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Authority of Medicare to Limit Coverage of FDA-Approved Products
Legal and Policy Considerations

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IMPORTANCE When the US Food and Drug Administration (FDA) approves a drug or medical device on the basis of limited clinical evidence, the Centers for Medicare & Medicaid Services (CMS) must decide whether the therapy is "reasonable and necessary" for coverage among Medicare beneficiaries. However, the legal underpinnings of CMS's authority to shape coverage of FDA-regulated products under Medicare Part B are controversial. To clarify this area, we reviewed relevant legal precedents on CMS's approaches to limit coverage and recent decisions Medicare has issued affecting coverage for FDA-regulated products.

OBSERVATIONS The CMS continues to exercise considerable legal discretion to limit coverage of FDA-authorized products to only uses it determines are reasonable and necessary for patients with Medicare. Courts have upheld this discretion repeatedly, emphasizing the difference between Medicare's coverage criteria and the FDA's review standards. As more new drugs and devices come to market without solid evidence of efficacy on clinical outcomes, or have narrow benefit-risk considerations, CMS may increasingly rely on forms of limited or conditional coverage, including coverage with evidence development (CED), which provides reimbursement only in the context of a clinical trial or registry.

CONCLUSIONS AND RELEVANCE The ability of CMS to condition or limit coverage of FDA-approved products is a commonsense necessity for this crucial taxpayer-funded program. Although courts have thus far deferred to the authority of CMS to make such decisions on the basis of its clear statutory discretion and public health expertise, Congress may want to act to reaffirm statutory language giving CMS sufficient flexibility to craft coverage determinations that reflect the evidence for a product's use.

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Editorial

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Medicare, the largest health care payer in the US, covers about 65 million people, most of whom are older than 65 years, for about $829 billion, or 10% of total annual federal government spending.[1] Medicare Parts A and B, which pay for hospital costs and other medical services, are prohibited by law from paying for any medical products or procedures that are not "reasonable and necessary."[2] Historically, the determination of what is reasonable and necessary has been left up to the Centers for Medicare & Medicaid Services (CMS), the agency responsible for administering Medicare.[3]

For a limited number of major coverage decisions, CMS issues a National Coverage Determination (NCD), a statement of policy that supersedes local decision making and determines whether Medicare reimburses for a given product or service nationwide.[4] Depending on determinations by CMS of what coverage is appropriate under the statute, NCDs can require coverage, deny coverage, or place certain conditions on coverage. A fraction of NCDs limit coverage to only the context of approved clinical trials or registries, through a determination of "coverage with evidence development" (CED).[5] In 2022, CMS issued a CED for the class of drugs that includes aducanumab (Aduhelm), which was granted accelerated approval by the FDA based on unclear evidence of efficacy, requiring that covered patients be enrolled in a qualifying randomized trial.[6]

Recently, CMS announced a CED plan for another controversial Alzheimer drug of the same class, lecanemab (Leqembi), which in 1 trial slowed cognitive decline among patients with mild cognitive impairment or early-stage Alzheimer disease to a small degree that some experts consider not clinically meaningful, while presenting risks of brain swelling and bleeding.[7] Like aducanumab, lecanemab was initially granted accelerated approval from the FDA on the basis of a surrogate measure (changes in β-amyloid levels in the brain). Under the proposed plan, now that it has received full approval from the FDA in July 2023, CMS will cover lecanemab in the much broader context of clinicians' providing limited information to a clinical registry at the time of prescribing.

The ability of CMS to shape coverage of FDA-approved products carries substantial policy implications. As more new drugs and devices are approved by the FDA that lack solid evidence of efficacy on clinical outcomes, or have narrow benefit-risk considerations, CMS may increasingly rely on forms of limited or conditional coverage.[8] However, the legal underpinnings of CMS's authority to craft conditions on coverage of medical products under Medicare are controversial. The CED program has drawn scrutiny in the wake

of the aducanumab decision,[9] and a recently introduced "Access to Innovative Treatments" bill in the House of Representatives would create a new legal avenue to challenge CMS decisions that limit coverage of therapies including unproven drugs and devices.[10]

To clarify the legal authority of CMS regarding coverage determinations, we reviewed relevant legal precedents on CMS's past efforts to limit coverage, recent NCDs Medicare has issued limiting coverage for FDA-regulated products, and key legal and policy considerations, offering recommendations to policymakers seeking to ensure that CMS has sufficient flexibility to cover products in ways that reflect the evidence for a product's use.

Legal Framework

To identify cases in which federal courts interpreted Medicare's coverage discretion under the reasonable and necessary clause, as well as specifically with regard to FDA-regulated products, we conducted an iterative search of Westlaw, Google Scholar, and the relevant legal and policy literatures using combinations of the following search terms: *reasonable and necessary*, *Medicare*, *beneficiary or beneficiaries*, *coverage*, *denial*, *national coverage determination*, *coverage with evidence development*, *FDA*, *drug*, and *device*.

Courts' Recognition of Coverage Discretion

Since the creation of Medicare, beneficiaries, manufacturers, and hospitals have mounted legal challenges to decisions denying coverage. Such challenges generally fail because courts have deferred to the government's determination of what items and services are reasonable and necessary, including for FDA-approved products (Box).[2,3,11-17]

In declining to consider a Medicare coverage denial in 1984, the Supreme Court stated that the decision "as to whether a particular medical service is 'reasonable and necessary'" is "clearly [a] discretionary decision…" left up to the agency.[18] In that case, a Medicare beneficiary had sued the Health Care Financing Administration, CMS's predecessor agency, seeking to force the agency to conclude that bilateral carotid body resection surgery should be covered as reasonable and necessary. The agency had issued a national determination that Medicare would not pay for the surgery when performed to relieve respiratory distress, on the basis that it was not safe and effective for that purpose.

Although the Supreme Court declined for procedural reasons to address the agency's policy limiting coverage of that surgery, the Federal Court of Appeals for the 8th Circuit addressed the merits head-on in a subsequent case, upholding the agency's discretion to limit coverage and denying the challenge.[19] In that case, the court rejected the beneficiary's argument that the denial of coverage was unreasonable, or arbitrary and capricious, under the Administrative Procedure Act. Pointing to the complexities of the Medicare program and difficulty of determining the appropriate scope of coverage, the court stated, "It is precisely this type of decision—made within the context of an extremely technical and complex field—that courts should leave in the hands of expert administrators. This is not an appropriate occasion for judges to play doctor."[19] Lower courts have followed this reasoning in upholding Medicare's discretion to limit coverage in various settings, including against constitutional challenges,[20] noting that "The Medicare Act demonstrates a congressional intent to commit maximum discretion to the Secretary."[21]

Box. Defining "Reasonable and Necessary" Care

The Medicare Act prohibits reimbursement under Parts A and B of any "items or services" that are not "reasonable and necessary" for a medical purpose.[2] "Reasonable and necessary" is a narrower standard than the statutory requirements for safety and efficacy used by the FDA for the approval of drugs and some high-risk medical devices, meaning that not all FDA-approved products must be covered by Medicare.[11]

The Centers for Medicare & Medicaid Services (CMS) has endeavored for decades to offer a definition of "reasonable and necessary," but no lasting definition has emerged.[3] In particular, the question of whether to consider the cost of therapies has long vexed CMS officials and, combined with industry lobbying, hindered the development of national criteria.[12] In an illustrative attempt in 1989, CMS's predecessor proposed a definition that would have considered a therapy's cost-effectiveness, but this effort met with major pushback from industry and other stakeholders, and the plan was scrapped.[13]

In January 2021, in the final days of the Trump Administration, CMS issued a rule committing itself to a definition of "reasonable and necessary," the first such binding declaration in the history of the Medicare program.[14] It defined "reasonable and necessary" as an item or service that is "safe and effective," not experimental or investigational, and "appropriate for Medicare patients" under a list of criteria, which included, controversially, consideration of how private insurers cover the item or service. This same rule also committed Medicare to automatically covering all medical devices designated under FDA's breakthrough pathway as "reasonable and necessary" for 4 years.

After President Biden took office, CMS repealed the rule, including the definition, in November 2021. The reasons centered on the device coverage policy, which the agency determined was "not in the best interest of Medicare beneficiaries" because it may require CMS to "provide coverage without adequate evidence."[15] In also repealing the definition of "reasonable and necessary," CMS noted only that "further stakeholder engagement" was needed.

Without a national definition, CMS decides what is "reasonable and necessary" through a combination of National Coverage Determinations and guidelines for Medicare Administrative Contractors (MACs), which are private insurers that process claims from Medicare Parts A and B. If there is no national determination from CMS, coverage decisions fall to MACs to determine whether reimbursement is "reasonable and necessary" in accordance with CMS guidelines, using a combination of local coverage determinations, which set carrier-wide rules for specific products, and individual coverage determinations.[16] As a result, state-by-state coverage varies substantially.[17]

Limiting Coverage of FDA-Regulated Products

Courts have shown similar deference in upholding Medicare's authority to limit coverage of FDA-approved products. For example, the Fourth Circuit in 2012 upheld the denial of coverage of a medical device against a challenge brought by a manufacturer. Noting that the "reasonable and necessary" judgment required "a significant degree of medical judgment," the court found that "the very nature of the Medicare program suggests that the Secretary's determinations are entitled to deference from this court."[22] The manufacturer argued that the device was entitled to coverage because it had

been cleared by the FDA under the 510(k) pathway for medical devices that can demonstrate substantial equivalence with other devices on the market. The court disagreed that Medicare coverage should hinge on FDA action, noting that the Medicare statute "contemplates no role for the FDA," and that FDA clearance or approval "cannot tie the Secretary's hands"[22] given the different statutory missions the agencies pursue.

The Second Circuit in 2006 also emphasized the difference between the FDA's approval standards and CMS's coverage standards. In that case, Medicare had instructed medicare administrative contractors (MACs) not to reimburse any devices that had not been "approved for marketing by the FDA," a decision the court found was inadequately justified. The court found that CMS had failed to explain the link to FDA approval as a sufficient reason for Medicare coverage of medical devices, stating that "reasonable and necessary is not obviously the same standard as safe and effective, and the authority to determine which devices are reasonable and necessary" lies with Medicare, and "not the FDA—an agency with a separate statutory purpose and agenda."[23pg,74] The case is notable as a rare example of a court invalidating a CMS coverage determination.

CMS Coverage Policy

"Least Costly Alternative" Policy

Although courts have consistently upheld CMS's authority to decide whether to cover an item or service for a certain use, CMS lacks authority over the extent of reimbursement. In *Hays v. Sebelius* (2009), the DC Circuit Court struck down a coverage-limiting strategy that CMS employed called the "least costly alternative" policy, under which Medicare reimbursed for treatments only up to the price of an alternative treatment that is "reasonably feasible and medically appropriate."[24] MACs were required to follow the policy for durable medical equipment, and could choose to apply it to prescription drugs.

A Medicare beneficiary challenged a MAC's decision to apply this "least costly alternative" policy to DuoNeb, a combination asthma treatment comprised of albuterol and ipratropium, by only reimbursing for the cost of those 2 drugs separately, which was lower.

The CMS argued that the statute was ambiguous when it prohibited payment for "any expenses incurred for items or services which…are not reasonable and necessary." The phrase "reasonable and necessary," the agency argued, modifies "expenses," and not "items or services," as the beneficiary argued. Thus, CMS could cover DuoNeb's "expense" to the extent it is "reasonable and necessary"—in other words, the cost of the 2 components when purchased separately, but no more. Under the legal doctrine known as *Chevron*, courts are supposed to defer to an agency's interpretation of their enabling statute if the law is ambiguous and the interpretation is reasonable.[25] But in this case, the court found that the statute was clear, holding that "reasonable and necessary" unambiguously refers to items or services. The statute, the court argued, requires "a binary choice: either an item or service is reasonable and necessary, in which case it may be covered at the statutory rate, or it is unreasonable or unnecessary, in which case it may not be covered at all."[25] The court declined to decide whether, in making coverage decisions, CMS could consider cost. But the opinion upheld CMS's authority to deny coverage altogether, while preventing it from providing coverage that limited reimbursement to not exceed a reasonable comparator.

Coverage With Evidence Development

One strategy CMS has employed is CED, determinations that condition coverage on patients' participation in clinical trials or registries approved by CMS. The aim is to incentivize the production of evidence that might support a broader coverage determination while limiting inappropriate coverage.[26]

The CED program began with registries, when CMS required a small number of hospitals to submit data as a condition of coverage, including for fludeoxyglucose (FDG) positron emission tomographic (PET) scans for cancer in 2004.[27] A current CMS guidance document states that "While the intent…was to monitor the appropriateness of use of these items and services, we recognized that the data could also be used to generate useful clinical evidence."[28] Since then, CED has offered coverage in other research settings, including clinical trials. A review of the program found "significant variation" in how it is used.[29] Of the 27 CEDs issued as of 2022, 4 have been granted broader coverage after the production of confirmatory evidence.[29] The number of trials or registries associated with a CED ranged from 0 to 33, and for 4 CEDs, there were no registries or trials associated. The Centers for Medicare & Medicaid Services does not require a trial or registry to be in place to make a CED determination.

The Centers for Medicare & Medicaid Services has identified a statutory basis for the CED program.[28] Although the Medicare statute broadly prohibits payment for items or services that are not "reasonable and necessary" for a medical purpose, a separate, narrower provision prohibits payment for "research conducted" by the Agency for Healthcare Research and Quality which is not "reasonable and necessary to carry out" its clinical research mission.[30] The Centers for Medicare & Medicaid Services has not disavowed the first provision as a valid legal basis for the CED program, but it has more often relied on this second, research-based provision, which carries the implication, without outright stating, that payments for research may be appropriate even when clinical benefit is not established.

The authority of CMS to limit coverage to only research settings has been upheld in court. The DC District court in 2016 upheld CMS's CED determination for a β-amyloid PET scan involving an FDA-approved diagnostic drug, florbetapir (amyvid), used in the diagnosis of dementia. A group of Medicare beneficiaries sued, arguing that CMS's reason for denying coverage, that the results of the scan do not affect treatment, was an inadequate explanation for denying coverage of a diagnostic test. The court disagreed, holding that the words "reasonable and necessary" vested the agency with sufficient discretion to issue an NCD denying coverage of products except for "limited use in certain clinical studies."[31]

The court did, however, find that CMS failed to adequately distinguish between the β-amyloid scan, which it refused to cover outside clinical trials, and the FDG PET scan, which it had covered with only a data reporting requirement since 2004. Because the agency did not give strong enough reasons for this apparent discrepancy, the court found that its decision was arbitrary and capricious, in violation of federal law. Rather than force CMS to cover the scan, as the beneficiaries urged, the court returned the issue to CMS, giving it

an opportunity to reconcile the 2 coverage decisions. The agency provided such an explanation in response to the ruling,[32] and the scan has been subject to an ongoing CED ever since.

Although in the β-amyloid scan case CED offered coverage only in the context of a randomized clinical trial, the court's reasoning likely extends to CEDs that offer coverage in the broader context of registries collecting observational data from patients in noncontrolled settings, which is the sort of CED that CMS plans for lecanemab. Notably, the court did not even identify the FDG PET scan's registry requirement as a limitation on coverage, describing it as simply covered by Medicare and implying that registries are well within CMS's authority to require.

Recent CMS Decisions Limiting Coverage for FDA-Approved Products

In addition to CED, CMS employs others forms of conditional coverage as well. Non-overlap between FDA approval and CMS coverage has been long documented. A prior analysis of Medicare coverage determinations from 1999 to 2011 found that CMS covered Part B drugs and devices only 80% of the time, and often added conditions to coverage of FDA-approved products, especially for medical devices.[33]

From 2010 to 2021, CMS issued 23 restrictive NCDs related to FDA-regulated products (**Table**). Many determinations related to procedures involved FDA-regulated products, such as diagnostic tests or implantable devices. For example, extracorporeal photopheresis involves an FDA-approved device (the Therakos CELLEX system) and an FDA-approved drug (methoxsalen).

Two treatments were denied coverage altogether: collagen meniscus implant and outpatient intravenous insulin treatment. Another 10 were granted CED, including leadless pacemakers, stem cell transplantation for sickle cell disease, and transcatheter aortic valve replacement.

Finally, 12 limited coverage to only certain uses, employing a flexible approach to listing covered and noncovered uses of a treatment. For some treatments, such as screening for hepatitis C virus infection, CMS lists covered indications, with the remainder uncovered. For other treatments, such as ocular photodynamic therapy, CMS lists both covered and noncovered indications, allowing MACs to determine coverage for any remaining uses. For two NCDs, transcatheter mitral valve repair and extracorporeal photopheresis, CMS combined CED with indication-based limits, covering some indications entirely but others only in a research context.

While most restrictive NCDs involve devices, diagnostics, or procedures, a rare few have involved FDA-approved drugs and biologic products. In addition to the 2022 CED for monoclonal antibodies for use in Alzheimer disease approved based on changes to β-amyloid alone, CMS has issued limited coverage determinations for florbetapir (amyvid) for use in β-amyloid PET scans, aprepitant (emend) for chemotherapy-induced nausea, outpatient intravenous insulin treatment, and stem cell treatments.

Discussion

The CMS continues to exercise its considerable legal discretion to limit coverage of FDA-regulated products to only uses it determines are reasonable and necessary under Medicare Part B. Courts have upheld this discretion repeatedly, emphasizing the difference

Table. National Coverage Determinations (NCDs) Limiting Coverage of US Food and Drug Administration (FDA)-Approved Products, 2010 to 2021[a]

Product/procedure	Date(s) NCD implemented	Coverage determination
Collagen meniscus implant	2010	Noncovered
Outpatient intravenous insulin treatment	2010	Noncovered
Pharmacogenomic testing for warfarin response	2010	Coverage with evidence development (listed as "noncovered" in the report to Congress)
Allogeneic hematopoietic stem cell transplantation for myelodysplastic syndrome	2011	Coverage with evidence development
Home use of oxygen to treat cluster headache	2011	Coverage with evidence development (revoked, coverage deferred to MACs)
β-Amyloid positron emission tomography in dementia and neurodegenerative disease	2014	Coverage with evidence development
Percutaneous image-guided lumbar decompression for lumbar spinal stenosis	2015	Coverage with evidence development
Leadless pacemakers	2017	Coverage with evidence development
Stem cell transplantation (multiple myeloma, myelofibrosis, sickle cell disease)	2017	Coverage with evidence development
Transcatheter aortic valve replacement	2013, 2020	Coverage with evidence development
Transcatheter mitral valve repair (updated to transcatheter edge-to-edge repair in 2021)	2015	Coverage with evidence development; 2 noncovered indications
Extracorporeal photopheresis	2013	Coverage limited to 3 indications plus coverage with evidence development for another indication
Screening for hepatitis C in adults	2015	Covered for certain high-risk people or people within a certain age range, remainder uncovered
Aprepitant for chemotherapy-induced emesis	2014	Covered if: (1) in combination with 2 other drugs (an oral 5-HT3 antagonist and oral dexamethasone), and (2) for patients taking specified oncology drugs
		Not covered if used alone as a full replacement for intravenously administered antiemetic agents
		Remainder up to MACs' discretion
Microvolt T-wave alternans	2015	One covered indication
Ocular photodynamic therapy with verteporfin for macular degeneration	2013	Lists multiple covered and noncovered indications
Screening for HIV infection	2016	Lists multiple covered and noncovered indications
Screening for hepatitis C virus in adults	2015	Covered for certain high-risk people or people within a certain age range, with the remainder uncovered
Dermal injections for the treatment of facial lipodystrophy syndrome	2010	Limited to only HIV$^+$ patients with depression as a result of antiretroviral treatment

(continued)

Table. National Coverage Determinations (NCDs) Limiting Coverage of US Food and Drug Administration (FDA)-Approved Products, 2010 to 2021[a] (continued)

Product/procedure	Date(s) NCD implemented	Coverage determination
Percutaneous transluminal angioplasty of the carotid artery concurrent with stenting	2010	Lists multiple covered indications, remainder uncovered
Ventricular assist device	2011; 2014	Two covered indications, all other indications noncovered
Magnetic resonance imaging	2010; 2011	Lists multiple covered and noncovered indications
Next-generation sequencing for Medicare beneficiaries with advanced cancer	2021	Covered with conditions

Abbreviations: MACs, Medicare administrative contractors; 5-HT3, antipruritic potency of serotonin type 3 .

[a] Collected using Centers for Medicare & Medicaid Services (CMS) yearly reports to Congress on coverage actions, available on the CMS website. Positron emission tomography (PET) scans were not included because CMS removed all NCDs for PET scans in 2022. To determine involvement of an FDA-regulated product, we searched the National Coverage Analysis and the coverage Decision Memo. Only NCDs that restricted coverage in some way were included; determinations that required coverage generally or deferred to contractor discretion were excluded.

between Medicare's coverage criteria and the FDA's approval standards. Within the framework arising from federal courts' interpretation of the Medicare statute, CMS has over the past decade limited coverage of dozens of FDA-regulated products to only certain circumstances, most notably by restricting coverage to include or exclude certain indications, and through CED, while completely denying coverage just 2 times.

Notwithstanding the scope of CMS's discretion, inconsistent coverage determinations threaten Medicare's ability to provide conditional coverage. Industry challengers seeking to ensure maximum coverage will sue CMS on the basis that the agency's approach to their product is more restrictive than it has been in past cases. Courts will look to other similar NCDs to decide whether this coverage determination is lawful, as happened in the β-amyloid PET scan case.

Although CMS has discretion over coverage, it is limited with regard to setting appropriate reimbursement. The Medicare statute, as written, distorts research incentives and makes Medicare vulnerable to exorbitantly priced products that threaten the Medicare budget. For example, if CMS had covered aducanumab at its initial list price, it could have doubled total Medicare spending, forcing Medicare to announce the largest increase in Part B premiums in its history.[34] An amendment to the language of the statute could cure this defect, allowing CMS to also set reimbursement rates that it considers reasonable and necessary.[35]

As the FDA continues to approve novel treatments on the basis of pivotal trials using surrogate measures as outcomes, CMS will face growing pressure to cover products marketed to treat diseases that lack a clear base of evidence for their use.[36,37] An amendment clarifying CMS's coverage discretion could better help ensure that, in setting coverage for products regulated by the FDA, CMS will pay an appropriate amount for only products that are reasonable and necessary for a medical or research purpose.

These changes are especially needed in light of courts' increasingly nondeferential review of agency policymaking.[38] The deference that Medicare coverage policy has received thus far, premised on respect for agency expertise and the complexity of the Medicare program, may not be extended in future decisions.

Congress could also provide clearer statutory authorization for CMS's coverage with evidence development program. Courts have recognized CMS's authority to make CED determinations, but the program has not faced a head-on statutory challenge. As CMS continues to consider CED for high-profile therapies, Medicare beneficiaries or manufacturers seeking coverage will sue, arguing that CMS must cover the item as reasonable and necessary under all circumstances. An explicit grant of authority for CED would further validate CMS's approach, incentivizing the development and use of valuable therapies while protecting CMS's discretion to tailor coverage determinations to the specific benefits and risks of novel products.

Conclusions

Medicare's ability to limit payment for therapies that may not meaningfully benefit patients with Medicare is a commonsense necessity for this crucial taxpayer-funded program. Although courts have thus far deferred to CMS's authority to make such decisions on the basis of its clear statutory discretion and public health expertise, Congress may want to act to reaffirm it in light of a legal trend toward limiting agency discretion. As the FDA continues to authorize new drugs and devices on the basis of uncertain data and surrogate measures instead of clinical outcomes, lawmakers, policymakers, and courts must grapple with Medicare's proper role when deciding how to pay for products that lack robust evidence for their use.

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REFERENCES

1. Cubanski J, Neuman T. What to know about Medicare spending and financing. Kaiser Family Foundation. Jan 19, 2023. Accessed June 22, 2023. https://www.kff.org/medicare/issue-brief/what-to-know-about-medicare-spending-and-financing/

2. Social Security Act 1862(a)(1)(A); 42 U.S.C. 1395y.

3. Neumann PJ, Chambers JD. Medicare's enduring struggle to define "reasonable and necessary" care. *N Engl J Med*. 2012;367(19):1775-1777. doi:10.1056/NEJMp1208386

4. CMS.gov. Medicare Coverage Determination Process. March 3, 2022. Accessed June 22, 2023. https://www.cms.gov/Medicare/Coverage/DeterminationProcess.

5. CMS.gov. Coverage with evidence development. March 3, 2022. Accessed June 22, 2023. https://www.cms.gov/Medicare/Coverage/Coverage-with-Evidence-Development

6. Sachs R. Understanding Medicare's Aduhelm coverage decision. Health Aff Forefront. Jan 12, 2022. Accessed June 22, 2023. https://www.healthaffairs.org/do/10.1377/forefront.20220112.876687/full/

7. Sachs R. Prescription drug policy, 2022 and 2023: the year in review and the year ahead. Health Aff Forefront. Jan 9, 2023. Accessed June 22, 2023. https://www.healthaffairs.org/content/forefront/prescription-drug-policy-2022-and-2023-year-review-and-year-ahead

8. Dhruva SS, Ramachandran R, Ross JS. Perspective: Medicare's national coverage determination for aducanumab—a one-off or a pragmatic path forward? *N Engl J Med*. 2022;387(17):1539-1541. doi:10.1056/NEJMp2210198

9. Phillips KA. CMS coverage with evidence development—challenges and opportunities for improvement. *JAMA Health Forum*. 2022;3(9):e223061. doi:10.1001/jamahealthforum.2022.3061

10. Press release: Barragán and Joyce reintroduce the access to innovative treatments act. April 7, 2023. Accessed June 22, 2023. https://barragan.house.gov/2023/04/07/barragan-and-joyce-reintroduce-the-access-to-innovative-treatments-act/

11. Sachs R. Delinking reimbursement. *Minn Law Rev*. 2018;(102):2307-2356.

12. Bartlett Foote S. Why Medicare cannot promulgate a national coverage rule. *Journal of Health, Politics, Policy, and Law*. 2002;L:707. doi:10.1215/03616878-27-5-707

13. Fox JR. Medicare should but cannot consider cost: legal impediments to sound policy. *Buffalo Law Rev*. 2005;(53):609-613.

14. Department of Health and Human Services, Center for Medicare and Medicaid Services. Medicare program; Medicare coverage of innovative technology (MCIT) and definition of reasonable and necessary. *Federal Register*. 2021;86(9):2987-3010.

15. Final Rule, Dept of Health and Human Services, Centers for Medicare & Medicaid Services. Medicare program; Medicare coverage of innovative technology (MCIT) and definition of 'reasonable and necessary.' Nov. 15, 2021. Accessed June 22, 2023. https://www.cms.gov/newsroom/press-releases/cms-repeals-mcitrn-rule-will-consider-other-coverage-pathways-enhance-access-innovative-medical.

16. Medicare Program Integrity Manual. Chapter 13 – Local Coverage Determinations. Accessed June 22, 2023. https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/Downloads/pim83c13.pdf

17. Report, Office of Inspector General, Department of Health and Human Services. Local Coverage Determinations Create Inconsistency in Medicare Coverage. January 7, 2014. Accessed June 22, 2023. https://oig.hhs.gov/oei/reports/oei-01-11-00500.asp

18. *Heckler v Ringer*, 466 US 602 (1984).

19. *Wilkins v Sullivan*, 889 F.2d 135 (7th Cir 1989).

20. *Hatcher v Heckler*, 772 F.2d 427 (8th Cir 1985).

21. *St. Louis University v Blue Cross Hospital Service*, 537 F.2d 283 (8th Cir 1976).

22. *Almy v Sebelius*, 679 F.3d 297 (4th Cir 2012).

23. *Yale-New Haven Hospital v Leavitt*, 470 F.3d 71 (2nd Cir 2006).

24. Centers for Medicare & Medicaid Services., Medicare Benefit Policy Manual § 110.1.C.3 (Rev. 93, July 25, 2008).

25. *Chevron v Natural Resources Defense Council*, 467 US 837 (1984).

26. Guidance for the public, industry, and CMS staff. National Coverage Determinations with data collection as a condition of coverage: coverage with evidence development. July 12, 2006. https://www.cms.gov/medicare/coverage/coverage-with-evidence-development

27. Decision Memorandum, Centers for Medicare & Medicaid Services. Positron Emission Tomography (FDG) and Other Neuroimaging Devices for Suspected Dementia. September 15, 2004. Administrative File: CAG #00088R. Accessed June 22, 2023. www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=N&NCAId=104

28. Guidance for the Public. Industry, and CMS Staff: Coverage with Evidence Development. Centers for Medicare & Medicaid Services. November 20, 2014. Accessed June 22, 2023. https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?MCDId=27

29. Zeitler EP, Gilstrap LG. Coverage with Evidence Development: Where Are We Now? *Am. J. Managed Care*. 2022. Accessed June 22, 2023. https://www.ajmc.com/view/coverage-with-evidence-development-where-are-we-now-

30. Social Security Act Sections 1862(a)(1)(A) and (E) (42 USC 1395y).

31. *Kort v Burwell*, 209 F.Supp.3d 98, 101 (D.D.C., 2016).

32. Reconciling National Coverage Determinations on Positron Emission Tomography (PET) Neuroimaging for Dementia Based on *Kort v Burwell* Court Order of July 19, 2016. Accessed June 22, 2023. https://www.federalregister.gov/documents/2018/04/11/2018-07410/medicare-program-reconciling-national-coverage-determinations-on-positron-emission-tomography-pet

33. Chambers JD, May KE, Neumann PJ. Medicare covers the majority of FDA-approved devices and Part B drugs, but restrictions and discrepancies remain. *Health Aff (Millwood)*. 2013;32(6):1109-1115. doi:10.1377/hlthaff.2012.1073

34. Cohrs R, Parker JE. Will Biogen's new Alzheimer's drug's burden on Medicare be big, huge, or catastrophic? STAT News, (June 28, 2021). Accessed June 22, 2023. https://www.statnews.com/2021/06/28/biogens-alzheimers-drug-medicare-huge-or-catastrophic/

35. Fox J. The Hidden Role of Cost: Medicare decisions, transparency and public trust. *U Cin L Rev*. 2010;(79):1-52.

36. Gyawali B, Hey SP, Kesselheim AS. Assessment of the clinical benefit of cancer drugs receiving accelerated approval. *JAMA Intern Med*. 2019;179(7):906-913. doi:10.1001/jamainternmed.2019.0462

37. Kim C, Prasad V. Cancer drugs approved on the basis of a surrogate end point and subsequent overall survival: an analysis of 5 years of US food and drug administration approvals. *JAMA Intern Med*. 2015;175(12):1992-1994. doi:10.1001/jamainternmed.2015.5868

38. Daval CJR, Bendicksen L, Kesselheim AS. Eroding judicial deference to the FDA–consequences for public health. *N Engl J Med*. 2023;388(11):963-966. doi:10.1056/NEJMp2215193