

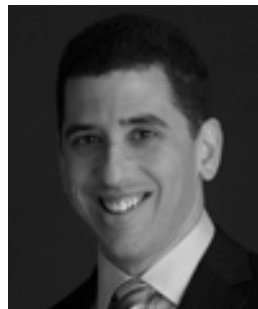


MEDICARE REPORT



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Is Provenge a Harbinger for Future CMS Decision Making?



By **SUNG HEE CHOE**, **TANISHA CARINO**
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Advances and innovations in medical science have allowed health care providers to prevent, diagnose and treat illness in ways that were previously impossible. Health technologies have extended life expectancy, improved quality of life, and increased productivity.

New technologies, however, pose a dilemma to public and private payers, which must reconcile the demands for access to these technologies with financial

realities of the current health care system. The Medicare program is no different and, as is well known, is headed towards insolvency.

As the gatekeeper to the Medicare program, the Centers for Medicare & Medicaid Services has long attracted public scrutiny. CMS is obligated to provide coverage to Medicare beneficiaries for health care services and items that are “reasonable and necessary,” an authority provided in Section 1862(a)1(A) of the Social Security Act.

CMS’s implementation of this legislative mandate, specifically its interpretation of what constitutes “reasonable and necessary,” has often been the subject of criticism and controversy. More recently, heightened sensitivity to ‘government rationing’ of health care has aroused additional concerns about how the agency fulfills its mandate.

This article explores CMS’s national coverage analysis (NCA) on the prostate cancer drug Provenge as a case study to determine how CMS decision-making may evolve in the future given mounting pressures on the program. It concludes by discussing potential strategic responses by innovator companies.

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Medicare's Decision-Making Process

First, some background on the Medicare decision-making process.

Medicare coverage decisions are made by both CMS' national office and by local Medicare contractors. National coverage decisions are made by CMS' Coverage and Analysis Group (CAG) in the Office of Clinical Standards and Quality.

A national coverage determination (NCD) issued by this group sets one national Medicare policy for a healthcare item or service and is binding on all local Medicare contractors. In the absence of an NCD, local Medicare contractors may develop a local coverage determination (LCD).

Coverage reviews conducted at the national level are generally reserved for more controversial, high-volume, and/or expensive procedures. As a result, the vast majority of Medicare coverage decisions are deferred to local contractors.

To inform its coverage decision, CMS may commission the Agency for Healthcare Research and Quality (AHRQ) to conduct a technology assessment and/or convene a Medicare Evidence Development & Coverage Advisory Committee (MEDCAC) meeting during which evidence is reviewed by a panel of independent experts from the healthcare field.

Potential outcomes from an NCD process are myriad, ranging from complete coverage to non-coverage. NCDs are rarely this cut and dry, however. Most decisions fall in between this range, often calling for coverage with certain conditions.

Burden on the Medicare Program of New Drugs

Drugs have historically not been the focus of NCDs. Avalere's analysis of NCDs completed during 2000-2010 reveals that only 7 percent of NCDs during that period were on drugs (only health education/behavioral interventions represented fewer NCDs during that period at 5 percent). By contrast, nearly 25 percent of NCDs over this period were on medical procedures and 16 percent on devices.¹

According to a recent study published by Avalere Health,² the Medicare program spent approximately \$15 billion on outpatient drugs under Medicare Part B in 2010. By 2019, spending on outpatient Medicare Part B drugs is expected to reach \$28 billion, representing 7.5 percent annual growth.

Given the heavy cost burden of Part B drugs on the Medicare program, it is not a big leap to assume that Medicare Part B drugs have piqued the interest of CMS' coverage group. In fact, during 2010, CMS opened NCDs on two high-profile drugs: erythropoiesis-stimulating agents to manage anemia in patients who have chronic kidney disease and sipuleucel-T (more familiarly known as Provenge).

Provenge

In June 2010, CMS opened an NCA on Provenge, a treatment for advanced prostate cancer, only two months following its FDA approval. CMS's reference to

informal inquiries received for an NCD on Provenge left experts speculating on the subject of those informal inquiries, chief among them, Provenge's \$93,000 price tag.

CMS' action on Provenge became one of the agency's most anticipated national coverage decisions. In its proposed decision memo issued on March 30, 2011, CMS proposed to cover the drug for its FDA-approved indication: asymptomatic or minimally symptomatic metastatic castration-resistant prostate cancer.

Contrary to the speculation of many analysts, CMS also chose not to make a uniform national coverage determination on whether to restrict Medicare coverage of Provenge for patients who fall outside its FDA-approved label. Instead, CMS left coverage of off-label use of Provenge to the discretion of local contractors.

But a closer read of the decision revealed some interesting and important policy choices by CMS. In particular, CMS' decision signals to its local contractors that it should implement non-coverage. CMS notes in its decision memo that it found no evidence from Phase III clinical trials designed to evaluate the health outcomes associated with Provenge for off-label uses, characterizing the evidence base as "virtually nil."³ CMS' support for this finding is the manufacturer's own acknowledgement that no such data currently exists.

CMS explicitly instructs its local contractors to judge the evidence, particularly new evidence that may develop on Provenge over time. By relying on its contractors to judge the evidence, CMS has innovated a new way to use the flexibility of the regional system, while still pursuing its interest in shining the spotlight on new, costly technologies through the national coverage process.

In its decision, the agency sets the expectation that its local contractors follow evidence-based care and restrict the off-label use of Provenge. Given this expectation, local contractors will have few incentives to allocate already limited resources and capacity to make coverage decisions on the off-label use of Provenge.

In fact, a review of existing local coverage policies on Provenge by Avalere shows that over 50 percent of contractors already limit coverage to on-label use. Unless new evidence emerges to the contrary, the number of contractors that restrict coverage of off-label use is likely to increase.

CMS also sends a clear message to Dendreon, the manufacturer of Provenge, that it hopes "unlabeled uses . . . will take place in the context of *bona fide* clinical studies [emphasis added]."

This sets a new—and somewhat ambiguous—bar for manufacturers to ensure that they are actively pursuing additional research studies to demonstrate benefit in additional indications. Further clarification of this standard will clearly be necessary for CMS to ensure that the resulting study outputs will be useful for future decisions on coverage of off-label uses by the local contractors.

Strategic Imperatives for Innovator Companies

While CMS's decision making is a well-established process today, it is likely to evolve in the future. Increasing collaboration with FDA and the emergence of

¹ Avalere Health analysis of Tufts Medical Center Medicare National Coverage Determinations (NCDs) Database

² *Federal Spending on Brand Pharmaceuticals*. Avalere Health. April 2011. <http://avalerehealth.net/wm/show.php?c=1&id=880>

³ Proposed Decision Memo for Autologous Cellular Immunotherapy Treatment of Metastatic Prostate Cancer (CAG-00422N). March 30 2011.

comparative effectiveness research (CER) are two significant trends that are likely to alter—and perhaps confound—the mechanics of the process.

Although CMS recently filled the Chief Medical Officer position, medical and evidence evaluation resources in the agency are stretched thin, hampering its ability to run an aggressive and sustained campaign around technology assessment at the federal level. Thus, CMS is more likely to take an incremental approach by continuing to send signals to the market to promote the changes it seeks.

To respond to this new environment, there are several steps innovator companies can take:

First, plan for greater scrutiny by CMS for pricier interventions earlier in a product's launch. While CMS is prohibited from making a coverage decision based on cost, it is not prohibited from using cost as a rationale for opening an NCD. Medicare clearly cares about technologies that will be widely used in the program, particularly those that could have significant budget implications.

For example, prostate cancer's prevalence in the elderly male population makes it likely that any new therapy that comes onto the market in this area is something CMS will seek to better understand. In the case of Provenge, CMS demonstrated that it will not necessarily wait to see how utilization patterns develop before initiating an NCD.

Second, work with the agency to better understand evidentiary hurdles. The bar on evidence is likely to head higher. CMS is seeking to promote the development of evidence that directly addresses its needs. To facilitate evidence collection of new interventions, CMS has readily exercised its coverage with evidence devel-

opment (CED) authority—in the last three years, CMS has applied CED to nearly 25 percent of NCDs; CED was applied in fewer than 10 percent of NCDs during the period 2000-2007.⁴ Intensifying CMS-FDA collaboration, such as the development of a parallel review process, could be another tool by which CMS can further encourage the generation of evidence that better meet its needs.

Finally, focus on local contractor decision making. As the national conversation on health care costs continues, the specter of 'government rationing' is likely to persist.

If CMS feels restricted in its ability to implement evidence-based coverage policies at the national level, it may increasingly look to its local contractors as partners in the application of evidence standards. Understanding local contractors, their process, capabilities, and frequency of policy changes will become increasingly important.

Conclusion

Innovator companies are rising to the call to demonstrate the value of its products within the health care system. They are generating evidence to support the safety, efficacy, and effectiveness of their products that accommodates the needs of payers across the globe.

As these data become available, payers will likewise need to rise to the call of making valuable technologies accessible to the beneficiaries who may benefit from them.

⁴ *Use of CER to Guide Medicare Coverage & Payment Decisions*. Avalere Health audio conference. April 28, 2011. <http://www.avalerehealth.net/conferences/mih-cer/>