PMC
PERSONALIZED
MEDICINE COALITION

May 5, 2016

Mr. Andrew Slavitt
Acting Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Hubert H. Humphrey Building, Room 445-G
200 Independence Avenue, SW
Washington, DC 20201

By electronic delivery

Re: Medicare Program; Part B Drug Payment Model [CMS-1670-P]

Dear Mr. Slavitt:

I am writing on behalf of the Personalized Medicine Coalition (PMC) to express serious concerns with the "Part B Drug Payment Model" proposed March 8 by the Centers for Medicare and Medicaid Services (CMS). PMC, which is comprised of more than 240 member institutions representing a wide range of stakeholders, believes that the proposal could impede patient access to targeted therapies and inhibit continued progress in personalized medicine.

PMC appreciates the Administration's commitment to advancing personalized, or precision, medicine as demonstrated by the recent announcements of both the National Institutes of Health (NIH)'s Precision Medicine Initiative (PMI) and Vice President Biden's National Cancer Moonshot initiative. As a nation, we find ourselves in a time of unprecedented opportunity, where scientific and medical advances are poised to transform the lives of patients.

Before focusing on the Part B Drug Payment Model, let me first thank you for engaging with the personalized medicine community. In December of last year, Marc Hartstein, Director, Hospital and Ambulatory Policy Group, spoke at PMC's Public Policy Committee meeting. He addressed community concerns related to the implementation of the Patient Access to Medicare Act of 2014 (PAMA) and outlined his desire to work with the community to engage laboratories and implement the law.

PMC is committed to helping CMS with that engagement. We appreciate CMS' work to advance high-value, individualized health care, and we believe personalized medicine has an important role to play in achieving this goal.[1]

However, we are concerned that the recently proposed Part B Drug Payment Model holds significant risk of hindering patient access to today's personalized medicines and slowing future progress. We therefore respectfully ask that you withdraw the proposed rule and engage the stakeholder community on an alternative plan through a transparent and public

[1] Abernethy, A., Abrahams, E., Barker, A., et al. Turning the Tide Against Cancer Through Sustained Medical Innovation: The Pathway to Progress. *Clinical Cancer Research*. March 2014.

Personalized Medicine Coalition
personalizedmedicinecoalition.org
1710 Rhode Island Ave., NW, Suite 700
Washington, DC 20036
P: 202.589.1770
F: 202.589.1778

process.

Personalized medicine is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient. By combining the data from those tests with an individual's medical history, circumstances and values, health care providers can develop targeted treatment and prevention plans.

Personalized medicine is already having tremendous impact for patients, particularly those with serious and life-threatening diseases. In 2015, 28% of the U.S. Food and Drug Administration's novel new drug approvals were personalized medicines, and 35% of novel new cancer therapeutics were personalized medicines.[2]

These advances are enabling more accurate diagnoses, better prediction of individual susceptibility to disease based on genetic or molecular factors, improved detection of disease at early stages, greater use of targeted treatments, and, more broadly, greater efficiency and effectiveness in health care delivery. Personalized medicine has become the standard of care in some diseases. For example, most Americans with breast or lung cancer benefit from targeted treatments. We expect future advancements to improve treatments in other disease states, as they have for these diseases and countless others, including cystic fibrosis.

Several specific, core features of the Part B Drug Payment Model, however — including the national scope of the proposed program, its accelerated timeline, its having been developed without input from affected stakeholders, the extent to which targeted therapies would face some of the steepest payment cuts in Phase I, and its reliance on static, one-size-fits-all measures of value in Phase II — are especially likely to negatively impact personalized medicine.

As the Administration reflects on the Medicare Part B Drug Payment Model, for the following reasons we respectfully urge you to withdraw the proposed rule and engage the stakeholder community on an alternative plan through a transparent and public process:

- The payment changes for Part B medicines outlined in Phase I of the proposal would result in some of the most innovative medicines — including advanced, personalized medicines targeted to smaller patient populations — facing the deepest payment cuts. Changing Part B reimbursement rates from the current structure, which sets rates at 6%, 4.3% after sequestration, of the drug's average sales price, to new rates that are calculated by adding a flat fee of $16.80 and 2.5% of the drug's average sales price, for example, could burden many of the most innovative medicines with the deepest payment cuts. As a result, the proposal will systematically disadvantage innovative treatments, including personalized medicines, and could deny access to a specific individual's "moonshot."

- CMS intends to begin Phase II immediately following the completion of Phase I, without the evaluative phase Congress envisioned when it established the Center for Medicare and Medicaid Innovation, and proposes unclear new payment standards based on complex, subjective standards of "clinical similarity" and cost effectiveness. Given the lack of clarity regarding Phase II, we are concerned that implementing any of the Phase II provisions without further study may hinder the adoption of personalized medicine through a failure to recognize important differences in patient subgroups and their responsiveness to particular treatments.

- Implementation of payment policies that institute one-size-fits-all mechanisms for assessing value and determining coverage run counterintuitive to the individualized nature of the advances we are witnessing in personalized medicine, and may reverse advances for patients. Further, this type of standard fails to recognize the way value emerges over time, as real-world evidence becomes available and is integrated

[2] Personalized Medicine Coalition, *2015 Progress Report: Personalized Medicine at FDA*. January 2016. Available at: http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/2015_Progress_Report_PM_at_FDA1.pdf.

into clinical practice and FDA adds additional information to therapeutics' labels. The standard therefore undervalues newer medicines, particularly the many precision medicines and other innovative therapies that are approved via accelerated review pathways.

- In Phase II, CMS is proposing to implement these sweeping changes to Medicare payment policy in 50% of the United States based on a 30-day, informal notice and comment period. The difficulty in appropriately applying clinical and cost effectiveness standards to personalized medicine, combined with the lack of consistency and clarity in policies proposed by CMS, dramatically increases the level of uncertainty for those considering whether to invest in high-risk research projects. This uncertainty threatens to discourage the development of breakthrough medicines that could drastically improve the lives of patients (such as those associated with the PMI and the Cancer Moonshot).

- Across both phases of the proposed rule, PMC is concerned that CMS has not sufficiently considered the unintended consequences and implications of the proposed provisions. For example, although CMS describes the proposed rule as a "demonstration project," its scope is national and its development lacked public input. Thus, this proposed rule might go beyond what Congress intended when it granted CMS the authority to create the Center for Medicare and Medicaid Innovation.

We are at a pivotal juncture in the personalized medicine community. While continuing scientific advances have the potential to transform health care for patients across the nation, the evolving policy landscape runs the risk of stalling progress. In oncology especially, the rapid pace of molecular and genomic innovation holds tremendous hope for the future.

That future has never been more promising. Today, 42% of medicines in the biopharmaceutical industry's pipeline have the potential to be personalized medicines, meaning that we will know, with more assurance than ever, that a drug will work for an individual. This progress is even more astounding in cancer, where 73% of oncology medicines have the potential to be personalized medicines.[3] Recent advances in cancer immunotherapy, for example, are already offering groundbreaking personalized medicine options for many cancer patients and are spurring a new era of cancer research that will have implications for other diseases.

To realize the full potential of emerging science for patients and the health system, we believe it is essential to ensure the entire health care regulatory framework — from FDA requirements to Medicare payment policy — is aligned with the principles of personalized medicine. We are deeply concerned that key elements of the Medicare Part B Drug Payment Model represent a step backward in this regard, and urge you to withdraw the proposed rule and engage the stakeholder community on an alternative plan through a transparent and public process.

Sincerely yours,

Edward Abrahams

Edward Abrahams
President

[3] Tufts Center for the Study of Drug Development, "Personalized Medicine Gains Traction but Still Faces Multiple Challenges," *Impact Report*. May/June 2015, Volume 17, Number 3.