

# The National Health Council Rubric to Capture the Patient Voice:

A Guide to Incorporating the Patient Voice into the Health Ecosystem



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## About the National Health Council

Founded in 1920, the National Health Council (NHC) brings diverse organizations together to forge consensus and drive patient-centered health policy. The NHC provides a united voice for the more than 160 million people with chronic diseases and disabilities and their family caregivers. Made up of more than 125 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient advocacy organizations, which control its governance and policy-making process. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses representing biopharmaceutical, device, diagnostic, generic, and payer organizations.

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# The NHC Rubric to Capture the Patient Voice

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# Executive Summary

The purpose of the National Health Council (NHC) Rubric to Capture the Patient Voice (Rubric) is to provide a tool the patient community or any other health care stakeholder can use to evaluate attributes of patient centeredness and to guide them on meaningful patient engagement throughout any activity they might undertake. It is broadly applicable and more general in scope as compared to prior patient-engagement rubrics. The Rubric is not intended to replace other patient engagement/centricity rubrics and guides that promote patient centricity in specific applications (e.g., the Patient Engagement Quality Guidance, which is intended to guide patient-centricity in medical product development).

Rubric development was undertaken by the NHC Rubric Committee to Capture the Patient Voice. The Committee met in February 2019 to evaluate past rubrics, consider the core domains of patient centeredness, and identify examples and up-front materials needed. The draft Rubric was initially sent to the Committee for feedback, revised, and then was opened for a 3-week public comment period. The Rubric includes level-setting definitions and two main sections:

- **Section I: Meaningful Patient Engagement Processes.** This section outlines characteristics of direct, meaningful patient engagement.
- **Section II. General Patient-Centeredness Considerations.** This section focuses on approaches that enhance patient centeredness throughout an activity but are not direct patient engagement.

The domains of patient centeredness include:

1. Patient Partnership
2. Transparency
3. Representativeness
4. Diversity
5. Outcomes Patients Care About
6. Patient-Centered Data Sources and Methods
7. Timeliness

To provide a prospective user with a practical tool, we include, for each characteristic, examples of what “meaningful” activities look like, as well as examples of what insufficient activities might look like. For example, for the Meaningful Patient Engagement Process component, in the Patient Partnership domain, a characteristic is that patients are recognized as partners.

## Patient Partnership

Characteristics of Meaningful Patient Engagement	Examples of Patient Partnership	
	Meaningful	↔

Patients are recognized as partners and integrated in all development phases.	A Patient and Family Advisory Council identified a challenge, co-developed a solution with hospital staff, implemented the planned solution, and measured the impact.	A Patient and Family Advisory Council identified a challenge, but hospital administrators and health care providers developed and implemented their solution without feedback from the Council.
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We envision that the Rubric will be broadly useful across a range of activities, including research; medical-product development, including biopharmaceuticals, devices, and diagnostics; health care delivery and shared decision making; value assessment; real-world data analyses; evidence generation; scientific exchange and communication; and health policy. This Rubric can serve as a general blueprint in a wide range of circumstances where patient centricity is desired.

# The NHC Rubric to Capture the Patient Voice

## Introduction

Today, after decades of hard work and innovation, there is finally broad consensus in the US and globally that patients should be engaged in all aspects of health, from research, to developing a new treatment, to care delivery, and in policy. While this cultural shift has been welcomed by many, especially those in the patient community, there is still more work to be done to achieve a patient-centered health ecosystem. Specifically, there remains confusion about and misuse of what it means to be patient centered and how to achieve meaningful patient engagement.

Patient perspectives can differ significantly from that of health care providers and payers, often integrating considerations beyond clinical outcomes and cost, such as a treatment's ability to help achieve personal goals.<sup>1</sup> To have true utility, health-focused efforts must incorporate these patient 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 as health care providers, payers, or policy makers as well as medical product developers seek to inform decisions that can affect patients' lives.

To support advances in patient centricity, the National Health Council (NHC), in a multi-stakeholder co-development effort, created this NHC Rubric to Capture the Patient Voice. This document builds on prior work done by the NHC, including the Patient-Centered Value Model Rubric and Dialogue on Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs.

## Uses and Intended Audiences

The purpose of the NHC Rubric to Capture the Patient Voice (Rubric) is to provide a tool the patient community or any other stakeholder can use to evaluate attributes of patient centeredness and to guide them on meaningful patient engagement throughout any activity they might undertake. It is broadly applicable and more general in scope as compared to prior patient-engagement rubrics, such as the Patient-Centered Value Model Rubric.<sup>2</sup>

Our goal is to provide a generic tool that helps users to further advance patient centeredness and meaningful engagement when looking at any facet of the health ecosystem. It is complementary to other patient engagement/centricity rubrics that capture the nuances of patient centricity in specific applications (e.g., PCORI's Engagement Rubric for Applicants). The Rubric is not intended to replace other patient engagement/centricity rubrics and guides that promote patient centricity in specific applications. For example, toolkits such as the Institute for Patient and Family Centered Care's (IPFCC) "Advancing the Practice of Patient- and Family-Centered Care in Hospitals: How to Get Started Guide" and Patient-Focused Medicines Development's (PFMD) "Patient Engagement Quality Guidance" can help ensure that an activity meets the expectations of this rubric.<sup>3,4</sup> This Rubric can provide a foundation for those interested in developing specific tools in areas where they do not exist. We did not use a scoring mechanism for this rubric, as it is intended to be a guide to the field of patient engagement as it evolves.

While not all aspects of the Rubric will be applicable to every circumstance, most elements can provide helpful guidance for achieving patient centricity. The circumstances where the Rubric may be applied include but are not limited to research; development of medical products, including

biopharmaceuticals, devices, and diagnostics; health care delivery and shared decision making; value assessment; real-world data analyses; evidence generation; scientific exchange and communication; and health policy. For example, in medical-product development, it may be applied to guide selection of outcome endpoints in patient-focused drug development or study protocol co-development. In health care delivery, it may be applied to guide implementation of a patient-guided quality-improvement initiative. In health policy, it may be applied to guide the design of a new program by a government agency. It can serve as a general blueprint in a wide range of circumstances where patient centrality is desired.

## Definitions

### 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.

- A **patient** is someone having or at-risk of having a medical condition(s), whether or not they currently receive vaccines, medicines, or other therapies to prevent or treat a disease.<sup>5</sup> They are dependent on the health care system after the diagnosis of a medical condition or disability. A patient relies on the health care system to feel better and to have a longer, healthier, and more robust life. An individual patient’s views on health issues, such as the benefit and risk of new treatments, will vary depending on the severity of his or her condition and personal circumstances.<sup>i</sup>
- A **family caregiver** is someone caring for those with a medical condition(s).<sup>5,7ii</sup> Their care recipients are dependent on the health care system after the diagnosis of a medical condition or disability. A caregiver’s views on health issues, such as the benefit and risk of new treatments, will vary depending on the severity of the condition and personal circumstances of those they care for, as well as their own personal circumstances.<sup>iii</sup> The terms “patient-identified” or “patient-designated” care partners or caregivers are also used.<sup>8</sup>
- A **patient organization** is a 501(c)(3) organization that has a mission 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.<sup>5</sup> It engages in programs, such as research, education, advocacy, and service to individuals and communities. It generally takes a holistic view of the conditions for the

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<sup>i</sup> Patients who are also health care providers provide a “dual perspective.” While also important, this perspective is unlikely to represent non-health care-provider patient experiences navigating the health care system or evidence base. The vernacular used by patients with a dual perspective may also differ from other patients.<sup>6</sup>

<sup>ii</sup> “Family” is defined broadly in this context. “Patients define their “family” and how they will be involved in care, care planning, and decision-making. Family members, as identified by the patient, provide support, comfort, and important information during ambulatory care experiences, a hospital stay in critical care, medical/surgical, and specialty units, in an emergency room visit, and in the transition to home and community care.” - From the Institute for Patient and Family Centered Care (IPFCC)<sup>8</sup>

<sup>iii</sup> An alternative to a family caregiver is a patient-identified care partner<sup>9</sup>

patients it represents and seeks universal support from stakeholders for its mission and programs.

- **Online patient communities** are internet-based platforms that unite patients who have been diagnosed with a disease. They offer support, networking and/or information sharing.<sup>10,11</sup> Platforms may also help patients record health information and/or become involved in research. For researchers, online patient communities can be a useful resource for identifying patients to recruit as advisers or to participate in a study (e.g., qualitative study on patient experience, clinical trial participant, or surveys of opinions/experiences). Online communities are operated by patient organizations, provider groups, and others including for-profit organizations. For-profit organizations should not be confused with patient organizations, as they typically collect data but do not advocate for a specific constituency and utilize patient data primarily for commercial purposes.

The patient community is heterogeneous and brings to the discussion different perspectives informed by their experiences, trajectory or stage of disease, level of expertise, and many other personal, community, and societal factors.

## Consumers' and Patients' Perspectives Differ

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.<sup>5</sup> This difference may lead to perspectives on new treatments that vary in important ways. Both are important, but one cannot typically substitute for the other.<sup>iv</sup>

## Patient Engagement

**Patient engagement in research** refers to “the active, meaningful, authentic, and collaborative interaction between patients and researchers across all stages of the research process, where research decision-making is guided by patients’ contributions as partners, recognizing their unique experiences, values, and expertise.”<sup>12</sup> While this definition refers specifically to research, it is also applicable across stakeholders and engagement activities (e.g., participation in an interview, focus group).

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<sup>iv</sup> A typically healthy consumer may suffer an acute condition or illness thereby becoming a patient for a short period who can report her/his journey, experience, preferences, etc. with that acute illness.



## Insights Derived through Patient Engagement

**Patient-provided information** (PPI<sup>v,vi</sup>) broadly encompasses the entirety of information that can be collected from an interaction with a patient. This includes, but is not limited to, patient views on their disease(s)/condition(s), desired attributes for treatments; benefit- risk preferences, and desired goals and outcomes, as well as their experience with the disease and its management (including diagnosis, treatment, and unmet needs).

PPI includes, but is not limited to, information from patients about:

- Natural history of the disease
- Impact of the disease or chronic condition on patients and their family caregivers, and how it affects their daily activities, physical functions, and quality of life – overall and across key domains: social, physical, emotional, and functional
- Outcomes that are most important to the patient, both clinical and non-clinical (e.g., goals, daily activities, symptom reduction, or a standard of quality of life)
- Patients' preferences for treatment delivery methods and views on beneficial and negative aspects of treatment effects
- Experience on treatment(s) including symptoms and side effects and how the treatment impacts their daily activities, physical functions, and quality of life

As illustrated by this list, PPI covers a wide variety of input from the patient regarding the patient's experience, preferences, and needs. However, PPI does not include all information obtainable about a patient (see Figure 1). For example, pathology results are "patient information" and distinct from PPI because they are not conveyed directly by the patient.<sup>17</sup> Data stemming from wearables would be considered PPI, but is not patient-reported information. Additional relevant definitions are listed in Appendix I.

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<sup>v</sup> Note: patient-provided information is abbreviated PPI instead of PPI to avoid confusion with "patient preference information" abbreviated PPI by the FDA and "patient package insert", also abbreviated PPI by the FDA.<sup>13,14</sup>

<sup>vi</sup> Note: "patient-based evidence" is also used to describe PPI.<sup>15,16</sup>

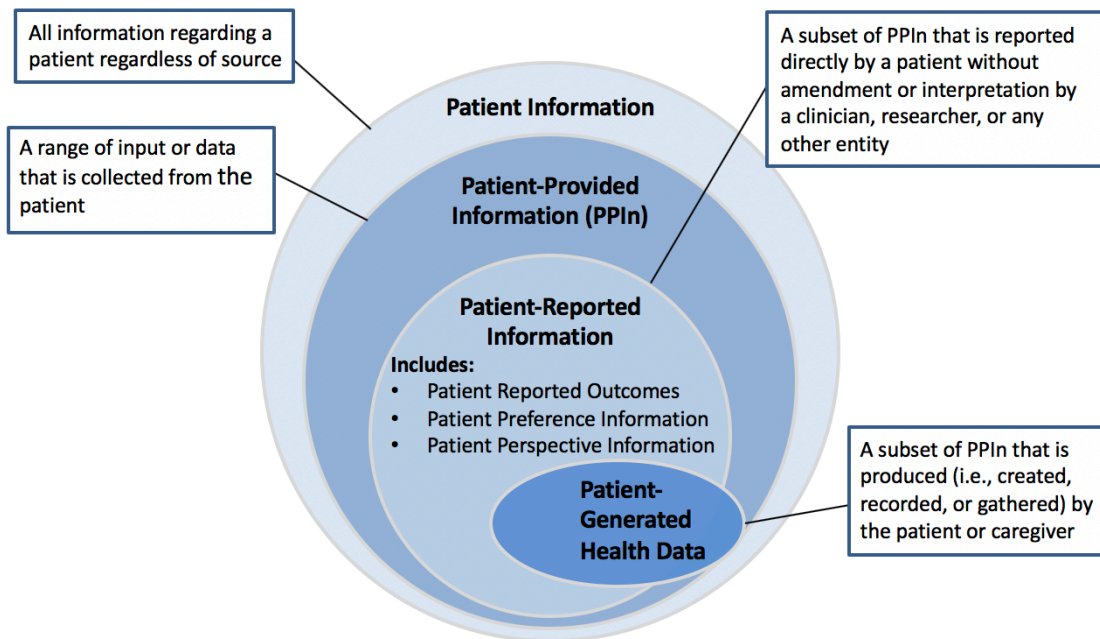


Figure 1. Types of Patient Information

## Patient Centric

The NHC defines **patient centered** as any process, program, or decision focused on patients in which patients play an active role as **meaningfully engaged participants**, and the central focus is on optimizing use of patient-provided information. Patient centered means doing things WITH – not FOR or TO – patients.<sup>5,18</sup> Meaningful engagement is an integral component of achieving patient centricity, but there are other activities one might conduct that contribute to patient centricity that are outside of direct patient engagement in the process.

**Patient-centered health care** is care that is respectful of and responsive to individual patient preferences, needs, and values in the context of their own social worlds. Patient centeredness is created by engaging, informing, and actively listening to patients at every point of contact – from the research bench to the bedside and everywhere in between.<sup>7,8</sup>

## Assessing Patient Engagement

**Meaningful Engagement** refers to direct relationships and partnerships that are bi-directional, reciprocal, and continuous. Communications are open, honest, and clear. Engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent.<sup>5</sup> A variety of “levels” of patient engagement exist and the appropriateness of engagement method selection is context specific.

It is assumed that “good” patient engagement would have a combination of actions (Figure 3); however, the ideal scenario is one where patients are key drivers of the effort instead of simply

observers to efforts on their behalf. Considering patients as study subjects or just having casual discussions misses the mark entirely.

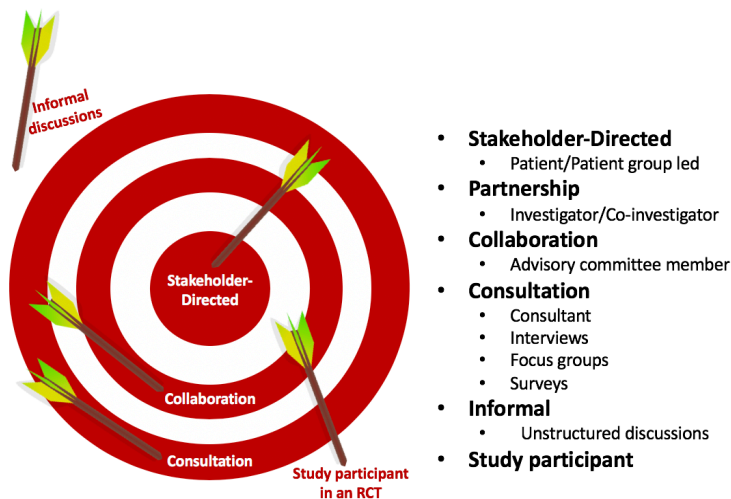


Figure 2. Examples of Levels of Engagement<sup>19,20</sup>

## Domains of Patient Centeredness

Over the past few years, the NHC has held several multi-stakeholder roundtables with the objective of capturing what it means to be patient centered. During the roundtables, participants discussed their patient-advocacy experiences, reviewed existing patient-engagement rubrics, and considered the hallmark characteristics of patient centeredness.

The following domains were considered in preparation of the Rubric to Capture the Patient Voice:

### 1. Patient Partnership

Patients should be involved in every step of the process, including planning and dissemination. Key characteristics of this partnership include: reciprocity, co-learning, co-development/creation, trust, transparency, honesty, and respect.<sup>21,22</sup>

Consideration should be given as to whether tools or other resources are needed to support patient partners (e.g., training on terminology). To facilitate sustainable partnerships, patient partners should be compensated for their time, and legal contracts should be understandable to laypeople. The NHC is currently building tools to help US-based stakeholders identify appropriate compensation and agreements based on the scope of engagement<sup>23</sup> Corresponding European efforts are being led by Patient-Focused Medicines Development (PFMD), WECAN, and Myeloma Patients Europe.<sup>24</sup>

### 2. Transparency

All activities should be conducted in an open way, and assumptions, inputs, processes, and results need to be disclosed to patients in plain language and a timely fashion.<sup>2,25</sup> The health literacy of the

target population should be considered when developing communication strategies.<sup>26</sup> The impact of patient engagement or patient input on the activity (e.g., study design or other process or outcome the engagement/input was intended to inform) should be shared back with participants.

### 3. Representativeness

Representativeness connotes that a sufficient number and types of people are included in the engagement activity to ensure that those engaged can speak on behalf of the target population. It refers to “who” and “how many” individuals to include in an interaction in order to, as closely as possible, engage with individuals that represent the broader, target patient population.<sup>27</sup>

Importantly, an effort to meet “representativeness” targets for an engagement is distinct from statistical sampling in that it focuses more on identifying individuals with the desired characteristics (and considering any challenges to doing so such as in the case of a rare disease), rather than meeting a known statistical threshold for the number of patient participants.

Representativeness is context specific, what the group initiating the interaction is aiming to accomplish affects the definition of patient representativeness for that effort. For example, what “good” patient representativeness looks like when the desired objective is to learn the patient perspective on how easy or difficult it is to follow a care regimen may vary greatly from what “good” representativeness looks like when the goal is to determine how patients with cancer define the value of a therapy. In some cases, engagement with only one individual patient may be sufficient to achieve representativeness, while other types of engagements may call for individuals from or representing a specific sub-population or all sub-populations relevant to a particular issue or question. Other cases (e.g., population-based questions) may require greater numbers of individual patients to represent an entire community or may require a statistically valid sample.<sup>vii</sup>

### 4. Diversity

The activity should consider differences among patients, including patient subpopulations, trajectory of disease, and stage of a patient’s life.<sup>29</sup> At every stage of the development of the activity, every effort should be made to ensure diverse representation of the patients as well as the characteristics listed below. The activity should attempt to represent the proportion of the population impacted by this disease, even if their participation may be difficult due to the circumstances of their illness or social determinants. Examples of characteristics that define subpopulations include:

- Age
- Comorbid conditions
- Ethnicity
- Gender
- Genetics
- Geographic location
- Health literacy

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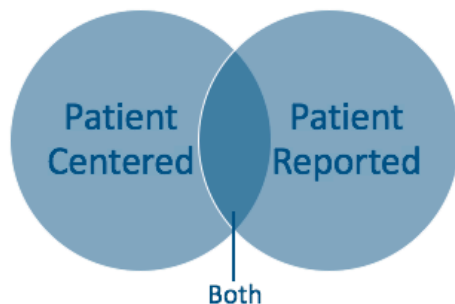
<sup>vii</sup> FDA’s draft guidance on “Patient-Focused Drug Development: Collecting Comprehensive and Representative Input Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders” provides extensive information on representativeness, including a review of sampling techniques relevant for medical product development.<sup>28</sup>

- Insurance coverage
- Rural, suburban, urban location
- Race
- Socioeconomic status
- Severity

## 5. Outcomes that Patients Care About

Whether the activity is research, policy, or care delivery oriented, the outcome(s) being measured should include those that patients state are important to them. Patient-centered (patient-prioritized) outcomes can only be identified by patients. Common methods for identifying patient-centered outcomes include but are not limited to interviews (e.g., concept-elicitation interviews) and focus groups. The FDA’s “Roadmap to Patient-Focused Outcome Measurement in Clinical Trials” provides useful considerations when identifying patient-centered outcomes.<sup>30</sup>

A common misconception is that all patient-centered outcomes are patient-reported outcomes (PROs) and all PRO’s are patient centered. Neither is true. Patients often prioritize outcomes such as pain or fatigue, which can only be captured through PRO measures (see Figure 2). However, patients also prioritize other outcomes that are captured using clinical measures such as biomarkers. Additionally, not all PROs were developed with patient input.<sup>31</sup> Some older measures that may still be in use today may not have been developed based on understanding what is most important to patients. It should be possible to update them to include and emphasize concepts most important to patients now. Use caution and do not assume a PRO tool is patient centered without understanding how it was developed or adapted to focus on what is important to patients.<sup>32</sup> As well, it should be relevant to patients experiencing the condition or disease today, which may be different based on the current therapeutic regimens, than in decades past, when a PRO instrument may have been developed.



### Patient-centered outcomes

- Outcomes patients report as important to them

### Patient-reported outcomes

- Outcomes reported only by the patient on how they feel or function

Figure 3. Patient-centered outcomes versus patient-reported outcomes

## 6. Patient-Centered Data Sources and Methods

Having a variety of credible sources can facilitate 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 in any effort should reflect the outcomes most important to patients and capture their experiences in the real world to the extent possible.

Patient centricity can be achieved by employing a range of methods, including both qualitative and quantitative. As technology evolves, so do the opportunities for innovative patient-engagement data collection.

## 7. Timeliness

The engagement should happen in alignment with the given objective of the activity. For example, if the objective of engagement is to solicit feedback from patients on how a clinical trial could be designed to reduce patient burden and improve enrollment, ideally, this engagement should occur as early as possible and well before the protocol has been drafted. Engaging patients when a protocol has already been developed may result in the need for costly protocol amendments or the inability to incorporate valuable insights.

## The NHC Rubric to Capture the Patient Voice

The Rubric to Capture the Patient Voice is intended to be a living tool to be refined over time based upon feedback from patients, patient groups, and other stakeholder-use experiences. The Rubric will require maintenance, updating, and enhancement as experience and knowledge is collected on its performance. It should be considered a guide for evolving patient-centeredness best practices.

Two sections comprise the NHC Rubric to Capture the Patient Voice:

- **Section I: Meaningful Patient Engagement Processes.** This section outlines characteristics of direct, meaningful patient engagement.
- **Section II. General Patient-Centeredness Considerations.** This section focuses on approaches that enhance patient centeredness throughout an activity but are not direct patient engagement.

The two sections of the Rubric to Capture the Patient Voice include a set of characteristics as well as examples of the type that represent meaningful or insufficient activity within each area.

As described, the intent is for the user to apply the Rubric to a wide range of activities that would be enhanced by patient engagement. Since that range is so broad, we have not enumerated all possible uses here. However, we encourage the user to identify and incorporate their intended use into the Rubric characteristics. For example, one characteristic under the Domain of Patient Partnership states:

Patients are recognized as partners and integrated in all development phases.

We encourage the user to think about phrasing it as:

- Patients are recognized as partners and integrated in all *[insert activity]* development phases.

Specific examples might include:

- Patients are recognized as partners and integrated in all *medical-product* development phases, or
- Patients are recognized as partners and integrated in all *policy-development* phases.

**Section I. A Meaningful Patient Engagement Process**

This section provides characteristics of meaningful engagement that illustrate efforts to incorporate the patient voice. While a clear understanding of “meaningful” may be elusive, for this document “meaningful engagement” refers to the definition outlined above.<sup>11,12,13,14</sup> Examples are organized into separate tables for each patient centeredness domain.

**\*Please note that the examples provided here are only intended to be illustrative of the characteristic. These examples are not intended to be exhaustive.**

**Domain: Patient Partnership**

Characteristics of Meaningful Patient Engagement	Examples of Patient Partnership	
	Meaningful	Insufficient/Low
Patients are recognized as partners and integrated in all development phases.	A Patient and Family Advisory Council identified a challenge, co-developed a solution with hospital staff, implemented the planned solution, and measured the impact.	A Patient and Family Advisory Council identified a challenge, but hospital administrators and health care providers developed and implemented their solution without input from the Council.
Patient partners are supported to enhance participation and have the capacity to engage.	Patient partners were provided with training and user-friendly, relevant materials, with adequate time to review them.	Patients were invited to participate one week prior to a project kick-off, while other participants received invitations well in advance, providing sufficient time to prepare.
Tools are available to patients to help them understand all aspects of the project.	A work group, with patient participation, crafted a communication strategy for equipping the patient collaborators.	No patient-specific communication materials were developed.
Patient expertise is acknowledged and relied upon.	A patient organization is invited to co-develop curriculum for medical students. The patient participants are encouraged to share suggestions on how care could be improved.	A single patient is invited to share his/her story with students over the course of a semester.

## Domain: Transparency to Patients

Characteristics of Meaningful Patient Engagement	Examples of Transparency	
	Meaningful	Insufficient/Low
The process for selection of patient partners/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. One or two may be invited close to the meeting date.
The patient community has early and timely continued opportunities for input on methods and drafts through multiple venues (e.g., direct access, public meetings or online comments).	Patient groups were invited and given at least three opportunities to review and provide comments without undue limitations on length (e.g., word count limits) or time to respond.	There is no effort made to ensure patient community comments are obtained.
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 health care providers. The tools were successfully tested with the users before public release.	A corresponding manuscript is published open access, but a patient-friendly summary is not included.

## Domain: Representativeness of Patients

Characteristics of Meaningful Patient Engagement	Examples of Patient Inclusiveness	
	Meaningful	Insufficient/Low
The objective of the engagement is aligned with the patient representative's(s') ability to provide useful information.	Researchers are interested in better understanding pathways to diagnosis for a broad range of patients. Through its registry, a patient group already collects patient-journey data from a diverse patient population across the US. A representative from the patient group is invited to present data from the registry to the researchers.	A single patient was invited to broadly describe the patient journey to diagnosis. The invited patient is pleased to present his own experience but is uncomfortable speaking on behalf of others.



## Domain: Diversity of Patients/Populations

Characteristics of Meaningful Patient Engagement	Examples of Diversity	
	Meaningful	Insufficient/Low
Diversity of the patient population is acknowledged and considered.	Thoughtful consideration was given to differences in patient perceptions across relevant patient sub-populations, including populations at-risk and those with early- and late-stage disease.	The project assumed the patient population is homogenous and takes a “one-size-fits-all” approach or is focused on the “average” patient.
Coordinated efforts are made to reach populations that are not always considered for patient input due to factors such as location or language barriers.	A health plan would like to improve the adherence rates of its beneficiaries to medication or treatment regimen. Through discussions with a variety of patients in urban, suburban, and rural settings and varying levels socioeconomic status, the plan identifies a variety of barriers to adherence. The plan co-develops solutions with representatives from each of the communities.	A health plan adopts a one-size-fits-all approach to improve adherence rates. The approach was developed with a local convenience sample near corporate headquarters from the same community.

## Domain: Outcomes Patients Care About

Characteristics of Meaningful Patient Engagement	Examples of Outcomes	
	Meaningful	Insufficient/Low
Outcomes important to patients are identified and incorporated, such as: <ul style="list-style-type: none"> <li>○ Functional status (mental/physical/societal)</li> <li>○ Symptoms</li> <li>○ Health-related quality of life, overall and in social, physical, emotional, and functional domains</li> <li>○ Well-being</li> <li>○ Clinical measures</li> <li>○ Survival</li> <li>○ Productivity</li> <li>○ Goals, expectations, aspirations</li> <li>○ Financial stress</li> </ul>	Researchers partnered with a patient organization to identify a disease-specific outcome measure. After speaking directly with patients, it was determined that the new measure should be a patient-reported outcome (PRO) measure. Patients were involved throughout the development and validation process.	A previously developed PRO measure was included in a trial as an exploratory endpoint to improve the patient centricity of a research study. However, the measure was not developed with patient input and did not reflect the outcomes currently most important to that patient population.

### Domain: Patient-Centered Data Sources

Characteristics of Meaningful Patient Engagement	Examples of Data Sources	
	Meaningful	Insufficient/Low
When gathering data, stakeholders consider how to best protect the patient's health privacy.	Data collected from a digital health application is anonymous, securely stored, and shared according to the patients' wishes.	Data was obtained from a digital health application and was used for research; the patients were not notified until after a report was completed.

### Domain: Timeliness

Characteristics of Meaningful Patient Engagement	Examples of Data Sources	
	Meaningful	Insufficient/Low
Engagement happens at the appropriate time(s) throughout the process.	A clinical practice guideline is being developed by a medical society. They would like to include a patient representative on their committee. The committee invites the patient representative to participate on the very first call and throughout the process.	After nearly completing development of a clinical practice guideline, a committee realizes that the guideline could benefit from the patient perspective. They invite a patient representative to participate on the committee just before the draft guideline goes out to the society's membership for final review.

## [Section II. General Patient-Centeredness Considerations](#)

Section II outlines considerations to enhance patient centeredness in general. Patients groups and other stakeholders can apply this section to evaluate the extent to which additional steps, beyond direct engagement efforts, have been taken to involve patients. These considerations enhance the patient centeredness of the processes, though direct patient engagement may not always be involved. 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.

### Domain: Patient Partnership

Patient Centeredness Considerations	Examples of Patient Partnership	
	Meaningful	Insufficient/Low
Rationale to substantiate the adequacy of the ratio of patient to non-patient participants is provided.	Of a 10-member advisory committee, two members were patients and one was a family caregiver, and a rationale was provided for stakeholder composition.	Of a 15-member clinical practice guideline development committee, the patient-perspective was provided by a single patient, while other stakeholder groups had at least two representatives. No rationale for stakeholder composition was provided.
Patients are engaged in pilot testing and refinement.	A disease-specific patient advocacy group partnered with a payer to test an intervention in practice.	No pilot testing with patient input was conducted.
Patients are engaged in providing technical assistance to activity end users.	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 the following: an adequate level of engagement occurred; the protocol reflected patient input; and improvements in engagement processes were offered.	No evaluation was conducted to assess patient engagement, and patients did not see any impact from their involvement.

## Domain: Transparency to Patients

Patient Centeredness Considerations	Examples of Transparency	
	Meaningful	Insufficient/Low
The purpose and goals are made clear to patients (including the intended audience and use) and are well-defined.	The goals are clearly represented and understandable to patients.	The goals are not clear to patients and do not include implications for patients.
The desired outcome of the engagement and implications for patients are made clear.	The desired outcome and implications for patient decision making are made clear, and the patients report they believe they have been included in the process.	Patients do not understand the desired outcome and fear it will hurt access for the most vulnerable patients.
The methodology(ies) used to engage patients is made transparent to patients in a timely manner.	Patients have timely access to detailed methods in understandable format if they want to review them.	Details of methodologic limitations were not disclosed to patients until after the project was completed.
All assumptions and inputs used are articulated in an understandable, patient-friendly way.	The assumptions and inputs were provided in a publicly accessible table, in a way a layperson can understand.	The assumptions and inputs were described in technical terms and are not easily retrievable.
Patient inputs considered but not used are described with the rationale for exclusion that patients can understand.	Methods described why certain patient registry data have been excluded.	Methods did not acknowledge existing data from a patient-advocacy survey of members that was excluded.
Results of 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 project.	Pilot testing with patients was conducted, but results were not disclosed to participants.
A clear distinction is made between pilot or interim stages/phases versus final in public communications that are accessible to patients.	The call for public comments was sent directly to relevant patient groups, was easily accessible to patients, and clearly described the project stage as final.	The call for public comment was difficult for patients to find on the website and did not disclose project stage or if there would be additional opportunities to comment.
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 if the activity is proceeding as intended are transparent and patients can understand them.	The methods for evaluating the project were described in detail, including goals and timeline, in a way that patients could understand.	No evaluation plan was provided.
Evaluation considers if deliverables/tools/products are being used as intended, achieving intended outcomes, and assessing unintended consequences for patients.	The evaluation led to changes 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 publicly accessible by the patient community.	Evaluation reports were only distributed to a small group that excluded patients.
Processes for updating and maintaining the activity are clear and accessible to the patient community.	The report explicitly listed factors that resulted 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.
Decision-making rationale for changes made to an activity is accessible, especially if it involves access or costs issues for patients.	Rationale for cost increases or decreases for specific services for patients were explicitly described and accessible to patients and their community.	A service used by patients suddenly jumped in price and patients could not obtain information on the cause.

## Domain: Representativeness of Patients

Patient Centeredness Considerations	Examples of Representativeness	
	Meaningful	Insufficient/Low
Opportunities for patients and patient organizations to participate are broadly disseminated and sufficient time is provided to respond.	A piece of legislation that, if passed, will have a substantial impact on patients, is released and broadly disseminated to the patient community. A 60-day comment period is provided to ensure that small and large patient organizations are able to appropriately respond.	A piece of legislation that, if passed, will have a substantial impact on patients, is released prior to a holiday weekend with only a ten-day comment period. Only patient groups with substantial resources are able to prepare comments.
Organizers consider which accommodations are needed for representative groups of patients to participate in an activity.	An externally-led Patient-Focused Drug Development meeting is held in Washington, DC. Grants to participate are offered and a live version is streamed over the web with many opportunities for those participating remotely to contribute to the meeting through a chat function and polls. Data collection is complemented by a survey in advance of the meeting.	An externally-led Patient-Focused Drug Development meeting is held in Washington, DC. While scholarships to travel to DC to participate are offered, the audio-visual equipment is insufficient to accommodate remote participation for those unable to travel.

## Domain: Diversity of Patients/Populations

Patient-Centeredness Considerations	Examples of Diversity	
	Meaningful	Insufficient/Low
Differences in health literacy are considered and reflected by the approach.	A clinical-practice guideline-development committee invited representatives from several patient groups to participate on the Committee. The Committee specifically asked the patient representatives to provide data on subpopulations, including by health literacy levels. Communications staff from those patient organizations were also asked to contribute their expertise communicating information to a variety of subpopulations.	Of a 15-member clinical-practice guideline-development committee a health care provider who had previously served on the committee for many years and was recently diagnosed with the condition was asked to provide the patient perspective.
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 update is necessary due to new or changing information.	No mechanism was offered for patients to suggest when an update is needed.
Applicability and limitations across patient subpopulations and disease trajectory are acknowledged and considered.	Information was provided by patient representatives on the model's limitations regarding the younger subpopulation of patients.	The project's limitations regarding applicability in the younger subpopulation were not addressed or acknowledged in the final report.

## Domain: Outcomes Patients Care About

Patient-Centeredness Considerations	Examples of Outcomes	
	Meaningful	Insufficient/Low
Clinical and economic inputs are considered in the context of a patient or caregiver's experiences.	Costs from a variety of stakeholder perspectives, including patient out-of-pocket costs or loss of productivity, were incorporated.	Only cost issues from the payer perspective were included without rationale for exclusion of patient costs or burden.
Processes are in place for identifying and incorporating emerging information on	A mechanism was described that allows patients and other stakeholders to suggest when an update is necessary	No mechanism was offered for patients to suggest when an update is needed, and no regular update is planned.

outcomes of importance to patients.	due to new or changing information.	
Optimal outcomes should consider recommendations by a patient proxy or caregiver, especially when the patient cannot provide information themselves.	Parents provided specific recommendations about disease trajectory for their infants and children, and these comments are incorporated into the study database.	A disease population is known to have family caregivers and few to none are asked for input on a project related to the disease.

## Domain: Patient-Centered Data Sources

Patient Centeredness Considerations	Examples of Data Sources	
	Meaningful	Insufficient/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.	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, while others were excluded.
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.	No systematic approach was described regarding identifying emerging data.
Existing sources of patient-generated health data (e.g., patient registries or patient-reported outcomes) are identified and considered while respect for patient privacy is maintained.	Data from a patient-generated patient registry contributes to the understanding of the natural history of a disease in a clinical practice guideline.	Physical function was identified by patients as the outcome of highest priority for them. A survey capturing data on physical functioning was identified, but not considered as part of a clinical practice guideline. No rationale was provided to explain why it wasn't used.



**Domain: Timeliness**

Patient Centeredness Considerations	Examples of Data Sources	
	Meaningful ←	→ Insufficient/Low
Engagement happens at the appropriate time(s) throughout the process.	<p>Researchers plan to apply for a research grant aimed to improve adherence to a medication. They reach out to the patient community six months before the proposal is due to provide sufficient time to understand why patients are non-adherent and to co-develop a proposed solution. The researchers invite members of the patient community to be co-investigators on the proposal.</p>	<p>Researchers independently identify an intervention to improve patients' adherence to a medication. Just before the proposal is due, researchers invite a patient to serve as co-investigator. From the patient community perspective, the proposed intervention doesn't adequately address barriers to adherence.</p>

## Real-World Vignettes

To assist with understanding how the Rubric can be applied, we offer these vignettes of patient engagement in various scenarios.

### Medical Product Development

In August 2018, for the first time, FDA cleared an expanded indication for a home hemodialysis machine. The device was originally cleared for hemodialysis at home, but only in the presence of a trained care partner. This meant that patients who did not have a trained care partner at home were not eligible for in-home hemodialysis. The sponsor worked with FDA and the Kidney Health Initiative to develop and conduct a [patient-preference study](#) to understand if patients are willing to accept the risks associated with hemodialysis at home without a qualified care partner. The FDA cited this survey in their decision based in part on asking kidney patients about their tolerance for risk.<sup>33–35</sup>

### Health Care Delivery

Example from the Institute for Patient and Family Centered Care's report on "Partnering with Patients and Families to Strengthen Approaches to the Opioid Epidemic"<sup>36</sup>

*Partnering with a PFAC to Inform Statewide Pain Management Education. :*

The Michigan Health and Hospital Association (MHA) Keystone Center has a Patient and Family Advisory Council (PFAC) that helps guide patient and family engagement-related work on a statewide level. Recognizing opioid misuse as a significant issue, MHA worked with its PFAC to change and improve pain management practices across the state. The PFAC identified several critical issues: a lack of appropriate education for patients about pain management, insufficient expectation-management regarding pain as a normal part of the healing process, and a lack of knowledge about how to safely dispose of leftover or expired pain medication.

MHA worked with its PFAC and a team of experts, including an anesthesiologist, pain management specialist, surgeon, and health literacy expert, to develop a resource entitled "Your Guide to Controlling and Managing Pain After Surgery." Released in 2013, the Guide includes tools for patients to document their pain management plan; tools for tracking pain, medications, and side effects; and information about how patients can participate in their care and why it is important to do so. MHA also worked with its PFAC on a public awareness campaign related to opioid use and pain management. The campaign included a Twitter thread that generated over 1.5 million impressions and included participation from the Michigan State Medical Society, state police, physicians' groups, and patients and families. Finally, learning from the PFAC that patients did not understand procedures for safe disposal of leftover pain medications, MHA worked with the Michigan Pharmacists Association and the Michigan Department of Environmental Quality to develop an education campaign and interactive map of "take back" locations across the state.<sup>36</sup>

### Policy-Making

The 21<sup>st</sup> Century Cures Act, signed into law in December of 2016, is a significant legislative change to the US health care system. While the bill was passed with bipartisan support in both chambers of Congress, it also enjoyed multi-stakeholder support across the health care ecosystem. This was thanks to the inclusive process

by which the bill was written. The development of the 21<sup>st</sup> Century Cures Act, much like the policy the legislation created, was focused on including the patient voice in the health ecosystem. While lobbyists and industry voices were certainly heard in the development of the bill, so too were patients' voices.

Informed by recommendations from patients, caregivers, and patient organizations, the bill ultimately included funding for President Obama's "Precision Medicine" initiative and makes regulatory changes at the Food & Drug Administration to foster patient-focused drug development. Months before the bill's final passage, more than 250 nonprofit organizations representing patients, family caregivers, health care providers, and researchers joined with the National Health Council in signing a letter of support for the 21st Century Cures Act to say:

"This bill is based on the hard work and thoughtful recommendations of the entire health community. This is a patient-focused bill that will advance the discovery and development of treatments, strengthen the patient voice in the regulatory environment, increase funding for the National Institutes of Health and Food and Drug Administration, and greatly improve our innovation ecosystem."<sup>37</sup>

### **Clinical Practice Guideline Development**

Example from the "American Society of Clinical Oncology's (ASCO) Outpatient Management of Fever and Neutropenia in Adults Treated for Malignancy Clinical Practice Guideline Update"

"This systematic review–based guideline product was developed by an Expert Panel with multidisciplinary expertise (Appendix Table A1, online only). A patient representative and an ASCO guidelines staff member with health research methodology experience were also included... The patient representative included in our Expert Panel highlighted the importance of communication between these health care providers and inpatients and outpatients regarding education about safety practices, what patients need to be aware of to communicate with health care providers, and expectations of patient and/or caregiver responsibility once the patient is discharged. Across the recommendations contained within this guideline, the patient representative highlighted that psychosocial and logistic requirements for outpatient management should be provided to patients and caregivers."<sup>38</sup>

## Appendix I. Definitions

Table 1. NHC definitions used in this document

<b>Consumer</b>	Generally healthy individual who will move in and out of the health care system as his or her needs change over time.
<b>Family Caregiver</b>	Someone caring for those with a medical condition(s). <sup>5,7viii</sup> Their care recipients are dependent on the health care system after the diagnosis of a medical condition or disability. A caregiver’s views on health issues, such as the benefit and risk of new treatments, will vary depending on the severity of the condition and personal circumstances of those they care for, as well as their own personal circumstances. The terms “patient-identified” or “patient-designated” care partners or caregivers are also used. <sup>8</sup>
<b>Meaningful Engagement</b>	Refers to direct relationships and partnerships that are bi-directional, reciprocal, and continuous. Communications are open, honest, and clear. Engagement goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent. <sup>5</sup> A variety of “levels” of patient engagement exist and the appropriateness of engagement method selection is context specific.
<b>Patient</b>	Someone having or at-risk of having a medical condition(s), whether or not they currently receive vaccines, medicines or other therapies to prevent or treat a disease. <sup>5</sup> They are dependent on the health care system after the diagnosis of a medical condition or disability. A patient relies on the health care system to feel better and to have a longer, healthier, and more robust life. An individual patient’s views on health issues, such as the benefit and risk of new treatments, will vary depending on the severity of his or her condition and personal circumstances.
<b>Patient Organization</b>	A 501(c)(3) organization that has a mission 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. <sup>5</sup> It engages in programs, such as research, education, advocacy, and service to individuals and communities. It generally takes a holistic view of the conditions for the patients it represents and seeks universal support from stakeholders for its mission and programs.
<b>Patient Community</b>	Broadly encompasses individual patients, family caregivers, and the organizations that represent them. The patient community is heterogeneous and brings to the discussion different perspectives informed by their experiences, trajectory or stage of disease, level of expertise, and many other personal, community, and societal factors.
<b>Patient Engagement in Research</b>	Refers to “the active, meaningful, authentic and collaborative interaction between patients and researchers across all stages of the research process, where research decision-making is guided by patients’ contributions as partners, recognizing their unique experiences, values and expertise.” <sup>12</sup> This definition is also applicable across stakeholders and engagement activities (e.g., participation in an interview, focus group).
<b>Patient-Provided Information</b>	Patient-provided information broadly encompasses the entirety of information that can be collected from an interaction with a patient. This includes, but is not limited to, patient views on their disease(s)/condition(s), desired attributes for treatments; benefit- risk preferences, and desired goals and outcomes, as well as their experience with the disease and its management (including diagnosis and treatment, and unmet needs).

<sup>viii</sup> “Family” is defined broadly in this context. “Patients define their “family” and how they will be involved in care, care planning, and decision-making. Family members, as identified by the patient, provide support, comfort, and important information during ambulatory care experiences, a hospital stay in critical care, medical/surgical, and specialty units, in an emergency room visit, and in the transition to home and community care.” - From the Institute for Patient and Family Centered Care (IPFCC)<sup>8</sup>

<b>Patient Centered</b>	Any process, program or decision focused on patients in which patients play an active role as meaningfully engaged participants, and the central focus is on optimizing use of patient-provided information. Patient centered means doing things WITH – not FOR or TO – patients. <sup>5,18</sup>
<b>Patient-Centered Health Care</b>	Care that is respectful of and responsive to individual patient preferences, needs, and values in the 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. <sup>39,40</sup>
<b>Online patient communities</b>	Internet-based platforms that unite patients who have been diagnosed with a diseases. They offer support, networking and/or information sharing. <sup>10,11</sup> Platforms may also help patients record health information and/or become involved in research. For researchers, online patient communities can be a useful resource for identifying patients to recruit as advisers or to participate in a study (e.g., qualitative study on patient experience, clinical trial participant, or surveys of opinions/experiences). Online communities are operated by patient organizations, provider groups, and others including for-profit organizations. For-profit organizations should not be confused with patient organizations, as they typically collect data but do not advocate for a specific constituency and utilize patient data primarily for commercial purposes.
<b>Representativeness</b>	Connotes that a sufficient number of and types of people are included in the engagement activity to ensure that those engaged can speak on behalf of the target population. It refers to “who” and “how many” individuals to include in an interaction in order to, as closely as possible, engage with individuals that represent the broader, target patient population. <sup>27</sup>

Table 2. Relevant FDA Definitions

<b>Patient Experience Data</b>	Defined in Title III, section 3001 of the 21st Century Cures Act, as amended by section 605 of the FDA Reauthorization Act of 2017 (FDARA) <sup>1</sup> and includes data that are collected by any persons and are intended to provide information about patients’ experiences with a disease or condition. Patient experience data can be interpreted as information that captures patients’ experiences, perspectives, needs, and priorities related to (but not limited to): 1) the symptoms of their condition and its natural history; 2) the impact of the conditions on their functioning and quality of life; 3) their experience with treatments; 4) input on which outcomes are important to them; 5) patient preferences for outcomes and treatments; and 6) the relative importance of any issue as defined by patients. <sup>41</sup>
<b>Patient Input</b>	Defined in the FDA’s Patient-Focused Drug Development Glossary: information that captures patients’ experiences, perspectives, needs, and priorities. <sup>41</sup>
<b>Patient Preference Information (PPI)</b>	Defined in the FDA Guidance on PPI for Medical Devices Document, Patient Preference Information are the assessments of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions. The methods for collecting PPI may be qualitative, quantitative, or mixed methods. <sup>13,41,40</sup>
<b>Patient-Provided Input</b>	Defined in the FDA’s Patient-Focused Drug Development Glossary: <i>Patient experience data</i> or other information that comes directly from patients. <sup>41</sup>
<b>Science of Patient Input</b>	Defined in the FDA’s Patient-Focused Drug Development Glossary: methods and approaches of systematically obtaining, analyzing, and using information that captures patients’ experiences, perspectives, needs, and priorities in support of the development and evaluation of medical products. <sup>41</sup>

## References

1. Perfetto EM, Oehrlein EM, Boutin M, Reid S, Gascho E. Value to Whom? The Patient Voice in the Value Discussion. *Value Health*. 2017;20(2):286-291. doi:10.1016/j.jval.2016.11.014
2. *The Patient Voice in Value: The National Health Council Patient-Centered Value Model Rubric*. National Health Council <http://www.nationalhealthcouncil.org/sites/default/files/Value-Rubric.pdf>.
3. Institute for Patient and Family Centered Care. Advancing the Practice of Patient- and Family-Centered Care in Hospitals: How to Get Started guide. [http://www.ipfcc.org/resources/getting\\_started.pdf](http://www.ipfcc.org/resources/getting_started.pdf). Accessed June 20, 2019.
4. Patient Focused Medicines Development. *The Patient Engagement Quality Guidance*. <https://patientfocusedmedicine.org/the-patient-engagement-quality-guidance/>. Accessed March 21, 2019.
5. Glossary of Patient Engagement Terms. National Health Council. <https://www.nationalhealthcouncil.org/resources/glossary-patient-engagement-terms>. Published February 13, 2019. Accessed March 19, 2019.
6. Johnson BH, Dokken DL, Abraham MR, Dardess P. Strategically Advancing Patient and Family Advisory Councils in New York State Hospitals. Institute for Patient- and Family-Centered Care. [http://www.ipfcc.org/bestpractices/NYSHF\\_2018\\_PFAC\\_Online\\_v3.pdf](http://www.ipfcc.org/bestpractices/NYSHF_2018_PFAC_Online_v3.pdf). Accessed June 3, 2019.
7. National Health Council, Genetic Alliance. Dialogue / Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs. <http://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf>. Accessed March 19, 2019.
8. Institute for Patient and Family Centered Care. Guidelines for the Presence and Participation of Families and Other Partners in Care—A Template. <http://www.ipfcc.org/bestpractices/guidelines-for-presence-of-families.pdf>. Accessed April 20, 2019.
9. Toolkit to Engage High-Risk Patients In Safe Transitions Across Ambulatory Settings. <https://www.ahrq.gov/professionals/quality-patient-safety/hais/tools/ambulatory-care/safetransitions.html>. Published December 8, 2017. Accessed June 3, 2019.
10. van der Eijk M, Faber MJ, Aarts JW, Kremer JA, Munneke M, Bloem BR. Using Online Health Communities to Deliver Patient-Centered Care to People With Chronic Conditions. *J Med Internet Res*. 2013;15(6). doi:10.2196/jmir.2476
11. Solberg LB. The Benefits of Online Health Communities. *AMA Journal of Ethics*. 2014;16(4):270-274. doi:10.1001/virtualmentor.2014.16.4.stas1-1404.
12. ISPOR Patient-Centered SIG. Defining Patient Centeredness and Engagement in HEOR: Proposed Definition and Stakeholder Response. [https://www.ispor.org/docs/default-source/presentations/1388.pdf?sfvrsn=ccb5658d\\_1](https://www.ispor.org/docs/default-source/presentations/1388.pdf?sfvrsn=ccb5658d_1). Published May 21, 2018. Accessed April 15, 2019.
13. Food and Drug Administration. Patient Preference Information (PPI) in Medical Device Decision-Making. *FDA*. May 2019. <http://www.fda.gov/about-fda/cdrh-patient-engagement/patient-preference-information-ppi-medical-device-decision-making>. Accessed June 3, 2019.
14. Commissioner O of the. Learn About Your Medicines. *FDA*. April 2019. <http://www.fda.gov/patients/learn-about-your-medicines>. Accessed June 3, 2019.

15. Staniszewska S, Crowe S, Badenoch D, Edwards C, Savage J, Norman W. The PRIME project: developing a patient evidence-base. *Health Expectations*. 2010;13(3):312-322. doi:10.1111/j.1369-7625.2010.00590.x
16. Staniszewska S, Werkö S. Patient-Based Evidence in HTA. In: Facey KM, Ploug Hansen H, Single ANV, eds. *Patient Involvement in Health Technology Assessment*. Singapore: Springer Singapore; 2017:43-50. doi:10.1007/978-981-10-4068-9\_4
17. National Health Council, Genetic Alliance. *Patient-Focused Drug Development – Recommended Language for Use in Guidance Document Development*. <http://www.nationalhealthcouncil.org/sites/default/files/NHC-GA%20Feb2017.pdf>.
18. What is PFCC? <http://www.ipfcc.org/about/pfcc.html>. Accessed June 20, 2019.
19. Forsythe LP, Ellis LE, Edmundson L, et al. Patient and Stakeholder Engagement in the PCORI Pilot Projects: Description and Lessons Learned. *J Gen Intern Med*. 2016;31(1):13-21. doi:10.1007/s11606-015-3450-z
20. Perfetto E, Gomel R. Engaging Patients at Multiple Levels. Presented at the: <https://www.pharmacy.umaryland.edu/media/SOP/wwwpharmacyumarylandedu/centers/PROEM/summer-institute/2016/engaging-patients-on-multiple-levels.pdf>.
21. Pomey M-P, Ghadiri DP, Karazivan P, Fernandez N, Clavel N. Patients as Partners: A Qualitative Study of Patients' Engagement in Their Health Care. *PLOS ONE*. 2015;10(4):e0122499. doi:10.1371/journal.pone.0122499
22. Patient-Centered Outcomes Research Institute. Engagement Rubric for Applicants. <https://www.pcori.org/sites/default/files/Engagement-Rubric.pdf>. Published June 6, 2016. Accessed June 19, 2019.
23. Tools to Support Sponsor-Patient Engagement: Fair-Market-Value Calculator and Engagement Templates. National Health Council. <https://www.nationalhealthcouncil.org/resources/fair-market-value-calculator>. Published March 29, 2019. Accessed June 3, 2019.
24. Patient Focused Medicines Development, National Health Council, Workgroup of European Cancer Patient Advocacy Networks (WECAN). Fair Market Value. <https://patientfocusedmedicine.org/fair-market-value/>. Accessed March 21, 2019.
25. TRANSPARENCY | meaning in the Cambridge English Dictionary. <https://dictionary.cambridge.org/dictionary/english/transparency>. Accessed March 21, 2019.
26. Protheroe J, Nutbeam D, Rowlands G. Health literacy: a necessity for increasing participation in health care. *Br J Gen Pract*. 2009;59(567):721-723. doi:10.3399/bjgp09X472584
27. National Health Council. *Tackling Representativeness: A Roadmap and Rubric*. <https://www.nationalhealthcouncil.org/sites/default/files/Representativeness%20in%20Patient%20Engagement.pdf>. Accessed March 11, 2019.
28. Food and Drug Administration. Patient-Focused Drug Development: Collecting Comprehensive and Representative Input. U.S. Food and Drug Administration. <http://www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-focused-drug-development-collecting-comprehensive-and-representative-input>. Published December 1, 2018. Accessed May 29, 2019.
29. National Health Council. Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool.

[http://www.nationalhealthcouncil.org/sites/default/files/NHCPatientInformationToolandinstructions\\_0.pdf](http://www.nationalhealthcouncil.org/sites/default/files/NHCPatientInformationToolandinstructions_0.pdf). Accessed March 29, 2019.

30. Food and Drug Administration. Roadmap to Patient-Focused Outcome Measurement in Clinical Trials. <https://www.fda.gov/media/87004/download>. Accessed May 29, 2019.
31. Oehrlein EM, Perfetto EM, Love TR, Chung Y, Ghafoori P. Patient-Reported Outcome Measures in the Food and Drug Administration Pilot Compendium: Meeting Today's Standards for Patient Engagement in Development? *Value Health*. 2018;21(8):967-972. doi:10.1016/j.jval.2018.01.004
32. Oehrlein EM, Perfetto EM, Hanna ML, Ganser TR, Chung Y, Ghafoori P. Patient-Centeredness in Clinical Outcomes Assessment. [https://www.ispor.org/docs/default-source/publications/value-outcomes-spotlight/january-february-2017/vos-patient-centeredness.pdf?sfvrsn=e961b854\\_2](https://www.ispor.org/docs/default-source/publications/value-outcomes-spotlight/january-february-2017/vos-patient-centeredness.pdf?sfvrsn=e961b854_2).
33. Hurst FP, Chianchiano D, Upchurch L, et al. Stimulating Patient Engagement in Medical Device Development in Kidney Disease: A Report of a Kidney Health Initiative Workshop. *Am J Kidney Dis*. 2017;70(4):561-569. doi:10.1053/j.ajkd.2017.03.013
34. Stroumbos M. Traditional 510(k) Premarket Notification. :8.
35. Medical Device Innovation Consortium. Using the MDIC PatientCentered Benefit-Risk Framework to Support an Expanded Indication:A Case Study of the Science of Patient Input Program of the Medical Device Innovation Consortium (MDIC). <https://mdic.org/wp-content/uploads/2019/04/NxStage-Case-Study.pdf>. Published February 1, 2019. Accessed June 3, 2019.
36. Dardess P, Dokken DL, Abraham MR, Johnson BH, Hoy L, Hoy S. *Partnering with Patients and Families to Strengthen Approaches to the Opioid Epidemic.*; :46. [http://www.ipfcc.org/bestpractices/opioid-epidemic/IPFCC\\_Opioid\\_White\\_Paper.pdf](http://www.ipfcc.org/bestpractices/opioid-epidemic/IPFCC_Opioid_White_Paper.pdf).
37. 21st Century Cures Patient Support Letter. <http://www.nationalhealthcouncil.org/sites/default/files/21CC-Patient-Support-Letter.pdf>. Published June 18, 2015. Accessed June 20, 2019.
38. Taplitz RA, Kennedy EB, Bow EJ, et al. Outpatient Management of Fever and Neutropenia in Adults Treated for Malignancy: American Society of Clinical Oncology and Infectious Diseases Society of America Clinical Practice Guideline Update. *J Clin Oncol*. 2018;36(14):1443-1453. doi:10.1200/JCO.2017.77.6211
39. Epstein RM, Street RL. The Values and Value of Patient-Centered Care. *Ann Fam Med*. 2011;9(2):100-103. doi:10.1370/afm.1239
40. Institute of Medicine (US) Committee on Quality of Health Care in America. *Crossing the Quality Chasm: A New Health System for the 21st Century*. Washington (DC): National Academies Press (US); 2001. <http://www.ncbi.nlm.nih.gov/books/NBK222274/>. Accessed December 18, 2018.
41. Food and Drug Administration. Patient-Focused Drug Development Glossary. <https://www.fda.gov/Drugs/DevelopmentApprovalProcess/ucm610317.htm>. Accessed April 25, 2019.