July 25, 2023

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Ave, SW
Washington, DC 20201

Submitted via http://www.regulations.gov

Medicaid Program; Misclassification of Drugs, Program Administration and Program Integrity Updates Under the Medicaid Drug Rebate Program (CMS–2434–P)

Dear Administrator Brooks-LaSure:

ARM appreciates this opportunity to comment 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]

The Alliance for Regenerative Medicine (ARM) is the leading international advocacy organization championing the benefits of engineered cell therapies and genetic medicines for patients, healthcare systems, and society. As a community, ARM builds the future of medicine by convening the sector, facilitating influential exchanges on policies and practices, and advancing the narrative with data and analysis. We actively engage key stakeholders to enable the development of advanced therapies and to modernize healthcare systems so that patients benefit from durable, potentially curative treatments. As the global voice of the sector, we represent more than 400 members across 25 countries, including emerging and established biotechnology companies, academic and medical research institutions, and patient organizations.

As of year-end 2022, 1,308 regenerative medicine and advanced therapies developers worldwide are sponsoring 1,200 clinical trials across dozens of

[1] 88 Fed. Reg. 34,238 (May 26, 2023).

indications, including rare monogenetic diseases, oncology, cardiovascular, central nervous system, musculoskeletal, metabolic disorders, ophthalmological disorders, and more.[2]

To date, the FDA has approved nine gene therapies – eight for rare genetic diseases, and six CAR-T cell therapies for various blood cancer indications. Transformative cell and gene therapies (CGTs) have been approved for rare genetic pediatric indications including Duchenne muscular dystrophy, spinal muscular atrophy, and cerebral adrenoleukodystrophy, as well as for the pediatric blood cancer acute lymphoblastic leukemia. These innovative therapies address high unmet medical needs; they can be life-saving; and many have the potential to reduce the need for burdensome and costly chronic care. The CGT pipeline for both rare and prevalent diseases is accelerating, with growing impacts on Medicaid. Notably, two gene therapies for sickle cell disease could be approved by the FDA in late 2023. And we've seen recent gene therapy approvals for Duchenne muscular dystrophy and Hemophilia A, and a cell therapy for type 1 diabetes, among others.

Ensuring Medicaid patients have timely access to the same transformative therapies that will become available to those with other forms of government and commercial insurance is critical to achieving CMS' goal of addressing health equity, including closing gaps in care for underserved populations and eliminating racial health disparities. Medicaid nationwide covered 66 percent of sickle cell disease hospitalizations in 2004 and 58 percent of emergency department visits for the disease between 1999 and 2007.[3] Not only does Medicaid pay for a majority of acute care for sickle cell disease patients, but those patients are overwhelmingly people of color.[4]

To help ensure access to these innovative therapies among Medicaid patients, ARM and its members are committed to advancing novel strategies including voluntary value-based arrangements (VBAs) with state Medicaid agencies. ARM believes that appropriately structured VBAs could address payer uncertainty regarding real-world efficacy that supports the durability and value of these cutting-edge therapies. For this reason, ARM supports CMS' efforts to address the government price reporting and operational barriers to VBA implementation by manufacturers and states, including the

[2] https://alliancerm.org/sector-report/2020-annual-report/
[3] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8550393/.
[4] https://www.cdc.gov/ncbddd/sicklecell/data.html#:~:text=SCD%20affects%20approximately%20100%2C000%20Americans,sickle%20cell%20trait%20(SCT).

final rule published on December 31, 2020,[5] and efforts by the Center for Medicare and Medicaid Innovation (CMMI) to reduce barriers to adoption of VBAs for CGTs specifically. We are concerned, however, that the Proposed Rule directly contravenes these aims. Specifically, by aggressively seeking to obtain greater federal and supplemental rebates from manufacturers, we are concerned that the Proposed Rule may inappropriately undervalue these therapies and discourage VBA adoption. We are also concerned that CMS has overstepped its statutory authority in outlining policies aimed at obtaining rebates for states in a manner not contemplated by controlling law.

While ARM members have concerns with many of the policies outlined in the Proposed Rule, our comments focus on the two policies that will have a disproportionately negative impact on CGTs. Specifically, as outlined in greater detail below, ***ARM urges CMS not to finalize its proposed modification to the regulatory Covered Outpatient Drug (COD) limiting definition and to abandon its proposed price verification survey process.*** Both proposals exceed CMS's statutory authority and represent misguided policies that would negatively impact access to CGTs for Medicaid patients. While not specific to CGTs, ARM further urges CMS not to finalize its proposed "stacking" policy, which not only exceeds the agency's statutory authority, but would be operationally infeasible and harmful to patients if finalized.

I. CMS's Proposed Interpretation of the "Limiting Definition" for Covered Outpatient Drug Strains the Bounds of the Statutory Text and is Likely to Interfere with Patient Access.

The term "Covered Outpatient Drug" (COD) is foundational to the MDRP, as it defines its scope. Specifically, only those products that fall within the definition of a COD are subject to the coverage and rebate provisions of section 1927 of the Social Security Act (the "Act").
As CMS outlines in the Proposed Rule, section 1927(k)(3) of the Act contains a "limiting definition," which provides that "[t]he term 'covered outpatient drug' does not include any drug, biological product, or insulin provided as part of, or as incident to and in the same setting as, any of [certain settings] (and for which payment may be made under [Medicaid] as part of payment for [services in such settings] and not as direct reimbursement for the drug)." Nearly a decade ago, in 2016,[6] CMS adopted the now-current regulatory definition of COD at 42 C.F.R. § 447.502, which, as CMS notes,

[5] Medicaid Program: Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and Supporting Value-Based Purchasing (VBP) for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability (TPL) Requirements, 87 Fed. Reg. 87,000 (Dec. 31, 2020).
[6] 81 Fed. Reg. 5170 (Feb. 1, 2016).

"substantially mirrors the statutory definition."[7] Indeed, paragraph (2) of this regulatory definition largely parrots the statutory limiting definition by restating nearly verbatim the parenthetical outlined above.

In the Proposed Rule, CMS proposes to make two changes to this regulatory definition. The first is a technical change to add "payment for" into this parenthetical text. ARM supports this proposed change, as it is consistent with the controlling statutory language and adds greater interpretive clarity. However, ARM is deeply concerned about CMS' second proposed change to the definition, which would interpret the statutory language "not as direct reimbursement for the drug" to refer not only to instances where the hospital actually seeks and obtains separate payment for a drug, but also where the drug is merely "separately identified" on a claim for payment. This interpretation conflicts with the controlling statutory text since, among other reasons, it ignores the reasonable interpretation of the statutory term "*direct* reimbursement" (emphasis supplied). Further, it is bad policy that conflicts with CMS' stated aim to create equitable access for Medicaid beneficiaries and will disproportionately impact patients seeking CGTs because many of these therapies are administered in the inpatient setting.

By way of background, for those CGTs that are currently administered in the inpatient setting, providers are generally paid a bundled rate (typically a DRG or per diem) that incorporates the hospital's cost for inpatient charges for the administration of the drug, including the cost of purchasing these innovative new therapies. Where drugs such as CGTs are administered and paid for in this way, such drugs are not considered "covered outpatient drug[s]" as defined in section 1927, and thus are not subject to that law's provisions.

The current bundled payment methodology is particularly problematic for hospitals seeking to administer CGTs, as the bundled payment is generally much lower than the acquisition cost of the drug for the hospital. Moreover, given the rarity of the diseases targeted by existing CGTs, the methodology for updating DRG payment rates is unlikely to be able to sufficiently account for the added cost of these therapies in a systematic manner. For this reason, hospitals seeking to administer these products to Medicaid patients risk significant financial losses, which can ultimately limit access to these therapies due to decreased provider uptake. Separate payment for CGTs – that is, reimbursement outside of the DRG by a state Medicaid program to the hospital that accounts for the hospital's actual cost of purchasing the CGT – encourages both acquisition and administration of these therapies by making hospitals whole for the costs incurred for purchasing CGTs.

[7] 88 Fed. Reg. at 34,252.

Furthermore, separate payment creates greater equity in reimbursement rates across settings of care (inpatient versus outpatient), allowing providers to make treatment decisions on the basis of the individual clinical circumstances of a patient.

For this reason, a limited number of states are beginning to pay hospitals separately, outside of the bundled payment for inpatient services, for their acquisition cost of CGTs through state plan amendments (SPAs) or administrative policies. Under CMS's longstanding interpretation of the COD limiting definition, these separate payment policies qualify as "direct reimbursement for the drug." Accordingly, any drugs paid through such separate payment policies qualify as CODs subject to the requirements of section 1927 of the Act, including the manufacturer's obligation to pay MDRP rebates. This arrangement is advantageous to all stakeholders: hospitals are paid adequately to ensure access, states have the benefit of federal rebates on the utilization, and Medicaid patients in turn benefit from increased access. Moreover, because the MDRP enables supplemental rebate agreements (SRAs), subjecting an inpatient administered drug to the MDRP will open the opportunity for a VBA on that drug to the extent that at state program has obtained approval from CMS for a VBA SRA.[8]

Concerningly, CMS' proposal to define "direct reimbursement" to include circumstances in which the drug is merely "separately identified" on a claim for payment would strip away these advantages to the detriment of timely patient access to CGT. First, as noted above, as a matter of statutory construction, we fail to see the rational relationship between the controlling statutory text "direct reimbursement" and this interpretation. Specifically, we fail to see how the term "direct reimbursement" can mean, as CMS seems to suggest, "separately identified" on a claim without some form of direct payment for the drug as requested by the provider. By referring to "payment" and "direct reimbursement," rather than the format of the claim, the plain language of the limiting definition's parenthetical language is clearly intended to consider only how the drug is actually *paid*. Merely including a line-item that identifies the amount the drug contributes to the overall bundled payment is not "direct reimbursement," as there is no direct payment for the drug itself.

Second, CMS' proposed change would presumably deem any drug that is administered inpatient as a "covered outpatient drug" simply by the inclusion of that drug on a claim form. As a result, states would presumably be authorized to seek MDRP rebates from manufacturers on such drugs by

[8] To date, 17 states have obtained such approval. *See* https://www.medicaid.gov/medicaid/prescription-drugs/downloads/vbp-sra-effective-dates-map-05162023.pdf.

simply identifying the product on the claim form, and without actually reimbursing the provider directly for the cost of the drug. In so doing, CMS undermines mutually beneficial separate payment arrangements outlined above, and instead creates an opportunity for states to merely add a line-item to an otherwise bundled payment, resulting in significant financial losses for hospitals or, alternatively, restricted patient access to CGTs. That is, CMS' proposal would override controlling statutory constraints as well as longstanding policy by enabling states to seek automatic MDRP rebates on inpatient-administered drugs, while still leaving hospitals at risk for significant financial losses, and patients at risk for access restrictions. Given the significant rebate exposure to manufacturers under this policy without any benefits in terms of increased access for patients, incentives for the negotiation of VBAs would also be eliminated.

For these reasons, ***we strongly urge CMS not to reinterpret the COD limiting definition as proposed.***

II. CMS's Proposed Drug Price Verification Survey Policy is in Excess of CMS' Authority, Ignores the Value of CGTs to Patients and the Medical System, Improperly Risks Disclosure of Confidential and Proprietary Information, and Represents Poor Public Policy.

In the Proposed Rule, CMS proposes to survey manufacturers and wholesalers that directly distribute their CODs to obtain information about the prices they are reporting under section 1927(b)(3)(A) of the Act (hereinafter the "Survey"). According to CMS, the purpose of the Survey is to "verify prices reported under section 1927(b)(3)(A) of the Act to assure that Medicaid payments and applicable rebates for CODs can be made, and that Medicaid payments are economical and efficient, as well as sufficient, to provide access to care."[9] This Survey would not apply to all drugs, however; rather, the proposed selection criteria (e.g., a launch price greater than $500,000) would disproportionately target CGTs, which are generally priced as a one-time treatment and thus have a higher upfront cost relative to chronic therapies, even though the chronic therapy may ultimately cost more over the lifelong course of the treatment.

As described in greater detail below, the Agency lacks the statutory authority to proceed with this policy. The proposal also evinces a clear misunderstanding of the value of CGTs to patients and ignores states' existing tools and authorities that can already be leveraged to negotiate rebates for these products. It instead imposes arbitrary and burdensome reporting requirements unrelated to CMS' reported price verification authority or the economy or efficiency of Medicaid payments, requires the

[9] 88 Fed. Reg. at 34,268.

disclosure of proprietary and confidential information unrelated to value and without sufficient protection from disclosure, and would ultimately hinder equitable patient access to CGTs.

ARM therefore strongly urges CMS not to move forward with the Survey. On its face, this proposal appears to prioritize an arbitrary notion of "cost" over a benefit/risk assessment for individual patients and eschews even robust cost effectiveness assessments. ARM contends this is shortsighted given the potential that it will limit Medicaid beneficiary access to CGTs, which can actually improve patient outcomes and decrease overall healthcare system costs over the short and long term. Instead, ARM recommends that CMS work with states and with manufacturers on a voluntary basis to develop alternative methodologies for accommodating the frontloaded cost of CGTs. These therapies should not be penalized simply because the entire value of the therapy must be included in the price for a single administration. Instead, CMS should aim to incentivize their appropriate use given that they can address the root cause of complex diseases through one-time administrations for patients who otherwise have limited or no other treatment alternatives. In doing so, CMS would preserve, rather than threaten, continued innovation and development of new, groundbreaking treatments.

To facilitate access, CMS should encourage states to work with willing manufacturers to negotiate VBAs before the products are approved. Our members frequently engage state Medicaid programs regarding their pipeline, requesting clinical meetings to provide an overview and discussing various potential contracting arrangements. However, states are often hesitant to engage early in the drug development process, and in many cases offer only limited opportunities for manufacturer interactions during the pre-approval period. Engaging in these negotiations and reaching even preliminary agreements on access and other contracting terms (whether through a VBA or otherwise) before approval can help ensure timely access for patients once a product is on the market and available for administration. CMS should work with state Medicaid programs and manufacturers, in particular, to identify how the Agency can act as a "bandwidth extender" for states to be prepared for novel CGTs coming to market.

CMS should also issue guidance encouraging more states to pay separately for inpatient administered CGTs at their actual acquisition cost to enable opportunities for VBAs for those products. Since only a minority of states administer separate payment or have obtained authority from CMS to negotiate VBAs, we have yet to experience the impact on full utilization of VBAs by states on cost and access. For this reason alone, an

unreasonably expanded use of the MDRP as a blunt tool for the collection of additional rebates is premature and unnecessary until further utilization of VBAs expands across states.

For the reasons stated below, we oppose the Survey in its entirety. However, if CMS nonetheless proceeds with the Survey, ARM urges CMS to expressly exclude certain categories of drugs from the process given their potential benefit to address the root cause of disease, not just its symptoms, and not expand the Survey requirements to include drugs approved via accelerated approval.

A. The Proposed Survey Exceeds CMS's Authority and Lacks a Rational Basis.

CMS relies on two sections of the Act, section 1902(a)(30)(B) and section 1927(b)(3)(A), as authority for the proposed Survey. Section 1902(a)(30)(A) requires that payments under the Medicaid program "are consistent with efficiency, economy, and quality of care and are sufficient to enlist enough providers so that care and services are available under the plan at least to the extent that such care and services are available to the general population in the geographic area . . ." Section 1927(b)(3)(B), in turn, authorizes the Secretary to survey wholesalers and manufacturers that directly distribute their CODs, when necessary, to verify manufacturer prices reported under section 1927(b)(3)(A).

CMS interprets the term "verify" in section 1927(b)(3)(B) of the Act as requiring manufacturers participating in the MDRP to *justify* their reported prices, since "in some of these situations, there is a need for more information or verification regarding how certain prices or charges reported to us for these high-cost CODs are calculated in order to make payment under Medicaid."[10] CMS further asserts that "there is little or no public information available about the factors that influence the pricing of drugs dispensed in non-retail community pharmacy settings in Medicaid, the prices that pharmacies or wholesalers pay for these CODs, whether the prices or charges bear any relationship to the cost components of the COD, or whether the costs of distribution or preparation methods are included in the prices reported to us." According to CMS, for these reasons, states can only assume that drug prices are reasonably set and based on value, but such assumptions "may not be accurate since how the manufacturer arrives at its price is generally opaque."

[10] 88 Fed. Reg. at 34,269.

Based on the above reasoning, CMS proposes to combine the statutory authority in section 1927(b)(3)(B) (authorizing the verification of prices), with that of section 1902(a)(30)(A) (requiring that state expenditures in Medicaid be of the Act be consistent with "efficiency and economy") to require through regulation that manufacturers "verify" the prices reported to CMS through mandated data disclosures.

CMS' proposal contorts and unreasonably expands the agency's limited price reporting verification authority under section 1927(b)(3)(B) of the Act, and thus exceeds the agency's regulatory authority. Section 1927(b)(3)(A) establishes manufacturer requirements for the regular reporting of AMP, best price, ASP, WAC and nominal pricing information for drugs under the National Drug Rebate Agreement (NDRA). While section 1927(b)(3)(B) does authorize CMS to survey manufacturers to verify manufacturer prices reported under subparagraph (A), that provision contemplates the sharing of information about "charges or prices" for purposes of confirming the accuracy of reported prices. Nothing in these provisions authorizes CMS to require the reporting of data justifying the development of the reported prices, let alone any of the proposed transparency data described in the Proposed Rule, such as product and clinical information, cost of production, R&D and marketing costs.

Further, CMS' reliance on section 1927(b)(3)(B) as a basis for CMS' proposed civil monetary penalties also has no merit. As noted, since section 1927(b)(3)(B) gives CMS no authority to promulgate the Survey process in the first place, it provides no basis for the assessment of penalties associated with those provisions. Accordingly, CMS is particularly precluded from engaging in any additional enforcement mechanism relative to the Survey, including referrals to the HHS Office of Inspector General (OIG) and invoking section IV of the NDRA.

There is similarly no reasonable interpretation of section 1902(a)(30)(A) that would authorize CMS to administer a mandatory price justification regulation such as the Survey. By its own terms, the requirement that payments in Medicaid programs be consistent with efficiency, economy and quality of care refers to payments made to providers serving Medicaid beneficiaries. Indeed, CMS repeatedly neglects in the Proposed Rule to reference the entire statutory provision on which it relies, which reads in full: "[A state plan for medical assistance must] provide such methods and procedures relating to the utilization of, and the payment for, care and services available under the plan . . . as may be necessary to safeguard against unnecessary utilization of such care and services and to assure that payments are consistent with efficiency, economy, and quality of care **and are *sufficient to enlist enough providers so that care and services are available***

under the plan at least to the extent that such care and services are available to the general population in the geographic area" (emphasis supplied). Inasmuch as section 1902(a)(30)(A) contemplates that states must adjust their payments to providers to conform to the referenced statutory standard (that is, "efficiency, economy and quality of care" and sufficiency "so that care and services are available under the plan"), such a requirement would be inapplicable in the context of the MDRP under section 1927 of the Act, which includes no legal basis for states to adjust prices charged by manufacturers.

Finally, the Survey is also invalid because it lacks a rational basis. As noted, CMS has no authority under existing law to require manufacturers to adjust drug prices as a condition of participation in the MDRP. Yet, CMS' proposal to compel manufacturer justification of reported prices of drugs in the MDRP serves no rational basis other than just that—to pressure manufacturers into deeper supplemental rebates or other previously voluntary business arrangements such as outcomes-based agreements to avoid further regulation and significant penalties. That this is CMS's primary objective is supported by the mechanics of the Survey itself, which would relieve manufacturers from disclosure requirements depending on their willingness to pay significant levels of supplemental rebates to states, among other conditions. CMS also reveals as much in the Proposed Rule, stating, for instance that " . . .transparency into a manufacturer's costs and process for establishing a drug price via the survey, along with other factors, would give States the ability to better negotiate supplemental rebates, and better understand the impact of the drug on its budget as supplemental rebates are negotiated."[11] CMS further states that ". . . our proposal to make certain manufacturer information publicly available (unless it is proprietary), would give States an additional tool to negotiate payment for Medicaid CODs consistent with section 1902(a)(30)(A) of the Act."[12] Such a proposal is legally improper, since under section 1927(a)(1), the decision of a manufacturer to negotiate an SRA with a state Medicaid program is entirely voluntary. Any regulatory scheme designed to compel such negotiations conflicts with this controlling law and would be invalid on its face.

Further, from a public policy perspective, the survey proposal is particularly misguided in light of the current tools available to states now to lower drug costs. Manufacturers are already required to offer significant rebates to states as a condition of coverage of their drugs under Medicaid. States are also entitled to rebates based on Best Price, matching the deepest discounts offered to payors in the commercial market. In this

[11] 88 Fed. Reg. at 34,268.
[12] *Id.*

regard, Medicaid programs are uniquely situated among all other payors in terms of access to discounted drugs. Indeed, as CMS notes in the Proposed Rule, manufacturer rebates collected by the states totaled $42.9 billion, or 55.3% of total drug spending for 2022. Most states are also authorized through a SPA to solicit traditional supplemental rebates. [13]

Finally, manufacturers are already subject to significant price reporting requirements under section 1927 of the Act. The Proposed Rule would only add to those burdens and impose significant financial costs – these additional regulatory and financial burdens are further barriers for manufacturers to the development and production of new and innovative therapies for patients in need, particularly those who are critically ill with complex medical conditions, and for whom CGTs are the only treatment options.

B. The Survey's Drug Selection Measures are Based on Unsupported Assumptions Regarding the Prices and Value of CGTs.

CMS' proposed drug price verification survey depends entirely on one assumption—which CMS leaves unsupported—that CODs launched at or above price levels arbitrarily selected by CMS, and CGTs in particular, are unreasonably priced, i.e., priced at levels that exceed their value. There is no basis in fact for this flawed assumption; instead, there is evidence to the contrary.

As an initial matter, CMS offers no rationale for considering factors such as the proposed WAC price of a therapy, its claim spending, or treatment costs as a basis for targeting the therapy for the Survey. And it cannot do so because these criteria are not reasonably related to any consideration of the degree to which a therapy addresses unmet medical need and they do not account for the totality of the impact of a therapy on avoiding downstream medical costs for Medicaid programs and their patients. CMS has no basis to presume that CGTs, which are typically one-time administered with potentially durable and curative effect, are unreasonably priced simply because they can be more expensive in their single/first administration than prior therapies. This is an unfair comparison, as these prior therapies are generally utilized for the ongoing treatment of chronic conditions. And, as noted above, these criteria may unfairly create a selection bias against CGTs priced as one-time treatments, compared to chronic therapies, even though chronic therapies may cost more over the course of a treatment regimen. In doing so, this proposal could increase total costs to the program over time.

[13] Source: https://www.medicaid.gov/medicaid/prescription-drugs/downloads/sra-table-mar-2023.pdf

That CMS does not intend to be constrained by any consideration of the value of the targeted therapy or the extent to which its use may offset downstream medical costs is revealed by the Agency's singular focus on price and failure to mention of value in the preamble text supporting the proposal.[14]

C. The Proposed Transparency Data Have no Connection to Value.

Even if CMS were authorized to require pricing justifications, which it is not, CMS is not structured to evaluate the relative value or even the price of a given therapy in an expert or detailed manner, and proposes to use an arbitrary set of factors to do so in the Proposed Rule.

CMS admits, for example, that it "would not be using the survey data to further assess either the clinical or cost effectiveness of the COD."[15] In fact, CMS urges states to assess the reasonableness of a drug's price based on arbitrary factors such as "information on the costs of production, research, and marketing of the COD" – none of which bear any relation to a determination of a product's clinical value.[16] CMS provides no justification (nor can it) for its conclusion that "it is important to understand" these costs and "how those costs are accounted for in the prices and charges" reported by manufacturers for the purposes CMS claims to be conducting the Survey in the first place—to verify the accuracy of prices reported under section 1927(b)(3)(A) or to ensure the efficiency and economy of Medicaid payment rates. To the contrary, using "[c]osts of production, research, and marketing" to assess the appropriateness of drug prices would be counterproductive as it would support higher costs for drugs with less efficient manufacturing or greater research and marketing expenses, rather than the therapy with more beneficial impacts on patients.
In sum, through the Survey and its proposed identification and transparency requirements, CMS would improperly coerce manufacturers either to lower prices or to negotiate significant supplemental rebates through a regulatory scheme that targets CGTs in particular without any regard to value of the identified therapy.

D. The Proposed Transparency Requirements Require Disclosure of Proprietary and Confidential Information Without Sufficient Protection, and are *De Facto* Price Controls Threatening

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[14] *See* 88 Fed. Reg. at 34,270 ("Gene and cell therapy drugs especially, while transformative in terms of therapeutic benefits, are being priced in the millions of dollars"; "States, with their limited budgets, are concerned about how they would be able to afford these medications"; "We believe this verification is extremely important given the significant number of high cost drugs and biologics, including cell and gene therapy drugs entering the market.").
[15] 88 Fed. Reg. at 34,268.
[16] *Id.* at 34,270.

Continued Research, Development, and Innovation in the CGT Sector.

CMS' proposed transparency requirements would compel the production of manufacturer pricing information that is confidential and proprietary for, among other reasons, dissemination to states. CMS has not provided sufficient notice as to how the agency intends to protect any of this sensitive information from unauthorized use or disclosure. Nor has CMS indicated how, if at all, the confidentiality protections under section 1927(b)(3)(D) would apply. These questions are particularly problematic given that states generally lack any laws offering specific protections against public disclosure of the categories of information that would be requested as part of the Survey. In an apparent attempt to address this critical issue, CMS merely notes its "understanding" that "some of the data proposed to be collected would be confidential and likely protected under section 1927(b)(3)(D) of the Act, in addition to other privacy and confidentiality provisions, including the Trade Secrets Act." Finally, since a substantial amount of the information CMS proposes to require manufacturers to submit would be confidential and proprietary, the proposed transparency requirements are likely to result in lengthy processes and even disputes as to the extent and applicability of these protections.

CGT research and development is inherently risky. Manufacturers rely on confidentiality protections to remain competitive and increase the ability to satisfy investor-backed expectations in connection with successful product development and launches. The improper disclosure of confidential, proprietary and competitively sensitive information can pose significant risks to a company's commercial viability, particularly for those manufacturers engaged in early stages of product development in disease areas subject to competition by giving competitors an undue advantage. Congress recognized this fact by establishing clear protections for reported drug pricing information in the context of the MDRP at section 1927(b)(3)(D) of the Act. However, as even CMS admits, the extent to which this statute would protect all of the information subject to disclosure under the Survey is, at best, unclear. CMS cannot proceed with any transparency disclosure requirements in the absence of clear and enforceable confidentiality protections that would apply to all information submitted under any verification survey that bears any relationship to a manufacturer's pricing strategies.

Further, we are concerned that the Survey is effectively an attempt by CMS to administer *de facto* price controls on behalf of state Medicaid programs through forced supplemental rebate negotiations on the threat of increased regulation and, as noted above, risked disclosure of confidential information.

As such, the Survey will add even more barriers to entry for an industry that is already facing significant challenges in bringing highly complex and innovative drug products from the clinic and to the patient. Draconian regulations in the nature of federally-sanctioned price controls create even more risks for investors in our sector, and will be particularly damaging to the commercial viability of our early-stage members. In order to continue to innovate and flourish at this next level of groundbreaking science, CGTs need to be priced and paid for accordingly.

E. If CMS Proceeds with the Survey, the Agency Should Carve Out Certain Categories of Drugs and Should Not Automatically Include Drugs Approved under Accelerated Approval.

Alternatively, if CMS insists over our objections on proceeding with the flawed Survey concept, ARM urges CMS to expressly exclude the following two categories of therapies:

- **Drugs with Offered Value-Based Purchasing Agreements.** CMS' stated purpose for conducting the Survey is to support state efforts to enter into supplemental rebates including via VBAs, such as the CMS-endorsed multiple best prices model. Consistent with this purpose, CMS expressly excludes from the Survey process any drug for which the manufacturer already participates in a value-based purchasing arrangement with a state Medicaid program and/or has offered a VBA via the multiple best prices approach via the CMS portal, but only to the extent that savings under these arrangements represent a certain "level of effort" by the manufacturer based on input from states and a complex formula (at § 447.510(k)(3)(ii) of the Proposed Rule). Such an approach does not provide manufacturers sufficient foreseeability or certainty in terms of possible inclusion or exclusion of their drugs from participation in the Survey. For example, manufacturers with drugs that would already be subject to VBAs as of the effective date of the Proposed Rule, either through a CMS approved-VBA SRA or a multiple best prices agreement, would have no basis for predicting whether they would be at risk for inclusion on the initial or final Survey list.

  There is no rational policy basis for subjecting manufacturers already seeking to negotiate VBAs to the Survey and its associated transparency requirements. Indeed, most states have not yet even requested authority from CMS to negotiate VBA SRAs and, based on feedback received from our membership, some of them have concluded that VBAs are too burdensome to implement. Manufacturers seeking to negotiate VBAs should not be penalized in the event a state elects not to pursue the manufacturer's VBA offering, or because a state has not yet obtained authority to negotiate a VBA.

- **Drugs With Certain Special Designations.** Additionally, CMS should exempt from the Survey any drugs that have received one of certain special designations from the US Food & Drug Administration, including orphan designation, breakthrough designation, and regenerative medicine advance therapy (RMAT) designation. FDA has provided these designations to support patient access to therapies for serious and/or rare conditions and subjecting these therapies to unnecessary scrutiny could threaten patient access.

Along these lines, we also strongly discourage CMS from automatically applying the Survey to drugs approved via accelerated approval. In the Proposed Rule, CMS solicits comment on "surveying manufacturers of certain CODs that are identified under the proposed criteria at § 447.510(k)(2)(i) through (iv) that are also granted accelerated approval by FDA."[17] This solicitation overlooks the lifesaving value of these drugs and the legitimate challenges companies may face in bringing products to market. It also bears noting that, from 2007 to 2020, spending on accelerated approval drugs accounted for well below one percent of total Medicaid spending.[18] **Targeting accelerated approval drugs also undermines the very purpose of the pathway, which is to bring drugs to market for high unmet need.**

The accelerated approval pathway makes critical and innovative therapies available to patients years earlier than under traditional approval, resulting in improved access and clinical outcomes.[19] Established in 1992 and codified in 2012, the Accelerated Approval Program (AAP) allows FDA to approve products that treat serious or life-threatening diseases for which there is unmet need, based on data that demonstrates efficacy using a surrogate or intermediate endpoint that is believed to predict clinical benefit for the disease or condition. Categorically, accelerated approval drugs come in disease areas where there is high unmet need at the time of approval, including those that disproportionately affect vulnerable populations (e.g., HIV and cancer).

FDA's authority to approve drugs via the accelerated approval pathway was recently reinforced through legislation.[20] This legislation addressed recent criticism regarding the accelerated approval pathway by allowing the FDA to

[17] 88 Fed. Reg. at 34,272.
[18] Thorpe, Kenneth E., Quantifying Impact of Accelerated Approval Drugs, May 2022, https://www.fightchronicdisease.org/sites/default/files/FINAL%20Quantifying%20Impact%20-%20White%20Paper%20v6.pdf.
[19] Beakes-Read, G., Neisser, M., Frey, P. et al. Analysis of FDA's Accelerated Approval Program Performance December 1992–December 2021. Ther Innov Regul Sci (2022). https://doi.org/10.1007/s43441-022-00430-z.
[20] Enacted on December 29, 2022, the Consolidated Appropriations Act includes the Food and Drug Omnibus Reform Act (FDORA).

require confirmatory studies to be underway at the time of approval and to initiate expedited withdrawal when necessary. Given that this legislation was recently enacted, it seems premature to implement further policy changes focused on completion of confirmatory studies before the impact can be determined.

III. CMS Lacks the Statutory Authority to Require "Stacking" for Purposes of Reporting Medicaid Best Price, and its Implementation would be Infeasible and Harmful to Patients.

CMS proposes to revise 42 C.F.R. § 447.505(d)(3) to require manufacturers to "stack" *all* price concessions among *all* Best Price-eligible customers for a single drug unit as follows:

> The manufacturer must adjust the best price for a drug for a rebate period if cumulative discounts, rebates, or other arrangements to best price eligible entities subsequently adjust the price available from the manufacturer. Cumulative discounts, rebates, or other arrangements must be stacked to determine a final price realized by the manufacturer for a covered outpatient drug, including discounts, rebates, or other arrangements provided to different best price eligible entities.[21]

As a threshold matter, this proposed policy is inconsistent with CMS's statutory authority and, in particular, with any reasonable interpretation of the definition of Best Price in the MDRP statute.[22] Moreover, it would be infeasible to implement as manufacturers have no method of reasonably of tracking and aggregating multiple price concessions on a single drug unit throughout the pharmaceutical supply chain. Resulting exposures to duplicate rebate liability would further pressure manufacturers to withdraw price concessions altogether, which would not only undermine the benefit of Best Price for states, but would also restrict access by increasing the cost of medications for patients. ***For these reasons, ARM urges CMS to abandon its proposed "stacking" policy.***

ARM is confident that meaningful improvements in clinical outcomes and cost reduction can be accomplished through CGTs. ARM believes that CGTs have the potential to heal people suffering from complex medical conditions with limited or no other treatment options, and in the process bend the health cost curve toward lower long-term costs and higher quality outcomes. This trend is already evidenced by several approved and marketed first-generation regenerative medicine products that are demonstrating both

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[21] 88 Fed. Reg. at 34,292–93.

[22] Social Security Act § 1927(c)(1)(C).

clinical and cost reduction value. Accordingly, we could substantially reduce overall healthcare expenses by reducing hospital care, the need for physician, clinical and professional services, nursing, and home healthcare.

ARM believes that these fundamental principles should serve as the foundation for establishing any Medicaid payment policies. ***Contrary to the approach outlined in the Proposed Rule, CMS should take steps to help states accommodate the upfront costs of CGTs to ensure equitable access to CGTs for Medicaid patients.***

We thank CMS for its consideration of our comments. Please free to contact me at ecischke@alliancerm.org with questions.

Sincerely,

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