

Cell & Gene Therapy (CGT) Access Model Overview Webinar
February 6, 2024

>>**Arbre'ya Lewis, SEA:** Good afternoon and thank you for joining today's CGT Access Model Overview Webinar. There are few housekeeping items I'd like to discuss before we get started.

Today's presentation is being recorded and streamed live. If you have any objections, please hang up at this time. Furthermore, all participants will be in listen-only mode. Please feel free to submit any questions you have throughout today's presentation in the Q&A pod displayed on the bottom of the meeting room window. Given time constraints, we may not get to every question, but we will collect questions for future events and FAQs. You can also reach out to the model team with questions at CGTModel@cms.hhs.gov. There will also be a short survey available at the end of the event. We would greatly appreciate if you could spend one-to-two minutes filling it out so we can improve the quality of future events. Finally, this deck, a recording of today's presentation, and a transcript will be made available on the CGT Access Model website in the coming days. Next slide, please.

Before we dive into content, let me give a brief overview of the agenda for today's event. We will begin with a welcome from Ellen Lukens, Deputy Director of the CMS Innovation Center, and an introduction of today's speakers. Then the model team will provide background information about the CGT Access Model. They will then dive into how the CGT Access Model intends to address health equity. And finally, they will discuss the model's contracting structure. Following that, they will share more information about the application process and timeline. We will have about 30 minutes for a Q&A session where our team will answer questions submitted by audience members. As a reminder, you can submit questions using the Q&A function at the bottom right-hand corner of your screen.

Again, thank you for joining us today. We've got a great presentation planned for you. Now I'm going to pass the mic to Ellen to formally welcome you to today's event. Next slide, please.

>> **Ellen Lukens, CMS:** Good afternoon, everyone. I'm Ellen Lukens, the Deputy Director of the CMS Innovation Center and I'm so excited to welcome all of you to this webinar today. I wanted to express our gratitude first, for the time and effort that so many of you have taken over the past several months to help us build this model. We've benefited so much from hearing the perspectives of the people in this virtual room, including clinicians, patients, states, manufacturers, researchers, and others. Next slide, please.

Today's speakers include myself, Jason Petroski, the Director of the Division of Drug Innovation, and the Co-Leads of the model, Aurelia Chaudhury, Melissa Majerol, and Corinne Alberts. We also have several members of the CGT Access Model team on this webinar, and they have been working passionately and tirelessly over the past several months to develop this model. We're also delighted to have our colleagues from the Center for Medicaid and CHIP Services, who have been fantastic partners to us over the past year. But next slide, please.

So I'm now going to turn the mic over to Aurelia who will start to walk through the CGT Model background. And then she will turn it over to others on the team. Thank you again for participating today.

>> **Aurelia Chaudhury, CMS:** Thanks, Ellen. Hello, everyone, my name is Aurelia Chaudhury. I'm so excited that you've chosen to join us today and I want to echo Ellen's thanks. I've personally spoken with

many of you over the past year, and I'm just so delighted to have the opportunity to engage in this webinar. As Ellen mentioned, I'll give a brief overview of the model here. So next slide, please.

Let's begin with a little history of how we got to where we are today. As some of you may recall in October of 2022, the United States President issued the Executive Order on lowering prescription drug costs for Americans, which called upon the Secretary to propose testing new health care payment and delivery models that would lower drug costs and promote access to innovative drug therapies for beneficiaries enrolled in the Medicare and Medicaid programs. In February of 2023, the Secretary released a report in response to that order, which included a brief description of the Cell and Gene Therapy Access Model.

The vision for this model is a framework in which CMS would negotiate with manufacturers of cell and gene therapies on behalf of state Medicaid agencies for what we call outcomes-based agreements. That is, agreements that, among other things, tie what the manufacturer receives in payment to the actual clinical outcomes achieved by the therapy in Medicaid beneficiaries. The goal of the Cell and Gene Therapy Access Model is to increase access to these innovative cell and gene therapies for people with Medicaid by making it easier for states to pay for these therapies. And through that improved access, lower the long-term health care expenditure trajectory of these patients and improve their health outcomes. Next slide, please.

As you all may be familiar, cell and gene therapies are an exciting and growing class of therapies, many of which are one-time treatments that hold the potential to transform the lives of patients who receive them. To help patients gain access to these therapies, CMS will negotiate outcomes-based agreements with manufacturers to try to accomplish a few things as I mentioned earlier; tying payment to achieving specific outcomes, negotiating discounted prices, and developing a broader strategy to address potential barriers to equitable access to care.

I'll turn it now to my colleague, Melissa Majerol to talk about sickle cell disease as the initial focus of the model, as well as some of the health equity aspects of the model. Next slide, please.

>> Melissa Majerol, CMS: Thanks, Aurelia.

So as Aurelia just mentioned, the initial focus for the CGT model is on gene therapies for sickle cell disease, or SCD. SCD is a condition that affects over 100,000 Americans, the majority of whom are black. As you can see from this map, SCD is heavily concentrated in certain states, mostly in the southern and eastern regions of the US. SCD can be a profoundly challenging condition associated with acute pain episodes, also known as vaso-occlusive crises, that may lead to frequent hospitalizations and other types of organ damage, such as stroke or kidney disease.

Individuals with SCD have a life expectancy, on average, that is about 20 years shorter than the general population. The impacts of the disease can create substantial challenges in the lives of patients with SCD, affecting educational attainment, employment, and the ability to participate in their communities. Patients with SCD may also experience mental health challenges related to their disease. In addition to the impact on individuals, SCD also costs the U.S. health care system a staggering nearly three billion dollars annually, much of which accrues to Medicaid because roughly 60% of individuals with SCD are enrolled in Medicaid.

As many of you are aware, this past December, the FDA approved two gene therapies for SCD: Casgevy, by Vertex and CRISPR Therapeutics, and Lyfgenia, by bluebird bio. These therapies hold the promise of substantially reducing or eliminating a patient's experience of vaso-occlusive crises and thereby dramatically improving the lives of patients with SCD. Next slide, please.

Now I'll discuss how this model plans to address health equity. Next slide, please.

So a key goal of the model is enabling states to improve equitable access to gene therapies for SCD. The model will accomplish this in three key ways. First, through the CMS facilitated manufacturer negotiation process, we hope to be able to secure more favorable pricing that meaningfully ties payment to beneficiary outcomes. And this creates space for states to provide more expansive coverage for these therapies.

Second, through a Notice of Funding Opportunity, we will be offering states optional funding for activities that reduce access barriers for patients with SCD. We'll also be requiring participating manufacturers to pay for the costs of a defined scope of fertility preservation services. And this is because the care journey for gene therapy for sickle cell disease typically results in infertility for patients, and the out-of-pocket costs of fertility preservation services can present a significant barrier.

Third, by improving access to gene therapies for SCD, we hope to address health disparities that have historically affected the SCD community and have been associated with poor health outcomes and lower life expectancy. Next slide, please.

Now I'm going to turn it over to my colleague, Corinne Alberts, to discuss the model contracting structure. Next slide.

>> **Corinne Alberts, CMS:** Thank you, Melissa.

So as Aurelia began to describe, the model framework is one in which CMS negotiates the key terms of an outcomes-based arrangement with manufacturers of gene therapies for sickle cell disease. These key terms would include the pricing structure, reflecting both the outcomes-based components, and other types of discounts and coverage criteria for the product. States would then decide whether or not they wanted to sign to a deal with those key terms. The model is voluntary for both manufacturers and states.

In order for states to participate, a state or a territory must participate in the Medicaid Drug Rebate Program, or MDRP, and meet the requirements of model participation, which will be discussed in much more depth in our state webinar held later this week. The state would also then be required to sign a State Agreement with CMS. In order for manufacturers to be eligible participants, a manufacturer has to also participate in the MDRP, market an FDA approved or licensed gene therapy for sickle cell disease, and sign a Participation Agreement with CMS that reflects our negotiated arrangement.

CMS will play a few key roles throughout this process, including on the front end by negotiating an overall set of key terms that reflects a pricing structure that includes discounted pricing as well as tying payments to outcomes. And as Melissa mentioned, CMS will offer states optional funding to support activities that will promote equitable access to care. And also, through supporting states and operationalizing the model both through technical assistance to states and through collecting, analyzing,

and reconciling data required to adjudicate the amounts that are owed under these outcome-based agreements. Next slide, please.

The structure of our model involves three key relationships: between CMS and the manufacturer, between the manufacturer and participating states, and between CMS and the states. As was mentioned, first CMS will negotiate the key terms of an outcomes-based arrangement with the manufacturer, which the manufacturer would then turn around and make available to states CMS would, for any state that takes up the manufacturer on that offer, support the data collection and analysis related to the outcomes-based agreements, including collecting data from manufacturers to support that work.

Second, CMS would have an arrangement with the states, where-in states would provide data to CMS, relying as much as possible on existing data flows like the current T-MSIS system. As mentioned, CMS will provide technical assistance and funding to states to support their participation in the model, and help states address access barriers. States would, just like they do today, be responsible for their share of funding the cost of these therapies, although this would be at net prices negotiated by CMS under the key terms.

Third, manufacturers and states that choose to participate would enter into their own agreement, a Supplemental Rebate Agreement, reflecting the key terms negotiated by CMS. States and manufacturers would have the option of including CHIP populations in this agreement, subject to certain unique considerations, that can be discussed in the state webinar. Both states and manufacturers would have to abide by these key terms both in terms of the manufacturers' obligation to provide access to discounted pricing and in terms of state obligations to cover the therapy according to the access policy negotiated under the key terms and meet the other requirements of participation in the model. We can go to the next slide, please.

I'm now going to pass it back to Melissa to discuss key dates in the application timeline for the model.

>> **Melissa Majerol, CMS:** Thanks, Corinne. Let's go to the next slide, thank you.

Okay, so our goal is for states to have the option of beginning participation in the model as soon as January of 2025. However, states are welcome to begin participation on a rolling basis throughout 2025 and up until January 1, 2026. There are several milestones that will happen between now and then.

First, in early March, we plan to release the manufacturer Request for Applications, which will be due on May 1st. This will kick off the negotiation process between CMS and manufacturers. We expect the negotiation process to conclude by no later than November 2024, with signed Participation Agreements with manufacturers by late November or early December.

We have recently released to states a non-binding Letter of Intent, which is due on April 1st. And while not required, we strongly encourage states to submit to CMS because it will help inform the manufacturer negotiation process.

This summer, we will release the State Request for Applications, which will outline state requirements for participation in more detail. And in December 2024, we will disclose to states the results of the negotiation process. States that want to participate in the model are required to respond to the State Requests for Applications by February 28, 2025 and can begin performance in the model anytime

between, again, January 2025 and January 2026. State Agreements will be signed between December 2024 and June 2025.

There will also be an optional Notice of Funding Opportunity, which we will plan to release this summer, with applications accepted between December 2024 and February 2025. Funding will be available as early as June or July 2025. This general timeline allows for state and manufacturer supplemental agreements under the model to go into effect as early as January 1, 2025. Next slide, please.

We encourage anyone interested to go to our website, to view our fact sheet, read additional information about the model, or contact the model email address with any additional questions or comments. And again, we also encourage states to submit Letters of Intent and to attend the state webinar, which is being held on Thursday at noon Eastern Time. States should have received an email with a link to register. If you haven't, you can contact the model email address and ask for a registration link. Next slide, please.

I'll now pass it to Jason Petroski, who will moderate the Q&A section of the webinar. Next slide.

>> **Jason Petroski, CMS:** Thank you, Melissa.

Before we start the Q&A session, we did want to take a brief moment and ask everybody that's participating in the call, please complete the following survey. And Arbre'ya just put a link to that survey in the chat, so everyone can take a moment and please respond to that. Also, if you have additional input or, other, information or feedback on this session, can also send that to the CGT model team at CGTModel@cms.hhs.gov. And we wanted to thank everybody again in advance for taking the survey and giving feedback. Next slide, please.

So for the remainder of the call, our team will be collectively responding to questions submitted before and during the course of today's event. As a reminder, due to the high volume of the attendance, we may not be able to get to each and every question. We will take notes of each of the questions and try to ensure future materials help address any common themes. Also, as I mentioned, if you have additional questions or feedback, again, you can please submit it to the email address listed here on the screen.

Okay, so let's go ahead and get started with the question and answer part of the webinar. So, what I'll be doing is I'll be putting out the question and I'll be turning to our co-leads to help give responses to each of these questions. And I think we'll start with a funding question, this one I'll throw to Melissa.

Melissa, will the federal government provide additional funding support if states decide to participate in the CGT model?

>> **Melissa Majerol, CMS:** Thank you, Jason.

Yes, so CMS is going to be providing optional funding to states through a Notice of Funding Opportunity. This optional funding will support things like infrastructure, data needs, Medicaid FTEs to fulfill certain required aspects of the model. And then milestone funding will also be available to states that achieve certain milestone measures. As I mentioned earlier, the Notice of Funding Opportunity will be released in summer of 2024 and that will further detail the opportunities for funding. I do want to mention here that states are not required to respond to the Notice of Funding Opportunity if they are not seeking

optional funding. They can participate in the model by simply responding to the State Request for Applications and not seeking any additional funding.

>> **Jason Petroski, CMS:** Okay, thanks Melissa. Okay, so now we're going to turn to a couple questions in the category of access. These also I'll send your way, Melissa. The first one is: How can this program help states address access issues with sickle cell disease treatment within their states?

>> **Melissa Majerol, CMS:** Sure, happy to take this one too, Jason.

So again, the Notice of Funding Opportunity is going to provide funding to states to increase things like awareness of gene therapy access, and access to multidisciplinary and comprehensive care that will help address access barriers along what is really just a very long and rigorous journey for patients receiving gene therapy for sickle cell. So among other things, state will be able to use funding to partner with community-based organizations to provide even certain direct services to Medicaid beneficiaries.

> **Jason Petroski, CMS:** Thanks, Melissa. And I'm going to keep the Q&A with you on this next access question, which is: How will CMS facilitate out-of-state access by Medicaid beneficiaries? And how will geographic access challenges be minimized in the model?

> **Melissa Majerol, CMS:** Sure. So, if there is no in-state treatment center that's qualified to provide sickle cell gene therapy, existing law requires all states to pay for people with Medicaid to receive care from an out-of-state provider. Under existing law, states must also assure necessary transportation and related travel expenses for Medicaid beneficiaries traveling to and from providers. And under the CGT access model, CMS will provide support to states in establishing relationships with out-of-state providers to ensure access to care.

> **Jason Petroski, CMS:** Okay, thank you Melissa.

Okay, so let's shift gears a little bit. Let's talk, or let's comment on some questions about beneficiary populations and payers. Aurelia, I'm going to tag you with this next one. The question is: Is the model limited to fee-for-service beneficiaries? And, how would Manage Care Organizations participate?

>> **Aurelia Chaudhury, CMS:** No, the model is not limited to fee-for-service beneficiaries. We expect participating states to coordinate with their managed care entities to be able to eventually enter with all of their lives. We understand that coordinating with their managed care entities may be complex and look different as each state has a different structure of managed care programs, which is why the model allows for something of a rolling start for states. And states may begin participation earlier with fee-for-service lives, then with respect to their managed care lives. Under the rolling start framework for the model, the fee-for-service lives could come online any time after January 2025, and managed care lives could come online as late as January 2026. And we understand that the ways in which states may be aligning with their managed care lives will vary and we look forward to working with individual states to talk to their particular circumstances.

>> **Jason Petroski, CMS:** Thanks Aurelia. Okay, so speaking of different payers, this is a question I think that we've been getting a lot. So I'm going to turn this one to Corinne. How does this model impact other payers outside of Medicaid, Medicare?

>> **Corrine Alberts, CMS:** Yeah, so the Innovation Center's authority doesn't extend to the commercial insurance market. So however, we would say that we anticipate, and in fact we hope, that private insurers will observe the model and learn from our experience in operating this demonstration. I will add though, that the model will allow manufacturers to submit a proposal to CMS for participating state's separate CHIP programs. Like many aspects of this model, however, the exact contours of an outcomes-based arrangement that includes separate CHIP beneficiaries, is subject to negotiation with manufacturers.

>> **Jason Petroski, CMS:** Thanks, Corinne. Okay, so shifting to another question, I think that we've received a lot that's really important. And staying with Corinne here. How will this model be applied to other therapies, or other cell and gene therapies or indications outside of sickle cell?

>> **Corrine Alberts, CMS:** Yeah, so this is the Cell and Gene Therapy Access Model's first performance period, we're targeting to begin in 2025, focusing on gene therapies for sickle cell disease. However, CMS may choose to include cell and gene therapies for additional conditions in future years. However, should that occur, we will release a new Request for Applications for both states and manufacturers. This won't be a surprise.

>> **Jason Petroski, CMS:** Thanks Corrine. Okay, so thank you for all the questions everyone's submitting. We are trying to compile them and review them on the fly here. Let's just keep going with some questions here and let's shift gears a little bit to operations, and the mechanics of how this model will work. So, Aurelia, can you help on the next question, which is: How does CMS plan to operationalize the outcomes-based agreements and which infrastructure and solutions is CMS looking at?

>> **Aurelia Chaudhury, CMS:** Thanks, Jason. Yeah, so this is an important part of the model. And you've gotten a few questions about it in chat, so I'll be a little bit more expansive my answer here.

So with respect to claims data, the model intends to leverage the existing T-MSIS system as much as possible to operationalize the outcomes-based agreement. This is a framework that states have long participated in with CMS in terms of channeling claims data to us, and we view it as a really helpful framework to start from. We also plan to pursue partnerships with patient registries, and we may require additional data submissions from states and manufacturers, as may be required. But a key focus of ours here is really reducing the administrative burden on states as much as possible in trying to ensure that, as far as we can, we can utilize existing data infrastructure.

We look forward to continuing to work with the patient registries in this space, in particular ones that already play a role in tracking patients with sickle cell disease who go through transplant, for example, to ensure that we're minimizing the administrative burden on manufacturers, providers, states, and beneficiaries as we think about the data collection infrastructure here. In short, we understand that patients undergoing these therapies are going to be receiving a certain set of care and certain sort of testing and follow-up in the ordinary course of care for these therapies and we attempt to sort of figure out where that data lands and collect it where it's already going as much as we can.

>>**Jason Petroski, CMS:** Okay, thanks Aurelia. Thanks for all that information. So, here's another question somewhat related to outcomes-based agreements, which Aurelia just touched on a bit. I'll turn this one to Corrine. What happens if a state already has an existing, outcomes-based agreement? And will negotiations be confidential?

>>**Corrine Alberts, CMS:** Yeah. And I see there's been a number of questions in the chat about existing OBAs and what kind of happens in the interim and the very quick answer to that is, states are still able to negotiate their own agreements with manufacturers prior to participation in the model. The - participating in the Model or intent participating in the model doesn't preclude arrangements happening in the interim. To the second part of your question, yes, negotiations between CMS and manufacturers will be a confidential process.

>>**Jason Petroski, CMS:** Okay, thanks, Corrine. Corrine, I was wondering if you could also help with this next question which really has two pieces. So the first piece is, what will the eligibility criteria be for CGT? And the second piece is, what is the strategy for long-term therapy monitoring?

>>**Corrine Alberts, CMS:** Thanks, Jason. I'll take that question out of order as well.

So, starting with the second part, the FDA has requirements on long-term follow-up after the administration of gene therapies and this model is separate and apart from that process. We will, however, be using the data infrastructure that Aurelia touched on to support data collection regarding outcomes and performance across the length of our negotiated contract with manufacturers on gene therapy with respect to outcomes.

On the first part of your question, about eligibility criteria, CMS intends to negotiate a standard access policy that would apply across all participating states. Now the exact details of that access policy, to repeat myself, will be determined in our negotiations with manufacturers. But that access policy will include important details like eligibility and utilization management. Over the next two months, we are really eager to hear from interested parties such as patient advocates, providers and state leaders, really all members of this community, to help inform our negotiation, regarding all the different elements of that access policy.

>>**Jason Petroski, CMS:** Okay, thanks, thanks a lot, Corrine. Okay, so now turning to, a super important part of this model, a question about equity. Melissa, can you help on this one? How does this model address equity?

>>**Melissa Majerol, CMS:** Sure, so I think we touched on this a little bit in the webinar, but I think one of the most important things is the initial focus of this model, which is on sickle cell disease, which as mentioned is a disease that impacts really a very specific segment of the population with very significant health disparities over a very long period of time. These are groups that have experienced historic disparities associated with this disease. As I mentioned, black Americans are disproportionately affected by this disease, and often face disparities in accessing health care in general, as well as really critical specialized care and treatment for sickle cell.

I had also mentioned earlier with respect to the Notice of Funding Opportunity that, through that, through that, funding opportunity, there will be federal funding to states to help promote access to equitable, equitable access to gene therapy and also to close some of the care gaps along this very long and rigorous journey. So some of the things that we're considering is support for expanding or making certain benefits more robust to help individuals along this journey.

>>**Jason Petroski, CMS:** Okay, thank you so much, Melissa. Okay, so now we'll turn to a dual-eligible, question. This question Aurelia, can you help us with? It does not seem that dual-eligible patients are included in this model. Is that true? Will, those patients be paid by Medicare fee-for-service?

>Aurelia Chaudhury, CMS: Yeah, so this model is targeted at patients for whom Medicaid is the primary payer, which we expect will exclude duals from the model, as Medicare would be the primary payer for those patients. We are working closely with our colleagues in the Center for Medicare to ensure alignment between what we're doing here in the model as far as coverage and reimbursement policies and what the Center for Medicare is doing as far as coverage. And reimbursement, but they have their own process and timeline and we are working in parallel and trying to ensure harmony.

The reason for the model being focused at beneficiaries for whom Medicaid is the primary payer is not that the dual-eligible population is not of great interest to us, and of course very important from an equity standpoint, but just that the framework of CMS negotiation with manufacturers facilitate. CMS negotiation with manufacturers on behalf of states is one that structurally works differently for the duals such that it would be difficult to operationally include them in the model at this time.

>>Jason Petroski, CMS: Okay, thanks Aurelia for explaining that. Corrine, can you help with this next question, which is: Will states need to file State Plan Amendments, or do states already have the requisite authority to do so independently without CMS approval?

>>Corrine Alberts, CMS: Yeah, so this is, this is a great question. So, to participate in this model, states are going to require a CMS-approved State Plan Amendment that allows them to enter into value-based purchasing supplemental rebate agreements. The good news is that many states already have these SPAs in place. And for those states, there's no further action required, at least on that front.

States can pursue a value-based purchasing State Plan Amendment really at any time. CMS has their processes for moving through, to move through those. And states can pursue those SPAs even before we reveal the negotiated key terms of this model. Though I would emphasize that having a CMS-approved SPA to enter into these value-based purchasing supplementary rebate agreements puts no obligations on the state to join the model or to enter into any further agreements. And if other colleagues have anything else to add on the on the SPA process, recognizing that this is a pretty complicated issue, please feel free to jump in. Great. Thank you, Jason.

>>Jason Petroski, CMS: Thanks, Corrine. Okay, so let's see, here's a question I think that we received on the call today. To CMS, can you clarify whether CHIP programs that are not Medicaid plans, can they be included but states could opt to exclude them. Is that correct? Aurelia, can you help us with that one?

>>Aurelia Chaudhury, CMS: Yeah, so let me draw a distinction here between CHIP-funded Medicaid expansion populations and standalone CHIP populations. We expect CHIP-funded Medicaid expansion populations to be included for any participating state. With respect to standalone CHIP populations, we imagine these populations to be optional for both states and manufacturers. And we anticipate that there may be different key terms that CMS is able to negotiate with respect to standalone CHIP populations relative to CHIP funded medical expansion or ordinary Medicaid populations. And part of it has to do with the differential treatment of standalone CHIP populations under the existing scope of the Medicaid Drug Rebate Program. But if manufacturers opt to participate with respect to CHIP populations and a particular state opts to participate with respect to its CHIP populations, then they could be included in the scope of the model, although as I mentioned, potentially with a different scope of key terms.

>>**Jason Petroski, CMS:** Alright, thank you Aurelia. I'm so happy to have the co-leads here with me to answer these the nuances of these questions. I'm speaking of which we got some other questions about the NOFO or the funding opportunity. So, I'm going to turn this one to Melissa. Melissa the question is: When will we have more information on support services available as part of the model? Will these vary based on state or will they be required to be consistent for all states that participate?

>>**Melissa Majerol, CMS:** Good question. So, the Notice of Funding Opportunity is expected to be published sometime this summer, likely June or July of 2024. And in that Notice of Funding Opportunity, we will detail all of the optional activities that states may use this funding for. We are really looking at building in just a ton of flexibility into this Notice of Funding Opportunity. So, states will have the ability to, vary what they decide to focus on. This might depend on benefits maybe they already have in place versus ones that they want to focus on under this model. So, we do expect there to be a lot of variability state by state and those details will be disclosed later this summer.

>>**Jason Petroski, CMS:** Okay, thanks, Melissa. Okay, so, I think we're going to take a minute or two here for the model team to just take a pulse of where we're at with some of the questions that have come in, what we can answer. Give, give us a couple of seconds here to see what we'll answer next.

Okay, alright, so we have, we have a couple more questions to go through with our audience here. Okay. So Aurelia, I'm going to turn to you for this next one. Let's see. Question is: Does the model include ancillary costs like the stem cell collection and myeloablation components of the therapy? What about related costs for transportation, housing, and meals, travel to and from the designated treatment facilities?

>>**Aurelia Chaudhury, CMS:** So, I want to be clear that the structure of CMS's role in the model here is negotiating the outcomes-based agreement with the manufacturer and supporting the implementation, reconciliation, evaluation of that outcomes-based agreement with the manufacturer for states that choose to participate in the model. And so, it's not, it remains the case that states that participate in the model continue to retain the same responsibilities they have in the ordinary course in the world outside of the model for reimbursing providers for therapy and paying for necessary services. Of course, a state that participates in the model is bound by the terms of the outcomes-based agreement key terms as negotiated by CMS and thus the coverage policy for example or access policies that would be a part of that outcomes-based agreement.

So, I would be clear that, a state would still be responsible for covering medically necessary services that would be required by the coverage policy as negotiated by CMS, which would include costs related to gene therapy including stem cell collection or myeloablation, but that the model is not itself paying for those costs. Similarly, with respect to other obligations that states would have to pay for services related to gene therapy such as transportation, housing and meals as Melissa had mentioned earlier, states have current legal obligations to cover non-emergency medical transportation, including housing and meals in some circumstances. And states would have the support of the model team in ensuring that they are able to work with CMS to ensure that beneficiaries receive those services in the context of the model, in the context of gene therapies for sickle cell disease.

>>**Jason Petroski, CMS:** Thanks Aurelia. And I'm going to throw you another question here. Does the state have input on whether the outcomes being evaluated in the value-based agreement or outcomes-based agreements are fair and clinically appropriate?

>>**Aurelia Chaudhury, CMS:** Yes, so we'll discuss this more on the state by webinar on Thursday, but we look forward to engaging closely with states over the next several months as we prepare to enter negotiations with the manufacturer such that we can get exactly this kind of input from states on their perspectives on the appropriate clinical outcomes, what would be reasonable to contract for, how we would tie those outcomes to payments, benchmarks and thresholds we would use, etc. And we have already been having some of these conversations with states, with beneficiary groups, with clinicians, and we look forward to continuing that process.

>>**Jason Petroski, CMS:** Okay, thanks, Aurelia. Okay, give us one more second here to just compile some more questions. We really do appreciate all the questions. Again, any questions that we don't answer on the call today will be sure to include information addressing those questions or pertinent to those questions in other materials that we put out.

Okay, let's, how about Melissa, Melissa can you help me on this one? How will coverage of fertility preservation work? Given that HHS Office of the Inspector General has opined that CGT manufacturers cannot provide those services via their PAPs. Will there be a federal anti-kickback waiver or all for, all model participants?

> **Melissa Majerol, CMS:** Yeah, that's a great question. So this is something that we're still developing and still thinking through, but under the CMS model safe harbor, we are allowed to have certain waivers that are not necessarily available outside of the model. As noted in the question, coverage of fertility preservation by manufacturers, is generally speaking not allowed due to anti-kickback and patient inducement concerns. But, under the model, given the specificity of the model test and what we are looking to test we will, we are looking at including manufacturer coverage of fertility preservation.

> **Jason Petroski, CMS:** Okay, thanks, Melissa. Let's see, Corinne, how about you help with this one? It was mentioned that CMMI will seek comment from the sickle cell disease community on the access policy? How will this work? How, how do we engage?

> **Corrine Alberts, CMS:** Thank you, Jason. And I think this is a really important question to repeat often. So we're really eager to hear from individuals and groups in the sickle cell disease community, and the best way to reach out is through the email that's on the screen. So, CGTModel@cms.hhs.gov. Please reach out, we're monitoring that inbox and we have found the input of the community incredibly valuable in the development of this model. I recognize this is just the beginning of the conversation and want to make sure that we're continuing to hear from all of you. And please feel free to reach out to that email inbox.

> **Jason Petroski, CMS:** Thanks, Corrine. Okay, so, Aurelia, here's a question on data collection monitoring: Who will support data collection monitoring, the states or the manufacturers?

> **Aurelia Chaudhury, CMS:** So as I mentioned, the focus here is really reducing the administrative burden on states as much as possible by shouldering that burden, by CMS directly or working with patient registries to obtain clinical data from providers, leveraging existing data infrastructure, existing registries and existing relationships as much as we can. There is funding in the Model, both centrally from the model and available through states Notice of Funding Opportunity to support any data collection that states may have to do related to the model. And additional funding that we may be able to provide to patient registries that participate in the data collection that could support this effort by providers to track longitudinal outcomes for patients who undergo gene therapy for sickle

cell disease. Manufacturers will also have data that they report into the system as well. But we anticipate that this would largely track what their existing, the existing data that they will have available in the ordinary course.

>>**Jason Petroski, CMS:** Okay, thank you, Aurelia. Aurelia, I'm going to come back with, another question, which is about payments. Aurelia, how does the reimbursement work for this model with providers? Are providers paid the APR DRG, will the cost continued to be bundled in that APR DRG?

>>**Aurelia Chaudhury, CMS:** So, we will be making clear in the State Request for Application, which we expect to release this summer, what the requirements for state participation will be. One of the requirements for state participation will be to separately reimburse for the gene therapy, separately from the inpatient stay during which it is provided. We understand that some states already engage in this type of separate reimbursement and we expect the participating states would do so.

This is important in a couple of ways. First, it's important for a state to be able to separately reimburse for the therapies, such that it is considered a covered outpatient drug for the purposes of the Medicaid Drug Rebate Program, which opens up the ability to use supplemental rate agreements to contract with manufacturers from outcomes-based agreements. But also, separate reimbursement for the therapies is important to ensure that hospitals remain appropriately incentivized to provide these therapies and do not encounter tremendous financial losses as might occur if we were to use the existing APR DRG, or MS DRG, whatever, whatever DRG system or per diem system that states currently used to pay for these therapies. And so we anticipate providing more detail about what exactly that separate reimbursement policy would look like in that State Request for Application this summer.

>>**Jason Petroski, CMS:** Thanks, Aurelia. Okay, so here's a couple, there's a couple questions about the outcomes-based agreements and the mechanics there. I'm going to turn this to, I think, Corinne. Corinne, are the outcomes-based agreements the only thing that CMS is negotiation or negotiating, doesn't that mean that states only benefit if the product fails?

>>**Corrine Alberts, CMS:** Yeah, this is a great question. I think to be clear, the key terms that we're discussing here would include a number of things. The outcomes-based arrangement ties rebates to the outcomes of the therapy, but also additional supplemental rebates. So, we're not necessarily saying that the only rebates that CMS will be negotiating here are going to be those tied to outcomes, we are also pursuing additional rebates that would be on top of those outcomes-based components.

>>**Jason Petroski, CMS:** Thanks, Corrine. And then, a follow up question on, again, on OBAs, how and when will the agreed upon terms of the negotiated OBAs be disclosed to interested states?

>>**Corrine Alberts, CMS:** Yes, and so that information will also be in the slide deck that will be posted later, but the answer is December. We are going to be releasing a Request for Applications for manufacturers shortly, that will initiate a negotiation period and at the conclusion of that negotiation period in the late fall, no later than December of this year, the negotiations both can be concluded and key terms will be disclosed to states.

>>**Jason Petroski, CMS:** And so, I think we're about rounding it out here in terms of the questions that we're able to answer today. As I mentioned, we'll be compiling the full list of questions and comments that we received and making sure that, again, we update our materials accordingly to address the content received. I think we'll, we'll see if we can do one or two more questions here and then and then

we'll proceed. Just give us one more second here to compile these questions. Okay, so, fundamental question here, I'll turn this one to Aurelia. What's the incentive for manufacturers to participate in the model?

>>Aurelia Chaudhury, CMS: So, we've been really heartened by our partnership with both of the manufacturers, who we've been talking to extensively over the past year. We think there are a couple of major incentives for manufacturers to participate.

One, we think manufacturers may benefit significantly from the ability to have a standardized outcomes-based agreement, a standardized outcomes policy across the various states that choose to participate in the program, is that'll simplify their market access and might reduce their administrative burden.

Second, we think that there's a real potential for increased participation by patients through the participation of the model that comes from all of the things that the model is doing to attempt to improve equitable access to the therapies. That spans from allowing manufacturers to cover the cost of fertility preservation, which as Melissa mentioned is not something that they will be able to do outside the context of the model, but also, from the other things we'll be doing. For example, working with states in collaborating with their managed care organizations and working with in and out of state providers and changing their reimbursement policies, in streamlining their prior authorization processes, in streamlining their prior authorization processes, etc. to ensure, you know, that as much as possible, that equitable access is being delivered for these therapies.

We understand that in cell and gene therapies that have come to market already, we have not necessarily always seen the equitable access emerge and we have seen lots of points of friction along what can be a long and difficult care journey. And so, we look forward to being able to partner with manufacturers as both we and they have an interest in reducing these points of friction and potential barriers to access.

>>Jason Petroski, CMS: Thank you, Aurelia. Okay, I think with that question, we're going to conclude the Q&A portion of the presentation. Thank you so much to everyone for your attention and for engaging during this session. If we could, next slide please.

Again, I just want to thank everybody for attending today's webinar. We hope this was an informative session for those interested in applying to the model in the near future, and will now close out the session. Next slide, please.

I want to just start by saying thank you to our presenters and to the audience for your engagement and questions again. Please submit any additional questions you have to the model email address which is listed here on this slide, CGTModel@cms.hhs.gov, and the team will respond to your questions or your comments as quickly as possible. As always, we look forward to working with you as we continue to develop the model and implement it. We genuinely thank you for your collaboration with us as we embark on this journey together to improve the care and support for people living with sickle cell disease. Next slide, please.

Okay, thank you. This concludes today's presentation

###

