PERSONALIZED MEDICINE COALITION

October 15, 2021

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
Mailstop C4-26-05
7500 Security Blvd.
Baltimore, MD 21244-1850

Sent electronically

Re: Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of "Reasonable and Necessary" (CMS-3372-P2)

Dear Administrator Brooks-LaSure:

The Personalized Medicine Coalition (PMC) has appreciated the Centers for Medicare & Medicaid Services (CMS)' efforts to expedite access to medical products designated as breakthrough devices by the U.S. Food and Drug Administration (FDA) through a new Medicare Coverage of Innovative Technology (MCIT) pathway. PMC submitted previous comments[i,ii] on the scope of the pathway and the proposed standards detailed for making "reasonable and necessary" (R&N) determinations under Section 1862(a)(1)(A) of the *Social Security Act* for items and services furnished under Medicare Parts A and B. We appreciate CMS' request for additional comments as it considers repealing the final rule.[iii]

PMC continues to strongly support implementation of the MCIT pathway as outlined in the final rule based on its potential to help expedite patient access to future breakthrough devices. As such, we are disappointed that CMS' proposal to repeal the final rule would cancel implementation of this new pathway for breakthrough devices that may include diagnostic and screening tests underpinning personalized medicine, as well as other new technologies supporting personalized medicine. PMC is pleased, however, that the proposal to repeal the final rule would resolve for now our significant concerns with the criteria that had been included in the final rule for defining R&N. In the following comments, we reiterate that CMS should address these concerns before proceeding in the future with any new attempt at regulatory implementation of the definition for "reasonable and necessary" to ensure that any such rule does not unintentionally limit patient access to personalized medicine by excluding from receiving Medicare coverage items and services that can play a role in delivering personalized health care.

Personalized Medicine Coalition
personalizedmedicinecoalition.org

1710 Rhode Island Ave., NW, Suite 700
Washington, DC 20036

P: 202.589.1770
F: 202.589.1778

PMC, which represents innovators, scientists, patients, providers, and payers, promotes the understanding and adoption of personalized medicine concepts, services, and products for the benefit of patients and the health care system.

We define personalized medicine as 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 helping to shift the patient and provider experiences away from trial-and-error treatments of late-stage diseases in favor of more streamlined approaches to disease prevention and treatment, which will lead to improved patient outcomes, a reduction in unnecessary treatment costs, and better patient and provider satisfaction.

PMC's members are leading the way in developing and delivering personalized medicine for patients. Our comments on the proposed repeal of the MCIT/R&N final rule are intended to highlight how a pathway for coverage of breakthrough devices can better support this growing field. To do so, we provide suggestions on the scope of the MCIT pathway and refinements to the definition of "reasonable and necessary" that we urge you to consider before codifying this or future definitions.

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 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 proposed rule to repeal the voluntary MCIT pathway and definition for "reasonable and necessary."

Section I: Establishing the MCIT pathway for breakthrough devices

PMC believes implementing the MCIT pathway would facilitate patients' access to new technologies underpinning personalized medicine, including diagnostic and screening tests and digital health technologies that leverage artificial intelligence. We continue to strongly support the portion of the final rule that would establish a voluntary MCIT pathway extending coverage for breakthrough devices immediately upon the date of FDA market authorization for up to four years. For devices addressing areas of unmet medical need, the newness of the device, and in some cases small patient population sizes, can create challenges to gathering the clinical evidence needed for coverage and reimbursement determinations, subsequently increasing the time between introduction to the market and patient access. The MCIT pathway would mitigate the upfront evidence burden required to meet the current coverage standard and prioritize patients' unmet medical needs.

The current MCIT pathway would create an opportunity for timely Medicare coverage of breakthrough devices, which would include in vitro diagnostic (IVD) test kits as well as laboratory-developed tests (LDTs) in the event a laboratory voluntarily seeks breakthrough designation and clearance or approval

from FDA. PMC appreciates that CMS had recognized diagnostic tests would be eligible for this pathway and supports their continued inclusion in the MCIT pathway and any future proposed pathways. Under the MCIT or any future proposed pathway for coverage, however, PMC believes CMS should avoid including provisions that can be construed as a requirement to seek FDA approval as a necessary precondition for Medicare coverage of all tests either within or outside of the new coverage pathway. In addition, the current MCIT pathway should not be expanded to apply to drugs or biologicals.

PMC supported CMS' earlier decision that breakthrough devices should be eligible for coverage if they fall within a Medicare benefit category and are not otherwise excluded from coverage by statute. PMC believes clinical tests should be eligible for coverage, whether they be used for diagnostic or screening purposes, as long as they fall within a benefit category. When making benefit category determinations for items and services eligible for coverage under MCIT or an alternative, we encourage CMS to continue to include all benefit categories under Medicare Parts A and B for consideration, as we continue to believe this is in line with the goals of CMS to facilitate patient access to new technologies.

While we are disappointed in CMS' proposal to repeal the final rule, we appreciate the agency's willingness to consider alternatives to facilitate patient access to new technologies either through new or existing coverage pathways. Should CMS finalize its proposal to repeal the MCIT pathway, we would welcome the opportunity to submit comments on future proposals that may improve the predictability of coverage for new technologies. Future CMS policymaking should consider how to improve patient access to not only breakthrough devices, but also existing diagnostic and screening tests, including LDTs.

Section II: Defining "reasonable and necessary"

PMC continues to have concerns with codifying the definition for "reasonable and necessary" as written in the MCIT/R&N final rule, which could adversely impact patient access to personalized medicine where the path to coverage may already be smooth and well-understood. To the extent that the current proposed rule would repeal the final rule that defined "reasonable and necessary" as including a "safe and effective" standard, PMC supports such repeal.

Currently, drugs and biologicals are generally covered by Medicare for all of their medically accepted uses, including all FDA-approved indications and any off-label uses that are supported by compendia or peer-reviewed literature. Medicare coverage policy, as applied to drugs and biologicals, has been effective in ensuring Medicare beneficiaries have access to innovative drugs and biologicals without creating undue burden on the Medicare program itself or manufacturers of drugs and biologicals. Unlike drugs and biologicals, devices face greater coverage scrutiny and do not have the same statutory protections. Therefore, we reiterate that CMS should exempt drugs and biologicals from the criteria in any future rule defining R&N.

CMS indicated in its final rule that the definition for "reasonable and necessary" would extend beyond breakthrough devices to include all items and services covered under Medicare Parts A and B,

including but not limited to drugs, devices, and biologics. We have outstanding concerns with the broad application of this definition. We appreciate CMS' request for comments on the proposed repeal of this definition, and we ask the agency to continue to work with stakeholders to refine the definition before applying regulatory standards to "reasonable and necessary." Before proceeding with any regulatory implementation in the future, we also ask the agency to give special consideration to the impact of language in the final rule on LDTs. PMC's additional suggestions on refining the criteria included in the final rule are discussed below.

Interpreting "safe and effective" standards

Some personalized medicine tests are LDTs. Since the "safe and effective" standard is the same standard used by FDA to evaluate drugs and devices for marketing approval, PMC remains concerned, as we explained in our previous comments, that retaining this criterion for defining "reasonable and necessary" could be interpreted as meaning that LDTs would need to be approved by FDA as a condition for coverage by Medicare. Since any such interpretation would be inappropriate and likely would result in significant loss of access to medically necessary laboratory services for Medicare beneficiaries, this criterion should not be applied to LDTs in a manner that would require FDA clearance or approval as a condition of Medicare coverage.

While the "safe and effective" standard is appropriate for products distributed in interstate commerce that are designed for and intended to produce a direct therapeutic impact, LDTs are services, developed and performed by the same laboratory entity, that do not create a direct therapeutic impact, but rather provide information to inform treatment decisions. LDTs are therefore qualitatively different from the tangible goods with direct therapeutic impact that FDA may regulate as "devices" and to which the standard "safe and effective" appropriately applies. When CMS' predecessor agency set forth its interpretation of "reasonable and necessary" in the context of making National Coverage Determinations (NCDs), it recognized that "[n]ot all of the criteria are necessarily pertinent to every coverage issue and each criterion is not necessarily given equal consideration in reaching a final decision."[iv] (Indeed, almost none of that proposed rule's discussion of what is meant by "safe and effective" is relevant to LDTs.)

In the MCIT/R&N final rule, CMS acknowledges concerns about items and services not regulated by FDA but does not adequately address the impact that finalizing this language would have on LDTs and other such items and services. The agency justified retaining this criterion based on its historical inclusion in the *Program Integrity Manual (PIM)*. While we recognize this criterion has been included in the *PIM* for some time, we support the agency's proposal to repeal the final rule to the extent that doing so would repeal regulatory codification of this criterion. If CMS should decide to proceed with such codification in the future, it is important for CMS to acknowledge and clarify in the regulatory text that this first criterion will not be interpreted now or in the future to require LDTs to have FDA approval or clearance before Medicare can cover them. Codified regulations carry more weight than sub-regulatory guidance such as the *PIM*, and it is important that neither this nor any future regulation be left open to this interpretation.

Determining medical "appropriateness"

To determine the "appropriateness" of an item or service, the final rule enumerated a number of sub-elements to the definition of "reasonable and necessary," including whether an item or service is "at least as beneficial as an existing and available medically appropriate alternative" for Medicare patients. What is beneficial and appropriate to one patient, however, may not be to another patient with the same condition or diagnosis. We continue to believe this language, as written, could limit patient access to personalized medicine where the use of an item or service (test, treatment, or other intervention) would otherwise be based on a patient's unique biology, values, and circumstances. It would be particularly problematic if CMS used its discretion to interpret "beneficial" as including cost and cost-effectiveness analyses. As we have commented previously, PMC continues to recommend removing this sub-element from this or any future definition.

Considering commercial coverage policies

Removing the consideration of commercial coverage policies from the MCIT/R&N final rule would not address our concerns with the definition for "reasonable and necessary" outlined in the final rule. In fact, PMC appreciates CMS' receptiveness to considering additional avenues for determining the appropriateness of an item or service.

In our previous comments, we encouraged the agency to further engage stakeholders to refine its proposal because we were concerned the proposal to consider commercial health insurers' coverage policies, without additional details regarding how the agency would select and analyze those policies, could limit patient access to personalized medicine. We were also concerned such analyses could become de facto CMS policy if this pathway for determining appropriateness was developed. Commercial policies often use comparative effectiveness research and cost-effectiveness research to establish coverage standards, whereas CMS does not. We thank CMS for acknowledging these concerns in the final rule and for proposing a process to refine its methodology for considering commercial insurers' policies by issuing draft sub-regulatory guidance that would allow for additional public comment. Providing a more detailed outline of how the agency intends to select and evaluate commercial coverage policies would ensure there is transparency in how commercial coverage policies are selected and evaluated and that this policy will be beneficial to patients.

PMC has encouraged CMS to create an "additive" pathway for coverage by considering commercial coverage policies in which the interests of serving a beneficiary's medical needs sets the bar for coverage. We have suggested that the existence of one positive commercial coverage policy should be sufficient for CMS to consider expanding coverage for an item or service, and when considering multiple commercial coverage policies, the most restrictive coverage policies should never be used. We also commented that a product currently covered by Medicare, either through CMS or Medicare Administrative Contractors (MACs), should not lose coverage once commercial coverage policies are considered. In the final rule, CMS proposed considering commercial coverage to the extent items or services are covered by a majority of commercial insurers when there is insufficient evidence to meet the other criteria in the definition for "reasonable and necessary." As the agency considers repealing the final rule and future rulemaking opportunities, PMC encourages the agency to continue to engage

stakeholders to ensure this or any future coverage pathway taking into account commercial coverage policies would be "additive." In addition, in the case of LDTs, CMS and MACs should be open to considering changes in existing policies in an expedited manner based on the presentation of commercial policies that provide greater access to tests used for diagnostic and screening purposes.

In addition, PMC appreciated that CMS responded to feedback and removed language from an earlier version of the final rule that would have excluded the consideration of commercial health insurers' coverage policies where "evidence supports the differences between Medicare beneficiaries and commercially insured individuals are clinically relevant." PMC was concerned this policy would result in denial of Medicare coverage for needed personalized medicine items or services. The commercially insured and Medicare populations are not the same or easily comparable. The Medicare population includes patients aged 65 and older as well as those with disabilities, whereas the commercially insured population is more diverse in terms of age, gender, and risk factors. Clinically relevant differences would therefore manifest in any comparison. PMC therefore encourages CMS to refrain from re-inserting this language in any future rule defining R&N or in any future draft sub-regulatory guidance. If, however, CMS chooses to include similar language in the future, the agency should be required to at least establish there is evidence indicating clinically relevant differences between Medicare beneficiaries and comparable commercially insured individuals, and the public should have the opportunity to comment on such analyses.

Conclusion

Thank you for considering PMC's comments on the agency's proposed rule to repeal the MCIT pathway for breakthrough devices and definition of "reasonable and necessary." PMC welcomes the opportunity to serve as a resource for you as you consider how to proceed with this and other policies that impact beneficiary access to personalized medicine tests and treatments. If you have any questions about the content of this letter, please contact me at 202-499-0986 and cbens@personalizedmedicinecoalition.org or David Davenport, PMC's Manager of Public Policy, at 804-291-8572 and ddavenport@personalizedmedicinecoalition.org.

Sincerely,

Cynthia A. Bens
Senior Vice President, Public Policy

[i] Personalized Medicine Coalition. "Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of 'Reasonable and Necessary' (CMS-3372-P)." November 2, 2020. http://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC_on_CMS_MCIT_Pathway_11.02.20.pdf.

[ii] Personalized Medicine Coalition. "Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of 'Reasonable and Necessary;' Delay of Effective Date; Public Comment Period (CMS–3372–IFC)." April 16, 2021. https://www.personalizedmedicinecoalition.org/Userfiles/PMC-Corporate/file/PMC-Comments_MCIT-Interim-Final-Rule_2021.04.16.pdf

[iii] *Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of "Reasonable and Necessary" [CMS-3372-P2]*. 42 CFR 405. Vol. 86, No. 176. September 15, 2021. https://www.federalregister.gov/d/2021-20016.

[iv] *Medicare Program; Criteria and Procedures for Making Medical Services Coverage Decisions That Relate to Health Care Technology*. 54 Fed. Reg. 4302, 4307. January 30, 1989.