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August 2, 2024

The Honorable Diana DeGette
United States House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Larry Buschon
United States House of Representatives
2313 Rayburn House Office Building
Washington, DC 20515

RE: 21st Century Cures 2.0 Request for Information, June 2024

Dear Representatives DeGette and Buschon,

On behalf of the [Alliance for Aging Research](http://www.agingresearch.org) (the "Alliance"), we applaud your commitment to improving medical research and healthcare delivery in the United States. The Alliance was honored to provide input on, and support passage of, the 21st Century Cures Act. Our organization advocated for the \$10 billion increase in NIH funding, the NIH provisions for young and emerging scientists to encourage careers in medical research and adjust loan repayments, and creation of the [Inclusion Across the Lifespan](#) effort.

We appreciate your continued passion for clinical and regulatory science, and your dedication to ensuring it is as well-funded, efficient, and meaningful as possible for the patients and families it is supposed to help. We have supported the 21st Century Cures 2.0 (Cures 2.0) effort and appreciate the opportunity to provide feedback on the questions issued in the request for information (RFI) and how additional legislation could best serve the needs of older adults.

Since the introduction of Cures 2.0, there has been significant progress in a number of areas. However, in 2023 the Centers for Medicare and Medicaid Services (CMS) introduced the Transitional Coverage for Emerging Technologies (TCET) program, which fails to meet the goals of Sec. 404 of Cures 2.0 and instead is likely to create additional barriers to care. Our feedback focuses on these concerns and the **urgency of engaging with CMS to ensure key changes are made to the proposed TCET pathway, including remedying the agency's reliance on coverage with evidence development (CED) coverage restrictions.**

Title IV – Medicare and Medicaid Services

Coverage and Payment for Breakthrough Technologies (Sec. 404)

The Alliance opposes CMS’s current proposal of the TCET pathway.¹ While we appreciate CMS’s efforts to bring transparency, predictability, and expedited national coverage for eligible Breakthrough Devices, the proposed TCET pathway will not achieve those goals as its end result will restrict rather than facilitate Medicare beneficiaries’ long-term access to innovative technologies.²

We instead urge Congress to restore the prior language included in Cures 2.0, as well as CMS’s prior Medicare Coverage of Innovative Technology (“MCIT”) language included in Cures 2.0 that would presumptively cover eligible Breakthrough Devices for a period of four years.

The FDA’s breakthrough status allows expedited review for devices that provide “more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions.”³ These devices often provide a treatment option when no clear alternatives exist or that have significant advantages over existing treatments. In many circumstances, a breakthrough device may deliver a therapeutic pathway for a patient when pre-existing technologies for an indication are not clinically appropriate.

FDA only grants a breakthrough device designation to a device upon a showing that the product provides a more effective treatment or diagnosis for a life-threatening or irreversibly debilitating disease or condition and meets one of the following: (i) represents breakthrough technology; (ii) presents the only option (i.e., no approved or cleared alternatives exist); (iii) offers significant advantages over existing approved or cleared alternatives; or (iv) its availability is in the best interest of patients.⁴

CMS previously finalized the MCIT final rule (since withdrawn) that would have covered FDA-designated breakthrough devices for Medicare beneficiaries immediately upon FDA approval or clearance for up to four years.⁵ In that rulemaking, CMS presumed that FDA-designated

¹ Centers for Medicare and Medicaid Services. CMS-3421-NC: Transitional Coverage for Emerging Technologies. Federal Register Vol. 88, No. 122. 27 June 2023. <https://www.govinfo.gov/content/pkg/FR-2023-06-27/pdf/2023-13544.pdf>

² Alliance for Aging Research. RE: Transitional Coverage for Emerging Technologies Notice (CMS-3421-NC). 28 Aug 2023. <https://www.agingresearch.org/wp-content/uploads/2023/08/AAR-Comment-Letter-to-TCET-Notice-FINAL-8.28.pdf>

³ U.S. Food and Drug Administration. Breakthrough Devices Program. 16 May 2019. <https://www.fda.gov/medical-devices/how-study-and-market-your-device/breakthrough-devices-program#s1>

⁴ U.S. Food & Drug Admin., Breakthrough Devices Program: Guidance for Industry and Food and Drug Administration Staff, at 8-14 (Dec. 2018), <https://www.fda.gov/media/108135/download>.

⁵ 86 Fed. Reg. 2987, 2991 (Jan. 14, 2021).

breakthrough devices satisfied the Act's "reasonable and necessary" standard and declined to impose additional clinical studies.⁶ CMS explained that the MCIT pathway would "accelerate the coverage of new, innovative breakthrough devices to Medicare beneficiaries."⁷ CMS even lauded that the MCIT pathway "was completely supported by the public comments," including ours.⁸

However, the TCET proposal takes the program in the opposite direction. CMS claims the TCET pathway will expedite access to technologies for Medicare beneficiaries. However, the reality is that TCET fails to respect the FDA's qualified decision-making and will permit coverage only when manufacturers agree to allow their devices to be subject to CMS's "coverage with evidence development" (CED) requirements.

Under a CED, Medicare denies coverage for an FDA-approved item or service, except for treatments provided through a very narrowly constructed clinical study. That means only a fraction of estimated eligible Medicare beneficiaries will have access to the FDA-approved treatments included. The reason, although CMS equivocates on this, is to save Medicare money. CMS is a payer, and CED has become an efficient way to ration items and services estimated to create a higher cost burden in Medicare Part B, which covers drugs administered in clinics and doctor's offices.

It's important to understand that CMS created CED in agency guidance—Congress did not provide statutory authority for it. As we documented in our February 2023 report, "Façade of Evidence: How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity,"⁹ the current CED process is fundamentally broken, and is used by CMS to deny coverage. CMS has applied CED rationing to procedures for severe hearing loss (cochlear implants), less-invasive heart valve replacement, and stem cell transplant for certain cancers and sickle cell disease.

Once CMS places a treatment in CED, it's extraordinarily difficult to reverse the decision. Between 2005 and 2023, CMS issued a total of 27 CEDs—in that time, the agency retired just five of them. CMS's April 7, 2022, Alzheimer's coverage decision was the first time CMS applied CED to an on-label use of an FDA-approved drug. There are strong signals from CMS that it won't be the last, as gene and stem cell therapies for cancer and rare diseases continue to progress through clinical development.

⁶ Id. at 2992.

⁷ Id. at 2988.

⁸ Id. at 2992; see Comment from Alliance for Aging Research to CMS (Nov. 2, 2020), available at <https://www.regulations.gov/comment/CMS-2020-0098-0291>. The Alliance supported CMS's proposal to align Medicare coverage for breakthrough medical devices with FDA market approval for a period of up to four years, but did not support CMS's proposal to codify a definition for "reasonable and necessary."

⁹ Peschin S, et al. Façade of Evidence. February 2023. <https://www.agingresearch.org/wp-content/uploads/2023/02/Facade-of-Evidence-CED-2-13-2023.pdf>

Although CMS proposes to invite manufacturers of eligible Breakthrough Devices to “voluntarily” participate in the TCET pathway,¹⁰ we are concerned that there is no real choice at all, and that patients appear to be excluded from the process. CMS downplays the reality that manufacturers who do not follow through with the TCET pathway and subject themselves to CED requirements are virtually excluded from Medicare coverage altogether without regard to the implications for beneficiaries resulting from lack of access. Further, there is nothing “voluntary” about the pathway for beneficiaries, who are left in the balance as CMS and manufacturers engage in the proposed process for beneficiaries to have access to these FDA-approved devices. Manufacturers who are driven to bring their innovative, new technologies to beneficiaries as soon as possible must undertake the TCET pathway – despite the coverage restrictions imposed – or patients will not have the option to access an item with clinical benefit.

Further, CMS has said that a maximum of five device candidates will be able to utilize TCET each year. This limitation is arbitrary and threatens to restrict timely access in areas of high unmet need. Rather than expending program resources on restricting access – and with insufficient staff to meet the need for timely and thorough review that would be generated by the TCET – we encourage Congress to consider that CMS simply has no clinical basis to second-guess FDA’s determination or to suggest that FDA’s determination is not reliable for the Medicare population. Congress intended to authorize FDA to carry out the Breakthrough Devices Program and provided FDA with the resources and personnel with expertise to make these determinations, including review of the extensive clinical information that must be provided without CMS’ influence.

Notwithstanding the determination by its sister agency that a breakthrough device offers significant advantages over existing treatments and FDA’s decision that access is in the best interest of patients, CMS contemplates that most—if not all—of the eligible Breakthrough Devices¹¹ will be subject to CED requirements. CMS claims that such products generally have insufficient evidence of clinical benefit in the Medicare population at the time of FDA marketing authorization to support national coverage.¹² CMS’s presumption that FDA-designated breakthrough devices are not “reasonable and necessary” under Section 1862(a)(1)(A) of the Social Security Act flies in the face of FDA’s determinations and undermines FDA’s mission to protect patients. And to be clear, the legal standard for breakthrough devices is more than “safe and effective.” There is a multi-step process test, noted above, that includes many of the factors that CMS should consider in its reasonable and necessary evaluation for coverage.

¹⁰ *Id.* at 41638.

¹¹ CMS defines “eligible Breakthrough Devices” as medical devices that are: (i) FDA-designated Breakthrough Devices; (ii) determined to be within a Medicare benefit category; (iii) not already the subject of an existing Medicare NCD; and (iv) not otherwise excluded from coverage through law or regulation). 88 Fed. Reg. at 41639.

¹² *Id.* at 41637.

The Alliance has not always opposed the use of CED for devices;¹³ however, the shortcomings and harmful impacts of CMS's application of CED have become difficult to ignore and necessitate a new approach.¹⁴ The proposed TCET pathway will undermine Congress's goal, enacted in the 21st Century Cures Act to bring innovative technologies to patients sooner through the FDA's Breakthrough Devices Program.¹⁵ The goal of the breakthrough designation was to get patients expedited access to new devices fulfilling an unmet need and allowing FDA, once essential safety criteria were met and other clinical evidence presented, to approve or clear these products for patient use. The proposed TCET pathway conflicts with Congressional intent and will create a two-tiered system with non-beneficiaries securing access to these devices but requiring Medicare beneficiaries outside the "evidence development" trials created by CMS having to wait for treatments. We appreciate that CMS's coverage and FDA's approval standards differ.¹⁶ That said, we urge Congress to evaluate how to enable faster access to potentially life-saving technologies for Medicare beneficiaries while remaining aligned with the purpose of the Breakthrough Devices Program. Quite simply, approval without the reasonable ability to access is not meaningful from the perspective of the beneficiary.

Due to the detrimental impact that TCET will have on timely beneficiary access, and the clear advantages of the original breakthrough devices proposal in Cures 2.0, **we urge Congress to oppose the TCET proposal and instead advance the prior legislative language to accelerate access to meaningful and potentially life-saving technologies for beneficiaries.** We also broadly encourage Congress to avoid codifying coverage with evidence development in legislation, given the existing challenges in the program and that the current basis for CED relies on regulatory precedent rather than statute.

¹³ Comment from Alliance for Aging Research to CMS. 11 Aug 2021. <https://www.agingresearch.org/wp-content/uploads/2021/08/Alliance-for-Aging-Research-Comment-on-CMS-NCA-CAG-00460N-8-11-2021-FINAL.pdf>.

¹⁴ On the whole, the agency has not illustrated that CED has created an environment conducive to the development of additional clinical data in a timely fashion. As a result, rather than being a tool to facilitate access for therapeutics while additional evidence is developed, it too often has the practical effect of limiting data collection and artificially rationing beneficiary access. A thorough evaluation of CMS's coverage with evidence development program can be found at <https://www.agingresearch.org/wp-content/uploads/2023/02/Facade-of-Evidence-CED-2-13-2023.pdf>

¹⁵ See 21st Century Cures Act, Pub. L. 114-255 § 3051 (codified at 21 U.S.C. § 360e-3) (as amended by Sec. 901 of the FDA Reauthorization Act of 2017, Pub. L. 1152).

¹⁶ See Statement of Rep. Guthrie, Hearing on Innovation in Medicare (July 18, 2023), available at <https://energycommerce.house.gov/posts/health-subcommittee-chair-guthrie-opening-statement-on-innovation-in-medicare> (stating that the TCET proposal "significantly narrows the number and type of products that can use the Breakthrough Devices program for streamlined Medicare coverage" and "is also undermining our innovative ecosystem through actions taken to limit Medicare access to FDA approved breakthrough medical devices and technologies. Instead of rewarding this innovation by providing a streamlined path to Medicare coverage for these novel products, like the ... Medicare Coverage of Innovative Technologies rule would have done, the [proposal] is reducing access to cures for patient policies through its proposed Transitional Coverage of Innovative Technologies rule.").

Alliance for Aging Research

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Conclusion

Thank you for considering our views related to the evolution of policies included in Cures 2.0. If you have questions related to our comments, please contact me at mward@agingresearch.org.

Sincerely,

A handwritten signature in brown ink that reads "Michael Ward". The signature is written in a cursive, flowing style.

Michael Ward, MS

Vice President of Public Policy and Government Relations