PERSONALIZED MEDICINE COALITION

July 2, 2024

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Department of Health and Human Services
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Sent electronically to IRARebateandNegotiation@cms.hhs.gov

Re: Medicare Drug Price Negotiation Program: Draft Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price in 2026 and 2027

Dear Deputy Administrator Seshamani:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 200 institutions from across the health care spectrum, thanks the Centers for Medicare & Medicaid Services (CMS) for the opportunity to submit comments on CMS' draft guidance for implementation of the Medicare Drug Price Negotiation Program for the initial price applicability year (IPAY) of 2027.[i] This draft guidance includes some improvements compared to the draft guidance for IPAY 2026. We believe, however, that it continues to lack transparency and clear descriptions for procedures and methodology that will be used to negotiate a drug's maximum fair price (MFP). Because few details are provided on how personalized medicine will be considered and in light of recent studies demonstrating the potential negative impacts of the program, our comments build upon those shared with CMS on IPAY 2026.[ii] We urge CMS to take every step possible to prevent, monitor, and correct for potential unintended impacts of the program on patients and the health care system.

Personalized medicine is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients. Personalized medicine is playing an important role in transforming care and patient outcomes for a range of serious and life-threatening diseases and conditions, helping to shift patient and provider experiences away from trial-and-error medicine and toward a more streamlined process for making clinical decisions.

After initial approval of a small-molecule drug, biologic, orphan drug, or genetically targeted therapy by the U.S. Food and Drug Administration (FDA), further research can provide greater understanding of patients' responses to treatment based on results

from molecular diagnostics. This research leads to new or improved treatment indications that contribute to progress in personalized medicine.

We believe PMC and CMS share the goal of achieving better health outcomes and removing patient access barriers. We urge CMS to refine its negotiation process so that it does not disrupt the innovation ecosystem and patient access to personalized medicine by ensuring that:

- CMS establishes processes to prevent, monitor, and correct for any unintended, downstream impacts of the program on patient access to personalized medicine and on pipelines for new personalized medicine treatments and expanded indications;
- CMS' methodology to determine a selected drug's MFP recognizes the clinical and societal benefits of personalized medicine and incorporates patients' perspectives on care value;
- CMS' methodology and negotiation process establish consistency and transparency by communicating how factors considered are weighed and how external data are factored into its decisions;
- CMS establishes procedures that allow a robust exchange of information with manufacturers, patient organizations, and other stakeholders in determining the MFP throughout the negotiation process; and
- Patients do not face additional barriers in accessing negotiated medicines and their treatment alternatives, as well as non-negotiated medicines.

Statement of Neutrality

Many of PMC's members will present their own responses to the *Medicare Drug Price Negotiation Program Draft Guidance for IPAY 2027 and Manufacturer Effectuation of the MFP in 2026 and 2027* and will actively advocate for those positions. PMC's comments are designed to provide feedback so that the general concept of personalized medicine can advance, and are not intended to impact adversely the ability of individual PMC members, alone or in combination, to pursue separate comments with respect to the draft guidance and/or any that follows.

Monitoring Unintended Impacts on Personalized Medicine

Personalized medicines have accounted for at least a quarter of new drug approvals for each of the past nine years.[iii] Medicare's drug price negotiation program could have an outsized effect in discouraging the pharmaceutical industry from bringing additional personalized medicines and expanded indications to the market. Multiple analyses, including those from the Congressional Budget Office (CBO), have called attention to the potential consequences of the Medicare drug price negotiation program, such as canceled research and development and disincentives to invest in small-molecule medicines and therapeutic areas that require incremental innovation.[iv,v,vi,vii] Legislators have also questioned CBO's initial analysis for underestimating such impacts of the program.[viii] **CMS should take every step possible to prevent, monitor, and correct for potential impacts of the program on patients and the health care system.**

Indications for smaller patient subpopulations

Due to smaller patient subpopulations, personalized medicines that address the root causes of disease can sometimes be expensive and risky to develop. In 2023, a record 61 percent of new personalized medicines approved by the FDA were to treat rare diseases, with 27 percent indicated for certain

cancers.[ix] There are more than 10,000 rare diseases, including rare cancers, and more than 90 percent of them do not have an FDA-approved treatment.[x] With companies expected to focus on treatments for larger patient populations where return on investment can be easier, the pursuit of indications for smaller patient populations could be delayed or forgone. Thus, treatment pipelines for cancers and rare diseases could be especially impacted by Medicare's drug price negotiation program.[xi,xii] **CMS should monitor impacts of the program on the development of personalized medicines for smaller patient subpopulations, including patients with unmet medical needs.**

Small-molecule drugs

Many targeted cancer therapies that deliver personalized medicine to patients are small-molecule drugs.[xiii] According to *IRA* statute, small-molecule drugs are eligible for negotiation nine years after approval versus 13 years for biological, or large-molecule, products. PMC is concerned that implementation of these differential timelines will disincentivize investment in small-molecule over large-molecule drugs. Small-molecule drugs comprise 70 percent of the drugs selected for negotiation in IPAY 2026. Such drugs are likely to make up 93 percent of the drugs selected for IPAY 2027 and 87 percent of the drugs in IPAY 2028.[xiv] Small-molecule oncology therapies are also estimated to be predominantly affected during the program's first few negotiation cycles.

These dynamics may impact the growing pipelines of personalized medicines available to patients, including patients from communities already experiencing disproportionately high incidence and mortality rates of certain diseases like cancer. One analysis estimates 79 fewer small-molecule drugs and 188 fewer indications coming to market over the next 20 years.[xv] To reduce the impact of differential timelines for drugs and biologics on clinical development for small molecules and patients who need these critical therapies, PMC supports Congress amending the *IRA* to establish equal timelines for the negotiation of both drugs and biologics at 13 years. **CMS should also monitor impacts of the negotiation program on the development of small-molecule personalized medicines.**

Post-approval research and expanded indications

In identifying drug products for negotiation, CMS broadly interprets the statute to aggregate drugs for selection based on a single active moiety, or ingredient, across multiple New Drug Applications (NDAs) or Biologics License Applications (BLAs). As drug products age and approach eligibility for price negotiation, companies may be disincentivized to pursue additional indications, which can require additional approvals after the original NDA or BLA approval. PMC is concerned that the negotiation program will deter incremental innovation supported by post-approval research, including the development of expanded indications that provide patients with personalized medicine treatment options.

Research conducted after approval of a new drug is important for advancing personalized medicine. After initial approval of a targeted therapy by FDA, further research provides greater understanding of patients' responses to treatment based on results from molecular diagnostics. This research leads to new or improved treatment indications that contribute to progress in personalized medicine. But smaller patient subpopulations can make it difficult to recoup investment in this research, which can require additional clinical trials and NDAs or BLAs. One white paper examining six products in chronic diseases, rare diseases, and cancer found that nearly half (seven out of 15) of the applications for expanded indications were approved at about the same time or after the product could have been selected to begin negotiation (at seven or 11 years).[xvi] Another peer-reviewed analysis of 50 drugs with the highest Medicare Part D

spending in 2020 found that 30 were small-molecule drugs, and 56 percent of these small-molecule drugs received FDA approval for expanded indications more than seven years after their initial FDA approval.[xvii]

Over the past nine years, PMC has identified more than 130 expanded indications significant to advancing personalized medicine.[xviii] Notably, these expanded indications have had an upward trend in the average time since a drug's initial approval. Since these expanded indications can increase the product's aggregated utilization and risk for earlier selection, the drug price negotiation program can alter manufacturers' decision-making for investing in researching new uses for a drug post approval, potentially affecting patients with serious conditions or unmet needs. **CMS should monitor impacts of the negotiation program on post-approval research into expanded indications for both small- and large-molecule drugs. CMS should also consider opportunities to implement its statutory requirements in a way that does not undermine incentives for post-approval research critical to personalized medicine.**

Orphan drug products and additional rare designations

Currently, certain orphan drugs are excluded from the Medicare Drug Price Negotiation Program, but the exclusion only applies to orphan drugs that treat one rare disease or condition. If an orphan product has designations for multiple diseases, even if these are also orphan designations, then it loses its exclusion from negotiation. The agency will use the earliest date of approval or licensure to determine when the product is eligible for negotiation. Only about one quarter of all orphan drugs approved in the last two decades have a single indication.[xix] Researching additional orphan indications for existing rare disease treatments plays an important role in identifying new treatments for patients with rare diseases who do not have treatments available to them.

PMC is concerned that this narrow exclusion could stifle post-approval research into additional orphan indications for rare diseases. Even when making investment decisions among multiple potential orphan indications, manufacturers may be incentivized to prioritize indications for rare diseases with larger patient populations over indications for very rare diseases. PMC believes this narrow exclusion contradicts the goals of the *Orphan Drug Act* to foster the development of new treatments for rare diseases. PMC recognizes CMS is limited by the *IRA* and supports legislation to broaden the orphan drug exclusion in statute by ensuring orphan drugs treating one or more rare diseases or conditions are excluded and by clarifying that the countdown to eligibility for price negotiation would begin only when an orphan drug loses its exclusion. **Still, we encourage CMS to monitor impacts of the new program on the development of orphan products and research into additional orphan designations for patients with rare diseases.**

Genetically targeted therapies

Genetically targeted therapies (GTTs) work by either delivering healthy copies of genes to target cells, permanently changing the genetic code, or manipulating gene expression. If a GTT silences a gene, it is regulated as a drug, but if a GTT adds to a gene, it is regulated by the FDA as a biologic. Despite differences in their pathways for regulatory approval, GTTs are similar in time of development, therapeutic action, and complexity of manufacturing. As a result of the unequal negotiation timelines, GTTs regulated as drugs would be negotiated after only nine years, whereas GTTs regulated as biologics would be negotiated after 13 years. These different timelines under the *IRA* impose an artificial

distinction that could lead to a lack of parity in the development of these novel therapies.

Of the dozen or so GTTs with an approved NDA to-date, all are personalized medicines that treat patients with rare diseases. While only a limited number of GTTs are on the market now, the underlying technology is expected to generate novel therapies for non-rare diseases in the future. To ensure the even advancement of all GTTs in this promising area of personalized medicine, PMC would support statutory changes treating all GTTs as biologics that could be negotiated after 13 years. **Meanwhile, CMS should monitor for disparate impacts of the program on the development of GTTs regulated as drugs versus biologics.**

Collecting information

PMC asks CMS to collect information on the unintended impacts discussed above to ensure the negotiation program does not disincentivize the development of new treatments for unmet medical needs; research on expanded indications that provide additional benefits to patients; patient access to personalized medicine through cost-control practices, like prior authorization or step therapy; or have other impacts on health equity. Related data CMS could consider tracking include changes in NDAs and supplemental NDAs and changes in formulary placement and utilization management for negotiated versus non-negotiated drugs, as discussed below.

Recognizing the Clinical and Societal Value of Personalized Medicine

Drugs with personalized medicine treatment strategies create considerable benefits for patients and society since they are used in a manner that directs them toward patients who are most likely to benefit and away from those who are not. Value assessment frameworks (VAFs) often draw sweeping conclusions, however, about the economic worth of a particular treatment, typically based on analysis of its safety and effectiveness at a population level. In many cases, value assessment methodologies fail to adequately account for the safety and effectiveness benefits that may be realized by individual patients or patient subpopulations. When assessing value, it is important to consider the holistic benefits of a treatment at the patient, subpopulation, and societal levels, including to underserved or underrepresented populations facing inequities in access to care.

PMC appreciates CMS' reference to patient experiences in its discussion of the clinical benefits of selected drugs and their therapeutic alternatives in Sec 60.3.3 of the draft guidance, as well as CMS' proposal to evaluate health outcomes for specific populations, including through an access and equity lens. Although CMS has broadened its consideration of patient experiences to now include caregiver perspectives; changes to productivity, independence, and quality of life; and other factors of importance to patients and caregivers, it is still unclear how input from patients, caregivers, and providers will influence CMS' analysis of clinical benefit and whether CMS may consider the benefit of personalized medicine. **In general, PMC urges CMS to consider the following aspects of clinical and societal value related to personalized medicine that advance patient-centered care,** [xx] **ensuring that the value of personalized medicine to direct patients toward or away from treatments based on their likelihood to benefit from them is factored into determining the MFP for a selected drug:**

1. **Diagnostic testing strategies:** Diagnostic tests can help guide treatment decisions and determine which treatments will be most effective and safest for any given patient. Such testing is a crucial element of the personalized treatment regimen. For example, the use of companion diagnostics

can help define subpopulations of patients who may benefit from a treatment, and those who will not. The availability of diagnostic tests and consideration of test results that help inform treatment decision-making for drugs with biomarker implications must be figured into the value assessment methodology for personalized medicines. **PMC encourages CMS to consider the value of applicable diagnostic strategies in its evaluation of unmet medical need and clinical effectiveness.**

2. **Heterogeneity of treatment effects:** Some patients will experience more or less benefit from a treatment than suggested by the averages reported within clinical trials and population-based data. Health care policies based on averages misjudge and undervalue personalized medicines simply because the data required for value-based decision-making do not account for patient subpopulations or because long-term efficacy data is not yet available. **PMC encourages CMS to consider the full range of patient outcomes and benefits that may not be represented in population average-based data.**

3. **Patient values and circumstances:** Personalized medicine depends not only on the consideration of a patient's molecular and biological characteristics but also on individual values, clinical and economic circumstances, and the potential impact of a therapy for that patient over the long term. Fundamental patient values and preferences, including the impact of treatment on quality of life, quantity vs. quality of time, functional ability related to illness or treatments, cost of supportive care, and other patient costs of treatment are weighed by patients and their caregivers when deciding on a treatment in consultation with health care providers. Although CMS attempts to broaden its definition for "unmet medical need" under IPAY 2027, we believe this definition continues to be too narrow to appropriately assess the value personalized medicines provide to patients with unmet medical needs. **PMC encourages CMS to further expand its definition of "unmet medical need" proposed in guidance to formally consider a broad range of patient outcomes and impacts, including unmet medical needs unique to individual patients and to patient subpopulations.**

4. **Treatment efficiency:** Although value assessments generally focus on improvements in effectiveness, they do not generally consider avoiding ineffective or harmful treatment options and reducing the downstream expenses associated with rapid disease progression and/or adverse events. In order to capture economic as well as clinical value, value assessments need to consider costs and outcomes across health care. **As CMS evaluates the costs and benefits of personalized medicines to society, PMC encourages the agency to formally consider a broad range of economic impacts beyond just the proposed consideration of changes to a patient's productivity, including broader cost offsets and societal benefits, like treatment efficiency.**

It is clear both in the statute and in CMS' guidance that quality-adjusted life years (QALYs) will not be used as a basis for evaluations. The QALY and other similar metrics do not sufficiently account for the broad heterogeneity of clinically relevant characteristics and preferences across patients and diseases, nor do they consider aspects of value defined by patients and their families. These measures rely on population averages that do not consider the heterogeneity of patient populations, even within the same condition.

While CMS states it will follow statute, the revised IPAY 2026 guidance suggested CMS may explore

QALY-like alternatives and the draft IPAY 2027 guidance indicates that CMS still plans to separate and exclude QALY metrics from evaluations of research that otherwise factor in QALYs when such content is "relevant and allowable." PMC is concerned that this approach may not effectively separate QALYs from CMS' analysis because CMS may continue to rely on studies that employ QALY or QALY-like data from secondary sources, or that CMS may exclude analyses that are otherwise helpful in establishing the value of a drug for a patient. Regarding CMS' *Negotiation Data Elements Information Collection Request* that asks the public to submit information on a selected drug, we appreciate that CMS now asks submitters to indicate whether their submission contains information from studies that use QALYs and to provide a short description of any cost-effectiveness measures included in the research they submitted and how they believe the data avoid the use of the QALY measure. **However, PMC also requests that CMS specify how it will exclude QALY-based and other similar metrics from its analysis of such evidence, how it will determine such content is relevant and allowable, and how this evidence would be weighed. PMC also requests that CMS highlight when and how the agency removed QALY-based metrics from consideration in its public explanation of a drug's MFP.**

For IPAY 2026, CMS requested input on what alternative measures to QALYs might be appropriate or inappropriate. PMC believes the agency would be better served by focusing on the factors related to comparative clinical outcomes and unmet need that are described in statute, which can better capture the benefits of personalized medicine, rather than seeking an alternative to the QALY or using another metric based on the QALY. There is not one measure of value or one VAF that holistically captures the value and benefits of any medical treatment or outcomes important to patients in every disease area. VAFs have strengths and limitations relative to different stakeholder perspectives and circumstances that can bolster or undermine their usefulness and applicability to personalizing patient care. A single measure will not be sufficiently comprehensive.[xxi] **We continue to encourage CMS to consider a wide variety of measures consistent with CMS' statutory focus on comparative effectiveness research and unmet need, especially those driven by patient experience data, patient input, and patient-centeredness.**

Establishing a Consistent and Transparent Process for Gathering and Evaluating Evidence

CMS indicates it will consider real-world evidence, peer-reviewed research, expert reports or white papers, clinician expertise, and patient experiences when reviewing the clinical benefit of a selected drug and its therapeutic alternatives (Sec. 60.3.3). Considering that all medicines for which CMS will set an MFP will have a minimum of nine years since their original FDA approval, **PMC encourages CMS to consider as broad an array of evidence sources and outcomes as possible to help fill gaps in population-based data sources and capture the full range of personalized medicine's benefits to patients and the health care system discussed above.** We thank CMS for considering information on underserved and underrepresented populations that may be experiencing disparities in health outcomes or access to a selected drug.

Although CMS' draft guidance lists aspects related to the quality and completeness of evidence sources it will consider, such as peer review, study limitations, risk of bias, and study population, among others, CMS does not describe requirements for the quality and completeness of this data, nor how CMS would consistently evaluate this evidence in determining the MFP. For example, since studies using RWE are designed fit-for-purpose, CMS' methodology should consider the extent to which the evidence it considers was designed to answer the value questions it is asking. The approach outlined in the initial guidance is too vague to create consistency across negotiations. **To ensure that the agency is evaluating these elements in a way that considers the value of personalized medicine to patients, CMS should**

refine its methodology through notice-and-comment rule-making to provide more clarity on how the agency intends to leverage negotiation factors outlined in Sec. 50.2. For real-world evidence in particular, CMS should describe what data sources it plans to use and create guidelines to ensure that the data used are robust and correctly utilized.

Specifically, CMS should outline a consistent methodology for how it will synthesize evidence and for how data related to therapeutic alternatives will result in changes to an initial offer or final negotiated MFP. In addition, CMS should not use cost as a criterion for selecting therapeutic alternatives. While multi-criteria decision analysis (MCDA) may not be feasible for CMS because it requires extensive time, resources, and expertise, CMS may be able to incorporate elements from, for example, the cost-consequence approach model to compare evidence on outcomes for certain therapies. CMS should continue to consider opportunities to adopt elements from MCDA into its framework for evaluating evidence, as the agency identified in its revised guidance for IPAY 2026.[xxii] As part of CMS' methodology, we ask CMS to prioritize data related to the factors described above for recognizing the full range of personalized medicine's benefits to patients and the health care system. Given the discount already reflected in a selected drug's ceiling price, **we recommend that when these factors are taken into consideration, the MFP for a selected drug be set at the ceiling if it demonstrates significant patient, clinical, or societal benefit.**

Even though CMS intends to employ a qualitative approach to considering the evidence between different selected drugs, CMS' methodology should clearly explain how each data element is weighted in determining the initial offer and final MFP. **To account for the clinical and societal benefits of personalized medicine and incentivize continued research and development for this field, CMS should place more weight on the factors related to the benefits of the selected drug for patients, caregivers, and society – including evidence on its benefit to patients experiencing health disparities – over, for example, non-clinical manufacturer-specific data elements.**

Establishing a clear and consistent process for gathering and evaluating evidence, including the information provided by stakeholders during the patient-focused listening sessions, can help manufacturers, patient groups, and other third parties better understand the evidence they may need to prioritize or collect for CMS' future consideration. Transparency can also build beneficiaries' confidence that their preferences and values are important to the agency.

Facilitating Meaningful Stakeholder Engagement

PMC thanks CMS for responding to previous stakeholder feedback by establishing patient-focused listening sessions in the IPAY 2026 revised guidance. We recognize that CMS has a tight timeline for drug selection and price negotiation. However, in order to ensure MFPs adequately reflect the value of selected treatments for patients and to limit unintended consequences on patients' access to personalized medicine, **CMS must meaningfully engage patients, caregivers, providers, manufacturers, regulators, and other third parties throughout the negotiation process. Third parties, including patients and patient organizations, should be allowed ample time and opportunities to share data and experiences related to selected drugs, and they should be informed by CMS about how their input is being used during the negotiation process.**

CMS solicits comments on how to improve and potentially restructure its patient-focused listening sessions in IPAY 2027. Organizations like the National Health Council have published recommendations

for how CMS can improve patients' experiences in these listening sessions and their overall engagement in the negotiation program.[xxiii] **PMC recommends CMS consider how to foster robust, bi-directional communication between public stakeholders and the agency, and we encourage the agency to adopt recommendations from patient advocacy organizations for how they and individuals from underrepresented communities can be most meaningfully engaged in the negotiation process.** In addition, although we appreciate CMS' intention to consult with clinical and academic experts to help evaluate clinical benefit of a selected drug, **we ask CMS to outline how clinical and academic experts would be identified and consulted during the negotiation process.** For example, CMS could establish a panel of patients, clinicians, and other stakeholders to provide feedback throughout each drug negotiation.

We appreciate CMS' interest in improving the submission of information from the public through its *Negotiation Data Elements Information Collection Request*, such as by grouping and revising questions to align with a respondent's area of expertise and by soliciting information about the factors patients care about most in assessing the value of a drug. CMS' proposed timeline, however, only allows one month from when the list of selected drugs is announced for the public to provide written information on the selected drug and therapeutic alternatives to inform CMS' initial offer. We believe this short and singular timeframe for written public input does not allow a sufficient window for stakeholders who may have information on the value of a treatment to their patient population to collect and provide information that could improve CMS' decision-making. In addition, this timeframe will disadvantage patients and caregivers from or organizations working with underserved communities, who have fewer resources and may find it challenging to respond in such a short timeframe. **CMS should consider the burden of data collection and submission on stakeholders. We ask CMS to allow patients, caregivers, clinicians, and organizations representing these groups additional time to submit the requested data in writing after the list of selected drugs is published. In addition to informing CMS' initial offer for a selected drug, CMS should allow this information to be submitted during subsequent steps of the negotiation process, if initiated, to inform CMS' decision-making.** Flexibility with the submission of public information would facilitate the inclusion of a broad range of patient perspectives, including those of communities underrepresented in existing studies and published literature.

To help build public trust in the process and ensure predictability informs stakeholder participation in listening sessions and data submission during future years of the negotiation program, CMS must be transparent about how it considers information provided. We thank CMS for intending to publish an explanation of the factors that had the greatest influence in determining a drug's MFP, including a narrative explanation and redacted information regarding the negotiation data elements received, exchange of offers and counteroffers, and the negotiation meetings. (Sec. 60.6.1). We remain concerned, however, that the explanation may not provide adequate detail to be meaningful to the public and that its timing – after stakeholders will have submitted data to inform the next cycle of negotiations – will make it irrelevant for stakeholders seeking to inform CMS' next cycle of negotiations. **In CMS' explanation for the MFP, we ask the agency to explain which information submitted by the manufacturer and the public was or was not considered in the final MFP; the benefits and impacts considered; the data sources considered; how evidence influenced the MFP up or down, including the extent to which real-world evidence and patient-centered data elements like patient experience data were used; which third parties were engaged, both formally and informally by CMS; and, as discussed above, the extent to which and how any evidence used to inform the MFP was separated from a QALY-based metric. In addition, so that stakeholders understand how the information they provide in one negotiation cycle is used before they submit information to the next, we support**

CMS' efforts to publish the explanation of the MFP earlier than its statutory deadline.

Ensuring Coverage Policies Facilitate Patient Access to Negotiated Drugs

Medicare's drug price negotiation program could narrow patients' access to existing treatment options in personalized medicine. PMC has previously submitted comments to CMS on the difficulties utilization management practices, such as prior authorization and step therapy, can create for patients in accessing the latest treatments and standards of care informed by personalized medicine.[xxiv,xxv,xxvi] We share CMS' concerns that plans may be incentivized to disadvantage selected drugs with utilization management that is not based on medical appropriateness, potentially exacerbating an already growing trend in the use of step therapy and its embedding in prior authorization requirements.[xxvii,xxviii,xxix] Because negotiated drugs are being offered to plans at a lower price, **PMC believes negotiated drugs should not face additional cost-control practices that could limit eligible Medicare beneficiaries' access to them. While PMC thanks CMS for identifying several criteria the agency will use to assess whether plans meet requirements for covering negotiated drugs through its existing formulary review process, including instances where plans impose more restrictive utilization management for a selected drug compared to a non-selected drug in the same class, we disagree with CMS' proposal to defer on implementing explicit policy requirements. We request CMS clarify specifically the extent to which any utilization management will be permitted for negotiated drugs.**

Although Medicare plan sponsors will be required to include selected drugs on their formularies, without additional guardrails, plans could use restrictive utilization management or other cost-control practices to manage their increased liability by preferring non-negotiated drugs or denying coverage for negotiated products vital to a patient's personalized health care. **To ensure patients are protected from plan attempts to offset costs, CMS should establish robust guardrails and conduct oversight to ensure the clinical appropriateness of any utilization management or formulary changes and to mitigate unintended consequences on beneficiaries' access to both negotiated and non-negotiated drugs and the narrowing of patients' treatment options. In particular, CMS should ensure that patients who are stable on their current medications maintain access to these medications as the negotiation program is being implemented, whether these medications are negotiated or non-negotiated drugs.** Following CMS' recent final rules regarding nondiscrimination protections[xxx] and collecting data regarding the health equity implications of utilization management under Medicare Advantage,[xxxi] **PMC also encourages CMS to address how it plans to monitor and address the real-world impacts of any utilization management changes on health equity.**

Conclusion

As the agency continues to implement the drug price negotiation program, we urge CMS to carefully consider these comments for this and future guidance. PMC looks forward to working with you and your colleagues to ensure the program maintains the ecosystem for innovation in personalized medicine and fosters patient access to needed personalized medicine treatments. If you have any questions about the contents of this letter, please contact me at 202-499-0986 or cbens@personalizedmedicinecoalition.org, or David Davenport, PMC's Manager of Public and Science Policy, at ddavenport@personalizedmedicinecoalition.org or 804-291-8572.

Sincerely,

Cynthia A. Bens
Senior Vice President, Public Policy

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[i] Center for Medicare. *Medicare Drug Price Negotiation Program: Draft Guidance, Implementation of Sections 1191 – 1198 of the Social Security Act for Initial Price Applicability Year 2027 and Manufacturer Effectuation of the Maximum Fair Price (MFP) in 2026 and 2027*. May 3, 2024. https://www.cms.gov/files/document/medicare-drug-price-negotiation-draft-guidance-ipay-2027-and-manufacturer-effectuation-mfp-2026-2027.pdf. (Accessed July 2, 2024.)
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