



# Risk in Perspective

## The FDA's Oversight of Pharmacoeconomic Claims

*While the FDA has an obligation to protect society from the adverse consequences of misleading promotional material, it also has a responsibility not to impinge on the free and fair exchange of potentially useful information.*



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As competitive pressures rise in the United States health care system, pharmaceutical companies are increasingly disseminating pharmacoeconomic (PE) studies to provide evidence that their products are cost-effective. These studies, which assess the resources consumed and health benefits associated with the use of drug therapies, offer potentially valuable insights to decision makers in health care. However, given the sponsorship of many studies by the pharmaceutical industry, some observers have complained about the poor quality of some analyses, and the potential for biased results. The issue concerns the Food and Drug Administration (FDA), because of the Agency's authority to ensure that promotional materials involving pharmaceuticals are not inaccurate or misleading.

This issue of *RISK IN PERSPECTIVE* explores the issues involved in evaluating and regulating claims made about the cost-effectiveness of pharmaceuticals. In particular, we assess the role that the government should play in regulating information, and how a risk-analytic viewpoint can be of assistance.

### The FDA and Pharmacoeconomics

Until recently, there has been little guidance from the FDA over what constitutes a valid PE claim. The Agency has now issued draft guidelines for public comment, which would require that PE studies satisfy a specified standard of scientific rigor and validity—typically demonstrated by two well-controlled studies—to support cost-effectiveness claims. In releasing the guidelines, FDA officials noted that the risk in failing to ensure accuracy in PE claims was an increase in health costs and a deterioration of care. Their argument is that without a strong regulatory presence, drug companies may make misleading or false statements, and that users of cost-effectiveness information—physicians, patients, and payors—could select an inferior or harmful medication.

### The Costs of Regulation

When assessing the draft guidelines, it is useful to consider the role of regulation in promoting efficiency in the health sector. While the FDA has a legal obligation to protect society from the adverse consequences of misleading promotional

material, it also has a responsibility not to impinge on the free and fair exchange of potentially useful information. There are penalties borne by society when the government pursues one objective at the expense of the other. The challenge for the FDA is to identify a policy that strikes an appropriate balance.

The societal costs of any regulation can be separated broadly into two categories: costs associated with adherence, and costs associated with unfavorable events. The costs of adherence include the costs incurred by the government in administering and enforcing regulations, and costs incurred by manufacturers in complying with them. The costs of unfavorable events are the detrimental effects associated with "mistakes." Both categories of cost are sensitive to the stringency of the regulatory environment but in opposite directions: less stringent regulations impose low costs of adherence, but increase the risk (and costs) associated with adverse outcomes; greater regulatory oversight raises the costs of enforcement, but lowers the chances (and costs) of unfavorable events.

In our view, the proposed FDA guidelines may impose unnecessarily high costs in several ways. They may drive up adherence costs by stipulating in too much detail the nature of information that will be needed to support PE claims (e.g., two well-controlled studies). The downside is that fewer claims will be made, thus providing less information to health care decision makers. In fact, there may be other worthwhile, and less expensive, configurations of the information that could support PE claims (e.g., epidemiological and cost analysis based on secondary data). Overly prescriptive guidelines are also likely to be costly to administer for the FDA. Since drug prices and treatment strategies vary across payors and change over time, the Agency will likely have a difficult time discerning whether a claim is valid in any particular circumstance.

In contrast, there are fewer grounds to worry about the unfavorable consequences of using a cost-ineffective drug. The chance of making unwise choices has diminished in recent years, since pharmaceuticals are increasingly sold to, and evaluated by larger, more sophisticated

purchasers. Such purchasers include pharmaceutical benefit managers and managed care plans' pharmacy and therapeutic committees.

# The Case for Private Sector Review

In our view, the FDA should function only as a policing and enforcing authority, attempting to ensure that promotional materials are not misleading. The FDA should not, however, *evaluate* cost-effectiveness claims itself, and should instead leave this function to the private sector.

Managed care plans could determine for themselves the validity of PE claims. Alternatively, other private firms could evaluate PE information and package it in a useful form for consumers. Market forces, not the FDA, would dictate the appropriate format and content of the information. In fact, this model works exceedingly well for a number of other industries. Consider two examples: the securities and consumer products markets.

In the case of securities, the Security and Exchange Commission (SEC) serves as an enforcement agency, and regulates the format and dissemination of the bond issuer's prospectus. However, private firms such as Moody's and Standard and Poor's evaluate the quality of bonds, essentially conducting "cost-effectiveness analyses" for investors. Other firms publish a variety of informational products, many of which constitute cost-effectiveness analyses of sorts.

A similar situation exists for consumer products, though with different institutional arrangements. The government establishes and enforces minimal safety standards. Agencies such as the Consumer Products Safety Commission (CPSC) promulgate regulations (e.g., flammability standards for children's sleepwear). A separate agency, the Federal Trade Commission (FTC), oversees truth-in-advertising laws. But private organizations, such as Underwriters Labs evaluate the quality of products and disseminate information. Private publications, such as *Consumer Reports*, rate the "cost-effectiveness" of products.

The integrity of these markets stems from three conditions: 1) the existence of informed buyers and sellers; 2) the free exchange of information; and 3) the existence of a government entity establishing minimal standards and enforcing truth-in-advertising laws. If the conditions are

satisfied, the source of funding for the evaluator becomes secondary.

The question for the pharmaceutical market is whether an analogous situation might be expected—or encouraged—to blossom. The rise of managed care purchasers provides cause for optimism, since they would help ensure that evaluators are independent and objective. The FDA could still formalize minimal standards over PE claims, requiring, for example, that communications be truthful and contain appropriate disclosures. Furthermore, other system safeguards, such as the right to sue over misrepresentative advertising, would still exist.

But drug companies should have their technical analyses certified for authenticity by independent private firms or consultants with expertise in cost-effectiveness analysis. Congress could even compel companies to certify their PE claims from an accredited organization for a "zone of reasonableness" before the claims were made. The certification would create the legal presumption of a lawful claim. The FDA would regulate the behavior of the certification industry, though not the claims themselves.

# Conclusions

The changing health care environment, with new forms of promotional materials such as PE claims, force us to reconsider the government's role. On the part of the FDA, there is a need for both caution and flexibility. Above all, regulation of pharmacoeconomic claims needs to recognize the usefulness of cost-effectiveness analyses—in a variety of forms—to assist decision makers in making resource allocation choices.

This issue of *RISK IN PERSPECTIVE* is based on research conducted at the Program on the Economic Evaluation of Medical Technology (PEEMT), which was recently formed at the Harvard Center for Risk Analysis. The mission of the program is to promote informed decision making with regard to the use of pharmaceuticals, devices, and medical procedures. PEEMT's work will expand upon the Harvard School of Public Health's historic strength in the application of risk-benefit and cost-effectiveness analyses to medical practices.

Requests for a full report on this topic should be sent to Peter Neumann, Harvard Center for Risk Analysis.

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# FURTHER READING:

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from the AIDS Experience,"  
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B. Kuhlik. "The FDA's  
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Communications in the Context  
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# PEER REVIEWERS:

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TABLE 1:  
Regulatory Authority and Evaluation of Information Across Industries

	INDUSTRY		
	Securities	Consumer Products (Toaster, In-Line Skates)	Pharmaceuticals
Federal Authority	SEC	CPSC/FTC	FDA
Evaluator	Moody's/S&P	Underwriters Labs Consumer Reports	??
Institutional Purchasers	Investment Houses	Department Stores Warehouse Clubs	HMOs/Provider Networks
Consumers	Private Investors	Consumers	Employers/Patients