Study examines use of clinical and cost-effectiveness data for drug coverage decisions

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A comparison of national agencies that play a role in determining drug coverage decisions in Britain, Canada and Australia finds that uncertainty regarding clinical effectiveness is a key issue in coverage decisions, with other factors including the ability to negotiate price and societal values, according to a study in the October 7 issue of *JAMA*.

"Expenditures on pharmaceuticals are the fastest growing sector within health care in developed countries, including Canada, the United Kingdom, Australia, and the United States, where federal expenditures for Part D of Medicare and Medicaid are projected to reach $4299 billion cumulatively from 2010 to 2014," the authors write.

"In an attempt to control expenditures and to assess the value of new drugs, many countries, including Britain (National Institute for Health and Clinical Excellence [NICE]), Australia (Pharmaceutical Benefits Advisory Committee [PBAC]), and, most recently, Canada (Common Drug Review [CDR]) have established agencies to determine whether new pharmaceutical treatments should be listed in public formularies," according to background information in the article. "NICE, PBAC, and CDR have included cost-effectiveness as part of drug coverage decisions, whereas drug reimbursement decisions within publicly funded health care (Medicare and Medicaid) in the United States largely exclude consideration of cost and cost-effectiveness at present."

Fiona M. Clement, Ph.D., of the University of Calgary, Canada, and colleagues examined the key issues facing three national agencies that use effectiveness and cost-effectiveness data in evidence-based coverage of pharmaceuticals. The researchers analyzed data through December 2008 from the CDR, NICE, and PBAC, and noted listing recommendations for drugs by disease indication.

The researchers found that NICE recommended 87.4 percent (174/199) of submissions for listing compared with 49.6 percent (60/121) for the CDR and 54.3 percent (153/282) for the PBAC. "The list rates for CDR and PBAC were lower when there was considerable clinical or economic uncertainty. In addition, the use of a relevant clinical end point was associated with a higher probability of recommending coverage for the CDR and PBAC."

"Significant uncertainty around clinical effectiveness, typically resulting from inadequate study design or the use of inappropriate comparators and unvalidated surrogate end points, was identified as a key issue in coverage decisions. Recommendations varied considerably across countries, possibly because of differences in the medications reviewed; different agency processes, including the willingness to negotiate on price; and the approach to 'me too' drugs. The data suggest that the 3 agencies make recommendations that are consistent with evidence on effectiveness and cost-effectiveness but that other factors are often important," they write.

"What can be learned from this study by the United States or other health care systems regarding pharmaceutical reimbursement? First, the existence of these 3 agencies confirms that it is feasible to establish an agency that considers comparative effectiveness in pharmaceutical reimbursement decisions. … Second, the differences that exist in the processes of these agencies confirm that they can be adapted to local health care circumstances. … Third, a primary concern in the United

States appears to be that the use of comparative effectiveness and cost-effectiveness would reduce choice in therapeutic options. As illustrated by ranibizumab [a high-cost medication for age-related macular degeneration that each of the agencies recommended listing], the use of cost-effectiveness in coverage decisions need not be an undue barrier to drug funding, even for expensive medications, when there is robust evidence of effectiveness, at least in some patient subgroup, or where there are factors that appeal to the values of decision makers beyond the simple metric of cost and health gain."

"Perhaps the main lesson from the experience of the 3 countries is that systematic, durable, and widely accepted decisions can be made using comparative effectiveness and cost-effectiveness, although it is evident that other information beyond these 2 criteria can be incorporated into decision-making. Given that the number of expensive, targeted pharmaceuticals for cancer and other chronic conditions is increasing, pharmaceutical reimbursement will continue to be a key challenge to formularies in all countries," the authors conclude.

More information: *JAMA*. 2009;302[13]:1437-1443.

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