VIEWPOINT

From Scientific Discovery to Covered Treatments
Understanding the Payer Perspective as a Keystone to Achieving High-Value Care

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The mission of the National Institutes of Health (NIH) is to translate scientific discovery into health by designing interventions that target the mechanisms of disease.[1] Translational research seeks to move discoveries from the earliest proof of concept through the therapeutic product development pipeline to establish safety, efficacy, and effectiveness. Approval of novel medications or devices by the US Food and Drug Administration (FDA) is a critical milestone in translating scientific discovery to clinical care. However, requirements to fulfill insurance coverage are often not considered until after FDA submission and may be even more challenging for behavioral interventions that extend beyond FDA authority. Recent discussions about drug pricing, inability of some patients to access indispensable medications, and pervasive health inequities have brought to the forefront the role of insurance coverage in the treatment development pipeline.[2,3]

This Viewpoint outlines some of the coverage requirements of Medicare,[3] the largest payer for health care services in the US, as an exemplar of the nuances of this key translation pipeline step. A better understanding of this process could help develop research programs, including those funded by NIH, that would generate evidence that better meets evidentiary requirements and that translate scientific discoveries into care and services that improve the health of individuals and populations. It may also stimulate interest in understanding the coverage determination procedures of other large payers. Research efforts to generate evidence that meets payer requirements (ie, relevance to covered populations, improvements in health outcomes) through design and methodological clarity could accelerate the translation of scientific discoveries into clinical care and reduce health inequities.

In contrast to the FDA, which generally determines whether devices or medications are safe and effective, Medicare coverage authority is based on determining whether there is sufficient evidence that a treatment or service is reasonable and necessary to diagnose or treat an illness or injury, and often focuses on the Medicare beneficiary population.[3] Evidence sources may include clinical trials and observational studies. The Centers for Medicare & Medicaid Services (CMS) makes the final determination in the case of national coverage determinations, whereas Medicare administrative contractors serving the local geographic jurisdiction are authorized to develop local coverage determinations under the statutory standards. Local coverage determination may not conflict with a national coverage determination.

To aid in making national coverage determinations, CMS can, but is not required to, commission a technology assessment through the Agency for Healthcare Research and Quality, or convene the Medicare Evidence Development and Coverage Advisory Committee to review and consider the sufficiency of the existing evidence. In the case of local coverage determinations, the local Medicare administrative contractors issue local coverage determinations to provide the evidence-based coverage criteria and follow a process that includes opportunity for public comment and may convene a contractor advisory committee to aid in making determinations. If existing evidence suggests that the treatment or service is promising but not reasonable and necessary, Medicare may elect through a national coverage determination to provide limited coverage as additional evidence is accrued ("coverage with evidence development") in approved implementation studies. Coverage in the context of ongoing clinical research protocols or with additional data collection can expedite earlier beneficiary access to innovative technology while ensuring that systematic patient safeguards, including assurance that the technology is provided to clinically appropriate patients, are in place to reduce the risks inherent to new technologies or inherent to new applications of older technologies.

For Medicare, national coverage determinations are largely dependent on the evidentiary strength and relevance to the Medicare beneficiary population. A study of 173 unique national coverage determinations made between 1999 and 2013 found that 7 (4%) resulted in full coverage of the intervention, 123 (71%) resulted in coverage of the intervention for Medicare beneficiaries meeting particular conditions, 16 (9%) resulted in CMS deferring coverage to regional Medicare administrative contractors, and 27 (16%) resulted in no coverage.[4] Another review of national coverage determinations between 2005 and 2016 that considered high-risk medical devices, moderate-risk medical devices, pharmaceuticals, or biologics reported that of 11 national coverage determinations, 8 (73%) were covered, including 3 (38%) that were covered with evidence development; the remaining 3 were not covered.[5]

There are several reasons that coverage requests are denied or delayed, despite FDA approval of a device or treatment.[3-5] Studies may have sufficient evidence to meet safety and efficacy requirements for FDA approval but may not have included research participants representative of the Medicare beneficiary population (typically ≥65 years) or with prevalent comorbidities. This leaves an evidentiary gap in determining if, and for whom, a device or treatment is clinically useful or potentially harmful, which is an important consideration for Medicare coverage. For example, studies on acupuncture effectiveness for chronic low back pain initially included few patients 65 years or older, leading to a proposed national coverage determination to provide Medicare coverage only in certain qualified clinical trials. However, after considering public comments and because of the opioid public health emergency and because

younger patients with chronic low back pain showed improvements in function and pain, CMS made a positive coverage determination that took into account an assessment of benefits and harms. Medicare also noted that although there was variation in covered indications and frequency of services, a number of large private payers provide some coverage of acupuncture for certain indications.[5]

Importantly, in addition to meeting evidentiary requirements, a treatment or service must fit into a benefit category (such as a device, physician service, or durable medical equipment) to lead to a positive national coverage determination. Approaches such as needle exchange programs or certain digital therapeutic interventions, even if efficacious, may not fit into an existing benefit category. For example, Medicare coverage of contingency management, an effective intervention for stimulant use disorders,[6] will be similarly challenging, unless defined as a treatment or service in a benefit category. It is also important to understand that a national coverage determination is not dependent on the cost of the intervention and does not set the payment level.

However, studies are seldom designed with the goal of collecting the information that is needed for insurance or payer policy purposes. In the case of Medicare coverage, prior to submitting a formal request for a national coverage determination of a treatment or service under Medicare Part A or B, potential requesters may (although are not required to) communicate with CMS staff in the Coverage and Analysis Group within the Center for Clinical Standards and Quality. This allows for the identification of additional information that might be needed or helpful in the request, including the potential benefit category. Preliminary discussions may also include consideration of study designs and protocols whose results will be later submitted to support the request, if relevant.

A better understanding of the payer perspective could facilitate achieving higher-value service delivery at multiple levels of the health care system. At the population level, improved understanding of coverage determinations could encourage the conduct of studies that support or refute the use of specific items or services in specific populations, and thus minimize risk and optimize benefits to specific populations. For example, clinical trials often exclude older adults and populations enriched with comorbidities for safety and efficacy reasons.[5,7] Yet, clinical trials and other research that do not sufficiently recruit and enroll study participants who are representative of racial, geographic, and other key factors relevant to the people whose lives are covered may limit confidence that the intervention improves health outcomes equitably.

From the perspective of clinicians and patients, research aligned with coverage and payment requirements could help eliminate the perception of arbitrariness that sometimes arises regarding treatment decisions and reimbursement[8] and could help inform discussions of risks vs benefits as part of clinical decision-making by patients and their clinicians. Furthermore, the evidence generated to address coverage requirements could better bridge discovery to coverage and eliminate the research-to-practice gap or access delay. For health systems, addressing coverage evidentiary requirements could support prioritization of services for specific populations and facilitate processes to further support high-value over low-value care. Moreover, this same evidentiary throughput can also extend support to prior-authorization policies applicable to treatments and services when details are available, such as indications for specific subpopulations, frequency, duration, and end points.

In addition, research studies with expanded eligibility criteria and intentional recruitment to address social determinants of health would inform coverage determination by payers and could enhance access to needed treatments and services that would otherwise not be covered without that evidence.[9] Enhanced access to behavioral and mental health treatments and services are critical to eliminating disparate health outcomes. It is also necessary to delineate what health outcomes are meaningful and measurable in the population, which in the case of behavioral health issues include misuse of substances and related unplanned care or death. Attending to these issues in the design of pilot, efficacy, and effectiveness studies may enhance the likelihood of a positive coverage determination and thus accelerate translation from discovery to clinical care.

In summary, translating research into overall and behavioral health is a complex endeavor that requires attention and planning for each step of the process. Attention to the evidentiary requirements from the payer perspective from the early phases of the translation pipeline could lead to a better alignment between research and treatment access and improve outcomes efficiently, effectively, and equitably.

ARTICLE INFORMATION

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