The Patient Voice in Value:

The National Health Council
Patient-Centered Value Model Rubric

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Introduction

The U.S. health care system is undergoing a transformation in the way it delivers and pays for care. As the traditional fee-for-service payment system gives way to more value-based payment arrangements, understanding and defining the value of health care treatments and interventions has become a national priority.

Value models (also known as frameworks) have emerged as the latest tools to help health care stakeholders assess the value of new treatments. 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[4] – released frameworks that developers have described as intended to support physicians and/or payers in assessing the value of treatments. While the subject of these initial models is drugs, models to evaluate other health care interventions are poised to proliferate given the heightened focus on value.

The patient community, like other stakeholders, is eager to take part in the value discussion. Yet, it is not apparent that individual patients or patient organizations were engaged throughout the creation of these frameworks or contributed to their development from conception.

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.[5] To have true utility, value models must incorporate these other value-influencing factors, and the only way to achieve this is by having robust processes in place to incorporate the patient voice. Such action is particularly important if physicians and payers look to value models to inform decisions that can affect the treatment options available to a patient.

To inform work in this area, the National Health Council (NHC), with stakeholder input, has created this Patient-Centered Value Model Rubric. The purpose of the Value Model Rubric is to provide a tool that the patient community, physicians, health systems, and payers can use to evaluate the patient centeredness of value models and to guide value model developers on the meaningful incorporation of patient engagement throughout their processes.

Assessing Patient Engagement in Value Models

On February 1, 2016, the NHC held a multi-stakeholder roundtable with the objective of creating a Value Model Rubric capturing the characteristics of a patient-centered value model. During the roundtable, participants discussed recent patient advocacy experiences with value models, reviewed other patient-engagement rubrics, and considered the hallmark characteristics of patient centeredness in assessments of value.

The Patient Community Represents a Broad Range of Voices

In this rubric, "patient community" broadly encompasses individual patients, family caregivers, and the organizations that represent them. For the purposes of the rubric, individual patients are those who have or are at risk of having a medical condition(s) whether or not they currently receive medicines or vaccines to prevent or treat a disease. A family caregiver is defined as an individual who provides unpaid care to a family member or friend who needs assistance with everyday activities.[6] Patient advocacy organizations are comprised of individuals, both lay and professional, and their mission is to combat a particular disease, disability, or group of diseases and disabilities, or to improve and protect the health of a particular group of people. The patient community is heterogeneous and brings to the value discussion different perspectives that have been informed by their experiences, trajectory or stage of disease, level of expertise, and many other factors.

It is important to note the distinction between patients and consumers in this context. Certainly, patients and consumers share many concerns but their perspectives on health issues can differ. A person with a chronic disease and/or disability relies on the health care system to enable a longer, healthier, and more robust life. A consumer is a generally healthy individual who will move in and out of the health care system as his or her needs change over time. This difference may lead to perspectives on the value of new treatments that vary in important ways.

Patient-centered health care is care that is respectful of and responsive to individual patient preferences, needs, and values in context of their own social worlds. Patient centeredness is created by engaging, informing, and actively listening to people with chronic conditions at every point of contact – from the research bench to the bedside and everywhere in between.[7,8]

Defining Value

Value means different things to different people. Establishing a definition of value that is broadly supported across the health care system has thus been elusive.

Today, the value of a treatment is commonly viewed by health care payers (i.e., both public and private insurers) in terms of its effectiveness and cost.[9] For patients, value is individualized and disease-dependent, and can evolve with the disease trajectory or stage of a patient's life. For example, a recent study of patients with metastatic breast cancer showed that more patients emphasize value in terms of their personal benefit (such as being able to maintain rich relationships with family members) than in economic terms.[10]

The Value Model Development Process

The value model development process can be broken down into five distinct phases:

- the *planning* stage during which the model's purpose and goals are established (e.g., statement of the question(s) the model is intended to help answer);
- the *drafting* stage, including subsequent refinements to reflect input and testing;
- the *dissemination and implementation* stage to encourage its use by the intended audience in the intended way;
- the *evaluation* stage to ensure it is achieving its stated purpose; and
- the *update and maintenance* stage to reflect changes or new knowledge in the disease, treatment, and evidence landscapes as well as to revisit the overall methodology.

At each stage of the process, there are opportunities and touch points for reaching out and involving patients, family caregivers, and patient advocacy organizations. Throughout this document, reference to the development process includes the stages described above.

Domains of a Patient-Centered Value Model

Roundtable participants agreed that because perceptions of value to patients are likely to differ significantly from perceptions of value to payers, providers, and consumers, value models must integrate the patient voice to have practical utility. The participants also agreed that any value model must be constructed with six key domains in mind:

1. **Patient Partnership.** Patients should be involved in every step of the value model development and dissemination process.
2. **Transparency to Patients.** The assumptions and inputs into the value model itself – and each step in the process – should be disclosed to patients in an understandable way and in a timely fashion.
3. **Inclusiveness of Patients.** The value model should reflect perspectives drawn from a broad range of stakeholders, including the patient community.
4. **Diversity of Patients/Populations.** The value model should account for differences across patient subpopulations, trajectory of disease, and stage of a patient's life.
5. **Outcomes Patients Care About.** The outcomes integrated into the value model should include those that patients have identified as important and consistent with their goals, aspirations, and experiences.
6. **Patient-Centered Data Sources.** The value model should rely on a variety of credible data sources that allow 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. The data sources included should reflect the outcomes most important to patients and capture their experiences to the extent possible.

The NHC Patient-Centered Value Model Rubric

The Value Model Rubric described below is intended to be a living document or tool to be refined over time based upon feedback from the patients, patient groups, model developers, and other stakeholder use experiences. The roundtable participants emphasized that it is too soon to declare the rubric as final and acknowledged they do not have enough information to develop a system for scoring or rating models. The rubric will require maintenance, updating, and enhancement as experience and knowledge is collected on its performance. It should be considered a guide for patient-centeredness best practices. The Value Model Rubric comprises two sections:

- **Section I: Meaningful Patient Engagement in the Value Model Development Process.** This section outlines characteristics of meaningful engagement in the value model development process.
- **Section II. Patient-Centeredness Considerations in General.** The second section focuses on activities that enhance patient centeredness as the model is being developed throughout the phases depicted above, but may not be directly related to patient engagement.

The two sections of the Value Model Rubric include a set of characteristics that correspond to the six domains of a patient-centered value model, as outlined above. Examples of the type of activities that represent high or low activity within the domain are also included.

Section I. Meaningful Patient Engagement in the Value Model Development Process

This section provides characteristics of meaningful engagement that illustrate efforts for incorporation of the patient voice. While a clear understanding of "meaningful" may be evasive, for this document "meaningful engagement" refers to direct relationships and partnerships that are bidirectional, reciprocal, and continuous. Communications are open, honest, and clear. Engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent.[11,12,13,14] *Please note that the examples provided here are only intended to be illustrative of the characteristic. These examples are not intended to be exhaustive.

1. Patient Partnership

Characteristics of Meaningful Patient Engagement	Examples of Patient Partnership*	
	High	**Low**
Patients are recognized as partners and integrated in all aspects of model development phases	Patient input was sought and used throughout the process, from planning to updating the model	Patients only responded as part of a public comment period
Patient partners are supported to enhance participation and capacity to engage	Patient partners were provided with training and user-friendly relevant materials, with adequate time to review	No patient-specific resources were developed or provided
Direct input is collected from a wide range of patients through mixed methods as suited to the disease, population, and context (surveys, focus groups, structured interviews, shadowing, etc.)	Processes were established for conducting a patient survey, interviews of disease-specific patient advocacy group staff, and use of a disease-specific registry	Processes only included use of data from randomized controlled trials reporting clinical outcomes. There was no direct patient engagement or contact

Characteristics of Meaningful Patient Engagement	Examples of Patient Partnership*	
	High	Low
Tools are available to patients to help them understand all aspects of the model and to communicate the model to other patients	A work group, with patient participation, crafted a communication strategy for patients	No patient-specific communication materials were developed

2. Transparency to Patients

Characteristics of Meaningful Patient Engagement	Examples of Transparency*	
	High	Low
The process for selection of patient representatives is transparent	The specific criteria used to identify, select, and invite patient representatives were disclosed, along with a rationale	No systematic process and/or criteria for selecting patient representatives was provided
The patient community has early opportunities for review of and comment on model inputs, methods, and drafts through multiple venues (such as public meetings or online comments)	Patients, as part of public comment, were given at least three opportunities to review and provide comment without undue limitations as to length (e.g., word count limits) or time to respond	Patients were given one opportunity to provide input after the draft was fully developed

3. Inclusiveness of Patients

Characteristics of Meaningful Patient Engagement	Examples of Inclusiveness*	
	High	Low
Representatives from the patient community are involved throughout the process, as required or expected given the condition/population	A rationale was provided for the patient community perspectives that were sought and incorporated throughout the process	Input was sought from stakeholders without consideration of the type of stakeholders that would be most appropriate given the condition/population
Model results (data) are translated into usable and meaningful information for patients	User-friendly tools were developed with patient input and made available to patients and families for shared decision making with clinicians. The tools were successfully tested with the users before public release	The model was intended for shared decision making between patients and providers, but no tools for the patient community were provided

4. Diversity of Patients/Populations

Characteristics of Meaningful Patient Engagement	Examples of Diversity*	
	High	**Low**
Diversity of the patient population is acknowledged and considered	Thoughtful consideration was given to differences in patient perceptions of value across relevant patient subpopulations, including populations at-risk and those with early- and late-stage disease	The model assumed the patient population is homogenous and takes a 'one-size-fits-all' approach

5. Outcomes Patients Care About

Characteristics of Meaningful Patient Engagement	Examples of Outcomes*	
	High	**Low**
Outcomes important to patients are identified and incorporated into the model, such as: o Functional status (mental/physical/societal) o Health-related quality of life o Well-being o Clinical Measures o Survival o Productivity o Goals, expectations, aspirations o Financial stress	A clear link was described between the outcomes incorporated into the model and their importance to patients	Only clinical outcomes were considered in the model without the context of importance to patients

6. Patient-Centered Data Sources

Characteristics of Meaningful Patient Engagement	Examples of Data Sources*	
	High	**Low**
Existing sources of patient-generated health data (e.g., patient registries or patent-reported outcomes) are identified and considered	Data on patient-reported outcomes were used, and the sources well described	No effort was made to identify sources of patient-reported data on physical function, though this was identified by patients as the outcome of highest priority

Section II. Patient-Centeredness Considerations in General

Section II outlines considerations to enhance the patient centeredness of the model in general. Patients, providers, and payers can apply this section to evaluate the extent to which additional steps, beyond engagement efforts, have been taken throughout the development of the value model to involve patients. These considerations enhance the patient centeredness of the model and the development processes, though patient engagement may not always be direct. These are critical considerations when relevant. But if deemed not relevant, a clear rationale should be provided. ***Again, the examples provided below are not intended to be exhaustive.**

1. Patient Partnership

Patient-Centeredness Considerations	Examples of Patient Partnership*	
	High	**Low**
Rationale to substantiate the adequacy of the ratio of patient to non-patient participants is provided	Of a 10-member advisory committee, 2 members were patients and 1 was a family caregiver, and rationale was provided for stakeholder composition	Of a 15-member team, no patients served and no rationale was provided for stakeholder composition
Patients are engaged in pilot testing and refinement of the model	A disease-specific patient advocacy group partnered with a payer to test the model in practice	No pilot testing with patient input was conducted
Patients are engaged in providing technical assistance to model end-users on implementation	A patient-informed implementation plan was provided	The implementation plan was reviewed by patients after it was constructed by others
Patient engagement in the development process is evaluated, including an assessment of whether patient expectations have been met and if patients realize/see the impact of their engagement	The development team learned upon evaluation that patient partners reported: an adequate level of engagement occurred; the model reflected patient input; and improvements in engagement processes were offered	No evaluation was conducted to assess patient engagement

2. Transparency to Patients

Patient-Centeredness Considerations	Examples of Transparency*	
	High	**Low**
The purpose and goals of the model are made clear to patients (including the intended audience and use) and are well-defined (includes caution on how the model should not be used)	The goals of the model are clearly represented and understandable to patients	The goals of the model are not clear to patients and do not include implications for patients

Patient-Centeredness Considerations	Examples of Transparency*	
	High	**Low**
The desired outcome of using the model and its implications for patients are made clear	The desired outcome of using the model and the implications for patient decision making are made clear	Patients do not understand the desired outcome of using the model and fear it will hurt access for the most vulnerable patients
The methodology is made transparent to patients in a timely manner	Patients have timely access to detailed methods if they want to review them	Details of methodologic limitations were not disclosed to patients until after the model was drafted
All assumptions and inputs used are articulated in an understandable, patient-friendly way	The model's assumptions and inputs were provided in a publicly accessible table, in a way a layperson can understand	The model's assumptions and inputs were described in technical terms and are not easily retrievable
Inputs considered but not used are described with the rationale for exclusion that patients can understand	Model methods described why certain patient registry data have been excluded from the model	Model methods did not acknowledge existing data from a patient-advocacy survey of members that was excluded from the model
Results of model pilot test(s) are disclosed and subsequent refinements are clearly indicated so patients can understand the sequence	Pilot testing results with patients were released with a plan for how the results will affect future iterations of the model	Pilot testing with patients was conducted but results were not made public
A clear distinction is made in public communications that are accessible to patients about the model development stage (e.g., undergoing pilot testing versus finalized)	The call for public comments was sent directly to relevant patient groups, was easily accessible to patients, and clearly described that the model was still in its drafting stage	The call for public comment was difficult for patients to find on the website and did not disclose stage of model development or if there would be additional opportunities to comment
Developer responses to public comments are made public to allow the patient community to understand how its input has or has not been used	Each new draft included a section explaining how patient community comments were addressed	No information was provided on how public comments informed revisions
Processes for evaluating that the model performs as intended are transparent and patients can understand them	The methods for evaluating the model were described in detail, including goals and timeline, in a way patients can understand	No evaluation plan was provided
Model evaluation considers if it is being used as intended, achieving intended outcomes, and assesses unintended consequences for patients	The evaluation led to changes in the model as it was learned that there were unintended consequences for patients	The evaluation process missed significant unintended consequences for patients
Results of any evaluation are made public	Evaluation reports were posted to a website publically accessible by the patient community	Evaluation reports were only distributed to a small group that excluded patients

Patient-Centeredness Considerations	Examples of Transparency*	
	High	**Low**
Processes for updating and maintaining the model are clear and accessible to the patient community	The report explicitly listed factors that result in updates outside of routine maintenance, including new data on patient outcomes	The process for updates outside of routine maintenance were not described
Patient partners are acknowledged as contributors/authors to the process	The report provided a list acknowledging all contributors, and the patient partners are listed among them with roles	The report did not identify patient partners, leading to questions regarding any patient involvement
All potential conflicts of interest are disclosed, including those of patient partners	Potential conflicts of interest for work group members were posted to a publicly accessible website that the patient community could access	Potential conflicts of interest for work group members were not publicly available for patients to access
All funding sources are publicly disclosed	Funding sources were explicitly acknowledged and patients could easily access the information	Funding sources were not disclosed and patients could not obtain the information

3. Inclusiveness of Patients

Patient-Centeredness Considerations	Examples of Inclusiveness*	
	High	**Low**
A role for a medical ethicist is considered	Including a medical ethicist on the team was discussed, and the rationale for not including one was provided in the report	Inclusion of a medical ethicist was not mentioned in supporting documents
The draft model is vetted with a broad coalition of stakeholders, including patients	A broad coalition of patient organizations was given appropriate time to vet the model	Notification of public comment period(s) was not widely distributed
Patient partners are engaged to support the dissemination and implementation of the model	Patient partners have been acting as ambassadors to communicate the model	Patient partners have not been involved in the dissemination and implementation of the model

4. Diversity of Patients/Populations

Patient-Centeredness Considerations	Examples of Diversity*	
	High	**Low**
Differences in patient perceptions of value, that shift over time as patient circumstances change, are acknowledged and considered (reflects expected stages over time)	Since different perceptions of value were proved by patients as the disease progressed, the model accounted for this change over time	Consideration was not given to patient-reported shifts in perceptions of value based on disease progression

Patient-Centeredness Considerations	Examples of Diversity*	
	High	Low
Applicability and limitations across patient subpopulations and disease trajectory are acknowledged and considered	Information was provided on the model's limitations with regard to the younger subpopulation of patients	The model's limitations regarding applicability in the younger subpopulation were not addressed
Processes are included for identifying and incorporating new knowledge regarding patient subpopulations and disease trajectory	A mechanism was described that allows patients and other stakeholders to suggest when an model update is necessary due to new or changing information	No mechanism was offered for patients to suggest when an update is needed

5. Outcomes Patients Care About

Patient-Centeredness Considerations	Examples of Outcomes*	
	High	Low
Economic inputs are considered in the context of a patient's experience	The model incorporated costs from a variety of stakeholder perspectives, including patient out-of-pocket costs	Only cost issues from the payer perspective were included without rationale for exclusion of patient costs
Processes are in place for identifying and incorporating emerging information on outcomes of importance to patients	A mechanism was described that allows patients and other stakeholders to suggest when an model update is necessary due to new or changing information	No mechanism was offered for patients to suggest when an update is needed

6. Patient-Centered Data Sources

Patient-Centeredness Considerations	Examples of Data Sources*	
	High	Low
Data beyond randomized controlled trials are considered (e.g., natural history of disease, patient views, outcomes and/or treatments, patient preferences regarding outcome or treatment characteristics)	The report described all data sources used, including data from a patient registry and a health-related quality-of-life study	The model only included clinical trial data submitted to FDA as part of a new drug application
Rationale for the inclusion or exclusion of available data sources is provided and information is provided in a patient-friendly way	Supporting documents clearly included a discussion of the work group's decision to exclude a data source on patient-reported outcomes after discovering substantial study limitations	No rationale was provided for why one particular patient registry was included in the model, while others were excluded

Patient-Centeredness Considerations	Examples of Data Sources*	
	High	Low
Processes are in place for identifying and incorporating emerging data sources, in particular patient-generated health data	The report clearly described the process for identifying and incorporating emerging data and how and when it will be included in an updated model	No systematic approach was described regarding identifying emerging data

Conclusion

Value models can help advance the national dialogue on value in health care, but only if these frameworks incorporate the patient voice. Value models must provide enough information such that patients and other stakeholders can assess the model's patient-centeredness in order to determine if the model should be factored into health care decision making.

The National Health Council Value Model Rubric can assist all stakeholders, especially the patient community, in assessing the level of patient centeredness and engagement in a given value model. It is also designed to support model developers in conceptualizing plans for meaningfully engaging patients. This Value Model Rubric is the first step in structuring truly patient-centered value models that patients and their families can rely on.

The National Health Council invites use of this model by health care stakeholders and welcomes comments based on use experiences that can help us strengthen the Value Model Rubric.

Appendix A. The National Health Council thanks the following organizations for providing their assistance and expertise with this initiative.*

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American Cancer Society Cancer Action Network, Inc.

Aetna Inc.

American Society of Anesthesiologists

Amgen Inc.

Asthma and Allergy Foundation of America

Bristol-Myers Squibb Company

Cancer Support Community

Eli Lilly and Company

FH (Familial Hypercholesterolemia) Foundation

National Consumers League

National Multiple Sclerosis Society

National Patient Advocate Foundation

Parent Project Muscular Dystrophy

Patient-Centered Outcomes Research Institute (PCORI)

Sanofi

Society for Women's Health Research

*The list of participants will be continuously updated on the National Health Council website.

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