The National Health Council Patient Engagement Rubric:

A Guide to Incorporating the Patient Voice into the Health Ecosystem

NATIONAL HEALTH COUNCIL

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The NHC Patient Engagement Rubric

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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 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, we still see 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 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, 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 clinicians, payers, or policy makers seek to inform decisions that can affect the treatment options available to patients.

To support advances in patient centricity, the National Health Council (NHC), with multi-stakeholder input, has created this Patient Engagement Rubric. 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 Patient Engagement Rubric is to provide a tool the patient community or any other stakeholder can use to evaluate patient centeredness and to guide them on meaningful patient engagement throughout their processes. It is broadly applicable and less specific in scope as compared to prior rubrics, such as the Patient-Centered Value Model Rubric. Our goal is to provide a more 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 many other patient engagement/centricity rubrics, that capture the nuances of patient centricity in specific applications (e.g., PCORI's Engagement Rubric for Applicants).

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; medical product development, including biopharmaceuticals and devices; 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. In health care delivery, it may be applied to guide the charter of a Patient and Family Advisory Council for an inpatient facility. 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 centricity 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.

1
2 ▪ A **patient**[1] is someone having or at-risk of having a medical condition(s), whether or not they currently
3 receive medicines or vaccines to prevent or treat a disease. They are dependent on the health care
4 system after the diagnosis of a medical condition or disability. A patient relies on the health care system
5 to feel better and to have a longer, healthier, and more robust life. An individual patient's views on
6 health issues, such as the benefit and risk of new treatments, will vary depending on the severity of his
7 or her condition and personal circumstances.[i]
8
9 ▪ A **family caregiver**[1,2,ii] is someone caring for those with a medical condition(s). Their care recipients
10 are dependent on the health care system after the diagnosis of a medical condition or disability. A
11 caregiver's views on health issues, such as the benefit and risk of new treatments, will vary depending
12 on the severity of the condition and personal circumstances of those they care for.[iii] The terms "patient-
13 identified" or "patient-designated" care partners or caregivers are also used.[3]
14
15 ▪ A **Patient Advocacy Organization**[1] is a 501(c)(3) organization that has a mission to combat a
16 particular disease, disability, or group of diseases and disabilities, or to improve and protect the health
17 of a particular group of people. It engages in programs, such as research, education, advocacy, and
18 service to individuals and communities. It takes a holistic view of the conditions for the patients it
19 represents and seeks universal support from stakeholders for its mission and programs. While a patient
20 advocacy organization may advocate for patient access to care, they do not have prescribing authority;
21 formulary control, responsibility, or decision-making authority; or make drug purchases.
22
23 The patient community is heterogeneous and brings to the discussion different perspectives
24 informed by their experiences, trajectory or stage of disease, level of expertise, and many other
25 factors.
26
27 ### Consumers' and Patients' Perspectives Differ
28 It is important to note the distinction between patients and consumers in this context. Certainly,
29 patients and consumers share many concerns but their perspectives on health issues can differ.
30 A person with a chronic disease and/or disability relies on the health care system to enable a
31 longer, healthier, and more robust life. A consumer is a generally healthy individual who will
32 move in and out of the health care system as his or her needs change over time. This difference
33 may lead to perspectives on the value of new treatments that vary in important ways. Both are
34 important, but one cannot typically substitute for the other.

35 # Patient Engagement
36 **Patient engagement in research** refers to "the active, meaningful, authentic and collaborative
37 interaction between patients and researchers across all stages of the research process, where
38 research decision-making is guided by patients' contributions as partners, recognizing their unique
39 experiences, values and expertise."[4]

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[i] Patients who are also healthcare providers provide a "dual perspective." While also important, this perspective is unlikely to represent non-healthcare-provider-patient experiences navigating the healthcare system or evidence base. The vernacular used by patients with a dual perspective may also differ from other patients.(need a cite – IPFCC?)

[ii] "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)

[iii] An alternative to a family caregiver is a "Patient-identified caregiver." (need a cite-IPFCC?)

While this definition refers specifically to research, it is applicable across stakeholders and engagement activities.

Insights Derived through Patient Engagement

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).

PPI includes, but is not limited to, information from patients about:
- Natural history of the disease
- Impact of the disease or condition on patients and their family caregivers, and how it
- Affects their daily activities, physical functions, and quality of life
- 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 opinions about side 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). For example, pathology results are "patient information" and distinct from PPI because they are not conveyed directly by the patient.[5] Additional relevant definitions are listed in Appendix I.
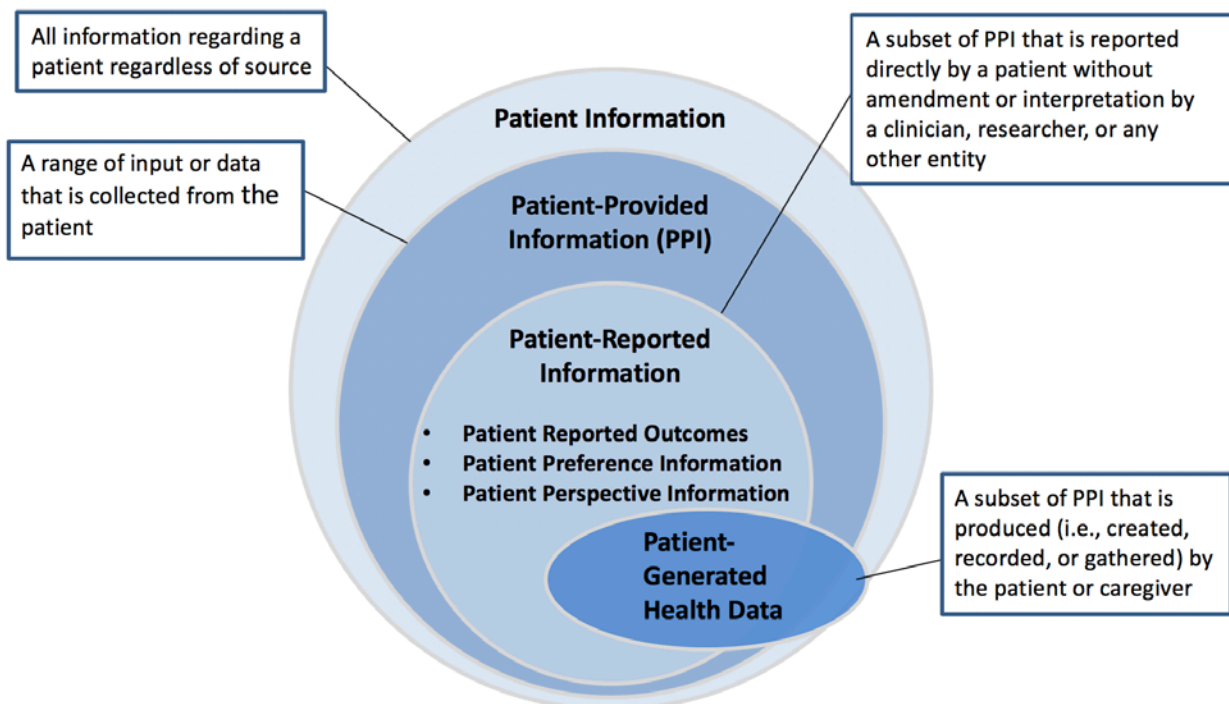
Figure 1. Types of Patient Information

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. 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 people with chronic conditions at every point of contact – from the research bench to the bedside and everywhere in between.[7,8]

Assessing Patient Engagement

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.[1] A variety of "levels" of patient engagement exist (see Figure). The appropriateness of engagement method selection is context specific.

The ideal scenario is one where patients are key drivers of the effort (hitting the bullseye) instead of just observers to efforts on their behalf. Considering patients as study subjects or just casual discussions misses the mark entirely.

- **Stakeholder-Directed**
  - Patient/Patient group led
- **Partnership**
  - Investigator/Co-investigator
- **Collaboration**
  - Advisory committee member
- **Consultation**
  - Consultant
  - Interviews
  - Focus groups
  - Surveys
- **Informal**
  - Unstructured discussions
- **Study participant**

Meaningful patient engagement is a bi-directional relationship between a patient or patients and another stakeholder or stakeholders. Communication, in a meaningful patient engagement undertaking, is open, honest, and clear. All goals, participants, methods, desired impacts, and actual impacts are clearly outlined and transparent.

Figure 2. Levels of Engagement[6,7]

Domains of Patient Centeredness

Over the past few years, the NHC 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 with, reviewed exiting patient-engagement rubrics, and considered the hallmark characteristics of patient centeredness.

The following domains were considered in preparation of the Patient Engagement Rubric.

1. Patient Partnership
Patients should be involved in every step of the process, including planning and dissemination.

Key characteristics include:
- Partnership
- Reciprocity
- Co-learning
- Co-development/creation
- Trust
- Transparency
- Honesty
- Respect

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 developing a fair-market-value calculator to help US-based stakeholders identify appropriate compensation based on the scope of engagement. Sample patient-friendly legal contracts are also under development. A corresponding European-focused project is being led by Patient-Focused Medicines Development (PFMD), WECAN, and Myeloma Patients Europe.[8]

2. Transparency
All activities should be conducted in an open way and assumptions, inputs, processes, and results need to be disclosed to patients in an understandable way and in a timely fashion.[9,10]
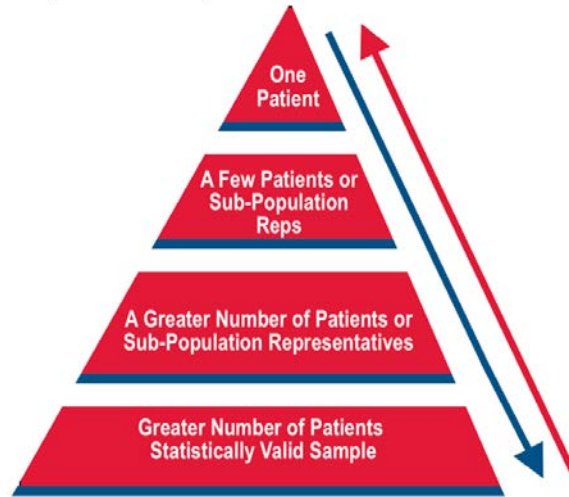
3. Representativeness
Representativeness connotes that a sufficient number of and types of people are included in the engagement activity to ensure that those engaged can speak on behalf of the target population. It refers to "who" and "how many" individuals to include in an interaction in order to, as closely as possible, engage with individuals that represent the broader, target patient population.[11]

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 limitations to doing that), rather than meeting a known statistical threshold for the number of patient participants.

Representativeness is context specific, what a group initiating the interaction (the "sponsor") is trying 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 difficult it is to follow a care regimen may vary greatly from what "good" patient representativeness looks like when the goal is to determine how patients with cancer define the value of a therapy. As illustrated by Figure 2 below, for some cases, engagement with only one individual patient may be sufficient to achieve

representativeness (top of the pyramid), while other engagements may call for individuals from or representing a specific sub-population or numerous sub-populations, and still others (e.g., population-based questions) can require greater numbers of individual patients to represent an entire community or may require a statistically valid sample (bottom of the pyramid).

Figure 3. Representativeness pyramid

4. Diversity

The activity should consider differences among patients, including patient subpopulations, trajectory of disease, and stage of a patient's life.[12] Examples of characteristics that define subpopulations include:

- Age
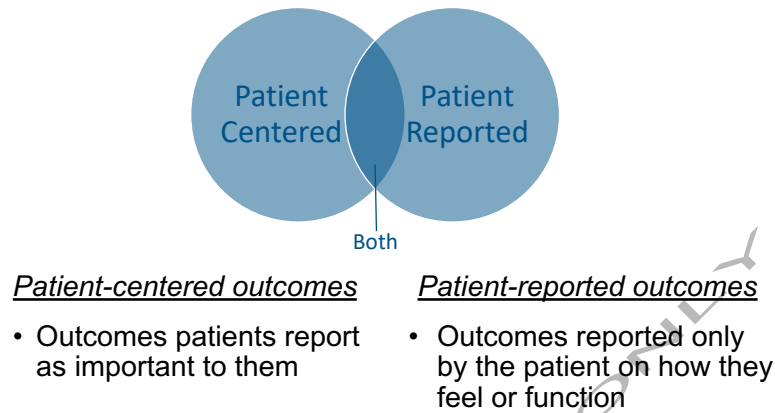- Comorbid conditions
- Genetics
- Geographic location
- Insurance coverage
- Socioeconomic status
- Severity
- Health literacy

5. Outcomes that Patients Care About

Whether the activity is research, policy or delivery oriented, the outcome(s) being measured should be those that patients state are important to them. Patient-centered (Patient-prioritized) outcomes can only be identified through patient engagement. Common methods for identifying patient-centered outcomes include but are not limited to interviews (e.g., concept elicitation interviews) and focus groups.

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 x). However,

patients also prioritize other outcomes that are best captured using clinical measures such as biomarkers. Additionally, not all PROs were developed with patient input.[13] Older measures – many still in use today – may not have been developed based on understanding what is most important to patients. Some older measures can be updated to include and emphasize concepts most important to patients. Use caution and do not assume a PRO tool is patient centered until you understand how it was developed or adapted to focus on what is important to patients.[14]

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 4. Patient-centered outcomes versus patient-reported outcomes

6. Patient-Centered Data Sources and Methods

A variety of credible sources is needed 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 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 to do the opportunities for innovative patient-engagement data-collection methods.

7. Timeliness

The objective of the activity needs to be aligned with the timing of the engagement and should happen at the appropriate time(s) given the objective throughout that 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 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 Patient Engagement Rubric

The Patient Engagement Rubric 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 future patient-centeredness best practices.

The Patient Engagement Rubric comprises two sections:

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

The two sections of the Patient Engagement Rubric include a set of characteristics and examples of the type of activities that represent high or low activity within each area are also included.

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 the specific uses here. But, 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. Example 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 evasive, for this document "meaningful engagement" refers to the definition outlined above.[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.

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 whether the impact.	A Patient and Family Advisory Council identified a challenge, but hospital administrators and clinicians developed and implemented their solution without feedback from the Council.
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.	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.

1	Patient expertise is acknowledged and relied upon	A patient organization is invited to co-develop curriculum for medical students. It is 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.

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 opportunities for input on method and drafts through multiple venues (e.g., direct access, 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),time to respond, or non-patient friendly language.	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 clinicians. 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.

(Row numbers in left margin: 2, 3, 4, 5)

Representativeness of Patients

Characteristics of Meaningful Patient Engagement	Examples of Inclusiveness*	
	Meaningful	**Insufficient/Low**
The objective of the engagement is aligned with the patient representative(s) ability to provide	Researchers are interested in better understanding pathways to diagnosis for a broad range of patients. Through their registry, a patient group already collects patient journey data from a diverse patient population located across the United States. A representative from the	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.

(Row numbers in left margin: 6, 7)

8		patient group is invited to present results from their registry to the researchers.	

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 subpopulations, 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
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 a medication. 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 convenience sample that all stemmed from the same community.

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: 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	Researchers partnered with a patient organization to develop 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 PRO measure was included in a trial as a secondary 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 most important to that patient population.

Patient-Centered Data Sources

	Examples of Data Sources*

Characteristics of Meaningful Patient Engagement	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.

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 clinical 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 thee 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 societies 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 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.

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, 2 members were patients and 1 was a family caregiver, and rationale was provided for stakeholder composition.	Of a 15-member clinical practice guideline development committee, a clinician who had previously served on the committee for many years, was listed.
Patients are engaged in pilot testing and refinement	A disease-specific patient advocacy group partnered with a payer to test the project in practice.	No pilot testing with patient input was conducted.
Patients are engaged in providing technical assistance to test 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, and patients did not see any impact from their involvement.

Transparency to Patients

Patient-Centeredness Considerations	Examples of Transparency*	
	Meaningful	**Insufficient/Low**

1	The purpose and goals 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 are clearly represented and understandable to patients.	The goals are not clear to patients and do not include implications for patients.
2	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.	Patients do not understand the desired outcome and fear it will hurt access for the most vulnerable patients.
3	The methodology(ies) used to engage patients 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 project was completed.
4	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.
5	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.
6	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.	Pilot testing with patients was conducted but results were not made public.
7	A clear distinction is made in public communications that are accessible to patients (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 the project stage.	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.
8	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.
9	Processes for evaluating if the project performs 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 patients can understand.	No evaluation plan was provided.
10	Evaluation considers if it is being used as intended, achieving intended outcomes, and assesses 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.

1	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.

DRAFT – FOR COMMENT ONLY – NOT FINAL

Patient-Centeredness Considerations	Examples of Transparency*	
	Meaningful	**Insufficient/Low**
Processes for updating and maintaining the project 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.
Decision making rationales for budgetary and cost changes in medication, products, or services for parents are accessible to the patient community	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.

Inclusiveness of Patients

Patient-Centeredness Considerations	Examples of Inclusiveness*	
	Meaningful	**Insufficient/Low**
A role for a medical ethicist is considered	The inclusion of a medical ethicist on the project 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 is vetted with a broad coalition of stakeholders, including patients, who should comprise a significant amount of the stakeholders	A broad coalition of patient organizations was given appropriate time to vet the project.	Notification of public comment period(s) was not widely distributed and no patients were asked to review documents.
Patient partners are engaged to support the dissemination and implementation of the project	Patient partners have been acting as ambassadors to communicate the project and know that the project includes their voice in the final report.	Patient partners have not been involved in the dissemination and implementation of the project.

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 clinician 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 acknowledge in the final report.

Outcomes Patients Care About

Patient-Centeredness Considerations	Examples of Outcomes*	
	Meaningful	Insufficient/Low
Economic inputs are considered in the context of a patient or caregiver's experiences	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 update is necessary due to new or changing information.	No mechanism was offered for patients to suggest when an update is needed.

Optimal outcomes should consider any recommendations given by the patient proxy or caregiver	Caregivers give specific recommendations about a disease trajectory and their comments are incorporated into the data for the projects final report.	A disease population is known to have family caregivers and few to none are asked for input on a project related to the disease.

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	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.

Timeliness

Characteristics of Meaningful Patient Engagement	Examples of Data Sources*	
	Meaningful	**Insufficient/Low**

| 8 | Engagement happens at the appropriate time(s) throughout the process. | 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 co-develop a proposed solution. The researchers invite members of the patient community to be co-investigators on the proposal. | Researchers independently identify an intervention to improve patients' adherence to a medication. Just before the proposal is due, researchers invite members of the patient community to serve as co-investigators. From the patient community perspective, the proposed intervention doesn't adequately address barriers to adherence. |

Real-World Vignettes

Medical Product Development

In August 2018, for the first time, FDA cleared an expanded indication for a home hemodialysis machine by NxStage so it could be used without a care partner being present, a decision based in part on asking kidney patients about their tolerance for risk.[15] The sponsor worked with FDA and the Kidney Health Initiative to construct and conduct a patient preference study. The objective was to expand the labeling for a home hemodialysis device that required patients with end-stage renal disease to have a caregiver present in order to use the system. The information collected was used to expand the labeling, allowing for patients to independently dialyze themselves.

Healthcare 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"*[16]

Partnering with a PFAC to Inform Statewide Pain Management Education: MHA Keystone Center:

"The Michigan Health and Hospital Association (MHA) Keystone Center has a 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 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 titled "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 chat 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."

Policy-Making

The 21st Century Cures Act, signed in to law in December of 2016, is the most significant legislative change to the US healthcare system since the Affordable Care

Act. While the bill was passed with bipartisan support in both chambers of congress, it also enjoyed multi-stakeholder support across the healthcare ecosystem. This is thanks to the inclusive process through which the bill was written. The development of the 21st Century Cures Act, much like the policy the bill creates, was focused on including the patient voice in determining patient care. While lobbyists and industry voices were certainly heard in the development of the bill, so too were patients'. Using recommendations by patients, caregivers, and patient organizations, the bill ultimately included funding for President Obama's "Precision Medicine" initiative which directs funding to patient-centered research as well as making regulatory changes to the FDA (Food & Drug Administration) that allows the agency to be more nimble when it comes to incorporating patient preferences in the drugs they approve and the information they disseminate. 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."

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 clinicians and inpatients and outpatients regarding education about safety practices, what patients need to be aware of to communicate with clinicians, 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."[17]

Appendix I. Relevant Definitions from FDA's Patient-Focused Drug Development Glossary[18]

Patient experience data	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)[1], 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. [18]
Patient input	Information that captures patients' experiences, perspectives, needs, and priorities. [18]
Patient preference information (PPI)	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 generating PPI may be qualitative, quantitative, or mixed methods. [18] (*Source:* FDA Guidance on PPI for medical devices)
Patient-provided input	*Patient experience data* or other information that comes directly from patients. [18]
Science of patient input	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. [18]

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