ONLINE APPENDIXES

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Enhancing Medicare's ability to innovate

ONLINE APPENDIX 1-A

Some issues associated with implementing reference pricing, performance-based risk-sharing strategies, and coverage with evidence development

In this appendix, we discuss some of the issues the Secretary and the CMS administrator would need to consider if they were given more flexibility to use reference pricing, performance-based risk-sharing strategies, and coverage with evidence development (CED).

Implementing reference pricing strategies

If the statute gave Medicare more flexibility to use reference pricing policies under Part A and Part B, a number of issues would need to be considered, including: (1) defining groups of products or services that are clinically similar, (2) addressing different units and frequency of administration of clinically similar services, (3) calculating and updating the reference price, and (4) developing an exceptions process.

Defining groups of products or services that are clinically similar

A key issue for all reference pricing schemes is defining which products and services should be considered clinically similar and, on that basis, be assigned the same price. With drugs used as an example, a group could be narrowly defined to include an innovator drug and its generics. Alternatively, a group could be more broadly defined based on drugs' pharmacologic equivalence. For example, such a group might consist of the different drugs used to treat prostate cancer. Medicare's contractors have applied least costly alternative (LCA) policies to these drugs after concluding that there was no difference in clinical efficacy among them (National Government Services 2009b).[1]

Similar decisions would need to be reached when grouping nondrug products, such as medical supplies and devices. For example, one Medicare contractor applies a LCA determination to cover wheelchairs, grouping six products as similar and paying all six at the rate for the LCA unless specific medical criteria are met (National Government Services 2009c).

Standardizing units and frequency of administration of clinically similar services

If reference pricing policies group products and services that are clinically similar but not identical, common units must be defined for the products and services to which the policies apply. Such an issue arose when CMS applied a functional equivalence policy (and set the same payment rate) for epoetin alfa and darbepoetin alfa. Because the two biologics are dosed in different units, CMS developed a factor (conversion ratio), with assistance from the product developers and an independent contractor, to convert units of darbepoetin alfa to epoetin alfa.

In addition to unlike units, clinically similar products and services may not necessarily have the same frequency of administration. The Commission raised this issue in its discussion of the LCA determination for prostate cancer drugs (Medicare Payment Advisory Commission 2007). Some prostate cancer drugs are administered once per year, while others are administered more frequently (monthly or every three, four, or six months). To address the Commission's comments about the different treatment schedules, one Medicare contractor applied a LCA policy for two time periods of drug treatment: 12 months and less than 12 months (National Government Services 2009a).

Calculating and updating the reference price

A key aspect of reference pricing policies is calculating the reference price for each group. One method is to use the LCA. Others include those used internationally (Table 1-A1). For example, in Germany, the reference price is calculated as a price above the lowest third of the market prices of drugs in a therapeutic class (after adjustments for differences in dosing and formulation). Other ways to calculate a reference price include basing it on the median or the drug considered to be the most cost effective within the group. In addition, in establishing the reference price, some countries consider the prices of drugs set by other countries.

The frequency of updating the reference price would need to be considered. One option is to link the updating of the reference price to the payment method for the product or service. For example, the reference price of Part B drugs would be updated quarterly, the time interval that Medicare uses to update the payment rate of all Part B drugs. At issue is the effect on beneficiaries when an update of the reference price results in a new product or service being the LCA. For example, because the average sales price changes quarterly, the prostate cancer drug chosen as the LCA may vary from one quarter to another (Medicare Payment Advisory Commission 2007).

Developing an exception process

Finally, reference pricing strategies need a process to ensure access to the more costly product or service when it is the clinically appropriate course of treatment for the

TABLE 1–A1 **Methods used to set the reference price vary internationally**

Country	Method used to set the price	International comparison
Australia	Lowest of the drugs within the therapeutic group	New Zealand and UK
Canada	Prices generally cannot exceed cost of existing drugs in the therapeutic group	Cannot exceed France, Germany, Italy, Sweden, Switzerland, UK, US
Germany	Statistically derived from regression analysis; price set at the lowest third of the price in the therapeutic group	No
Italy	Lowest price product in the therapeutic group	Other European Union countries, particularly France and Spain
Netherlands	Price of the drug equal to or directly below the average of the prices within the therapeutic group	Maximum price cannot exceed average wholesale price in Belgium, France, Germany, and the UK
Spain	Mean of the three lowest cost drugs	Selected countries within the European Union

Note: UK (United Kingdom). Patented drugs include small molecule drugs and biologics.

Source: Australian Government Department of Health and Ageing 2009, Kanavos and Reinhardt 2003, Österreichisches Bundesinstitut Für Gesundheitswesen 2006, Organisation for Economic Co-operation and Development 2008a, Organisation for Economic Co-operation and Development 2008b, Patented Medicine Prices Review Board 2009.

beneficiary. Under the current local coverage decision process, Medicare already has in place a process to make exceptions. If there are true medical necessity indications that require the use of a more costly agent, Medicare considers payment for the difference in cost if an invoice and documentation of the medical necessity are submitted.

Implementing performance-based risk-sharing strategies

Implementation issues associated with performance-based pricing strategies include: (1) deciding who should collect, maintain, and analyze the data necessary to implement the arrangements; (2) establishing the information systems necessary to collect the needed data; and (3) defining objective measures.

One issue is whether CMS or the product developer would collect, maintain, and analyze the clinical evidence necessary to implement performance-based pricing. On the one hand, given sufficient funding, CMS could lead the analytical effort. On the other hand, with sufficient safeguards, including providing CMS access to the data, the product developer could lead the effort. Ensuring that the outcome data are validated clinical evidence data and developing the necessary pharmacy and medical data systems would also be key.

It would be necessary to establish the infrastructure to collect health care utilization data, laboratory results, and clinical outcome data. For example, a case study discussed in Chapter 1 of the June 2010 report (p. 12) noted that the payer already had the infrastructure in place that collected both the pharmacy data and hemoglobin A1c laboratory results necessary to establish a risk-sharing contract between the product developer and the payer. The future availability of electronic medical records would greatly assist in developing these strategies.

Other implementation issues associated with performance-based pricing include defining objective measures of outcomes that are not heavily confounded by patient characteristics or by other treatments, and developing and maintaining a mechanism to track patients' outcomes, such as clinical registries and electronic medical records (Garber and McClellan 2007). The effects of providers' practice patterns and patients' adherence to the prescribed regimen are other variables that need to be considered when designing performance-based pricing strategies.

Implementing CED

If the statute gave Medicare a clear legal foundation to use CED, a number of issues would need to be considered including: (1) developing a process to identify medical services as potential candidates for CED, (2) establishing a timeframe to consider CED for a particular service and to reconsider coverage for a service studied under CED, and (3) funding and managing the CED effort.

Developing a process to identify potential candidates for CED

Currently, Medicare lacks a process that would actively identify and determine which medical services—new services or new indications of existing services—would be suitable candidates for CED. Some health plans in the United States and internationally (e.g., Great Britain) have developed such a capability (Institute of Medicine 2008). Although the Agency for Healthcare Research and Quality (AHRQ) intends to sponsor such a process as part of its comparative-effectiveness research activities funded by the American Recovery and Reinvestment Act of 2009, this function will not be specific to Medicare (Agency for Healthcare Research and Quality 2010).

Such a process might include criteria (e.g., disease prevalence, mortality, morbidity, practice variation, information gaps, and duplication with existing research efforts) for evaluating whether a service is a candidate for CED. Another option is to conduct a value-of-information analysis to calculate the probability that new evidence would suggest a better treatment decision and the expected gain in benefits from that improved treatment decision (Claxton et al. 2005).

Establishing a timeframe to consider CED

Another implementation issue is whether CMS has sufficient time to consider applying CEDs. The agency deliberates on CEDs in the national coverage determination (NCD) process under the following deadlines the Congress established: (1) six months to issue an initial draft of a NCD that does not require a technology assessment or deliberation from the Medicare Evidence Development and Coverage Advisory Committee and (2) nine months for a NCD that requires such an assessment or deliberation. At issue is whether CMS is able to develop well-considered methods for CED implementation under this timeframe.

CMS lacks a specific timeframe as to when it will reevaluate Medicare's coverage for a service studied under CED. There have been only two instances to date in which CMS reconsidered coverage of a service studied under a CED. The concern is that without timelines, the goal of CED—to evaluate the clinical effectiveness of a service—may not be achieved. That is, a service whose clinical effectiveness is not well established could be covered under a CED indefinitely.

Funding and managing CED efforts

In some, but not all, instances the lack of a designated funding source to pay for the research costs of CED studies has delayed the start of the data collection effort. Medicare pays for the cost of services being studied under CED. However, Medicare generally does not fund clinical research and data collection activities. The lack of Medicare funding means that other public sources, such as the National Institutes of Health, or private sources, such as medical societies, providers, and product developers, are needed to cover a CED's research costs (Mohr et al. 2010).

Opinion differs as to whether the private sector or Medicare should incur a CED's research costs and be responsible for collecting and managing the data. One option is that product developers who realize increased revenue from newly allowed product sales should bear the cost. If private groups with a vested interest in the outcome of the CED research continue to help fund some or all of the research costs, it would be important to ensure that they cannot influence the study results. Laying out clear standards for designing studies, collecting and validating data, and analyzing data would be one way to help ensure that private funding sources do not bias CED efforts. Some standards do exist; for example, AHRQ sponsored a user's guide to registries evaluating patient outcomes (Gliklich and Dreyer 2007). In addition, Medicare would need access to the collected data to replicate and confirm data analyses. If private groups maintain the registries housing CED clinical evidence, it would also be important that Medicare establish a transparent process by which health services researchers can gain access to the data.

Another option is that Medicare should cover these costs because of the benefit to be gained from more appropriate utilization based on the evidence generated (Health Industry Forum 2007). There is concern that the private sector may not always provide an impartial and predictable source of funding.

Some observers contend that funding should be shared by all parties because both Medicare and the product developers gain from the clinical evidence that is collected. In addition, some leading analysts contend that for CED to be successful, an organization with scientific credibility, political independence, and technical expertise should be established to manage the CED efforts (Tunis and Pearson 2006). Ensuring the confidentiality of patient-level data would be key to whether CED efforts are managed by public or private groups. ■

Endnotes

1 The products include: goserelin acetate, leuprolide acetate, and triptorelin pamoate.

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