PERSONALIZED MEDICINE COALITION

October 12, 2021

Chiquita Brooks-LaSure
Administrator
Centers for Medicare and Medicaid Services
Department of Health and Human Services
Attention: CMS-5528-P
7500 Security Boulevard
Baltimore, MD 21244-1850

Sent electronically

Re: Most Favored Nation (MFN) Model (CMS-5528-P)

Dear Administrator Brooks-LaSure:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 200 institutions across the health care spectrum, thanks the Centers for Medicare and Medicaid Services (CMS) for the opportunity to comment on the proposed rule to rescind the Most Favored Nation (MFN) Model.[i] The model is intended to reduce the cost of prescription drugs in the United States, doing so by establishing prices for the top 50 Medicare Part B drugs with reference to the lowest available price in economically similar countries.

PMC supports CMS' decision to withdraw the MFN Model, which we believe is deeply flawed. In particular, the approach adopted by this model may reduce patient access to the treatments that are most appropriate to their needs, interfering with use of therapies that are best suited and most promising to their personal clinical situation and thereby cutting the efficacy of their care.

The proposed rule states that the withdrawal of the MFN Model "does not reflect any judgement by HHS regarding future policy." Therefore, we remain concerned that CMS will continue pursuing similar models or policies that rely on reference pricing to reduce drug costs. PMC urges CMS to avoid such approaches and to instead recognize the value of tailored approaches to individual patient care, developing strategies that preserve patient access to personalized medicine and facilitate its rapid adoption in 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 cause disease. 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.

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Personalized medicine is helping to shift the patient and provider experiences away from trial-and-error care of late-stage disease in favor of more streamlined strategies for disease prevention and treatment. PMC's members are leading the way in personalized medicine and recommend that patients who may benefit from this approach undergo appropriate testing and tailored treatment as soon as possible during their clinical experiences.

Based on the potential of personalized medicine to target treatments to those will benefit, we believe this approach holds the greatest potential for improving patient outcomes and reducing overall health care costs without jeopardizing patient access to the health care interventions they need. Accordingly, we urge CMS, as it considers how best to address drug costs in the future, to sustain and support personalized medicine.

Statement of Neutrality

Many of PMC's members will present their own responses to CMS and will actively advocate for those positions. PMC's comments are designed to provide input that can help advance the concept of personalized medicine in general and are not intended to constrain the ability of individual PMC members, alone or in combination, to pursue separate comments with respect to the MFN Model.

Preserving Patient Access to Current and Future Personalized Medicines

The number of personalized medicines approved by the U.S. Food and Drug Administration has grown from five in 2008 to 286 in 2020.[ii] PMC's annual analyses of new drug approvals also highlight that personalized medicines have accounted for more than 25 percent of newly approved drugs each of the last six years.[iii] Some of the advances we have observed that are beginning to transform patient care include novel cell and gene therapies, additional targeted therapies for rare and infectious diseases, and the first tissue-agnostic cancer therapies that act on specific biomarkers present in a patient as opposed to a tumor or cancer type.

Based solely on their costs, a number of drugs that are essential to the delivery of personalized medicine would be included in the MFN Model and could be included in similar models that rely on reference pricing. These drugs include life-saving targeted treatments and immunotherapies for diseases most commonly experienced by older adults and other vulnerable Medicare beneficiaries. Subjecting these treatments and those like them to reference pricing would reduce their availability. For example, CMS' Office of the Actuary determined that the MFN Model could contribute to a significant reduction in patient access to medications that might cause them to accept less effective treatments or forgo care altogether.[iv]

Experts continue to quantify the anticipated shifts in drug development that could result from reference pricing policies. A recent study from researchers at the University of Chicago found that price controls such as reference pricing would reduce drug research and development by as much as 60 percent by the year 2039, which might result in as many as 340 fewer new drugs coming to market.[v] In an analysis of one legislative proposal aimed at reducing drug costs, the Congressional Budget Office (CBO) wrote that due in part to international reference pricing, CBO would expect to see between eight and 15 fewer

drugs reaching the market in the coming decade alone.[vi] While it is difficult to know in advance the nature of the drugs that might be affected or to quantify the effect of foregone innovation on health outcomes, one study forecast that the reductions in drug development would occur disproportionately in hard-to-test conditions requiring long outcomes trials, such as Alzheimer's disease, cardiovascular disease, and rare diseases.[vii]

Instead of contemplating approaches that will limit access to many available treatments and put new discoveries at risk, CMS can advance personalized medicine in ways that allow patients and their providers to access the treatments that will be most effective, maximizing improvements in individual patient outcomes by tailoring care to a patient's genetics and other unique factors.

Recognizing and Valuing Patient Heterogeneity

Some patients will experience more or less benefit from treatments than suggested by averages reported from clinical trials. While advancements in personalized medicine have led to a greater understanding of the genetic and molecular heterogeneity underpinning variable treatment effects, many widely accepted health technology assessments (HTAs) and methodologies utilized for assessing value focus on population health, thereby overlooking efficiencies in individual patient care. Relying on such methodologies will have negative consequences on the ability of patients to access effective care.

By not accounting for patient heterogeneity, HTAs and current methodologies for assessing value neglect to adequately consider the fundamental element that comprises value in health — the patient. PMC appreciates the Department of Health and Human Services (HHS)' acknowledging in its recent report to the White House that "there are important concerns about the equity implications of certain methodologies, such as Quality Adjusted Life Years (QALYs)."[viii] We agree that drug pricing policies and new models of care should avoid methodologies that adversely impact access to needed medications, especially for vulnerable populations.

Ultimately, decisions about which therapy is most appropriate for a patient must be left to the patients working with their providers; involve consideration of each patient's clinical circumstances and preferences; and involve consideration of a therapy's long-term impact. Utilizing personalized medicine strategies, providers can identify individuals within larger populations who are more or less likely to respond to certain therapies. CMS should consider how these strategies can facilitate improvements and efficiencies at the population level by ensuring that patients who are most likely to benefit from new therapies receive them.

Appropriate Consideration of Diagnostic Tests in Future Models

The detection or measurement of biomarkers plays an important role in determining value across numerous clinical scenarios. Scientific knowledge of these biomarkers is rapidly advancing, and appropriate diagnostic testing is crucial for realizing the potential of personalized medicine. Diagnostic testing helps define personalized medicine's value and underline its potential to improve outcomes while reducing costs. The MFN Model did not consider diagnostic testing intended to help guide treatment decisions where appropriate or the value they provide in getting the right medicine to a patient as early

as possible.

We believe the importance of diagnostic testing and biomarker detection within certain clinical scenarios should be recognized in any drug pricing model's methodology. Therefore, it is imperative that future model development consider diagnostic testing an integral part of clinical decision-making. Failure to explicitly address this important aspect of care will undermine the ability of personalized medicine to maximize patient benefits while reducing avoidable and unnecessary health care costs.

Conclusion

Thank you for considering our views. We support CMS' withdrawal of the MFN Rule and would oppose similarly misguided drug pricing policies that would interfere with the delivery of personalized medicine. PMC welcomes the opportunity to serve as a resource for you to assist in shaping policies that improve beneficiary access to personalized medicine tests and treatments so that they achieve the goal the Coalition shares with CMS of delivering appropriate, efficient, and accessible health care to patients. If you have any questions about the content of this letter, please contact me at 202-499-0986 or cbens@personalizedmedicinecoalition.org.

Sincerely yours,

Cynthia A. Bens
Senior Vice President, Public Policy

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[i] Centers for Medicare & Medicaid Services. *Most Favored Nation (MFN) Model*. August 10, 2021. https://www.federalregister.gov/d/2021-16886.

[ii] Personalized Medicine Coalition. *2020 Personalized Medicine Report: Opportunities, Challenges, and the Future.* http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_The_Personalized_Medicine_Report_Opportunity_Challenges_and_the_Future.pdf.

[iii] Personalized Medicine Coalition. *Personalized Medicine at FDA: The Scope & Significance of Progress in 2019.* http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PM_at_FDA_The_Scope_and_Significance_of_Progress_in_2019.pdf.

[iv] Centers for Medicare & Medicaid Services. *Most Favored Nation (MFN) Interim Final Rule with Comment Period.* November 27, 2020. https://www.federalregister.gov/documents/2020/11/27/2020-26037/most-favored-nation-mfn-model.

[v] T.J. Philipson and T. Durie. *Issue Brief: The Evidence Base on the Impact of Price Controls on Medical Innovation.* September 2021. https://cpb-us-w2.wpmucdn.com/voices.uchicago.edu/dist/d/3128/files/2021/08/Issue-Brief-Price-Controls-and-Drug-Innovation-Philipson.pdf.

[vi] Congressional Budget Office. *Effects of Drug Price Negotiation Stemming From Title 1 of H.R. 3, the Lower Drug Costs Now Act of 2019, on Spending and Revenues Related to Part D of Medicare.* October 11, 2019. https://www.cbo.gov/system/files/2019-10/hr3ltr.pdf.

[vii] Vital Transformations. *H.R. 3 – Medicare D Reform: Calculating the Impact of International Reference Pricing on California's Biopharmaceutical Innovation Ecosystem*. October 30, 2019. http://vitaltransformation.com/wp-content/uploads/2021/04/HR3_4.5.21_v10.1.pdf.

[viii] *Comprehensive Plan for Addressing High Drug Prices: Report to the White House Competition Council.* September 9, 2021. https://aspe.hhs.gov/sites/default/files/2021-09/Drug_Pricing_Plan_9-9-2021.pdf.