



# 2021 METHODS SUMMIT

*Designing Equitable Measures and Methods for Patient Priorities in Healthcare Value Assessment*

Pre-Read Brief

[www.thevalueinitiative.org](http://www.thevalueinitiative.org)

# Welcome to the 2021 Methods Summit

The Innovation and Value Initiative (IVI) is a nonprofit, research organization committed to advancing the science, practice, and use of value assessment in healthcare to make it more meaningful to those who receive, provide, and pay for care. IVI's annual Methods Summit convenes health policy leaders, health care executives, patient leaders, and researchers to address challenges in the methods and practice of patient-centered value assessment.

This brief is intended to provide information on why this focus for the Methods Summit is important, a review of key challenges that have historically limited progress in the field, and brief insights into some of the exciting work happening around collection and use of patient perspectives. Key questions are included to guide your thinking in advance, as well as a few notes about what to expect at the summit. Thank you for joining us for the 2021 IVI Methods Summit!

This convening is partially funded through a Patient-Centered Outcomes Research Institute® (PCORI®) Eugene Washington PCORI® Engagement Award (#EAIN-21156).

Jennifer Bright, MPA  
Executive Director



Rick Chapman, PhD  
Chief Science Officer



2021

# Executive Summary

Inclusion of inputs and perspectives from patients is essential to ensure that the research enterprise—from clinical and comparative effectiveness to outcomes and value assessment—captures the diversity of patient preferences and treatment experiences in the real world, and yields credible and relevant insights to inform decisions. IVI is convening the 2021 Methods Summit as a multi-day, virtual event to drive consensus on what matters most to patients in their health and health care and how to measure it.

As the data generated by our complex health care system rapidly increases, it is worth taking time to explore what information we already have at the ready that helps us understand what matters to patients and what data we need to truly inform patient-centered decision-making.

Below are a few highlights from this brief that we hope you will keep in mind as you prepare for and participate in the 2021 Methods Summit:

**Direct Input from Patients is Necessary:** Incorporating patient perspectives is both a primary gap in the current practice of value assessment and a priority for stakeholders in the health care system.

**Finding the Patient in the Maze of Healthcare Data:** We are in a “sea” of health care data, but it is locked in complex and siloed systems that hinder the use of this data for insight.

**Patient-Centered Research (PCOR/CER) Can Help Guide Decisions:** CER is generating valuable patient perspective research and data to inform decision-making, but additional areas of priority are critical to understanding the full range of impacts to patients.

**Incorporating Patient Perspectives Can Help Address Health Disparities:** Collection and use of inputs from a representative patient population will guide decision-making that closes the gap in access and outcomes.

**New Research and Initiatives Point to Solutions:** There is great work happening in the field of patient preferences and patient-centered outcomes, but consensus on where to start in bringing it into practice is needed.

## IVI Methods Summit Focus:

**Patient perspectives – including patient preferences, patient-reported outcomes, and impacts – are not adequately incorporated in patient-centered research or value assessment and they must be if we are to transition to an equitable, value-driven healthcare system.**

**If it is well established that this is a necessary realignment of the health care system, then what is stopping us?**

# Goals and Origins: 2021 IVI Methods Summit

Aligning value-based care with patient input, such as preferences for treatment options and reducing financial burden to patients in using care, is critical to achieving improved outcomes, reducing the burden patients experience in their care, and reducing overall health care costs. This refocusing of the health care system will require the intentional and explicit integration of patient perspectives and impacts into patient-centered research, quality measurement, clinical decision-making, and value-based purchasing programs.

This alignment is also necessary to illuminate and address health disparities and inequities. Consensus on the most important data inputs are a needed first step in making patient-centered research and value assessment relevant to today's complex health care decisions.

In February 2020, IVI convened the inaugural IVI Methods Summit, a multi-stakeholder forum in Washington, D.C., to explore unmet needs and gaps in value assessment methods and development priorities to make value assessment more meaningful to all stakeholders. IVI brought together leaders from research, patient advocacy, and across the health care system to build consensus on priorities and action. A summary of the convening is available [here](#). What emerged was agreement on the most critical gap: **lack of data on patient perspectives and impacts**.

To help fill that gap, IVI seeks to explore critical questions on what patient inputs – including preferences for treatment, patient experiences, and barriers in accessing or adhering to treatment – can be represented in data and in models to support decision-making.

IVI is convening the 2021 Methods Summit as a multi-day, virtual event to drive consensus on what matters most to patients in their health and health care and how to measure it. This convening is partially funded through a Patient-Centered Outcomes Research Institute (PCORI) Engagement Award (EAIN-21156). IVI has convened a [Steering Committee](#) over the past five months to support planning for this 2021 Methods Summit. The roster of Steering Committee members is included in the Appendix of this brief - IVI wants to thank each member for generously giving their time and perspectives to support planning of this event.

# Understanding the Full Range of Patient Impacts

## Direct Input from Patients is Necessary

Incorporating patient perspectives is both a primary gap in the current practice of value assessment and a priority for stakeholders in the health care system. This may be due in part to the fact that patients have been largely excluded from contributing to what is included in the design of clinical effectiveness research and value assessment models.

IVI has adopted the hierarchy of patient inputs published by the FDA as part of the Patient Preference Information Initiative (illustrated in Figure 1 to the left) as a foundation for how we describe what currently exists and where the gaps are in information available to support decision-making (HHS, 2016).

As the data generated by our complex health care system rapidly increases, it is worth taking time to explore what information we already have at the ready that helps us understand patient inputs and what data we need to truly inform patient-centered decision-making. Patient perspectives on value may differ from that of medical providers or other decision-makers.

IVI believes that incorporating patient perspectives in value assessment will unlock its potential to be a more relevant tool for informing equitable health care decision-making. However, the data to drive insight is a primary gap. Value assessment currently sits outside of traditional healthcare decision-making, being conducted through a handful of independent value assessment organizations or experts.

There is a growing national dialogue on the need to ensure the sustainability of U.S. health care systems by better linking the cost of healthcare to the benefits they provide to patients and establishing capabilities at a national level to guide these decisions. However, before national policy directions on how to do this are set, we must first ensure that we have the right data and methods to incorporate flexible and patient-centric approaches to examining value.

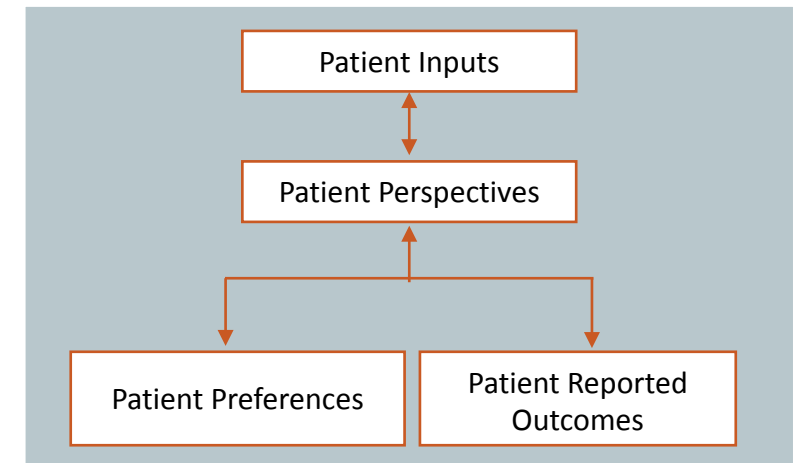


Figure 1. Hierarchy of Patient Inputs. FDA Final Patient Preference Guidance Document. [Available Online](#). (adapted)



# Finding the Patient in the Maze of Healthcare Data

## Finding the Patient in the Maze of Healthcare Data

The landscape of health care data is highly complex and siloed. Disparate data collection processes and systems hinder the use of this data for broader insights, due to a complex web of regulatory standards and market forces. Figure 2 to the right offers a generalized schematic of systems and the flow of data across them.

In this maze of data systems and processes, it is challenging to align measurement towards the patient impacts that are most important. While measurement currently focuses on care delivery processes and clinical outcomes, data collection must be expanded to include patient inputs that represent impacts on family and community, patient preferences for treatment, and financial impacts to patients and caregivers (Allen, 2017).

A significant amount of progress has been made in standardizing metrics representing patient-reported clinical outcomes; however, many gaps remain in comparable measures for assessing patient perspectives on other aspects of their experience of healthcare.

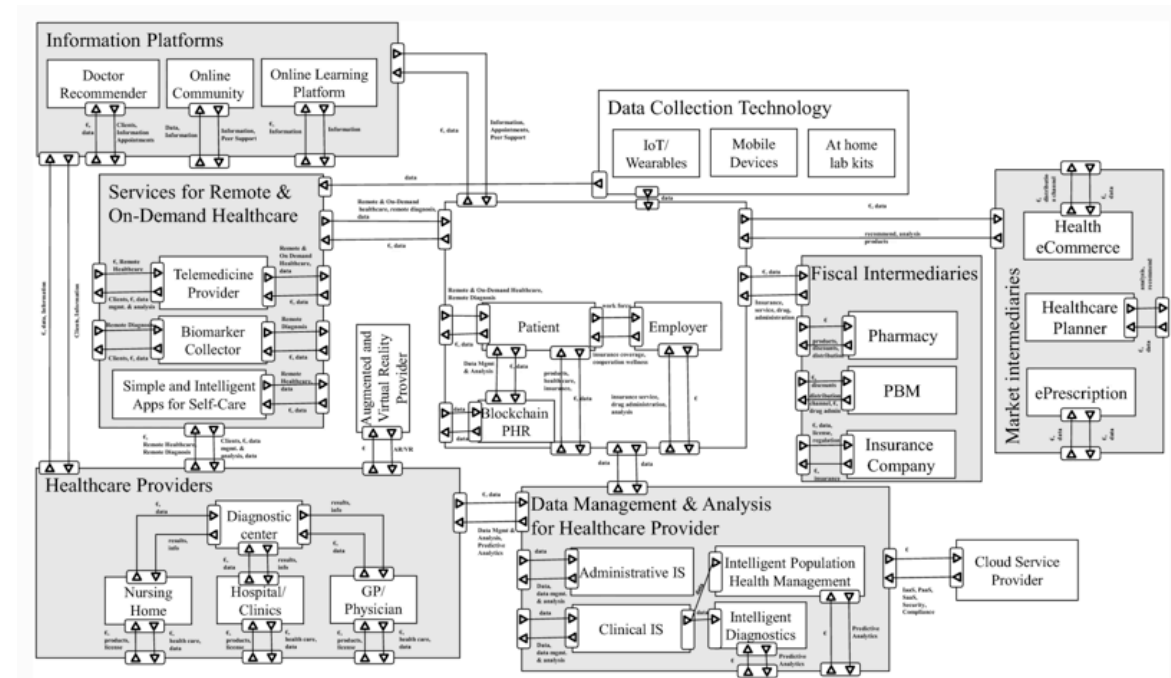
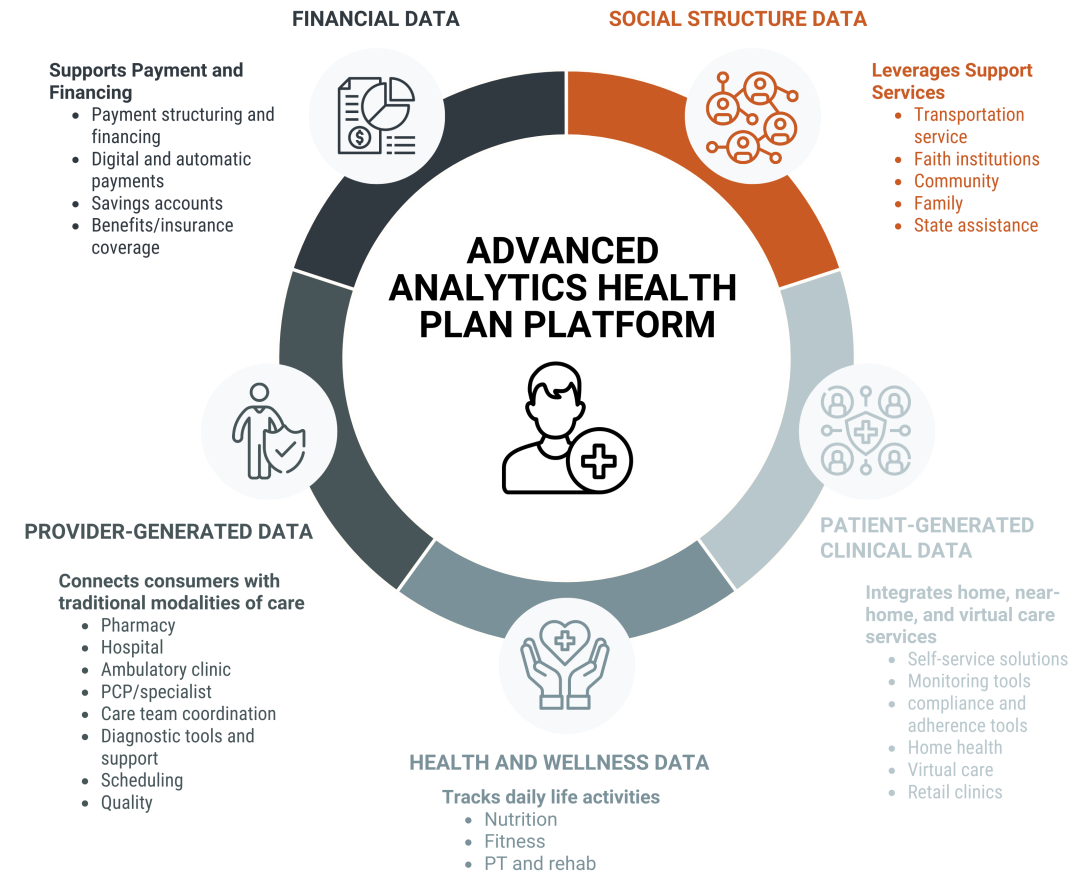


Figure 2. Schematic of Flow of Data Ecosystem Across Health Landscape: Ecosystem visualization of the digital healthcare industry. Market segments have been depicted as actors for better readability. The grey rectangles around multiple market segments represent generic roles. From: Hermes, S., Risanow, T., Clemons, E.K. et al. The digital transformation of the healthcare industry: exploring the rise of emerging platform ecosystems and their influence on the role of patients. Bus Res 13, 1033–1069 (2020). <https://doi.org/10.1007/s40685-020-00125-x>.

# Building the Ecology of Patient Centered Data Through Research

While the focus of IVI's stakeholder engagement and research is centered on improving patient-centricity in value assessment, IVI recognizes the critical contributions of Patient-Centered Outcomes Research (PCOR) and Comparative Effectiveness Research (CER) in generating valuable patient perspective research and data that may be utilized in decision-making. PCOR/CER is viewed as an important part of the data collection ecology.

Engaging patients from diverse patient subgroups in the data generation and collection process is equally important. In existing patient-centered assessment and studies (e.g., surveys), patients of color and lower socioeconomic status (SES) have historically been underrepresented, reducing representativeness of the evidence base and limiting their usefulness in guiding real-world healthcare decision-making. The current pandemic has highlighted the urgency to address the widening health and economic disparities across different subgroups in contemporary American society. Including direct patient inputs from underrepresented patient populations is the necessary first step to achieve such an objective.



Credit: McKinsey & Company. Source: <https://www.mckinsey.com/industries/healthcare-systems-and-services/our-insights/the-next-wave-of-healthcare-innovation-the-evolution-of-ecosystems#>

# Progress In The Field

Exciting collaborations that bring together researchers, patient advocates, and policy leaders are emerging to build capacity in the healthcare system to collect and use patient-centered data. A few examples are described below.



**Principles for the Consideration of the Full Range of Outcomes Data in PCORI-Funded Research:** PCORI® released these principles in March 2021 to ensure that PCORI-funded research considers the full range of outcomes data—including, as appropriate, potential burdens and economic impacts related to the utilization of healthcare services.



**Patient-Centered Core Impact Sets (PC-CIS):** National Health Council is developing a blueprint and inventory gathered from patients, caregivers, and other stakeholders, of the broad range of disease and treatment impacts on a patient's life. Core impact sets are envisioned as including the clinical outcomes currently used as endpoints or evaluative measures in PCOR/CER research, but also include other elements that impact patients, such as family stress or financial impacts.



**Patient Preferences in Treatment for Depression:** IVI is partnering with the PAVE (Patient-Driven Values in Healthcare Evaluation) Center at the University of Maryland to develop attributes and criteria for a patient-informed value assessment for treatment and outcomes among individuals with Major Depressive Disorder (MDD).



**Medical Device Innovation Consortium (MDIC) Science of Patient Input:** This program advances the art and science of patient engagement in the development, premarket approval, and post-market evaluation of medical devices. This work expands on the MDIC Patient Centered Benefit-Risk (PCBR) project, which was launched in 2013. The Framework report, "A Framework for Incorporating Information on Patient Preferences Regarding Benefit and Risk into Regulatory Assessments of New Medical Technology," was [published](#) in May 2015.



**Defining Patient-Important Outcomes:** IVI is partnering with RAND to pilot test the use of Goal Attainment Scaling to capture rheumatoid arthritis patient preference inputs and explore its application in multi-criteria decision analysis (MCDA). This exploratory research is looking for new methods to directly incorporate patient perspectives into optimal treatment choice.



# Building Novel Methods that Drive Insights Centered on Patient Inputs

Health technology assessments (HTA) and value assessments often use cost-effectiveness analysis (CEA) to assess the relative value of available treatment options. However, existing CEA models typically fail to capture a comprehensive range of patient perspectives, partly due to lack of patient engagement in model development and the lack of appropriate measurement of patient inputs. To date, a standardized framework and methods for the comprehensive inclusion and scaling of inputs on patient perspectives in CEA are lacking. Efforts to bring other analytic tools – such as Multi-Criteria Decision Analysis (MCDA) – into value assessment are underway. Such efforts hold the promise for inclusion into value models of additional types of data inputs that reflect the full range of impacts on patients.

IVI seeks to explore the barriers that currently prohibit inclusion of patient perspectives in patient-centered research and value assessment. **If it is well established that this is a necessary realignment of the health care system, then what is stopping us?** Figure 3 to the right identifies four scenarios that may contribute to lack of movement in the field.

While patients are increasingly engaging in the value assessment process, there are existing gaps in methods and practice for eliciting and incorporating patient perspectives, including patient preferences and other patient-reported outcomes.

Key challenges for the systematic use of patient input include: lack of appropriate data, inaccessible data, lack of transparency and trust in the data and methods, inability to capture and represent patient heterogeneity, and inconsistent processes and methods for eliciting and applying patient preferences and other patient-derived factors.

## We are not measuring the right data:

- Unclear what we should be measuring
- Unclear who should be measuring it

## We are not sharing the right data:

- Data on relevant patient impacts exist, but
- Barriers exist to accessing, sharing, and using it

## We are not able to collect the right data using current measurement:

- Uniform data not available
- Unclear who can or should standardize and collect uniform data

## We are not able to evaluate the data using current methods

- Clear and valid data are collected and accessible, but
- Methods for incorporating the data into HTA are not well-formed

Figure 3. Four scenarios hindering progress on incorporation of patient perspectives.

# Key Questions to Consider and Additional Reading List

1

What are the **most important** data inputs to represent patient perspectives that move beyond what is traditionally measured? What matters most to patients?

2

Thinking across disease areas, are there priorities for improving data inputs regarding the patient's lived **experience**? Regarding patient **outcomes**? Regarding **costs** to patients and caregivers?

3

What priority **action steps** need to occur to build trust in the reliability and validity of these types of data?

4

What **new methods or processes** are needed to make full use of data on patient perspectives and impacts to ensure these are reflected in decision-making?

## Recommended Reading List

**How to integrate evidence from patient preference studies into health technology assessment: a critical review and recommendations** (Kevin Marsh, Cambridge University Press, [access here](#))

**Making Alternative Payment Models Work For Patients** (Purva Rawal, Annie Cloke, and Lu Zawistowich, Health Affairs, [access here](#))

**The digital transformation of the healthcare industry: exploring the rise of emerging platform ecosystems and their influence on the role of patients.** (Hermes, S., Riasanow, T., Clemons, E.K. et al., Business Research, [access here](#)).

**An Equity Agenda for the Field of Health Care Quality Improvement** (Margaret O'Kane, Shantanu Agrawal, Leah Binder, Victor Dzau, Tejal K. Gandhi, Rachel Harrington, Kedar Mate, Paul McGann, David Meyers, Paul Rosen, Michelle Schreiber, and Dan Schummers, [access here](#)).

**Proposed Framework for Patient-Centered Outcomes-Based Measures in Alternative Payment Models** (Jabbarpour Y, Coffman M, Habib A, et al, [access here](#)).

**Limited role of patient input in specialty drug coverage policies** (Brittany D'Cruz, Jennifer S Graff, Ari D Panzer, James D Chambers, [access here](#))


**New Commission To Tackle How National Health Data Are Collected, Shared, And Used.** (Alonzo Plough Gail C. Christopher, [access here](#))

**First-Hand Perspectives in Rheumatoid Arthritis: Insights to Improve Healthcare Research & Value Assessment.** (Courtney, C., Phillips, L., Masco, R., O'Hara Levi, S., Bright, J., Eller, J., Hyde, A., Kelly, M. and E. Malik, [access here](#))



# Contact Us





## Headquarter

 917 Prince Street  
Alexandria, VA 22314

## Email Address

 [rick.chapman@thevalueinitiative.org](mailto:rick.chapman@thevalueinitiative.org)  
 [jennifer.bright@thevalueinitiative.org](mailto:jennifer.bright@thevalueinitiative.org)

## Website

 [www.thevalueinitiative.org](http://www.thevalueinitiative.org)  
 [www.ivimethodssummit.com](http://www.ivimethodssummit.com)

# Appendix:

## IVI Methods Summit Steering Committee

- Ashley Valentine, Sick Cells
- Arturo Cabra, ISPOR
- Barry Liden, Edwards Lifesciences
- Eleanor Perfetto, National Health Council
- Erin Holve, DC Department of Healthcare Finance
- Jessica Brooks Woods, Pittsburgh Business Group on Health
- Jon Campbell, Institute for Clinical and Economic Review
- Juan Marcos Gonzales, Department of Medicine, Duke University
- Kristi Mitchell, Avalere Health; Health Equity Outcomes
- Lizheng Shi, Tulane university School of Public Health and Tropical Medicine
- Maggie Jalowsky, Sick Cells
- Margaret Rehayem, National Alliance of Healthcare Purchaser Coalitions
- Margo Edmunds, Academy Health
- Michael Stancil, Pittsburgh Business Group on Health
- Patrick Gleason, Prime Therapeutics
- Simu Thomas, Alexion Pharmaceuticals

# Appendix:

## Chart of In Scope and Out of Scope Key Words

This table is intended to guide the scope of our discussions during the 2021 IVI Methods Summit. This has been designed to frame the key issues and questions that we intend to take up during this meeting. Our goal is building consensus on an action-oriented research agenda that answers critical questions on what patient inputs can be represented in data and in models and other resources to support decision-making.

To that end, we propose that our focus remain on the concepts outlined below as in scope. Discussion about the mechanics or merits of specific methods lie beyond the scope of the Summit's purpose.

In Scope	Out of Scope
Value Assessment/Health Technology Assessment (HTA)	Quality-adjusted life year (QALY)
Patient inputs	Cost-effectiveness analysis
Patient impacts	Methods for decision analysis
Patient perspectives	Multi-criteria decision analysis (MCDA)
Patient preferences	Distributional Cost-Effectiveness Analysis (DCEA)
Patient-reported outcomes	"It's too complicated"
Patient-centered outcomes research	Health inequity*
Comparative effectiveness research	
Representativeness	
Health/care disparity	
Costs to patients and caregivers	

\* Health inequity will be outside the scope of this summit. Addressing inequities requires an intentional approach to the application of patient inputs and patient data into the practice of health care decision-making.

With improved understanding of gaps from an equity lens, reformed approaches to resource allocation towards historically under-resourced communities can be implemented. Given the importance of this, IVI seeks to build this as a focus of convenings in the future.



# Appendix:

## Glossary of Terms

Terms	Definition
Health technology assessment (HTA)	A multidisciplinary process that uses explicit methods to determine the value of a health technology at different points in its lifecycle. Health technology assessment (HTA) is intended to inform decision-making, including decisions regarding which health technologies should or should not be reimbursed. A health technology is the application of organized knowledge and skills in the form of devices, medicines, vaccines, procedures, and systems developed to solve a health problem and improve quality of lives for individuals affected.
Value assessment (VA)	Comparison of the relative benefits to the costs of a given technology or service for a specific person or population.
Health economic modeling	A set of analytic approaches in health economic analysis that synthesize clinical, epidemiological, and economic evidence from different data sources into an evaluation framework that will enable researchers or decision makers to generate estimates for specific outcomes of interest. Models are usually a simplified representation of the real world to inform decision-making by characterizing uncertainty in projecting outcomes.
Cost effectiveness analysis	A method to examine both the costs and health outcomes of one or more interventions. It compares an intervention to another intervention (or the status quo) by estimating how much it costs to gain an additional unit of some health outcome, such as a life year gained or a case prevented.
Value element	Refers to specific aspects or components that stakeholders may consider to be part of an overall assessment of value (e.g., different mode of administration, reduced risk, lower cost).

Terms	Definition
Patient inputs	Include a wide range of information and perspectives from patients including but not limited to: informal comments; patient opinions, including through social media; patient responses to qualitative surveys; and quantitative measurements of patient-reported outcomes (PROs). Used to describe the elicitation from patients of a spectrum of impacts a disease and its treatments have on a patient's life, inclusive of clinical endpoints and PROs, as well as impacts such as stress on family, caregiver burden, and financial toxicity.
Patient perspective	A specific type of patient input describing patients' experience with a disease or condition and its management.
Patient preference	Qualitative or quantitative assessment of the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions.
Patient impacts	Factors that are collected from patients and others to be used across health care research and decision-making. Identification and prioritization of disease and treatment impacts are guided by patient contributions as partners.