909 E Street NW, 2nd Floor
Washington D.C. 20004
gene-therapies.org

July 25, 2023

VIA ELECTRONIC DELIVERY

Administrator Chiquita Brooks-LaSure
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Attention: CMS-2434-P
P.O. Box 8016
Baltimore, MD 21244-8016

RE: IGT Comments on Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program [CMS-2434-P]

Dear Administrator Brooks-LaSure:

The Institute for Gene Therapies (IGT or "the institute") appreciates the opportunity to submit these comments to the Centers for Medicare and Medicaid Services (CMS or "the agency") on the Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program proposed rule (the "Proposed Rule").[1] 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 Corporate Advisory Council, Patient Advocacy 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 life-altering 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.

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. In the past 12 months (August 1, 2022 through July 25, 2023), the Food and Drug Administration (FDA) has approved six gene therapies.[2] Scores of gene therapies are currently in clinical trials in the US; dozens of which would be administered in the inpatient setting, addressing rare and ultra-rare diseases, central nervous system diseases, and conditions disproportionately impacting historically disadvantaged populations.[3] In many cases, gene therapies halt but cannot reverse the effects of a disease by addressing the underlying genetic cause. For this

[1] 88 Fed. Reg. 34,238 (May 26, 2023).
[2] In chronological order, see: (1) https://www.fda.gov/vaccines-blood-biologics/zynteglo; (2) https://www.fda.gov/vaccines-blood-biologics/skysona; (3) https://www.fda.gov/vaccines-blood-biologics/vaccines/hemgenix; (4) https://www.fda.gov/vaccines-blood-biologics/vyjuvek; (5) https://www.fda.gov/vaccines-blood-biologics/tissue-tissue-products/elevidys; and (6) https://www.fda.gov/vaccines-blood-biologics/roctavian.
[3] See ClinicalTrials.gov.

reason, any delay in access to an approved gene therapy can result in patients continuing to suffer irreversible damage caused by their disease that may otherwise be avoided. IGT commends CMS for taking action in recent years to facilitate policies that bolster access to critical therapies for rare diseases, including finalization of the Medicaid "multiple best prices" regulation that will enable greater adoption of value-based payments.[4] We look forward to continuing to work with CMS and with the Center for Medicare and Medicaid Innovation (CMMI) to advance value-based payment models and innovative payment arrangements in the Medicaid program to ensure immediate and continued access to transformative therapies for the most vulnerable patients.

However, we raise significant concern that, if implemented, the Proposed Rule will directly contradict the agency's efforts to facilitate value-based payments, stymie timely patient access to the most innovative therapies, and negatively impact longer-term investment in gene therapies. These threats arise primarily from the proposals related to the drug price verification survey and the definition of a covered outpatient drug (COD), which not only are counterproductive from a policy perspective but overstep the agency's own legal authority. IGT details our serious concerns with these two specific proposals in the balance of this letter.

I. The Drug Price Verification Survey Is Not Authorized Under Existing Statute and Will Disproportionately Impact Access to Care for the Most Vulnerable Medicaid Beneficiaries

The agency is authorized by the Social Security Act (the "Act") to survey wholesalers and manufacturers engaged in the direct distribution of CODs in order "to verify" pricing metrics reported to the government, including Average Manufacturer Price (AMP), Best Price, Average Sales Price (ASP), and Wholesale Acquisition Cost (WAC).[5] Under the guise of this survey authority, CMS now proposes an extensive and exhaustive survey, which the agency suggests is intended to help understand "the factors that influence the pricing of drugs dispensed in non-retail community pharmacy settings…the prices that pharmacies or wholesalers pay…or whether the costs of distribution or preparation methods are included in the prices reported to us."[6]

Under newly proposed §447.150(k), CMS essentially is proposing a National Prescription Drug Price Accountability Board designed to specifically target gene therapies while ignoring the immense value that gene therapies provide to patients, families, and the healthcare system.[7] This is because the proposal, if finalized, would require manufacturers of certain CODs to submit much more than just the information to fulfill the statutory requirement of "verify[ing]" pricing metrics. Instead, CMS proposes a survey that would include information pertaining to: (1) pricing, charges, distribution, and utilization; (2) product and clinical information (including manufacturer data concerning the safety, efficacy, and outcomes associated with the drug); and (3) the costs of production, research, and marketing associated with the drug.[8] CMS then proposes to post information received on a public website for "further verification" and public comment, and manufacturers that refuse to provide information may be subject to civil monetary penalties.[9]

We agree with CMS that arrangements between manufacturers and industry middlemen can affect drug coverage costs for government and other payors. However, much of the information sought by the proposed survey far exceeds what would be necessary to verify the metrics addressed by the Act. Manufacturers can offer little insight into many of the transactions undertaken by entities noted by CMS as contributing to its difficulties

[4] 85 Fed. Reg. 87,000 (Dec. 31, 2020).
[5] Social Security Act §1927(b)(3)(B).
[6] 88 Fed. Reg. at 34269-70.
[7] See https://www.gene-therapies.org/value-of-gene-therapies.
[8] 88 Fed. Reg at 34295.
[9] *Id.*

in verifying manufacturer reported prices. To truly achieve the statutory goal of verification, IGT recommends that CMS should focus its efforts on establishing reporting requirements that encourage transparency on the part of the participants in the healthcare system that CMS itself lists in the preamble including wholesalers, health plans, PBMs, retail pharmacies, as well as non-retail providers such as specialty pharmacies, physician practices, home infusion pharmacies, hemophilia treatment centers, and clinics.

Instead of simply verifying AMP as allowed by statute, and particularly in view of the proposal to refine the list of drugs surveyed by removing those whose manufacturers demonstrate a "willingness to negotiate further rebates,"[10] CMS appears to be engaged in an attempt to coerce price reductions by means of a threatened disclosure of sensitive and proprietary information, much of which bears no direct connection to the transactions that CMS is authorized by statute to verify. CMS' proposed survey focuses in particular on product safety and efficacy, information more appropriately disclosed through existing pathways established by the FDA. This suggests that CMS is seeking "justification" of prices charged, rather than "verification" of prices reported. This is a clear case of overreach on CMS' part, which can only be corrected by a withdrawal of this proposal.

CMS' drug price verification survey proposal would create an unnecessary bureaucracy and falsely assumes that manufacturers are not engaging state Medicaid programs. A recent white paper published by the American Society of Gene and Cell Therapies (ASGCT) reports that many states are limiting the number of meetings a manufacturer may have with the state to discuss their pipeline; one state reportedly gives only one 30-minute meeting per year to each manufacturer to discuss all access issues and drugs in clinical development; and some states are not even willing to meet with manufacturers at all.[11] This proposal is not the solution to encourage greater coordination between states and manufacturers. Rather, CMS should focus its efforts on providing resources for states so that they are able to maximize collaboration with manufacturers, the use of innovative contracting, and planning for new transformative therapies ahead of launch. IGT stands ready to partner with CMS in these efforts.

II. The Proposal to Modify the Definition of Covered Outpatient Drug is Contrary to Statutory Authority and Threatens Provider Participation and Medicaid Beneficiary Access to Care

The Act excludes from the definition of "covered outpatient drug" any drug, biologic, or insulin "provided as part of, or as incident to and in the same setting as [certain services] (and for which payment may be made under this title as part of payment for the [services] and not as direct reimbursement for the drug)."[12] CMS proposes to revise and add to the regulatory definition of "covered outpatient drug" the following sentence: "Direct reimbursement for a drug may include both reimbursement for a drug alone, or reimbursement for a drug plus the service, in one inclusive payment if the drug and the itemized cost of the drug are separately identified on the claim."[13]

This proposal, if finalized, unduly expands the scope of the MDRP beyond its statutory bounds and would undermine efforts to adopt value-based payment models and innovative payment arrangements. Simple "itemization" on a claims form is not the equivalent of "direct reimbursement." This proposal would make it more difficult for providers to be reimbursed adequately for the cost of administering gene therapies. This cost has not been included in the development of a bundled payment rate for the underlying service and may not be

[10] *Id.* at 34272.
[11] Medicaid coverage practices for approved gene and cell therapies: Existing barriers and proposed policy solutions (June 2023). Available at: https://www.cell.com/action/showPdf?pii=S2329-0501%2823%2900077-3.
[12] Social Security Act §1927(k)(3).
[13] 88 Fed. Reg. at 34291.

for years after launch given the low volume of claims associated with rare diseases. If providers are not reimbursed adequately, they in turn will not offer these therapies, thus negatively impacting patient access and outcomes.

Through Medicare's Hospital Inpatient Prospective Payment System (IPPS), IGT has encouraged CMS to utilize innovative reimbursement methods to ensure that providers will offer, and be adequately reimbursed for, gene therapies.[14] CMS should similarly encourage state Medicaid programs to implement innovative reimbursement methodologies for gene therapies that adequately cover both the direct gene therapy costs and the patient care costs for services incident to that therapy. We are deeply concerned that modifying the definition of a COD as proposed will incentivize states to require itemization of drug and service charges in a variety of treatment settings, solely for the state to capture increased Medicaid rebate revenues at the expense of providers. The transformational promise of gene therapies cannot be realized without an adequate supply of providers ready and willing to administer the treatments and this proposed change to the definition of a COD should be withdrawn.

III. Conclusion

The Institute for Gene Therapies strongly opposes the Proposed Rule's drug price verification survey and change to the definition of covered outpatient drug. These two proposals are not supported by statute, single out gene therapies, and would stifle innovation. Simply put, these proposals will inhibit access to transformative therapies for the most vulnerable and disadvantaged individuals in the US – those who rely on Medicaid. IGT welcomes the opportunity to engage with CMS over the coming years regarding broader payment concepts to ensure a strong future for gene therapy across payer systems. We would be pleased to serve as a resource on gene therapy issues and answer any questions regarding these comments.

Sincerely,

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

[14] https://www.gene-therapies.org/_files/ugd/b11210_d01b7e91290c4dc2b1d12df3d72a6b23.pdf?index=true.