

VIA ELECTRONIC SUBMISSION

Chiquita Brooks La-Sure
Administrator
Centers for Medicare and Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

October 15, 2021

RE: Medicare Program; Medicare Coverage of Innovative Technology (MCIT) and Definition of “Reasonable and Necessary” (CMS-3372-P2)

Dear Administrator La-Sure,

The Robert J. Margolis, MD Center for Health Policy at Duke University (Duke-Margolis) appreciates the opportunity to comment on the Centers for Medicare and Medicaid Services (CMS) proposed rule to repeal the Medicare Coverage of Innovative Technology (MCIT) pathway and definition of “Reasonable and Necessary” final rule.

The Duke-Margolis Center analyzes evidence across the spectrum of health policy and supports the triple aim of better care, better health, and lower cost. A core mission is to increase the value of biomedical innovation to patients. Our experts are engaged in policy research and development efforts to improve the processes and infrastructure needed at CMS to ensure efficient access to new and innovative technologies by addressing challenges related to coding, coverage, and payment.

Our comments are informed by an independent analysis of the proposal and convenings with a broad set of stakeholders. Duke-Margolis supports the important goal to provide Medicare patients timely and appropriate access to breakthrough devices that receive Federal Drug Administration (FDA) approval. We agree with CMS that there are significant challenges in the current design of the MCIT pathway that may prevent it succeeding in its intended goals. We also commend CMS for considering additional rulemaking to accommodate coverage processes intended to provide timely access to breakthrough devices. We believe that the success of an expedited coverage pathway depends on the extent to which CMS can guide evidence development to meet a “reasonable and necessary” threshold for Medicare coverage. Our comments describe steps CMS can take to provide Medicare patients timelier access to innovative medical devices through evidence-based policies through the following key recommendations:

- Revise the final MCIT rule *or* propose new expedited coverage process to provide timely and appropriate access to breakthrough devices following FDA approval through evidence-based coverage policies informed by sustained stakeholder engagement.
- Increase resources and capacity to support coverage, coding, and payment activities in order to provide Medicare patients timely access to biomedical innovation.
- Undertake a separate rulemaking process to determine if codifying a definition of “reasonable and necessary” is the best way to improve internal CMS processes while supporting evidence development and promoting medical innovation.

Our comments below detail these recommendations:

Part I. Goals of an Expedited Pathway to Coverage and Access

The FDA’s Breakthrough Devices Program is an expedited regulatory pathway for devices that are novel, breakthrough, and provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions.ⁱ The intent of this pathway is to provide timely commercial access to novel devices by speeding up their development, assessment, and regulatory review. Because of the expedited nature of the pathway, at the time of device approval there may be limited evidence on the device’s effects on health outcomes, its long-term adverse events, and the treatment durability. Further, the available evidence may not be relevant to Medicare beneficiaries. Even though breakthrough devices may provide Medicare patients with much-needed treatments or diagnostics, these evidence gaps are a concern for CMS. Medicare coverage depends on the determination that an item or a service is “reasonable and necessary” to improve Medicare patients’ health outcomes. Combined with siloed processes for clinical evidence development, regulatory review and approval, coverage determinations, coding, and payment, there is a gap between the time of FDA approval for a breakthrough device and Medicare reimbursement, which impacts access for Medicare patients.

There are some mechanisms that facilitate the adoption of breakthrough devices in the Medicare system that build off of existing coverage and payment processes, however they still result in access gaps after a breakthrough device receives FDA approval. First, Medicare has used the longstanding policy of Coverage with Evidence Development (CED) to provide early access to novel technologies, including breakthrough devices, when there is insufficient evidence to substantiate “reasonable and necessary” determination for Medicare coverage.^{ii,iii,iv} While CED ensures Medicare access, it necessitates a national coverage determination (NCD) process and requires the availability of a data collection infrastructure. These may be deterrents for manufacturers who do not want to risk a negative coverage determination following a national coverage assessment, and for manufacturers with limited resources who may not be able to establish a data collection infrastructure to support CED. Second, recent changes have streamlined how breakthrough devices are incorporated into the Medicare fee schedules, such as the modified application criteria for new technology add-on payments (NTAP) and pass-through payment status. However, these payment processes are not necessarily aligned with coding processes that define units of service across the different Medicare fee schedules. Further, many coding and payment processes require FDA approval as an eligibility requirement. Despite mechanisms in which FDA approved breakthrough devices can be adopted in the Medicare system, there are notable gaps in access immediately following FDA approval.

The intent of the MCIT pathway was to address these access gaps for breakthrough devices that receive FDA approval. It was proposed to address the concerns that the statutory processes to determine Medicare coverage, application requirements, and review cycles for coding and payment impact the timeliness in which patients may access breakthrough devices. However, the current MCIT pathway, as written, does not define any procedure to align coding and payment processes to facilitate claims processing at the time of FDA approval. Thus, the MCIT pathway does not guarantee immediate access to breakthrough devices, even in the presence of Medicare coverage. Nor does the pathway guarantee that Medicare beneficiaries will have long-term access to breakthrough technologies, as there is no requirement to generate evidence to substantiate a “reasonable and necessary” determination for long-

term Medicare coverage. As such, the MCIT pathway, as written, does not improve access for breakthrough devices. Duke-Margolis has detailed these challenges with the MCIT pathway design in previous public comment opportunities,^{v,vi} and we note that in this proposed rule, CMS has raised the same concerns.

In order to address CMS's stated concerns of the MCIT pathway, the goal of providing timely access for breakthrough devices should be balanced with equal efforts to inform and develop evidence-based coverage policies and, as needed, evidence generation to meet "reasonable and necessary" Medicare coverage criteria. We believe that an expedited pathway should include the following guiding principles:

- Earlier engagement between manufacturers, CMS, FDA, and other stakeholders prior to FDA approval to identify evidence gaps and determine how to address them.
- Post-market evidence generation to support coverage should not duplicate FDA post-marketing data collection requirements to minimize provider burden.
- Predictable and transparent operating procedures with opportunities for public comment and stakeholder engagement.

Earlier engagement between manufacturers, CMS, FDA, and other stakeholders prior to FDA approval to identify evidence gaps and determine how to address them. Early engagement between manufacturers, CMS, and FDA will allow CMS to identify evidence gaps relevant to Medicare beneficiaries and provide guidance on relevant pre-market evidence development to help assure timely and predictable coverage. In cases where an entirely new class of products has significant uncertainties around coverage and evidence expectations, a pre-approval process that includes CMS engagement with external experts, including the Medicare Evidence Development & Coverage Advisory Committee (MEDCAC), the National Institutes of Health (NIH), the Patient Centered Outcomes Research Institute (PCORI), and Agency for Healthcare Research and Quality (AHRQ), and (with manufacturer agreement) relevant health care providers and private organizations will help ensure that key evidence needs for Medicare beneficiaries can be addressed before or soon after approval. In cases where additional evidence generation is warranted, earlier engagement will allow manufacturers and other stakeholders to develop a data collection infrastructure and minimize operational delays following FDA approval.

Post-market evidence generation to support coverage should not duplicate FDA post-marketing data collection requirements to minimize provider burden. Duke-Margolis appreciates the growing burden of data collection at the provider level. Providers currently face many data collection requirements to satisfy quality and performance metrics under other quality and payment frameworks. The prospect of collecting additional data to support permanent coverage for a breakthrough device may prove challenging and potentially discouraging to providers. Early engagement between manufacturers and both agencies is an opportunity to leverage the same data collection infrastructure to satisfy evidence generation requirements while minimizing provider burden.

Predictable and transparent operating procedures with opportunities for public comment and stakeholder engagement. Finally, any new coverage process should be modeled after existing coverage processes in that they are predictable, reliable, and include opportunities for public input. A predictable and reliable process will be beneficial in establishing transparency, as well as time and resource expectations for both manufacturers and CMS.

We recommend CMS revise the MCIT rule or open a new rulemaking process to define an expedited coverage pathway that includes these guiding principles. Should CMS opt to open a new rulemaking process, we recommend they provide a clear timeline of when to expect a new proposed rule.

Part II. Operational Framework to Support an Expedited Coverage Pathway

In order to fulfill the intended goals of an expedited pathway, it will be beneficial for stakeholders to operate under a framework, or standard operating procedures (SOP), that will define the level of engagement between stakeholders, as well as time and resource expectations. As CMS gains experience in the MCIT pathway, the SOP can establish accountability and identify ways to improve performance. Our recommended framework (see Appendix A) for operationalizing an expedited coverage pathway includes the following elements:

- Defined time window in which a manufacturer applies for expedited coverage. We recommend that manufacturers notify CMS after receiving the breakthrough designation from the FDA and no later than when manufacturers submit pivotal investigational device exemption (IDE) trial results to the FDA for market approval. To provide clarity about the expected content of a successful evidence submission, CMS should provide an opportunity for consultation and guidance much earlier in the development process. CMS can expand existing interactions with manufacturers that request Medicare coverage for IDE studies. Manufacturers' applications can include a summary of the existing evidence base and plans for ongoing clinical data development.
- Defined, predictable timeline for coverage assessments, including whether expedited coverage is appropriate, and if additional evidence generation is necessary. Following manufacturers' application, CMS will have a defined timeline for activities to reach a coverage determination, which include:
 - a Medicare benefit category determination,
 - a determination on whether expedited coverage is needed and appropriate (including guidance to the manufacturer about coverage if CMS determines a special process is not needed),
 - a preliminary evidence assessment to identify evidence gaps,
 - through the new Technology Coding and Pricing Group, guidance on navigating coding and payment processes to facilitate claims processing immediately following FDA approval.

This initial evidence analysis will enable CMS to operationalize and implement coverage quickly following FDA approval. It will also allow CMS to determine whether additional evidence development will be necessary, and, if so, the most efficient way to develop such evidence. CMS could require CED if additional evidence development is necessary to assess health outcomes in Medicare beneficiaries. Throughout this process, CMS would engage with the manufacturer, the FDA, and potentially other entities, including NIH, MEDCAC, PCORI, AHRQ, clinicians, and other stakeholders to inform the evidence assessments and identify evidence needs for Medicare beneficiaries. Consistent with Medicare past coverage determinations,

CMS could cover breakthrough devices in a way that includes follow on and iterative devices. This will expedite the approval to coverage process while still incentivizing innovation in the device class.

- Public comment period. To align with existing coverage processes, CMS will release a proposed coverage determination immediately following FDA approval with a 30-day public comment period. The proposed coverage determination will include a review of the relevant evidence underlying the coverage decision. In cases that require CED, CMS will include guidance on how to develop relevant evidence and the conditions to end the evidence generation requirement. Following the public comment period, CMS will have a defined timeline to finalize the coverage determination and issue implementation instructions.
- CED process for cases that need additional RWE collection. For the subset of breakthrough products that require further RWE generation, manufacturers will be required to share regular interim reports with CMS on how evidence development is progressing. These reports will help CMS plan for long-term coverage, including any outstanding evidence generation needs, and work with the manufacturer to address any challenges in evidence development. If these requirements are not met, CMS would have the authority to terminate coverage under the expedited program. CMS or the MACs could then determine coverage through the existing coverage processes, such as NCD, LCD, or claim by claim adjudication.

This proposed framework addresses many of CMS's stated concerns with the current MCIT rule (see Appendix B). The framework allows CMS to make quick—yet thorough—coverage decisions based on the safety implications for Medicare beneficiaries while minimizing the gap between FDA approval and coverage. CMS can retain its authority to terminate coverage for devices that may have negative safety implications. The requirement that manufacturers collect necessary post-market evidence in the event of a CED ensures CMS will not cover devices without data demonstrating benefits for Medicare beneficiaries.

Early engagement between all stakeholders helps address concerns around coding and payment processes, as well as resourcing evidence development. Manufacturers and CMS can work together before approval to determine coding and payment to ensure a smooth transition to Medicare reimbursement after approval, furthering the ultimate goal of minimizing the gap between approval and coverage. This framework can also help mitigate CMS's concern that the MCIT pathway would disincentivize innovation of non-breakthrough devices by covering breakthrough devices in a way that includes follow on and iterative devices.

The design of the MCIT pathway can be amended to incorporate this recommended framework for expedited coverage. However, should CMS choose to repeal MCIT, this type of framework can be the basis of a new expedited coverage pathway. Further, elements of this framework can also be incorporated into existing coverage processes. Early engagement with CMS and other key stakeholders can help manufacturers inform their clinical trials and evidence development plan. Early engagement also does not prevent the public from voicing their opinions or concerns with coverage for a medical product; it merely minimizes the time between approval, comment periods, and final decision-making.

We recommend CMS revise the final MCIT rule or propose new expedited coverage processes to provide

timely and appropriate access to breakthrough devices following FDA approval through evidence-based coverage policies informed by sustained stakeholder engagement.

Part III. Increasing Resources to Support Expedited Coverage Activities

Any new coverage process will create new requirements for CMS's time, capacity, and expertise, but especially an expedited process that relies on early and sustained stakeholder engagement, such as the framework recommended above. These recommendations will have the greatest impact if accompanied by steps to ensure adequate resources and capacity for CMS to implement and operationalize them.

Our framework aims to provide more clarity around the process—and thus the resources—that will be needed to support predictable, timely, and productive interactions between CMS, manufacturers, and other stakeholders to meet program expectations. We believe these resource requirements to support expedited coverage will be modest relative to their impact on innovation and Medicare patients' health outcomes. CMS resources in areas related to new technology assessment and access have declined over the past decade, even as breakthrough innovation has expanded and the resources to support it at FDA have grown rapidly.^{vii} This mismatch will be exacerbated by the growing number of devices receiving breakthrough designation, and expedited coverage is likely to further increase manufacturer interest in developing breakthrough therapies. Ensuring that CMS has adequate resources and capacity to undertake evidence assessments and other coverage activities will be crucial to the success of an expedited pathway. **We recommend CMS increase resources and capacity to support coverage, coding, and payment activities in order to provide Medicare patients timely access to biomedical innovation.**

In the short-term, CMS could rely on existing interactions to coordinate with FDA and manufacturers, since the operational framework is based on existing points of contact between CMS and manufacturers that can be used to fulfill expectations for MCIT. CMS may also leverage existing collaboration with the FDA for subspecialty expertise to inform evidence development.

However, short-term considerations may not be sufficient for long-term sustained success for this pathway. As the pipeline of breakthrough therapies grows, existing resources and mechanisms may not be sufficient. CMS will need medical officers with subject matter expertise, and a greater number of personnel to manage operational elements of MCIT and downstream coverage assessments. CMS should consider further steps within its administrative authority to make such personnel shifts to support all MCIT activities. We also recommend CMS consider different mechanisms to increase resources to support coverage activities that include dedicated appropriations or user fees. To ensure appropriate use of these resources, there could be a requirement for an assessment of the impact on key goals, such as the timely occurrence of meetings and development of clear guidance to manufacturers on coverage-related issues, and time to coverage.

Part IV. Duke-Margolis Comments on Codifying the Definition of "Reasonable and Necessary"

Duke-Margolis agrees with the CMS assessment that expanding the definition of "reasonable and necessary" to include consideration of commercial insurer coverage would present multiple challenges

to the implementation process, limit flexibility, and potentially limit CMS's ability to offer equitable access to new technologies.

There are a number of benefits to having a codified definition of "reasonable and necessary." With a codified definition, CMS could then define what is considered "appropriate" for offering coverage in different disease areas. This could help manufacturers design their clinical trials around the types of outcomes that would matter to CMS, given the disease area or type of technology. A codified definition of "reasonable and necessary" would also direct CMS to provide evidence development guidance for applications for coverage and increase CMS's role in providing evidence development guidance for new medical products. However, codifying a definition of "reasonable and necessary," current or amended, would affect CMS processes outside of coverage for devices and thus merits its own separate rulemaking process and consideration. **We recommend that CMS have a separate rulemaking process to determine if codifying a definition of "reasonable and necessary" is the best way to improve internal CMS processes while supporting evidence development and promoting medical innovation.**

Conclusion

An expedited coverage pathway has the potential to provide broad and timely access to breakthrough devices, but the current MCIT rule limits CMS's ability to ensure the safety of Medicare beneficiaries and lacks the necessary enforcement mechanisms to ensure proper data collection. Our recommendations describe opportunities in which CMS can succeed in the intended goals of an expedited pathway with a supporting process framework. Duke-Margolis appreciates CMS's consideration of our comments, and the Administration's support for advancing high-value, affordable healthcare. We and our colleagues would be pleased to provide more information on these issues if that would be helpful.

Sincerely,

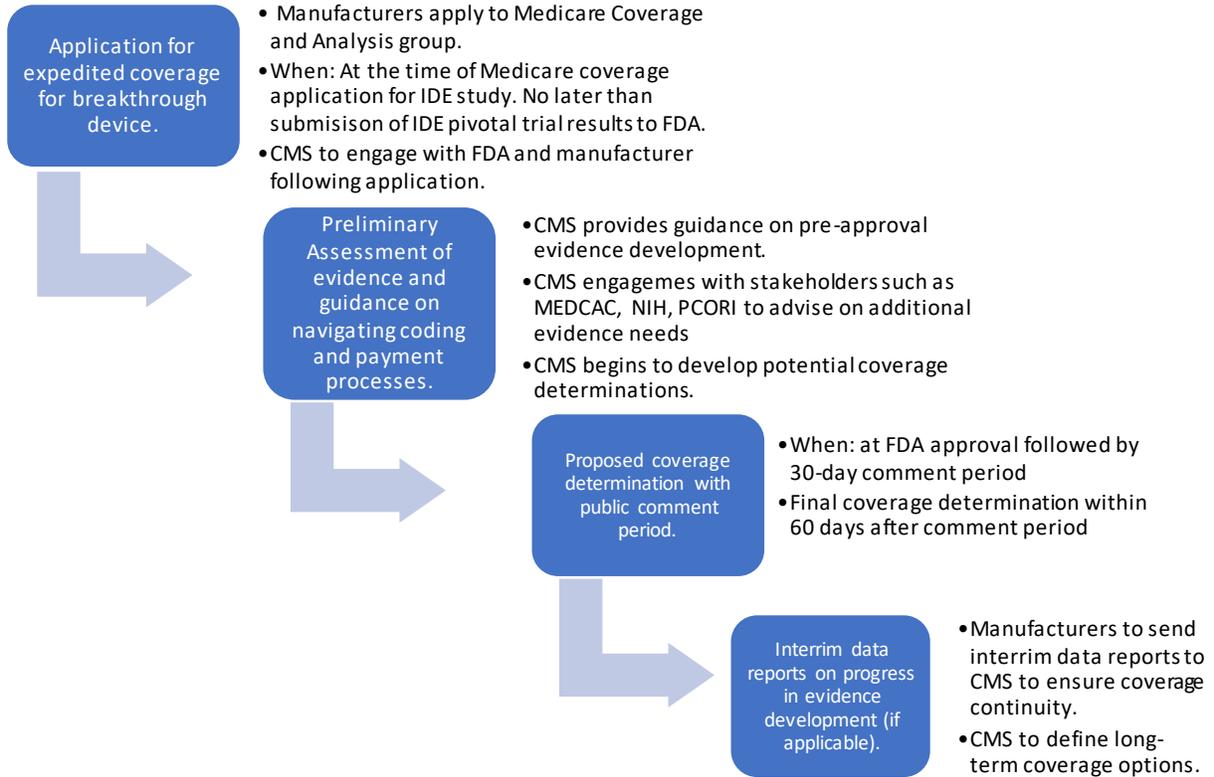
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Appendix A



Appendix B

CMS's concerns with MCIT	Suggested modifications to MCIT or design for new expedited coverage pathway
<p>Limited ability of CMS to ensure safety of Medicare beneficiaries</p>	<ul style="list-style-type: none"> • Manufacturer applies for expedited coverage breakthrough designation to begin early CMS engagement for guidance on key evidence questions for Medicare beneficiaries. • CMS retains authority to rescind national coverage during the expedited pathway process if safety concerns are found.
<p>May provide coverage without supporting data of benefit for Medicare populations.</p>	<ul style="list-style-type: none"> • Early and sustained engagement between CMS, FDA, and manufacturers during pre-approval process to identify gaps in evidence.
<p>Voluntary data collection can lead to insufficient evidence for permanent coverage determinations.</p>	<ul style="list-style-type: none"> • Requires evidence development for breakthrough devices that have significant evidence gaps through Coverage for Evidence Development or similar evidence generation component.
<p>Unfair advantage not available to subsequent market entrants, potentially disincentivizing innovation.</p>	<ul style="list-style-type: none"> • Consistent with Medicare past coverage determinations, coverage for a breakthrough device will include follow on and iterative devices.
<p>No defined path to minimize gaps in access from coding and payment.</p>	<ul style="list-style-type: none"> • Coordination with Office for Technology Policy, Coding, Pricing to begin coding and payment processes prior to approval.

References

- ⁱ Center for Devices and Radiological Health, “Breakthrough Devices Program,” *FDA*, January 5, 2021, <https://www.fda.gov/medical-devices/how-study-and-market-your-device/breakthrough-devices-program>.
- ⁱⁱ Centers for Medicaid & Medicare Services, “Medicare Coverage Document - Guidance for the Public, Industry, and CMS Staff: Coverage with Evidence Development,” CMS, November 20, 2014, <https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?MCDId=27>.
- ⁱⁱⁱ Tamara Syrek Jensen et al., “National Coverage Determination for Transcatheter Edge-to-Edge Repair (TEER) for Mitral Valve Regurgitation,” Centers for Medicaid & Medicare Services, January 19, 2021, CAG-00438R, <https://www.cms.gov/Medicare/Coverage/Coverage-with-Evidence-Development/TMVR>.
- ^{iv} Tamara Syrek Jensen et al., “National Coverage Determination for Vagus Nerve Stimulation (VNS) for Treatment Resistant Depression (TRD) Reconsideration” (Centers for Medicaid & Medicare Services, February 15, 2019), CAG-00313R2, <https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=N&NCAId=292&type=Closed&bc=ACAAAAAAQAAA&>.
- ^v Duke-Margolis Center for Health Policy, “Comment Letter on MCIT Proposed Rule: CMS-2020-0098-0304,” *Regulations.gov*, November 12, 2020, <https://www.regulations.gov/comment/CMS-2020-0098-0304>.
- ^{vi} Duke-Margolis Center for Health Policy, “Comment Letter on MCIT Delay: CMS-2020-0098-0548,” *Regulations.gov*, June 7, 2021, <https://www.regulations.gov/comment/CMS-2020-0098-0548>.
- ^{vii} Mark B. McClellan et al., “Access For 21st Century Cures: Updating Medicare’s Approach To Coverage And Payment,” *Health Affairs* (blog), December 18, 2019, <https://www.healthaffairs.org/doi/10.1377/hblog20191217.27121/full/>.