PERSONALIZED MEDICINE COALITION

April 16, 2021

The Honorable Liz Richter
Acting 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''; Delay of Effective Date; Public Comment Period (CMS–3372–IFC)

Dear Acting Administrator Richter:

The Personalized Medicine Coalition (PMC) appreciates 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. In November 2020, PMC submitted comments[i] to the proposed rule[ii] on the scope of the pathway and the proposed standards detailed for making "reasonable and necessary" determinations under Section 1862(a)(1)(A) of the *Social Security Act* for items and services furnished under Part A and Part B. We appreciate CMS' interim final rule[iii] requesting additional comments before implementation of the final rule.

While we 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, which may include diagnostic and screening tests underpinning personalized medicine, we continue to have significant concerns with the criteria for defining "reasonable and necessary." We encourage CMS to address these concerns before proceeding with implementation of the definition for "reasonable and necessary" to ensure that the final 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.

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.

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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 MCIT final rule are intended to highlight how it can better support this growing field. To do so, we provide suggestions on the scope of this pathway and refinements to the definition of "reasonable and necessary" that we urge you to consider before implementing the final rule.

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 final rule to establish a voluntary MCIT pathway and to define and codify in statute "reasonable and necessary" standards.

Section I: Establishing the MCIT pathway for breakthrough devices

PMC strongly supports the portion of the final rule that outlines 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. We applaud CMS' prioritization of patients' unmet medical needs and willingness to facilitate patient access to breakthrough devices through this pathway.

The 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 recognizes diagnostic tests would be eligible for this pathway and supports their continued inclusion in the MCIT pathway, as written in the final rule. If CMS revises the final rule before implementation, however, PMC holds that the MCIT pathway should not be changed to include 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 MCIT pathway, nor should the pathway be expanded to apply to drugs or biologicals.

Implementing MCIT

In the interim final rule, CMS specifically requested comments on resolving operational issues, such as benefit category determinations, coding, and payment levels, that may arise when implementing the MCIT pathway.

PMC supports CMS' 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 eligible MCIT items and services, we encourage CMS to continue to include all benefit categories under Part A and Part B for consideration, as we continue to believe this is in line with the goals of the MCIT pathway.

Finally, since all coding, payment, and bundling rules would continue to apply under MCIT and since CMS routinely addresses coding and payment issues when operationalizing coverage for items and services, PMC believes these will not present significant additional barriers or delays to patient access when implementing Medicare coverage for items and services under the MCIT pathway.

Section II: Defining "reasonable and necessary"

PMC recognizes that CMS has moved to codify criteria for "reasonable and necessary" determinations in response to Executive Order 13890 to clarify the application of standards and streamline coverage for innovative products. We also recognize that many of these criteria are included in CMS' *Program Integrity Manual (PIM)*. However, as written in the final rule, the criteria in the definition for "reasonable and necessary" could adversely impact patient access to personalized medicine where the path to coverage may already be smooth and well-understood.

Currently, drugs and biologicals are generally covered by Medicare for all of their medically accepted uses, which includes all FDA-approved indications and any off-label use that is 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, in our proposed comments, we asked CMS to exempt drugs and biologicals from the proposed criteria. We also asked the agency to give special consideration to the impact of language in the final rule on LDTs.

CMS indicates in the final rule that this definition would extend beyond breakthrough devices to include all items and services covered under Medicare Part A and Part B, including but not limited to drugs, devices and biologics. We reiterate our concerns with the broad application of this definition and ask the agency to continue to work with stakeholders to refine the definition before applying regulatory standards to "reasonable and necessary." 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 comments on the proposed rule, 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 final rule, CMS acknowledges concerns about items and services not regulated by FDA but does not adequately address the impact finalizing this language would have on such items and services, like LDTs. The agency justifies retaining this criterion based on its historical inclusion in the *PIM*. While we recognize this criterion has been included in the *PIM* for some time, 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 the regulation not be left open to this interpretation.

Determining medical "appropriateness"

To determine the "appropriateness" of an item or service, the final rule enumerates 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. In addition, it would be particularly problematic if CMS used its discretion to interpret "beneficial" as including cost and cost effectiveness analysis. As we commented on the proposed rule, PMC continues to recommend removing this sub-element from the definition. The remaining sub-elements of the proposed "reasonable and

necessary" definition should be sufficient to establish the "appropriateness" of an item or service for Medicare patients.

Considering commercial coverage policies

PMC appreciates CMS' receptiveness to considering additional avenues for determining the appropriateness of an item or service. In our comments on the proposed rule, 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 is 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 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 will ensure there is transparency in how commercial coverage policies are selected and evaluated and that this policy will be beneficial to patients.

In our comments on the proposed rule, PMC 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 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." PMC encourages the agency to consider how this pathway can be "additive" when drafting sub-regulatory guidance. In the case of laboratory tests, 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.

PMC also appreciates that CMS responded to feedback and removed language from the proposed rule that would exclude 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 this rule or in

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 final rule to establish the MCIT pathway for breakthrough devices and to codify criteria for making reasonable and necessary determinations. 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

cc: Norris Cochran, Acting Secretary, Department of Health and Human Services

[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] *Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of Reasonable and Necessary [CMS-3372-P]*. 42 CFR Part 405. Vol. 85, No. 170. September 1, 2020. https://www.federalregister.gov/documents/2020/09/01/2020-19289/medicare-program-medicare-coverage-of-innovative-technology-mcit-and-definition-of-reasonable-and

[iii] *Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of "Reasonable and Necessary"; Delay of Effective Date; Public Comment Period [CMS-3372-IFC]*. 42 CFR 405. Vol. 86, No. 50. March 17, 2021. https://www.federalregister.gov/d/2021-05490

[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.