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Value to Whom? The Patient Voice in the Value Discussion

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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 refined 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.

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

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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 frameworks (and economic models they may include), capturing expectations and delineating consistent criteria for patient-centeredness. For this project, the term "rubric" describes a tool with criteria and levels of achievement in patient-centeredness and patient engagement. Examples of existing, publicly available patient-engagement rubrics were reviewed as models, including the Patient-Centered Outcomes Research Institute's (PCORI) Patient and Family Engagement Rubric and the University of Maryland's Center of Excellence in Regulatory Science and Innovation's Rubric on Assessing Meaningful Patient Engagement in Drug Development [33–35]. The second part included the group being asked to describe the hallmark characteristics of patient-centeredness and frame those characteristics in the context of value assessments. Storyboarding was used throughout to ensure key points were accurately captured. The third part of the group discussion was dedicated to reviewing the storyboards, analyzing and grouping patient centricity characteristics, and creating illustrative examples of high versus low engagement in the context of the grouped themes.

The storyboards, themes, and illustrative examples were subsequently used by the NHC staff to craft a draft rubric, which was reviewed by the roundtable participants who provided comments, edits, and additions. The next iteration of the draft was vetted through multistakeholder peer review, including representatives from patient advocacy organizations, payers, professional societies, biopharmaceutical companies, and policy institutes. The draft was sent to 33 organizations (12 patient organizations, 3 payers, 7 professional or policy organizations, 9 biopharmaceutical organizations, and 2 research organizations), comprising NHC members and others recommended by roundtable participants. Input from three value model developers was specifically sought.

Results

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,