Emerging Good Practices for Transforming Value Assessment: Patients' Voices, Patients' Values

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ABSTRACT

Background: Patient engagement is a transformative strategy for improving value assessment. US value framework developers have increased engagement activities, but more needs to be learned about how to best achieve meaningful patient engagement in value assessment. The objective was to glean good practices in patient engagement emerging from patient community experiences, to be used in value assessment. **Methods:** The National Health Council Value Workgroup conducted a survey and held a focus group with its member advocacy organizations to gather experiences with value framework developers and views on emerging good practices. **Results:** Ten of 13 organizations completed the survey; reporting 13 interactions with four framework developers. Most rated experiences as "good" to "very good." Emerging good practices included (1) engage early; (2) engage a range of patients; (3) leverage patient-provided information, data resources, and outreach mechanisms; (4) be transparent; and (5) appreciate and accommodate resource constraints. Twelve of 13 organizations participated in the focus group, and this produced 30 emerging good practices in four areas: (1) timing; (2) methodology and data; (3) partnering; and (4) characterizing engagement. **Discussion:** Patient engagement was limited in early development of value frameworks but has increased in the past few years. Patient groups report positive experiences that can serve as emerging good practices. These groups also reported experienced challenges in their interactions and recommended good practices to mitigate those challenges. **Conclusions/Recommendations:** The growing pool of patient engagement experiences can be translated into good practices to advance a patient-centered, value-driven health care ecosystem. Lessons learned from these early experiences can help establish recommend emerging good practices that can eventually result in best practices and standards in the field.

Keywords: economic, health technology assessment, patient(s), value.

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Background

Value frameworks and assessments have proliferated and gained attention in the United States over the past few years. In 2015, four organizations—the American Society of Clinical Oncology (ASCO) [1], the Institute for Clinical and Economic Review (ICER) [2], the National Comprehensive Cancer Network (NCCN) [3], and Memorial Sloan Kettering Cancer Center (MSK) [4]—released frameworks that they describe as intended to support physicians and/or payers in assessing the relative value of treatments. At their inception, the US frameworks were developed and value assessments were produced with little to no patient engagement [5,6]. Currently, there is no one perfect model or framework for value assessment or even one that earns support across all stakeholders [7]. There is agreement among stakeholders that value frameworks need to better incorporate the patient perspective [8].

To increase and improve patient engagement, the National Health Council (NHC) in 2016 launched a Value Initiative to support its patient-advocate membership [9]. The value initiative (1) a Value Model Rubric [5] that provides clarity to the patient community and framework developers/assessors on what constitutes patient-centeredness and engagement in value assessment; (2) a Get-Ready Checklist [10] that provides a guide for patient groups to prepare for engagement; (3) a Value Workgroup (Workgroup) that provides networking and information sharing through voluntary meetings of patient-organization staff with recent experience interacting with value framework developers; (4) a Qualitative Research Study that provides insights on patients' definitions of value; and (5) a Health economics educational program that provides patient groups basics on economic and value-assessment terms and principles.

A fundamental premise of the NHC's Value Initiative is that meaningful patient engagement—in all aspects of value

assessment—is a transformative strategy that can improve evaluations of the value of health care interventions to advance a value-driven health care ecosystem in the United States. Meaningful engagement is defined as interactions with the patient community (i.e., patients, caregivers, advocates, and patient advocacy groups) exemplified by direct relationships and partnerships that are bidirectional, reciprocal, and continuous; where communications are open, honest, and clear; and where engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent [5].

When meaningful engagement takes place, a stronger, more comprehensive value assessment results that is accepted by a greater number of stakeholders. For example, a disease-specific patient group provides information to a framework developer describing heterogeneity treatment response, which has been recognized but not fully understood. The patient group convincingly conveys the downside of step therapy [11], providing its own data on patient experiences. The result is a value assessment that highlights the importance of heterogeneity in the condition and recommends avoidance of step therapy. If this report is used for decision making by clinicians, payers, or patients, it will be more widely accepted, have greater utility in real-world care, and will not be disputed by patient and clinicians for not considering the heterogeneity in treatment effect they deal with routinely. Credibility and relevance are enhanced.

There is recent evidence that patient engagement in value assessment has improved considerably in the last few years [11,12]. However, questions remain about the best means to achieve meaningful and effective patient engagement in value assessment and to understand its impact on value assessment findings. This study's objectives were to (1) gather the patient-community's first-hand experiences with value framework developers and (2) glean from those experiences emerging good practices in patient engagement that can be disseminated, improved upon, and replicated.

Methods

NHC Value Workgroup

Starting in November 2016, the NHC began convening a voluntary Value Workgroup (Workgroup) comprising patient-organization staff members that had interacted in the past and/or currently are interacting, or anticipated interacting with value framework developers. Twenty patient-organization staff members from 13 organizations participate in monthly meetings via teleconference. The participants tend to be senior management-level staff from patient advocacy organizations (e.g., CEO, VP, manager titles). They also tend to be patients or caregivers themselves with personal experience with the disease or condition they represent. The objectives for the group are networking, information sharing, and education, and monthly updates help to inform and guide the NHC's Value Initiative. Workgroup members and NHC staff have learned a great deal from one another through these informal interactions. The Workgroup wanted to collect its views and experiences in a more formalized manner and decided to prospectively collect their experience reports and recommendations through a survey of Workgroup members and by using one of their meetings to hold a focus-group discussion.

Survey

From April 17 to April 21, 2017, the NHC Value Workgroup conducted an online survey to understand the experiences its members had with value framework developers. The SurveyMonkey questionnaire was designed by the NHC staff and University of Maryland, Baltimore, faculty with Workgroup input and pilot testing. The questionnaire (see Appendix A) was sent to each participating organization (N = 13) with the request that only one survey be completed for each organization. Topics included (a) activities comprising engagement experiences; (b) ratings of experiences with value framework developers; (c) experiences considered an emerging good practice (e.g., positive experience that should be replicated and disseminated); (d) proposed good practices recommended to mitigate challenges experienced; and (e) observed/perceived impact of patient input on value-assessment reports or final products. Open-ended questions also allowed respondents to provide details or other reflections on experiences. Workgroup members were sent four email requests to complete the survey. The group also was reminded about completing the survey during one of its teleconferences. Responses were anonymous.

Focus Group

On April 21, 2017, the Workgroup held a 2-hour, focus group discussion via teleconference with members. The purpose was to discuss specifically responses to survey questions (c) and (d) to drill down further on experienced and recommended emerging good practices. The Workgroup discussed positive and challenging experiences with value framework/assessment organizations and further discussed if the positive experiences represented emerging good practices that should be recommended for dissemination. It delved into challenging experiences and good practices that could be recommended to mitigate the challenges. The group came to consensus on experienced and recommended emerging good practices.

Because the maximum number of participants anticipated in this study could only reach 13, only descriptive analyses were planned.

Results

Survey

Ten of the 13 organizations (77%) completed the SurveyMonkey questionnaire. Of the three non-completers, one organization reported that it did not have any experiences with framework developers and could not answer the questions; one organization experienced a staffing change resulting in loss of Workgroup participation; and one organization did not respond.

Ten patient organizations reported 13 interactions with four value framework developers. The organizations reported the most common activities experienced while interacting with framework developers (Table 1). The top three most common activities were providing advice/consultation on a condition/disease, patient recruitment, and question/problem development. The least common activities were clinician recruitment, providing input for an economic model, and serving as a member of a governing committee/board.

Experience ratings on a list of value framework developers are presented in Table 2. Five groups had interacted with Framework Developer A, seven groups had interacted with B, one group had interacted with C, and one group had interacted with D. No interactions were reported with Framework Developers E and F. An excellent or very good rating was usually accompanied by such comments as "responsive," "receptive," "genuinely interested," or "gets us." Poor or fair ratings were accompanied by such comments as "unresponsive," "problems with transparency," "poor or no processes," "cherry picking what they will/will not use or respond to," or "provide no response without explanation."

Experienced good practices reported in the survey are listed in Table 3, and recommended good practices are listed in Table 4. Through the survey, the groups enumerated several ideas on