



Risk in Perspective

ARE PHARMACEUTICALS COST-EFFECTIVE? LESSONS FOR THE MEDICARE DRUG BENEFIT DEBATE



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"Our research reveals that some drugs save money or are cost-effective, but the issue depends critically on the context in which the drug is used and the intervention with which it is compared."

Are prescription drugs cost-effective? The question has been part of the ongoing debate in Washington over whether and how to add an outpatient prescription drug benefit to the Medicare program, as well as the larger national discourse over appropriate coverage and reimbursement policies for prescription drugs.

Some have argued that drugs provide good value -- even saving money in the long run in many cases by averting other more expensive health care services -- and thus warrant their prices. Indeed, officials from the pharmaceutical industry have argued that drug expenditures have risen faster than other types of health care spending in recent years precisely because pharmaceuticals are cost-effective (and therefore their use has been encouraged more heavily by cost-conscious managed care plans).

Cost-effectiveness-like arguments have also been mentioned in other

arenas. For example, the New York Times recently quoted an internal Clinton Administration document stating that for every dollar spent on drugs there would be \$3.50 in savings on hospital expenditures, a claim repeated elsewhere by analysts in the popular press.

This issue of Risk in Perspective reports on an extensive review of published research on this question by investigators at the Harvard Center for Risk Analysis. The research reveals that some drugs save money or are cost-effective, but the issue depends critically on the context in which the drug is used and the intervention with which it is compared. Further, the terms cost-effectiveness and cost-savings have important distinctions that are sometimes confused in the debate.

Defining terms. Over the years, people have often confused the terms cost-effective and cost saving. A drug that is "cost-

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saving" is one that saves more money than it costs to administer. For example, drug A is cost saving if it costs \$1, but the health care system saves more than \$1 in averted hospital care.

"Cost-effective" drugs would include cost-saving drugs but also drugs that do not save money but are valued because their health benefits outcomes are judged to be worth their additional costs.

Measuring the cost-effectiveness of health and medical interventions has been an active field of research in recent years, with many analyses focused on pharmaceuticals. While the evaluations have taken on a number of forms, cost-effectiveness analysis has emerged as the recommended analytic technique for the field. The appeal of cost-effectiveness analysis is that it provides a convenient means of quantifying both economic and health benefits in a single ratio. Further, if the numerators and denominators of cost-effectiveness ratios are reported in standard terms and obtained using comparable methods, then they inform decisions about how to allocate health-care resources efficiently across many competing health interventions.

An important point is that a drug is not intrinsically cost-effective or cost-ineffective. It is only meaningful to say that a drug is cost-effective compared to something else. A drug prescribed to lower an individual's blood pressure may in fact be cost-effective compared to the option of no treatment, but not necessarily when compared to an alternative intervention, such as an intensive program of diet and exercise, or another medication. Similarly, claims of cost-effectiveness often depend on the population under investigation. For example, statin drugs used to lower an individual's cholesterol have been found to

be relatively cost-effective as secondary prevention in persons with existing heart disease, but considerably less cost-effective as primary prevention. Therefore, it is not meaningful to say that drugs as a class are "cost-effective," as some observers have asserted or implied.

In recent years, leaders in the field have recommended that cost-effectiveness analysis take the form of "cost-utility analysis," in which results are presented in terms of costs per quality-adjusted life years (QALYs) gained. QALYs provide a convenient means for capturing gains from both prolongation and quality of life in a single health outcome, and explicitly incorporate the value that people place on different health outcomes.

Developing a database of cost-utility analysis. Researchers at the Center for Risk Analysis recently developed a database of published cost-utility analysis. The database was constructed through an extensive search for original cost-utility analysis using publication databases such as MEDLINE through the year 1997. For each study, detailed descriptive data were abstracted on: the intervention under investigation, the methods used to estimate costs, effects, and preference weights, and the degree to which the article met recommended protocols for reporting and discussing findings. The final database contains 228 studies and over 700 cost-utility ratios (the number of ratios exceeds the number of studies, because some articles contained more than one usable comparison.)

The cost-effectiveness of pharmaceuticals. Our database reveals that cost-utility analysis of pharmaceutical therapies have been published widely in recent years. Of the 228 studies in our database, 73 (32%) are of pharmaceutical therapies, compared

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Table 1: Selected Cost-Effectiveness Ratios for Pharmaceuticals

Reference	Intervention vs. Base Case in Target Population	\$/QALY (1998\$)
Sarasin FP & Eckman MH, 1993	Long-term anticoagulant therapy vs. observation in lung cancer patients with acute deep venous thrombosis	Cost-saving
Tsevat J et al., 1995	Captopril therapy vs. No captopril in 80-yo patients surviving myocardial infarction	\$4,000
Rose DN et al., 1990	One-year course of isoniazid (INH) chemoprophylaxis vs. No INH chemoprophylaxis in 55 yo white male tuberculin reactors w/no other risk factors	\$18,000
Oster G et al., 1994	Ticlopidine vs. ASA in 65 yr old with high risk of stroke	\$48,000
Desch CE et al., 1993	Chemotherapy vs. No Chemotherapy in 75-yo with breast CA	\$58,000
Zbrozek AS et al., 1994	Antiemetic therapy with ondansetron vs. Antiemetic therapy with metoclopramide in 70-kg patient receiving cisplatin chemotherapy (≥ 75 mg/sq.m) who had not been previously exposed to antineoplastic agents	\$460,000

Adapted from PJ Neumann *et al.* "Are Pharmaceuticals Cost-Effective? A Review of the Evidence" *Health Affairs* 2000; 19(2): 92-109. www.projhope.org/HA/ Reprinted with permission.

to 41 (18%) surgical procedures, and 26 (11%) diagnostic procedures.

Table 1 displays cost-utility ratios for selected drug therapies. The Table shows that the results vary substantially across therapies from those that are cost saving (e.g., long-term anticoagulant therapy vs. observation in lung cancer patients with acute deep venous thrombosis) to those that have positive but relatively low cost-effectiveness ratios (e.g., captopril therapy versus no treatment in 80-year-old patients surviving myocardial infarction with a ratio of \$4,300/QALY) to those that have relatively high cost-effectiveness ratios (e.g., antiemetic therapy with ondansetron versus antiemetic therapy with metoclopramide in certain patients receiving cisplatin chemotherapy, with a ratio of \$460,000/QALY).

Policy implications. The data presented here underscore several important points about the cost-effectiveness of pharmaceuticals. First, a great deal of information on the topic has become available to policymakers in recent years.

Unlike many unsupported assertions made over the years about the "cost-effectiveness" of drugs or other medical practices, these studies quantify costs and health effects using clinical and economic data and a standard, well-accepted methodological technique.

Second, according to published, peer-reviewed articles, many drugs are indeed cost-effective. Examples in our database of cost-utility analysis include warfarin therapy to prevent stroke in patients with atrial fibrillation, immunosuppressive drugs for patients with kidney transplants, and treatment with mood-altering drugs for patients suffering from depression. These interventions provide good value in the sense that they produce health benefits for relatively little cost, or may actually save money for the health care system.

Third, as noted, cost-effectiveness does not mean cost-saving. For similar reasons, one would not expect a Medicare drug benefit to save money, despite the fact that individual drugs may produce savings in certain situations. The reason is that adding

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FURTHER READING

Neumann PJ, Sandberg EA, Bell CM, Stone PW, Chapman RH. Are pharmaceuticals cost-effective? A review of the evidence. *Health Affairs*. 2000;19(2):92-109.

Chapman RH, Stone PW, Sandberg EA, Bell C, Neumann PJ. A comprehensive league table of cost-utility ratios and a sub-table of "Panel-worthy" studies. *Medical Decision Making*. In press.

Neumann PJ, Stone PW, Chapman RH, Sandberg EA, Bell CM. The quality of reporting in published cost-utility analysis, 1976-1997. *Annals of Internal Medicine*. In press.

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a drug benefit for the millions of elderly Americans without coverage will result in expanded use of cost-increasing drugs, including expensive break-through products that are likely to be cleared for marketing in the near future. Some of these drugs will be "cost-effective" in the sense that their health benefits will be worth their additional costs, though it is also likely that some will be used in situations where they greatly increase costs at little or no additional health benefit.

In interpreting our data, it is important to emphasize that cost-utility analysis represent only a subset of all economic evaluations. Moreover, some have criticized the use of cost-utility analysis on theoretical and practical grounds.

Nonetheless, cost-utility analysis has the distinct advantage over other approaches of incorporating a set of methodological standards and permitting meaningful comparisons across diverse interventions. While debate continues over measurement and conceptual issues, consensus has emerged over the basic methodology, and the technique has been recommended by leaders in the field in the U.S. and abroad.

Options for Medicare. With respect to cost-utility analysis, Medicare might consider several options. One is to conduct or fund such analysis and disseminate the information to managed care plans on to the private sector-pharmacy benefit managers who may

oversee the drug program and negotiate discounts in the future. In the U.S., several managed care plans have already adopted or are considering formal pharmacoeconomic guidelines, to help guide coverage and payment policies, a popular approach overseas. Medicare might also convene an advisory group on the issue to learn from the experience of other payers and to chart a course for the future.

A number of changes would help the future discourse on the issue. One involves more precise use of the term cost-effectiveness. For cost-effectiveness analysis to be useful to policymakers, they should be framed as specifically as possible. There is also a need for better studies in the field. Independence on the part of study investigators is also important.

A great deal of information about the cost-effectiveness of pharmaceutical therapies has become available to policymakers in recent years. Our new database of cost-utility analysis underscores the mixed results: some drugs reduce costs and improve health outcomes, while others increase costs and improve health by varying degrees, but the issue depends critically on the context in which the drug is used and the intervention to which it is compared.