MEMORANDUM

To: Subcommittee on Health Members and Staff
From: Committee on Energy and Commerce Majority Staff
Re: Health Subcommittee Hearing on July 18, 2023

The Subcommittee on Health will hold a hearing on Tuesday, July 18, 2023, at 10:30 a.m. (ET) in 2322 Rayburn House Office Building. The hearing is entitled "Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology."

I. Witnesses

- **Dr. Natalia Rost, M.D.**, President Elect, American Academy of Neurology
- **Dr. Thomas MacGillivray, M.D.**, President, Society of Thoracic Surgeons
- **Dr. Lishan Aklog, M.D.**, Chairman and Chief Executive Officer, PAVmed
- **Dr. Todd Brinton, M.D.**, Corporate Vice President, Advanced Technology Chief Scientific Officer, Edwards Lifesciences
- **Ms. Sue Wronsky**, Alzheimer's Association Advocate
- **Dr. Brian Miller, M.D.**, Nonresident Fellow, American Enterprise Institute, Assistant Professor of Medicine, Johns Hopkins University School of Medicine

II. Background

Overview

Since 1990, more than 50 million life-years have been saved through medical innovation.[1] The United States ranks eleventh in the World Index of Health Care Innovation, which is a ranking system of 32 national health care systems on their quality, choice, science and technology, and fiscal sustainability.[2] The United States has consistently ranked a clear first in the "science and technology" category thanks to its world-renowned research institutions and biotechnology sector and continues to lead the world in the number of clinical trials.[3]

It is estimated that it takes an average of 4.7 years for certain new medical devices to clear the regulatory national coverage process, while it takes an average of 3.6 years to receive a local coverage determination (LCD) policy through a local Medicare Administrative Contractor

[1] Worthy, Stacey. "Fifty Million Life-Years Saved Through Medical Innovation." 2014. Fifty million life-years saved through medical innovation | The Hill
[2] Roy, Avik. "About the FREOPP World Index of Healthcare Innovation." 2021. https://freopp.org/about-the-freopp-world-index-of-healthcare-innovation-c84200df79b7
[3] U.S. National Library of Medicine. *Map of All Studies on ClinicalTrials.gov*. https://classic.clinicaltrials.gov/ct2/search/map?map=

(MAC).[4] Furthermore, it takes an average of 2.6 years to acquire a new code for the medical device and an average of 3.3 years to establish Medicare payment. This hearing will examine challenges and opportunities to improve Medicare coverage processes in order to encourage innovation and increase access to care.

Medicare Coverage Pathways for Innovative Drugs, Medical Devices & Technologies

Reasonable and Necessary Standard

Section 1862(a)(1)(A) of the Social Security Act (SSA) prohibits the Secretary from covering items or services under Medicare Parts A or Part B that are not "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member," and provides the Secretary the authority to determine whether a particular medical item or service is "reasonable and necessary."[5]

In making coverage determinations, the Centers for Medicare and Medicaid Services (CMS) has historically considered whether an item or service is safe and effective (not experimental or investigational) and appropriate.[6] These criteria are also included in the Medicare Program Integrity Manual (PIM).[7] Medicare currently uses several different coverage pathways for items and services, all of which serve under the reasonable and necessary standard.

National Coverage Determinations (NCDs)

Section 1862(l)(6)(A) of the SSA defines a national coverage determination (NCD) as "a determination by the Secretary with respect to whether or not a particular item or service is covered nationally under this title." NCDs are essentially policy statements by the Medicare program to identify the circumstances and manner in which items are covered under the program. CMS has historically relied on (1) health outcomes data and (2) an analysis of whether an item or service falls under an existing Medicare benefit category to make its determination.

Requestors seeking national Medicare coverage for an item or service must go through the NCD process, which requires a comprehensive review of the item or service involving public participation and feedback. The NCD process is statutorily prescribed to take between 9 to 12 months to complete, depending on whether the NCD analysis includes an external technology assessment or Medicare Evidence Development and Coverage Advisory Committee (MEDCAC) meeting, which allows the extra three months of analysis.[8]

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[4] Health Management, Policy, and Innovation. "The Need for Accelerated Medicare Coverage of Innovative Technologies: Impact on Patient Access and the Innovation Ecosystem." 2021. HMPI The Need for Accelerated Medicare Coverage of Innovative Technologies: Impact on Patient Access and the Innovation Ecosystem - HMPI

[5] https://www.ssa.gov/OP_Home/ssact/title18/1862.htm

[6] Federal Register. 54 FR 4307. 1989. https://archives.federalregister.gov/issue_slice/1989/1/30/4298-4318.pdf

[7] U.S. Centers for Medicare and Medicaid Services. "Medicare Program Integrity Manual." Chapter 13 – Local Coverage Determinations. https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/downloads/pim83c13.pdf

[8] U.S. Centers for Medicare and Medicaid Services. "National Coverage Determination Process & Timeline." 2023. National Coverage Determination Process & Timeline | CMS

CMS last submitted its statutorily required annual report to Congress on Medicare NCDs in 2021.[9] Additionally, CMS has not updated its NCD dashboard since September 16, 2020, which is intended to provide information about requests in their various stages.[10] This leaves open questions about the number of requests that are (1) currently under review, (2) on a waiting list to be reviewed, or (3) otherwise remain open.

In some cases, CMS may determine coverage of an item or service only in the context of clinical studies in addition to those required by the FDA. This pathway is called "Coverage with Evidence Development" (CED). CMS attempted to utilize conditional coverage policies in the 1990s, but it more formally established its CED policy in two guidance documents issued in 2005 and 2006.[11] Subsequent administrations have deliberated about the legality of the CED paradigm.[12]

Currently, there are more than 20 medical products covered under CED.[13] Between 2005-2022, CMS issued a total of 27 NCDs requiring CED and only four have been retired by the Agency.[14] On average, the CED process takes 11.5 years to be completed, meaning products are required to invest over a decade more time and resources in order to establish a more permanent and predictable form of Medicare coverage.[15] Stakeholders have raised concerns about the time and resource intensive requirements and actions required to maintain CED registries.[16] Notably, CMS also sets "conditions of coverage" for health providers participating in CED studies and registries, which effectively limit access to products in less affluent or densely populated communities.[17]

Local Coverage Determinations (LCDs)

Another pathway to Medicare coverage for an item or service is through the Local Coverage Determination (LCD) process. MACs develop LCDs based on its analysis of the Social Security Act "reasonable and necessary" requirements that only apply in their geographic regions. MACs follow specific guidelines for developing LCDs based on the CMS Program

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[9] U.S. Centers for Medicare and Medicaid Services. *Report to Congress on Medicare National Coverage Determinations For Fiscal Year 2021*. 2021. Report to Congress on (cms.gov)
[10] U.S. Centers for Medicare and Medicaid Services. *National Coverage Determination Dashboard*. 2020. NCD Dashboard (cms.gov)
[11] Neumann, Peter. Chambers, James. "Medicare's Reset on Coverage with Evidence Development." 2013. Medicare's Reset On 'Coverage With Evidence Development' | Health Affairs
[12] Kelly, Cathy. "Medicare's CED for Alzheimer's Drugs May Exceed Statutory Authority, Former HHS Attorneys Say." 2022. https://pink.pharmaintelligence.informa.com/PS145677/Medicares-CED-For-Alzheimers-Drugs-May-Exceed-Statutory-Authority-Former-HHS-Attorneys-Say
[13] U.S. Centers for Medicare and Medicaid Services. "Coverage with Evidence Development." 2023. Coverage with Evidence Development | CMS
[14] Alliance for Aging Research. "*Façade of Evidence: How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity*." 2023. Facade-of-Evidence-CED-2-13-2023.pdf (agingresearch.org)
[15] Partnership to Fight Chronic Disease. "Leaving Medicare Beneficiaries in Limbo." http://www.fightchronicdisease.org/sites/default/files/PFCD%20-%20Leaving%20Medicare%20Beneficiaries%20in%20Limbo_v5.pdf
[16] Partnership to Fight Chronic Disease. "Patient Registries Under CED: Checklist of Endless Difficulties." http://www.fightchronicdisease.org/sites/default/files/PFCD%20-%20CED%20Checklist%20Infographic%20final.pdf
[17] Alliance for Aging Research. "*Façade of Evidence: How Medicare's Coverage with Evidence Development Paradigm Rations Care and Exacerbates Inequity*." 2023. Facade-of-Evidence-CED-2-13-2023.pdf (agingresearch.org)

Integrity Manual. MACs must finalize proposed LCDs within 365 days from opening according to the PIM and can take between 9 to 12 months to develop.[18]

Stakeholders often cite concerns with inconsistencies in LCD policies that may be at odds with either existing NCDs or their interpretation of the reasonable and necessary standard.[19]

Claim-by-Claim Adjudication

If there is no applicable NCD or LCD, MACs make coverage decisions based on the applicability of Section 1862(a)(1)(A) of the Social Security Act. The majority of Medicare claims are handled through this process.

Clinical Trial Policy (CTP) Coverage

The Clinical Trial Policy (CTP) coverage pathway can be used for coverage of routine care items and services (but typically not the underlying technology under trial investigation) in a clinical trial that is supported by certain federal agencies. This pathway has been in use since 2000.[20]

Parallel Review

Parallel Review is a process by which the Food and Drug Administration (FDA) and CMS simultaneously review submitted clinical data to help decrease the amount of time between the FDA's approval of a premarket application (PMA) or granting of a de novo classification and the subsequent CMS NCD policy. The process has two stages: (1) FDA and CMS meet with the applicant to provide feedback on the trial within the FDA pre-submission process and (2) FDA and CMS concurrently review the clinical trial results of the PMA or de novo request. The parallel review has not been utilized often and has achieved disappointing results.[21]

Alzheimer's Disease

In April 2022, Medicare took an unprecedented step by issuing an NCD restricting Medicare coverage of an entire class of FDA-approved drugs that target amyloid for Alzheimer's disease.[22] The NCD established a range of criteria and requirements within its National Coverage

[18] U.S. Centers for Medicare and Medicaid Services. *Medicare Program Integrity Manual*. 2019. https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/downloads/pim83c13.pdf
[19] American Physical Therapy Association. "Win: Medicare Contractors Will Continue to Pay for Remote Therapeutic Monitoring. 2023. https://www.apta.org/news/2023/05/24/rtm-win
[20] U.S. Centers for Medicare and Medicaid Services. *Routine Costs in Clinical Trials*. https://www.cms.gov/medicare-coverage-database/details/ncd-details.aspx?NCDId=1&fromdb=true.
[21] Podemska-Mikluch, Marta. "FDA-CMS Parallel Review – A Failed Attempt at Spurring Innovation." 2019. https://www.emerald.com/insight/content/doi/10.1108/JEPP-D-18-00059/full/html
[22] U.S. Centers for Medicare and Medicaid Services. "Monoclonal Antibodies Directed Against Amyloid for the Treatment of Alzheimer's Disease (AD)." 2023. https://www.cms.gov/medicare/coverage-evidence-development/monoclonal-antibodies-directed-against-amyloid-treatment-alzheimers-disease-ad

Analysis (NCA).[23] The NCD essentially requires that most providers and patients participate in a registry (or a separate CMS-approved study or registry), without a clear timeframe for completion, in order to receive the approved treatment.[24] Patient stakeholders expressed continued concerns about CMS breaking precedent to restrict coverage of a drug for its FDA-approved indications and restricting coverage for an entire class of drugs based on clinical trial data for a single drug before any data on other drugs in the class became available.

On July 6, 2023, the FDA converted Leqembi from the accelerated approval pathway (approved in January 2023) to traditional approval after a confirmatory trial which verified clinical benefit for Alzheimer's patients.[25] In 2012, Congress passed the Food and Drug Administration Safety Innovations Act (FDASIA), which allows the FDA to base accelerated approval for drugs for serious conditions addressing an unmet medical need on whether the drug has an effect on a surrogate or intermediate clinical endpoint.[26] Prior to Leqembi's traditional and accelerated approvals, Aduhelm was approved by the FDA in June of 2021 using the accelerated approval pathway.

In February 2023, the CMS Innovation Center announced it would test a model to develop new payment methods for drugs approved under the accelerated approval pathway to reduce Medicare spending on certain accelerated approval drugs.[27]

Medicare Coverage of Innovative Technology (MCIT)

To expedite the Medicare coverage process for innovative technologies in response to Executive Order 13890, CMS finalized the Medicare Coverage of Innovative Technology (MCIT) and Definition of "Reasonable and Necessary" (R&N) rule in January 2021.[28] This rule created a new Medicare coverage pathway for innovative medical devices under the FDA Breakthrough Devices Program. The MCIT rule allowed for devices cleared for market authorization by the FDA to be immediately covered by Medicare for 4 years, eliminating the lag time between FDA approval and Medicare coverage known as the "valley of death."[29] Patient stakeholders, provider organizations, and innovators were generally supportive of the predictable coverage pathway and its potential impact on medical innovation and patients.[30]

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[23] U.S. Centers for Medicare and Medicaid Services. "Monoclonal Antibodies Directed Against Amyloid for the Treatment of Alzheimer's Disease. https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=N&ncaid=305

[24] U.S. Centers for Medicare and Medicaid Services. *Monoclonal Antibodies Directed Against Amyloid for the Treatment of Alzheimer's Disease CED Study Registry*. https://qualitynet.cms.gov/alzheimers-ced-registry

[25] U.S. Food and Drug Administration. "FDA Converts Novel Alzheimer's Disease Treatment to Traditional Approval." 2023. FDA Converts Novel Alzheimer's Disease Treatment to Traditional Approval | FDA

[26] U.S. Food and Drug Administration. "Accelerated Approval." 2023. Accelerated Approval | FDA

[27] U.S. Centers for Medicare and Medicaid Services. "HHS Secretary Responds to the President's Executive Order on Drug Prices." 2023. HHS Secretary Responds to the President's Executive Order on Drug Prices | CMS

[28] U.S. Centers for Medicare and Medicaid Services. "CMS Unleashes Innovation to Ensure Our Nation's Seniors Have Access to the Latest Advancements." 2021. CMS unleashes innovation to ensure our nation's seniors have access to the latest advancements | CMS

[29] U.S. Centers for Medicare and Medicaid Services. "CMS Acts to Spur Innovation for America's Seniors." 2020. https://www.cms.gov/newsroom/press-releases/cms-acts-spur-innovation-americas-seniors

[30] AdvaMed. "Patient Groups Support Giving Seniors Access to Life-Saving Medical Technologies Through MCIT." 2021. https://www.advamed.org/industry-updates/news/patient-groups-support-giving-seniors-access-to-life-saving-medical-technologies-through-mcit/

As part of the MCIT rule, Medicare also established more clear regulatory standards to determine whether innovative devices or items and services furnished under Medicare Parts A and B qualify as "reasonable and necessary" since stakeholders have historically raised questions about the lack of clarity around that standard, especially as new and emerging technologies take shape and do not fit neatly into existing benefit categories or require different types of evidence to determine safety and efficacy for the Medicare population.

However, prior to the rule's implementation, CMS delayed the rule in March of 2021 and again in May of 2021 and then repealed the MCIT/R&N final rule in November 2021.[31] CMS argued that the MCIT rule would not adequately evaluate the effect of new devices on the Medicare population and that it would take future action to address the concerns.

Transitional Coverage for Emerging Technologies (TCET):

On June 22, 2023, Medicare issued a proposed notice with public comment known as the "Transitional Coverage for Emerging Technologies" (TCET).[32] This proposed sub-regulatory framework is a new coverage process that adds onto the existing NCD and CED pathways by creating new mechanisms for manufacturers to communicate with the FDA and CMS earlier in the approval and coverage processes. While this framework would allow CMS to engage with certain FDA-designated Breakthrough Devices prior to coming to market, it would still require innovators to go through a lengthy evidence development process without a predictable or transparent coverage pathway. It remains to be seen whether and how lessons from the failures of the parallel review pathway will be applied to make the TCET process more successful. [33]

This hearing will allow for the Committee to hear directly from witnesses who understand the tradeoffs involved in these various approaches and how delays in access to innovative medical technologies can be harmful to patients and their providers.

III. Staff Contacts

If you have questions regarding this hearing, please contact Alec Aramanda or Caitlin Wilson of the Committee staff at 202-225-3641.

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[31] U.S. Centers for Medicare and Medicaid Services. "CMS Repeals MCIT/R&N Rule; Will Consider Other Coverage Pathways to Enhance Access to Innovative Medical Devices." 2021. https://www.cms.gov/newsroom/press-releases/cms-repeals-mcitrn-rule-will-consider-other-coverage-pathways-enhance-access-innovative-medical

[32] U.S. Centers for Medicare and Medicaid Services. " Notice with Comment – Transitional Coverage for Emerging Technologies (CMS-3421-NC). 2023. https://www.cms.gov/newsroom/fact-sheets/notice-comment-transitional-coverage-emerging-technologies-cms-3421-nc

[33] Holtzman, Jessica N. *Harmonizing Standards and Incentives in Medical Device Regulation: Lessons Learned From the Parallel Review Pathway*. 2019. https://dash.harvard.edu/bitstream/handle/1/41971472/HOLTZMAN-SCHOLARLYPROJECT-2019.pdf?sequence=1&isAllowed=y