Value to Whom? The Patient Voice in the Value Discussion

Eleanor M. Perfetto, MS, PhD1,2,*, Elisabeth M. Oehrlein, BA1,2, Marc Boutin, JD1, Sarah Reid, BA3, Eric Gascho, BA1

1National Health Council, Washington, DC, USA; 2University of Maryland, Baltimore, MD, USA

Abstract

Background: Professional societies and other organizations have recently taken a visible role trying to define treatment value via value frameworks and assessments, providing payer or provider recommendations, and potentially impacting patient access. Patient perspectives routinely differ from those of other stakeholders. Yet, it is not always apparent that patients were engaged in value framework development or assessment. Objectives: To describe the development and content of the National Health Council’s (NHC’s) Rubric, a tool that includes criteria for evaluation of value frameworks specifically with regard to patient-centeredness and meaningful patient engagement. Methods: The NHC held a multistakeholder, invitational roundtable in Washington, DC, in 2016. Participants reviewed existing patient-engagement rubrics, discussed experiences with value frameworks, debated and thematically grouped hallmark patient-centeredness characteristics, and developed illustrative examples of the characteristics. These materials were organized into the rubric, and subsequently vetted via multistakeholder peer review. Results: The resulting rubric describes six domains of patient-centered value frameworks: partnership, transparency, inclusiveness, diversity, outcomes, and data sources. Each domain includes specific examples illustrating how patient engagement and patient-centeredness can be operationalized in value framework processes. Conclusions: The NHC multistakeholder roundtable’s recommendations are captured in the NHC’s Rubric to assess value framework and model patient-centeredness and patient engagement. The Rubric is a tool that will be redefined over time on the basis of feedback from patient, patient group, framework developer, and other stakeholder-use experiences. Keywords: clinical decision making, decision making, patient-centered care, patient outcome assessment, patient preference.

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Introduction

The US health care system is undergoing a transformation in the way it delivers and pays for care. As traditional fee-for-service payment gives way to more value-based payment arrangements, understanding and defining the value of health care treatments and interventions has become a national priority [1]. Value frameworks have emerged to aid health care stakeholders in assessing the value of new treatments. Although somewhat new to the United States, value assessments have long been a routine approach used more formally in health care decision making and priority setting in many other countries [2,3]. For example, in countries with public health insurance, such as Canada, Australia, and European countries, national and/or regional health technology assessment agencies are well established [4,5]. In Asian and Latin American countries, health technology assessment processes are becoming more widespread [6,7]. In 2015, four organizations in the United States released value frameworks described as intended to support physicians and/or payers in assessing treatments [8–11]. These developers, including professional societies and think-tank organizations, are taking an increasingly visible role defining value and providing recommendations to payers and providers [12]. Unlike international efforts, the US-based frameworks are not mandated by law or endorsed by public payers.

Although most stakeholders agree that spiraling health care costs must be addressed and discussions on value are necessary, framework developers have been criticized for “dangerously oversimplifying complex issues” and potentially limiting patient access to valuable treatments [13]. Developers have largely adopted a conventional health economic approach to value in terms of treatment effectiveness and cost [14]. Value frameworks often include quantitative, economic models, deriving cost-effectiveness or utility, and/or budget impact. For patients, however, value is individualized and disease-dependent, and can evolve with the disease trajectory or a patient’s stage of life [15–18]. Importantly, patient perspectives on value can differ significantly from that of physicians and payers, often integrating considerations beyond clinical outcomes and cost, such as a treatment’s ability to help patients achieve personal goals [19]. Similarly, even among patients with the same condition, individual values and preferences may differ [20]. Despite lack of agreement over how value is defined and assessed, value frameworks have gained recent attention in the United States, raising
concerns regarding their appropriateness for determining access to care [21]. In countries or regions with established public health decision-making bodies, stakeholders have long echoed these concerns, resulting in formalized procedures to involve patients in health care priority setting [22]. Despite these efforts, even in countries with ongoing efforts to include patients in decision making, concerns persist that patient involvement efforts are insufficient or “rhetoric” [23,24].

To the patient community, discussions regarding value are not a theoretical exercise and if value frameworks are to be used to inform decisions affecting treatment access, the patient community wants to have a central role in defining value and in value assessment [25–27]. Thus, robust processes are needed to incorporate the patient voice in value assessment. Yet, it has not always been apparent if individual patients or patient organizations were engaged in new value framework creation or use [28].

As developers work to improve the patient centricity of their frameworks and accompanying economic models, both in the United States and in other countries, bridging any gap between cost-effectiveness interpretations of “value” in health care and patient perceptions of value is increasingly important [29–31]. The patient advocacy group members of the National Health Council (NHC) expressed that they were not sure how to help bridge these gaps as they were navigating new waters [32]. To assist in this effort, the NHC convened a value roundtable to construct a rubric as a tool to support understanding of patient-centeredness in value assessment. This article provides an overview of the development and content of and the expected uses for the NHC Patient-Centered Value Model Rubric (Rubric), which conceptualizes patient-centeredness and meaningful incorporation of the patient voice throughout value framework (and related economic model) development [26].

Methods
A convenience sample of 28 organizations (12 patient organizations, 3 payers, 6 professional or policy organizations, 5 biopharmaceutical organizations, and 2 research organizations) was invited to participate in a daylong roundtable discussion on February 1, 2016, in Washington, DC. The roundtable discussion was divided into three parts. The first involved reviewing the objective for the roundtable, to produce a rubric for value framework, and model) and agreed on how they would be used in their discussion and in the Rubric (see Online Supplement 1 in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2016.11.014). Participants grouped the characteristics they listed into six key domains of patient-centeredness: partnership, transparency, inclusiveness, diversity, outcomes, and data (Table 1). They discussed that direct patient engagement is the ideal goal, in which the patient community has an active role as a co-investigator, partner, advisor, or consultant. Nevertheless, indirect means of engagement also contribute to patient-centeredness and should not be overlooked. Indirect means include such things as using existing data sets that capture patient views and preferences.

The roundtable participants included seven patient groups representing diseases/treatments with value assessment released in the previous year and those anticipating release in the coming year, and one representative from a consumer group. There were also representatives from three biopharmaceutical companies, two from a professional association and one from a not-for-profit research organization.

The group members discussed their views on the terms being used (e.g., patient, patient community, patient-centeredness, value framework, and model) and agreed on how they would be used in their discussion and in the Rubric (see Online Supplement 1 in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2016.11.014). Participants grouped the characteristics they listed into six key domains of patient-centeredness: partnership, transparency, inclusiveness, diversity, outcomes, and data (Table 1). They discussed that direct patient engagement is the ideal goal, in which the patient community has an active role as a co-investigator, partner, advisor, or consultant. Nevertheless, indirect means of engagement also contribute to patient-centeredness and should not be overlooked. Indirect means include such things as using existing data sets that capture patient views and preferences.

The Patient-Centered Value Model Rubric
On the basis of the roundtable discussion, a draft Rubric resulted describing the two complementary, direct and indirect, pathways for addressing the six domains of patient-centeredness [26]. Specific examples from the discussion representing high or low activity were included. Of the 33 organizations invited to submit comments on the draft Rubric, 11 organizations (1 payer, 3 professional or policy organizations, and 7 biopharmaceutical organizations) responded and their comments were incorporated. In Table 1, excerpts from the final Rubric document’s content are provided (for the full Rubric, see Section 2 in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2016.11.014). The characteristics capture aspects of direct engagement and indirect patient-centeredness and illustrative examples for each of the six key domains of patient centricity. The Rubric was released publicly on March 28, 2016, as part of a public Webinar held by the NHC [36].

Discussion
The roundtable participants were generally supportive of the intent behind value assessments and stated that they do not wish to waste money or other resources on unnecessary procedures and/or prescriptions. But, patients want information to make informed decisions on the basis of what is or is not valuable to them. Participants agreed that to be considered patient-centered, value frameworks must have the key characteristics that resulted in six domains of patient-centeredness. The Rubric formed around these domains promotes patient-centeredness in the value discussion by engaging, informing,
<table>
<thead>
<tr>
<th>Domains of a patient-centered value model</th>
<th>Example characteristics of meaningful patient engagement</th>
<th>Example patient-centeredness considerations</th>
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<tr>
<td><strong>Patient partnership:</strong> Patients should be involved in every step of the development and dissemination processes</td>
<td><strong>Characteristics</strong></td>
<td><strong>Considerations</strong></td>
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<td></td>
<td>Patients are recognized as partners and integrated in all aspects of development</td>
<td>Patient input was sought and used throughout, from planning to updating</td>
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<td>Transparency to patients: The assumptions and inputs (and each step in the process) should be disclosed in an understandable way and in a timely fashion</td>
<td>The patient community has early opportunities for review of and comment on inputs, methods, and drafts through multiple venues</td>
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<td>Inclusiveness of patients: Perspectives drawn from a broad range of stakeholders, including the patient community, should be reflected</td>
<td>Representatives from the patient community are involved throughout the process, as required or expected given the condition/population</td>
<td>A rationale was provided for the patient community perspectives sought and incorporated throughout the process</td>
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<tr>
<td>Diversity of patients/populations: Differences across patient subpopulations, trajectory of disease, and stage of a patient’s life should be accounted for</td>
<td>Diversity of the patient population is acknowledged and considered</td>
<td>Consideration was given to differences in patient perceptions of value across relevant subpopulations, including populations at risk and those with early- and late-stage disease</td>
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Applicability and limitations across patient subpopulations and disease trajectory are acknowledged and considered

Information was provided on limitations with regard to the younger patient subpopulation

Limitations regarding applicability in the younger subpopulation were not addressed
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<th>Outcomes patients care about: The outcomes integrated should include those that patients have identified as important and consistent with their goals, aspirations, and experiences</th>
<th>Outcomes important to patients are identified and incorporated</th>
<th>A clear link was described between the outcomes incorporated and their importance to patients</th>
<th>Only clinical outcomes were considered without the context of importance to patients</th>
<th>Processes are in place for identifying and incorporating emerging information on outcomes of importance to patients</th>
<th>A mechanism was described that allows patients and other stakeholders to suggest when an update is needed</th>
<th>No mechanism was offered for patients to suggest when an update is needed</th>
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<tr>
<td>Patient-centered data sources: Various credible data sources are used allowing for timely incorporation of new information and account for the diversity of patient populations and patient-centered outcomes, especially those from real-world settings and reported by patients directly</td>
<td>Existing sources of patient-generated health data (e.g., patient registries or PROs) are identified and considered</td>
<td>Data on PROs were used, and the sources well described</td>
<td>No effort was made to identify sources of patient-reported data on physical function, although this was identified by patients as the outcome of highest priority</td>
<td>Data beyond randomized controlled trials are considered (e.g., natural history, patient views, outcomes and/or treatments, preferences regarding outcome or treatment)</td>
<td>The report describes all data sources used, including data from a patient registry</td>
<td>Included only clinical trial data submitted to the FDA as part of a new drug application</td>
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FDA, Food and Drug Administration; NHC, National Health Council; PRO, patient-reported outcomes.

* Please note that the examples provided here are intended to be only illustrative of the characteristic. These examples are not intended to be exhaustive.
and actively listening to people with chronic conditions at every point of contact [18,37].

The participants were enthusiastic about the construction of the Rubric as a learning tool, but stopped short of defining a scoring system or conducting full evaluations of the frameworks that exist. Their rationale was that there is not enough US-based experience with value frameworks; therefore, a benchmark for scoring or rating did not exist. They advised caution in that the Rubric should not be viewed as punitive, but rather as informative and educational, much in line with the way the PCORI implemented its original patient and family engagement rubric [38]. Similar to PCORI’s Engagement Rubric, which illustrates “how input from patient and stakeholder partners can be incorporated throughout the entire research project,” the NHC Rubric can guide framework developers from the planning stages through the update and maintenance stage [34]. The recommendation from the group was to disseminate the Rubric and engage with patient groups and developers to get their feedback on its usability, validity, and impact, and update it in the future as deemed necessary, instituting a scoring system if needed.

Since the public release of the Rubric, framework developers have acknowledged that elements of high value to patients may be missing from their work. For example, the American Society of Clinical Oncology’s revised framework states that “the task force is well aware that there are many elements that might be important to individual patients in assessing the relative value of their treatment options that are not taken into account in our model” [30]. The authors justify leaving patient views out by stating that elements of high value to patients fit under the patient-reported outcomes (PROs) umbrella; reliable PRO assessments are, however, not yet available. This statement demonstrates that developers have become more sensitive to the need for frameworks to be more patient-centered. Nevertheless, it also demonstrates that there is more work to be done to promote patient-centeredness. Patient-centeredness extends beyond capturing PRO data, and the outcomes that matter most to patients are not always PROs. In fact, depending on the particular patient population, individual patients may prioritize changes in biomarkers, improvements in clinician-reported outcomes, or other observable symptoms [39].

The Institute for Clinical and Economic Review recently solicited input from the health care community on how its methodology should be modified to include “important patient-relevant and patient-centered outcomes, especially those not adequately captured in the clinical trial data” [40]. These efforts to improve the patient-centeredness of value frameworks are particularly important if physicians and payers look to frameworks to inform decisions that can affect the treatment options available to a patient.

In a more general sense, the domains and attributes included in the Rubric could apply to patient-centeredness and engagement in contexts beyond value assessment [41]. Efforts to involve patients in value assessment align with other patient centricity efforts, such as involving patients in research, as is promoted by PCORI and increasingly by the Food and Drug Administration through its patient-focused drug development initiative [41–43]. Although value frameworks have a different purpose and intended audience, each of them will likely have an impact on the care or access to care that patients experience. Application of the Rubric in many of these other contexts can help to ensure processes reflect patient priorities, opportunities, and touch points for reaching out and involving patients, family caregivers, and patient advocacy organizations.

One outstanding question that still needs to be addressed is that of representativeness [44]. If developers are to be inclusive in the patient populations they engage, there must be clarity about what representativeness means. Future efforts must help to articulate clearly how to judge when a representative sample of patients have or have not been engaged in the processes of value framework development and assessment. Again, the relevance of representativeness is broad and can apply to value assessments as well as patient-focused drug development and patient-centered health care.

Importantly, for patient-centered value assessment to be successful, the onus is not only on value framework developers, but on other stakeholders as well. For example, the research community must participate by pursuing and publishing research examining health outcomes that matter to patients. Methods for defining and enrolling heterogeneous populations in studies must be developed and patient-centered data sources should be used. Although patient advocacy organizations are undoubtedly experts on their condition of interest, they must also be prepared to participate in value discussions, for example, by soliciting and providing information from their diverse memberships regarding the impact of disease on their lives, natural history of disease, preferred outcomes, and related epidemiologic information.

There are a number of limitations to the Rubric that should be noted. The Rubric content was based on the roundtable discussion and vetting among a group of peer reviewers. The participants and reviewers were a convenience sample of NHC members, colleagues, and others who had been recommended by the roundtable participants. This approach may not have captured the views of all stakeholders or the breadth of representation within any one stakeholder group. The Rubric has not been formally tested and needs to be used by various stakeholders to fully capture its utility, validity, and impact. Finally, although the domains of patient centricity and examples of high versus low patient engagement may be broadly applicable, this initiative was developed in the context of the US health care system and frameworks recently published in the United States. The Rubric may require cultural adaptation before its use by international audiences.

Conclusions

Value frameworks can help advance the national dialogue on value in health care, but only if these frameworks incorporate the patient voice. The NHC Rubric is a first step toward creating patient-centered value assessments patients and their families can rely on. It is intended to assist all stakeholders, especially the patient community, in assessing the level of patient-centeredness and engagement in a given framework or model. It can be a guide to support developers in conceptualizing plans for meaningfully engaging patients.

At present, the Rubric should be considered a guide for patient-centeredness good practices. It is intended to be a living document, to be refined over time on the basis of feedback from patient, patient group, framework developer, and other stakeholder-use experiences. It will require maintenance, updating, and enhancement as experience and knowledge grow on its use.

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Supplemental Materials

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REFERENCES