COST-EFFECTIVENESS ANALYSIS IN HEALTH POLICY

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I am a strong advocate of the formal tools of regulatory analysis, such as risk analysis, decision analysis, cost effectiveness analysis and cost benefit analysis. I believe these tools can help us accomplish more public health and medical protection at less cost than will occur when decisions are made without good analysis. Although formal analyses do have much insight to offer, it is fair to say that their influence on practical decision making in both the public and private sectors has been limited to date. The following are steps that groups like ISPOR can take to increase the influence of these analytic tools in the policy process. I am tempted to suggest, after ten months of experience, that the real reason lies in the ratio of professional affiliations in the regulatory arms of the government, which run about five lawyers for one analyst, or at least that is what I have been sensing in my initial experience in government. But I do think there are some deeper intellectual and institutional reasons why these types of analysis that we champion have a limited impact, and I would like to suggest a few of those ideas for your consideration.

First, there is the enormous challenge of assessing the likelihood and severity of a health threat, particularly an emerging health threat. Sometimes there is ample basis for public or clinical concern about a hazard, but there may be very limited historical basis for determining the precise magnitude, whether measured in probability or severity. Examples that easily come to mind are bio-terrorism, mad cow disease, and antibiotic resistance. In order to perform good risk assessments of these threats, we need much better information about the most susceptible subgroups in society because exposures to these groups may determine the overall public health significance of an emerging hazard.

A closely related issue is identifying the most important sources or causes of health problems. Thinking back from being raised as a young child I recall being instructed by my parents of the merits of margarine relative to butter, and of course now we are increasingly learning of the heart disease risks associated with trans fatty acids, often of significant concentration in margarine. We need to have risk assessment information, very precise risk assessment information on what does one gram per day of trans fat mean in your diet, compared to one gram per day of saturated fat. We suffer the same needs for risk assessment information in the environmental health field. In the case of particulate air pollution, we often make the simple assumption that all fine particles in the air are equally toxic regardless of their precise diameter or chemical composition and yet fine particles from difference sources, say motor vehicles and power plants, vary somewhat in their typical size distribution and their chemical content. As modelers, we

often make the assumption that all particles are equal because it is analytically tractable, not because it is biologically plausible. Indeed it is quite possible that some particles are more toxic than others, information that could play an important role in setting priorities and doing good analysis.

Which institutions in society should be responsible for this applied risk assessment research function? One might think that university-based scientists could offer analytic solutions to these challenges and they often do. Yet these emerging health issues, including the identification of sources and causes, often require collaboration of scientists from multiple disciplines. And having spent 20 years in three universities, my experience tells me it is often times difficult to get the university community organized to offer those multidisciplinary teams. It also entails a willingness to develop and validate mathematical models that provide speculative yet useful forecasts about what is possible or probable. Yet in many universities, this type of modeling exercise is not necessarily the most attractive academic pathway. I am encouraged that organizations like the National Science Foundation and the NIH do make available certain types of grants to support this applied risk assessment research, and I am aware that many of the emission-oriented agencies also do. But I also believe that it is critical for the public and private sectors to work together to support first-rate peer reviewed science in these areas. While very diverse, organizations such as the Health Effects Institute, the International Life Sciences Institute, Research Triangle Institute, the Chemical Industry Institute of Toxicology and the Electric Power Research Institute have one common feature, which is a commitment to bring public and private sector people together to work with university scientists on these issues. We need to work harder to strengthen the scientific quality, credibility, and policy relevance of these applied research organizations to inform the public and private sectors.

Second, going beyond health risk assessment, we need better tools to compare the health benefits of policies to their economic costs. You might think of this field as health risk evaluation. By evaluation I mean the analytic process of scoring or rating health affects in terms of their overall burden on the patient and on society and quantifying the benefits of health policies in monetary or other units that capture the preferences of the public. The central intellectual challenge is to account for the adverse affects of both morbidity and mortality in some single numerical index, recognizing that some bouts of sickness are more severe than others, and some deaths may be considered more tragic than others.

Although answers to these questions require value judgments, the social sciences have much to offer in providing possible answers. In the developing world, the World Health Organization has promoted a metric called the disability adjusted life year, or the DALY. Diverse health problems ranging from infectious diseases to trauma are scored in terms of the number of DALYs that a society loses. The scoring of each health condition is based on three factors: the number of life years lost compared to the Japanese experience, weights applied to each healthy life year to reflect productivity at work and at home, and weights for each unhealthy life year are applied to capture the degree of functional limitation imposed on a person. Although my description of the DALY has been very

simplistic, the tool continues to be refined in various ways and its use is increasing around the world.

A precursor to the DALY, often favored by my colleagues at the Harvard School of Public Health, the quality adjusted life year, or QALY, is now the standard measure of health policy effectiveness used by many academics. It is similar to the DALY but differs in three important ways. First, the loss of life expectancy from each condition is derived either from actuarial or experimental data or modeling for a relevant target population, rather than from the Japanese experience. Second, each year of healthy life is weighted the same, regardless of when in the lifespan that loss occurs. This assumption is defended on grounds of fairness and analytic simplicity, though it is a very different assumption that that embedded in DALYs. Finally, the weights for different health states are typically derived in surveys of community residents or patients who have experienced the condition.

Early work on QALYs was very simplistic in its use of utility theory, clinical understanding and insights of survey methods, but I think it is fair to say that the sophistication of QALY-oriented research is improving. Both the DALY and QALY methods, due to their very assumptions, place more emphasis on health risks affecting middle age adults and young people than risks of chronic diseases of old age. This emphasis is not necessarily consistent with the current patterns in the U.S. health care system, where we invest billions of dollars late in the lifespan. And of course there are economic critics of DALYs and QALYs who argue that these methods depart from classical economic assumptions in ways that current investment patterns reveal. For example, a standard economic view is that consumer sovereignty should be respected. If well informed senior citizens who often have substantial assets and previous few remaining life years have a large demand for modest health gain, so be it. And there is a growing literature in economics aimed at quantifying the monetary benefits of reducing different diseases and health impairments.

Yet, these willingness-to-pay tools face a host of ethical and technical criticisms. I heard them each spring from my students at the Harvard School of Public Health, when we taught these analytic methods. At the most basic ethical level, concerns have been raised about whether a person's income level or asset position should be permitted to influence how much a governmental study values their health status. Others have argued that a fair allocation of public resources throughout the lifespan should be determined in a so-called veil of ignorance where people's views are not influenced by current age or health status. Yet there are certainly some practical problems with implementing that idea. At a technical level, substantial progress has been made in estimating willingness to pay for health protection. Questions have also been raised about whether stated willingness to pay by survey respondents is adequately sensitive to the amount of risk reduction and the content of the risk reduction. In light of these many difficulties you may find it interesting that OMB does not currently require agencies to value health gains and losses in monetary units. Some agencies such as the Occupational Safety and Health Administration and the National Highway Traffic Safety Administration have refrained from this practice for several decades. I believe that we need to have a greater degree of

consensus from within the analytic community about how we should proceed on these health risk evaluation questions and I urge that some form of conference be assembled and generated, sponsored by several federal agencies, to try to bring some degree of road map for the field and to lay out some guidelines for practice. I think this professional society could be a very important contributor to that activity.

What has happened in OMB in this general area of analytic practice? We see health policy occurring at a variety of federal agencies, FDA (Food and Drug Administration), EPA (Environmental Protection Agency), USDA (US Department of Agriculture), CMS (Center for Medicaid and Medicare Services), CDC (Centers for Disease Control), to name just a few. These agencies are now using a wide rage of analytic practices and quite frankly they are not always mutually consistent. Rarely are these analytic practices rooted in statute, which in a sense is good news because it means there is opportunity -- without changing underlying laws -- to bring some more reason and consistency to this process. OMB does have an important role to play in fostering some degree of consistency. Yet agencies sometimes have important reasons for doing things differently, and OMB's challenge is to appreciate when these distinctions have merit. In the Bush administration, we support development of health policies and regulations that are based on sound science and economics. We are certainly not allergic to regulation; indeed, we view it as a critical tool of national health policy, as critical as government spending and taxation. My office in particular strives to prompt and approve good health regulations, while improving flawed ones and stopping harmful ones. We see better formal analysis as a critical step towards more effective, fair, and efficient healthy policies.

In my first year at OMB we have taken two modest steps to promote more analytic rigor and consistency in health policy throughout the Federal Government. You can learn more about these issues on the OMB web site; however, let me conclude by summarizing them. First, OMB recently established analytic guidelines for health risk assessment that cover all the federal agencies. The guidelines are based on two rounds of public comment and interagency review and were derived from principles found in the Safe Drinking Water Act Amendments of 1996. And although that might seem very specific, if you consult those amendments, you will find that there is a wide degree of general applicability to those principles. When these guidelines take effect in October of this year (2002), the public will be provided an opportunity to challenge any health risk information, disseminated by a federal agency that does not adhere to these guidelines. Second of all, in collaboration with the Council of Economic Advisors, OMB has initiated a process to refine the guidance we provide to agencies on how to do good regulatory analysis, economic impact analysis, decision analysis and so forth.

In the first phase we have asked for public comment by the end of this month on specific analytic issues, including health risk evaluation, that should be addressed in OMB's refinement of its existing guidance. And we would look forward to comments from any of you on that process. By the end of May 2002, you can comment through the internet. We will prepare a proposed revision of OMB's analytic guidance and we will release it for public comment, interagency review, and external peer review. My office will use the

OMB guidance to judge whether the regulatory analysis prepared by agencies are adequate.

Looking forward, in order to enhance the roles of science and economics and health policy, we must do something that is as obvious as it is critical: Improve both the technical and ethical foundations of the tools we use to inform policy makers. Organizations like this one have a critical role to play in that process and we at OMB look forward to working with you.