ISPOR Task Force Report


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ABSTRACT

Concerns about rising spending on prescription drugs and other areas of health care have led to multiple initiatives in the United States designed to measure and communicate the value of pharmaceuticals and other technologies for decision making. In this section we introduce the work of the International Society for Pharmacoeconomics and Outcomes Research Special Task Force on US Value Assessment Frameworks formed to review relevant perspectives and appropriate approaches and methods to support the definition and use of high-quality value frameworks. The Special Task Force was part of the International Society for Pharmacoeconomics and Outcomes Research Initiative on US Value Assessment Frameworks, which enlisted the expertise of leading health economists, concentrating on what the field of health economics can provide to help inform the development and use of value assessment frameworks. We focus on five value framework initiatives: the American College of Cardiology/American Heart Association, the American Society of Clinical Oncology, the Institute for Clinical and Economic Review, the Memorial Sloan Kettering Cancer Center, and the National Comprehensive Cancer Network. These entities differ in their missions, scope of activities, and methodological approaches. Because they are gaining visibility and some traction in the United States, it is essential to scrutinize whether the frameworks use approaches that are transparent as well as conceptually and methodologically sound. Our objectives were to describe the conceptual bases for value and its use in decision making, critically examine existing value frameworks, discuss the importance of sound conceptual underpinning, identify key elements of value relevant to specific decision contexts, and recommend good practice in value definition and implementation as well as areas for further research. Keywords: cost-effectiveness analysis, QALYs, value assessment frameworks.

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Need for Value Assessment in Health Care

Concerns about rising spending on prescription drug prices and in other areas of health care have led to multiple initiatives in the United States designed to measure and communicate the value of pharmaceuticals and other health care technologies for decision making. Among the most visible organizations proposing and implementing a “value assessment framework” (or “value framework” for short) are the American College of Cardiology (ACC)/American Heart Association (AHA), the American Society of Clinical Oncology (ASCO), the Institute for Clinical and Economic Review (ICER), the Memorial Sloan Kettering Cancer Center (MSKCC), and the National Comprehensive Cancer Network (NCCN) [1–6]. Notably, unlike the plethora of efforts to assess value in many countries around the world, these initiatives are privately financed and administered, and might be seen as a response to the absence of US government activity in the area.

These entities differ in their missions, scope of activities, and methodological approaches. For example, some (e.g., ASCO, MSKCC, and NCCN) focus on cancer drugs, whereas others (e.g., ICER) have a broader purview, not only focusing on new drugs but also considering certain medical devices, procedures, and health

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The emergence of these five recently proposed value assessment frameworks, as listed in part A of Table 1, provided the initial motivation for this effort and represents important attempts in the US health care system to provide better information on value of prescription drugs—and more broadly of other health care technologies and interventions, including devices, procedures, diagnostics, and health programs—to payers, providers, and patients. They complement other previous and ongoing approaches listed in part B of Table 1 (e.g., the European Society for Medical Oncology, the AMCP Format for Formulary Submissions, Avalere/FasterCures, SMART Vaccines, US Preventive Services Task Force, Advisory Committee on Immunization Practices, Institute of Medicine, European network for Health Technology Assessment, and efforts by organizations around the world that use economic evaluation to guide decisions); they have also endeavored to measure or summarize the value of medical interventions [7–20]. Various organizations and groups have formulated principles that they believe constitute good principles for value frameworks; see part C of Table 1 for examples of these as well [21–27].

Nevertheless, these recent new frameworks also raise important questions and potential concerns. Because they are gaining visibility and some traction in the US marketplace, it is essential to scrutinize whether the frameworks use approaches that are transparent as well as conceptually and methodologically sound. By attempting to simplify the problem of value assessment, these new frameworks could end up making ad hoc assumptions and simplifications not supported by theory or evidence, and thus may not deliver promised value. Possibly, they could even make value assessment more difficult and less rational, that is, we could be optimizing the wrong aspects. Furthermore, given that they are addressing different decisions and stakeholders, there is the possibility of inconsistency or misalignment across the frameworks. Finally, it is critical to investigate these value frameworks because of the signals they send to innovators. Value-based approaches can encourage firms to produce more of what is being optimized in the frameworks and can discourage them from bringing to market products that do not produce good value. Ideally, that means that the society will benefit from medical products and health care technologies that efficiently improve the health and welfare of the population according to consistent and well-founded measures of value. Conversely, ill-conceived frameworks could produce long-lasting harms by encouraging innovators to develop treatments that fail to produce real value.

At the outset, we note that existing value assessment frameworks tend to focus on drugs and to a lesser extent on medical devices, which neglects the fact that most of the nation’s health spending involves physician and hospital services; the latter are not typically subject to formal economic evaluation. Focusing at the margin on new technologies may ignore or downplay waste and inefficiency in the existing system and in an important sense penalizes new technologies [28]. Notably, the purview in this report is broader than pharmaceuticals alone, and encompasses devices, diagnostics, procedures, and health programs. We recognize that there may be characteristics of nonpharmaceutical interventions that pose special challenges for value assessments. For example, data on the expected effectiveness of drugs (a key ingredient for CEAs) are generally based on the efficacy observed in randomized controlled trials, whereas such data are not available for many physician and hospital services and for some medical devices given the rapid and incremental nature of innovation [29]. Moreover, patent protection and exclusivity reward innovators of drugs with potentially high prices, which in turn increase the demand for CEAs, whereas there are no such property rights in most other areas of health and medicine [29]. Finally, the value of certain interventions, such as some devices and procedures, may depend on clinical and patient learning effects, which makes value assessment challenging.

### Importance of Defining Value and Perspective

A broad range of disciplines—economics, health services research, psychology, philosophy, design theory, and anthropology, for example—have developed constructs for considering what constitutes “value.” Across these fields, value is often recognized as a multidimensional concept, encompassing utility, social significance, emotional and spiritual meaning, and monetary expenditure [30]. Specific attributes may vary in importance depending on the perspective of the individual or organization.

The importance of perspective is reflected in the new value frameworks. Some of the constructs have adopted the perspective of the provider (as the patient’s agent) or of the patient. For example, the ASCO value framework states that its purpose is “to provide a standardized approach to assist physicians and patients in assessing the value of a new drug treatment for cancer as compared with one or several prevailing standards of care” [2]. The implied reasoning is that those parties most immediately affected by the treatment are in the best position...
to judge its relative merits. Nevertheless, this reasoning holds only if the decision maker faces all the costs and benefits of the product. Insured patients almost never face the full price of the products they consume, and physicians typically have relatively little direct economic stake in the prescribing decision. In certain specialties, however, where doctors are allowed to purchase and “sell” the drugs they prescribe to insurers at a margin over the purchase price, they have a stake in the decision.

Value frameworks with perspectives that do not consider the full costs and benefits of a treatment necessarily result in decisions that are distorted from a societal welfare perspective. Such decisions can then create spillover effects, externalities, that affect those not involved in the transaction. Occasionally, these impacts can be positive (e.g., as in vaccines), but in most situations they are negative and reduce overall societal welfare. Furthermore, in a market-oriented health care financing and delivery system such as in the United States, it is well understood that this “marketplace” is not unfettered, and is at risk of many kinds of “market failure” that are addressed (imperfectly) through a wide range of regulations and laws. In a seminal work in 1963, Nobel Laureate Kenneth Arrow [31] remarked, “... the special economic problems of medical care can be explained as adaptations to the existence of uncertainty in the incidence of disease and the efficacy of treatment.” In health care, uncertainty is an important source of market imperfections and regulatory response. Indeed, these value frameworks could well be interpreted as efforts by particular stakeholders to correct or adjust for some perceived market (or governmental) failures.

Health economists define value on the basis of microeconomic principles, recognizing that value is best defined as what individuals (or others acting on their behalf) would be willing to pay to acquire more health care or other goods or services. This has to be compared with “opportunity cost” in terms of what benefit, income, or other resources they are willing to forgo to obtain them. Nevertheless, although providing a well-defined framework for decision making with the objective of efficient resource allocation, the economic approach may not easily accommodate circumstances relevant in some health care decision contexts, such as health plan enrollment or provider-patient shared decision making about treatment choice, and may not address issues of equity across subpopulations.

Leaders in the field of economic evaluation in health care have long recommended that analysts seeking to inform resource allocation decisions approximate the value of interventions in terms of incremental cost per QALY gained [8,10,14,32,33]. (Note that some studies use disability-adjusted life-years [DALYs] rather than QALYs to measure health gains. QALYs and DALYs are closely related health metrics, although we recognize differences and their potential implications; essentially, QALYs weight survival using measures of preference, whereas DALYs weight survival on the basis of measures of disability [34].) These ratios are types of incremental cost-effectiveness ratios, and when specifically using the QALYs gained in the denominator, it is often labeled “cost-utility analysis”, although in this report we generally refer to such analyses as cost-per-QALY analyses or as CEs with QALYs as the measure of effectiveness. Thousands of these cost-per-QALY analyses have been carried out and catalogued [35]. Health economists, however, also recognize that this metric, although capturing the key driver of health gain in terms of length and quality of life, has limitations: QALYs may not always fully capture the health (or well-being) of patients, or incorporate individual or community preferences about the weight to be given to health gain, for example, about disease severity, equity of access, or unmet need. Indeed, QALYs were not developed to capture elements such as equity or unmet need. Moreover, in cases in which it has been applied, the cost-per-QALY metric has served as an input into complex and multifaceted health policy decisions. Frameworks aiming to inform physician-patient shared decision making raise their own concerns. What are the appropriate elements or dimensions of value? How should those elements be weighted to derive an overall “score”?

As we highlight in this Task Force report, the particular “perspective” considered in the frameworks is a central question. Is the audience the patient, the payer, or society at large? The role of health insurance in the US mixed private/public health care system is also critical. In theory, a framework could help insurers choose which value measures are most suitable for and attractive to insured, premium-paying individuals or employers, or those who are buying insurance by paying taxes for it.

Despite being the agent for the plan member—and hence the patient—the insurer perspective is, however, likely to differ from the perspective of a patient when ill. Because patients reveal their preferences in part through their choice to enroll in health plans, payers will presumably want to understand how patients value health outcomes, and hence QALYs should reflect factors such as patient preferences for health states and treatment as accurately as possible. Nevertheless, a tension can exist in defining value from the perspective of an individual patient who, when ill, may desire all potentially beneficial health care regardless of cost because they face only a fraction of the cost and because their illness is now virtually a certainty and not just a probability. In contrast, premium-paying plan members and taxpayers face the full cost for the entire health plan population and must consider the value for money from spending on health versus other non-health-related goods and services.

Methods

The International Society for Pharmacoeconomics and Outcomes Research (ISPOR) is a multistakeholder and multidisciplinary organization with the mission to promote scientific excellence in health economics and outcomes research. In the spring of 2016, ISPOR’s Board of Directors approved an Initiative on US Value Assessment Frameworks to inform the shift toward a value-driven health care system by promoting the development and dissemination of high-quality, unbiased value assessment frameworks, by considering key methodological issues in defining and applying value frameworks to health care resource allocation decisions. This initiative enlisted the expertise of leading health economists, including several who are not ISPOR members, focusing on what the field of health economics can provide to help inform the development and use of value assessment frameworks. The deliberations of the ISPOR Special Task Force (STF) were vetted at all stages with the broad range of stakeholders, including other outcomes researchers, patient and industry representatives, and academic experts.

The Initiative on US Value Assessment Frameworks proceeded in several stages (see Fig. 1). First, an External Advisory Board (EAB) and a Stakeholder Advisory Panel (SAP) were created to provide general guidance and stakeholder input for the initiative. The EAB mainly comprised academic-based experts in health economics and outcomes research, whereas the SAP included representatives from the life sciences industry (both pharmaceutical and devices), patient groups, payers, professional societies, and contract research organizations.

An STF was then formed to collaborate on a white paper, heretofore termed “report,” to review relevant perspectives and appropriate approaches and methods to support the definition and use of high-quality value frameworks. ISPOR task forces are initiated to develop consensus guideline reports on good practice standards for outcomes research (clinical, economic, and patient-reported outcomes) and on the use of this research in health care
decision making. An STF is a version of a task force formed for a time-sensitive, science policy-related task, typically first producing a report to be responsive to external science policy developments. Both types of task forces must be approved by ISPOR’s board of directors. The STF for the Initiative on US Value Assessment Frameworks, cochaired by Louis P. Garrison of the University of Washington and Peter J. Neumann of Tufts Medical Center, comprised 13 individuals with extensive expertise in health economics and economic evaluation to allow for in-depth methodological discussions. The members were drawn from academic institutions in the United States and, in two cases, the United Kingdom to reflect non-US perspectives, and also included Richard Willke, ISPOR’s Chief Science Officer. Beginning in the late summer of 2016, the STF convened through two in-person meetings and several teleconferences. Section authors met on a more regular basis. A leadership group, comprising Drs. Garrison, Neumann, and Willke, convened frequently to provide oversight and coordinate the work.

To obtain thorough and timely expert and stakeholder input to this effort, the STF consulted the EAB and SAP on several occasions. The EAB and SAP were first surveyed before the beginning of the STF work, in May and June 2016, on general value definitions, key perspectives, and strengths and weaknesses of five current value frameworks; 19 of 24 EAB members responded to the survey and 29 of 45 (requested) SAP members responded. On the basis of this input, the STF drafted a scope of work for its report, and the EAB and SAP were surveyed again to provide input on this draft scope of work, with 16 and 21 respondents, respectively. A stakeholder conference was held on September 23, 2016, in Washington, DC, to further discuss the proposed STF scope of work, drawing more than 250 attendees.

After this conference, the SAP was expanded to 72 members on the basis of individual requests. In addition, to solicit further research and perspectives on value assessment frameworks as input to STF deliberations, a call for abstracts on topics relevant to this area was issued in May 2016, and 135 abstracts were received. Thirty-five abstracts were selected for full article submissions; after peer review, 22 articles were published in a special themed issue of *Value in Health* in February 2017, including 5 articles presenting stakeholder group perspectives [36]. In early May 2017, a draft report of the STF report was made available to the EAB and the expanded SAP for comment; 18 sets of comments were received. Key aspects of the draft report were presented and discussed in two sessions available to all attendees at ISPOR’s 22nd Annual International Meeting on May 22, 2017, in Boston, MA. On the basis of this feedback, the report was revised and a new draft distributed to the entire ISPOR membership in early July, with a subsequent 1-month comment period. To address more than 25 sets of comments from members, the STF revised the report one final time, giving extra attention to the short set of key recommendations in the article by Garrison et al. [37].

Funding for the STF came exclusively from ISPOR, including support, such as travel expenses for STF members to attend in-person meetings. All the STF members volunteered their time for this effort. The Pharmaceutical Research and Manufacturers of America provided partial support for a May 2016 EAB meeting, but did not fund the work of the STF.

**Goals and Organization of This ISPOR STF Report**

The objectives of this report are to describe the conceptual bases for value and its use in decision making, critically examine existing value frameworks, discuss the importance of sound conceptual underpinning, identify key elements of value relevant to specific decision contexts, and recommend good practice in value definition and implementation as well as areas for further research.

The article by Garrison et al. [38] provides an overview of value and perspective, and the one by Lakdawalla et al. [39] discusses key elements of value. In the article by Danzon et al. [40], we take up issues about reaching decisions through consideration of budget constraints, thresholds, and opportunity costs. The article by Phelps et al. [41] examines potential options to aggregate these elements at both the individual and population levels and reviews decision-making processes. The article by Willke et al. [42] discusses recent value frameworks in light of the points made in the previous articles, and the article by Garrison et al. [37] provides a summary and key recommendations.

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