AMERICAN ACADEMY OF NEUROLOGY®

201 Chicago Avenue
Minneapolis, Minnesota 55415

Tel: (612) 928-6100
Fax: (612) 454-2744

AAN.com

July 26, 2023

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

RE: Medicare Program; Transitional Coverage for Emerging Technologies [CMS-3421-NC]

Dear Administrator Brooks-LaSure,

The American Academy of Neurology (AAN) is the world's largest neurology specialty society representing more than 40,000 neurologists and clinical neuroscience professionals. The AAN is dedicated to promoting the highest quality patient-centered neurologic care. A neurologist is a physician with specialized training in diagnosing, treating, and managing disorders of the brain and nervous system. These disorders affect one in six people and include conditions such as multiple sclerosis (MS), Alzheimer's disease, Parkinson's disease, stroke, migraine, epilepsy, traumatic brain injury, ALS, and spinal muscular atrophy.

The Centers for Medicare and Medicaid Services (CMS) is proposing to establish a new Transitional Coverage of Emerging Technologies (TCET) pathway for certain eligible devices designated as "breakthrough" by the US Food and Drug Administration (FDA). In establishing this optional coverage pathway, CMS states that "the TCET pathway can support manufacturers that are interested in working with CMS to generate additional evidence that is appropriate for Medicare beneficiaries and that may demonstrate improved health outcomes in the Medicare population to support more expeditious national Medicare coverage."[1] CMS justifies the establishment of the TCET pathway noting that "Medicare beneficiaries are often older, with multiple comorbidities, and are often underrepresented or not represented in many clinical studies" and that "the potential benefits and harms of a device for older patients with more comorbidities may not be well understood at the time of FDA market authorization."[2]

The AAN concurs with CMS that "new approaches are needed to make decisions on certain new items and services, such as medical devices, more quickly to provide expedited access to new and innovative medical

[1] 88 Fed. Reg. at 41637
[2] 88 Fed. Reg. at 41636

technologies."[3] Access barriers exist for all types of therapies, but the obstacles for patients obtaining therapeutic medical devices can be much steeper than those for pharmaceuticals. This is partially attributable to the fact that despite collaboration between the FDA and CMS, both agencies consider different legal authorities and apply different statutory standards when making marketing authorization, as opposed to coverage decisions for medical devices. The FDA makes marketing authorization decisions based on whether the relevant statutory authority for safety and effectiveness is met, whereas CMS makes decisions based on whether a device meets the agency's "reasonable and necessary" threshold.

Further compounding this disparity is that FDA designation of Breakthrough Devices status must meet the agency's regulatory standard of "reasonable expectation" of safety and effectiveness of a device based on "literature or preliminary data (bench, animal, or clinical)" that is assessed prior to the submission of that pr device for consideration of FDA for marketing authorization.[4] For example, clinical "preliminary data" considered by FDA as sufficient for Breakthrough Devices designation could be limited to an "early feasibility study" including as few as 5 normal human subjects.[5]

Furthermore, subsequent FDA marketing authorizations of medical devices must meet a separate statutory "reasonable assurance" standard, wherein Congress requires that FDA adheres to a "least burdensome" standard that has been defined as requiring collection of "the minimum amount of information necessary to adequately address a relevant regulatory question or issue through the most efficient manner at the right time."[6] CMS has previously stated that devices cleared by the FDA under this standard, and via the most common authorizing pathway, i.e. 510(k), "generally does not involve clinical data showing safety and effectiveness".[7] In other words, neither FDA designation of Breakthrough Devices status, nor FDA marketing clearance via 510(k), should be regarded as determinations that a device is either safe or effective, particularly for Medicare populations. Consequently, the AAN supports the underlying concept for TCET and the CED NCD processes.

The AAN supports the development of a voluntary, time-limited pathway for manufacturers and recognizes that Coverage with Evidence Development (CED) can be an integral tool in informing care and coverage decisions. The AAN encourages CMS to explore the most appropriate uses for this pathway, including potential prioritization, recognizing that it is possible that demand for this pathway may substantially outmatch agency bandwidth. The AAN supports that the TCET pathway would utilize the existing CED NCD process and that safeguards that allow for stakeholder input on coverage decisions would remain in place. The AAN appreciates CMS' recognition that medical specialty societies "have valuable expertise

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[3] 88 Fed. Reg. at 41634

[4] "Investigational Device Exemptions (IDEs) for Early Feasibility Medical Device Clinical Studies, Including Certain First in Human (FIH) Studies." Food and Drug Administration, Oct. 2013, www.fda.gov/media/81784/download.

[5] Id.

[6] Center for Devices and Radiological Health, and Center for Biologics Evaluation and Research. "The Least Burdensome Provisions: Concept and Principles Guidance for Industry and FDA Staff." U.S. Food and Drug Administration, Feb. 2019, www.fda.gov/regulatory-information/search-fda-guidance-documents/least-burdensome-provisions-concept-and-principles.

[7] 54 Fed. Reg. at 4307

and first-hand experience in the field that will help CMS develop Medicare coverage policies."[8]

B. TCET General Principles

In explaining the TCET pathway, CMS sets forth several key principles upon which the pathway is based. The AAN's comments on select principles are as follows:

- Prior to FDA marketing authorization, CMS may initiate discussions with manufacturers to discuss any evidence gaps for coverage purposes and the types of studies that may need to be completed to address the gaps, which could include the manufacturer developing an evidence development plan and confirming that there are appropriate safeguards for Medicare beneficiaries.

The AAN has fervently advocated for improving the representation of diverse populations in clinical trials. The AAN recommends that manufacturers utilizing the TCET pathway should be required to demonstrate the methodology for recruiting and retaining diverse participant populations, across a wide range of patient characteristics, as supported by health care institutions around the world and in the USA in alignment with National Institutes of Health standards, including Guiding Principles for Ethical Research[9] and Ethics in Clinical Research.[10] In support of this effort, CMS should work to address disparities based on geography, race, and other socio-economic factors by providing resources to address barriers to trial participation for historically underrepresented populations.

- If CMS determines that further evidence development (that is, CED) is the best coverage pathway, CMS will work with the manufacturers to reduce the burden on manufacturers, clinicians and patients while maintaining rigorous evidence requirements. CMS will work to ensure we are not requiring duplicative or conflicting evidence development with any FDA post-market requirements for the device.

The AAN lauds CMS for the specific mention of reducing burden for clinicians and patients. The AAN firmly believes that evidence generation should not come at the cost of increased reporting or administrative burden for patients and providers. The burden of evidence generation should fall on the manufacturer seeking broader coverage for their particular product. In designing CED requirements, CMS should prioritize evidence gathering methodologies that can be operationalized with minimal effort for practices and patients, including claims-based methodologies, registry-based studies that can collect interoperable data elements directly from the EHR, and fit-for-purpose studies that make use of real-world data. In all cases, manual data entry for patients and providers should be minimized.

- CMS does not believe that an NCD that requires CED as a condition of coverage should last indefinitely, including under the TCET pathway. If the evidence supports a favorable coverage decision under CED, coverage will be time-limited to facilitate

[8] 88 Fed. Reg. at 41638
[9] Guiding Principles for Ethical Research | March 16, 2016
[10] Patient Recruitment: Ethics in Clinical Research | October 21, 2021

the timely generation of sufficient evidence to inform patient and clinician decision making and to support a Medicare coverage determination under section 1862(a)(1)(A) of the Act.

The AAN concurs with CMS that an NCD that requires CED as a condition of coverage should not last indefinitely. The AAN is deeply concerned by the fact that there are CED requirements that have remained in place for longer than a decade. CMS must prioritize expeditious data gathering and continuous evaluation of the appropriateness of CED. The AAN believes that the goal of CED should be to identify the most appropriate patients for a product covered by CED and expeditiously transition patients from being subjected to CED requirements to receiving broad, equitable, and unfettered coverage, as long as they meet evidence-based criteria. The AAN believes it is critical for CED to have a predetermined timeline for interim data analysis, and if that analysis demonstrates that the study endpoints have been met, that the CED should be stopped, and full coverage be established.

D. Procedures for the TCET Pathway

Request for Specific Stakeholder Input on the Evidence Base and Conditions of Coverage

CMS notes that "[s]ince the evidence base for these emerging technologies will likely be incomplete and practice standards not yet established, we believe that feedback from the relevant specialty societies and patient advocacy organizations, in particular their expert input and recommended conditions of coverage (with special attention to appropriate beneficiary safeguards), is especially important for technologies covered through the TCET pathway."[11] The AAN concurs with this statement and firmly believes the input of relevant specialty societies is critical for all CMS coverage decisions. The AAN appreciates CMS' recognition that "[w]hile CMS prefers to have this information during the initial public comment period upon opening the NCD, we realize that in many cases it may take longer for these organizations to provide their collective perspectives to CMS since these technologies will have only recently received FDA market authorization."[12] The AAN urges CMS to communicate with specialty societies regarding relevant opportunities to provide feedback, and encourages the agency to be flexible regarding the time it takes specialty societies to collect evidence and determine consensus perspectives as they pertain to coverage decisions.

Coverage of Similar Devices

Recognizing that breakthrough devices frequently serve as a predicate for the approval of subsequent similar devices, CMS indicates that the agency believes "that it is important to let physicians and their patients make decisions about the best available treatment depending upon the patient's individual situation."[13] As such, CMS is soliciting comments on whether coverage of similar devices using CED would establish a level playing field and avoid delays in access that would occur if a separate NCD were required to ensure coverage. The AAN is concerned that mandating that similar devices fall under the CED requirements established under the TCET would undercut the voluntary nature of this proposal. Furthermore, the AAN

[11] 88 Fed. Reg. at 41642
[12] Id.
[13] Id.

requests clarification regarding how CMS will determine the applicability of a pre-existing CED to similar devices and whether and how evidence generation specific to one device covered under CED will apply to all devices potentially subject to CED requirements under a particular coverage policy. For example, will impacted manufacturers have the opportunity to discuss a similar device designation with CMS? The AAN notes that it is likely that products brought to market will have unique features and CMS should be aware of the need to study each product's unique features individually.

Additionally, if a predicate or other device covered by CED is recalled due to safety concerns, how will CMS approach coverage for all other devices covered under a particular NCD? The AAN recognizes substantial gaps in clinical testing and post-market surveillance for products approved by the FDA based on substantial equivalence to a previously approved and legally marketed device. The AAN supports requiring new evaluation of CED covered products when similar devices are introduced after a predicate is recalled.

Conclusion

Promoting timely and appropriate access to care is a top priority for the AAN. The AAN appreciates the opportunity to comment on the proposed Transitional Coverage for Emerging Technologies pathway. The AAN firmly believes that reforms are needed to safely provide expedited access to new and innovative medical technologies. If you have any questions regarding these comments or seek further input, please contact Matt Kerschner, Director, Regulatory Affairs and Policy at mkerschner@aan.com or Max Linder, Government Relations Manager at mlinder@aan.com.

Sincerely,

Carlayne E. Jackson, MD, FAAN
President, American Academy of Neurology