

The Effect of Formularies and Other Cost Management Tools on Access to Medications: An Analysis of the MMA and the Final Rule

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Introduction

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) provides for an outpatient prescription drug benefit to be delivered through private prescription drug plans and Medicare Advantage plans. The law puts the plans at risk for the cost of the drug benefit (although it includes some provisions for the government to share that risk). Plans thus have a clear incentive to control the cost of the benefit, both to protect their own bottom line and to offer a favorable premium in marketing to potential enrollees.

In the private sector, employers and health plans offering drug benefits employ a variety of tools to manage costs. They typically contract with pharmacy benefit managers (PBMs) to design and implement these tools. Many of these tools are centered on the use of a drug formulary, defined as a list of drugs selected as those that are most useful in patient care, based on both clinical effectiveness and cost considerations. In some cases, formularies are closed, thus excluding coverage for some drugs. In other cases, the formulary is open and all drugs are covered. In either situation, drugs may be arrayed into tiers and incentives intended to encourage use of drugs in the preferred tiers. These incentives can take the form of differences in the cost sharing faced by plan enrollees or procedures such as prior authorization or step therapy that make the use of non-preferred drugs more difficult.

In general, the MMA presumes that most or all of the tools that are commonly employed in the private sector will be available to Medicare drug plans. The law and the final regulations to implement the law address the need for certain constraints on the use of these tools, such as the law's provision that the use of formularies and other cost containment tools do not discourage enrollment of certain types of beneficiaries and that the use of tiered cost sharing be actuarially equivalent to the basic benefit design in the law. The final rule generally provides only limited amplification of these policies, leaving many key decisions subject to other avenues for providing guidance.¹ Immediately after publication of the final rule, the Centers for Medicare and Medicaid Services (CMS) issued guidance that addressed many issues concerning formularies and the role of pharmacy and therapeutics (P&T) committees.²

A number of small but significant changes were made between the proposed and final regulations. Refinements were made, for example, to the requirements that a plan's formulary must meet. More important than these changes, however, was the elaboration in guidance of the standards that CMS intends to use in reviewing whether formularies meet the regulatory requirement that the benefit design should not discourage enrollment of certain types of beneficiaries. Nevertheless, the tight timetable for this review will present the agency with a significant challenge.

¹ Department of Health and Human Services, Centers for Medicare & Medicaid Services. "42 CFR Parts 400, 403, 411, 417, and 423: Medicare Program; Medicare Prescription Drug Benefit; Final Rule," *Federal Register* 70(18), Part II: 4193-4742, January 28, 2005. Page citations in the text are from this reference.

² "Medicare Modernization Act Final Guidelines — Formularies; CMS Strategy for Affordable Access to Comprehensive Drug Coverage; Guidelines for Reviewing Prescription Drug Plan Formularies and Procedures," January 24, 2005, www.cms.hhs.gov/pdps/FormularyGuidance.pdf.

This policy brief examines the provisions of the law, the final rule, and the CMS guidance with regard to the use of cost management tools. In particular, it considers implications of these provisions for beneficiaries' access to needed medications and for the ability of plans to manage overall costs. The first half of this brief reviews the major cost management tools and the statutory and regulatory provisions that might affect their use. The second half lays out several major issues, most of which affect more than one of the tools.

The Use of Formularies

Formularies have become a nearly universal tool in the management of drug benefits. In the private sector, nearly 90 percent of health plans use a formulary of some sort.³ It seems certain that Medicare drug plans will have some kind of formulary, but that they may employ these formularies in very different ways.

The MMA anticipates this use of formularies and includes just two basic requirements. First, Medicare will establish a therapeutic classification system that can serve as the basis for plan formularies. Such a system includes a listing of drug classes and categories to which all drugs can be assigned. The law called for US Pharmacopeia (USP) to develop a model classification system. USP submitted that system to the Secretary on December 31, 2004.⁴ Plans are not required to use the model system, but there are certain incentives to do so.

Second, the Secretary has the authority to disapprove a drug plan whose design or benefits (explicitly including the formulary) substantially discourage enrollment of certain beneficiaries. This nondiscrimination rule, as discussed below, is potentially a key mechanism for protecting beneficiaries. Those plans that use the model classification system will be protected from further secretarial review for nondiscrimination, but plan formularies and other design features can still be reviewed for the selection of drugs within categories and classes.

In addition to these major requirements, the law also requires that a plan's formulary must be developed and revised by a P&T committee. The committee must include at least two independent members (one practicing physician and one practicing pharmacist), and the committee must base decisions, including those about which drugs are included on the formulary, on scientific evidence and standards of practice.

The Use of Closed Formularies

In a closed formulary, plans limit the set of drugs on the formulary and offer coverage only for those drugs. This approach has become rare in the private sector, used by only 2 percent of

³ *The Prescription Drug Benefit Cost and Plan Design Survey Report*, 2003 edition. Pharmacy Benefit Management Institute, Inc., 2003.

⁴ United States Pharmacopeial Convention, Inc., "Medicare Prescription Drug Benefit Model Guidelines: Drug Categories and Classes in Part D," December 31, 2004, <http://www.usp.org/pdf/drugInformation/mmg/finalModelGuidelines2004-12-31.pdf>.

private plans.⁵ It has remained, however, more common in Medicare Advantage (previously Medicare+Choice) where about one-third of plans use closed formularies.⁶

The MMA requires that a drug plan's formulary include drugs in each therapeutic category and class, a standard that is clarified in the final rule to require at least two drugs (as long as there are two drugs in the class) (p. 4538).⁷ For beneficiaries, the significance of this requirement is twofold. They will have to pay the full cost out of pocket for any drugs not on the formulary, and the costs incurred for these drugs will not count toward the true out-of-pocket (TrOOP) amount that triggers the catastrophic benefit.

The impact of the two drugs per class requirement depends heavily on the therapeutic classification system in place. As noted above, USP submitted its model guidelines to the Secretary. Plans, however, will not be required to use this system. Plans will face several strategic decisions, including whether to use the model classification system, whether to close their formulary in general or for particular classes, and what range of drugs to include on formulary in classes they choose to close.

One potentially significant change in the final rule was the addition of a provision calling for a plan's formulary to "include adequate coverage of the types of drugs most commonly needed by Part D enrollees, as recognized in national treatment guidelines" (p. 4538). The preamble notes further the need to "offer complete treatment options for a variety of medical conditions" such as asthma, diabetes, depression, lipid disorders, hypertension, and HIV (p. 4260). This requirement, which stems from the nondiscrimination requirement in the statute, is further amplified in the CMS guidance (discussed below).

Although the law did not provide specifically for an exceptions process for beneficiaries who want access to a drug that is excluded from the formulary, the final rule would apply it to this situation (pp. 4565-6). The preamble indicates that, since such requests are subject to external appeal, it makes sense also to require a process for requesting exceptions (p. 4354).

The Use of Formularies with Tiered Cost Sharing

Plans may apply different levels of cost sharing for drugs in different tiers of their formulary, regardless of whether a particular drug class is closed (i.e., only some drugs are covered) or open (all drugs are covered). A plan could even choose to cover all drugs in all classes (thus, in effect not have a formulary), while applying tiered cost sharing or prior authorization to encourage use of preferred drugs in some or all of the classes.

In the private sector, tiered cost sharing has become a dominant approach to cost management. In 2004, 65 percent of covered workers in the private sector faced three-tier copayments, where

⁵ *The Prescription Drug Benefit Cost and Plan Design Survey Report*, 2003 edition. Pharmacy Benefit Management Institute, Inc., 2003.

⁶ Achman, Lori, and Marsha Gold, *Trends in Medicare+Choice Benefits and Premiums, 1999-2002*, The Commonwealth Fund, November 2002, and personal communication.

⁷ The final rule includes an exception for a category or class with only two drugs available. In such a case, the rule allows a plan to cover only one drug if it demonstrates that one drug is clinically superior to the other drug.

generics are placed on the lowest-cost tier with an average copayment of \$10 or a coinsurance of 20 percent. The second tier typically includes preferred brand-name drugs with an average copayment of \$21 or a 26 percent coinsurance. The third tier includes non-preferred brand-name drugs for \$33 or 31 percent. Another 20 percent of workers saw two-tier systems, with generics only or generics and preferred brand-name drugs on the first tier. A few plans have experimented with four-tier systems, where the fourth tier (\$48 copayment or 31 percent coinsurance) is typically reserved for very high-cost drugs (e.g., biotech drugs) or drugs that treat symptoms for conditions that are unlikely to have life-threatening consequences (e.g., allergies or sexual dysfunction). Most plans use flat copayments, although there has been some increase in the use of percentage coinsurance.⁸

Under the MMA, tiered formularies must meet the actuarial equivalence test set forth in the statute, in addition to being subject to the nondiscrimination rule. The statute includes several criteria that must be met to preserve some elements of the deductible, coverage gap, and catastrophic coverage established for the Part D benefit. The key is that plans may modify the flat 25-percent coinsurance that otherwise would apply between the deductible and initial coverage limit in establishing tiered cost sharing. There is no restriction on the absolute level of cost sharing for a given drug or against substituting flat copayments for coinsurance as long as the actuarial equivalence test is met. The law also makes it clear that beneficiaries incurring higher cost sharing under a tiered system may apply these additional payments to meeting the TrOOP test.

The MMA provides that plans have a process for requesting exceptions to the tiered formulary for beneficiaries wishing to get access to a higher-tier drug at a lower cost. The final rule specifies that a plan must grant an exception whenever it determines that the non-preferred drug is medically necessary. But CMS did not establish specific exceptions criteria, choosing not to be overly prescriptive on the plans in order to preserve flexibility and the ability of plans to enforce cost containment.

The final rule does indicate some of the procedures that a plan must follow, for example that a request be based on a physician's determination that the preferred drug is less effective or would have adverse effects on the beneficiary (while noting that other stricter standards might be permitted). It also states that when an exception is granted, the non-preferred drug would be available at the cost-sharing level for a preferred drug, but not at the level for a generic drug (pp. 4564-5). The rule, however, denies beneficiaries the right to request a tiering exception for certain high-cost drugs, such as genomic and biotech products, if the plan has established a separate formulary tier (e.g., in a four-tier formulary) for these products.

⁸ *Employer Health Benefits, 2004 Annual Survey*, Kaiser Family Foundation and Health Research and Educational Trust, 2004.

Other Cost Management Tools

In addition to tiered cost sharing, health plans may employ other tools to enforce their formulary or to manage the cost of drugs more generally. In the private sector, about three-fourths of plans use prior authorization, where the plan must grant permission before a particular prescription can be filled. About half of plans use therapeutic interchange, a program designed to switch a patient from one medication to another that is on a preferred drug list or formulary (either accomplished at the point of sale or after an initial prescription is filled with the drug originally prescribed). About one-fourth of plans use step therapy, where payment for a drug is restricted unless certain other drug therapies have been tried first (e.g., a Cox-2 inhibitor for arthritis might be available only to patients who do not respond successfully to less costly non-steroidal anti-inflammatory drugs or NSAIDs).⁹ For any of these tools, the plan has the authority to deny payment for the drug, but a physician must authorize any change in the drug used by a particular patient. These tools may be used in conjunction with tiered cost sharing or as an alternative to that approach.

The MMA does not explicitly single out any of these techniques, but the final rule makes clear that their use by Medicare Part D plans is permitted. For example, the rule states that P&T committees would be involved in reviewing policies that guide their use (p. 4538), and the guidance issued by CMS notes that this review role is considered a best practice in the industry. In addition, use of these cost management tools would be subject to review under the nondiscrimination rule. The final rule, however, does not set forth specific criteria for plans' use of cost management tools, preferring to offer broad flexibility to participating plans.

The law and the regulations make clear that high use of generic medications is a goal of the program. The law requires that information be provided to the beneficiary (at the point of sale for retail customers) on the savings available if he or she switched to the generic alternative. In addition to lower cost sharing for using generic drugs, private-sector plans sometimes pay higher dispensing fees to pharmacists when filling a prescription with a generic drug, especially effective in the roughly 40 states where state law allows pharmacists to fill a prescription with a generic drug unless the prescribing physician indicates on the prescription "no substitution" or that the brand-name drug is "medically necessary."¹⁰ Other plans have engaged in education campaigns aimed at convincing either beneficiaries or physicians on the wisdom of substituting generic drugs where available. Some observers have suggested that seniors are often unwilling to challenge their doctor on what is prescribed and may not understand that generic drugs can generally be substituted without adverse consequences.

Another tool that is used by some private-sector plans is to encourage filling some prescriptions by mail order or even to require that prescriptions for certain maintenance medications be filled by mail. Where voluntary programs are encouraged, about 11 percent of prescriptions are filled by mail, and where some drugs must be filled by mail, the use of mail order rises to 34 percent.¹¹ The MMA allows the use of mail order but insists that any prescription (even 90-day supplies) can be filled at retail. The final rule, however, allows plans to have different (presumably,

⁹ *The Prescription Drug Benefit Cost and Plan Design Survey Report*, 2003 edition. Pharmacy Benefit Management Institute, Inc., 2003.

¹⁰ NIHCM Foundation, *A Primer: Generic Drugs, Patents and the Pharmaceutical Marketplace*, June 2002.

¹¹ *The Prescription Drug Benefit Cost and Plan Design Survey Report*, 2003 edition. Pharmacy Benefit Management Institute, Inc., 2003.

lower) cost sharing when a prescription is filled by mail (p. 4537). The use of cost-sharing incentives to encourage filling prescriptions by mail is subject to the actuarial equivalence test, and the higher amounts paid by the beneficiary for 90-day supplies at retail would count toward meeting the TrOOP test.

Finally, some plans – especially in certain Medicaid programs or Medicare Advantage plans – have controlled costs through quantity limits, for example, limiting some prescriptions to a certain number of pills or capping the number of prescriptions that may be filled in a month. The final rule makes it clear that quantity limits are allowed by listing this tool among the policies that P&T committees should review (p. 4538). Neither the final rule nor the preamble provides additional detail on allowed use of quantity limits, and it would appear that the only constraint would be a determination that such a limit would discourage enrollment by certain beneficiaries.¹²

Issue: The Nondiscrimination Criterion

The nondiscrimination rule could be one of the most significant tools for protecting beneficiaries from inappropriate use of cost management tools. It gives the Secretary the authority to reject bids from plans whose proposed use of these tools is aimed at discouraging enrollment of certain beneficiaries, such as those with costly medical conditions. Should its application prove limited, however, beneficiaries who have serious chronic conditions may find few if any plans that provide them affordable access to the drugs they need. In addition, plans using tools that discourage enrollment of sicker beneficiaries will likely experience favorable selection and achieve an unfair advantage in market competition.

The statement in the law is broad, allowing disapproval of a plan bid if the “design of the plan and its benefits (including any formulary and tiered formulary structure) are likely to substantially discourage enrollment by certain part D eligible beneficiaries under the plan.”¹³ In the final rule, the regulatory text addressing this provision simply repeats the basic requirement in the law (p. 4545). It is important to note that neither the law nor the regulatory text uses the word “discriminate” although it appears in the preamble and the formulary guidance a number of times. In fact, the preamble explains that a benefit design is not “discriminatory” if it does not discourage enrollment “on the basis of health status, including medical condition (related to mental as well as physical illness), claims experience, receipt of health care, medical history, genetic information, evidence of insurability, and disability.” The preamble goes on to say that the Secretary will review plans for features that, when applied, would have “differential impacts on beneficiaries with particular medical conditions” (p. 4297).

Although this language addresses broadly the idea that the rule bars designs that discriminate based on medical conditions, it could be interpreted to leave open some discriminatory approaches. Suppose, for example, that a plan chose to exclude from its formulary or assign to a higher tier all drugs that cost more than a given amount (with exceptions only as needed to meet the statutory requirement of covering two drugs in each therapeutic class). The plan might argue

¹² The formulary file record layout included in the CMS guidance has a set of fields in which the plan must specify, by drug, any quantity limits (e.g., 9 pills every 60 days).

¹³ Medicare Modernization Act, 1860D-11(e)(2)(D)(i).

that its formulary cannot be judged discriminatory since the criterion is strictly one of cost.¹⁴ Those arguing that this formulary design should be excluded would have the more difficult task of showing that it had the effect of excluding beneficiaries with conditions that could only be treated with expensive drugs.

This language might also make it difficult to exclude a formulary that was judged to discriminate against certain racial or ethnic groups. There is clinical evidence that beneficiaries of different races or ethnic groups respond differently to certain drugs, for example, those used to treat hypertension. If a formulary design excluded those drugs considered more effective for African American beneficiaries, it might be judged not to discourage enrollment on the basis of health status, again leaving it more difficult to deny participation to that plan or require that its formulary be modified.

Enforcement of the nondiscrimination standard may also be a significant challenge for the Secretary. The task is made more difficult because the standard lacks a specific definition in the statute or the rule. Neither participating health plans nor beneficiaries can know what the standard really means until there is a record from the reviews that are conducted.

In theory, it should be relatively easy to apply the rule (whatever its regulatory interpretation) in looking at the assignment of drugs to a formulary and to tiers on that formulary. Reviewers can look at which drugs are excluded or assigned to high cost-sharing tiers and make judgments about whether certain types of beneficiaries will need those drugs. But the absence of a clear standard will make this determination difficult in practice. If a particular drug is indisputably the preferred treatment for a particular condition, then its exclusion from a formulary should be grounds for rejection of that formulary. But where evidence is mixed about which of several different drugs is a preferred treatment or when patients respond differently, CMS will have a difficult decision.

Decisions become even more complex when considering the varying impact of cost sharing on those at different income levels. For example, a cost-sharing tier where the beneficiary pays 50 percent of the cost of the drug will have a different impact if the prescription costs \$10 or \$200, and that impact will vary according to the income of the beneficiary. Those with the lowest incomes are protected by the subsidies in the law, but those just above the threshold for subsidies (150 percent of the federal poverty level) will have a hard time paying half the cost of a \$200 drug each month.

In addition, it will be even harder to apply the nondiscrimination rule to cost-management tools other than the formulary itself. The final rule added the plan's "utilization management program" (in addition to its formulary and any tiered formulary structure) to the provision that requires enforcement of the nondiscrimination rule (p. 4545). Determining that use of a tool like prior authorization might discriminate will require, however, a judgment about how the process works. The preamble to the final rule generally indicates an intention to review formal requirements, which might include both the list of drugs subject to prior authorization and the

¹⁴ Jeanne M. Lambrew, "Highlights from the Medicare Prescription Drug Regulation: The Good, the Bad, and the Ugly," Center for American Progress, August 12, 2004.

criteria for obtaining approval (e.g., whether the physician has to provide written documentation of the patient's need or just place a request by phone). But it will also be important to review how easily authorization is granted (e.g., whether most requests are authorized quickly or whether many are rejected or sent back for more documentation). Evidence of these variations may be unavailable until the system has been in place for many months. The preamble does note that, after the initial year of the program, CMS will review the history of enrollee appeals of plan formulary requirements to identify issues with the plan's formulary, and the final rule added a requirement that plans provide CMS with information on the procedures and performance of their drug utilization management programs (p. 4540).

Figure 1. Timetable for Prescription Drug Plans, 2005

February 18	– Notice of intent to apply
March 23	– Applications due to CMS
April 18	– Formularies due to CMS for review
May/June	– Decisions on contract eligibility
May 16	– Preliminary approval of formularies
June 6	– Bid submissions due
September 2	– CMS completes review of bids
October 15	– CMS information campaign starts
November 15	– Open season starts
January 1, 2006	– Part D Benefit begins

Ultimately, the means of enforcement may be the key to the effectiveness of the nondiscrimination rule. The final rule signals more strongly than did the proposed regulations that the Secretary intends to enforce this provision, and the guidelines published by CMS in January provide some additional information on the proposed process for enforcement.¹⁵ There it states that the goal for ensuring that plans provide high-quality cost-effective drug benefits can be met by an agency review strategy that “facilitates appropriate beneficiary access to all medically necessary Part D covered drugs along with plan flexibility to develop efficient benefit designs” and draws on the best practices in the private sector.¹⁶ CMS states specifically in the guidelines that the two-drug requirement is viewed as a floor rather than an absolute standard and lays out a series of review standards, described in the next section below.

The most significant challenge for CMS may be fitting its review of plan formularies into a tight timetable (Figure 1). The schedule for submitting and approving plan bids will constrain this process in the best of circumstances, since plans that intend to participate in the program must submit their formularies, including tier placement, to CMS by April 18, 2005. CMS has indicated that it will provide preliminary approval of the formularies by May 16. The agency created this tight schedule presumably so that plans will know their formularies are acceptable before submitting bids to meet the June 6 deadline.

If there are even three plans per region on average, CMS will need to approve formularies for over 100 plans in just four weeks, including a review of whether there are two drugs per class, its use of prior authorization or other utilization management tools, whether the formulary would not discourage enrollment of certain types of beneficiaries, and whether it generally provides adequate coverage. These reviews should involve a detailed look at the adequacy of coverage

¹⁵ It is important to note that the guidelines are an interpretation of the final rule, but do not have the same legal standing. For example, guidelines can be modified without going through a formal rulemaking process.

¹⁶ “Medicare Modernization Act Final Guidelines — Formularies; CMS Strategy for Affordable Access to Comprehensive Drug Coverage; Guidelines for Reviewing Prescription Drug Plan Formularies and Procedures,” January 24, 2005, www.cms.hhs.gov/pdps/FormularyGuidance.pdf.

across 41 major categories of drugs and well over 1,000 separate pharmaceutical products (not counting separate brand and generic versions). Apparently the review of the plan's cost sharing structure and its actuarial equivalence will take place between June 6 and early September, after bids are submitted.

Especially in the first few years, CMS will not be able to draw on past experience with drug plans on the effect of different formulary designs. CMS has not provided any indication of what resources might be devoted to review of the formularies and enforcement of the nondiscrimination rule. Finally, there is no indication of how the Secretary will review midyear changes to the formulary or other cost management tools with regard to any potential discriminatory effect, although the preamble to the final rule states that such changes will be reviewed.

Issue: The Therapeutic Classification System

As required by the statute, US Pharmacopeia issued model guidelines for therapeutic categories and pharmacologic classes on December 31, following a process of publishing draft guidelines in August and soliciting public comments.¹⁷ The model guidelines lay out a classification system with 146 distinct drug classes – a number larger than that advocated by PBMs and other potential drug plan sponsors, but smaller than that preferred by pharmaceutical manufacturers. The guidance issued by CMS in January provides further help to health plans and beneficiaries on the standards the agency expects plan formularies to meet.¹⁸

Beneficiaries will have the greatest opportunity to obtain drugs they need if there are more distinct drug classes, since the formulary would include more drugs based on the rule requiring the availability of two drugs per class. In this one respect, beneficiary interests tend to be aligned with those of drug manufacturers. These aligned interests might break down, however, if beneficiaries gain access to more drugs but incur higher costs either because plans have less ability to bargain for lower prices or if they place the additional covered drugs in higher cost-sharing tiers.

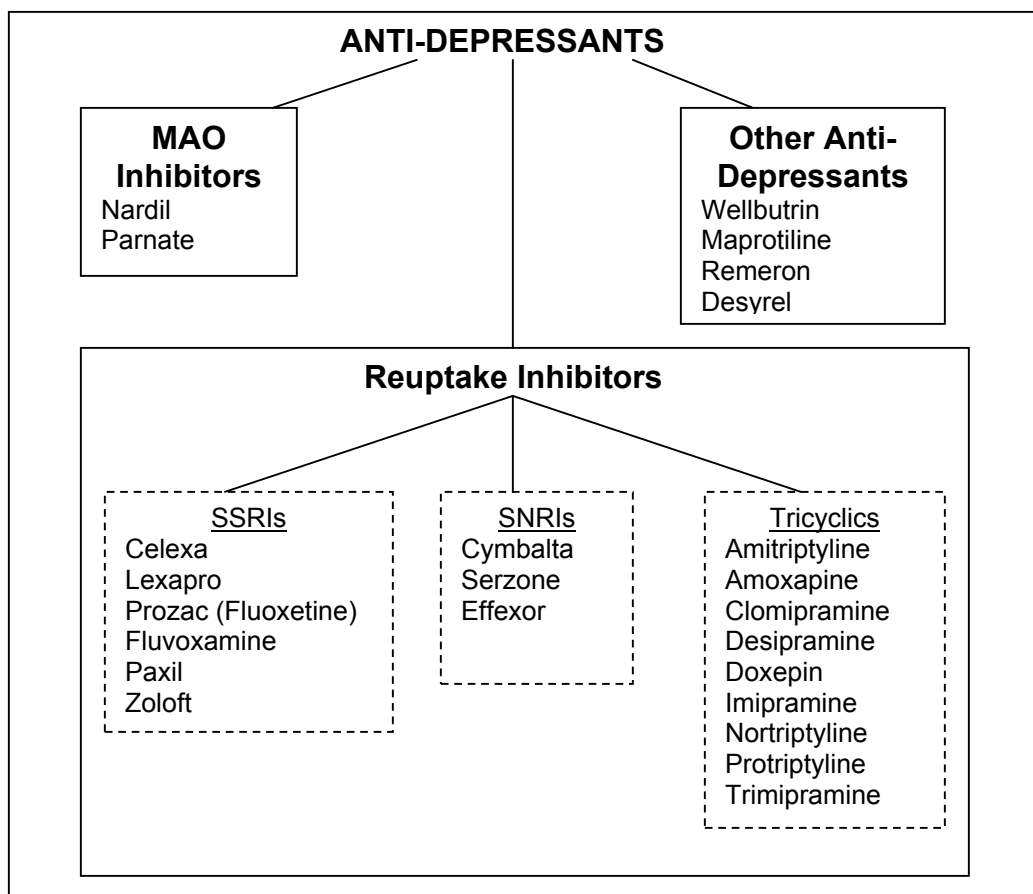
To illustrate the effects of formulary classes, reuptake inhibitors are one of three pharmacologic classes in the model guidelines within the larger therapeutic category of anti-depressants (Figure 1). The reuptake inhibitor class contains three types of anti-depressants: the older tricyclics (e.g., amitriptyline and nortriptyline), the selective serotonin reuptake inhibitors or SSRIs (e.g., Celexa, Paxil, Zoloft, and Prozac), and the serotonin and norepinephrine reuptake inhibitors or SNRIs (e.g., Cymbalta and Effexor). Because a plan could meet the requirement of two drugs in the class by offering two tricyclics on its formulary, a patient needing an SSRI or SNRI could be denied coverage. Among the other drug classes where groupings in the model guidelines could affect large numbers of patients are non-steroidal anti-inflammatory drugs or NSAIDs, where

¹⁷ United States Pharmacopeial Convention, Inc., "Medicare Prescription Drug Benefit Model Guidelines: Drug Categories and Classes in Part D," December 31, 2004, <http://www.usp.org/pdf/drugInformation/mmg/finalModelGuidelines2004-12-31.pdf>.

¹⁸ "Medicare Modernization Act Final Guidelines — Formularies; CMS Strategy for Affordable Access to Comprehensive Drug Coverage; Guidelines for Reviewing Prescription Drug Plan Formularies and Procedures," January 24, 2005, www.cms.hhs.gov/pdps/FormularyGuidance.pdf.

Cox-2 inhibitors are grouped with older NSAIDs, and cardiovascular drugs, where ACE inhibitors are grouped with ARBs (although the group that includes these two types of drugs is distinct from beta-blockers, calcium channel blockers, and diuretics, all of which are also used to treat hypertension).

Figure 2. Therapeutic Category of Anti-Depressants and its Three Pharmacologic Classes



In the draft model guidelines, the anti-ulcer class had a similar mix, containing both the older H2 blockers (e.g., Pepcid, Tagamet, and Zantac) and the newer proton pump inhibitors or PPIs (e.g., Prilosec, Prevacid, and Nexium). The final guidelines were modified to create separate classes of anti-ulcer drugs — one of several such changes made. Plans, however, could still propose to cover two H2 blockers in a low cost-sharing class and two PPIs at higher cost sharing.

As described above, CMS put forward in its January formulary guidelines a higher standard than the statutory requirement of two drugs per class.¹⁹ The paper outlines a series of six checks that the agency intends to use in reviewing plan formularies. One review standard is whether a

¹⁹ “Medicare Modernization Act Final Guidelines — Formularies; CMS Strategy for Affordable Access to Comprehensive Drug Coverage; Guidelines for Reviewing Prescription Drug Plan Formularies and Procedures,” January 24, 2005, www.cms.hhs.gov/pdps/FormularyGuidance.pdf.

formulary has at least one drug in each of a set of “key drug types” identified by USP in comments provided to CMS.²⁰ These key drug types are in effect a third-level class for the model classification system and would ensure that a formulary had at least one tricyclic, one SSRI, and one SNRI in the anti-depressant class, since each of these are designated as key drug types. As a result, a plan’s formulary would need a minimum of seven (instead of six) different anti-depressants. Another check would extend that standard further, stating the expectation that formularies cover a majority of all anti-depressants (thus upping the standard from seven drugs to thirteen, based on USP’s list of 24 drugs in that category). The standard for a majority of drugs in the class would also apply to other key drug classes used to treat mental health conditions and HIV disease, conditions for which many clinical experts believe less substitution of drugs is appropriate. CMS also indicates that it will review the drug lists to see whether beneficiaries will have “appropriate access” to drugs cited in widely accepted national treatment guidelines for another two dozen conditions (although it does not list the guidelines or the drugs in them) and some different measures of drugs commonly used by the Medicare population.

Because CMS expects tiered formularies to be used by many plans, it also states its expectation that these designs will follow standard industry practices. Specifically, its review will consider whether drugs placed in a high cost-sharing tier have therapeutically similar drugs available in “more preferable positions on the formulary.”²¹ This standard could take on particular importance if a plan chooses to place certain high-cost drugs in some classes on a separate tier with higher cost sharing. Because plans are not required to grant requests for tiering exceptions for such a tier, the absence of a therapeutically similar drug in a lower tier should be grounds for rejecting that formulary.

The standards in the guidance appear to strengthen considerably the requirements that plan formularies must meet and represent a statement of the agency’s intention to enforce the nondiscrimination rule. Still the standards in the guidelines are not rules, and each plan will have the opportunity to present clinical justifications for why its particular formulary deviates from the standards. It also remains to be seen whether the limited time for agency review will result in most formularies being approved as submitted, although this result is unlikely to be transparent to outside observers since draft formularies will not be made available to the public. Furthermore, beneficiaries do not benefit only from having a large number of classes and a large number of drugs on the formulary. They will also be better off if plans can use well-designed management tools and the resulting competition among drugs in a larger class to obtain lower prices, thus presumably lowering both beneficiary copayments and plan premiums. The anti-ulcer drugs illustrate this tension. Some experts suggest that PPIs are over-prescribed and that many who use them would be better treated with the less expensive H2 blockers. Yet for some patients, especially those with gastroesophageal reflux disease or GERD, the H2 blockers are not satisfactory treatments. By contrast, mental health experts typically argue that patients who have been stabilized on a particular anti-depressant or anti-psychotic drug face adverse consequences

²⁰ United States Pharmacopeial Convention, Inc., “USP Comments to the Draft Guidance -- CMS Formulary Review for the Medicare Prescription Drug Benefit,” letter to Mark McClellan, Administrator, CMS, December 30, 2004, <http://www.usp.org/pdf/drugInformation/mmg/commentstotheGuidance2004-12-30.pdf>.

²¹ “Medicare Modernization Act Final Guidelines — Formularies; CMS Strategy for Affordable Access to Comprehensive Drug Coverage; Guidelines for Reviewing Prescription Drug Plan Formularies and Procedures,” January 24, 2005, www.cms.hhs.gov/pdps/FormularyGuidance.pdf.

if switched away from that drug. CMS reflected these differences by having stricter guidelines for mental health drugs (and HIV drugs) than for drugs used for other health conditions.

While an effective exceptions process could guarantee that these patients gain access to a PPI or a particular anti-depressant at a reasonable price, it is unclear whether the number of patients in this situation would burden the exceptions and appeals processes or whether plans would have any real incentive to grant exceptions allowing the purchase of drugs that are considerably more expensive. Nor is it clear from the final rule that the exceptions and appeals processes envisioned are adequate to suit the needs of these patients.²²

Issue: The Actuarial Equivalence Standard

Because tiered cost sharing is expected to be a major tool for Medicare Part D drug plans, the actuarial equivalence test will have considerable importance in setting boundaries on allowable cost sharing. A plan is allowed to lower the \$250 deductible, change the \$2,250 initial coverage limit, and apply different coinsurance (or flat copayments) up to the initial coverage limit, as long as its assessment of the actuarial value remains equal to that of the standard benefit. Included in this test is the requirement that not only the value of the total coverage be the same as for the standard benefit, but also the value of coverage below the initial coverage limit and the value of unsubsidized coverage be the same as those segments of standard coverage. The final rule indicates that a qualified actuary (a member of the American Academy of Actuaries) must certify the plan's actuarial valuation as part of the bid the plan submits to the Secretary (p. 4544).

Within the bounds of actuarial equivalence and the application of the nondiscrimination standard, neither the law nor the regulations restrict the tiered cost-sharing design. Thus, there are no absolute limits on how tiers can be structured. For example, a plan could provide for higher coinsurance for spending between the \$250 deductible and \$1,000 in drug costs and lower coinsurance between \$1,000 and the \$2,250 initial coverage limit. Alternatively, based on its formulary, a plan could set the coinsurance for some nonpreferred drugs at 90 percent or even 100 percent, and it could set different tier structures in each class. The calculations necessary for the actuarial equivalence test will determine, for example, whether a 90-percent tier for some drugs must be offset by a 5-percent tier or a 15-percent tier for other drugs. But the regulations do not require this test to be met within each class of drugs. The test must take into account expected behavioral responses to different cost-sharing structures, although there is little if any empirical evidence in the literature to estimate beneficiary response to different plan designs, especially those that are not used often in the private sector.

CMS has indicated that, in enforcing the nondiscrimination standard, it will review the design of tiered cost sharing. As noted above, one of the review standards in the CMS guidelines will focus on drugs in nonpreferred tiers without commonly used therapeutically similar drugs appearing in more preferred tiers. For example, this would appear to prevent a plan from placing all the PPI drugs or SSRIs on its formulary on a non-preferred tier, although "therapeutically similar" is only defined as drugs that provide similar treatment outcomes. But a design that

²² More discussion of issues relating to exceptions, grievances, and appeals in the proposed regulations is in a paper by Sara Rosenbaum, "Grievance and Appeals Procedures: An Analysis of the MMA and Proposed Regulations," Kaiser Family Foundation, September 2004, updated in March 2005.

passes muster on this standard (especially if the standard is interpreted narrowly) and that meets the actuarial equivalence test may still force some beneficiaries to choose between incurring high coinsurance payments to continue a drug they have been using and making a switch to an unfamiliar drug.

Enforcement of the actuarial equivalence test also raises some issues. The final rule relies on an actuary's certification that the benefit design meets the test, and the preamble suggests that additional information will be provided in the form of interpretive guidance. The preamble also talks generally about specifying data elements to be submitted, so that the Secretary can "evaluate the analysis and assumptions for compliance and reasonableness" (p. 4290). As in the case of evaluating therapeutic classifications and the potential for benefit designs and formularies to discriminate against certain classes of beneficiaries, the actuarial equivalence test moves CMS into relatively uncharted territory. Some concerns, such as whether CMS will allow tiers based on the level of drug spending or extremely high cost sharing for certain non-preferred drugs, have not been addressed directly in the final rule or guidelines and are thus left to a general review for actuarial equivalence and nondiscrimination. Regardless of the regulatory requirements, the impact of the actuarial equivalence provision will be seen largely in how it is enforced.

Issue: Role of P&T Committees

As noted above, the law requires a role for the plan's pharmacy and therapeutics committee in developing and revising the formulary. A majority of P&T committee members must be practicing physicians or practicing pharmacists, and at least two members must be experts in the care of elderly and disabled individuals. In addition, at least one practicing physician and one practicing pharmacist on the committee must be independent experts. In the proposed regulations, the Secretary asked for comments on several options: clarifying that the committee's role is binding on the plan, expanding the law's requirement for having independent members, and involving the committee in reviewing other cost management tools such as prior authorization.

The final rule added language requiring that the P&T committee review policies regarding utilization management processes and explicitly includes drug utilization review, quantity limits, generic substitution, and therapeutic interchange (p. 4538).²³ Preamble language makes clear that, while P&T committee recommendations on which drugs should be on the formulary are binding, recommendations on tier placement or utilization management are advisory and not binding (p. 4256).

The final rule makes no expansion of the statutory requirement of two independent members, although it does clarify that independent means free of conflict with both the plan sponsor and any pharmaceutical manufacturer. Although there are additional requirements in the CMS guidance that all members must reveal to other members any conflicts of interest and recuse themselves from discussions of any particular drug that presents a conflict, the rules fall short of

²³ The language in the final rule does not explicitly include prior authorization or step therapy. But the use of the word "including" and language in the preamble seem to make clear that these fall under the mandated review by the P&T committee.

creating a fully independent committee. In addition, because neither the law nor regulations speaks to the size of the committee, it is unclear how strong a role the two independent members will play. Current industry practice suggests that plans are unlikely to create a committee that is fully independent and whose decisions are binding on the plan (except as required). Over time, it will be important to see whether other steps should be taken to ensure that beneficiaries' interests are well represented in the deliberations of the P&T committees.

The law also requires that the committee's decisions should be based on "the strength of scientific evidence and standards of practice."²⁴ There is growing interest in basing formularies on unbiased scientific evidence, and the directive at least points in this direction. As noted above, beneficiaries might be better off when plans put equivalent drugs in competition to obtain lower prices, but evidence suggests that some private-sector plans place a drug on a preferred tier based on the size of a rebate rather than on an overall lower price. It may be important to consider whether greater reliance on evidence-based decision making (as well as transparency of pricing) will help align the plan's interests with those of beneficiaries in terms of obtaining lower prices. Although it is unlikely that regulatory requirements can accomplish this alone, they might push plans to move in this direction.

Conclusion

The array of cost management tools will be a major factor in determining what the drug benefit really means to the beneficiary. Formularies, cost sharing, and the use of tools like prior authorization will be critical to whether beneficiaries can get the drugs they need. For beneficiaries with modest drug needs who do not fall into the coverage gap, these factors will probably be the most important aspect of their benefit, driving whether their drugs are covered and how much they cost. For those with more substantial drug costs, formularies and cost-sharing tiers will help determine the burden imposed by the coverage gap and when catastrophic protection kicks in. The way these tools are used will also speak to the hassle factor faced by beneficiaries and their physicians, that is, how often needed drugs are unavailable and when exceptions or appeals must be requested.

As noted earlier, there is a potential tradeoff between what helps individual beneficiaries at any given time (e.g., being able to obtain the drugs their doctors prescribe) and what helps beneficiaries more broadly (e.g., lower prices and lower premiums). For medical conditions like depression, bipolar disorders, HIV, or hypertension, treatment decisions are relatively individualized, that is, the drug that works for one patient may not work for another. For beneficiaries with these conditions, less restrictive formularies or fewer drugs on a high-cost tier may be important to maximizing quality of care. For other medical conditions such as allergies, arthritis pain, or ulcers, the choice of drugs may be less critical. But there are still some patients, especially those with certain accompanying conditions, for whom the preferred drug may not work. The average beneficiary in these circumstances may see reduced premium costs if plans use their market leverage to bargain for the best price among a set of drugs where good evidence on comparative effectiveness concludes that the drugs are generally equivalent. For these drug

²⁴ Medicare Modernization Act, 1860D-4(b)(3)(B)(i).

classes, a good exceptions process may be the key to protecting beneficiaries with special circumstances.

The MMA and the final rule that directs implementation of the new drug benefit provide some help in addressing these issues, but they leave some key questions to less formal guidance from CMS. The formulary guidelines, released by CMS in January, offer plans and beneficiaries many additional answers. But the real test will come in decisions by plan sponsors about the design of their benefits and the rigor of oversight of those plans by CMS. As implementation of the Medicare drug benefit proceeds, it seems critical that careful consideration be given to making sure that key beneficiary protections are workable: that the review of plan formularies and use of cost management tools with regard to the nondiscrimination test is meaningful and can be enforced, that the therapeutic classification system provides appropriate access to drugs that beneficiaries need, that the actuarial equivalence standard prevents the use of cost sharing that is prohibitive to beneficiaries, that P&T committees can base decisions on scientific evidence, and that their role in advising plan sponsors is more than symbolic.



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