a Cooperative Agreement with CMS.

2.3 Model Population

The Model population includes Medicaid and Medicaid expansion CHIP beneficiaries in fee-for-service and Medicaid managed care who do not have other coverage that is the primary payer for a Model Drug (hereinafter, "Medicaid beneficiaries"). Manufacturers and States will have the option to include separate CHIP beneficiaries alongside Medicaid beneficiaries. See Section 2.3.1 for more information on the optional inclusion of separate CHIP beneficiaries in the Model.

Model beneficiaries are beneficiaries in the Model population who are deemed eligible for (i.e., are clinically eligible for and meet all negotiated prior authorization criteria) and receive a Model Drug that is covered and paid for by either (1) a participating State Medicaid program as a covered outpatient drug where Medicaid is the primary payor, or (2) if the Manufacturer and State engage in a separate VBP arrangement for separate CHIP beneficiaries, a separate CHIP that participates in the Model.

2.3.1 Children's Health Insurance Program (CHIP)

Inclusion of beneficiaries in Title XXI-funded Medicaid expansion CHIPs (that is, CHIPs in which the State receives Title XXI funding to expand Medicaid eligibility to optional targeted low-income children) is

required. All requirements for Medicaid beneficiaries in this RFA apply to both Title XIX-funded Medicaid and Title XXI-funded Medicaid expansion CHIP.

Inclusion of beneficiaries in separate CHIPs is optional for Manufacturers and States. To facilitate the inclusion of the separate CHIP population, the Manufacturer should specify in their response to this RFA their willingness to offer a VBP arrangement for separate CHIP beneficiaries in addition to Medicaid beneficiaries. If so, CMS and the Manufacturers may, during Key Term negotiation, structure supplemental Key Terms that constitute a VBP arrangement that meets the definition of such an arrangement at 42 CFR § 447.502 (hereinafter, "separate CHIP Key Terms"). The separate CHIP Key Terms would be distinct from the Key Terms for Medicaid program supplemental rebates and would satisfy requirements under CMS's existing "multiple best price" reporting framework.

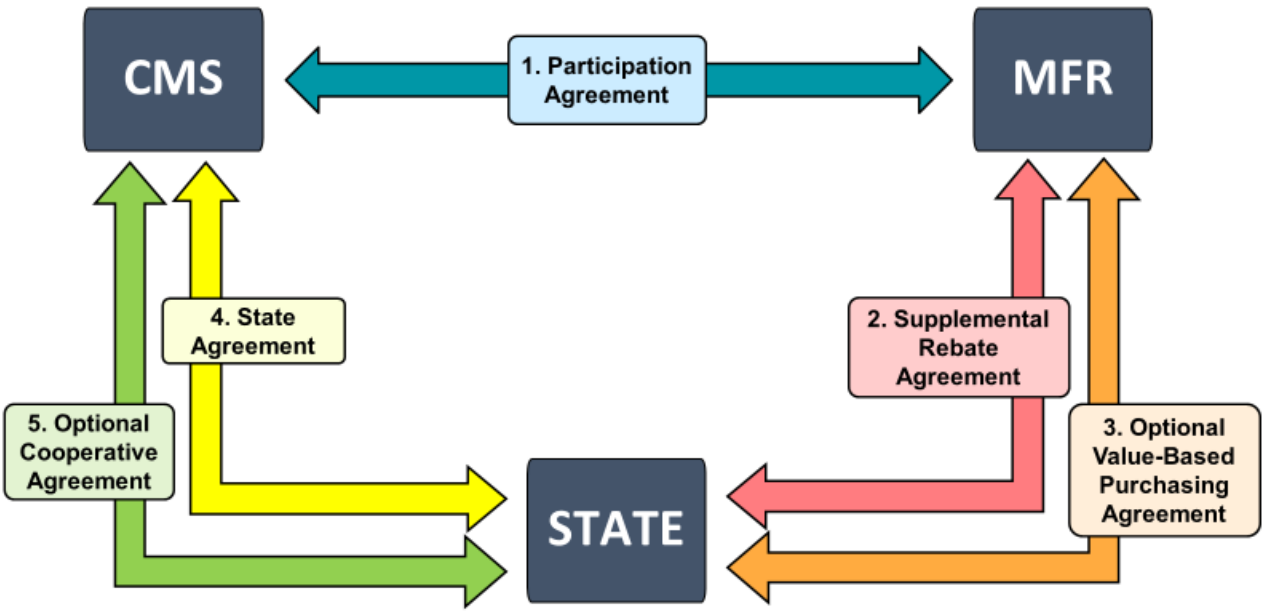
Negotiated Key Terms regarding volume rebates or guaranteed rebates will not qualify under the "multiple best price" reporting framework and may be excluded in the agreement reached for separate CHIP beneficiaries. Unless explicitly noted (or otherwise not permissible by law), all requirements regarding the Key Terms discussed throughout this RFA must apply to any agreement reached for the separate CHIP population.

See Section 1.3 for a discussion of Model-specific waivers related to exclusion from the calculation of ASP units of Model Drugs administered to participating separate CHIP beneficiaries.

2.4 Legal Agreements

This Model will include a partnership among CMS, participating Manufacturers, and participating States. This partnership will be executed through multiple legal and contractual mechanisms. Legal relationships are enumerated in both Figure 1 and the table below.

Figure 1: Legal Agreements



1. CMS and Manufacturer: Manufacturer Participation in Model (Participation Agreement)
Effective Dates: No later than January 1, 2025 – December 31, 2035 **Description:** 1) Formalizes Manufacturer participation in the Model. 2) Specifies "Key Terms" negotiated with CMS, which will be included in the OBAs established with participating States. Key Terms may address, but are not limited to: a. Duration of OBA Term, Measurement Period, Volume Accrual Period, and Reconciliation Period b. Pricing related to Outcome-Based Rebates, Guaranteed Rebates, Volume-Based Rebates c. Outcome Measures and Outcome Measure Benchmarks d. Fertility Preservation Services e. Access Policy f. CMS Responsibilities g. Rebate Documentation h. Termination, Renewals, Renegotiation or Alterations i. Coverage Shifts 3) Specifies terms of CMS and Manufacturer data exchange. 4) Specifies terms of potential Model renewal.

2. Manufacturer and State: Outcomes-Based Agreement (Supplemental Rebate Agreement)

Anticipated Effective Dates: January 1, 2025* – December 31, 2030**

Description:

1) Formalizes supplemental rebates as negotiated by CMS and the Manufacturer.
2) Specifies "Key Terms" negotiated by CMS and the Manufacturer. Key Terms may include, but are not limited to:
    a. Duration of OBA Term, Measurement Period, Volume Accrual Period, and Reconciliation Period
    b. Pricing related to Outcome-Based Rebates, Guaranteed Rebates, Volume-Based Rebates
    c. Outcome Measures and Outcome Measure Benchmarks
    d. Fertility Preservation Services
    e. Access Policy
    f. CMS Responsibilities
    g. Rebate Documentation
    h. Termination, Renewals, Renegotiation or Alterations
    i. Coverage Shifts

* May begin on a date of the State's choosing from January 1, 2025 to January 1, 2026.
** Ends at the conclusion of the OBA Term, which will be part of the Key Terms subject to CMS-Manufacturer negotiation.

3. *(Optional)* Manufacturer and State: Inclusion of separate CHIP Population (Value-Based Purchasing Agreement)

Anticipated Effective Dates: January 1, 2025* – December 31, 2030**

Description:

1) Formalizes the rebates related to the OBA as negotiated by CMS.
2) Specifies "separate CHIP Key Terms" negotiated by CMS and the Manufacturer. Separate CHIP Key Terms may include, but are not limited to:
    a. Duration of OBA Term, Measurement Period, and Reconciliation Period
    b. Pricing related to Outcome-Based Rebates
    c. Outcome Measures and Outcome Measure Benchmarks
    d. Fertility Preservation Services
    e. Access Policy
    f. CMS Responsibilities
    g. Rebate Documentation
    h. Termination, Renewals, Renegotiation or Alterations
    i. Coverage Shifts

* May begin on a date of the State's choosing from January 1, 2025 to January 1, 2026.
** Ends at the conclusion of the OBA Term, which will be part of the Key Terms subject to CMS-Manufacturer negotiation.

4. CMS and State: State Participation in Model (State Agreement)

Effective Dates: January 1, 2025*** – December 31, 2035

Description:

1) Formalizes the terms of State participation in the Model.
2) Requires States to include Medicaid beneficiaries in the Model when Medicaid is the primary payer for a Model Drug. Beneficiaries enrolled in fee-for-service Medicaid must be included by the beginning of the Performance Period.[18] Beneficiaries enrolled in Medicaid managed care plans must be included by no later than January 1, 2026.
3) Allows States to include separate CHIP beneficiaries in the Model by no later than January 1, 2026, subject to separate, optional agreement with Manufacturers.
4) Establishes State requirements for Model participation. For instance, States must:
    a. Have, or obtain, the necessary authority to implement the Model, including CMS approval of a SPA to enter into a VBP SRA.
    b. Establish a standardized Model Drug access policy consistent with the CMS-Manufacturer negotiated Key Terms.
    c. Carve Model Drugs out of an inpatient payment bundle, if necessary, and pay for the Model Drugs in a manner such that rebates under the MDRP apply.
    d. Require providers to follow Model-specific requirements related to registry participation and claims submission.
    e. Ensure that managed care plan policies align with Model requirements.
    f. Execute a VBP SRA with a participating Manufacturer that incorporates the CMS-Manufacturer negotiated Key Terms.
    g. If applicable, execute a VBP agreement for separate CHIP beneficiaries with a participating Manufacturer that incorporates the CMS-Manufacturer negotiated separate CHIP Key Terms.
    h. Attest that included beneficiaries will have access to gene therapy care with at least one qualified SCD gene therapy provider within the state or in another state.
    i. Attest that the State will ensure necessary transportation (and related travel expenses to Model beneficiaries (and their caregivers, as applicable).
    j. Meet minimum data requirements and conduct data quality activities.
    k. Submit reports to CMS on Model implementation.

*** States will sign SAs on a rolling basis following CMS acceptance of their applications (which may be submitted between December 2024 and February 2025).

[18] For each participating State, the Performance Period will begin on a date of the State's choosing from January 1, 2025 to January 1, 2026.

5. *(Optional)* CMS and State: Model Funding (Cooperative Agreement)
Anticipated Effective Dates: June/July 2025 – December 31, 2035 **Description:** 1) Outlines funding for activities related to Model implementation (e.g., data collection, coordination with managed care plans and out-of-state providers, partnerships with community-based organizations, etc.). 2) Describes performance milestones for which states may receive funding related to equitable access to care and beneficiary receipt of behavioral health services, fertility preservation, and follow-up care.

2.5 Key Terms

Manufacturers that submit a timely and complete response to this RFA will be eligible to participate in negotiations with CMS to determine the Key Terms of the Model. Key Terms means the central parameters of the agreement negotiated with CMS, including rebate calculation and amounts, the duration of the agreement, data sharing arrangements, and any options or variations, that will form the basis for individual SRAs between the Manufacturer and participating States. In its application to this RFA, the Manufacturer must include proposals related to each of these Key Terms. The Manufacturer should not consider the list below as being exhaustive and may propose additional Key Terms as an attachment to their Model application. The full application template for this RFA is included as Appendix A.

Key Term	Description
Access Policy	The State coverage policy for the Model Drug, including utilization management policies, such as prior authorization criteria. Such access policies would be standardized across all participating State's Medicaid fee-for-service and Medicaid managed care beneficiaries unless necessary to diverge to comply with state law.[19] States may create their own criteria and policies so long as they are no more restrictive than the standardized access policy.

[19] Manufacturers and States will have the option of including their separate CHIP beneficiaries in the OBA, discussed further in Section 2.3.1.

CMS Responsibilities	CMS's role in operationalizing the Key Terms. This includes, but is not limited to, supporting States and Manufacturers with the implementation of the Model and the OBA. CMS will be responsible for gathering, aggregating, and analyzing data, as well as assessing whether the Outcome Measure Benchmarks are met. CMS will determine the resulting financial obligations and share reports with States and Manufacturers.
Coverage Shifts	How to consider patients who have a change in healthcare coverage after receiving the Model Drug but before the collection of all data relevant to Outcome Measures.
Fertility Preservation Services	CMS will require participating Manufacturers to provide payment for fertility preservation services for individuals who receive a Model Drug.
Guaranteed Rebates	Rebates provided for the Model Drug that are applied to all units, regardless of outcomes or volume. These guaranteed rebates are specific to the Model and are in addition to the existing statutory rebates required under the MDRP.
Measurement Period	The time period following administration of the Model Drug during which Outcome Measures for an individual or cohort will be monitored.
OBA Term	The time period for which the Key Terms are applicable. In other words, the OBA term is the period of time during which State financial arrangements with the Manufacturer governed by the OBA apply for beneficiaries who receive the Model Drug. The Manufacturer may propose a duration of the OBA Term in their response to this RFA, so long as that term is less than 6 years, and no related obligations extend beyond 2035.
Outcome Measure Benchmarks	The measurable thresholds at which the Outcome Measure performance will result in a rebate. Outcome Measure Benchmarks will be assessed at agreed upon performance assessments throughout the Measurement Period. The number and timing of performance assessments are encompassed within this Key Term.
Outcome Measures	The agreed-upon measures that are linked to rebates. If certain outcomes following treatment with a gene therapy are not achieved, then the outcomes-based rebate is triggered.

Outcome-Based Rebates	The rebate amount paid by the Manufacturer due to failure to reach an Outcome Measure Benchmark. Can be a calculation based on pricing (see Appendix A).
Rebate Documentation	The materials and data required to confirm that an outcome-based or volume-based rebate is owed to the State.
Reconciliation Period	The time period following the conclusion of the Measurement Period in which final performance measurement, financial settlement and payment of rebates will occur. The Reconciliation Period may include interim and final calculation and payment of rebates.
Termination, Renewals, Renegotiation or Alterations	How to proceed with any termination, renewals, alterations, or renegotiations of the Key Terms throughout the duration of the Model. This includes processes for Manufacturer or State withdrawal from Model participation.
Volume Accrual Period	The time period in which additional units of the Model Drug count towards the volume-based rebate before resetting to baseline.
Volume-Based Rebates	The amount paid by Manufacturer based on the number of units of the Model Drug counted during the Volume Accrual Period.

2.5.1 Outcomes Based Rebates

In responses to this RFA, Manufacturers must include a proposal for outcome-based rebates. Manufacturers must propose Outcome Measures and Outcome Measure Benchmarks in their response to this RFA (see Appendix A) that will be tied to outcomes-based rebates for Medicaid beneficiaries. In proposing Outcome Measures and Outcome Measure Benchmarks, Manufacturers must consider:

(1) **Broad Applicability to Medicaid Beneficiaries who have SCD.** To enhance the feasibility and simplicity of the scoring and weighting of Outcome Measures, proposed measures must be applicable to the population of SCD patients who are eligible to receive the Model Drug.

(2) **Acceptability to Interested Parties.** Proposed Outcome Measures must be supported by robust evidence and agreed upon by a consensus of SCD experts.[20]

(3) **Related to Longer-Term Outcomes.** Proposed Outcome Measures must be closely linked to SCD progression and be indicative of the impact that the Model Drug is expected to have across the patient's lifetime.

[20] The Manufacturer may, in their response to this RFA, attach evidence or testimonials to establish scientific consensus.

Below are four Outcome Measures that CMS has identified as primary outcomes of interest for SCD gene therapies. Manufacturers' proposed Outcome Measures may overlap with, or diverge from, these measures, but must be responsive to the three considerations listed above.

1. Occurrence of red blood cell (RBC) transfusions to treat SCD.
2. Occurrence of vaso-occlusive crisis (VOC) identified by medical utilization.
3. Anti-sickling hemoglobin levels as determined by an electrophoresis or high-performance liquid chromatography (HPLC) test.
4. Patient-reported outcome measures (PROMs) that assess the impact of gene therapy on beneficiary quality of life (including, but not limited to, home management of VOCs and reduction in fatigue).

Manufacturers must include in their application a list of proposed Outcome Measures and Outcome Measure Benchmarks that, if finalized, will be tied to supplemental rebates (Outcomes-Based Rebates), and that identifies which measures would be analyzed at a patient-level and/or population-level (discussed in more detail below). For each Outcome Measure, the Manufacturer must include valid and empirical evidence to support the use of that Outcome Measure in the Key Terms as an attachment in their RFA responses. CMS encourages Manufacturers to consider both patient-level and population-level Outcome Measures in their responses to this RFA. Manufacturers are encouraged to consider how the patient perspective can be incorporated into the Model to the fullest extent possible.

The application must also include details regarding the preferred data source, how the Outcomes-Based Rebates will be calculated, how Outcome Measure Benchmarks will be identified, the intervals at which performance assessment will occur, and the length of the Measurement Period. In proposing a preferred data source, the Manufacturer must consider and provide evidence of the reliability and accuracy of collected data.

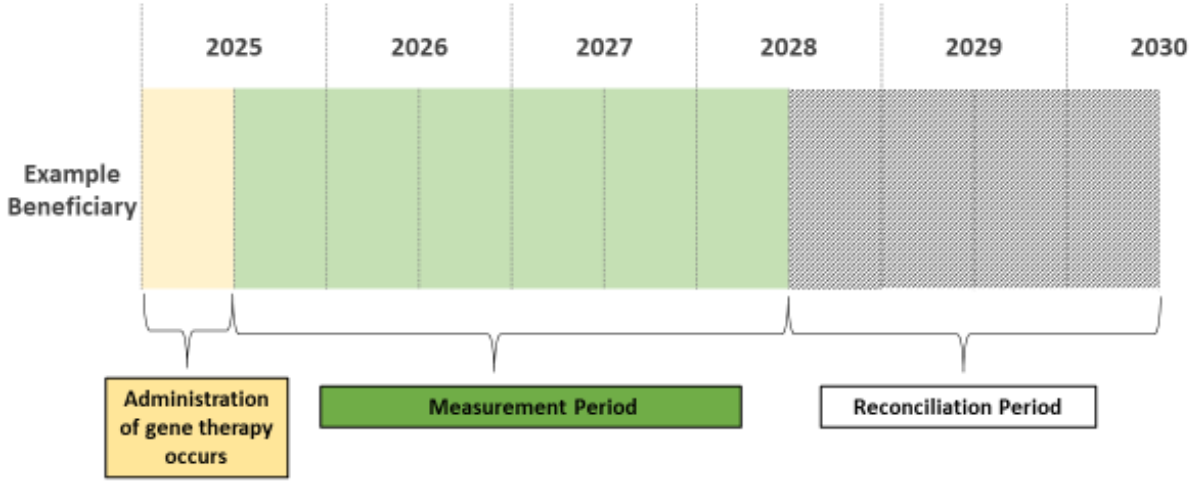
The Manufacturer application must also propose and include the rationale for the number of and timing of the Outcome Measure Benchmark performance assessments that will occur throughout the Measurement Period. If a baseline assessment is required, the Manufacturer must specify the timing and type of data required to establish that baseline.

Manufacturers must also clearly describe how the results of an Outcome Measure Benchmark performance assessment should be captured, and what documentation will be necessary to substantiate if Model rebates are owed to the States (Rebate Documentation). Manufacturers may refer to the data support provided by CMS as described in Section 2.5.6 in their response. Figure 2, included below for reference only, illustrates the potential duration of the OBA Term, Measurement Period, and Reconciliation Period for a single beneficiary.

- **Example:** In this example, the Key Terms include an OBA Term of six years, a Measurement Period of three years that begins 6 months following the administration of the Model Drug, and a Reconciliation Period of two years. Under this example framework, a beneficiary who receives treatment on January 1, 2025, would have a Measurement Period that begins on July 1, 2025,

and concludes on July 1, 2028. The final financial settlement for that beneficiary would occur no later than July 1, 2030.

Figure 2: Example Patient-Level Structure



Population-level Outcome Measures would allow Model participants to tie rebates to the impact of the Model Drug over a "cohort" of beneficiaries. If a proportion of a cohort meets (or fails to meet) a certain threshold (the Outcome Measure Benchmark), as negotiated and agreed upon by CMS and Manufacturers, then a rebate would be due.

The Manufacturer must propose a precise definition of "cohort" for each proposed population-based Outcome Measure. The Manufacturer must also specify how rebates will be allocated across participating States for each cohort. For example:

- **Example 1:** The cohort includes beneficiaries in single Model participating State who receive the Model Drug in a single calendar year. Population-level rebates owed are allocated to an individual participating State according to the performance of the cohort in that State alone.
- **Example 2:** The cohort includes all beneficiaries in all Model participating States who receive the Model Drug over three calendar years. Population-level rebates owed are allocated proportionally to the number of beneficiaries from a State in that cohort.

Figure 3 in section 2.5.3 illustrates a potential population-level Outcome Measure structure and timeline.

2.5.2 Volume Based Rebates

CMS is interested in negotiating as part of the Key Terms volume-based rebates, wherein the rebate for increases with additional units over a defined period of time. Volume-based rebates would apply prior to any additional rebates realized due to failure to meet Outcome Measure Benchmarks.

In responses to this RFA, Manufacturers must include a proposal for a) volume-based rebates, b) guaranteed rebates (see Section 2.5.3), or c) both volume-based rebates and guaranteed rebates. A response that does not include either volume-based rebates or guaranteed rebates for Medicaid

beneficiaries will be considered incomplete. Manufacturers are not required to propose volume-based rebates or guaranteed rebates for separate CHIP beneficiaries.

In their response to this RFA, Manufacturers should include information on proposed volume-based rebates, including:

1) How rebates will be tied to volume – meaning how volume will be aggregated (for example, aggregated across all Model participating States, or within a single participating State), and the calculation for determining the rebate.
2) The length of time in which units will count towards the volume rebate before the Volume Accrual Period concludes.
3) Proposed rebate amounts based on volume (for example, an increasing percentage rebate per volume tier to a maximum amount during the Volume Accrual Period.)

Manufacturers may propose a volume-based rebate structure that accounts for the uncertainty, at the time of the release of this RFA, regarding which States may ultimately participate in the Model. For example, Manufacturers may propose multiple volume-based rebate structures tied to the percentage of Medicaid beneficiaries with SCD in a participating State, or the number of participating States that are in the Model. The Manufacturer is not limited to the two options described in this example and may make other such proposals related to the volume-based rebate structure.

2.5.3 Guaranteed Rebates

CMS is interested in negotiating as part of the Key Terms guaranteed rebates. These rebates would apply to units of the Model Drug prior to and regardless of additional rebates realized due to volume or performance on Outcome Measures. Guaranteed Rebates must be structured to likely result in a supplemental rebate amount that is greater than $0 per unit utilizing either of the two calculation options below or as mutually agreed upon by the Manufacturer and CMS during negotiation and included in the final Key Terms:

- **Option 1:** Guaranteed Rebate = A percentage of Wholesale Acquisition Cost (WAC)
- **Option 2:** Guaranteed Rebate = WAC – MDRP statutory rebate – Contracted Rate[21]

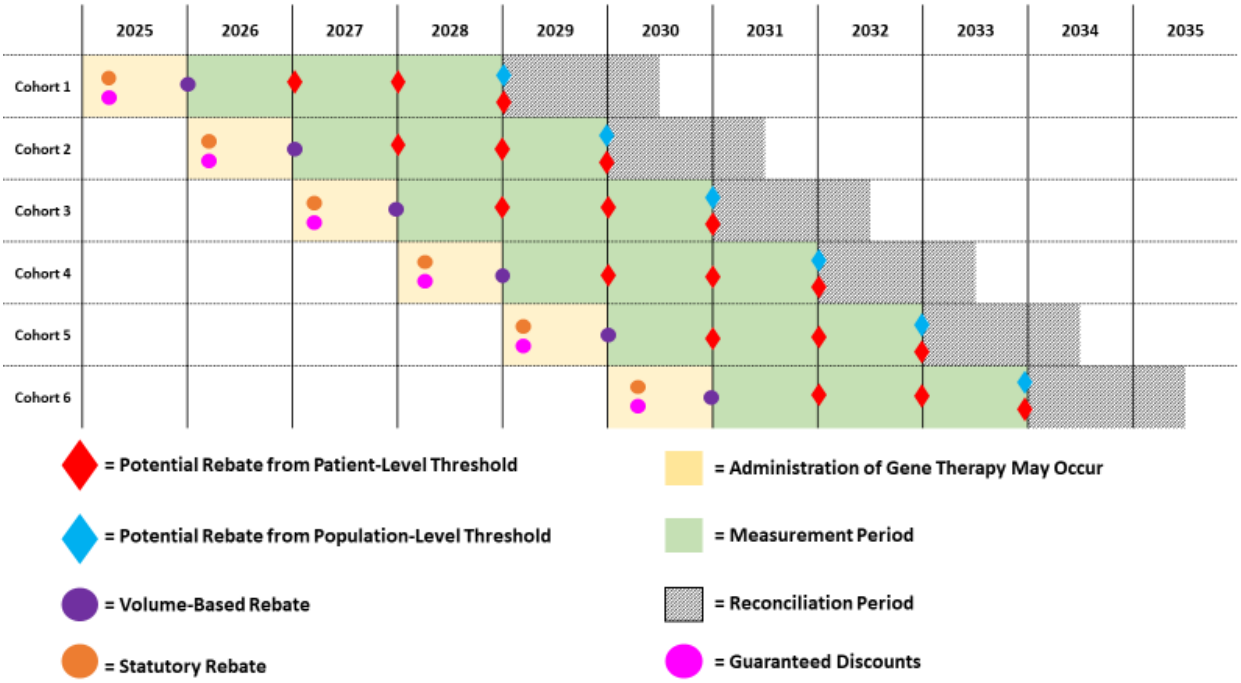
Manufacturers who propose a Guaranteed Rebate must specify in their RFA response the proposed Guaranteed Rebate calculation, specifying the proposed formula and components (i.e., percentage of WAC, Contracted Rate). The manufacturer must also propose whether adjustments or additional provisions would adjust the Guaranteed Rebate formula or components due to conditions such as inflation or changes to WAC, and the timing and frequency of such adjustments.

As discussed in Section 2.5.2, Manufacturers must include a proposal for a) volume-based rebates, or b) guaranteed rebates, or c) both volume-based rebates and guaranteed rebates in their responses to this RFA. A response that does not include either volume-based rebates or guaranteed rebates for Medicaid beneficiaries will be considered incomplete.

[21] "Contracted Rate" will be determined in negotiations and may be proposed in the Manufacturer's response to this RFA.

Figure 3, included below for reference only, illustrates the potential timeline for both patient-level and population-level outcome measures with an OBA Term of six years (from 2025-2030), and a Measurement Period of three years for each annual cohort, and potential timing of Guaranteed Rebates, Volume Rebates, and Outcome Measure benchmark performance assessments.

Figure 3: Example Cohort Structure with Outcome-Based Rebates, Guaranteed Rebate and Volume-Based Rebates



2.5.4 Fertility Preservation

Participating Manufacturers must financially support fertility preservation services at no cost to beneficiaries who receive a Model Drug within the Model or to other payers.[22] Manufacturer payment for fertility preservation services would only be required (and permitted) related to beneficiaries who receive a Model Drug during the relevant period of the Model test (from January 1, 2025 – December 31, 2030, or as determined in negotiations) and for whom Medicaid is the primary payer in a participating State. Fertility preservation services encompass the following services:

- **Egg Harvesting (mature eggs), Freezing, and Storage:** The process of harvesting mature eggs takes approximately four to six weeks. During that time, the beneficiary completes ovarian reserve testing and physician consultation, followed by several weeks of taking birth control pills and injecting ovarian stimulating hormones. During this time, the beneficiary goes to the fertility clinic for regular outpatient monitoring appointments, which include bloodwork and

[22] Administration of gene therapy for SCD typically involves a chemotherapy regime that results in infertility.

ultrasounds. At the end of the stimulation period, the beneficiary receives a final trigger injection, two days after which the beneficiary has their eggs retrieved. The retrieval consists of IV sedation in an outpatient setting, while the eggs are collected during an intra-vaginal procedure. Once retrieved, embryologists evaluate the eggs to determine which ones are matured. Matured eggs are frozen using vitrification, an ultra-rapid cooling process, and are then stored in liquid nitrogen for long-term storage.[23]

- **Egg Harvesting (immature eggs), Freezing, and Storage:** In pre-pubescent girls, ovarian tissue may be removed laparoscopically to access immature eggs. The tissue is carefully divided into small pieces, frozen, and stored.[24] Ovarian tissue cryopreservation is already considered an accepted technique for female fertility preservation given its success in restoring ovarian function and fertility.[25]

- **Sperm Collection, Freezing, and Storage:** The process of collecting sperm in preparation for freezing typically consists of the beneficiary providing a sperm sample either at a fertility clinic, or at home and delivered to a lab within 24 hours. In some cases, aspiration of sperm, an outpatient procedure involving local anesthesia, may be necessary. Some beneficiaries must undergo microdissection testicular sperm extraction (MicroTESE), which is performed with the aid of a surgical microscope in the operating room with general anesthesia.[26] The sperm is then frozen and stored.

- **Testicular Tissue Extraction, Freezing, and Storage:** In pre-pubescent boys, testicular tissue may be obtained by a unilateral open testicular biopsy under general anesthesia. Testicular tissue samples are then transferred, frozen, and stored for future use. Clinical outcomes of testicular tissue transplantation in human subjects are still unavailable.[27]

The Model requirements for Manufacturers related to financially supporting fertility preservation services are anticipated to include:

1. The Manufacturer must pay providers of fertility preservation services directly or through a third party, without payment by the beneficiary or other payer. Under no circumstances will payments be made to beneficiaries or their caregivers.

2. The Manufacturer must provide comprehensive disclosures to beneficiaries regarding the limited scope of fertility preservation services that are payable under the allowable Model fertility preservation services. The Manufacturer must also detail the services that are not payable under the allowable Model fertility preservation services but are typically required to achieve a live birth using cryopreserved reproductive material, including, for example, the costs of cryopreservation and storage following the

[23] Imudia, A. "The egg freezing cycle in 5 steps." Shady Grove Fertility, 2022, available here.
[24] "Ovarian Tissue Freezing (Cryopreservation)." Johns Hopkins Medicine, accessed 2023, available here.
[25] Chen, L., Dong, Z., and Chen, X. "Fertility preservation in pediatric healthcare: a review." Front. Endocrinol., 2023, available here.
[26] "Sperm Retrieval Procedures." Johns Hopkins Medicine, accessed 2023, available here.
[27] Chen, L., Dong, Z., and Chen, X. "Fertility preservation in pediatric healthcare: a review." Front. Endocrinol., 2023, available here.

negotiated storage period (see below), in-vitro fertilization, and ovarian and testicular tissue transplantation for pre-pubescent individuals, as applicable.

3. The Manufacturer must pay cryopreservation and storage fees for a negotiated period that must be at least five years and may be up to fifteen years in an advanced sum so that any costs for cryopreservation and storage for the full negotiated period are not passed to the beneficiary or other payer.

4. The Manufacturer must engage a third-party vendor to contract with fertility preservation clinics and providers to the Model population. This third-party vendor must be responsible for providing a reasonable set of fertility preservation clinic/provider options to beneficiaries who qualify for fertility preservation under the Model. The options offered to an individual beneficiary may be based on factors such as the geographic location of the beneficiary so long as the options offered do not steer the beneficiary to any particular provider. Beneficiaries must be free to choose a clinic or provider that is not included among offered options, so long as the total cost to the Manufacturer (inclusive of travel support, as described below) is equal to or less than the total cost of the most expensive clinic or provider on the third-party vendor's list.

5. The Manufacturer (or a relevant third-party vendor engaged by the Manufacturer), must provide financial reimbursement to certain eligible beneficiaries[28] for certain costs related to travel, lodging, and meals, if necessary for the receipt of fertility preservation services. Specifically, the Manufacturer must (i) provide reimbursement for mileage, tolls, and parking upon presentation of a valid receipt; (ii) arrange for transportation via air travel, train, bus, or rental car for eligible beneficiaries and a caregiver to and from the fertility preservation provider; (iii) if lodging is necessary, arrange for a modest single hotel room for a length of time not to exceed two days per visit; and (iv) if an overnight hotel stay is necessary, provide a reasonable per diem amount for food expenses for the eligible beneficiary and caregiver on the days where the overnight hotel stay takes place. The financial support expectations described in this section pertain only to travel, meals, and lodging support related to fertility preservation services for beneficiaries.

6. The Manufacturer must ensure the delivery of comprehensive beneficiary education regarding the provided fertility preservation services. Beneficiary education material must be written and delivered at a 5th grade or lower reading level. Materials must include comprehensive disclosure of the scope and limitations of available services, potential extended storage costs and transfer of responsibility for storage costs to the

[28] To be eligible for travel-related assistance for the purposes of receiving fertility preservation services, the beneficiary must (i) must live more than 2 hours driving distance or more than 100 miles from their fertility preservation provider, and (ii) not also be the recipient of free assistance from an independent third party related to travel and lodging for this service.

beneficiary after Manufacturer pre-payment has elapsed, and the likelihood of a live birth if a beneficiary decides to use the cryopreserved reproductive material.

7. The Manufacturer must document for each beneficiary who receives its Model Drug under the Model whether that beneficiary received an offer of fertility preservation services prior to receiving myeloablative conditioning in preparation for being infused with the modified stem cell product.

8. The Manufacturer must, in contracting with authorized treatment centers that administer a Model Drug, ensure that the providers involved in administration of Model Drugs discuss with beneficiaries the likely infertility caused by the gene therapy treatment protocol and the range of potential reproductive outcomes and risks associated with fertility preservation.

9. The Manufacturer must adhere to all transparency and records retention requirements regarding fertility preservation services as specified in the PA.

10. The Manufacturer must provide periodic reports to CMS that demonstrate compliance with CMS' requirements included in the PA. These reports will be part of CMS' monitoring of Manufacturer activities. CMS will provide additional specification regarding the format, required elements, and frequency of these reports in the PA.

11. The Manufacturer must retain records regarding the provision of and payment for fertility preservation services to an individual beneficiary who receives fertility preservation services through this Model and must be made available to the beneficiary upon request. Additional requirements regarding monitoring and duration of record retention will be specified in the finalized PA.

In their response to this RFA, the Manufacturer must provide details regarding their proposed scope of fertility preservation services, including but not limited to: the length of time (within five to fifteen years) that cryopreservation and storage will be financially supported, the maximum number of egg or sperm retrieval cycles that will be financially supported for beneficiaries, how fertility preservation clinics/providers will be identified, a plan for providing transportation, lodging, and meals (as necessary), planned beneficiary education and disclosure regarding financially supported fertility preservation services, a plan for ensuring that authorized treatment centers that administer their Model Drug discuss with beneficiaries the likely infertility caused by the gene therapy treatment protocol, and the range of potential reproductive outcomes and risks associated with fertility preservation. The Manufacturer may not, through this Model, provide payment for in-vitro fertilization (IVF), and should not include proposals related to the provision of IVF in their response to this RFA.

As stated in Section 1.4, CMS-sponsored safe harbor protections for activities in this Model apply solely to the Cell and Gene Therapy Access Model. The Manufacturer is not permitted to engage in additional contractual relationships related to the provision of fertility preservation services for the Model population outside of those specified in the finalized PA. Should CMS learn of additional contractual relationships (related to the provision of fertility preservation services for the Model population) outside

of those specified in the finalized PA, then the arrangement will be ineligible for safe harbor protection under the "CMS-sponsored model" safe harbor at 42 CFR § 1001.952(*ii*) and may be subject to additional action by CMS or other federal entities.

2.5.5 Access Policy

The Manufacturer must describe in detail a proposed State access policy in their response to this RFA. This response should include proposed prior authorization policies for the Model Drug, any utilization management processes, provider qualifications, and eligibility for the Model Drug. The access policy described in the Key Terms will be standardized across all participating States and all included beneficiaries. Participating States may create their own criteria and policies so long as they are no more restrictive than the standardized access policy described in the Key Terms.

- In their response to this RFA, the Manufacturer may propose criteria for States that chooses their Model Drug as the only preferred drug. The Manufacturer may propose the same or other criteria if a participating State chooses to prefer that manufacturer's Model Drug as well as other Model Drugs. The Manufacturer should include in their response to this RFA information regarding additional contract provisions that will be available to participating States that grant preferential status to that Manufacturer's Model Drug in either scenario.

2.5.6 CMS Responsibilities

CMS' responsibilities will be specified in the Key Terms and in the PAs and SAs. At a minimum, CMS will be responsible for compiling, monitoring, and analyzing data necessary to support the Model, including utilization data, claims data, clinical records, and PROMs.

Sources of data utilized by CMS will include, but are not limited to:

- The Transformed Medicaid Statistical Information System (T-MSIS) for utilization and claims information.
- Patient registries, such as the Center for International Bone Marrow Transplant and Research (CIBMTR), for clinical information that is not captured on patient claims.
- Patient-level data provided by participating Manufacturers, such as the name of the beneficiary, the date the Model Drug was shipped to the treatment center, and the date of administration.

If the Manufacturer proposes Outcome Measures in their response to this RFA that rely upon data other than those described in this subsection, then the Manufacturer must fully describe such data and propose a) what the source of that data will be, and b) how that data would be acquired and received by CMS.

2.5.7 Rebate Documentation & Reconciliation

The Key Terms must clearly specify, for each type of rebate (Outcomes-Based Rebates, Volume-Based Rebates, and Guaranteed Rebates), what is required for acceptable Rebate Documentation, and the deadline by which Rebate Documentation must be provided to the Manufacturer from CMS. The Manufacturer must describe, for each Outcome Measure or rebate proposed in its RFA response, what is necessary for Rebate Documentation. CMS will have the responsibility of establishing, through its review of data as described in the Key Terms, whether Outcome Measure Benchmarks or volume

thresholds have been met for an applicable performance assessment and will be responsible for the transmission of Rebate Documentation to the Manufacturer on behalf of participating States.

For the purposes of reconciliation of all rebates related to the Model, CMS will provide Rebate Documentation to both the relevant participating State and participating Manufacturer pursuant to the timeline agreed upon in the Key Terms. Participating States and Participating Manufacturers shall have 45 business days after the transmission of Rebate Documentation to review and evaluate the Rebate Documentation. Within those 45 days, either party may notify CMS to assert that there is a deficiency in the Rebate Documentation or to question CMS's conclusions regarding the amount of rebate that is payable under the terms of the State VBP SRA. Parties may dispute based upon factors such as whether the Rebate Documentation was properly completed, or the presence of data errors. However, Participants may not dispute the underlying rebate methodology, as agreed upon in Key Terms. If neither the participating State nor participating Manufacturer objects to the Rebate Documentation within the 45 days, then the conclusions of CMS are accepted.

In the event that a party believes there is a deficiency in the Rebate Documentation, either the participating State or the participating Manufacturer may request from CMS supporting documentation and additional information used to produce the Rebate Documentation, to the extent allowed by law and otherwise not prohibited to be shared with Model Participants (see Section 5.2). Furthermore, either party may request a meeting to include participants from CMS, the participating State, and the participating Manufacturer, to resolve any discrepancy. In the event that the deficiency is not resolved following a review of supporting documentation or these consultations, then the participating Manufacturer and participating State may advance to the state-based dispute resolution process outlined in their VBP SRA, such as the Medicaid Drug Rebate Dispute Resolution Program (DRP). The Manufacturer may comment on the reconciliation process described above in their response to this RFA. The PA resulting from this RFA will include language finalizing the interim and final reconciliation processes.

2.6 Changes to Model Design in Current or Future Model Years

CMS retains the right to modify any Model policy or parameter on an annual basis, or more frequently, in accordance with procedures to be agreed upon in the PA and SAs.

2.6.1 Modification of Key Terms

CMS understands that participating States may have nuanced and individualized contracting processes that may require, among other accommodations, annual renewals for the VBP SRA. These annual renewals are allowable under the Model, as long as the Key Terms are adopted at each renewal (barring exceptions as described in Section 5.1). The Manufacturer must agree to offer the Key Terms, as agreed by the Manufacturer and CMS, each year to States for the duration of the OBA Term as a term of Model participation.

CMS and Manufacturers will negotiate standard language regarding termination and renewals of the Key Terms, and the Manufacturer may propose such terms in their response to this RFA. The Manufacturer will agree to offer the Key Terms to States, subject to annual VBP SRA renewals, during the OBA Term. A

participating State may include additional language regarding termination and renewals in their VBP SRA as required by State laws or regulations.

The Key Terms will specify the circumstances in which renegotiation would occur (e.g., changes in the FDA labeling, new clinical evidence). If renegotiation between CMS and the manufacturer results in prospective change to the Key Terms, participating States would have an opportunity to execute new VBP SRAs any participating Manufacturer or terminate Model participation with respect to future performance years.

2.6.2 Termination

The PA resulting from this RFA shall commence on January 1, 2025, and continue until the end of the Model on December 31, 2035, subject to earlier termination as provided for in the PA. CMS reserves the right to terminate a participating Manufacturer's PA at any point during the Model for reasons associated with poor performance, new safety or efficacy data regarding the Model Drug, program integrity issues, non-compliance with the terms and conditions of the applicable PA, or as otherwise specified in the PA or required by Section 1115A(b)(3)(B) of the Social Security Act. A participating Manufacturer may voluntarily terminate their PA and participation in the Model, subject to terms that will be outlined in the PA.

3. Quality and Performance Monitoring

As part of both Model implementation and evaluation, CMS will monitor the impacts of the Model on the Medicaid program and separate CHIP (as applicable) spending and quality. Specifically, CMS will monitor the Model's impact on beneficiary access to Model Drugs, beneficiary access to other types of care relevant to Model Drugs, beneficiary health outcomes, beneficiary experience, and any potential impacts on affordability and adherence due to the Model.

CMS will collect additional information regarding beneficiary claims, clinical outcomes, and PROMs beyond what is necessary to monitor Outcome Measures throughout the Model. This information will be used to monitor and evaluate the performance of the Model and will not be tied to the VBP SRAs. The Innovation Center reserves the right to monitor and validate information and data submitted by Model participants to the Innovation Center for the purposes of either Model implementation or Model evaluation. Model participants will be required to comply with any and all monitoring activities and validation efforts as part of their Model participation. The data sharing requirements of Model participation will be detailed in full in the PAs and SAs.

3.1 Enrollee Protections and Oversight

CMS will conduct regular monitoring to review Model participant compliance with the terms of the Model, particularly related to beneficiary quality of care. CMS will monitor for compliance using existing data sources to the extent practicable, and may seek additional information from Model participants, particularly in the even that CMS receives a high number of complaints or other indicators of poor performance. CMS expects Model participants to cooperate to the fullest extent possible in requests for relevant data and information. CMS will closely monitor Model implementation to ensure that performance is consistent with Model parameters. CMS will also monitor the impact the Model has on other CMS initiatives. CMS reserves the right to investigate a participating State or participating Manufacturer if there is evidence that indicates that participation in the Model is adversely impacting enrollee quality of care or failure to provide required information and exercise all available remedies in appropriate instances, including potential termination from the Model.

4. Evaluation

CMS will use an independent contractor to conduct an evaluation of the Model, which will examine the Model's implementation and assess the Model's impact on Medicaid program and CHIP spending and the quality of care. All Model participants will be required to participate in any evaluation activities if requested. CMS anticipates primarily relying on the data sources also utilized in adjudicating rebates in the evaluation of the Model.

In certain situations, Model participants will be required to cooperate with primary data collection activities, which may include participation in surveys, interviews, site visits, and other activities that CMS determines necessary to conduct a comprehensive formative and summation evaluation. When the evaluation uses non-publicly available data, only aggregated results would be reported. CMS does not anticipate that confidential, commercially valuable information will be used in the evaluation.

5. Application

5.1 Application Process and Selection

Manufacturers seeking to participate in the Model must complete and submit the application template in Appendix A in either PDF or Word format by 11:59pm EDT on May 1, 2024, according to the instructions provided in Appendix A. CMS will acknowledge receipt of the application to the Primary Application Contact (see Appendix A) and will, within 45 days of the submission response to this RFA by an eligible Manufacturer, respond to the Manufacturer with a request for a meeting and, if applicable, a counterproposal to the Key Terms submitted by the Manufacturer. All eligible Manufacturers that submit a response to this RFA will be individually invited to participate in Key Term negotiations with CMS, and, if, upon conclusion of negotiation an agreement is reached, will be selected as a Model Participant.

If an agreement is reached with a Manufacturer, CMS will enter into a PA with the Manufacturer and a fully executed PA must be completed on or before November 29, 2024. As stated in Section 2.2.2, States will be required to adopt the Key Terms from January 1, 2025, to January 1, 2026, and must sign a SA with CMS by January 1, 2026. States that do not join the Model by January 1, 2026, will not be allowed to participate in the Model except at CMS discretion.

If additional Manufacturers receive FDA approval for gene therapies for SCD after May 1, 2024, CMS may open a new application cycle to allow eligible Manufacturers to participate in negotiation with CMS. In the future, CMS may release an additional RFA pertaining to other conditions for manufacturers that market FDA-approved gene therapies for such conditions. Additional manufacturers may be eligible to participate in negotiation under this Model with CMS and join the Model at that time.

5.1.1 Meetings between CMS and Manufacturers

Representatives of CMS and Manufacturers may meet as needed, subject to agreement between parties, between the submission of the Manufacturer's response to this RFA and November 29, 2024, to discuss the Manufacturer's application or subsequent offers provided by either CMS or the Manufacturer. After the Manufacturer has submitted a response to this RFA, either CMS or the Manufacturer may, at any point, request an in-person, virtual, or hybrid meeting prior to the end of the Model pre-implementation period.

5.2 Rights in Data and Intellectual Property

CMS may use any data obtained pursuant to the Model to evaluate the Model and to disseminate quantitative results to State Medicaid Agencies and to the public. Data to be disseminated may include savings information, results of beneficiary experience of care and quality of life surveys, as well as measures based upon claims and medical records. Participating States and participating Manufacturers will be permitted to comment on evaluation reports for factual accuracy, where appropriate, but may not edit conclusions or control the dissemination of reports.

Notwithstanding any other provision in the PA, all proprietary trade secret information and technology of the Manufacturer is, and shall remain, the sole property of the Manufacturer and, except as required by federal law, shall not be released by CMS without express written consent. The regulation at 48 CFR

§ 52.227-14, “Rights in Data-General” is hereby incorporated by reference into this RFA. CMS does not acquire by license or otherwise, whether express or implied, any intellectual property rights or other rights to the Manufacturer’s proprietary information or technology.

If the Manufacturer maintains any information that should not be publicly disclosed because the Manufacturer considers such information to be proprietary and confidential, the Manufacturer should submit to CMS a form, using either the template attached as Appendix B, or a form substantially the same as Appendix B, identifying specific examples of information the Manufacturer considers to be proprietary and confidential. The Manufacturer must notify CMS, in a form and manner to be specified by CMS, of any updates to this form. If the participating Manufacturer does not submit such a form, it will be deemed to be confirmed that the Manufacturer has no information in its response to this RFA it considers proprietary and confidential.

5.3 Submission Information

Information required by CMS in response to this RFA regarding the Key Terms and parameters of Model participation is included in Appendix A. While Appendix A includes the minimum information required per this RFA, Manufacturers may, at their discretion, include additional information or Key Terms they wish to present to CMS.

5.4 Model Timeline

A summary of the Model's timeline is provided below:

Milestone	Target Date
Release of State Letter of Intent Template (LOI)	January 30, 2024
Release of Manufacturer Request for Applications (RFA)	March 1, 2024
State LOIs due (optional, non-binding)	April 1, 2024
Manufacturer Applications due	May 1, 2024
CMS—Manufacturer Negotiations Ongoing	May 2, 2024 – November 29, 2024
Release of State RFA and Notice of Funding Opportunity (NOFO)	Summer 2024
CMS Acceptance/Rejection of Manufacturer Final Offers (PA is signed)	November 29, 2024
Announcement of Participating Manufacturers & Disclosure of CMS-Manufacturer Negotiated Key Terms to States	December 2024 (no later than)
OBA Term begins	January 1, 2025
State RFA Applications due	February 28, 2025 (rolling)
State NOFO Applications due	February 28, 2025
Cooperative Agreement Funding begins	June or July 2025
Publication of First Public Evaluation Report	Q2 of 2027 (Negotiation Report)
Latest Date of OBA Term	December 31, 2030
End of Model Evaluation Period	No later than Q3 2035

5.5 Withdrawal of Application

Prior to 11:59 pm EDT November 29, 2024 a Manufacturer that submitted an application may withdraw from participating in the pre-implementation period by submitting a written request on the organization's letterhead that is signed by one of the following: (1) the chief executive officer (CEO) of the Manufacturer, (2) the chief financial officer (CFO) of the Manufacturer, (3) an individual other than a CEO or CFO, who has authority equivalent to a CEO or a CFO, or (4) an individual with the directly delegated authority to perform the certification on behalf of one of the individuals mentioned in (1) through (3).

To submit a withdrawal request, the Manufacturer must send the request in a PDF format by email to CGTModel@cms.hhs.gov.

Appendix A: Application Template

The Centers for Medicare & Medicaid Services (CMS) is seeking applications for a voluntary Model (the Cell and Gene Therapy Access Model, or "the Model") that tests whether a CMS-led approach to developing and administering outcomes-based agreements (OBAs) for cell and gene therapies improves Medicaid beneficiary access to innovative treatment, improves health outcomes for Medicaid beneficiaries, and reduces health care expenditures.

CMS will safeguard the information provided in submitted applications in accordance with the Privacy Act of 1974, as amended (5 U.S.C. § 552a).

CMS provides no opinion on the legality of any contractual or financial arrangement that the applicant may disclose, propose, or document in this application. The receipt by CMS of any such information during the application process or otherwise shall not be construed as a waiver or modification of any applicable laws, rules, or regulations, and will not preclude CMS, the Department of Health and Human Services (HHS), the HHS Office of Inspector General, a law enforcement agency, or any other federal or state agency from enforcing any and all applicable laws, rules, and regulations.

CMS will provide Manufacturers with a secure platform where the completed application template and supporting documents must be submitted. Manufacturers must contact CMS at CGTModel@cms.hhs.gov to receive instructions regarding accessing the secure application platform and may do so at any time following the release of this RFA.

Manufacturers seeking to participate in the Model must submit the completed application template and any supporting documents in PDF or Microsoft Word format by 11:59pm EDT on May 1, 2024. Text responses in the application template are limited to no more than 1,000 words for each response.

Questions about the application for the Model should be directed to CGTModel@cms.hhs.gov.

I. BACKGROUND INFORMATION

a. Applicant Company Information

Please provide the following information regarding your company. The information provided in this application must be the same information as provided for participation in the Medicaid Drug Rebate Program (MDRP).

FIELD	RESPONSE FORMAT
Manufacturer Name:	*Text*
Doing Business As (DBA):	*Text*
Prior DBA(s) if applicable:	*Text*
Organization TIN/EIN:	*Text*
Organization DUNS:	*Text*
Website, if applicable:	*Text*
MAILING ADDRESS:	
Street Address:	*Text*
City:	*Text*

State:	*Text*
ZIP Code:	*Text*

b. Contact Information

Please include information for the Primary Application Contact and Secondary Application Contact. These two individuals will be whom CMS will contact to confirm receipt, direct follow up questions, and schedule meetings regarding the Model. The primary means of communication will be via E-mail.

For Primary Application Contact

FIELD	RESPONSE FORMAT
Full Name:	*Text*
Title/Position:	*Text*
Business Phone Number:	*Text*
Business Phone Number Extension:	*Text*
Alternate Phone Number:	*Text*
Email Address:	*Text*

For Secondary Application Contact

FIELD	RESPONSE FORMAT
Full Name:	*Text*
Title/Position:	*Text*
Business Phone Number:	*Text*
Business Phone Number Extension:	*Text*
Alternate Phone Number:	*Text*
Email Address:	*Text*

c. Product Information

FIELD	RESPONSE FORMAT
Proprietary Name of Model Drug	*Text*
Generic Name of Model Drug	*Text*
FDA Submission Tracking Number(s) (STN)	*Text*
NDC(s)	*Text*

II. **DURATION:** Please describe the proposed length of the OBA Term. The OBA Term is the time period for which the Key Terms are applicable. The Manufacturer may propose a duration of the OBA Term, so long as that term is less than 6 years, and no related obligations extend beyond 2035.

RESPONSE FORMAT
Text

III. **MODEL REBATES:** Please refer to the templates on the three following pages to describe the rebates the Manufacturer proposes as part of the Model. The Manufacturer must offer Outcomes-Based Rebates (Template 1), and either Volume Rebates (Template 2), or Guaranteed Rebates (Template 3), or both Volume Rebates and Guaranteed Rebates. The Manufacturer may offer multiple rebates of any of these three types. Please copy and repeat the templates as necessary to provide information on all proposed rebates.

SUPPLEMENTAL REBATE TEMPLATE 1: OUTCOME-BASED REBATES

<table>
<tr><th colspan="3">REBATE [#]</th></tr>
<tr><td colspan="3">Outcome Measure Description:</td></tr>
<tr><td colspan="3">Outcome Measure Specification (please include information regarding numerator, denominator, inclusion and exclusion criteria, as applicable):</td></tr>
<tr><td colspan="3">REBATE TYPE</td></tr>
<tr><td>Applies at the individual/per-patient level.

Yes [ ]
No [ ]</td><td>Applies at a population level:

Yes [ ]
No [ ]</td><td>Other (please specify):</td></tr>
<tr><td colspan="3">If the measure is based on outcomes achieved at a population/cohort level, please define the cohort:</td></tr>
<tr><td colspan="3">MEASUREMENT PERIOD</td></tr>
<tr><td colspan="3">The Measurement Period shall encompass [#] calendar quarters. It shall commence on [DAY/WEEK/QUARTER] following administration of the Model Drug. It shall conclude on [INSERT].</td></tr>
<tr><td colspan="3">OUTCOME BASED BENCHMARKS</td></tr>
<tr><td colspan="3">Please list and fully describe the outcomes-based benchmarks that will be used to evaluate the performance of the Model Drug. Describe the timing of interim and final performance assessments.</td></tr>
<tr><td>CALCULATION TYPE</td><td colspan="2">REBATE PER UNIT</td></tr>
<tr><td>{Specify WAC, GNUP, AMP other}</td><td colspan="2">%, $, other</td></tr>
<tr><td colspan="3">MAXIMUM REBATE AMOUNT</td></tr>
<tr><td colspan="3">No rebate per-unit is to exceed:</td></tr>
<tr><td colspan="3">COVERAGE SHIFTS</td></tr>
<tr><td colspan="3">Please describe (if necessary) the proposed approach to account for patients for whom data is unavailable:</td></tr>
<tr><td colspan="3">REBATE DOCUMENTATION</td></tr>
<tr><td>Please check all applicable data sources:

Claims Paid [ ]
Electronic Medical Records [ ]
Patient Registry [ ]
Other [ ]</td><td colspan="2">Please describe what is required for Rebate Documentation:</td></tr>
<tr><td colspan="3">Please include additional narrative here as necessary. Attachments supporting this narrative, or the information above may be referenced, but should not exceed 20 pages per measure.</td></tr>
</table>

SUPPLEMENTAL REBATE TEMPLATE 2: VOLUME REBATES

REBATE [#]		
Description (please include information regarding numerator, denominator, and inclusion and exclusion criteria, as applicable):		
POPULATION DEFINITION		
Volume rebates will accrue across participating states: *Yes [ ]* *No [ ]*		
Further specify the population/cohorts, as necessary:		
VOLUME ACCRUAL PERIOD		
The Volume Accrual Period shall encompass [#] calendar quarters. It shall commence on [DATE]. It shall conclude on [DATE].		
VOLUME THRESHOLD	**CALCULATION TYPE**	**REBATE PER UNIT**
Please identify the applicable volume thresholds (e.g., 1-50 units, 51-100, etc.).	*{Specify WAC, GNUP, AMP other}*	*%, $, other*
MAXIMUM REBATE AMOUNT		
No rebate per-unit is to exceed:		
REBATE DOCUMENTATION		
Please check all applicable data sources: *Claims Paid [ ]* *Electronic Medical Records [ ]* *Patient Registry [ ]* *Other [ ]*	*Please describe what is required for Rebate Documentation:*	
Please include additional narrative here as necessary. Attachments supporting this narrative, or the information above may be referenced, but should not exceed 20 pages.		

SUPPLEMENTAL REBATE TEMPLATE 3: GURANTEED REBATES

<table>
<tr><th colspan="2">REBATE [#]</th></tr>
<tr><td colspan="2">Description (please include information regarding numerator, denominator, and inclusion and exclusion criteria, as applicable):</td></tr>
<tr><th colspan="2">FORMULA</th></tr>
<tr><td colspan="2">Please specify the full proposed Guaranteed Rebate formula.</td></tr>
<tr><th colspan="2">% OF WAC (IF APPLICABLE)</th></tr>
<tr><td colspan="2">Please specify the % of WAC</td></tr>
<tr><th colspan="2">CONTRACTED RATE (IF APPLICABLE)</th></tr>
<tr><td colspan="2"></td></tr>
<tr><th colspan="2">INFLATION PROVISIONS</th></tr>
<tr><td colspan="2">Please describe adjustments (if any) or additional provisions to adjust the Guaranteed Rebate due to either inflation, changes to WAC, or other conditions, and the timing and frequency of such adjustments.</td></tr>
<tr><th colspan="2">REBATE DOCUMENTATION</th></tr>
<tr><td>Please check all applicable data sources:

Claims Paid [ ]
Electronic Medical Records [ ]
Patient Registry [ ]
Other [ ]</td><td>Please describe what is required for Rebate Documentation:</td></tr>
<tr><td colspan="2">Please include additional narrative here as necessary. Attachments supporting this narrative, or the information above may be referenced, but should not exceed 20 pages.</td></tr>
</table>

IV. ADDITIONAL REBATES

a. Please specify any additional supplemental rebates proposed to be offered as part of the Model that are not otherwise specified in this application.

CALCULATION TYPE	REBATE PER UNIT	NARRATIVE
{Specify Wholesale Acquisition Cost (WAC), Guaranteed Net Unit Price (GNUP), Average Manufacturer Price (AMP), other}	*%, $, other*	*Please fully describe the additional rebate, and how the additional rebate would relate to other proposed rebates.*

V. FERTILITY PRESERVATION SERVICES

a. Please fully describe the scope of fertility preservation services the Manufacturer will financially support for beneficiaries in the Model.

RESPONSE FORMAT
Text

b. Please specify the number of years of storage of cryopreserved reproductive material (between five and fifteen years) that the Manufacturer will financially support as part of the fertility preservation services offered to each beneficiary in the Model.

RESPONSE FORMAT
Text

a. Please specify the number of cycles of egg or sperm retrieval that the Manufacturer will financially support as part of the fertility preservation services offered to beneficiaries in the Model.

RESPONSE FORMAT
Text

b. Please describe the proposed Manufacturer process for funding and disseminating beneficiary education and disclosure related to fertility preservation services financially supported by the Manufacturer in the Model.

RESPONSE FORMAT
Text

c. Please describe how the Manufacturer will engage a third-party vendor to contract with fertility preservation clinics and providers to the Model population. Describe how the Manufacturer will ensure compliance and integrity to the requirements of the Model.

RESPONSE FORMAT
Text

d. Please describe the proposed Manufacturer plan for ensuring that beneficiaries under the Model receive an offer of fertility preservation prior to undergoing myeloablative conditioning.

RESPONSE FORMAT
Text

e. Please describe the process for providing beneficiaries with financial support for transportation, food, and lodging, as necessary, to receive fertility preservation services.

RESPONSE FORMAT
Text

f. Please describe the proposed Manufacturer plan for ensuring that treatment centers and providers that administer a Model Drug within the Model provide appropriate education and discuss with beneficiaries the likely infertility caused by gene therapy and the range of potential outcomes associated with fertility preservation. Please describe how the Manufacturer will monitor treatment center compliance with this Model requirement.

RESPONSE FORMAT
Text

VI. ACCESS POLICY

a. Please describe your proposed access policy that participating States would adopt as part of the Model. This may include criteria for eligibility for the Model Drug; step therapy; other terms for prior authorization. Describe how the proposed access policy compares to the FDA-approved labeling and pivotal clinical trial inclusion criteria and provide a rationale for any differences.

RESPONSE FORMAT
Text

a. Please describe your proposed policy for rebates associated with beneficiaries who lose eligibility for Medicaid after receiving the Model Drug but before collection of all relevant outcomes data for each performance assessment. Please propose policies for the following scenarios: 1) the individual is no longer a Medicaid beneficiary of the original participating State, but transfers to another form of coverage for which CMS receives claims data (i.e., Medicaid or Medicare); 2) the individual transfers to commercial coverage, but information regarding the individual's health care utilization and/or patient registry information continues to be accessible to CMS; and 3) the individual has a change in coverage that renders information regarding the individual's health care utilization and/or patient registry information no longer fully accessible to CMS.

RESPONSE FORMAT
Text

VII. REBATE DOCUMENTATION & RECONCILIATION

a. Please include any comments regarding the reconciliation process described in the RFA.

RESPONSE FORMAT
Text

VIII. PREFERRED STATUS

a. Please describe any additional financial arrangements or alterations to the responses to questions III – V that would apply if the Manufacturer's Model Drug is granted Preferred Status by a participating State. The Manufacturer may attach additional versions of Supplemental Rebate Template 1, 2, or 3 if necessary. The Manufacturer may also include alternatives as applicable throughout their responses to questions III – V.

RESPONSE FORMAT
Text

IX. ADDITIONAL PAYERS

a. Is the Manufacturer interested in negotiating Key Terms for the separate Children's Health Insurance Program (CHIP)? If yes, please provide as a separate attachment a value-based arrangement proposal for this population, utilizing the following elements of Appendix A: Question II, Supplemental Rebate Template 1, Questions V, VI, VII, VIII, IX, XI, XII, XIII, XIV.

Yes [ ] No [ ]

X. TERMINATION, RENEWALS, RENEGOTIATION OR ALTERATIONS

a. Please review the language below regarding the duration and renewals of the Model participation agreement between CMS and Manufacturer.

"This Agreement shall commence on January 1, 2025, and continue thereafter until December 31, 2025, and shall thereafter automatically renew for additional one (1) year terms unless either Party provides written notice of nonrenewal at least sixty (60) days prior to the end of any renewal term, until the end of the Model on December 31, 2035, subject to earlier termination as provided herein.

CMS, or the Manufacturer, may terminate this Agreement upon written notice to the other Party: (i) if the other Party breaches any term of this Agreement and such breach is not cured within sixty (60) days of written notice thereof; or (ii) if the Manufacturer files a petition in bankruptcy, is adjudicated bankrupt, makes a general assignment for the benefit of its creditors, or is voluntarily or involuntarily dissolved."

b. Is this language regarding duration and renewal of the contract resulting from this RFA acceptable?

RESPONSE FORMAT
Yes [ ] No [ ]

c. If no, please propose alternative language:

RESPONSE FORMAT
Text

XI. ADDITIONAL INFORMATION

a. Please describe the patient and community support that is offered to patients without federal health insurance (i.e., who are commercially insured or uninsured) who begin and/or complete treatment with the Model Drug.

RESPONSE FORMAT
Text

b. Do you currently offer monetary patient support? If so, please describe. For each type of support, please clarify if it is restricted by payor (i.e., only available to patients with or without certain types of insurance coverage).

RESPONSE FORMAT
Text

c. Do you provide non-monetary patient support? If so, please describe. For each type of support, please clarify if it is restricted by payor.

RESPONSE FORMAT
Text

d. Please describe the distribution process for your gene therapy product. Please describe the roles of each entity in the distribution process and the nature of financial relationships among those entities.

RESPONSE FORMAT
Text

e. Does the distribution process for your gene therapy product support financing options, such as payment-over-time or payment after successful engraftment, for hospitals and payers? If yes, please describe the options that may be available to states.

RESPONSE FORMAT
Text

f. Does the distribution process for your gene therapy product support an arrangement between a specialty pharmacy (or other distributor) and state in which the Model Drug is sold to that specialty pharmacy (or other distributor) rather than directly to the hospital at which administration of the gene therapy would occur? If yes, please describe.

RESPONSE FORMAT
Text

XII. **SUPPLEMENTAL INFORMATION:** Please submit additional materials, including but not limited to: clinical trial data, white papers, and patient or provider testimonials, that you believe support your proposal. Supplemental information is not to exceed 50 pages.

XIII. **ADDITIONAL KEY TERMS (OPTIONAL):** If desired, please describe any additional Key Terms that you would like to propose to CMS for consideration. Additional Key Terms are not to exceed more than 10 pages.

XIV. SIGNATURE

g. An individual eligible to certify this submission on behalf of the Manufacturer must be one of the following: (1) the chief executive officer (CEO) of the Manufacturer, (2) the chief financial officer (CFO) of the Manufacturer, (3) an individual other than a CEO or CFO, who has authority equivalent to a CEO or a CFO, or (4) an individual with the directly delegated authority to perform the certification on behalf of one of the individuals mentioned in (1) through (3).

I hereby certify, to the best of my knowledge, that the information being sent to CMS in this submission is complete and accurate, and the submission was prepared in good faith and after reasonable efforts. I reviewed the submission and made a reasonable inquiry regarding its content. I also certify that I will timely notify CMS if I become aware that any of the information submitted in this form has changed. I also understand that any misrepresentations may also give rise to liability, including under the False Claims Act.

Yes [ ]
No [ ]

[DATE]
[Signature block]

Appendix B: Manufacturer Proprietary and Confidential Information

The following are specific examples, without limitation, of what the Manufacturer considers proprietary and confidential information currently maintained by the Manufacturer that should not be publicly disclosed:

1)

2)

3)

In accordance with Section 5.2 of the RFA, this information shall remain the sole property of the Manufacturer and, except as required by federal law, shall not be released by CMS without the express written consent of the Manufacturer.