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# Approaches to Aggregation and Decision Making—A Health Economics Approach: An ISPOR Special Task Force Report [5]



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#### ABSTRACT

The fifth section of our Special Task Force report identifies and discusses two aggregation issues: 1) aggregation of cost and benefit information across individuals to a population level for benefit plan decision making and 2) combining multiple elements of value into a single value metric for individuals. First, we argue that additional elements could be included in measures of value, but such elements have not generally been included in measures of quality-adjusted life-years. For example, we describe a recently developed extended cost-effectiveness analysis (ECEA) that provides a good example of how to use a broader concept of utility. ECEA adds two features—measures of financial risk protection and income distributional consequences. We then discuss a further option for expanding this approach—augmented CEA, which can introduce many value measures. Neither of these approaches, however, provide a comprehensive measure of value. To resolve this issue, we review a technique called multicriteria

decision analysis that can provide a comprehensive measure of value. We then discuss budget-setting and prioritization using multicriteria decision analysis, issues not yet fully resolved. Next, we discuss deliberative processes, which represent another important approach for population- or plan-level decisions used by many health technology assessment bodies. These use quantitative information on CEA and other elements, but the group decisions are reached by a deliberative voting process. Finally, we briefly discuss the use of stated preference methods for developing "hedonic" value frameworks, and conclude with some recommendations in this area.

**Keywords:** aggregation, cost-effectiveness, equity, multi-dimensioned benefits.

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### Introduction

This section identifies and discusses two types of aggregation issues. One arises from the aggregation of cost and benefit information across individuals to a population level for benefit plan decision making. The other deals with the combination of multiple elements of value into a single value metric for individuals. We assess both issues here.

Regarding the first, cost-effectiveness analysis (CEA) and (more broadly) cost-benefit analysis (CBA) share common origins and common deficits. Because they originate in utility maximization for a single individual, they cannot accommodate societal issues that involve (among other things) the distribution of costs and benefits across a population. For example, the US 1939 Flood Control Act specified that water projects could be undertaken only when "the benefits, to whomever they accrue, [be] in excess of the estimated costs." These "distributional issues"

remain outside the domain of traditional CEA and CBA. Thus, the problem is how best to aggregate the benefits enjoyed and the costs borne by individuals into a societal statement of value. Bator [1] proposes (in effect) a benign dictator whose utility function includes the utilities of individuals in the population, allowing different weights for different people, but this is seldom if ever operationalized meaningfully.

Even the question of "distribution" can have multiple meanings. For example, regarding the health of different groups in a population, does "equity" mean equal access to health care or does it mean equal health outcomes? The latter approach implies a greater emphasis on caring for population subgroups with poor health status than does the former. These different meanings of equity have different implications for the way one might prioritize various health interventions.

The second class of aggregation issues appears when the definition of value has more than a single element or dimension.

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CEA and CBA each have a single metric of value—cost per quality-adjusted life-year (QALY) (or some similar measure of health benefit such as disability-adjusted life-year [DALY] or others) or net monetary value (in CBA). Some people object to having the analyst monetize health benefits as required by CBA, and hence prefer CEA. When multiple dimensions of value arise, CBA and CEA have no way to formally incorporate them (except, in CBA, by monetizing them when feasible). Thus, this raises the issue of aggregation across value components.

In the article by Garrison et al. [2], we described a number of decision contexts and perspectives potentially relevant to considering and estimating value in a microeconomic framework. The two types of aggregation issues are then related in that the aggregation of elements of value at the individual level becomes a necessary input for the aggregation at the population level. As mentioned, most CEAs—at least those assessing new biopharmaceutical products—have been oriented to the normative question of whether a health plan should adopt a new technology. Thus, they can be an important element in health technology assessment (HTA), which often considers a broader range of components or issues.

We discuss these issues here, organizing this section as follows. In the first section, we argue that additional elements could be included in measures of value, but such elements have not generally been included in measures of QALYs. As a specific example, in the second section we describe a recently developed methodology called "extended" CEA (ECEA) that provides a good example of how to use a broader concept of utility, which has been applied to some specific interventions. ECEA adds two features-the element of financial risk protection (one part of insurance value assessed in the article by Garrison et al. [2]) and income distributional consequences. The third section discusses further options for expanding this approach (what we call "augmented" CEA [ACEA]). In the fourth section we discuss another, even broader approach called multicriteria decision analysis (MCDA) that has some intuitive appeal, especially for decision makers who consider other elements or components not easily measured in CEA or CBA. This approach requires that decision makers place quantitative weights on the various criteria, depending on their relative importance for a decision. This exercise can make group decision making more transparent. The fifth section discusses budget-setting and prioritization in these frameworks, building upon the previous discussion in the article by Danzon et al. [3] discussing the situation in which budget allocations and decision-making cutoffs are not perfectly aligned. The sixth section discusses deliberative processes that represent another important approach for population- or planlevel decisions that is used by many HTA bodies. It uses quantitative information on CEA and other elements, but in the end, the group decision is reached by a deliberative voting process. This section also includes a brief discussion of alternative voting methods that deliberative bodies might use.

# Additional Value Elements: Conceptually Appealing but Currently Impractical to Implement

There remain a number of these other elements, some discussed in the article by Lakdawalla et al. [4], that analysts in concept could include in a CEA (or CBA) structure, but cannot meaningfully do so because relevant data do not exist, and are impractical for real-world analysts at present. Consider, as an example, a vaccine against or the treatment of a virulent disease such as Zika and Ebola or historical predecessors such as leprosy, tuberculosis, poliomyelitis, severe acute respiratory syndrome, and AIDS. Public policy in these cases is often driven by issues such as public fear of contagion that are not readily captured in

traditional CEA models. Such models normally do not include the entire at-risk population and their disutility from the threat of the disease. The missing element is the loss of utility associated with the potential risk of exposure to the disease—the "fear factor." This fear has dominated public policy toward these diseases for millennia, resulting in isolation colonies for people with leprosy, quarantine rules for tuberculosis, travel bans during severe acute respiratory syndrome epidemics, and many other similar actions. An associated issue is the utility gained from permanently eliminating the disease, raising a further important issue—namely, aggregating costs and benefits across future generations of the population.

An additional array of potential value elements fall into this general category of "conceptually feasible but generally impractical" for use in CEA or CBA without further research and testing. This includes, for example, things such as fit with existing infrastructure/programs; availability of requisite trained personnel; improvement in children's school participation and highest grade attainment (and hence future earnings); ethical considerations (e.g., involving end-of-life alternatives or manipulation of genetic material); and—for an individual patient—fears associated with specific types of therapies (e.g., radiation and genetic modification). It also includes other elements discussed in the article by Lakdawalla et al. [4]. In what follows, we discuss methods that could be used to include both these less tractable and the more traditional value elements in a consolidated valuation

#### **Extended Cost-Effectiveness Analysis**

In principle, one can conceive of estimating or eliciting from individuals the total willingness to pay (WTP) for a defined bundle of attributes. In practice, this is often difficult to do because some key elements (e.g., the WTP for scientific spillovers, for elimination of fear, for added clinical certainty, or for less inequality) remain unknown. Absent this perfect solution, some authors have proposed strategies for aggregating a few key elements of value into a systematic framework.

An approach that also captures important aspects of equity, albeit not within a fully aggregated value measure, is ECEA [5]. ECEA aims to describe the value of medical interventions and policies along three specific dimensions: health gains, financial risk protection, and social cost, thus extending beyond the traditional CEA measures of health gains and costs. As a further refinement (separate from gathering information about these added dimensions of value), ECEA collects this information for different subpopulations of interest, thus allowing consideration of distribution of benefits and costs (equity and fairness). Financial risk protection reflects the reduction in the risk of financial distress due to health care costs, or the financial costs of poor health. It represents a subset of the "insurance value" concept discussed in the article by Lakdawalla et al. [4]. Cost is measured from the perspective of the society as a whole, as is common in CEA. One could envision including additional measures of value, but ECEA consists of these three. Equity reflects the value of promoting a more just distribution of access to health care or health outcomes themselves or conversely, the loss arising from expanding inequality.

ECEA evaluates the distributional effects of a medical intervention by specifying a set of population subgroups of interest. For instance, one might evaluate groups that vary by income, education, baseline health, disease status, or some other characteristics of interest. By subgroup, ECEA then reports the health gains, private expenditures avoided, and financial risk protection. Health gains can be measured using QALYs or other suitable units. Private expenditures avoided reflect the reduction in

out-of-pocket spending by subgroup. Theoretically, this includes the totality of effects on private spending, including changes in non-health-related spending. Financial risk protection is measured as expenditures avoided suitably scaled by an individual's income. The concept is that a given level of avoided expenditures has value in proportion to a person's or household's income or assets.

Concerning the concept of risk protection, to substitute for the conceptually desirable "risk premium," various metrics are possible, including expenditures averted as a fraction of income; the change in the share of individuals above a certain threshold of health care spending as a fraction of income; change in the number of individuals with disposable income falling below poverty thresholds; the WTP for the reduction in expenditures faced in a given health state, in the context of a specific consumer utility function; and so on.

The purest measure from standard welfare economics analysis would be the Pratt-Arrow risk premium, that is, the product of the absolute risk-aversion measure (r) and 0.5 times the variance of remaining out-of-pocket expenditures [6,7], both of which are difficult to measure or even approximate at the individual level. Garber and Phelps [8] reference the corporate finance literature to estimate  $r^* = -r^*$  income between 1 and 4. New work [9] using labor supply data puts the value of  $r^*$  slightly below 1, and rules out values greater than 2. Variances are difficult to estimate even with large data sets in health care because of the large coefficients of skewness and kurtosis in medical expenditure data, and the requirement of having data representing expenditure distributions for people with similar or identical insurance coverage and health risks. Much can be written about each of these approaches, but the common feature is some measure of expenditures avoided, relative to the individual's willingness to bear financial burden. None of these alternatives measure perfectly the aspect of risk that is captured by variance in financial outlays and embedded in the standard risk-aversion measure.

ECEA then reports these three measures of benefit by subgroup and then scaled by the total cost of the intervention. Thus, one might report total cases of poverty averted per dollar of total cost, or dollars of financial risk protection per dollar of total cost. These measures are analogous to the more conventional cost-effectiveness ratio of health gains (e.g., QALYs gained) per dollar of cost, but there is no standard measure of WTP for these that is equivalent to a cutoff value for cost per QALY or other CEA investment rule.

Furthermore, ECEA provides little guidance on how to choose a single metric to combine these elements of value. Indeed, the absence of a single unifying economic framework for ECEA makes this a matter of judgment rather than analysis. Although some may view the multidimensional nature of ECEA as advantageous, others may view it as a limitation, because ECEA offers no obvious way to compare dimensions such as financial risk protection and health gains. Nonetheless, ECEA adds an important tool to the literature on health care value, particularly for analysts who care about and wish to quantify issues relating to equity and risk, and it may provide a useful stepping stone to more complete approaches that we discuss in a later section.

#### **Augmented Cost-Effectiveness Analysis**

Going beyond the three dimensions of utility chosen in ECEA, one could consider adding still more measures of value in addition to health gains, social costs, and financial risk protection. To differentiate this from ECEA, we describe this approach as an ACEA, emphasizing the extra dimensions of utility captured beyond health gains, costs, and financial risk. These added elements of value might include (as examples, recognizing the risk of potential double counting) the value of physical risk protection,

scientific spillovers, the value of reducing diagnostic uncertainty for patients, option value, financial insurance value, the value of preventing incurable diseases (such as polio, Ebola, or Zika), or the value of completely eliminating a contagious disease from the earth (as with smallpox, and nearly so with polio).

There are several potential approaches to including these added elements. One is to create a separate set of elementby-element comparisons, perhaps by subgroup if relevant. Nevertheless, this approach—as with ECEA—would still make it impossible to rank one intervention above another except in cases of pure dominance (one is better than the other on all measures of value), and that becomes increasingly unlikely as more and more elements of value are added to the model. A second approach is to selectively, and systematically, add elements to either the numerator (net cost) or the denominator (net benefit as measured in QALYs or utility) as most appropriate. This approach has the advantage of retaining a cost-per-outcome ratio form, but is likely to affect what threshold is applicable. A third approach is to monetize all benefits so that a net monetary benefit can be calculated. Nevertheless, many health policy analysts and their clients find the step of monetizing health and related benefits objectionable. Indeed, that very objection led to the adoption of CEA over the previously developed CBA to valuing health-related investments. Net monetary benefit accomplishes the aggregation across different dimensions of value by using the same potentially objectionable step of monetizing each type of benefit. A fourth approach solves this problem by providing a way to aggregate the multiple elements of value into a single, nonmonetary metric—the topic we consider next.

#### **Multicriteria Decision Analysis**

Neither ECEA nor ACEA provides a single measure of value, because they cannot fully aggregate across the various dimensions of value that they describe. One approach does provide such aggregation: MCDA, building on the work of Keeney et al. [10], Saaty [11], von Winterfeldt and Edwards [12], and Barron and Edwards [13]. In general, these approaches elicit from the "decision maker" the trade-off values to incorporate issues that cannot or have not been included in CEA or CBA. Thus, these models provide a unified one-dimensional measure of value of alternative choices using a multi-attribute metric that combines the preference weights specified by the decision maker and the performance of alternative "candidates" along each of the dimensions of value. Although other approaches exist, the two most common MCDA methods used in health care are the analytic hierarchy process (AHP) and the multi-attribute utility theory (MAUT). In the simplest form (linear MAUT), the value score is a weighted linear sum of each candidate's performance along each relevant dimension. More complex models use multiplicative models. The largest differences between these approaches appear in the processes used to elicit the decision makers' value structures.

The key components of these MCDA models are the value weights supplied by the decision maker(s) and measures of performance of each intervention candidate along each specified criterion. In effect, MCDA models attempt to re-create a systematic "utility function" of the decision maker that formally expresses trade-offs between attributes of medical interventions that create value—"goods" in the usual economic sense. Nevertheless, rather than estimating them from observed behavior, as economists might normally do, they seek to elicit the trade-offs through various structured processes. These weights will likely differ considerably across different stakeholders (e.g., provider, payers, or patients) and may well differ even within any of these groups.

MCDA models have a series of specific advantages compared with less formal processes [14]. In particular, using formal MCDA models

- 1. makes the decision-making process more transparent;
- 2. guides investment in data improvement (to where it most affects decisions);
- 3. allows a "test drive" of alternative program specifications in multidimensional value space;
- 4. allows "reverse engineering" to improve product specifications;
- 5. bypasses many cognitive errors made in intuitive human judgments; and
- 6. may assist in decision convergence for divergent interests.

To be clear, we believe that specific value measures (such as QALYs) are necessary core elements in any broader MCDA model of value in health care. In some cases, these elements may dominate the model, whereas in other decision contexts, other attributes of the choices may assume greater importance (larger weights). We view MCDA primarily as a method to expand upon the elements and dimensions of value that are not readily captured in CEAs. The most obvious "omitted element" concerns issues of distribution (otherwise known as "fairness," "equity," or "disparities"). Other important elements may enter these models, particularly when they are difficult or impossible to capture in traditional CEA models. Some of these issues were discussed in greater detail earlier in the first section.

MCDA models readily allow analysts to use different perspectives while using the same basic model, merely by changing the dimensions of value (criteria or attributes) that are included in the model and the weights they receive in the value structure. Consider five of the potential perspectives outlined in the article by Garrison et al. [2]: the public (societal), providers (doctors, hospitals, etc.), payers (insurers), patients, and producers (those who create and produce medical drugs and devices). Each might choose different dimensions of value, or could share some, but with different weights. Others with more defined self-interests (providers, producers, and payers) would likely have differing perspectives [14].

Different stakeholders may well have different perspectives (and hence weights) in an MCDA, but the proper use of two perspectives seems clear to us. First, MCDA models should always be presented—at a minimum—using a societal or payer perspective for the same reasons that the societal or payer perspective should always be presented as one option in CEA models. In a specific example, health plans (either public or private) should use a societal or payer perspective in determining coverage of medical interventions (what is covered and at what cost to the patient) and reimbursement to providers. But additional (separate) perspectives can also be developed within the same MCDA structure.

Second, once a health plan determines coverage and reimbursement, a separate viewpoint may be useful—that of the individual patient. Here, MCDA models using individual patient-specific value weights could help patients choose among available medical interventions—those chosen for coverage by their health plan—using both their own value weights and their out-of-pocket costs. Such formal models might help patients choose among complex alternatives such as alternative chemotherapies for cancer, surgical versus medical intervention for some disorders, or various strategies for mental illnesses. And in creating MCDA-to-cost ratios comparable with cost-effectiveness ratios in CEA, a societal view and the patient view could readily use different measures of cost.

MCDA models differ from ECEA models in two ways. First, ECEA incorporates only a fixed set of criteria beyond health gains and social cost—the dimensions of protection against individual

risk and issues of distribution (equity). MCDA in concept could incorporate not only these but also other dimensions of value for decision makers. Second, and most distinctly, ECEA (and ACEA as well) does not provide a basis for assessing trade-offs between gains in these desirable criteria, whereas MCDA explicitly elicits decision makers' preferences and uses them to provide a one-dimensional measure of value. From this perspective, ECEA and ACEA can be seen as a subset of MCDA, but both lacking flexibility in the number of criteria included in the depiction of value as well as a method for measuring trade-offs between desirable criteria.

Another use of MCDA allows incorporation of things considered valuable to the decision maker but which cannot readily be accommodated in standard CEA, as the first section discusses earlier. In MCDA, at least some of these can be incorporated using subjective measures of how well each competing technology performs on these other dimensions of value. For example, in a vaccine program, one could state the "fit" with existing vaccine schedules on (say) a 0 to 10 scale, and incorporate that fit attribute with a chosen weight. In concept, one could incorporate this in elaborate measures of the cost of introducing alternative vaccine programs, but in practice, such detail may be impossible.

MCDA can capture other issues that remain beyond the attainable data for a full CEA or ACEA model. Most of these fall into the category of states of the world where we have no existing utility measurements (and where analysts cannot reasonably expect to acquire such measurements). Consider as an example the "fear factor" from a highly dangerous and virulent disease (such as Ebola or Zika). MCDA can incorporate subjective judgments about the ability of technologies to avoid such fear (with an appropriate weight included in the model) in lieu of attempting to measure population-level utilities when people are confronted with varying degrees of risk from such diseases. Nevertheless, such utilities, if available, could be included in an ACEA model.

Existing MCDA models suffer from a number of curable defects, which are detailed in the following subsection. Some of these affect only AHP, whereas others affect only MAUT, the two most commonly used measurement models in health care decision support. We view none of these defects as "fatal," and urge continued research to address these issues to improve MCDA performance and ease of use.

## **Budget-Setting and Resource Allocation with MCDA**

MCDA models suffer from a remaining problem. They do not currently provide a clear method for making "investment choices," for example, deciding which new technologies to add to the health benefit package. And, in general, they provide no basis for guiding budget-setting itself, in contrast to CBA and CEA.

Consider first the simplest rule of CBA: invest in or include all projects in which the benefit-to-cost ratio exceeds 1. In CEA, the standard rule says to invest if the cost per health unit gained (e.g., QALY or DALY) is smaller than the decision makers' predetermined cutoff value (and, in parallel, reduce investments in interventions with cost-effectiveness ratios exceeding the predetermined cutoff). The World Health Organization has recommended using a decision cutoff of 1 to 3 times per capita gross domestic product, although it has more recently been reviewing alternative threshold estimation approaches [17]. In England, the National Institute of Health and Care Excellence uses a cutoff of £20,000/ QALY, increasing to £30,000, and even to £50,000 in special circumstances. Other alternative approaches lead to lower or higher cutoff values (e.g., see Neumann et al. [18]).

CEA measures have and can be used to help resource allocation decisions without formally using a specific cutoff. The

common use of "league tables" to help people understand where a medical intervention or technology "fits" in the general realm of common use and practice can inform some decisions about health technology use. In these settings, an implicit CEA cutoff is used in lieu of an explicit measure. This approach, however, tacitly assumes that existing interventions in common use have passed an implicit or explicit cost-effectiveness test, which is not necessarily true.

In the world of MCDA models, one approach to prioritize investments assumes that an exogenously given investment budget is available, and recommends investing in projects with the highest multi-attribute utility score first, and proceeding down the list of available projects until the investment budget is consumed [19]. But, as noted, this approach provides no guidance as to how large the investment budget should be.

A recent International Society for Pharmacoeconomics and Outcomes Research Good Practice Task Force report on MCDA addressed the problem of budget-setting (or the parallel problem of how to recommend the best investments), discussing two specific options. These approaches either 1) include "cost" in the MCDA model with a specific (negative) weight or 2) measure the MCDA value of interventions thought to be susceptible to being removed from use [20]. The Task Force concluded that neither of these approaches was wholly satisfactory, and recommended further research.

To be clear, we recommend that MCDA models do not include costs as a (negative) attribute. As noted, using cost as an attribute was one of the approaches considered in the earlier Task Force report assessing MCDA. That report concluded (and we agree) that using costs as an MCDA attribute implies knowledge of the appropriate WTP for the value bundle, yet the MCDA framework provides no basis for making such judgments [20]. Instead, users of MCDA models should treat them as a comprehensive benefit (value) measure, independent of cost. Then (as we discuss shortly) research must develop the best methods to determine the investment budget or an acceptable threshold of MCDA value-to-cost ratio to guide investment decisions.

One new approach has subsequently been suggested [16]. This approach builds upon the availability of a WTP decision cutoff in a CEA context (such as \$100,000/QALY) and provides potential guidance for MCDA models that contain QALYs as an important attribute of value. In brief, this approach scales the CEA "cutoff value" WTP for a QALY upward to account for other dimensions of value not measured in the CEA framework. Thus, for example, if QALYs accounted for two-thirds of the weight (or value) in an MCDA model, and the WTP for a QALY CEA cutoff value was \$100,000/QALY, then the MCDA value-tocost cutoff would be 3/2 times the WTP for a QALY value CEA cutoff, or (in this example) \$150,000 per unit of MCDA value. In effect, agreed-upon values for a WTP for a QALY CEA cutoff (such as may exist) serve as a numeraire to estimate the total value of the expanded multicriteria value index. By analogy, it would be like adding new safety features to automobiles and then re-evaluating them taking the new features into account. The overall WTP for any automobile should expand with new safety features added, but the rankings of value would shift depending on how much new safety enhancement was built into any particular model, and each vehicle would then be ranked on the ratio of its new MCDA value to its cost.

In some settings, a separate issue arises: nonalignment between budgets allocated for provision of health services and the decision rule to determine acceptability of medical interventions. First, consider this issue in the familiar context of CEA. It might arise, for example, when a prototypical "Minister of Health" establishes the CEA cutoff, whereas a "Minister of Finance" establishes the budget for the health care system. Even if initially in balance, new technologies or health care interventions might

emerge that had an "acceptable" CEA cutoff, yet their inclusion into the package of services offered to enrollees might greatly exceed the budget. Drugs to treat hepatitis C appear to have created this issue in some situations, and (for example) the emergence of an effective treatment for Alzheimer's disease could well cause it to appear in the future. This issue has been described as the problem of "affordability." In this situation, the budget creates an implicit cost-effectiveness cutoff that is more stringent than the official cutoff. Economists call this the "shadow price."

In the long run, the most desirable solution would bring the budget-setting process and the CEA cutoff determination (or an expanded MCDA cutoff determination) into alignment. The official cutoff and the shadow price should be one and the same. Achieving that alignment, however, may be difficult to impossible, at least in the short run. When a misalignment occurs, it would appear that any of four logical solutions are available (none exclusionary to the others): 1) increase the budget (Future budget expansion may not be inevitable. One could imagine cases in which a high expenditure for an intervention "today" reduced subsequent-year budgets, e.g., through elimination of a contagious disease or for reduced future liver transplant costs from lower alcoholic cirrhosis. Proper intertemporal budgeting with discounting would accommodate such issues, but politically set budgets commonly do not have the appropriate temporal horizon, and thus cannot accommodate such issues readily.); 2) tighten the official CEA (or MCDA) cutoff; 3) cut back on or eliminate interventions with excessive CEA ratios (or in an MCDA world, with poor MCDA value-to-cost ratios); or 4) as a variant on point 2, estimate the shadow cutoff value implied by the budget and use that as the "real" CEA (or MCDA) cutoff. In situations such as these, resolving the lack of alignment between cutoff rules and budgets must-at least in the short and intermediate runaccount for costs of change and other issues discussed previously in the article by Danzon et al. [3].

This issue could become more prominent with the adoption of MCDA value measures, particularly when they expand measures of value beyond traditional health outcomes. This could place more pressure on budgets than would arise in a pure CEA-driven world. If the cutoff value should expand in MCDA models beyond that used for CEA, then (other things equal) budgets will commonly have to expand to accommodate the costs of acquiring the additional elements of value. Some of the added value (as measured by the MCDA approach) may well lie outside the domain of health, for example, elements of value arising in the domains of education, social justice, or others. Budgets of health care providers or payers would not normally incorporate these "other" issues, hence exacerbating nonalignment between budgets and prespecified MCDA cutoffs. Offsetting these potential increases, some existing interventions might fall in overall value as measured by the MCDA approach than previously ranked using only CEA. In this situation, pruning out some interventions that score poorly on the MCDA value-to-cost index could relieve budgets.

None of these solutions are easy to achieve in real-world situations. In many cases, CEA ratios are not available for the entire portfolio of existing interventions, and so deciding when to "cut back" cannot rely entirely on using incremental CEA ratios. This would happen even more frequently when an MCDA model was used, if for no other reason than the more extensive data demands of MCDA (beyond those of CEA) would make it less likely that all interventions in the portfolio had been evaluated using the appropriate metric. In situations such as these, the most likely outcome is to turn to deliberative bodies to resolve the dilemma, a topic to which we turn to next.

Another approach exists once an MCDA metric is chosen: allow people to vote on the appropriate cutoff for the bundle of benefits as defined by a particular MCDA weighting structure. In

this setting, people would "vote" with a number for the cutoff value to define an acceptable investment. For a number of reasons, the median value is the most appropriate to use in such a vote [21,22]. The same approach, of course, could be used directly to choose a CEA cutoff through population voting.

#### **Deliberative Processes**

A deliberative process is characterized by the careful, deliberate consideration and discussion of the advantages and disadvantages of various options [23]. Hence, a deliberative process is best considered as an aid to thought and judgment. Properly executed, it will be—compared with an ad hoc process—more comprehensive in the relevant issues embraced, more consistent in the way they are embraced, and more engaging of the people affected by the outcome. In making health care resource allocation decisions, deliberative processes have been useful in incorporating wider dimensions of value, for example, 1) social and cultural values, such as other types of outcome beyond health gain; 2) other social and personal values not typically taken into account, such as issues of equity and fairness; and 3) practical issues of operational feasibility [24].

Currently, we have identified several examples of deliberative processes in regular use. For example, the method used by the National Institute for Health and Care Excellence for evaluating health care technologies in England and Wales includes formal submissions from interested parties as well as consultations and invited commentaries from consultees and commentators, systematic reviews, technical modeling exercises, and multiparty representation in the (large) deliberative committee that hears witnesses [25]. Rawlins and Culyer [26] and Rawlins et al. [27] discuss how this process has been used to incorporate several social judgments, including equity considerations, into decisions of the National Institute for Health and Care Excellence.

Also, the Scottish Medicines Commission uses several "modifiers" in appraising new medicines, which are sometimes used to justify accepting a higher cost per QALY (see the article by Danzon et al. [3]). In Australia, George et al. [28] argue that the Pharmaceutical Benefits Advisory Committee has taken account of factors other than cost effectiveness in reaching its reimbursement decisions, including severity of disease, the absence of any other effective therapy, and the financial burden on the patient if the therapy was not reimbursed. Examples of bodies in the United States that use deliberative processes to incorporate other considerations when formulating policy recommendations are the Advisory Committee on Immunization Practices [29] and the US Preventive Services Task Force [30].

Although the evidence is not strong, the possible advantages of deliberative processes are as follows: 1) decision makers acquire a better grasp of the strengths and weaknesses of the underlying cases and can better defend their decisions; 2) consensus building is enhanced; 3) the revelation of evidence gaps helps to inform downstream research programs; 4) stakeholders and their peers are more likely to accept and implement decisions that they have had a hand in shaping; 5) possible selection bias through the membership of decision-making panels becomes relatively more obvious; and 6) context-free evidence can be re-interpreted in relevant contexts [24]. Daniels and Sabin [31] argued that the reasonableness or legitimacy of the process was crucial to obtaining acceptance of certain health plan decisions. It can be argued that a transparent deliberative process can increase the legitimacy of decision making compared with a black box process in which it is unclear what has been considered and how the decision was reached.

Nevertheless, deliberative processes may be relatively informal and unstructured. Thus, without a formal set of prompts, key

issues may be overlooked, decisions may be reached in an unstandardized way, and the biases of decision makers may not be adequately contested. There may also be a lack of quantification of elements other than health system costs and health gain, which might in principle be taken into account. For these reasons, it may be valuable to deliberative bodies to incorporate an explicit framework such as MCDA. Some HTA bodies or analysts, such as the Institute for Clinical and Economic Review in the United States, have used this approach. As in all such uses, MCDA analyses do not *make* decisions. Rather, they inform the process and help quantify thinking Box 1.

If a deliberative process were to use MCDA to help structure its decisions, then that deliberative body must set the weights for the MCDA model in advance. Otherwise, the MCDA model has no meaning in the context of the deliberation. Whether used to augment deliberative processes or as stand-alone processes, using MCDA models in the context of group decision making requires voting or ranking procedures for groups to choose among alternatives. Box 2 contains a brief discussion of some pertinent issues for these "choice-related" procedures.

Deliberative processes, no matter what voting methods are used, have participants integrating a considerable amount of possibly complex information to reach a single conclusion. As is now increasingly understood in the field of behavioral economics, human decision making is often distorted by how questions are framed, by inaccurate assessment of probabilities, and by other important human fallibilities [36]. The formal structure of MCDA may avoid at least some of these issues that may persist in less structured deliberative processes.

#### Other Value Elicitation Methods

The previous section discussed the use of MCDA models to aggregate multiple dimensions of value into a single metric. The commonly used models such as MAUT and AHP have specific methods embodied in them to elicit the needed value trade-offs—primarily arising from the work of people in the field of decision science. These methods have their own strengths and weaknesses (as discussed earlier), but other approaches may prove fruitful, some of which arise from the methods of economics.

Because health itself cannot be bought and sold in a market setting, and because health care markets are distorted by insurance or government subsidies, direct valuation of a health intervention or a health insurance product as a differentiated good through observed market prices—as economists might normally do-is difficult. In a recent article, Basu and Sullivan [37] discussed the rationale of using stated preference methods for developing "hedonic" value frameworks for health insurance products to inform the decision on whether a product should be covered or subsidized by insurance, given its price. They propose that discrete choice experiments in a nationally representative sample be used to elicit WTP for health insurance products that would cover a new health intervention with specific attributes. These elicitations should be carried out among the patients who are the direct beneficiaries of this intervention as they are diagnosed with the specific clinical condition as well as the healthy individuals who do not have that specific clinical condition but face varying risks of being diagnosed with that condition in a given year. This combination of values from both patients and nonpatients would reflect the true value of a health care intervention because it would incorporate the value of health insurance covering that intervention [38]. On the basis of these elicitations, a WTP value index could be developed relating to each of the dimensions of a health care intervention.

This value index will not reflect marginal value at market equilibrium, as would appear in hedonic pricing estimates, but it

Box 1-Aggregation via MCDA and Deliberative Processes: Limitations and Approaches

#### **Limitations to Current MCDA Models**

The methods of value elicitation in MCDA differ from model to model. Standard AHP processes ask "the decision maker" to evaluate all attributes in pairwise fashion, asking (a) which is more important and then (b) on a scale of 1-9 (typically) how much more important is the better attribute. The number of questions needed to elicit the weights rises quadratically with the number of dimensions of value; for N dimensions, there are N\*(N-1) questions that must be answered. In group decision-making processes, simple voting rules are normally used to elicit the "group value" structure. The problems from this approach are well known. Most importantly, this independent pairwise evaluation process admits inconsistencies in the final value structure, which must either be ignored or resolved through re-evaluation. Second, the process for converting the data to usable weights relies on mathematical methods that are difficult for most people to understand, and hence create a sort of "black box" methodology. And finally, at least some forms of AHP are subject to rank reversal (the Independence from Irrelevant Alternatives issue), although some variants of AHP avoid this issue.

Other approaches resolve some of these issues, but at the expense of creating other concerns. A standard approach sets boundary values for the highest and lowest levels of utility created in single dimensions and then elicits "swing weights" to capture relative value of each attribute, known as the Simple Multi-Attribute Rating Tool using Swings (SMARTS) [13]. This model and variants are widely used in MAUT. An easier refinement elicits the value structure by simply asking "the decision maker" to list the attributes in rank order, and then creates a set of weights that give the average of all possible weights consistent with the rank order. This approach (known as SMARTER) has been shown to closely approximate the results using exact measures of value rather than these simple approximations [15]. But this process brings its own difficulties. Most importantly, this MAUT approach requires that "the decision maker" specify for each attribute a lower bound (the amount of the attribute that creates zero added utility) and an upper bound (the amount of the attribute that creates maximum utility). This approach violates the standard economic concept that "more is always better" by setting an upper bound on utility. More importantly, these "boundary" values create a metric to measure performance of each candidate on each dimension of value, typically scaled to 0-1 or 0-100, and the value weights interact with the "measuring stick." Thus if the MAUT software's "measuring stick" metrics differ from those held in the minds of "the decision maker," the results do not represent what the decision maker intended. More detailed discussions of these issues are available [13,16].

#### Box 2-The Choice of Formal Voting Methods

Both the use of MCDA models and formal deliberative methods require that groups agree on choices. For example, AHP requires a series of N\*(N-1) pairwise votes to choose the more important of each possible pair of N value dimensions, and then another vote to specify the relative importance (ranging from 1 to 9 typically). MAUT using the SMARTER process requires that the group agree on a rank-order listing of the importance of the N value dimensions [13]. Multiple voting techniques are available to accomplish these tasks, and they (not uncommonly) give different answers from the same group of voters.

Formal deliberative processes also require group agreement to choose among alternatives. When three or more choices exist, no ranking method exists that meets four simple criteria [32] and all available voting methods to choose among three or more choices are subject to strategic voting manipulation [33,34]. Even if the choices are reduced to pairwise comparisons using some structured technique as Roberts' Rules of Order, the opportunity for strategic manipulation of the outcome through agenda control looms in many settings [35]. While we have no particular recommendation about choice of voting rules, we believe that groups undertaking such votes either with MCDA models or deliberative processes should carefully consider the voting methods they adopt and understand the potential consequences for their choices. A large literature on social choice theory (too large to reference here) can help guide these choices.

would capture the distribution of marginal value in the population. This approach can inform two specific decision-making processes: 1) whether a health insurance budget could be expanded to cover a new technology and 2) which existing technology can be displaced to accommodate the new technology that maximizes value in the health plan population. If either or both cannot accommodate the budget impact of the new technology, then coverage may not be feasible. Basu and Sullivan [37] lay out a research agenda that can help develop this concept of hedonic valuation for health care value frameworks.

#### **Conclusions and Recommendations**

No existing method completely or perfectly solves the two aggregation problems that we consider in this section. It is widely understood that CEA, CBA, and related models cannot deal with aggregation across individuals (equity, fairness, disparities, etc.) and, in actual practice (if not in concept), cannot incorporate some important components of value (and occasionally components of cost). Consensus-building approaches (i.e., deliberative

methods) are often unstructured, idiosyncratic, and easy for individuals to influence or control through agenda manipulation, power of status, or personality. ECEA and even further expansions of such models (such as ACEA) gather information about additional outcomes beyond those considered in standard CEA, but provide no mechanism for aggregating across the relevant elements or dimensions of value. They provide uncontestable answers to questions about coverage of an intervention only when pure dominance emerges, that is, one candidate exceeds another on every possible dimension of value. Thus, these are not satisfactory methods to carry into the future without further effort to improve upon them.

Public and private insurers use deliberative decision making for payer coverage and reimbursement decisions. A transparent deliberative process can increase the legitimacy of decision making. Currently, such processes often lack transparency, and it is unclear what factors have been considered and how decisions were reached. Deliberative processes today are often informal and unstructured. Thus, without a standardized approach, key issues may be overlooked, decisions may be reached in an unstandardized way, and potential biases of decision makers may not be

adequately explored. This can be improved, both by the introduction of cost-per-QALY evidence and by the use of more structured decision making to take account of preferences about the weight to be given to health gain—for example, about disease severity, equity of access, or unmet need. A systematic comparison of the processes used by various private and public pharmacy and therapeutic committees and HTA programs may further help others determine best practices for their own setting.

MCDA models may provide the best opportunity for improvement, but they have not yet been perfected. To improve these methods, we urge progress on two fronts. First, we must expand the use of MCDA models in real-life decision settings and learn from these experiences how well they work. We may learn that they seldom differ from standard CEA in the investment advice they give, or we may learn that formally incorporating these "other issues" importantly changes many decisions. We cannot know until we "run the experiment."

Second, we also need more research on key aspects of MCDA modeling and use. Just as the current "criterion standard" of CEA did not begin in its current form, we can expect that MCDA will evolve for the better in the future. In CEA, both for lack of the proper conceptual framework and for lack of data, earlier efforts focused on things that were easily measurable. These first included "deaths averted" or similar measures, and soon expanded to "life-years saved" or similar measures. Then came the notion of quality adjustment, leading to the current metric of the QALY based on work that relies on population-based estimates of reported quality adjustments for different health-related conditions. A similar but not identical measure—DALY—is used by the World Health Organization, the World Bank, and others: for this measure, expert judgment has been used (at least initially) rather than population survey data to create the adjustment factors [17], although DALY estimates increasingly turn to population-based metrics when available. A third related measure is the capabilitiesadjusted life-year, using a capabilities index instead of a health state utility, initially proposed by Amartya Sen, progressed by Nussbaum [39], and operationalized by Anand et al. [40].

We likely stand at a similar point in the evolution of MCDA models with important issues to resolve before they reach their full potential. Some have issues in ease of use. Some have methodological flaws such as the risk of "rank reversal" as new technology options emerge. Little is known about important human factor issues associated with the use of various MCDA models, including ease of use, susceptibility to strategic manipulation, and ease of comprehension of the methods (and hence acceptance of the results). And finally, these approaches are quite data-intensive compared with CEA modeling because they require measuring each candidate technology on multiple dimensions of value rather than on the single dimension of QALYs.

We recommend greater testing and use of MCDA models, pushing the frontiers of their use and continuously comparing their results with those of standard CEA and similar models. Using ECEA or even more broadly—ACEA—models may provide a halfway-house step to MCDA by facilitating data acquisition and refinement. But ECEA provides no way to combine multiple dimensions of value into a single index of merit. MCDA provides the logical basis for this next step. An important missing element is a universally acceptable method to elicit value weights. Current approaches have known defects (as discussed earlier), but have nevertheless been demonstrated to assist in decision making in complex health care settings, but further improvement is needed to bring MCDA models to full flower.

What are the best methods for acquiring value weights, particularly in settings with groups acting as decision makers? AHP and MAUT offer different approaches with different strengths and weaknesses. Discrete choice experiments using representative populations offer another approach to establishing

proper weights [37]. Other approaches may emerge as well. All should be tested and compared both for methodological soundness and for human factors (ease of use etc.).

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#### REFERENCES

- [1] Bator FM. The simple analytics of welfare maximization. Am Econ Rev 1957;47:22–59.
- [2] Garrison LP, Pauly MV, Willke RJ, Neumann PJ. An overview of value, perspective, and decision context—a health economics approach: an ISPOR Special Task Force report [2]. Value Health 2018; 21:124–30.
- [3] Danzon PM, Drummond MF, Towse A, Pauly MV. Objectives, budgets, thresholds, and opportunity costs—a health economics approach: an ISPOR Special Task Force report [4]. Value Health 2018;21:140–5.
- [4] Lakdawalla DN, Doshi JA, Garrison LP, Phelps CE, Basu A, Danzon PM. Defining elements of value in health care—a health economics approach: an ISPOR Special Task Force report [3]. Value Health 2018;21:131–9.
- [5] Verguet S, Kim JJ, Jamison DT. Extended cost-effectiveness analysis for health policy assessment: a tutorial. Pharmacoeconomics 2016;34:913–23.
- [6] Arrow KJ. Aspects of the Theory of Risk Bearing in the Theory of Risk Aversion. Helsinki, Finland: Yrjo Jahnssonin Saatio, 1965. Reprinted. In: Essays in the Theory of Risk Bearing. Chicago, IL: Markham Publishing Co., 1971:90-109
- [7] Pratt JW. Risk aversion in the large and in the small. Econometrica 1964;32:122–36.
- [8] Garber AM, Phelps CE. Economic foundations of cost-effectiveness analysis. J Health Econ 1997;16:1–31.
- [9] Chetty R. A new method of estimating risk aversion. Am Econ Rev 2006;96:1821–34.
- [10] Keeney RL, Raiffa H. Decisions with Multiple Objectives: Preferences and Value Tradeoffs. New York, NY: Wiley, 1976.
- [11] Saaty TL. The Analytical Hierarchy Process, Planning, Priority. Resource Allocation. RWS Publications, 1980.
- [12] von Winterfeldt D, Edwards W. Decision Analysis and Behavioural Research. New York, NY: Cambridge University Press, 1986.
- [13] Edwards W, Barron FH. SMARTS and SMARTER: improved simple methods for multiattribute utility measurement. Organ Behav Hum Decis Process 1994:60:206–325.
- [14] Phelps CE, Madhavan G. Using multi-criteria approaches to assess the value of health care. Value Health 2017;20:251–5.
- [15] Barron FH, Barrett BE. Decision quality using ranked attribute weights. Manage Sci 1996;42:1515–23.
- [16] Phelps CE, Madhavan G. Resource allocation in decision support frameworks. Cost Eff Resourc Alloc. 16(S1), in press, 2018.
- [17] Tan-Torres Edejer T, Baltussen RM, Adam T, et al. Making Choices in Health: WHO Guide to Cost-Effectiveness Analysis. Geneva, Switzerland: World Health Organization, 2003.
- [18] Neumann PJ, Sanders GD, Russell LB, et al. Cost-Effectiveness in Health and Medicine. New York, NY: Oxford University Press, 2016.
- [19] Phillips LD, Bana e Costa CA. Transparent prioritisation, budgeting and resource allocation with multi-criteria decision analysis and decision conferencing. Ann Oper Res 2007;154:51–68.
- [20] Marsh K, IJzerman M, Thokala P, et al. Multiple criteria decision analysis for health care decision making—emerging good practices: report 2 of the ISPOR MCDA Emerging Good Practices Task Force. Value Health 2016;19:125–37.
- [21] Galton F. Vox populi. Nature 1907;75:450–1.
- [22] Balinski M, Laraki R. Majority Judgment: Measuring, Ranking, and Electing. Cambridge, MA: MIT Press, 2010.
- [23] Hajer MA, Wagenaar H, editors. Deliberative Policy Analysis: Understanding Governance in the Network Society. Cambridge, UK: Cambridge University Press, 2003.
- [24] Culyer AJ, Lomas J. Deliberative processes and evidence-informed decision making in health care: Do they work and how might we know? Evid Policy 2006;2:357–71.
- [25] National Institute for Health and Care Excellence. A guide to the methods of technology appraisal. 2003. Available from: http://www. nice.org.uk/aboutnice/howwework/devnicetech/guidetothemethod softechnologyappraisal.jsp. [Accessed December 5, 2013].
- [26] Rawlins MD, Culyer AJ. National Institute for Clinical Excellence and its value judgments. BMJ 2004;329:224–7.

- [27] Rawlins M, Barnett D, Stevens A. Pharmacoeconomics: NICE's approach to decision making. Br J Clin Pharmacol 2010;70:346-9.
- [28] George B, Harris A, Mitchell A. Cost-effectiveness analysis and the consistency of decision-making: evidence from pharmaceutical reimbursement in Australia (1991–1996). Pharmacoeconomics 2001;19:1103-9.
- [29] Smith JC. The structure, role and procedures of the US Advisory Committee on Immunization Practices (ACIP). Vaccine 2010;285:A68–75.
- [30] Agency for Healthcare Research and Quality. US Preventive Services Task Force Procedure Manual. AHRQ Publication No. 08-05118-EF. Rockville, MD: Agency for Healthcare Research and Quality. July, 2008. Available from: https://www.uspreventiveservicestaskforce.org/Home/ GetFile/6/7/procmanual/pdf. [Accessed January 4, 2018].
- [31] Daniels N, Sabin JE. Accountability for reasonable priority setting. BMJ 2008;9:a1850.
- [32] Arrow KJ. A difficulty in the concept of social welfare. J Polit Econ 1950;58:328–46.

- [33] Gibbard A. Manipulation of voting schemes: a general result. Econometrica 1973;41:587–601.
- [34] Satterthwaite MA. Strategy-proofness and Arrow's conditions: existence and correspondence theorems for voting procedures and social welfare functions. J Econ Theory 1975;10:187–217.
- [35] Plott CR, Levine ME. A model of agenda influence on committee decisions. Am Econ Rev 1978;68:146–60.

  [36] Kahneman D. Thinking, Fast and Slow. New York: Farrar, Straus and
- Giroux, 2011.
- [37] Basu A, Sullivan SD. Towards a hedonic value framework in health
- care. Value Health 2017;20:261–5.
  [38] Lakdawalla D, Malani A, Reif J. The insurance value of medical innovation. J Public Econ 2017;145:94–102.
- [39] Nussbaum M. Capabilities as fundamental entitlements. Fem Econ 2003;9:33-59.
- [40] Anand P, Hunter G, Carter I, et al. The development of capability indicators. J Human Dev Capabil 2009;10:125–52.