Alliance for Regenerative Medicine

July 20, 2020

Ms. Seema Verma, Administrator
Centers for Medicare & Medicaid Services
U.S. Department of Health and Human Services
P.O. Box 8016
Baltimore, MD 21244-8016

Re: CMS Proposed Rule on Value-Based Purchasing for Drugs Covered in Medicaid, CMS-2482-P

Dear Administrator Verma,

The Alliance for Regenerative Medicine ("ARM") appreciates the opportunity to provide comments to the June 19, 2020 Proposed Rule, which offers several innovative concepts that advance the use of value-based purchasing ("VBP") for drugs covered under the Medicaid program. We applaud and welcome CMS's efforts to address current barriers that impede greater adoption of VBP arrangements in Medicaid and look forward to working with you on this important issue.

About the Alliance for Regenerative Medicine

ARM is an international advocacy organization dedicated to realizing the promise of regenerative medicines and advanced therapies. ARM promotes legislative, regulatory, and reimbursement initiatives to advance this innovative and transformative sector, which includes cell therapies, gene therapies and tissue-based therapies. Early products to market have demonstrated profound, durable and potentially curative benefits that are already helping thousands of patients worldwide, many of whom have no other viable treatment options. Hundreds of additional product candidates contribute to a robust pipeline of potentially life-changing regenerative medicines and advanced therapies. In its 11-year history, ARM has become the voice of the sector, representing the interests of 350+ members worldwide, including small and large companies, academic research institutions, major medical centers, and patient groups.

ARM and its members have long recognized the need for alternative payment models to make regenerative technologies and therapies available in the U.S. health care system, and have actively engaged with CMS as well as private payors to discuss the adoption of more innovative approaches to reimbursement. VBP strategies are particularly well-suited for regenerative treatments due to complexities in how such therapies are administered, the severity of the

conditions treated, and the fact that many of the potentially curative treatments proposed will realize their full clinical and economic value over time.

The Need for Change in Current Regulations to Address VBP

ARM obviously is not alone in recognizing the importance of VBP in health care. CMS has consistently acknowledged the positive impact of such arrangements on patient access to life-saving drug therapies. At the same time, however, current law governing Medicaid best price reporting creates uncertainty for reimbursement models where (1) the cost of treatment could be refunded if the therapy did not meet certain committed outcomes for a particular patient or group of patients, or (2) the cost of treatment would be financed over time after an initial payment upon administration, whether those subsequent payments are fixed or vary based on the achievement of defined clinical outcomes. ARM members seek to negotiate novel pricing solutions for its regenerative technologies and therapies consistent with HHS's own goals and priorities, but have been reluctant to bear the risks that come with a reliance on ambiguous and undefined legal and regulatory compliance standards governing such arrangements.

We thank CMS for taking a significant step towards filling in the gaps described above with the issuance of this Proposed Rule. While ARM and its members have worked diligently with CMS and state agencies to explore various options for VBP, we foresee more rapid and meaningful progress when all stakeholders may operate from consistent federal standards. This is especially important for the types of groundbreaking technologies that ARM members offer through newly developed and forthcoming cell and gene therapies. As stated in the Proposed Rule, a lack of further regulation from CMS "may hinder Medicaid access to the care and services provided as part of these VBP arrangements (for example, to gene therapies and potentially curative orphan drug treatments) that are available in the general population and that are consistent with efficiency, economy, and quality of care in accordance with section 1902(a)(30)(A) of the Act."[1] ARM agrees wholeheartedly with your comments in a recent *Health Affairs* publication, that "with dozens of gene therapies in the development pipeline, it's time we allow more creative approaches to drug reimbursement that promote wider access for patients."[2]

General Comments to Proposed Rule Related to the Support of VBP for Drugs Covered in Medicaid

We include below the collective comments from ARM and its members on certain aspects in the Proposed Rule that are critical to the increased adoption of VBP in Medicaid, and consequently, other government and commercial health care programs. As we offer our thoughts on specific elements of the Proposed Rule, we emphasize several overarching themes that are reinforced time and again as we examine the technical details involved:

- Regulations that address VBP should be constructed to allow for the greatest amount of flexibility to accommodate the wide range in the types of cell and gene technologies available.

[1] 85 Fed. Reg. 37286, 37291 (June 19, 2020).

[2] "CMS's Proposed Rule on Value-Based Purchasing for Prescription Drugs," Health Affairs, June 17. 2020.

- Manufacturers and payers should be allowed considerable discretion to negotiate the terms of their VBP arrangements, as they are the parties best situated to determine the ideal model for payment that achieves their combined goals. This includes the ability to enter into a VBP arrangement without the need to file a Medicaid state plan amendment ("SPA"), or at the very least allowing states the flexibility to determine whether a SPA is necessary to enter into a VBP arrangement with manufacturers.

- The solution cannot be worse than the problem. Manufacturers and/or state Medicaid agencies will remain hesitant to enter into VBP arrangements if the proposed alternatives to address best price reporting and other barriers are too administratively burdensome or cause further confusion and uncertainty in the setting of drug prices.

- Changes to Medicaid best price reporting may have unintended consequences for other drug price reporting measures, including AMP and ASP, that in turn, will impact payments for drugs reimbursed under Medicare Part B or offered through the 340B Drug Pricing Program. CMS must take into account how the changes discussed in the Proposed Rule may be pulled through to these other drug pricing measures and programs. If the Final Rule does not appropriately address these issues, it will be less likely to promote the goals of VBP.

- CMS should also consider how pay-over-time arrangements may be factored into best price, AMP and other price reporting, especially as (1) each installment payment may stretch across multiple rebate reporting periods; (2) installment payments may not necessarily be contingent on outcome-based measures, but may merely spread fixed payments over time in order to avoid higher upfront costs or investment and better finance the transaction; and (3) manufacturers may use multiple contracting strategies with different payers for the same covered outpatient drug in the same quarter.

New Definition for Value-Based Purchasing Arrangement at 42 C.F.R. § 447.502

ARM is in full support of CMS's proposal to codify by regulation a formal definition for VBP arrangements. We believe the suggested definition for VBP arrangements is broad enough to include many different forms of VBP arrangements that are worth exploring by manufacturers and payers, including those that employ measures that are both evidence-based and outcome-based.

It is critical that any rules governing the use of VBP in Medicaid not be overly restrictive so as to eliminate reimbursement models from consideration. These arrangements are carefully negotiated between the manufacturer and payer based on the nuances presented with each type of drug therapy under consideration. Some drug therapies may require multiple administrations over several days or may be administered over the course of several months or even several years. Certain treatments may be intended for patients with rare diseases, where the total pool of patients eligible for such therapies is extremely small or where measurable outcomes are difficult to define for the purposes of a VBP arrangement because of the slow progression of the disease and a heterogeneous patient population. Clinical results for some drugs may be obtained

rather easily, and observed over a shorter period of time, while other drug outcomes take years to reveal. Manufacturers and payers must have a full range of VBP arrangement options at their disposal.

CMS solicits feedback on one consideration related to the definition of VBP arrangements: how to interpret the term "substantially" as used in the proposed definition, and more specifically, how much of the drug's final cost should be associated with evidence-based and outcome-based measures in order to be considered VBP. We understand CMS's concern that the allowances made for VBP arrangements in this Proposed Rule may be applied in arrangements with only minimal links to evidence-based or outcome-based measures, or that other types of rebates, refunds and price concessions that are not performance-driven would be improperly characterized as VBP. However, ARM does not believe that there is a substantial risk that the definition of VBP arrangements will be misapplied or misconstrued. In addition, we feel that any attempts to impose arbitrary thresholds undercuts the benefits of a broad definition of VBP.

First, the use of the term "substantially" does not have a material effect on the reading and interpretation of the definition of VBP arrangements. Evidence-based and outcome-based measures used in this context must by their very nature "link" drug costs and prices to effectiveness, outcome, and performance. The reliance on a "substantial" link does not help further define or clarify the relationship, and attempts to impose thresholds like tying 90% of the cost of the drug to such measures in order for the arrangement to be considered to be VBP are likely to be arbitrary and overly restrictive. For example, consider one drug therapy where patient outcomes can be easily monitored through a real-time tracking device, like a glucose monitor, and where we are able to confirm within a matter of weeks that the treatment was and will continue to be effective. These arrangements are best suited for VBP arrangements where a higher percentage of a drug's cost are tied to outcome-based measures, especially where such outcomes are easy to detect and verify in a short period of time.

At the same time, another drug therapy, equally beneficial or even curative for a certain patient population, requires regular medical assessments by a physician or at a specialized facility over the course of several years in order to ascertain clinical outcomes. Manufacturers would need to account for higher rates of non-participation and non-compliance among its tracked patient population, and as such, may link a smaller proportion of the drug cost with outcome-based measures than in the previous example. Using an arbitrary percentage of drug costs or other thresholds to demonstrate the existence of "substantial" links between drug costs and these measures unfairly favors VBP arrangements for the first type of drug therapy over the second.

Ultimately, ARM believes that VBP arrangements are sufficiently defined in the proposed regulation, and that the term "substantially" can and should be stricken from the definition entirely. VBP arrangements should reflect the terms that the manufacturer and payer feel are most appropriate given the type of drug therapy and the clinical benefits expected, without the hindrance of arbitrary thresholds to define payment models that are clearly value-based. In addition, given that commercial insurers will likely serve as primary adopters of VBP, CMS has a

model from which to assess what is standard and customary in the marketplace, thus reducing the need to add further context or definition in the regulation.

Revision of the Definition of Bundled Sale to Include VBP

ARM supports the modification of the existing definition of a bundled sale in 42 C.F.R. § 447.502 to include VBP arrangements to help ensure that a price concession associated with one failed outcome is not reflected as the lowest price for best price reporting. We recommend, however, that the definition of a bundled sale explicitly incorporate by reference the new proposed definition of VBP arrangement as described above. The revision to the definition of a bundled sale as currently proposed would utilize an abbreviated version of the definition of VBP arrangements by just citing to arrangements with "a performance requirement such as an outcome(s) measurement metric."[3]

The bundled sale proposal is important to facilitate VBP arrangements under certain circumstances, but we urge CMS to finalize both the bundled sale and the multiple best price approach in the Final Rule given that manufacturers and payers, including Medicaid, must have flexibility to tailor a VBP arrangement to a specific approved therapy and patient population. Consider the following example: the benefit of the bundled sale concept is to "smooth" the impact of a failed outcome-based measure by allocating the price concession associated with the failure across all the drugs in the bundle subject to the performance requirement. While this has clear benefits for drug therapies that have a larger number of patients and/or dispensed units, the smoothing effect will not be as effective with drug therapies treating rare and orphan diseases. It is conceivable that some drug therapies may be prescribed and administered to only a few patients each year, so there would not be a high number of units across which to allocate a large price concession associated with a failed outcome. In fact, there may very well be scenarios where drugs intended to treat certain rare conditions may be prescribed to only one patient in a given state, thus literally lacking any other sales to bundle. CMS should ensure other methods are available to accommodate these unique circumstances. Specifically, in addition to finalizing the multiple best price approach, discussed in more detail in the next section, CMS also should work with stakeholders to identify additional, acceptable model options that fit within the flexibilities created by a Final Rule. CMS can use the full range of regulatory actions available, including reasonable assumptions discussions and guidance, to do so.

Reporting of Multiple Best Prices

ARM believes the use of multiple Medicaid best prices for a drug creatively accommodates VBP arrangements where options for regulatory change are somewhat limited given the applicable statutory requirements in place. While we fully support and promote the ultimate inclusion of VBP within the list of recognized exemptions to current Medicaid best price reporting, ARM understands that this overarching approach falls outside the scope of the Proposed Rule.

[3] 85 Fed. Reg. 37292 and 37319.

We believe that manufacturers reporting multiple best prices for a specific drug based on a VBP arrangement complies with Medicaid best price reporting rules and accommodates instances even where 100% of the cost of the drug was rebated and reported as such. The issues that ARM raises in this section concerning the use of multiple best prices across multiple price points are operational in nature. Without thoughtful clarification from CMS in the ensuing Final Rule, it would be challenging to implement VBP arrangements in the way CMS intends moving forward. We respectfully request that CMS commit in the Final Rule to issuing more detailed, timely guidance through the various mechanisms it has available, including through State Medicaid Director letters and Participating Drug Manufacturer Releases. We also ask CMS to ensure opportunities for additional stakeholder input, whether through industry and stakeholder calls or future RFIs. Nevertheless, ARM provides what we believe are key considerations with regard to this approach below:

- **Operational and Administrative Impacts**. Despite current capabilities to report multiple best prices manually, all systems and reporting mechanisms used in Medicaid best price reporting will likely need to be upgraded, if not overhauled, to be able to track and report sets of best prices based on particular pricing structures. The Proposed Rule does not provide technical interpretation or guidance behind the concept of multiple best prices, and this in turn may result in a great deal of inconsistency in how various state Medicaid programs will obtain, interpret and use any data manufacturers may provide. At a certain point, manufacturers and/or state Medicaid agencies may opt out entirely if the requirements are too complex to effectively manage

- **Delays in Implementation**. The Proposed Rule acknowledges that "it will take us time to make such changes." We are concerned that the operational changes necessary to accommodate multiple best prices are so significant that we could see delays of at least 2-3 years before implementation, which only serves to further postpone greater adoption of VBP. Clarifications in the Final Rule would help mitigate that potential delay, as would iterative, subsequent guidance issued by CMS to tackle details that may be too technical for rulemaking. We also urge CMS to undertake this process in a way that allows for stakeholder feedback and engagement.

- **Consistency between AMP and Best Price**. CMS intends to "provide consistency between AMP and best price," and discusses making certain conforming changes to 42 C.F.R. § 447.505(d)(3) and its determination of AMP to match the language in 42 C.F.R. §447.504(f)(3). It is unclear how these proposed changes address the entirely new concept of multiple best prices proposed. We address these concerns in more detail in a subsequent section, where we respectfully suggest further guidance or rulemaking to appropriately address such implications.

- **Implications for 340B Drug Pricing Program**. Under the 340B Drug Pricing Program for eligible "safety net" providers, the 340B ceiling price is the "maximum price that covered entities may permissibly be required to pay for the drug,"[4] and is calculated as the AMP for the drug minus the Medicaid unit rebate amount, i.e. the sum of the basic and additional rebate, for the same quarter. It is unclear how the use of multiple best prices and the associated unit rebate amounts will be factored into 340B drug pricing. However, ARM believes that the 340B ceiling price for a given drug therapy should not factor in prices based on multiple best price or VBP arrangements, but rather be calculated based solely on manufacturers' non-VBP pricing. If a drug therapy is

[4] Public Health Service Act, § 340B(a)(4). *See also* 42 C.F.R. § 10.3.

offered exclusively through VBP, then the 340B ceiling price should reflect the aggregate negotiated price in the VBP arrangement prior to any refunds or reimbursements triggered by non-responding patients. Recognizing that CMS does not have sole jurisdiction over the 340B program, we respectfully ask CMS to work with the Health Resources and Services Administration ("HRSA") to issue clarifying guidance for manufacturers to facilitate the uptake of VBP in the short term.

Exceptions to the 12 Quarter Period for Adjustments to Best Price and AMP

ARM fully supports CMS's proposal to allow an exception to the 12 quarter rule for the adjustment of AMP or best price reporting data to account for VBP arrangements and pay-over-time models. As CMS states in the Proposed Rule, many of these types of arrangements "may be better suited for periods longer than 12 quarters, and manufacturers entering into such arrangements may need to adjust AMP and best prices beyond the 12 quarters because the evidence-based or outcomes-based measures are being measured beyond a period of 12 quarters."[5] We agree with CMS's decision not to simply increase the number of quarters, but to generally allow payment adjustments outside of the 12 quarter window when a VBP arrangement includes outcomes that must be evaluated during longer intervals of time. ARM is in favor of setting reasonable parameters tied to the length of the contract term to both accommodate the need for time beyond the 12 quarters but not demanding constant resubmission and adjustment of best price and AMP in perpetuity.

Consequences for Calculating and Reporting AMP

ARM emphasizes that Medicaid best price and all the various drug reporting measures are interconnected, so that changes to how Medicaid best price is reported must also be factored into how other price reporting, including for AMP, is calculated. For instance, while CMS's proposed guidance addresses to some extent how pay-over-time arrangements, whether outcome-driven or not, should be calculated for best price reporting purposes, the Proposed Rule does not offer sufficient guidance as to how AMP should reflect pay-over-time or installment payment arrangements. Would manufacturers report AMP as the price paid at the time of sale, which would include only the first installment payment and represent only a portion of the drug's total purchase price? Would subsequent installment payments be reported in future quarters rather than recognized upfront? For installment payments that are contingent on outcomes set forth in VBP arrangements, how would manufacturers report AMP if expected payments did not occur because VBP outcomes measures were not achieved?

Most critically, if CMS were to offer regulatory guidance on one price reporting measure and not another, this would generate significantly different price reporting results for the same drug under the same agreement. In order to ensure a greater degree of consistency, ARM believes that manufacturers should be permitted to report AMP as the full price of the drug at the time the drug is administered, even if installment payments would extend to subsequent quarters.

[5] 84 Fed. Reg. 37301.

This aligns with CMS's proposed changes for best price reporting. For VBP arrangements where installment payments are contingent on patient outcomes, any expected installments that are not paid due to failure should be treated as a price concession and reported as such not only for best price reporting purposes, but also for AMP.

Other Items Related to VBP that Should Be Addressed by CMS or HHS

ARM appreciates CMS's efforts to clear current regulatory barriers that serve to restrict widespread adoption of VBP in Medicaid and other settings. We would like to take the opportunity to raise other items related to VBP that should be addressed by CMS or HHS in order to further advance the use of these arrangements, including the following:

- **Drug Therapies that Fall Outside the Definition of "Covered Outpatient Drug**." Only drug therapies that fit the definition of a "covered outpatient drug" as defined in Section 1927(k)(2)-(4) of the Social Security Act are subject to the Medicaid Drug Rebate Program. We recognize that therapies that fall outside of this statutory definition and its regulatory interpretation are not in scope for this rulemaking. Nevertheless, several of ARM's members are developing innovative cell and gene therapies appropriate for inclusion in a VBP arrangement, but, for one reason or another, may not fit the definition of a covered outpatient drug and are not subject to the Medicaid Drug Rebate Program. Given the potential of these therapies to improve patient health outcomes and overall health care costs in the short and long term, we encourage CMS to work with stakeholders to consider ways to implement VBP with any interested manufacturer for an FDA-approved therapy that treats Medicaid beneficiaries, and to account for these important drug therapies in future rulemaking.

- **ASP and Medicare Part B Reimbursement**. Manufacturers participating in the Medicaid Drug Rebate Program must report to CMS on a quarterly basis the ASP of certain of the manufacturer's covered outpatient drugs. Similar to best price and AMP reporting, ASP must include price concessions offered by the manufacturer, including those that may result from VBP arrangements. We encourage CMS to provide corresponding guidance for calculating ASP and Medicare Part B reimbursement in light of VBP as well.

- **Patient Portability Issues**. Patient portability issues remain a significant challenge in the ability to monitor patient outcomes over longer periods of time. In many cases, a patient that is lost to follow-up efforts may reflect treatment success, and that information is critical to collect not just to determine VBP payments but for clinical and therapeutic considerations. Although not specifically addressed in the Proposed Rule, CMS should consider options it may have at its disposal to require all payers to track or assist in the tracking of patient outcomes over the term of the VBP arrangement, even as patients change plans and move in and out of coverage. Alternatively, if a manufacturer pays a third party to track patient outcomes over time, fees associated with those administrative services should be excluded from best price and AMP calculations and reporting.

- **Anti-Kickback Statute Safe Harbor**. ARM has previously submitted comments to the OIG to advocate for an explicit VBP regulatory safe harbor to the federal anti-kickback statute. While existing safe harbors may offer some degree of protection for VBP arrangements, the very nature

of VBP precludes the use of safe harbors that could otherwise protect such arrangements from liability under the anti-kickback statute. In addition, OIG's continued reliance on a case-by-case examination of potential anti-kickback implications does not provide enough assurance to manufacturers or payers desiring to enter into these arrangements. CMS should work with OIG to ensure that rulemaking and guidance related to VBP for both the Medicaid Drug Rebate Program and the anti-kickback statute are harmonized. The OIG has taken the first critical step by issuing the October 17, 2019 Proposed Rule to revise safe harbors under the anti-kickback statute to accommodate VBP and to codify an exception to civil monetary penalty rules against beneficiary inducements.[6] As Secretary Azar noted in his remarks to the American Health Lawyers Association on March 20, 2019, "the rules we need to have governing our payments should look different, because our payments look different – and we want them to look more different still."

Exclusion of Certain Manufacturer-Sponsored Patient Assistance Programs from Best Price and AMP

ARM would like to take the opportunity to provide brief comments on CMS's proposal to exclude certain manufacturer-sponsored patient assistance programs from best price and AMP determinations. It appears from the Proposed Rule that CMS is concerned that manufacturers are improperly leveraging exclusion criteria for programs that provide a drug discount or co-payment assistance to *patients* in order to also exclude price concessions that benefit health plans instead. CMS suggests as a remedy to exclude manufacturer patient assistance only if the manufacturer ensures that the full value of the assistance is passed through to the patient and that no benefits are passed through to the plan. We believe that this course of action inappropriately assumes manufacturers can require health plans to share this type of information with them. If finalized, this proposal would only serve to exacerbate patient access and ability to pay issues, as manufacturers will be hesitant to commit themselves to offering such programs if they have difficulty tracing and guaranteeing that that 100% of the assistance goes to the patient alone. ARM urges CMS not to finalize this particular proposal.

We would be pleased to engage in further discussion with CMS on the above. Thank you in advance for your consideration of the comments and recommendations included in this letter. If you have any questions or need any additional information, please contact Robert Falb at 202-320-7602 or rfalb@alliancerm.org.

Sincerely,

Robert J. Falb

Robert Falb
Director, U.S. Policy and Advocacy
Alliance for Regenerative Medicine

[6] 84 Fed. Reg. 55694.