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This report was developed by Avalere and FasterCures staff, based on critical input received from representatives of the Steering Committee outlined below.

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Patient-Centered Outcomes Research Institute
Pharmaceutical Research and Manufacturers of America
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In addition to the above organizations, leadership and staff from across the Centers for Medicare and Medicaid Services (CMS) have participated in meetings and provided input to the framework.
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GLOSSARY

Patient-Perspective Value Framework (PPVF): The PPVF is made up of a set of patient-centered domains, technical criteria, data sources, and measurement methodologies that, with additional analyses, can be used in a variety of applications. The PPVF will not be an automated tool into which data can be entered to produce a score/assessment.

Application: a real-world use case of the PPVF. There are four initial types of applications of the PPVF: shared decision making, applied to existing value frameworks, support for public healthcare programs, condition-specific public analysis.

Domain: The PPVF includes five broad domains of patient value that represent core components of the patient’s perspective on value. The five domains are:

- **Patient Preferences**: assesses a patient’s personal goals and preferences. This domain functions as a lens through which the PPVF views the Patient-Centered Outcomes, Patient & Family Financial Considerations, and Quality & Applicability of Evidence associated with different healthcare options.

- **Patient-Centered Outcomes**: assesses the clinical, functional, and quality of life benefits and drawbacks of different healthcare options to the patient.

- **Patient & Family Financial Considerations**: assesses the medical, non-medical, and future out-of-pocket costs and other financial considerations associated with different healthcare options.

- **Quality & Applicability of Evidence**: assesses the strength and consistency of the evidence, as well as the degree to which the evidence applies to the individual patient. This domain functions as a lens through which we view the Patient-Centered Outcomes and Patient & Family Financial Considerations domains.

- **Usability & Transparency**: serves as a foundation for the PPVF and assesses the usability of the framework for its intended audience and transparency of the framework’s approach. This domain determines how the weighted assessments of the other domains are communicated through a particular application.

Criteria: narrower components of values that constitute a domain. As the PPVF is operationalized for a particular application, the criteria are used to assess each domain.

Measures: the specific factors used to measure the criteria within the Patient-Centered Outcomes (PCO) and Patient & Family Financial Considerations (PFFC) domains.

Methodological Considerations: the specific considerations taken into account when measuring the criteria within the Quality & Applicability of Evidence and Usability & Transparency domains.
Methods: the specific methodology for assessing the PPVF criteria and domains using a range of measures and data sources; and for weighting the domains, criteria, and measures based on patient preferences.

Shared Decision Making: a process in which clinicians and patients work together to make decisions among different healthcare options based on clinical evidence that balances risks and expected outcomes with patient preferences and values.¹

SECTION I. INTRODUCTION

Background and Impetus

At the 2015 Partnering for Cures (P4C) conference, Avalere Health and FasterCures held a workshop to discuss value frameworks and the degree to which they incorporate the patient perspective. In response to consensus of the necessity for a value framework centered around the patient perspective, we launched the Patient-Perspective Value Framework (PPVF) Initiative. Over several months of conversations with various constituencies, we formed a multi-stakeholder Steering Committee comprising 23 organizations. Representatives from patient groups, payers, life sciences companies, and other policy and research organizations have been meeting monthly since June 2016 to discuss this critical issue and collaborate to build the proposal outlined in this report.

Why Now?

2015 was marked by a proliferation of new models for assessing the value of treatments—including those of the American Society of Clinical Oncology (ASCO), the Institute for Clinical and Economic Review (ICER), the National Comprehensive Cancer Network (NCCN), and Memorial Sloan Kettering’s DrugAbacus. Each of these models relies primarily on traditional definitions of value that do not adequately incorporate the patient perspective, and many were not created for a patient-centered purpose. Some of these organizations have acknowledged this limitation and have begun to address it through their own processes. The PPVF Initiative aims to fill this gap by developing a framework for conducting patient-centered value assessments, and is actively engaging with other framework development organizations to identify synergies between the PPVF Initiative and each of their efforts.

Structure of the Initiative

As mentioned above, the PPVF Initiative is a multi-stakeholder collaborative guided by the PPVF Steering Committee (for a list of organizations represented on the Steering Committee, please visit this link2). Beginning in June 2016, the Steering Committee has met monthly. The majority of the sessions have been held over conference calls, with the exception of one in-person working session held in Washington, DC, in September 2016. Moreover, each Steering Committee member engages with Avalere and FasterCures on an individual basis each month through a phone call. Avalere and FasterCures also provide draft materials to the Steering Committee for feedback on a regular basis. These consistent communication efforts are designed to ensure that the Steering Committee has adequate opportunity for input at each stage of the brainstorming and drafting process.

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Practical Uses of the PPVF

In June 2017, Avalere and FasterCures will release a condition-agnostic framework to guide the assessment of the value of different healthcare options (i.e., drugs, devices, diagnostics, and other interventions) from the patient’s perspective and in a patient-centered way. It is important to note that this framework will not be a “tool” or a software application. The framework by itself will not allow for the assessment of a drug/device/diagnostic/intervention and provide a final “score” output, however, it will include detailed patient-centered domains, a set of technically specified criteria, and a set of measures, data sources, and associated preliminary methods for their evaluation. In order to fully operationalize the PPVF as a value assessment tool, we will need to apply it to a specific use case that pertains to a condition and healthcare options. In this draft methodology document, we do not build out any specific applications. Instead, we outline the ways in which the PPVF could function differently in different situations.

Ultimately, we envision that the PPVF will function in a variety of applications. For example, the PPVF could be incorporated into clinical decision support (CDS) systems and used as a platform for shared decision making (SDM) between patients and clinicians. In another application, the PPVF could be used for de novo population-level assessments of a set of therapies and other healthcare services. The potential future applications of the PPVF will be outlined in detail in Section V of this report, however, it is first important to note that there are two distinct buckets of potential applications of the PPVF:

- **Population-level applications**, which assess the value of a healthcare option for a population of patients and are largely used for policymaker and payer decision making
- **Individual-level applications**, which assess the value of a healthcare option for an individual patient, at the point of care, and are largely used by patients and clinicians in the clinical setting

Because the construct and methodology of the PPVF will vary based on the type of application, the Steering Committee largely agreed that the first application of the PPVF should be at an individual level, for shared decision making between patient and clinicians. However, this does not mean that the PPVF will not be applicable to other uses, and it will therefore ultimately describe how its construct and methodology will shift when used in a population-level application.

Over the next seven months, Avalere and FasterCures will continue to solicit extensive feedback, conduct additional research with patient groups, and begin developing specific applications. Our processes for these efforts are described in Sections V and VI. The information gained through feedback and through the development and testing of specific applications will inform our efforts to develop the PPVF. We plan to release version 1.0 of the PPVF by June 2017.
The Draft PPVF and Purpose of This Report

The purpose of this draft methodological report is to explain the methodological underpinnings of the draft PPVF. We have two primary motivations for releasing this level of detail in draft format:

1) As a call to action for patients, patient advocates, and other experts in this space to provide detailed feedback on this draft and offer suggestions as to how it might be applied. This feedback will be critical to the development of the PPVF, and we felt it was important to solicit it early in the process.

2) To ensure that our process and methodology are transparent.

In this report we will present the three draft components of the PPVF as they are outlined in the infographic associated with this report: 1) the domains and criteria; 2) the measures, data sources, and preliminary methods; and 3) the applications. Section II will provide an overview and summary of the three components. The domains and criteria; measures, data sources, and methods; and the applications will be presented in depth in Sections III, IV, and V, respectively. Finally, Section VI will provide further detail on our process for public input and provide a copy of our feedback questionnaire. This questionnaire is also available online.
SECTION II. SUMMARY: THE THREE PPVF COMPONENTS

The draft Patient-Perspective Value Framework (PPVF) comprises three components, which are described below.

Component 1: Domains and Criteria

The draft PPVF proposes five domains to represent five broad considerations that are important to patients when making healthcare decisions. The five domains of the PPVF are: Patient Preferences; Patient-Centered Outcomes; Patient & Family Financial Considerations; Quality & Applicability of Evidence; and Usability & Transparency. As proposed, these domains serve different purposes within the PPVF and add different kinds of information to the framework, but are all equally important to patient decision making.

Each domain is made up of a set of technically specified criteria that explain the factors to be considered when measuring a domain. For example, the Patient-Centered Outcomes domain includes the following criteria: quality of life, complexity of regimen, efficacy & effectiveness, treatment-related side effects/adverse events & complications. Further detail on the domains and a full list of the criteria included in each domain will be provided in Section III: Domains and Criteria.

Component 2: Measures, Data Sources, and Methods

The draft PPVF proposes to assess each criterion through a set of data sources, measures, and methods. For instance, quality of life, a criterion within the Patient-Centered Outcomes domain, will be measured by using available data sources to assess measures such as: health-related quality of life, functional/cognitive status, and palliation of symptoms/symptom-free intervals. Wherever possible, we propose to use data sources that incorporate patient-reported data.

The draft PPVF methodology starts with a measure of Patient Preferences and proposes to weight measures, criteria, and domains in accordance with measures of Patient Preferences. The methodology also includes a measure of heterogeneity of treatment effect (i.e., differences in how interventions affect different people) as a part of the Quality & Applicability of Evidence domain, which considers subpopulation data in an effort to measure the degree to which a particular treatment effect will occur for a particular patient.

In Section IV: Measures, Data Sources, and Methods, we provide further detail on our methods for selecting measures and data sources, and for assessing each criterion.

Component 3: Applications

The PPVF Initiative has proposed four potential future categories of applications for the PPVF. These categories include: deploying the PPVF as a shared decision making tool; incorporating the PPVF into existing value frameworks; using the PPVF to support
public healthcare programs; and using the PPVF for condition-specific public analyses. Avalere and FasterCures, in accordance with the Steering Committee’s guidance, propose to move forward with initially constructing the PPVF for use in a shared decision making application. In Section V: Roadmap to Applications, we outline how we envision moving from the draft PPVF to specific applications and why a shared decision making application was identified as a good starting point for the PPVF.
SECTION III. DOMAINS AND CRITERIA

The Patient-Perspective Value Framework (PPVF) consists of five broad draft domains of patient value. These draft domains were developed through a year-long iterative process that began at the 2015 Partnering for Cures (P4C) Conference, where we received input and feedback from an audience made up largely of patient advocates and life sciences companies. For additional information about the genesis of this project, please see the Avalere/FasterCures 2015 White Paper titled “Integrating the Patient Perspective into the Development of Value Frameworks.”³

Within each domain is a set of draft technically specified criteria that have been developed over the last five months with guidance from the PPVF Steering Committee. In the next phase of the framework development process, we will pressure test the draft domains and criteria with patients to ensure that: 1) they are measuring what is most important to patients when making care decisions; and 2) we are using language that is understandable and meaningful to patients. Through this draft document, we are seeking public input on the draft domains and the criteria within them. Specifically, we would value input on the following questions:

- Do the proposed five domains and criteria within them adequately capture the information most relevant (or important) to patients?
- Are there other elements that may be relevant for specific disease states that are not captured?

In this section, we first list the five draft domains and the draft criteria that sit within each one. Each draft domain contributes a different type of information to the framework and, as a result, each functions differently within the framework’s methodology. In this section we will therefore outline our proposal for how the five domains function, interact, and impact one another. For a detailed description of our proposed methodology for assessing each domain and criteria, and for creating a model for assessing various healthcare options, please see Section IV.

The PPVF’s Domains and Criteria

Below we have listed the five draft domains and the draft criteria within each domain. Each of the five domains is described in terms of the question it answers.

- **PATIENT PREFERENCES:** How do the patient’s personal goals and preferences influence their healthcare options?

  **Criteria:**

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• **PATIENT-CENTERED OUTCOMES**: What are the clinical, functional, and quality of life benefits/drawbacks of different healthcare options to the patient?

  *Criteria:*
  - Quality of life
  - Complexity of regimen
  - Efficacy & effectiveness
  - Side effects & complications

• **PATIENT & FAMILY FINANCIAL CONSIDERATIONS**: What are the overall costs of different healthcare options to the patient and family?

  *Criteria:*
  - Medical out-of-pocket (OOP) costs
  - Non-medical costs
  - Future costs of care

• **QUALITY & APPLICABILITY OF EVIDENCE**: What level of confidence does the patient have that a healthcare service will have specific effects for them?

  *Criteria:*
  - Quality of evidence
  - Consistency of evidence
  - Differences in treatment effect

• **USABILITY & TRANSPARENCY**: Are the framework and its applications usable and transparent in construct, content, and format?

  *Criteria:*
  - Transparent approach
  - Meaningful information
Assessment: How the Five Domains Function

Each domain contributes a different type of information on patient value. The ultimate purpose of the final PPVF will be to describe how each domain fits together to render an assessment for a healthcare service, in the format appropriate for a given application. In this section, we describe the different types of information each domain contributes and how each domain influences the others. **Figure 1** below depicts how the different domains impact each other.

**Figure 1: The Five Domains of the PPVF**

Patient-Centered Outcomes and Patient & Family Financial Considerations domains: These domains contribute objective information to the framework on the benefits and costs associated with different healthcare options.

The Quality & Applicability of Evidence domain touches both the Patient-Centered Outcomes and Patient & Family Financial Considerations domains. The quality of the evidence and the degree to which the evidence indicates that a healthcare option will have a particular effect for an individual patient is a key factor in how the PPVF considers outcomes and costs.

The Patient Preferences domain surrounds the Patient-Centered Outcomes, Patient & Family Financial Considerations, and Quality & Applicability of Evidence domains. The Patient Preferences lens shapes how one assesses the information contributed by each of these inner domains. For example, Patient Preferences determine the weighting of
each criterion within Patient-Centered Outcomes, and the PPVF’s assessment of different healthcare options is determined based on Patient Preferences. One can also imagine that different patients will have different preferences regarding the level of cost they are willing and able to bear, and the level of certainty of the evidence they require to move forward. The PPVF is organized so that Patient Preferences is a lens through which we view each of these domains.

Finally, Usability & Transparency rests beneath the other four domains as a foundation for the PPVF. This domain represents the PPVF’s commitment to ensuring that the framework has a transparent approach and that the information displayed through each application is appropriate for, accessible by, and useful and meaningful to its intended audience. This domain is critical to determining how the weighted assessments of Patient Preferences, Patient-Centered Outcomes, Patient & Family Financial Considerations, and Quality & Applicability of Evidence are communicated through a particular application. As indicated by its criteria, the Usability & Transparency domain contributes information about the audience that determines the type and level of information displayed. For example, a shared decision making application of the PPVF intended to facilitate a conversation between a patient and a clinician could display qualitative information about each criterion, rather than a single score output.
SECTION IV. MEASURES, DATA SOURCES AND METHODS

Overall Process for Healthcare Option Comparison

In this section, we will outline a draft proposed methodology for assessing each of the Patient-Perspective Value Framework’s (PPVF) domains: Patient Preferences, Patient-Centered Outcomes (PCO), Patient & Family Financial Considerations (PFFC), Quality and Applicability of Evidence (QAE), and Usability & Transparency, as well as a proposed methodology for presenting healthcare options. Figure 2 below outlines the PPVF’s overall proposed methodology for assessing healthcare options.

Figure 2: The PPVF’s Overall Process for Healthcare Option Comparison
Patient Preferences

Patient-centered care involves the active engagement of patients and full incorporation of patient preferences into clinical decisions through shared decision making, product development, and everything in between. Patient preferences can be defined as patient statements about the relative desirability of particular treatment options, treatment characteristics, and health states.\(^4\)

The incorporation of individual- and population-level patient preferences to assess the value of a healthcare option is a key differentiating component of the PPVF, rendering this domain central to the framework. The Patient Preferences domain assesses a patient’s values, needs, goals, expectations, and openness to financial trade-offs. This domain aims to answer the following question: “How do the patient’s personal goals and preferences influence their healthcare options?” These criteria represent the factors that will need to be measured at the outset, when operationalizing the framework into a specific application, to determine a patient’s preferences.

How do the patient’s personal goals and preferences influence their healthcare options?

How patient preferences will be elicited in the PPVF will vary according to the application. As demonstrated in Figure 2, the primary methodological consideration when operationalizing the Patient Preferences domain is whether the framework is being applied at the individual or population level. Therefore, a methodology that can successfully operationalize patient preferences at both the individual and the population level is vital to the development and application of the PPVF. In developing a proposed methodology for the Patient Preferences domain, we performed a review of available decision aid tools to inform an individual-level methodology and a review of patient preferences literature and available data sources to inform a population-level methodology. From this research, we identified which practices can be extrapolated and applied for the purposes of this framework and where gaps exist.\(^5,6\) We then outline our plans to conduct additional research as a next step in the framework development process, including new analysis that leverages patient experience registries. This research can be used to inform both individual- and population-level patient preference-eliciting tools, for future incorporation into the PPVF.

In the sections below, we outline approaches as to how patient preferences can be elicited and incorporated into the PPVF for both the individual- and population-level

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\(^5\) Ibid.

applications and then describe how the Patient Preferences domain impacts the other PPVF domains.

**Incorporating Individual-Level Patient Preferences into the PPVF**

When used as part of an individual-level application, where patients and providers use the PPVF to engage in a shared decision making conversation, we will determine Patient Preferences by asking the patient. To determine the best methodology for eliciting individual patient preferences for any given application, we would look to existing tools and resources, such as decision aids, designed to help patients describe their preferences and engage in the process of shared decision making with their clinicians. In this section, we include a short review of existing decision aids.

Decision aids can be grouped into two categories: 1) instruments designed for the pre-encounter visit and 2) encounter tools (e.g., option grids). Pre-encounter decision aids can be used to help patients make more informed and thoughtful choices on healthcare options by providing patients with information on the options and outcomes relevant to the person’s health status prior to meeting with the physician.\(^7\) In comparison, encounter tools are designed for use at the time of a clinical encounter. For example, option grids are a form of encounter tool that are meant to create a collaborative conversation between a patient and a clinician by outlining comparison answers to a patient’s frequently asked questions for different healthcare options.

Importantly, the literature indicates there is a need for these patient preference tools to be further developed and tested. For example, according to one study, we should focus on developing tools that are more tailored to a patient’s preferred style of assessment, whether analytical decision aids in the form of computer software or more intuitive instruments such as weight scales.\(^8\) Many organizations and researchers are working to address this gap by testing ways of systematically incorporating the criteria impacting patient preferences into new tools and decision aids that can help elicit patient preferences at decision points more readily and effectively.

**Incorporating Population-Level Patient Preferences into the PPVF**

Methods of operationalizing population-level patient preferences into assessments of value lag behind individual-level decision aids. The challenge in operationalizing patient preferences at the population level stems from the underlying question of who can realistically represent the patient voice, given that patient preferences are unique to the individual. However, a thorough review of the literature suggests that—within a specific disease state—there are patterns in patient preferences that may allow for some

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population-level assessment of patient preferences. There is a lot more work to do in this space, however, below we outline several potential methodologies for ascertaining population- and subpopulation-level patient preferences, depending on the availability of the data and the particular application of the PPVF.

A commonly used method for eliciting preferences is the standard gamble in which a choice is posed to a patient with a certain outcome and a gamble. The time trade-off is another technique in which patients choose between two certain outcomes and then the patients are asked how many years in a healthy state would be equivalent to a certain number of years in a poorer state. Rating scale instruments can be employed in which the patient might be asked to define the best and worst states of health for both ends of the scale and then the patient rates their desirability for different health states on the scale that they defined. Other techniques include the stated preference survey and discrete choice experiments (DCE). The stated preference survey can provide a systematic approach to quantitatively assessing the preference for features of cancer screening tests such as cost, efficacy, and process. DCEs are another technique that involves asking individuals to state their preference for different hypothetical scenarios, goods, or services. Methodologically sound population-level approaches have also recently been identified through the Medical Device Innovation Consortium’s (MDIC) work to catalog methods for incorporating patient preferences on benefits and risks into the regulatory assessment of medical technologies.

The Food and Drug Administration (FDA) has begun to take strides to incorporate patient perspectives in approval determinations. For instance, in August 2016, the FDA released

a guidance document outlining ways in which patient input, in particular perspectives on risk tolerance and benefit value, should be relevant in FDA decisions regarding medical device premarket approval and de novo classifications. This progress is illustrative of the progress being made in this space, and the ways in which regulatory processes will help drive the development of additional patient preferences data.

The PPVF could also draw from data sources such as patient experience registries, and other data that health plans collect from their members, to ascertain population-level patient preferences for a particular condition. For example, the Cancer Support Community’s (CSC) Cancer Experience Registry collects patient preference data to gain a greater understanding of the social and emotional needs of both the people impacted directly by cancer as well as their caregivers. Recent research conducted by CSC, with 679 cancer survivors in the Cancer Experience Registry, highlighted the relative importance of factors such as length of life, quality of life, impact on family, and financial cost of care for cancer patients (including non-metastatic breast, metastatic breast, prostate, ovarian, and other cancers). This type of data and research could be central in informing patient preferences when applying the PPVF in a population-level application.

Addressing Gaps in Patient Preferences Tools at the Individual and Population Level

As we continue to refine this draft and develop PPVF Version 1.0, Avalere and FasterCures are committed to working with patient groups within the PPVF’s Steering Committee and beyond to build on our existing knowledge base. Within the field of decision aids, we will work to determine the best patient preference-eliciting tools, resources, and/or interventions to incorporate into the PPVF. Finally, we will work with clinicians and patients to better understand the limitations and barriers to introducing preference and shared decision making tools at the point of care, including impact on clinician workflow and patient priorities. Improving decision aids is a challenge, but the PPVF creates a significant opportunity to define

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The Cancer Support Community is currently collecting patient preference data in its Cancer Experience Registry to gain a greater understanding of the social and emotional needs of both the people directly impacted by cancer as well as their caregivers.

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value from the patient’s perspective, and improved decision making tools will allow us to significantly impact patient and clinician decision making at the point of care.

We will also work to improve the available resources regarding patient preferences at a population level by partnering with patient groups and researchers to identify existing data sources and gaps in those data sources. Where gaps and limitations in available data persist, we will work with patient groups to outline recommendations for further research and actively seek opportunities to participate in that research.

Much of this work will happen in the context of building a particular application. Despite progress in this field of research, which is producing helpful proxies for measuring patient preferences at the population level, developing population-level applications will often require partnering with relevant patient groups to conduct new research and fill gaps in the literature with regard to patient preferences for specific conditions and disease areas.

**How the Patient Preferences Domain Impacts the Other PPVF Domains**

Once patient preferences have been elicited at either the individual or population level, this domain will serve as a weighting mechanism throughout the PPVF. This process will ensure that patient preferences are at the beginning, middle, and end of the entire value assessment process. The PPVF will use the Patient Preferences domain to weight and rank at three different levels within the PPVF: 1) within criteria, 2) among criteria, and 3) among domains.

**Patient Preferences weighting within the Patient-Centered Outcomes domain.**

Patient preferences can be used to weight and rank within the Patient-Centered Outcomes criteria; for example, within the complexity of regimen criterion, patients will have an option to indicate whether they have a preference over a treatment’s route of administration, its site of care, and its length or dosing schedule. A patient will also have the option of weighting and/or ranking among the criteria in the Patient-Centered Outcomes domain, based on whether a patient has a preference over a treatment’s impact on their overall quality of life, the complexity of a treatment’s regimen, a treatment’s efficacy and effectiveness, and a treatment’s side effects/adverse events and complications.

**Patient Preferences weighting within the Patient & Family Financial Considerations domain.**

Patient preferences will be used to weight and/or rank within the Patient & Family Financial Considerations criteria. For example, within the non-medical costs criterion, the patient will have an option to indicate whether they have a preference over a healthcare option’s effect on their wages, the associated cost of travel, or the costs of child and/or elder care, among other factors. A patient will also have the option of weighting and/or ranking among the criteria in the Patient & Family Financial Considerations domain, based on whether a patient has a preference over a healthcare option’s impact on their medical OOP costs for the entire episode of care, their non-
medical OOP costs, or the potential cost offsets and downstream OOP cost implications of the healthcare option.

**Patient Preferences weighting among the PPVF domains.** Finally, a patient will have the option of weighting and ranking each of the PPVF domains, based on their personal preferences among the clinical, functional, and quality of life benefits and drawbacks of a healthcare option (i.e., the Patient-Centered Outcomes domain); the overall costs they will incur for a healthcare option (i.e., the Patient & Family Financial Considerations domain); and the certainty that a healthcare option will have its purported effects for them (i.e., the Quality & Applicability of Evidence domain).

**Addressing Limitations: Shared Decision Making**

We recognize that these three levels of weighting and ranking could present a potential administrative or time burden to the patient and the clinician in the context of a shared decision making application; however, we felt that the benefits of creating a framework that is flexible enough for a patient to drill down as far as possible on all aspects of decision making, based on their level of comfort and understanding, outweighed such concerns at this juncture. We anticipate that decision support tools generally will have default weighting for those patients who do not wish to engage at that level of decision making. As part of our research moving forward and as we develop specific applications, we will pay special attention to overcoming the limitations of introducing preference tools at the point of care with regard to their impact on patient and clinician burden and workflow.

**Patient-Centered Outcomes**

Understanding the clinical benefits and risks of different therapy options is central to any value assessment. Existing value frameworks largely fall short of consistently measuring outcomes that matter to patients, such as a therapy’s effect on functional and cognitive status and a regimen’s complexity of administration.\(^{23,24,25,26}\) Reasons for non-inclusion of these outcomes can often be found in the inclusion/exclusion criteria that are used to assess the evidence base for informing the framework regarding the comparators’ benefits and risks. For example, the current frameworks’ general reliance on randomized control trial (RCT) design data can limit the ability to measure the clinical benefits/drawbacks of a specific therapy to a patient, given the limited generalizability of RCT data to real-world settings. Moreover, some existing value frameworks rely on the use of Quality Adjusted Life Years (QALY) to assess the value of a therapy, which has been criticized by various stakeholders as too rigid a measure that does not appropriately take into account the complex balance and

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\(^{25}\) NCCN Evidence Blocks. Available at: https://www.nccn.org/evidenceblocks/.

\(^{26}\) DrugAbacus. Available at: http://www.drugabacus.org/.
preference among quality and quantity of life for patients. The PPVF does not rely on the use of QALYs, and instead draws from a variety of patient-centered outcomes deemed important to patients when considering different healthcare options, as outlined below.

Though the PPVF does not focus on any particular condition, research in the oncology space provides important guidance. Many researchers have examined the outcomes that are important to patients when making decisions among different healthcare options, as cancer patients are often faced with a multitude of choices. For example, in the areas of breast and prostate cancer, studies note how patients often make healthcare decisions based on the invasive nature of the surgery, duration of the therapy, or onerous treatment regimens. Patients have also been shown to care about how a treatment might impact work and recreational activities, and interference with their sex life. Other studies cite factors surrounding treatment-related side effects such as avoiding infection, vomiting, and incontinence. Some studies also note that patients care about the clinical outcomes and efficacy of a particular treatment, as well as the strength of the evidence that they are being presented. For example, studies have shown that breast and prostate cancer patients often choose a particular healthcare option because it provides the “best cure” or “complete cancer removal” in order to avoid reoccurrence.

In the Patient-Centered Outcomes domain, the PPVF aims to answer the question: “What are the clinical, functional, and quality of life benefits and drawbacks of different healthcare options to the patient?” The criteria outlined in this domain reflect some of the important factors that patients consider when making decisions between

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different healthcare options, such as those outlined above: quality of life, complexity of regimen, efficacy/effectiveness, treatment-related side effects/adverse events/complications. While working toward version 1.0 of the PPVF, Avalere and FasterCures look forward to collaborating closely with the patient groups on the PPVF’s Steering Committee—who have offered their help and partnership—to test and confirm the PPVF’s domains and criteria with patients for their comprehensiveness and to ensure that they are truly patient-centered.

This section will first provide an overview of the proposed criteria and associated measures used to assess the Patient-Centered Outcomes domain. We will then outline an ideal set of data sources for each measure and highlight how data considered within this framework may differ from that of other existing frameworks, as well as the potential limitations of the data outlined. Finally, we will describe an assessment methodology for this domain. Figure 3 below summarizes the criteria, measures, and data sources for the Patient-Centered Outcomes domain and will be referenced throughout the sections below.

**Figure 3: Criteria, Measures, and Data Sources that Constitute the Patient-Centered Outcomes Domain**

<table>
<thead>
<tr>
<th>CRITERIA</th>
<th>MEASURES</th>
<th>IDEAL DATA SOURCES</th>
</tr>
</thead>
</table>
| Quality of Life (both generic and disease-specific instruments) | • Health-related quality of life (e.g., instruments that capture Patient Reported Outcomes (PROs) such as vitality, depression, fatigue)  
• Functional/cognitive status (e.g., instruments that capture PROs evaluating mental/physical/social functioning)  
• Palliation of symptoms *(Note: should also capture the duration/magnitude of palliation, e.g., through a continuous scale)*  
• Symptom-free intervals (e.g., pain) | • Reported in randomized control trial (RCT) and observational/registry data |
Critics have argued that existing value frameworks fall short on measuring outcomes that matter to patients, with regard to the clinical benefits/risks of different healthcare options. Existing frameworks largely focus on measuring outcomes such as efficacy and side effects/toxicities; and are inconsistent or incomplete in how they address factors such as palliation of symptoms, quality of life, and factors associated with the complexity of the regimen.  

<table>
<thead>
<tr>
<th>Complexity of Regimen</th>
<th>Efficacy/Effectiveness (long- and short-term)</th>
<th>Side Effects/Complications</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Dosing/treatment schedule</td>
<td>• Significant improvement in:</td>
<td>• Frequency, severity, and duration</td>
</tr>
<tr>
<td>• Treatment length (including need for rehabilitation)</td>
<td>o Primary end point (e.g., HbA1c for diabetes, impact on disease progression)</td>
<td>• Discontinuation (drop out) rates due to side effects/adverse events/complications</td>
</tr>
<tr>
<td>• Typical site of care/pharmacy channel</td>
<td>o secondary end point</td>
<td>• Reported in RCT and observational/registry data</td>
</tr>
<tr>
<td>• Route of administration/procedural process</td>
<td>o tertiary end point</td>
<td>• Drug/device label information</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Reported in RCT and observational/registry data</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Drug/device label information</td>
</tr>
<tr>
<td></td>
<td></td>
<td>• Surveillance data collected through Risk Evaluation and Mitigation Strategies (REMS) data (i.e., post-marketing safety studies for drugs)</td>
</tr>
</tbody>
</table>

Criteria and Measures

As outlined above, research shows that, when assessing the value of different healthcare options, patients care about a variety of outcomes beyond survival, side effects, and

39 NCCN Evidence Blocks. Available at: https://www.nccn.org/evidenceblocks/.
40 DrugAbacus Available at: http://www.drugabacus.org/.
effects on activities of daily living. The PPVF aims to capture a broad scope of patient-centered outcomes that are of central importance to patients when determining value and making a choice among two or more healthcare options. Columns 1 and 2 of Figure 3 summarize the criteria and measures that make up the Patient-Centered Outcomes domain, which are described in further detail below:

**The quality-of-life benefits of a treatment**, such as: how a treatment will impact a patient’s mobility, fatigue, or depression; functional and cognitive abilities to continue work and engage in recreational activities as usual; and whether a treatment will palliate a patient’s symptoms (e.g., pain).41,42

**The complexity of a treatment’s regimen**, such as: the dosing/schedule (e.g., one tablet vs. three tablets a day)43,44,45; the site of a treatment (e.g., in the clinic/hospital vs. at home); and the associated logistics (e.g., transportation, living arrangements, caregiving roles. [please note that these factors are addressed in the Patient & Family Financial Considerations domain below])46,47,48; the treatment’s route of administration/procedural process (e.g., IV, tablet, reconstructive surgery)49,50,51; and the treatment’s length/duration (e.g., months of chemotherapy, length of time spent with IV in arm).52,53

The treatment’s efficacy and effectiveness, such as whether a treatment shows improved clinical outcomes for a specific condition (e.g., overall survival for breast cancer, HbA1c for diabetes), and its impact on disease progression.\textsuperscript{54,55,56,57}

The side effects, adverse events, and complications associated with a treatment, such as: sexual dysfunction, bowel problems, exposure to radiation, joint and muscle pain, etc.\textsuperscript{58,59,60,61} Patients also value the associated frequency, severity, and duration of the side effects/adverse events/complications, and understanding the proportion of patients who discontinue/drop out of the treatment due to the side effects, adverse events, and/or complications.\textsuperscript{62,63}

The PPVF Initiative is seeking public feedback on whether the criteria in this domain (as outlined above) adequately capture the information that is most relevant and important to patients. For example, one specific issue that we invite public input on is how to consider non-financial burdens to family members/caregivers in the PPVF. In the Patient & Family Financial Considerations domain outlined below, we consider a healthcare option’s financial burden on the family/caregiver; however, the important issue of non-financial burdens to the family/caregiver (e.g., the effect of caregiving on a caregiver’s state of mind, need for caregiver clinical training) is not yet well represented in the framework. We believe that this issue could be addressed in the Patient-Centered Outcomes domain, likely in the form of a fifth, independent criterion.

Data Sources

A limitation of existing value frameworks is their inherent over-reliance on RCT data for assessing the clinical benefits and drawbacks of treatments, and general lack of

\begin{thebibliography}{99}
\bibitem{57}Mazur D, et al. How Patients’ Preferences for Risk Information Influence Treatment Choice in a Case of High Risk and High Therapeutic Uncertainty. October 1999. Available at: \url{http://mdm.sagepub.com/content/19/4/394.abstract}.
\bibitem{60}DiBonaventura M, et al. Patient Preferences and Treatment Adherence Among Women Diagnosed with Metastatic Breast Cancer. October 2014. Available at: \url{https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4268769/}.
\bibitem{61}Ishitobi M, et al. Preferences for oral versus intravenous adjuvant chemotherapy among early breast cancer patients. 2013. Available at: \url{https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3839808/}.
\end{thebibliography}
consideration for observational data.\textsuperscript{64,65,66,67} Relying on RCT data to assess the clinical benefits and drawbacks of a therapy for an individual patient serves a role in determining efficacy and identifying certain risks associated with therapies; however, RCTs are carried out in tightly controlled conditions among often homogeneous populations, and are thus often limited in their generalizability to populations that may actually be candidates for the treatment in the real-world setting.\textsuperscript{68} Moreover, most value frameworks only consider data from head-to-head clinical trials, meaning that they do not allow for the comparison of treatments across or beyond individual trials, which limits the body of evidence that might be considered to inform patient- and population-level decision making.

The PPVF differentiates itself from most existing frameworks by allowing for the consideration of broader sources of data and measures such as observational data (e.g., patient-reported outcomes (PROs) collected from clinical registries), in addition to the foundational data derived from clinical trials. The inclusion of observational data in the PPVF not only provides an opportunity for the collection of more patient-centered outcomes that may not be reported in clinical trials, but will also allow the PPVF to act as a mechanism for identifying and, ideally, spurring efforts to address gaps in the evidence base for specific patient-centered outcomes.

The PPVF will also incorporate a mechanism for patients and clinicians to consider data for a given measure that may be reported for one healthcare option, but may not be reported for the other healthcare option(s) under assessment, which may be of value to the patient in their decision making process. This will ensure that certain healthcare options are not overlooked or not considered due to a lack of comparable data across healthcare options.

Moreover, the PPVF will allow for the inclusion of sub-population data that may be most relevant to the patient, beyond just a study’s overall population data. This will serve to provide patients with a greater level of confidence that a healthcare option will have its purported clinical benefit or drawback for them. Where subpopulation data are available for a given outcome, patients will also have the option of weighting their importance with clinical guidance from the physician. The focus on introducing subpopulation data from the evidence base, where available, is a key distinguishing feature of the PPVF.

Finally, in terms of a preferred hierarchy of evidence assessment approaches, the use of sound and accepted methods such as meta-analyses for comparing interventions across a particular measure will be preferred and this study design will dominate others in terms of quality of evidence rankings. Additional considerations in assessing the evidence base

\textsuperscript{64} ICER Value Assessment Framework. Available at: https://icer-review.org/methodology/icers-methods/icer-value-assessment-framework.
\textsuperscript{66} NCCN Evidence blocks. Available at: https://www.nccn.org/evidenceblocks/.
\textsuperscript{67} DrugAbacus. Available at: http://www.drugabacus.org/.
\textsuperscript{68} Varadhan R., Seeger J. Estimation and Reporting of Heterogeneity of Treatment Effects. AHRQ. Available at: https://www.effectivehealthcare.ahrq.gov/ehc/assets/File/Ch_3-User-Guide-to-OCER_130129.pdf.
may include the need to consider both placebo-controlled trials and active controlled studies in order to provide information from studies that most closely aligns with patient-centered outcomes and patient preferences. It is likely that the quality and completeness of the evidence base will vary across therapeutic areas that are considered for application of this framework. In many cases, due to the limitations of each approach to evidence generation, the best “answers” will be derived by triangulating multiple data sources and methods.

Understanding the current status of the evidence base within each therapeutic area or across selected comparators will allow for assessment of the maturity and timeliness of existing evidence, and help us identify remaining evidentiary gaps. Through implementation of this framework, it will be crucial that we catalog areas where evidentiary gaps exist in order to encourage and target future research in both assessment and the data sources available to inform meaningful patient-centric decision making.

Column 3 in **Figure 3** above outlines the ideal data sources that the PPVF will rely on for assessing each criterion. However, should those data not be available, relevant proxies and alternative data sources will be outlined for the specific condition and application at hand.

**Assessment Methodology**

**Figure 4** below describes the proposed process for assessing the Patient-Centered Outcomes domain using the criteria, methods, and data sources mentioned in **Figure 3** above.

It bears mentioning that this domain, as is outlined in **Figure 2**, also integrates and operationalizes certain criteria from the Quality & Applicability of Evidence (QAE) domain, which will be described in more detail later in this document. Specifically, two criteria within the QAE domain—quality of evidence and differences in treatment effect (determined by relevant subpopulation data when available)—are operationalized as part of the assessment and weighting of the PCO domain. However, a third important criterion of QAE—consistency of evidence—is operationalized separately and is addressed in more detail later in this document.

The proposed assessment methodology for the PCO domain (**Figure 4**) includes 5 broad steps. This methodology begins with a collection of all relevant and available data on comparators (Step 1), focusing on identification of both population-level and subpopulation-level analyses and studies from the data sources described earlier in this section.
### Step 1. Determine completeness of data to assess across two or more healthcare options, and organize population/subpopulation data

- **a)** Determine where data are reported for relevant outcomes for each healthcare option and proceed to **b)**. If data are incomplete for particular outcomes across options, proceed to **a) i)**.
  
  - **i.** Assess where data are not comparable for particular outcomes across all healthcare options. These data will be identified and categorized as “non-comparable” and will be made available to the patient to inform his/her decision making with appropriate guidance as to the quality of the evidence and with appropriate caveats regarding non-comparability.

- **b)** Categorize comparable data by overall population and subpopulation analyses for each healthcare option, over each outcome, in each study (integration of QAE domain criteria).

- **c)** Identify any subpopulation data for an outcome(s) that reflect(s) the characteristics of the individual patient (e.g., race, income level, age, gender, comorbidity, disease stage). If subpopulation data that match patient characteristics do not exist, proceed to **Step 2)**.

- **d)** Determine which subpopulation data are most important from a patient preference/goal alignment standpoint, along with a clinical and disease-specific standpoint.

  *Note: In the case of a shared decision making application, if more than one subpopulation that matches the patient’s characteristics exists, clinicians and patients will work together to choose one set of subpopulation data based on preferences and the clinical significance of the subgroups.*

### Step 2. Assess the Quality of Life and Efficacy/Effectiveness criteria

*Note: The below method will be the same for assessing both the quality of life and Efficacy/Effectiveness criteria*

- **a)** For each healthcare option and each study, assess the percent improvement for each outcome over the comparator in each study (referred to as “incremental effectiveness” from here on)

- **b)** Weight each estimate of incremental effectiveness based on the quality (strength of evidence) of the study design (integration of QAE domain criteria)

- **c)** Weight each estimate from **b)** by a patient preference for each study endpoint (e.g., for Diabetes: HbA1c reductions vs. weight loss vs. microvascular/macrovascular complications)
**Step 3. Assess the Complexity of Regimen criteria**

<table>
<thead>
<tr>
<th>a)</th>
<th>Assess each measure within the Complexity of Regimen criterion (i.e., dosing schedule, treatment length, site of care, and route of administration)</th>
</tr>
</thead>
<tbody>
<tr>
<td>b)</td>
<td>Weight each measure by a patient’s preference to arrive at a quantitative estimate for each measure, for each healthcare option</td>
</tr>
<tr>
<td>c)</td>
<td>Sum the adjusted measures and divide by the number of total measures, to arrive at an average criteria estimate to assess the complexity of regimen for each healthcare option</td>
</tr>
</tbody>
</table>
| d) | Calculate healthcare option A’s % change in criteria estimate compared to healthcare option B with the following formula:  
\[
\frac{\text{Healthcare option A estimate} - \text{Healthcare option B estimate}}{\text{Healthcare option A estimate}} = \text{Healthcare option A’s % Change in Criteria estimate}
\]  
*Note: if more than two healthcare options are being assessed, each will be compared to the other comparators.* |

**Step 4. Assess the Side Effects/Adverse Events criteria**

<table>
<thead>
<tr>
<th>a)</th>
<th>For each healthcare option, multiply the frequency of each side effect or adverse event reported in each seminal clinical trial by its grade</th>
</tr>
</thead>
<tbody>
<tr>
<td>b)</td>
<td>Sum a) across the side effects for each healthcare option</td>
</tr>
</tbody>
</table>
| c) | Calculate healthcare option A’s % change in b) compared to healthcare option B according to the following formula:  
\[
\frac{\text{Healthcare option A estimate} - \text{Healthcare option B estimate}}{\text{Healthcare option A estimate}} = \text{Healthcare option A’s % Change in b) estimate}
\]  
*Note: if more than two healthcare options are being assessed, each will be compared to the other comparators.* |

**Step 5. Calculate the Patient-Centered Outcomes (PCO) score (for applicable applications)**

| a) | Sum the preference-weighted scores of each criterion for each healthcare option to arrive at the PCO score for each |
Patient & Family Financial Considerations

Financial considerations are a critical component of any value determination regarding healthcare options. Whereas traditional value assessment methodologies primarily focus on the cost to the healthcare system, the PPVF is primarily focused on costs to the patient and family. The PPVF’s Patient & Family Financial Considerations domain also differs from definitions of costs used in traditional value determination in that it considers not only medical out-of-pocket (OOP) costs but also non-medical costs to the patient and family, as well as downstream future costs of different healthcare options. This domain aims to answer the question: What are the overall costs of different healthcare options to the patient and family?

This section includes an overview of the criteria we propose to measure Patient & Family Financial Considerations and a proposed methodology for measuring these considerations. We also discuss our proposal, at this juncture, to focus on costs to the patient and family instead of system-wide costs, and how that approach could be altered as the PPVF evolves and applications of the PPVF are developed. Finally, we discuss the different types of cost data that are available and how our approach will change based on the available data in any given situation.

Patient & Family Financial Considerations: Criteria and Measures

Figure 5 describes the criteria and measures that make up the Patient & Family Financial Considerations domain.

Figure 5: Criteria and Measures that Constitute the Patient & Family Financial Considerations Domain

<table>
<thead>
<tr>
<th>CRITERIA</th>
<th>MEASURES</th>
</tr>
</thead>
<tbody>
<tr>
<td>Medical Out-of-Pocket (OOP) Costs</td>
<td>• Estimated OOP cost of treatments and related medical care based on patient’s plan design and site of care, including any patient assistance</td>
</tr>
<tr>
<td></td>
<td>• Estimated OOP cost of supportive care agents (e.g., blood transfusions, home health care)</td>
</tr>
<tr>
<td></td>
<td>• For devices: OOP cost of device maintenance, monitoring, and replacement</td>
</tr>
</tbody>
</table>
Non-medical Costs

- Lost wages (not applicable to retired or non-working patients)
- Lost wages for family/caregivers
- Time back to normal productivity/time back to work
- Cost of travel (including consideration of treatment schedule)
- Cost of child/elder care
- Administrative burden related to utilization management mechanisms (e.g., step therapy benefit design, applications for cost-sharing assistance)

Future Costs

- Subsequent OOP costs related to downstream healthcare utilization, including products or services that are no longer needed as a result of the intervention
- Changes in costs of therapies (in particular for chronic conditions)

System-wide Costs

As mentioned, the proposed PPVF focuses primarily on costs to the patient and family and does not currently include a measure of system-wide costs. However, we envision that future versions of the PPVF will include more detail in this area. By first focusing on costs to the patient and family, the PPVF will assess the evidence base necessary to define costs more comprehensively than is done under traditional assessments of value. For instance, the type of information considered as a part of this domain, including non-medical costs and future costs will be critical as future versions of the PPVF also consider system-wide costs.

Because the first application of the PPVF will be a shared decision making application, this proposed methodology also does not include a measure of direct medical costs to the payer. However, as the PPVF is used to develop applications beyond shared decision making, including applications to other value frameworks, to support public healthcare programs, and in the form of public analyses of particular conditions, we will need to measure direct medical, non-medical, and future costs more broadly. We understand that there are situations in which cost to the system is not only important to value assessments, but where patients may have a preference for a treatment that has a lower cost to the system. In the future, we plan for the PPVF to be able to address these system-wide costs—or more generally, costs to entities other than the patient. However, we will continue to incorporate the full range of patient costs that we detail in this domain, as we believe that is central to a patient-perspective value framework. As a part of this
The draft framework, we seek input on the best way to incorporate information on system-wide costs of different healthcare options.

**Assessing the Patient & Family Financial Considerations Domain**

The methodology by which we will assess each of these measures and criteria will be adapted based on circumstances related to the interventions and applications. Three of these circumstances are outlined in **Figure 2: The PPVF’s Overall Process for Healthcare Option Comparison** as “Methods Considerations.”

They are:

1) Population- vs. individual-level application
2) Chronic or acute condition
3) Data availability and completeness

Each of these methods considerations indicates situations that would impact the approach to measuring the Patient & Family Financial Considerations domain. For instance, for applications of the framework at an individual level (e.g., a shared decision making tool to be used by an individual patient with his or her clinician), we propose to incorporate patient-specific plan design information to measure the first criterion, *medical out-of-pocket (OOP) costs*. For population-level applications of the framework (e.g., an application to an existing framework that produces population-level analyses of different healthcare options), we would not have access to any patient-specific plan information. For this type of application, we would reference data from studies published in the literature that report cost estimates of direct medical OOP costs, and in some instances health plan cost data for a particular population could be incorporated.

The methodology for assessing this domain would also be adapted based on whether the particular application relates to an acute or chronic condition. For an acute condition, we would measure each criterion for an episode of care reflective of that acute condition, whereas for a chronic condition, we would measure costs over a defined time horizon that is appropriate for the particular condition (e.g., monthly, yearly).

Finally, as with other domains, we have both an ideal case—where we have access to all the data we need to measure a particular criterion—and alternative situations in which our data sources are incomplete, incompatible, and costs (particularly OOP costs) may be difficult to access. Below we go into detail about the different ideal data sources and how we would assess this domain in the event that those data sources are incomplete or unavailable.

**Data Sources**

As described above, we have identified ideal data sources—which may change in certain circumstances. Those ideal data sources are described in **Figure 6** below.
### Figure 6: Ideal Data Sources for Assessing the Patient & Family Financial Considerations Domain

<table>
<thead>
<tr>
<th>CRITERIA</th>
<th>MEASURES</th>
<th>IDEAL DATA SOURCES</th>
</tr>
</thead>
</table>
| **Medical OOP Costs** | • OOP costs of treatments and related medical costs to the patient | • Patient-specific plan design information, including cost-sharing requirements (for individual-level applications)  
• Estimates from literature on expected healthcare resource use; published list prices for products, services, or interventions payment benchmarks such as the Medicare Fee Schedule; and national averages for particular sets of services based on payment databases (e.g., FAIR Health) (for population-level applications)  
• Estimates of patient assistance program support and availability |
|               | • Supportive care agents                                                  | • Patient-specific plan design information, including cost-sharing requirements (for individual-level applications)  
• Estimates from literature (for population-level applications) |
|               | • Device maintenance                                                      | • Estimates from literature  
• Incorporates patient-specific plan design information (for individual-level applications) |
| **Non-medical Costs** | • Lost wages                                                             | • Estimates regarding expected time horizons for treatment and supportive care from physicians (individual-level applications) or literature (population-level applications)  
• Estimates from literature  
• Patient/family members’ income (individual-level applications)  
• Representative income brackets (e.g., Bureau of Labor Statistics data) |
<p>|               | • Family/caregiver lost income                                           |                                                                                  |
|               | • Time back to normal productivity/time back to work                     |                                                                                  |</p>
<table>
<thead>
<tr>
<th><strong>Future Costs</strong></th>
<th><strong>Limitations</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cost of travel</strong></td>
<td>There are a number of limitations based on the availability and completeness of data sources in given applications. For instance, for individual-level applications, the ideal data</td>
</tr>
<tr>
<td><strong>Cost of child/elder care</strong></td>
<td>sources in given applications. For instance, for individual-level applications, the ideal data</td>
</tr>
<tr>
<td><strong>Administrative burden related to utilization management mechanisms</strong></td>
<td>estimates from the literature, including observational data or modeling of offsets that accumulate over time based on effectiveness of treatment</td>
</tr>
<tr>
<td><strong>Subsequent OOP costs related to downstream healthcare utilization, including products or services that are no longer needed as a result of the intervention</strong></td>
<td>estimates from the literature, including burden of prior authorizations that might be expected or prescribing requirements that would require additional costs to access medication</td>
</tr>
<tr>
<td><strong>Changes in cost of therapies (in particular for chronic conditions)</strong></td>
<td>Use plan-specific information where available (individual-level applications)</td>
</tr>
</tbody>
</table>

**Limitations**

There are a number of limitations based on the availability and completeness of data sources in given applications. For instance, for individual-level applications, the ideal data...
source is plan-specific benefit design information that will allow the framework’s application to accurately predict the cost of different healthcare options; however, in many cases this information may not be knowable to the patient and provider at the point of care. Another common limitation that we anticipate is the availability of patient-specific information to inform the calculation of travel costs for different healthcare options. In the absence of patient-specific inputs, we would use averages similar to the approach for population-level applications, as outlined below.

In situations where access to ideal data sources is limited, the methodology will be adapted to develop reasonable estimates to inform the framework. For instance, where ideal data sources for measuring medical OOP costs are not available, the PPVF will consider incremental differences in component healthcare utilization rates (e.g., hospitalization, ER visits, physician visits) and make reasonable OOP estimations by applying OOP unit costs to incremental difference in utilization.

**Assessing Each Criterion**

In this section, we propose a methodology for assessing the measures within each criterion.

- **Medical out-of-pocket costs**
  
  - **Individual-level applications.** For each healthcare option, estimates for direct medical OOP costs for the patient over the entire episode of care will be developed based on the patient’s actual or estimated plan benefit design/cost-sharing. In most cases, we will assess all three measures—OOP costs for the actual treatment, OOP costs for supportive care, and any OOP costs associated with device maintenance—together as described in the below proposed methodology.

  - **Population-level applications.** For each healthcare option, literature will be assessed on expected healthcare resource use to gain an understanding of the products and healthcare services that must be “monetized.” Reported costs or alternative list prices for healthcare options will be utilized to assess incremental differences in costs of products (e.g., drugs, devices), and payment benchmarks such as the Medicare Fee Schedule and national averages of OOP costs based on payment databases (e.g., FAIR Health) to assess incremental differences in the costs of services (e.g., specialist visits, inpatient admissions). Finally, reasonable estimates of OOP cost attribution to the incremental differences will be applied across the various services to arrive at a population-level OOP estimate across comparators.
• **Non-medical OOP costs**
  
  o *Individual-level applications:* For each healthcare option, total lost wages will be calculated using expected time horizons needed for treatment and supportive care (based on estimates provided by the physician), along with a patient or family member’s income (e.g., salary, hourly wages). The cost of travel will be derived from patient self-reporting or calculated using regional estimates of costs of air, train, bus, or automobile travel to treatment or supportive care as appropriate. The cost of child or elder care will be derived from patient self-reporting or calculated based on the regional market rates. Where access to patient-specific information to inform travel and child or elder care costs is not available, an alternative approach similar to that outlined for population-level applications can be used.

  o *Population-level applications:* For each healthcare option, total lost wages will be calculated using expected time resources needed for treatment and supportive care (estimate provided by literature), along with pre-determined income brackets that are representative of the U.S. population. The cost of travel will be calculated using national averages for costs of air, train, bus, and automobile travel based on the typical site of care for different healthcare options and average for patient travel distance to those types of sites based on National Household Travel Surveys for medical care. Total cost of elder and child care will be estimated based on national averages of the amount of care needed, the cost of this care, and assumptions from the literature regarding the impact of different healthcare options on a patient’s ability to fulfill child and elder care duties.

• **Future costs.** Based on the peer-reviewed literature, estimates will be calculated based on the changes to healthcare utilization and downstream offsets and effects as a result of different healthcare options over an identified time horizon.

  o *Individual-level applications.* Estimated future healthcare utilization will be determined and apportioned based on the patient’s plan benefit design to calculate expected reductions or increases in utilization and OOP costs over an identified time horizon to determine estimated future costs.

  o *Population-level applications.* We will apply estimated future utilization to national payment benchmarks such as the Medicare Fee Schedule and average cost-sharing information using national estimates for OOP costs related to component of care to determine estimated future costs.
Assessing Patient & Family Financial Considerations

In this section, we propose a methodology for how the different criteria that make up the Patient & Family Financial Considerations domain can be brought together in a single assessment. While we envision some applications and situations in which this assessment will be a single score or monetary value, there will be many situations in which such a single output is not possible or desirable. Situations like this include:

- When available data sources do not allow for full assessment of each measurement
- When an application requires additional incorporation of patient preferences, including the option for patients to indicate a preference about the relative importance of any individual measures within the criteria (e.g., time out of work) compared to not only criteria within this domain but also to criteria in other domains
- For applications that do not require a single score output

The below proposed methodology describes an ideal case in which we would want to create a single score output.

Figure 7: Proposed Patient & Family Financial Considerations Domain Methodology

<table>
<thead>
<tr>
<th>Step 1. Assessment of the Medical OOP Costs of Treatment/Episode of Care</th>
</tr>
</thead>
<tbody>
<tr>
<td>a) Individual: Calculate the OOP cost of all necessary products (e.g., drugs, devices) and/or services (e.g., outpatient/inpatient visits, supportive care agents) according to the patient’s plan benefit design and site of care, including any patient assistance</td>
</tr>
<tr>
<td>b) Population: Calculate the OOP cost of all necessary products (e.g., drugs, devices) and/or services (e.g., outpatient/inpatient visits, supportive care agents) by utilizing published list prices for products and the Medicare Fee Schedule for services</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Step 2. Assessment of the Non-medical OOP Costs of Treatment/Episode of Care</th>
</tr>
</thead>
<tbody>
<tr>
<td>c) Lost Income of Patient/Family or Caregiver (Individual): Based on the expected time needed for treatment and supportive care, along with the patient’s income, wage structure (e.g., salary vs. hourly), and sick/vacation policies, calculate the amount of wages lost by the patient due to treatment and supportive care</td>
</tr>
</tbody>
</table>
d) **Lost Income of Patient/Family or Caregiver (Population):** Utilize estimates from the peer-reviewed literature on lost productivity combined with average U.S. income to calculate lost wages over a given time horizon.

e) **Cost of Travel (including consideration of treatment schedule) (Individual):** Based on likely site of care, number of visits, patient’s home, anticipated mode of transportation, and regional estimates of the cost of transportation, calculate total estimated cost for identified time horizon.

f) **Cost of Travel (including consideration of treatment schedule) (Population):** Based on national averages of transportation costs, typical site of care for different treatment options, and national averages of travel distances to the sites of care, calculate total estimated cost for identified time horizon.

g) **Time Back to Work:** Based on literature and physician estimates, report on time needed for treatment and estimates related to lost productivity (This will be difficult to quantify numerically, however, it is included in this domain as an important criterion from the patient perspective.)

h) **Cost of Child/Elder Care (Individual):** Based on expected impact of treatment options on ability to fulfill child and elder care responsibilities, and estimates of child and elder care costs, report on additional costs per non-school age child, school-age child, as well as elder in need of care, and sum those costs for a total.

i) **Cost of Child/Elder Care (Population):** Based on assumptions from the literature on treatment impact on need for child/elder care, and national averages of the cost of child/elder care, estimate total additional cost for child/elder care.

---

**Step 3. Assessment of OOP Cost Offsets or Future Medical Costs Associated with Treatment**

j) **Assessment of OOP Cost Offsets (Individual):** Based on estimates from the literature on the impact of an intervention on future healthcare utilization, and plan-specific cost-sharing requirements, calculate the total cost of utilization impacts.

k) **Assessment of OOP Cost Offsets (Population):** Based on estimates from the literature on the impact of an intervention on future healthcare utilization, and published list prices (products)/Medicare Fee Schedule (services), calculate the total cost of utilization impacts.

---

**Step 4. Allow for Weighting of OOP Cost Criteria**
Individual: The patient has the opportunity to directly weight three criteria

Population: The preference weights will be garnered from the literature if available, and will be set as equal in the absence of peer-reviewed evidence

Step 5. Calculate the Total OOP Costs for Each Framework Comparator (for applicable applications)

Sum the weighted total direct medical OOP costs for the episode of care, the non-medical costs, and future medical OOP cost offsets to arrive at a total OOP cost for each framework comparator (Note: As mentioned above, there will be applications and situations in which a single output is not possible or desirable. This final piece of the methodology does not pertain to those situations.)

Quality & Applicability of Evidence

The Quality & Applicability of Evidence domain measures three key criteria: the quality of the evidence, a measure of the strength of each study’s design; the consistency of evidence across studies; and the differences in treatment effect for different types of patients. The quality and consistency of evidence criterion is used in traditional evidence assessment processes to gauge the degree to which the body of evidence represents credible and robust information regarding outcomes of interventions. It is an important part of the process in determining how and what information should be considered when informing value assessment frameworks. The differences in treatment effect criterion identifies available subpopulation data to provide information on the differences in treatment effect for different types of patients. The overall aim of this domain is to answer the question: “What level of confidence does a patient have that a given healthcare option will have specific effects for them?”

What level of confidence does a patient have that a given healthcare option will have specific effects for them?

The Quality & Applicability of Evidence domain further differentiates the PPVF from traditional definitions of value by how it interacts with the other domains. In Figure 1, the Quality & Applicability of Evidence domain is positioned inside the Patient Preferences domain and alongside the Patient-Centered Outcomes and Patient & Family Financial Considerations domains. Different patients have different preferences with regard to evidence as described in the Patient Preferences section above. Furthermore, the evidence also impacts a patient’s perspective on the outcomes of different healthcare options and the cost of those healthcare options.
In this section, we outline the criteria and methods considerations that make up the Quality & Applicability of Evidence domain and our proposed methodology for how this domain will function as a part of the PPVF.

Assessing the Quality & Applicability of Evidence

Criteria, Measures, and Methods

Figure 8 describes the criteria, the associated methodological considerations, and measures that make up the Quality & Applicability of Evidence domain.

Figure 8: Criteria, Methods Considerations, and Measures that Constitute the Quality & Applicability of Evidence Domain

<table>
<thead>
<tr>
<th>CRITERIA</th>
<th>MEASURES</th>
<th>METHOD</th>
</tr>
</thead>
<tbody>
<tr>
<td>Quality of Evidence</td>
<td>● Adherence to generally accepted methods</td>
<td>● Consideration of established evidentiary grading scales (trial-based and observational data-based)</td>
</tr>
<tr>
<td>Consistency of Evidence</td>
<td>● Variability of study results—measures the degree to which different studies illustrate the same results</td>
<td>● Consideration of consistency in magnitude of effect that is reported across evidence base</td>
</tr>
<tr>
<td>Differences in Treatment Effect</td>
<td>● Considers heterogeneity across different subpopulations:</td>
<td>● Reported variance in outcome measure based on specific subpopulations</td>
</tr>
<tr>
<td></td>
<td>○ Demographics</td>
<td></td>
</tr>
<tr>
<td></td>
<td>○ Comorbidities</td>
<td></td>
</tr>
<tr>
<td></td>
<td>○ Disease stage</td>
<td></td>
</tr>
</tbody>
</table>

How the Quality & Applicability of Evidence Domain Interacts with Other Domains

As displayed in Figure 1, Quality & Applicability of Evidence provides a lens through which the patient views both Patient-Centered Outcomes (PCO) and Patient & Family Financial Considerations (PFFC), and is impacted by Patient Preferences. The evidence collected through the assessment process described earlier underpins the data used to compare interventions based on the measures found in the PCO and PFFC domains. This evidence must be evaluated for its level of quality (based on the strength of evidence according to the various types of study designs and data sources that are used) and consistency (the degree to which there is certainty of the results across studies).

Within the PCO domain, two elements of the QAE domain are integrated into the framework and operationalized by applying a quality of evidence assessment to the individual studies that report the measures being evaluated. Quality assessments will be based on evidence scoring tools that are commonly used to gauge the strength of the evidence based on the particular design that is employed (e.g., using hierarchical ranking of study designs), and will provide a weighting for each outcome based on the methodological rigor employed to produce results for the outcome.
As described earlier, the notion of differences in treatment effect is an important concept that is operationalized within this framework. This is achieved by identifying study results that report out at the subpopulation level and provide the opportunity to assess the alignment between the patient with the particular subpopulation and the resulting outcomes. At an individual level, patients would have the opportunity to consider the comparisons across interventions for those subpopulations that are most meaningful to them, thereby creating an inherent weighting toward the resulting comparisons for those particular outcomes.

This same approach to integrating these elements of quality of evidence and differences in treatment effect would also be applied to the Patient & Family Financial Considerations domain, to the degree that individual studies are available and can be assessed for the measures described in the domain, and also to the degree that subpopulation-level data is reported for those measures.

We propose to use the consistency of evidence criterion to describe how often similar findings are reported across studies. This criterion will be operationalized using an assessment of the results and by determining the degree of variance across these studies. Given that this criterion is also a way to operationalize the certainty with which a patient would expect to see results for the outcomes of interest across the comparison of interventions, this provides an opportunity for weighting the consistency of evidence criterion against the other domains to align with patient preferences.

As discussed in the Patient Preferences section, the PPVF creates an opportunity for patients to express differing levels of preferences relating to the Quality & Applicability of the Evidence they will accept. This will be discussed in more detail in the Assessing Healthcare Options section.

The reality in many circumstances may be that patients may not want to consider that level of detail. That does not mean that such information will be inconsequential to patients, particularly within certain subpopulations. Therefore, it is important to have options for addressing this level of distinction but also helpful that applications include default options that allow patients to bypass those decisions when data are limited, of limited relevance, or more detailed than is comfortable for those patients.

**Application-Specific Methodology Considerations**

The methodology by which each of these measures and criteria will be assessed would likely change based on certain circumstances, two of which are outlined in Figure 2: The PPVF’s Overall Process for Healthcare Option Comparison as “Methodological Considerations.”

They are:

1) Population- vs. individual-level application
2) Data availability and completeness
Population- vs. individual-level application significantly impacts the third criterion, *differences in treatment effect*, but not the first criterion, *quality of evidence*. In a population-level application, the PPVF will not be able to consider the individual characteristics of any given patient. Therefore, in order to convey information about the differences in treatment effect for different subpopulations, the PPVF will identify subpopulation data that is comparable for the different healthcare options under consideration. How this information is ultimately conveyed to the audience will depend on the specifics of the population-level application, or on the intended use of the healthcare option assessment.

In an individual-level application, the PPVF would collect information about the patient’s characteristics, including race, income level, age, gender, comorbidity, disease stage, and others. Based on that information, the PPVF would allow for identification of appropriate subpopulation data that is identified through the evidence assessment process. In circumstances where more than one subpopulation is appropriate for the patient, the PPVF would create a mechanism for the patient to indicate a preference as to which subpopulation data is more relevant to them. In a shared decision making application, this preference exercise would be part of a patient’s conversation with a clinician that incorporates the patient’s goals and preferences as well as the physician’s clinical evaluation about which characteristics, in this case, generate the most relevant data for the patient.

Data availability and completeness affects each domain. The ideal case is one in which we have access to a range of comparable data sources and detailed RCT data that indicates differences in treatment effect across subpopulations and is confirmed by real-world evidence. As one can expect, we will often not have access to this level of data, and therefore will need to shift the methodology for assessing the Quality & Applicability of Evidence based on what data sources are available. We seek public input on data sources that can be incorporated into the PPVF and ways in which we can account for situations where ideal data sources are not available.

**Assessing Healthcare Options**

After the Patient-Centered Outcomes, Patient & Family Financial Considerations, and Quality & Applicability of Evidence domains have been individually assessed, users of the framework will have to: 1) use patient preferences to weight between these domains; and 2) calculate the comparative value of the two or more healthcare options being considered.

**Weighting among Domains**

Before completing the final assessment of the healthcare options being considered, users of the PPVF must weight among the Patient-Centered Outcomes, Patient & Family Financial Considerations, and Quality & Applicability of Evidence domains for each
comparator (drugs/devices/diagnostics/interventions) being considered. In accordance with the approach described in Patient Preferences section, weighting method will differ based on whether the PPVF is applied at the individual vs. the population level and the available patient preferences data based on the application.

- **In an individual-level application**, in the context of shared decision making, patients will be asked to rank the above three domains in order of importance and magnitude, based on their personal preferences. Patients will have the opportunity to apply points or scoring that follows the assumption that more points will equate to higher preference and importance.

- **In a population-level application**, population-wide data collected on patient preferences or desirability of trade-offs for one domain over others will be utilized. In the absence of these data, all three domains will be weighted equally.

**Calculating the Comparative Value of Different Healthcare Options**

Once the domains have been weighted according to patient preferences, the preference-weighted domain-specific assessment scores would be summed to arrive at the “overall value assessment score” for each of the healthcare options (i.e., drugs, devices, diagnostics, interventions) being considered. These “overall value assessment scores” can then compared across the healthcare options being considered. A higher score will indicate more patient-centered value.

**Presenting the Final Healthcare Options to the End User**

As described in the section below, the format and means for presenting the final healthcare options to the end user is just as important as the information itself. For version 1.0 of the PPVF, further thought and research will need to be conducted on how the above information should be structured so that it can be best fed into an appropriate format for patients and clinicians for the purposes of shared decision making. Avalere and FasterCures look forward to working with patient groups on the Steering Committee and elsewhere to conduct this research in advance of publishing version 1.0 of the PPVF, and provide suggestions for what the PPVF could look like when operationalized for a shared decision making application.

**Usability & Transparency**

The final domain of the PPVF is Usability & Transparency. While all the domains contribute different types of information and function differently within the PPVF, Usability & Transparency stands apart as the foundation of the framework. Despite its different function, we have included it as one of the five domains.
of patient value to ensure that its importance will not be diminished at any point in the framework.

Is the framework and its applications usable and transparent in contract, content, and format?

This domain represents the PPVF’s commitment to ensuring that the framework has a transparent approach and that the information displayed through each application is appropriate for, accessible by, and useful and meaningful to its intended audience. It represents our commitment to publishing a detailed methodology and seeking public input, as well as our commitment to continuous self-evaluation as we test both the framework and individual applications through patient surveys and focus groups.

Usability & Transparency: Criteria and Methods Considerations

Figure 9 displays the criteria and methods considerations that make up this domain. This domain is an evaluative domain, and measures associated with each method consideration will be determined based on the audience and the application as described later in this section.

Figure 9: Criteria and Methods Considerations that Constitute the Usability & Transparency Domain

<table>
<thead>
<tr>
<th>CRITERIA</th>
<th>METHODS CONSIDERATIONS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Transparent Approach</td>
<td>• Clear methods and parameters</td>
</tr>
<tr>
<td></td>
<td>• Clear interpretation of results</td>
</tr>
<tr>
<td></td>
<td>• Clear justification of evidence</td>
</tr>
<tr>
<td></td>
<td>• Inclusive of all options</td>
</tr>
<tr>
<td></td>
<td>• Transparency of individuals involved</td>
</tr>
<tr>
<td>Meaningful Information</td>
<td>• Stays true to patient perspective</td>
</tr>
<tr>
<td>Accessible Format</td>
<td>• Education levels and health literacy</td>
</tr>
<tr>
<td></td>
<td>• Various ages</td>
</tr>
<tr>
<td></td>
<td>• Accessible to persons with disabilities</td>
</tr>
<tr>
<td>Usefulness</td>
<td>• Rate of utilization</td>
</tr>
<tr>
<td></td>
<td>• Satisfaction</td>
</tr>
<tr>
<td></td>
<td>• Patient engagement</td>
</tr>
</tbody>
</table>
Assessing the Usability & Transparency Domain

The Usability & Transparency domain serves a number of purposes. First, it determines how the information is communicated in a way that is appropriate to its audience and fits with the specific application under development. Second, it evaluates the framework and the application for usability and transparency. As shown in Figure 2: The PPVF’s Overall Process for Healthcare Option Comparison, there are two primary methodological considerations that impact how this domain will be addressed.

1) Audience. The applications of the PPVF will have a wide range of audiences, which will impact how the criteria within the Usability & Transparency domain are assessed.

2) Application. As will be discussed in detail in the following section, the PPVF has a range of potential applications. The particular application at hand will impact the framework throughout the methodologies associated with the other domains outlined above, but will play a particular role here as this domain determines how the information regarding different healthcare options is conveyed.

For example, a shared decision making tool will have an audience of patients and providers. That means that not only must the framework’s approach be transparent and true to the patient perspective, but it also must be accessible and useful to both the patient and the provider. In this case, considering the audience and the application, we would determine via surveys and patient focus groups what information is most useful to the type of patient and provider being served by the application, and then design a tool that displays that information. It may be that for this application the most useful way is to perform a healthcare option assessment based on the data available—including patient preferences as obtained from the individual patient, clinical data about the outcomes of different healthcare options, cost data based on the individual's insurance benefit, and other factors such as location of treatment center and availability of family caregivers, as well as an assessment of evidence to determine the likelihood of a given treatment effect occurring for that individual patient—and display the recommended healthcare option followed by information about other healthcare options, in the order of a patient's preference.

Who are the audiences of the PPVF’s applications?
- Patients/Caregivers
- Providers
- Payers
- Life science companies
- Policymakers
- People with varying levels of literacy/health literacy
- General public
- Researchers
- Other framework developers
SECTION V. ROADMAP TO APPLICATIONS

Future Potential Applications of the PPVF

As described throughout this methodological report, we intend to design the PPVF to be flexible so that it can be operationalized across a variety of individual- and population-level applications. Figure 10 outlines the proposed categories applications of the PPVF, which are described in further detail below:

1. The PPVF could be adapted and incorporated into clinical decision support (CDS) systems and used as a platform for shared decision making (SDM) between patients and clinicians. For this application to be developed and piloted, it would first be necessary to partner with an organization developing a shared decision making tool and/or resource, and work with that organization to define how the PPVF could help shape and inform it.

2. Detailed elements of the framework could be integrated into existing value frameworks (e.g., ASCO, ICER, NCCN, DrugAbacus) to help render them more patient-centered in their approach to value assessments. We look forward to continuing to work with framework developers to discuss how the PPVF can inform their iterative processes.

3. The PPVF could be applied to public healthcare programs to help inform work around shared decision making and beneficiary engagement. For instance, CMS has been modernizing its approach to paying providers by rewarding them increasingly on their performance. As a part of these efforts, CMS has publicly committed to holding providers accountable for more patient-centered outcomes measures. As CMS develops new measures for the merit-based incentive payment system (MIPS) and various alternative payment models, a tool to assess CDS and SDM efforts could be critically important for measuring provider performance in MIPS performance categories, including improvement activities and advancing care information.

4. The PPVF could be used for de novo assessments of therapies and other healthcare services. That is, the PPVF could be operationalized to generate a report that assesses the value of different healthcare options to patients for a specific condition.
Avalere and FasterCures, through guidance from the PPVF Steering Committee, propose to move forward with initially constructing the PPVF for its use in an SDM application. As displayed in Figure 10, this application is a good starting point from which to inform other future applications. For example, focusing on an SDM application will inform how the PPVF could be applicable to CMS programs. Moreover, starting at the individual level will allow us to elicit feedback from patients and patient groups on individual preferences, which can inform the patient preferences research that will be critical to a population-level application, such as introducing components of the PPVF into existing value frameworks.

**Next Steps: Getting from Draft PPVF to Version 1.0**

Much ground must be covered to deliver a framework in June 2017 that is truly condition agnostic and can be used across a variety of individual- and population-level applications, and we will continue to work with our Steering Committee on the PPVF’s overall construct. However, as touched on in the sections above, there are a number of specific next steps that will be taken to ensure that version 1.0 of the PPVF is truly patient-centered.
Actively Soliciting Feedback on the Draft PPVF’s Three Components

Continuous work with the patient groups on the Steering Committee, and beyond the confines of this Initiative is critical to ensure the PPVF’s domains and criteria comprehensively address the questions that are most important to patients when making care decisions. Several patient groups on the Steering Committee have offered to help research and test the factors that are most important to patients when making decisions between different healthcare options and care choices. This work will be undertaken to appropriately pressure test the draft PPVF and fill in any existing gaps. Our feedback process is described in detail in Section VI.

Testing the PPVF’s Language with Patients

The patient audience must be engaged to ensure the three components and concepts of the PPVF truly resonate with a patient audience, empowering them to understand the proposed framework and provide meaningful feedback on its further development. Avalere and FasterCures will work with patient groups on the Steering Committee, as well as other patient groups, to test the language used in the PPVF to ensure that it is transparent and meaningful to patients.

Outlining Future Research on Patient Preferences

As outlined in the “Patient Preferences” subsection in Section IV, as a next step in the framework development process, Avalere and FasterCures will work with patient groups to conduct original research on patient preferences that can be used to inform both individual- and population-level patient preference-eliciting tools for future incorporation into the PPVF.

We will also work with patient groups to continuously scan the landscape for meaningful data sources that provide information on patient preferences, including patient experience registries that can be leveraged for research and pulled into the PPVF. Finally, we will assess the full landscape of patient preferences research and outline important gaps in the literature that need to be filled.

Piloting the PPVF

As outlined above, deciphering the best formats through which the PPVF could be presented to patients and clinicians is a central component of this effort. Avalere and FasterCures will work with partners both on and off the PPVF Initiative Steering Committee to build applications of the PPVF and evaluate those applications to ensure they are truly patient-centric and adequately capture the patient perspective. Working closely with clinicians in the process of building and testing specific applications will also be key in ensuring that the PPVF can be successfully operationalized in routine clinical care. Ultimately, this work will be critical in ensuring that, when operationalized, the PPVF will be truly usable and meaningful to patients, clinicians, payers, researchers, life sciences companies, and other relevant users.
If you are interested in discussing how to partner with us in supporting this next phase of the work, please do not hesitate to contact us at DMasi@avalere.com.
SECTION VI. FEEDBACK

We need your help to develop the Patient-Perspective Value Framework. Avalere and FasterCures are soliciting input on the proposed concepts and planned applications of the PPVF through an online feedback questionnaire, which can be found at the following link: http://avale.re/2eISRTn. For your reference, we have copied the questions below – please visit the above link to input your answers.

If you have any additional feedback or are interested in partnering with us on the development of an application, please contact us as DMasi@avalere.com. Thank you for your feedback!

1. Please provide your name
2. Please provide your organization and title
3. Please indicate which of the below options best represents your perspective:
   a. Patient
   b. Patient Advocate
   c. Clinician
   d. Life Sciences Company
   e. Health Plan
   f. Government
   g. Healthcare Researcher
   h. Other
4. When you think about a healthcare decision that you have to make, what are the top three most important considerations?
   a. How will it impact my quality of life?
   b. Does some evidence show that it works?
   c. What are the side effects?
   d. How much will it cost?
   e. How much work will I miss?
   f. Will the treatment cause me to be a burden on my family?
   g. Will the treatment save me money in the long run by avoiding future medical costs?
   h. Other
5. How well do the five domains represent the broad key elements of decision making for patients? (Please refer to the back of the PPVF infographic for more information)
   a. Very Well
   b. Well
   c. Adequately
   d. Poorly
   e. Very Poorly
6. Is anything missing from the five domains?
7. Do the criteria within each of the five domains adequately capture the specific information most relevant and important to patients? Is anything missing? (Please refer to the Domains and Criteria section in the center-fold of the PPVF infographic).

8. Do the measures and methods considerations for each domain capture elements that you view to be critically important? (Please refer to the Measures, Data Sources and Methods section in the center-fold of the PPVF infographic).

9. In making decisions about what data sources to use, what considerations should the PPVF prioritize? Why? (Please refer to the Measures, Data Sources, and Methods section in the center-fold of the PPVF infographic).

10. Specifically, how can you envision the PPVF being operationalized in real-world situations? (Please refer to the Applications section in the center-fold of the PPVF infographic).

11. Please share any additional feedback about the proposed PPVF. (Please refer to either the PPVF infographic or the draft methodology report).
About Avalere

Avalere is a vibrant community of innovative thinkers dedicated to solving the challenges of the healthcare system. We deliver a comprehensive perspective, compelling substance, and creative solutions to help you make better business decisions. As an Inovalon company, we prize insights and strategies driven by robust data to achieve meaningful results. For more information, please contact info@avalere.com. You can also visit us at avalere.com.

About FasterCures

FasterCures, a DC-based center of the Milken Institute, is driven by a singular goal—to save lives by speeding up and improving the medical research system. We focus on cutting through the roadblocks that slow medical progress by spurring cross-sector collaboration, cultivating a culture of innovation, and engaging patients as partners. FasterCures brings together all stakeholders across the medical enterprise to ensure inclusion of multiple perspectives in vital cross-disciplinary problem-solving, with the ultimate goal of turning scientific advances into meaningful medical solutions for patients. Through our programs, we identify what’s working and what isn’t across the research ecosystem and share that knowledge so that every sector—and every patient—can benefit.

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