HEALTH CARE POLICY AND LAW

Statutory Authority for Medicare Coverage Decisions—
CMS Is an Independent Federal Agency

Francis X. Crosson, MD; Rita F. Redberg, MD, MSc

It is time for the public to recognize and strongly support the authority of the US Centers for Medicare & Medicaid Services (CMS) to make reasonable and necessary coverage decisions for Medicare beneficiaries and for Congress to codify that authority and specifically include drug and device costs as part of the reasonable determination. At present, although CMS has authority to make reasonable and necessary coverage decisions, the standard for determining which medical products are reasonable and necessary is unclear and subject to political haggling. Particularly fraught are 2 questions. First, is US Food and Drug Administration (FDA) approval sufficient to meet the CMS’s statutory requirement of reasonable and necessary? Second, should CMS consider for coverage fast-track and accelerated approval differently than the standard FDA approval?

In this issue of JAMA Internal Medicine, authors (and legal scholars) Daval and Kesselheim1 illuminate these complex issues by outlining the legal bases for, and limitations on, CMS’s ability to limit taxpayer coverage of FDA-approved drugs and other products. There is wide concern that pressure from the pharmaceutical and medical device industries and industry-supported disease advocacy organizations may be leading the FDA to approve drugs and devices that have little or no evidence in establishing clinical benefit. Daval and Kesselheim show that CMS has ample authority to refuse or limit coverage of these products within its mandate to cover only goods and services that are reasonable and necessary for treating Medicare beneficiaries.2

Daval and Kesselheim1 review the history of CMS’s efforts to develop tools to apply the statutory reasonable and necessary criteria to Medicare coverage, including national coverage determinations and coverage with evidence development (CED), an approach that conditions coverage on the manufacturer conducting further research on safety and efficacy. They describe one promising program from CMS that included drug cost as part of its reasonableness determination, which is called the least costly alternative initiative, that was invalidated in 2008 when challenged in court.3 The authors recommend that Congress strengthen CMS’s authority to include reasonable reimbursement rates for a drug or device as part of the reasonable and necessary standard.

Policy Context
The article from Daval and Kesselheim1 is critically important and timely. The development of biopharmaceuticals, including astonishingly expensive drugs designed to treat common disorders, such as Alzheimer disease, has changed the game for Congress and the Medicare program. For example, after the FDA granted accelerated approval to the Alzheimer drug aducanumab (Aduhelm; Biogen) in 2022, CMS issued a CED decision offering coverage only in the context of CMS-approved clinical trials; without that requirement, the projected cost to Medicare Part B for covering aducanumab would have been an astounding additional $57 billion per year for a drug of dubious clinical benefit and with no plans to obtain meaningful data.4

In January 2023, the FDA granted accelerated approval to another expensive Alzheimer drug with only marginal effectiveness, lecanemab (Leqembi; Biogen and Eisai). In July 2023, the FDA granted full approval based on the pivotal CLARITY-AD study. CLARITY-AD found that patients who received lecanemab deteriorated 0.45 points (on a 19-point scale) less than patients receiving a placebo on the Clinical Dementia Rating: Sum of Boxes scale.5 A clinically meaningful benefit on this scale is 1 to 2 points.6 The cost of lecanemab is estimated to be $26,500 per beneficiary per year. The CMS has signaled it will require registries that will indicate how the drug is being used, but not randomized clinical trials, which inform on how the drug should be used.7 After a fierce lobbying effort by the Alzheimer’s Association and other industry-supported advocacy groups, on July 6, 2023, CMS removed the requirement for meaningful evidence collection for lecanemab.8 Registries have been shown to be almost completely ineffective in identifying and removing dangerous or ineffective drugs (or devices) from the market.9 This lowering of the requirement for meaningful clinical data collection with full approval means that CMS (and thus all of us) will be paying (a lot) for new Alzheimer medications without ever learning whether lives are improved by getting them or if patients are more likely to be harmed.

The rush by FDA to approve lecanemab (and many more like it in the pipeline) with unclear value but clear and substantial fiscal effects poses a serious question: is CMS powerless to prevent taxpayers from footing the bill for and beneficiaries from receiving extremely high-priced drugs and devices of uncertain, marginal, or no proven clinical value? The substantial budgetary effect of CMS’s coverage of FDA-approved drugs pales compared with the potential health risks associated with some drugs with accelerated approval from the FDA. Accelerated approval of potentially toxic drugs (treatment with aducanumab and lecanemab is associated with brain swelling and hemorrhage and death in some patients) may be followed by a public health disaster, because rare but catastrophic adverse effects generally become evident only when a drug is widely used. One case in point was the 2022 delayed withdrawal of the seriously toxic oncology drug,
idelalisib, which had received accelerated FDA approval in 2014.18 Another prominent example of the perils of rushing a drug to approval without evaluating harms was the famous thalidomide disaster in the 1960s. Fortunately, an FDA scientist, Frances Oldham Kelsey, resisted public pressure and refused approval because of inadequate population-based evidence for thalidomide’s safety.19 Within a year it became clear that thalidomide, when used by pregnant persons, produced severe limb deformities in their offspring. As the FDA continues to focus more on speed to market and less on safety and efficacy of drugs and devices, it is more critical than ever that CMS step up safeguarding public health and patient safety by independently evaluating whether medical products are reasonable and necessary for Medicare beneficiaries.

When there is clear clinical benefit and reasonable assurance of safety, it can be advantageous for FDA and CMS to work in tandem on approval and coverage determinations. However, it is important to be mindful that the agencies have different mandates. The FDA’s mandate is to assure the public that new medical products are safe and effective. This is best assured through the use of high-quality, clinical outcome-based evidence. The FDA’s increasing reliance on unproven surrogate outcome markers is of questionable value. Kim and Prasad20 found that among cancer drugs receiving accelerated approval, just 1 in 15 was later shown to be associated with improved survival. The CMS’s mandate is to cover only those goods and services that have been shown to be reasonable and necessary for Medicare beneficiaries. The 2 agencies are guided by different standards, and they should remain so, for the benefit of all.

What Should Be Done?

We agree with Daval and Kesselheim6 that Congress must act, and soon, to uphold CMS’s ability to make drug and device coverage decisions that serve the interests of Medicare beneficiaries and the US population. There are efforts that would accomplish just the opposite, such as the written opposition to CMS’s requirement for CED for Alzheimer drugs by an advocacy group for patients with Alzheimer disease.13 The recent proposal by CMS for greater use of registries is well-intentioned, but inadequate to the task.

Congress should consider 3 actions. First, Congress should expand and codify CMS’s coverage determination authorities, including but not limited to CED. Second, Congress should find that CMS may in certain circumstances include considerations of cost in its reasonable and necessary determinations. There are many examples of how this can be done while protecting the interests of Medicare beneficiaries, including the work of the Institute for Clinical and Economic Review.14 Third, Congress should consider implementing I or more of a series of recommendations from the Medicare Payment Advisory Commission to eliminate perverse financial incentives in Medicare Part B for physicians to choose costly drugs (physicians are paid a percentage of the average sales price to administer such drugs) vs equally or more effective treatments that are less costly.15,16 The time for Congress to act to protect the health and well-being of Medicare beneficiaries and the future of the Medicare program is now.

**ARTICLE INFORMATION**

**Author Affiliations:** Health Systems Science, Kaiser Permanente School of Medicine, Pasadena, California (Crosson); University of California, San Francisco (Redberg).

**Corresponding Author:** Rita F. Redberg, MD, MSc, University of California, San Francisco, 505 Parnassus, MT180, San Francisco, CA 94143-0124 (rita.redberg@ucsf.edu).

**Published Online:** July 28, 2023. doi:10.1001/jamainternmed.2023.4082

**Conflict of Interest Disclosures:** Dr Redberg receives grants from the Arnold Ventures Foundation. No other disclosures were reported.

**REFERENCES**


