March 23, 2021

Melanie Bella, MBA, Chair
Medicaid and CHIP Payment and Access Commission
1800 M Street NW
Suite 650 South
Washington, DC 20036

Sent via: comments@macpac.gov

Re: Payment and Coverage of High-Cost Specialty Drugs: Report from the Technical Advisory Panel

Dear Chair Bella,

The Alliance for Regenerative Medicine (ARM) submits the following comments to the Medicaid and CHIP Payment and Access Commission's ("MACPAC") January 29, 2021 proposal and March 4 discussion to create a differential rebate for drugs receiving an accelerated approval by the Food and Drug Administration ("FDA") ("Proposal").[1]

ARM appreciates MACPAC's systematic effort to study the issue of prescription drug usage and access in the Medicaid program. However, we question the decision not to integrate stakeholder feedback more broadly throughout this process, which began with the convening of a technical advisory panel (TAP) to discuss the specialty drug pipeline, the impact these drugs present to Medicaid, and potential new payment and coverage models. For example, opening up one or more of the various TAP meetings (which we understood took place in November, December, and March) would have allowed key stakeholder groups like patients, providers, and innovative medicines developers to provide perspective and feedback on specific payment and coverage models, their design parameters, and the potential effect on these various stakeholders. Given the scope and potential impact of the resulting Proposal and the aforementioned process, ARM is concerned that Medicaid and CHIP patients may lose early access or face significant access delays to the very therapies most needed by many Medicaid and CHIP patients.

ARM is the leading 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

[1] https://www.macpac.gov/publication/payment-and-coverage-of-high-cost-specialty-drugs-report-from-technical-advisory-panel/

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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 global voice of the sector, representing the interests of 380+ members worldwide, including small and large companies, academic research institutions, major medical centers, and patient groups.

ARM and its members have long recognized that the current reimbursement paradigm can stymie timely patient access to the most innovative therapies, specifically cell and gene therapies, given that it was devised decades ago when these therapies were not yet a reality. We continue to advocate for modernizing payment systems to accommodate these therapies once approved while responsibly protecting healthcare system sustainability. For this reason, ARM is a firm advocate of the need for innovative payment models to make regenerative technologies and therapies available in the U.S. health care system. Among other activities, we have actively engaged with the Centers for Medicare & Medicaid Services (CMS). Congress and private payors to discuss the adoption of alternative approaches to access and reimbursement. All three of these major stakeholder groups have collaborated to advance the implementation of such models. In fact, ARM supported CMS' recent creation of a flexible value-based construct within the Medicaid Drug Rebate Program to facilitate these types of arrangements.[2]

Based on these experiences, we encourage MACPAC to explore alternative reimbursement and access models as a means to address the Commission's stated concerns around the completion of FDA confirmatory trial commitments. Such an approach is preferable to increasing manufacturer Medicaid rebate liability as proposed, which can: (A) harm some of the most vulnerable patient populations disproportionately (i.e., those for whom there are no, or limited, existing treatments and significant unmet medical need); and (B) deter investment in these therapies over the longer term, countermanding the intent of Congress in establishing the accelerated approval pathway in the first place.

I. Background: MACPAC's Proposal to Create a Differential Rebate for Dugs Approved under the FDA's Accelerated Approval Pathway

During MACPAC's March meeting the Commissioners heard a presentation on how states can better manage the costs of drugs approved through the FDA's accelerated approval pathway. Specifically, the TAP proposed, and the Commissioners discussed a policy recommendation for Congress to create a higher Medicaid drug rebate for all drugs that have been approved via the FDA's accelerated pathway that have not yet completed confirmatory trials. This increased rebate would be added as a statutory change to the Medicaid Drug Rebate Program and increase the minimum rebate above the current 23.1 percent of average manufacturer price. In support of this Proposal, the TAP states that "increasing the rebate would provide a lower net price to

[2] 85 Fed. Reg. 87,000 (December 31, 2020).

help account for the uncertainty that the product will produce the anticipate clinical benefit. Medicaid will pay less while there is a limited amount of evidence. Additionally, the higher rebate would create a financial incentive for manufacturers to complete confirmatory trials in a timely fashion."[3] Once manufacturers, however, complete their confirmatory trials the rebate would revert to the standard amount.[4]

II. The FDA Accelerated Approval Pathway maintains the Agency's gold-standard safety and efficacy standards while taking into account novel trial design necessary and appropriate for certain disease with high Unmet Medical Needs

In 2012, Congress passed the Food and Drug Administration Safety Innovations Act ("FDASIA"). Section 901 of FDASIA amends the Federal Food, Drug, and Cosmetic Act ("FD&C Act") to allow the FDA to base accelerated approval for drugs for serious conditions that fill an unmet medical need on whether the drug has an effect on a surrogate or an intermediate clinical endpoint. As part of this process, drug companies are still required to conduct studies to confirm the anticipated clinical benefit. These studies are known as Phase 4 confirmatory trials. If the confirmatory trial shows that the drug actually provides a clinical benefit, then the FDA grants traditional approval for the drug. If the confirmatory trial does not show that the drug provides clinical benefit, FDA has regulatory procedures in place that could lead to removing the drug from the market.[5]

ARM believes that the accelerated approval pathway aligns with FDA's sterling safety and efficacy standards. Moreover, it achieves the optimal balance of providing early access to life saving therapies for which there is an unmet medical need while also requiring the continued clinical data gathering exercise to determine the full scope of clinical benefit. Depending on the disease in question, it can take many years to learn whether a drug effects longer-term outcomes like survival and quality of life. A positive therapeutic effect that is clinically meaningful in the context of a given disease is known as "clinical benefit." A surrogate endpoint used for accelerated approval is a marker - a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit but is not itself a measure of clinical benefit. Likewise, an intermediate clinical endpoint is a measure of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on irreversible morbidity and mortality (IMM).[6] Using a surrogate or intermediate clinical endpoint under the accelerated approval pathway enables the FDA to speed safe and efficacious therapies to patients who often have few, or no, other treatment options.

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[3] https://www.macpac.gov/wp-content/uploads/2020/08/MACPAC-March-2021-Meeting-Transcript.pdf. At page 95.

[4] https://www.macpac.gov/publication/payment-and-coverage-of-high-cost-specialty-drugs-report-from-technical-advisory-panel/ At page 9.

[5] https://www.fda.gov/drugs/information-health-care-professionals-drugs/accelerated-approval-program

[6] Id.

The FDA bases its decision on whether to accept the manufacturer proposed surrogate or intermediate clinical endpoint on the scientific support for that endpoint. The studies that demonstrate a drug's effect on a surrogate or intermediate clinical endpoint must be "adequate and well controlled" as required by law. Using surrogate or intermediate clinical endpoints for serious diseases with unmet medical needs, and factoring in all other safety and efficacy evidence, provides for appropriate benefit/risk analysis in the drug approval process. For example, instead of having to wait to learn if a drug extends survival for cancer patients, the FDA may approve a drug based on evidence that the drug shrinks tumors, because tumor shrinkage is considered reasonably likely to predict a real clinical benefit. In this example, an approval based upon tumor shrinkage can occur far sooner than waiting to learn whether patients actually lived longer. This accelerated approval process has worked for many drugs in turn positively impacting thousands of lives.

ARM is concerned that MACPAC's Proposal to create a differential rebate for drugs approved under the accelerated approval pathway will disrupt the balance that Congress and the FDA have achieved to approve 141 new therapies covering a wide range of live saving therapies that treat many diseases improving the lives of thousands of patients.[7]

III. MACPAC's Proposal Lacks Detail and Therefore Needs Further Discussion Before Going to Congress

During its January and March meetings, MACPAC's Commissioners recognized the significant, negative impact that this proposal could have on patient access to newly approved drugs during their discussion of this Proposal.[8]

ARM believes that this is a significant issue such that the Commissioners should spend more time digesting and understanding instead of immediately proceeding to a vote at the April meeting. Additionally, ARM is concerned that the lack of clarity on the full economic impact that this Proposal could have on overall drug spending, taking into account the current healthcare spending on disease states most likely to have a treatment come to market through the accelerated approval pathway, before proceeding to a vote. As the Commissioners stated, this Proposal could actually lead to higher drug prices thereby defeating the purpose of providing financial relief to the states.[9]

Due to the need for more understanding on the impact that this Proposal could have on drug development, access to new drugs, and drug pricing for some of the most vulnerable patient populations, ARM urges MACPAC not to vote to move forward with the recommendation around increasing the rebate amount for accelerate approval therapies. As stated earlier, ARM believes that there are many other policies that could

[7] Global Data Drugs Database

[8] https://www.macpac.gov/wp-content/uploads/2020/08/MACPAC-March-2021-Meeting-Transcript.pdf At page 105.

[9] Id. At 111.

meet MACPAC's goals of reducing drug costs that also more effectively increase access to and maintains a robust innovative environment.

IV. Conclusion

ARM encourages MACPAC to continue to collaborate with the health economic field to monitor the potential future inclusion of these dimensions. ARM appreciates the opportunity to provide our perspective on these important issues. Please do not hesitate to contact me if you have any questions.

Sincerely,

Robert J. Falb,
Director, U.S. Policy and Advocacy