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December 19, 2023

VIA ELECTRONIC DELIVERY (LIZ.FOWLER@CMS.HHS.GOV)

Liz Fowler
Deputy Administrator and Director
Center for Medicare and Medicaid Innovation
Centers for Medicare and Medicaid Services
Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244

RE: Cell and Gene Therapy (CGT) Access Model Considerations

Dear Deputy Administrator Fowler:

We appreciated the conversation that you and your staff at the Center for Medicare and Medicaid Innovation (CMMI) had in March 2023 with the Institute for Gene Therapies (IGT or "the institute") to discuss the Cell and Gene Therapy Access Model (the "CGT Access Model"). As you may recall, IGT was launched in February of 2020 to advocate for a modernized regulatory and reimbursement framework that encourages the development of transformative gene therapies and promotes patient access. Through a Patient Advocacy Advisory Council, Corporate Advisory Council, and Scientific, Academic & Medical Council, the institute represents a wide array of patient advocacy groups, gene therapy manufacturers, and scientific, medical, and academic stakeholders seeking to advance the promise of gene therapies. IGT is devoted to promoting the value of transformative therapies and advocating for policies and practices to ensure patient access to these treatments. Our most vulnerable patients and their families anxiously wait for the transformational treatments that gene therapies will offer to some of the most debilitating or rare diseases. A full list of our members is available at https://www.gene-therapies.org/about-igt.

In 2023, the Food and Drug Administration (FDA) approved four gene therapies and a gene editing cell therapy.[1] Scores of gene therapies are currently in clinical trials in the US; most of which will address rare and ultra-rare diseases, central nervous system diseases, and conditions disproportionately impacting historically disadvantaged populations.[2] In many cases, gene therapies may halt but cannot reverse the effects of a disease by addressing the underlying genetic cause. For this reason, any obstacle to accessing an approved gene therapy can result in patients continuing to suffer irreversible damage caused by their disease that may otherwise be avoided.

[1] In chronological order, see: (1) https://www.fda.gov/vaccines-blood-biologics/vyjuvek; (2) https://www.fda.gov/vaccines-blood-biologics/tissue-tissue-products/elevidys; (3) https://www.fda.gov/vaccines-blood-biologics/roctavian; (4) https://www.fda.gov/vaccines-blood-biologics/lyfgenia and (5) https://www.fda.gov/vaccines-blood-biologics/casgevy.
[2] See ClinicalTrials.gov.

IGT is hopeful that the CGT Access Model will foster innovation, preserve and enhance access, and improve the lives of the most vulnerable patients in the US. The patients and families waiting for gene therapies deserve access to these treatments as soon as possible upon FDA approval.

In advance of additional details regarding the model, IGT offers several comments and suggestions outlined below. Our hope is that the CGT Access Model will serve patients and encourage state and manufacturer partnerships that help deliver on the promise of gene therapy.

I. Recommendations for CMMI Relating to the CGT Access Model

Model Flexibility. As we discussed in March, flexibility is key to any successful payment model demonstration. We hope that CMMI will pursue the CGT Access Model with flexibility as a foundational principle for success. Multiple components of the model, and ultimately patients, will be better served by flexibility. This could include rolling eligibility that allows states to participate when ready and manufacturers to join upon FDA approval, examining the financial and quality impacts over a longer period than 12 months (e.g., dictated by disease progression), and permitting waivers of federal fraud and abuse laws to facilitate a successful model.

Type of Value-Based Payment Arrangement (VBA). CMMI should not limit what type of VBA can be negotiated between states and manufacturers. The Medicaid multiple best price regulation defines a VBA as an arrangement intended to align pricing and/or payments to an observed or expected therapeutic or clinical value and includes (but is not limited to): (a) evidence-based measures that link cost to evidence of effectiveness and potential value for specific uses of a product; and/or (b) outcomes-based measures that link payment to the drug's actual performance in a patient or population, or a reduction in other medical expenses.[3] In order for the CGT Access Model to be successful, it should function in close coordination with the existing multiple best prices regulatory structure. CMMI should not limit "acceptable" VBAs solely to those categorized as outcomes-based arrangements as not all disease states and gene therapies, particularly those for progressive and degenerative diseases, are amenable to outcomes-based agreements. CMMI should allow states and manufacturers to enter into new and innovative VBAs without limiting the type of VBA prior to even initiating the model.

Disease States. We support CMMI's decision to evaluate multiple conditions for inclusion in the CGT Access Model, including sickle cell disease. However, we request that CMMI carefully consider which diseases should be included at the model's initiation. Some diseases, particularly progressively degenerative conditions, might not be as amenable to value-based contracting as other diseases. CMMI should use the early years of the model as a true test to inform future changes or model expansion.

Model Details. We hope that when CMMI releases additional details regarding the CGT Access Model, that an opportunity for public comment from all stakeholders will be permitted. Beyond meaningful opportunity for public comment, we hope that CMMI will have targeted conversations and seek feedback from patients, gene therapy manufacturers likely to participate in the model, and leading physician experts administering gene therapies. We appreciate the willingness of CMMI leadership to meet with us and other stakeholders to develop the model parameters over the past year and request that the next iteration of model details be open for public comment. For this model to be successful, which would help facilitate access to gene therapies by the most vulnerable patients, we must prevent unintended consequences or an inflexible model design. By providing for an opportunity for public comment and model refinement, all stakeholders can be assured that our voices have been heard.

[3] 42 C.F.R. § 447.502.

CMMI's Role. The CMMI was granted broad statutory authority by the Affordable Care Act (ACA) to test new and innovative payment models. However, the statute does not permit CMMI to negotiate Medicaid prescription drug prices, whether through direct or indirect negotiation or drug price verification or justification surveys. We hope that the CGT Access Model will focus CMMI's role on facilitating conversations between states and manufacturers, implementing the measures agreed to between these two parties, reconciling data, and evaluating the results. In addition, we request that CMMI reiterate previously issued guidance that state Medicaid agencies are required to cover and authorize treatment for all covered outpatient prescription drugs subject to a rebate agreement with the Secretary.[4] In the case of the CGT Access Model, states would still be required to cover and authorize treatment for a covered outpatient drug to its medically accepted indication regardless if the manufacturer of the covered outpatient drug participates in a CMMI demo.

II. Request for Meeting

IGT respectfully requests a meeting with you and CMMI staff to discuss the CGT Access Model and the concerns outlined above. As you noted in the one-year update of the Executive Order 14087, the CGT Access Model presents "[t]he opportunity to increase access to novel therapies among historically underserved populations and advance health equity…"[5] IGT agrees, and it is in the interests of all stakeholders to ensure that the model is a success from day one.

III. Conclusion

Gene therapies have the potential to transform how we approach disease in this country, particularly serious conditions with high unmet medical need, and to help reverse decades of health inequities and discrimination against disadvantaged populations including individuals with disabilities. The Institute for Gene Therapies appreciates CMMI's efforts to advance access to gene therapies through the CGT Access Model. IGT welcomes the continued opportunity to engage with CMMI and others at HHS and CMS and we are pleased to serve as a resource on gene therapy issues.

Sincerely,

John R. Feore, III
Director, Health Policy and Advocacy
Institute for Gene Therapies

Cc: Arielle Woronoff, Director, CMS Office of Legislation (ARIELLE.WORONOFF@CMS.HHS.GOV)

[4] https://www.medicaid.gov/sites/default/files/2022-07/cib07212022_0.pdf
[5] CMS Innovation Center's One-Year Update on the Executive Order to Lower Prescription Drug Costs for Americans (oct. 11, 2023), available at: https://www.cms.gov/blog/cms-innovation-centers-one-year-update-executive-order-lower-prescription-drug-costs-americans.