A Prescription for Savings: Reducing Drug Costs to Medicare

Special Committee on Aging
United States Senate

Testimony of:

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July 21, 2011

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Introduction

Mr. Chairman, Senator Corker, and other Senators of the committee, thank you for the invitation to appear before the committee today. The issues being discussed today are of central importance to the Medicare program and more generally to public health. The Medicare program can almost certainly spend less on drugs without any negative impact on health outcomes for Medicare beneficiaries. In my view, there are at least three important activities that can be pursued by the Medicare program to spend less and achieve the same or better health outcomes:

1) Medicare should have the authority to link drug prices more directly to health outcomes;
2) Medicare should implement additional policies to promote high priority clinical research; and
3) Medicare should develop a systematic policy approach to promoting health technology innovation.

Health care technologies, including drugs, devices, procedures, diagnostics and other services, receive a great amount of attention in discussions of health care costs because they are generally viewed as being responsible for up to 50% of the increase in health spending over time[i]. Along with the frequently cited estimate that up to 30% of health care services are unnecessary or of unknown effectiveness[ii], it is reasonable to conclude that substantial savings could be achieved simply by reducing payments for services that are not known to improve health outcomes.

In practice, it is often very difficult to know with a high level of certainty which drugs, devices or procedures are most effective, because the studies needed to answer those questions have never been done or are of poor quality. A recent report looking at the strength of scientific evidence supporting clinical recommendations on treatment of six common heart diseases by the American College of Cardiology and the American Heart Association showed that only about 10% of these recommendations were supported by high quality clinical studies[iii]. The number of situations in which compelling evidence is available to compare the benefits, risks and costs of alternative diagnostics and treatment approaches is limited, in part because high quality comparative studies are rarely done.

As Chief Medical Officer for the Medicare program, I was consistently struck by how much uncertainty existed about the relative benefits, harms and costs of widely used health technologies. Decisions about what to pay for and how much to pay were made substantially more difficult by these gaps in knowledge. Despite this uncertainty, there were plenty of examples where it seemed possible for Medicare to spend less for certain drugs and devices without any possibility of harm to Medicare beneficiaries. In some cases, one could be confident that it was possible to reduce drug costs to Medicare, without any sacrifice of health

outcomes for Medicare patients. For reasons discussed below, the pathway to achieving those savings was far from easy, and would be considerably advanced with several specific policy changes.

I recognize that the approaches to reducing Medicare drug spending being discussed today will not by themselves produce dramatic spending reductions in Medicare. More fundamental payment reforms and delivery system innovations will be required to ensure the long term solvency of the Medicare program. However, it would still seem worthwhile to consider changes to coverage and payment policy that could potentially reduce federal spending by 100 million dollars or substantially more per year in treating specific medical conditions, particularly when those savings could be achieved with the same or better health outcomes.

Medicare should have authority to link drug prices to health outcomes

One relative straightforward approach to reducing Medicare spending on drugs without negatively affecting patient health would be to restore the Agency's authority to pay the same price for drugs that produce similar benefits and harms.

Medicare does not generally attempt to factor a service's relative effectiveness or its cost relative to alternatives in setting prices for covered items and services[iv]. However, Medicare's regional contractors have been adjusting prices highly selectively based on clinical effectiveness evidence for more than 15 years. This has been through their use of a least costly alternative (LCA) policy for certain types of items, including durable medical equipment and Part B drugs. The policy's rationale is that Medicare, beneficiaries, and taxpayers should not pay more for a service when a similar service can be used to treat the same condition and produce the same outcome at lower cost[v].

Using this reference pricing approach, Medicare contractors have not paid the added cost of a more expensive service if a clinically comparable one exists in particular categories of items and services. Examples include manual wheelchairs, power mobility devices, seat lift mechanisms, supplies for tracheostomy care, and anti-androgen drugs for patients with advanced prostate cancer. Beneficiaries are allowed to obtain the more costly item if they choose to pay the difference between the approved payment amount for the reference item and the amount for the one they choose.

There is no statutory provision giving specific authority or prohibiting the application of LCA. CMS has considered its "reasonable and necessary" statutory language[vi] to provide the needed authority to adopt this approach for equivalent drugs and equipment. However, a recent court decision constrains Medicare's current use of LCA determinations. In a case involving LCA for inhalation drugs for asthma, the Court found that the pricing formula for part B drugs specified in the Medicare Modernization Act did not allow Medicare the flexibility to apply the LCA approach to drug pricing, and the Agency and its contractors have not pursued the approach since that court decision[vii].

In 2003, the Medicare program used a different legal authority to achieve comparable pricing for two similar drugs, Aranesp and Procrit, that were both approved to treat anemia caused by cancer chemotherapy. Because these drugs were very similar in their molecular structure, worked through the same biological mechanism, and had similar benefits and risks, Medicare decided to determine the price to be paid for Aranesp based on the price that was paid for Procrit, which resulted in a much lower level of payment for Aranesp than would have been determined through the standard methods of using average wholesale price. Medicare described these two drugs as being "functionally equivalent" to one another, and an existing payment authority, called "equitable adjustment" was used to establish the price.

This simple sounding idea turned out to be technically difficult, particularly the analysis required to determine the amount of each drug that produced the same improvement in the severity of anemia. There were few head to head studies of these two drugs, which significantly impaired the ability to calculate clinically equivalent dosing. The effect of this policy was estimated to have reduced Medicare spending by as much as $150 million dollars per year, without any negative impact on the health of cancer patients. The underlying logic of linking the price paid to the health benefits produced has intuitive appeal if one's intention is to buy as much health as possible with whatever resources are available, and ideally not to pay more than necessary to obtain a given amount of health benefit.

The basic premise is that we are generally inclined to pay similar prices for products and services that provide similar benefits. This is a fairly standard approach that consumers take to making purchasing decisions, and is the central premise behind the concept of value-based purchasing. We pay a higher price for things that provide more benefit, and less for items that offer less value. In the case of health care, we generally want to recognize better outcomes by paying more for them. That is why Medicare and private health plans are moving in the direction of tying the payments directed to clinicians, hospitals and other providers to the quality of the care that they provide. The same notion that motivates linking provider payments to outcomes can be applied to the prices paid for drugs, devices, procedures and other services. When health outcomes are better, prices are higher; when they are the same, the payment level is the same.

Clearly there are some reasonable concerns about the approach, including the possibility of reduced incentives for investment in new drug development and the challenges inherent in arriving at a definitive conclusion about equivalence of benefits and harms. There are also down sides to retaining a payment system that provides financial rewards for "newness" alone – in that the signal communicated to the product development community is that meeting the current standard of care will be financially rewarded. The signal that would ideally be sent is to guarantee significant rewards for substantial improvements over current therapy. Further discussion of the role of Medicare with respect to biomedical innovation appears below.

Medicare should implement additional policies to promote clinical research

The CATT results underscore the importance to Medicare of having the capacity to rapidly identify, design, and implement clinical trials on questions of substantial importance to the Medicare program. Current research and payment policies create substantial challenges to consistently and efficiently conducting these studies. The successful implementation of the CATT required extraordinary and persistent efforts by a number of individuals and organizations, including the American Academy of Ophthalmology, the National Eye Institute, CMS and others to overcome substantial barriers and delays in executing the study. Senator Kohl was instrument in addressing challenges with handling co-pays for patients enrolled in the trial, and helped to craft language addressing this problem in the Medicare Improvements for Patients and Providers Act of 2008. This law provided new authorities to the Secretary of Health and Human Services to develop alternative payment mechanisms that would reduce reimbursement barriers to future comparative trials, and it would be helpful for CMS to outline the process through which this new authority will be implemented[viii]. A more reliable, less burdensome approach will be essential to ensure that we efficiently learn how best to put new discoveries into clinical use to achieve good clinical outcomes.

There is a critical need to produce critical information for patients, consumers, clinicians, payers, particularly in the context of payment reforms and new insurance benefit designs that will increase the reliance on clinicians and patients to weight the benefits, risks and costs of alternative health care choices. Faced with these difficult choices, these decision makers are going to need credible, unbiased information on which treatments work best, and at the lowest available cost. Judging from past experience, credible and relevant evidence will frequently be unavailable[ix].

Proton beam therapy for prostate cancer[x] and vertebroplasty and kyphoplasty for vertebral compression fracture[xi] are examples of widely used technologies for common clinical problems for which Medicare spends hundreds of millions of dollars, with little or no evidence that these treatments are better than inexpensive conservative therapies. Over $900 million was spent in the US in 2008 on surgical procedures for vertebral compression fractures despite the recent clinical guidelines from the Academy of Orthopedic Surgery which noted strong evidence against the use of vertebroplasty and weak evidence to inform recommendation about the use of kyphoplasty.

To ensure that Medicare dollars are directed to clinical services that are likely to improve the health of Medicare beneficiaries, the Medicare program will need to become considerably more active in ensuring that adequate studies are conducted on interventions that are widely used in their covered populations. Collaborative relationships with the Agency for Health Care Research and Quality, National Institutes of Health, and Patient-Centered Outcomes Research Institute will need to be strengthened, ideally with direction from a well defined list of clinical research priorities reflecting the perspectives of the Medicare program.

Medicare can also promote critical research by making more systematic use of coverage with evidence development (CED). Coverage with Evidence Development (CED) is a policy tool that

links coverage of a technology with a requirement that patients receiving the service are enrolled in prospective clinical studies designed to inform future revisions to the coverage decision. The term was coined specifically for Medicare[xii], but is now part of a growing array of options for insurers to share in the costs of data collection in order to support their collective interest in reducing uncertainty when making coverage decisions[xiii]. Under CED, Medicare reimbursement is contingent on a beneficiary's participation in a clinical study as part of a systematic data gathering exercise.

CED remains a promising idea, for which the implementation has been done with great diligence, but there are still few unequivocally successful examples of CED leading to the generation of the type of relevant and reliable evidence originally envisioned. Although Medicare has applied CED in more than a dozen national coverage decisions in the last 15 years, data from the resultant studies have been used for policy in only two cases: for lung volume reduction surgery to treat late-stage emphysema in 2003 and the use of positron emission tomography (PET) for cancer in 2009. In both cases, Medicare made positive coverage policies that have been viewed as more permissive than was justified by the evidence generated from the studies[xiv,xv,xvi].In many other cases, appropriate studies were never designed, funded, or implemented, for a variety of reasons. In short, the promise of CED as a mechanism to support clinical research on urgent topics has not yet been realized.

Improving Medicare's use of CED will require explicit statutory authority for the Agency to apply this policy mechanism, as past efforts have had limited success largely due to the ambiguity of its legislative authority. Although CMS has issued guidance attempting to clarify the authorities for CED[xvii], each application has involved much internal legal debate. Without a clear legal mandate to pursue CED, CMS' efforts have, again, by necessity been *ad hoc* – with no formal process for selecting appropriate topics, little learning from one initiative to the next, limited resources and lack of dedicated staff skilled in navigating the political and operational issues raised by CED, including CMS' ability to require provider and supplier compliance with CED reporting requirements. This experience has dampened CMS' enthusiasm for pursuing this policy tool, as it requires considerable staff time and resources just to get approval, resulting in the failure to apply the policy for technologies which would most benefit from additional study. A series of steps through which Medicare can improve the implementation of CED is outlined in the most recent MedPAC annual report[xviii] and in the background paper on this topic prepared by the Center for Medical Technology Policy at the request of MedPAC[xix].

Medicare should develop a systematic policy framework to promote biomedical innovation

Sustaining innovation in the life sciences industry is clearly an important public health priority, and many analysts have made persuasive arguments regarding the health of the biomedical innovation ecosystem in the US. As has been frequently noted, the cost and time required to bring a new drug from initial discovery into clinical use are substantial, and appear to be increasing with time. Policy interventions by Medicare that aim to reduce the prices paid for some new drugs would have the potential to reduce the attractiveness of investments in early

stage products, as the probability and magnitude of large profits can only be reduced in this context, particularly for new drugs and other technologies that do not impart significant new clinical benefits.

To my knowledge, the goal of promoting biomedical innovation has never been an explicit objective in the strategic plan for the Medicare program. The currently stated mission on the Medicare web site states that the program is: "to ensure effective, up-to-date health care coverage and to promote quality care for beneficiaries." And to the extent that promoting innovation is a high priority for the Department of Health and Human Services, little or no attention has been focused on the role that the Medicare program might play in achieving that objective.

Given the current size of the Medicare program and the dominant impact that is has on the market for drugs and devices in the US and globally, it is impossible for Medicare to avoid having an impact on innovation, whether or not it is an explicit programmatic goal. It would therefore be important for the Medicare program to seriously and systematically consider the impact of various Medicare policies on innovation, much as was done by the FDA in their 2004 report: Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products[xx]. This white paper describes the concerns about the impact of regulatory policy on innovation, and outlined a number of policy tools and strategies that could be pursued by the FDA, along with private sector partners, to promote innovation while continuing to fulfill their primary institutional mission of ensuring that medical products are safe and effective. The FDA report concludes that the primary barrier to innovation is that the applied sciences for medical product development have not kept pace with biomedical discovery, and that new tools were needed to accelerate product development:

> "A new product development toolkit -- containing powerful new scientific and technical methods such as animal or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical evaluation techniques -- is urgently needed to improve predictability and efficiency along the critical path from laboratory concept to commercial product. We need superior product development science to address these challenges -- to ensure that basic discoveries turn into new and better medical treatments. We need to make the effort required to create better tools for developing medical technologies. And we need a knowledge base built not just on ideas from biomedical research, but on reliable insights into the pathway to patients."

The FDA has devoted substantial staff time and resources over the past 7 years to advancing the applied sciences for product development in the life sciences, working closely with private sector partners. The Critical Path Institute, a private, non-profit founded and led Dr. Ray Woosley, and supported in part by the FDA, has assembled a highly skilled staff of 55 scientists and clinicians to support pre-competitive collaboration in the life sciences industry to help develop the new product development science called for by the FDA. Many other professionals and organizations have also focused on carrying out the FDA's strategic vision.

It would be extremely valuable for the Medicare program to take a similarly comprehensive and structured look at the relationship between Medicare policy and biomedical innovation, and then carefully consider the full range of Medicare policy mechanisms through which Medicare innovation could be sustained at optimal levels. This is particularly critical as the Medicare program continues to face strong spending pressures, and is likely to undergo significant reforms in payment policy and benefit design that may have important impact on innovation.

In the absence of a thorough assessment of the range of Medicare policy levers that may impact innovation, it is unclear whether the optimal response to the recognized concerns about biomedical innovation are best addressed by retaining current pricing mechanisms that are linked to average sales prices rather than to clinical evidence of comparative benefits and harms. The argument that Medicare should continue to pay more for new drugs as a mechanism to promote innovation should be considered with the benefit of a more comprehensive framework that considers a full range of alternative policy mechanisms through which Medicare might promote innovation.

Recognizing the important impact of health care reimbursement on innovation, the National Institutes of Health and Clinical Excellence (NICE) commissioned a report to examine how their analytic approach to new drugs might better reflect the value of innovation. The final report, Appraising the Value of Innovation and Other Benefits[xxi], provided 25 specific recommendations to NICE and the National Health Service about how their decision making processes could be made more sensitive to biomedical innovation. A similar assessment of Medicare reimbursement policies and biomedical innovation would be extremely valuable.

An initial effort to provide an assessment of specific Medicare policies that could promote innovation was taken on by the National Venture Capital Association (NVCA) in their 2007 report: Proposal for a Reimbursement Critical Path for CMS[xxii]. This report paper provided 8 specific recommendations for changes in CMS policies and procedures that were viewed as having potential to promote innovation. Some examples of these recommendations included:

- Develop clear process descriptions through which new technologies achieve coding, coverage and payment
- Establish specific timelines for all phases of the reimbursement process
- Clarify evidence requirements necessary to obtain new technology add-on payments and to quality for a separate Medicare billing code
- Undertake a thorough review of the process through which new CPT-coded are assigned to new technologies
- Explore approaches to parallel review and approval by the FDA and CMS

CMS should more explicitly recognize the critical impact of the Medicare program on innovation, and systematically evaluate policy strategies to sustain and promote innovation, analogous to the FDA's efforts to identify and address barriers to innovation from their perspective as a regulator. As with the FDA, a serious effort by Medicare to develop a policy strategy and the applied reimbursement science needed to promote innovation will require

sustained attention and resources, and will likely require collaboration with a range of public and private experts and stakeholders.

Conclusion

The issues raised in today's hearing are of critical importance to the Medicare program and the health of the American public. The challenge of reducing drug costs to Medicare raises broader issues of how best to ensure that Medicare spends money on technologies and services that provide the most possible benefit to beneficiaries. Responding intelligently to this challenge becomes more urgent as health care cost trends inevitably promote greater downward pressure on spending. In the specific context of drugs and other medical technologies, we believe that there would be value in further exploring three policy strategies: 1) Medicare should be given authority to link drug prices more directly to health outcomes; 2) Medicare should implement additional policies to promote high priority clinical research; and 3) Medicare should develop a systematic policy approach to promoting health technology innovation.

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