PERSONALIZED MEDICINE COALITION

November 2, 2020

The Honorable Seema Verma
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-P)

Dear Administrator Verma:

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 its proposed Medicare Coverage of Innovative Technology (MCIT) pathway. PMC's comments on the recently released proposed rule for the MCIT pertain to the anticipated 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.[i]

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 that uses diagnostic tools to identify specific biological markers, often genetic, to help determine which medical treatments and procedures will be best for each patient. By combining this information with an individual's medical history, circumstances, and values, personalized medicine allows doctors and patients to develop targeted prevention and treatment plans. Personalized medicine is helping to shift the patient and provider experience 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.

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PMC's members are leading the way in developing and delivering personalized medicine for patients. Our comments on the MCIT proposed rule are intended to highlight how it can better support this emerging field. To do so, we provide suggestions on the scope of this pathway and refinements to the proposed definition of "reasonable and necessary" that we urge you to consider when issuing a 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 proposals to establish a voluntary MCIT pathway or to define and codify in statute "reasonable and necessary" standards.

Section I: Establishing the MCIT pathway for breakthrough devices

PMC strongly supports the proposed rule to 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 allow breakthrough device manufacturers to gather clinical evidence that can be later submitted to CMS. We applaud CMS' prioritization of patients' unmet medical needs and willingness to facilitate patient access to breakthrough devices.

The MCIT pathway would create an opportunity for timely Medicare coverage of breakthrough devices, which should 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 encourages CMS to develop this pathway. The MCIT pathway proposal, however, should not 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 proposed pathway apply to drugs or biologicals.

Finally, PMC supports CMS' position 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 also supports establishing clinical tests as eligible for coverage, whether they be used for diagnostic or screening purposes, as long as they fall within a benefit category. CMS suggests this broad approach for inclusion in the voluntary MCIT coverage pathway, and we believe it is in line with the goals of the proposed rule.

Section II: Defining "reasonable and necessary"

PMC acknowledges that CMS is proposing 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, the criteria in the proposed 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, we ask CMS to exempt drugs and biologicals from the proposed criteria. We also ask the agency to give special consideration to the impact of this proposal on LDTs.

Because CMS proposes that this definition extend beyond breakthrough devices, we ask the agency to continue engaging the public in refining it before codification. This will allow the agency time to work with stakeholders before applying regulatory standards to reasonable and necessary. PMC's suggestions on refining the proposed definition are highlighted 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 is concerned that 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."[ii] (Indeed, almost none of that proposed rule's discussion of what is meant by "safe and effective" is relevant to LDTs.)

While we recognize that 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 proposed 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 believe this language as written could therefore limit patient access to personalized medicine where the use of an item or service (i.e., test, treatment, 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 analysis. Since 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, PMC recommends removing this sub-element.

PMC appreciates CMS' receptiveness to considering additional avenues for determining the appropriateness of an item or service, but we are 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 encourage the agency to refine its proposed rule.

PMC also encourages CMS to engage stakeholders to understand how to create an "additive" pathway for coverage in which the interests of serving a beneficiary's medical needs sets the bar for coverage. For example, the existence of one positive commercial coverage policy should be sufficient for CMS to consider expanding coverage for an item or service. When considering multiple commercial coverage policies, CMS should avoid selecting the most restrictive coverage policies. Furthermore, 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 fact, 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.

In its proposal to consider commercial health insurers' coverage policies, CMS excludes instances where "evidence supports the differences between Medicare beneficiaries and commercially insured individuals are clinically relevant." PMC is concerned this would result in denial of Medicare coverage for needed personalized medicine items or services, and we suggest that CMS remove this exception. 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. If CMS chooses to include this language, the agency should be required to at least establish there is evidence indicating clinically relevant differences between Medicare beneficiaries and comparable commercially insured individuals. Additionally, the public should have the opportunity to comment on such analyses.

Finally, PMC is 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. Before issuing a final rule, CMS should provide a more detailed outline of how it intends to select and evaluate commercial coverage policies. This pathway will only be beneficial to patients if there is transparency in how commercial coverage policies are selected and evaluated.

Conclusion

Thank you for considering PMC's comments on the agency's proposals 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 continue to shape 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] *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

[ii] *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.