CMS Review Could Act as a Check on FDA Shortcomings

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The past eight years have been difficult ones for the United States Food and Drug Administration (FDA). Serious and legitimate questions have been raised about FDA’s conduct, its politicization of medical decision making and a failure to fulfill its primary mission of protecting the health of the American public. Critics have long contended that there is too much emphasis on “me-too” prescription drugs with lower efficacy standards and marginal benefits, and a misplaced focus on new drug approval at the expense of patient safety. On the legal side, FDA has for the first time taken a blatantly pro-industry stance on the issue of federal pre-emption of private citizen claims against pharmaceutical manufacturers for either direct injury from the medications themselves or from insufficient warning information in the drug label.

All of these possible misuses of regulatory power and public health failures raise important questions about whether FDA is capable of self-correcting. Congress has conducted dozens of hearings with senior FDA officials for more than five years to address agency shortcomings, but FDA has not even fully complied with the drug safety recommendations of the Congressionally-funded Institute of Medicine Report on Drug Safety which was released in 2000. At times FDA has acted as little more than a conduit for manufacturers to get new medical products to market with little regard for marginal efficacy, safety, cost to public health, or financial obligations traditionally incurred by third-party payers for expensive new prescription drugs, biologics, and devices.

It may be up to the Center for Medicare and Medicaid Services (CMS), another part of the Department of Health and Human Services, to step in to correct these problems. For many years, the pattern has traditionally been that of FDA approval of a medical product followed by a routine, perfunctory CMS decision to reimburse manufacturers for their product. Although there have always been two distinct, sequential hurdles for manufacturers of medical products in the United States to successfully sell their products

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1 UNITED STATES GOVERNMENT ACCOUNTABILITY OFFICE, REPORT TO CONGRESSIONAL REQUESTERS, FOOD AND DRUG ADMINISTRATION DECISION PROCESS TO DENY INITIAL APPLICATION FOR OVER-THE-COUNTER MARKETING OF THE EMERGENCY CONTRACEPTIVE DRUG PLAN B WAS UNUSUAL (November 2005).
3 Id.
6 Id. The Report itself can be found on the Institute of Medicine website.
8 Although products can be sold without CMS reimbursement, this limits the drug’s potential profits substantially by cutting off use by Medicare and Medicaid recipients.
– the approval decision by FDA and the subsequent reimbursement decision by CMS – there is already evidence that the balance of power has begun to change.9

Ironically, one of the biggest boons to the pharmaceutical industry by the United States government also contains the potential to offset some FDA failures. The 2003 Medicare Modernization Act (MMA)10 could, if amended, give the federal government the power to negotiate for lower prices with prescription drug and medical device manufacturers as part of Medicare’s Part D prescription drug program. Particularly if this were combined with a flexing of the untapped potential regulatory muscle of CMS, it could reign in a substantial component of current healthcare costs. A review of what and how FDA and CMS decide, and what CMS could decide were it so inclined illustrates how this could happen.

FDA’s Role Is Peripheral to Healthcare Costs

FDA approval is the necessary first step for medical product manufacturers, but it is likely no longer the most important step. Given the current money-crunch the federal government is facing with escalating medical expenses for its entitlement populations such as Medicare beneficiaries, the looming tidal wave of retiring baby boomers who will become eligible for Medicare within the next five years,11 and the exorbitant costs of both prescription drugs as well as of cutting edge diagnostic and therapeutic medical devices and biological drug products, there is no longer enough money in the federal pot anymore to pay for every new prescription drug, biological, medical device, or medical/surgical procedure for every eligible person.

The old economic reality of demand exceeding supply, and a new attention to whether the money being spent is worth the incremental, if any, benefit provided by the product (a byproduct of a combination of the new “pay for performance” mantra and a refusal by insurers to pay for costs incurred by preventable medical errors12) has now tilted the playing field sharply in CMS’s favor. The big hurdle for companies to go over, and the critical review to a product’s ultimate success, now goes through CMS, not FDA. The days of FDA approval alone being the golden ticket to enter the U.S. market are now gone. There are several key differences between FDA and CMS that account for this new reality.

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10 THE MEDICARE PRESCRIPTION DRUG, IMPROVEMENT, AND MODERNIZATION ACT OF 2003, Pub.L. 108-173, 117 Stat. 2066. This Act specifically forbade the Centers for Medicare and Medicaid Services (CMS) – in effect the largest health maintenance organization (HMO) in the world – from using its tremendous market share power to negotiate with the pharmaceutical industry for lower prices on prescription drugs for its 60 million beneficiaries.
First, although FDA directly or indirectly regulates industries (e.g. food, drugs) which affect close to 20 percent of every dollar in the United States gross domestic product (GDP), CMS is the largest health claims processor in the world, and the largest health care system of any Western nation with almost 85 million enrollees. Its fixed operating costs make it a substantial non-discretionary component of the annual federal budget every year. The only thing FDA and CMS have in common is that they are both understaffed and under-funded. Even their missions are different. CMS’s is to “ensure the health security of beneficiaries.” This clearly encompasses financial security so that money is not wasted on expensive medical products with little or no advantage over existing, less expensive ones.

Second, FDA makes its prescription drug and medical device decisions in a financial vacuum, but just the opposite is true for CMS. CMS functions as a gatekeeper or public insurance program, and must make 3 critical decisions: (1) coverage (which products and services are eligible for payment and on what terms); (2) coding (unique identifiers must be given so that new devices, drugs, and procedures are compensated); and (3) payment (CMS determines the rate at which it will pay for new medical products and procedures). If the latter is not high enough, FDA approval becomes irrelevant since the financial payoff no longer justifies a marketing effort, particularly since private insurance reimbursement is often tied to CMS reimbursement. In other words while the approval for marketing decision for FDA is one of benefit versus risk, the coverage for reimbursement decision for CMS is one of benefit versus cost.

Third, the decision-making standards for the two agencies differ as well. FDA’s standard for approval of a new drug or biologic is whether the medical product is, on balance, safe and effective for its labeled condition when compared to the risks from taking it. This safety and efficacy determination is not precisely defined by statute and is a judgment call ideally purely based on analysis of medical data derived from one or two randomized, controlled, prospective clinical trials. All of the data for an FDA approval decision is supplied by industry in the new drug, device or biologics application. In fact, the medical literature is never consulted; the approval decision is derived solely from the four corners of the documents submitted. Accusations of FDA “capture” by industry have focused in part on concerns that the inherently subjective nature of the individual medical reviewer’s approval decision has not emphasized legitimate safety concerns, particularly for new prescription drugs. An awareness of the potential power of CMS decision making may be seen in the new phenomenon of new drug or medical device applications

13 FDLI Conference, supra note 6.
14 Id.
16 FDLI Conference, supra note 6.
containing pharmaco-economic data on comparative cost even though this is not a requirement for a new drug, device, or biologic approval decision.

CMS, on the other hand, uses a “necessary and reasonable” standard. This is also not defined by statute but clearly is an inherently more expansive evaluation of a new medical product. In particular, this coverage/coding/reimbursement standard need not be, and virtually never is, driven by a simple evaluation of the medical efficacy versus safety calculus of FDA approval. Although much of the evidence CMS’s decisions are based on will be supplied by industry as well, the determination of whether a new medical product is reasonable and necessary for the Medicare population is based on the precise type of information FDA approvals are rarely based on – comparing the proposed new drug or device to those which are already on the market to determine if any incremental benefit exists, if at all, and whether the level of reimbursement for the product can be justified. What this means is that just because FDA considers something is “safe and effective” for a given medical purpose does not guarantee that CMS will consider is “reasonable and necessary.”

In other words, CMS must first decide that a new product offers a real benefit to its patient population before it will determine that it will pay for it. Because CMS decisions always follow FDA decisions, this means that manufacturers could easily invest hundreds of millions of dollars developing new follow-on drugs that could potentially end up being priced the same as the cheapest generic if CMS determines they have negligible health benefits for its beneficiaries. Decisions such as this are a double-edged sword and could truly change new drug development in the U.S. Low pricing decisions by CMS could deal a death-blow to the pattern of “me-too” drug development we’ve seen for more than a decade and at the same time provide manufacturers with the incentive they need to focus on developing truly innovative medical products.

CMS Independent Review of FDA Decisions

Even more critical is that CMS may now perform its own review of the FDA approval decision to determine if the approval was justified. This is a one-way street; FDA does not get to sit in on CMS determinations and ex post change the reimbursement decision. Although uncommon, this de novo review ability has already occurred on several occasions. Not only is FDA’s presumably “expert” review subject to later reversal by CMS, but we are now seeing a CMS presence at FDA meetings with industry prior to FDA’s approval decision. This has put manufacturers in the position of having to have comparative pharmaco-economic cost information about their products during the FDA approval stage to justify product development, and possibly the initial pricing decision, to CMS even before FDA has approved the product. The end result of this is that the status quos of agency independence, separate but equal power, and involvement by CMS only after FDA has completed its work are no longer the rule.

Analysis/Conclusion

Some health policy experts have contended that the fact that U.S. citizens pay so much more for brand-name prescription drugs than citizens in any other Western country is the real source of much of the bad press and controversy which hangs over the pharmaceutical industry like a cloud. CMS may be in a position to do a great deal about this. All it will take is leadership willing to use CMS’s real marketing and regulatory power for the physical and financial health of Medicare beneficiaries. Certainly, an aggressive approach to drug and medical device reimbursement by CMS coupled with an amended MMA allowing CMS to negotiate for lower prices for prescription drugs would be a potent combination for controlling health care costs.

*Health Law Perspectives* (September 2008), available at:
http://www.law.uh.edu/healthlaw/perspectives/homepage.asp