INTRODUCTION

Advancing High-Quality Value Assessments of Health Care Interventions

Assessing value in health care is not a new concept; nevertheless, discussion on using value assessment frameworks to support payers, physicians, and patients in health care decision making, particularly related to prescription drugs, has increased dramatically in the United States. Numerous value assessment frameworks have been proposed recently by several US organizations, such as the American College of Cardiology and the American Heart Association (ACC-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]. All these frameworks differ in their objectives, scope, approach, and the type of factors accounted for in measuring the value of new therapies. Each framework has its strengths and weaknesses, many of which have been raised by various stakeholders. At the same time, additional efforts are ongoing to develop new value assessment frameworks, particularly from the patient perspective [7].

Given this rapid evolution of a complex landscape of value assessment frameworks in the United States, the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) announced the Initiative on US Value Assessment Frameworks in May 2016 [8]. This initiative was created to inform the shift toward a value-driven health care system by promoting the development and dissemination of high-quality, unbiased value assessment frameworks. In collaboration with stakeholder representatives, this initiative has aimed to identify and discuss key methodological and process issues in defining and applying value frameworks to health care resource allocation issues, and to convene a special task force to collaborate on a white paper that reviews the breadth and depth of the topics addressed in this issue.

To complement and inform this ISPOR initiative as well as ongoing efforts on value assessments by other organizations, Value in Health announced a call for articles in May 2016 for a themed section dedicated to this topic. We called for submissions of articles that highlight challenges related to methodological, process, and ethical considerations in value assessment frameworks, and/or propose solutions to advance development and implementation of high-quality value assessment frameworks for health care interventions. We invited submissions of brief articles, such as commentaries, perspectives, or brief reports, as well as full-length original research or methodological articles. All interested authors were invited to submit an abstract for consideration in this themed section. We received an overwhelming response, with a total of 126 abstracts received from all over the world. The abstracts were reviewed by both guest editors, and in consultation with a Value in Health co-editor-in-chief. Authors of those articles that best fit the scope, vision, and goals of this call for articles were invited to submit full-length manuscripts for consideration in a newly planned themed issue. We had a rapid publication timeline planned for this themed issue to allow it to be timely and relevant in informing ongoing initiatives. Hence, each invited abstract was matched to two reviewers before formal submission so as to expedite the peer review process. We are thankful to all the reviewers for their time and effort in helping evaluate the manuscripts within a quick turnaround time. Of the 36 manuscripts invited for full submission, 22 were accepted for final publication in the present issue.

Although not meant to be comprehensive, we hope that this set of articles provides a broad array of perspectives on value assessments and an overview of key issues with (and alternatives or enhancements to) existing approaches to inform value-based decision making in health care. We now provide a brief overview of all the articles, grouped by sections, to provide a general sense of the breadth and depth of the topics addressed in this issue.

In the first section, “Evaluating Existing Value Frameworks,” we have three articles that evaluate existing value frameworks on various metrics and highlight their strengths and weaknesses. Mandelblatt et al. [9] first summarize a taxonomy of principles grounded in cost-effectiveness theory, but extend beyond this foundation, that could be used to evaluate value frameworks. They apply these principles to four recently published value frameworks, spanning various specialties including oncology (ASCO and NCCN frameworks), cardiology (ACC-AHA frameworks), and vaccines (European guidelines for vaccine value assessment). They highlight not only key strengths but also significant gaps that exist between some of these frameworks and well-accepted principles. Cohen et al. [10] developed case studies to explore the degree to which the MSKCC, ASCO, and ICER frameworks have face validity. To do so, they identify four challenges: 1) value is not always proportional to a therapy’s benefit; 2) value incorporates factors that may not be relevant to framework users (patients or payers); 3) attribute weights do not necessarily reflect user preferences; and 4) value estimates used to inform therapy prices do not reflect per-person benefit. Bentley et al. [11] assembled a panel of eight members representing a range of potential value framework users to conduct value assessments of five advanced lung cancer drugs using the ASCO, ESMO, ICER, and NCCN frameworks. This pilot study demonstrates fair to good convergent validity (i.e., extent to which each framework produced similar evaluations for the same list of drugs) but finds variable reliability (i.e., the reproducibility of the assessments by different panelists) across the frameworks.
All three articles highlight that further revisions of the existing frameworks are needed to improve their utility. Although cost-effectiveness analysis (CEA) is often cited as the criterion standard for economic evaluations of health care technologies and is widely used in decision making, it has been argued that this approach to measuring value has several shortcomings, and there are other elements of value and issues related to equity that deserve further consideration in resource allocation decisions. In the second section, “Addressing Gaps in Cost-Effectiveness Analysis,” we have a set of four articles that address a diverse set of issues identified with the CEA approach. Cookson et al. [12] provide guidance on the use of CEA to address health equity concerns by first introducing the “equity impact plane” that helps illustrate the trade-offs between traditional CEA objectives (i.e., improving total health) and equity objectives (i.e., reducing social inequality in health or prioritizing the severely ill). They then present two practical options for conducting equity-informative CEA that provides policymakers a better understanding of equity impacts and trade-offs. Garrison et al. [13] posit the need for an “expanded” CEA framework to incorporate elements of value over and above the core value drivers of health gains (typically measured in quality-adjusted life-years [QALYs]) and cost offsets. They specifically describe a subset of five elements related to the “value of knowing,” including reduction in uncertainty, insurance value, value of hope, real option value, and scientific spillovers. They argue that without appropriate consideration of such elements of value, payers may risk incorrect access decisions for patients or suboptimal investments in health care research and development. Shafirin et al. [14] provide some empirical data on one aspect of the value of hope discussed by Garrison et al. [13]. They show that patients with cancer have a significantly higher preference than physicians for treatments that have some probability of a durable (i.e., long-term) survival gain as opposed to those that do not, given equal mean survival for those treatments. Finally, Taylor et al. [15] conducted an experiment on a sample of individuals to highlight that the standard QALY approach that values health states rather than changes in health states could lead to suboptimal resource allocation by understating the value given to improvements from more severe health states. They suggest that their proposed alternative approach, which values changes in health states directly, would better reflect individual preferences.

Any value-based decision necessarily involves consideration of what the buyer—patient, payer, and/or society—can afford. Our third section, “Value and Affordability,” contains three articles that provide perspectives on how affordability might be judged. Ciarlet et al. [16] take issue with drug-specific fixed spending caps, at the payer or national level, such as referenced in the ICER value framework. They analyze the historical variability in total spending for drugs launched in the United States from 2003 to 2014, showing that only a small percentage of them exceeded a hypothetical proportional spending cap, and arguing that limiting spending on those drugs may have inefficiently shifted spending to less cost-effective alternatives. Sculpher et al. [17] discuss the importance of considering opportunity costs in any treatment funding decision, particularly when there is a fixed drug or overall health care budget, because any spending on a new treatment requires that spending on some other treatment be forgone. Funding only a new treatment whose incremental cost-effectiveness ratio is less than a relevant cost-effectiveness threshold, such as £30,000 per QALY saved, helps ensure that health gain due to the new treatment exceeds the health gain forgone—the opportunity cost—of shifting limited budget to the new treatment. Camejo et al. [18] provide a more in-depth discussion of the option value of a new product (as mentioned in Garrison et al. [13]) when new products may not only have a current societal value but also have a dynamic value on the basis of their expected scientific contribution to future innovation. To achieve longer term efficiency in decision making, they propose a way to incorporate this option of value into a dynamic value framework for product assessment, with the proviso that this future aspect of benefit may need to be funded by a separate global innovation budget that may be determined by disease-specific societal preferences for innovation.

Although CEA provides a specific, theoretically well-grounded structure for combining the costs and benefits of an intervention, there are alternatives that may be more responsive to varying stakeholder preferences and provide a basis for decision making [19,20]. In our fourth section, “Incorporating Multiple Dimensions of Value in Decision Making,” four articles describe such approaches. To provide some grounding in an actual process being used in Europe, Kristensen et al. [21] present a succinct overview of the HTA Core Model®, an international framework for assessing multiple dimensions of value developed within the European Network for Health Technology Assessment project, and discuss some recent developments there. Phelps and Madhavan [22] discuss models based on multicriteria systems analysis. This approach, often known as multicriteria decision analysis or MCDA, has also been the subject of a two-part ISPOR Task Force Report [23,24] and allows for consideration of criteria beyond what is present in standard CEA. Essentially, this approach is a stakeholder group process in which relevant aspects of value are identified, rated, and combined using user-defined weights to create an overall value measure for a given product. The authors describe the various advantages, such as transparency and explicit representation of preferences, of this approach as well as provide an illustrative example. Building on the MCDA approach, Baltussen et al. [25] propose “evidence-informed deliberative processes” that combine MCDA with another principle for priority setting called “accountability for reasonableness.” This process puts the specific MCDA valuation process in the larger context of public decision making by an HTA agency, involving public stakeholder identification, consultation, review, response, and appeal. The authors provide some examples of countries that have implemented some aspects of this process, and make some specific recommendations for its conduct by HTA agencies. In a different, more empirical approach to determining value, Basu and Sullivan [26] explain hedonic pricing schemes, which in well-functioning markets can estimate value on the basis of the attributes of the product under consideration; for example, a real estate appraiser may estimate that a chimney adds $1000 to the value of a house. They describe how hedonic pricing may be adapted to the less well-functioning health care market by using patient-based stated preference methods to inform the decision on whether a product should be covered or subsidized by insurance. They lay out a research agenda for how their conceptual approach may be operationalized and used to inform other value frameworks.

No health economics–based anthology on value would be complete without some discussion of alternative ways that governments or societies can make value-based decisions. In our fifth section, “Alternative Approaches for Value-Based Assessments,” we have three articles that describe alternative frameworks for assessing value of health interventions from the payer perspective, including some that have been implemented in ex-US health care systems. Mühlbacher and Sadler [27] describe the concept of the efficiency frontier (EF) adapted by the German Institute for Quality and Efficiency in Health Care as a framework for evaluating cost-effectiveness and informing reimbursement and pricing of health technologies. They further address methodological issues of current EF methods by calculating EFs on the basis of a single multidimensional overall benefit (as opposed to individual end points) and by taking parameter uncertainty into account. They illustrate the practical
implementation of this approach using the example of hepatitis C treatments. Connolly et al. [28] present a fiscal health analytic framework to examine the value of health care investments from a government perspective. The framework uses intertemporal and cross-sectoral consequences for health interventions on disability costs, lost taxes attributed to poor health, and reduced productive output, that are pertinent to public sector decision makers. This approach could serve as a useful complement to other value frameworks based on traditional CEA. In the next article, Pauly [29] reminds us that, like with any product, the perceived value of a health care treatment can vary from patient to patient, and so there is likely no single single-value-based price that applies to all patients. Patients, or insurance plans, will pay for a product if its price is less than their perceived value for it, thus defining the demand curve for the product. When a product is available from only one or a few sellers, the price is going to be set by the seller at a point that maximizes its net revenue given this demand curve. Within a cost-effectiveness framework, Pauly presents a possible solution to value-based purchasing in which different insurance plans will cover all products lower than a given cost-effectiveness threshold, with premiums that vary on the basis of the threshold used. Patients (or their employers) could choose from among these plans on the basis of their willingness to pay for different levels of coverage.

Understanding how patients and other key stakeholders perceive value is a necessary and important step in any discussion of how to measure "value" and use it in decision making. Although certainly not capturing all possible perspectives, in our final section, "Patient and Other Stakeholder Perspectives on Value," we are pleased to include five commentaries that present patient, shared decision making, regulatory, and multistakeholder perspectives. One patient perspective on value, from Allen et al. [30] at Friends of Cancer Research, emphasizes attention to patient preferences for features that improve patient accessibility to therapies with potential benefit, including valuing evidence from innovative trial designs or real-world data, as well as consideration of both direct and indirect costs of care. Perfetto et al. [31] focus on the National Health Council’s rubric for patient-centered value frameworks, whose key domains are partnership, transparency, inclusiveness, diversity, outcomes, and data sources. Their article describes the development and content of that rubric and contains examples as to how its domains can be operationalized in value assessment frameworks. Armstrong and Mullins [32] apply patient values to a shared decision-making context, identifying a taxonomy of global, decisional, situational, and external factors. By incorporating both general and situation-specific patient value considerations, this taxonomy may provide a basis for a decision aid for joint clinician-patient evaluation in treatment options.

Of course, a fundamental value assessment is made by the US Food and Drug Administration in its decisions regarding approval of new drugs and devices. Miller and Woodcock [33] describe principles underlying the Food and Drug Administration’s decision making, which is largely based on clinical risk/benefit assessment, but is seeking to be better informed by the patient voice via its Patient-Focused Drug Development Initiative. Finally, to help consolidate patient, clinical, payer, industry, and societal perspectives on value assessment, Sorenson et al. [34] propose a set of 10 high-level principles to be observed in any value assessment effort. Embracing elements such as broad stakeholder engagement, inclusion of the patient perspective, use of established and transparent methods for comparative and cost-effectiveness, use of long-term outcomes and available evidence, and more, these principles may help guide the creation and operationalization of specific value assessment frameworks.

We hope this overview will help readers navigate through the large number of articles in this issue and identify the ones they would like to review in more detail. We also hope that this collection of articles will broaden our thinking on the various perspectives and challenges associated with value assessments, inform the ongoing initiatives on value assessment frameworks, and ultimately contribute to solutions for supporting robust value-based decision making in health care.

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