A Blueprint for Patient-Centered Value Research

July 2025

Center for Innovation
& Value Research

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Our Mission

To advance the science, practice, and use of patient-centered **health technology assessment (HTA)** to support decisions that make healthcare more meaningful and equitable.

Our Vision

A learning healthcare system supported by patient-centered **health technology assessment (HTA)** and focused on high-quality, efficient, innovative, and equitable care for all people and communities.

Our Work

The Center drives innovation in HTA through **patient-centered value research** and partnerships focused on patient preferences, novel value methods, and model development. We collaborate with patients, researchers, employers, payers, and providers to define and assess value, serving as a laboratory for testing and refining patient-centered HTA methods.

For a more detailed explanation of the terms used in this document, please refer to the Glossary on page 16.

Purpose and Scope of the Blueprint

The ***Blueprint*** defines the Center's approach to patient collaboration in **value research** and provides a structured framework for integrating patient perspectives throughout the research process. This resource is designed primarily for researchers, patients and caregivers*, and patient advocacy organizations supporting them in developing and participating in meaningful, patient-centered value research. Other stakeholders, including payers, policymakers, purchasers, healthcare providers, and life sciences manufacturers are encouraged to use the Blueprint to strengthen patient partnership in their research processes. The Blueprint serves as a shared resource for all who seek to embed patient partnership into the design, execution, and dissemination of value research.

Researchers

Patients and Caregivers

Patient Advocacy Organizations

Payers / Purchasers

Healthcare Providers

Life Sciences Companies

Defining Patients and Caregivers*

Throughout the Blueprint, we use the term "patients and caregivers." This term refers to people with lived experience, such as patients, caregivers, family members, and community members that represent the population of interest. We use the term "patient" to refer to a person who has had a significant encounter with the healthcare system. We do not suggest that a person is their disease or that is a person's only area of experience or expertise, but use this term in recognition of their lived experience and the importance of this experience to improving the healthcare system.

The Impact of Partnering with Patients and Caregivers in Patient-Centered Value Research

Patients and caregivers offer invaluable insights and have significant expertise and experience to contribute. They should be recognized as equal and critical partners across all stages in the research process. Meaningful engagement with patients and caregivers from diverse backgrounds and perspectives is essential to advancing value-based healthcare and research. This approach underscores the important shift towards patient-centered care—one that acknowledges patient diversity and prioritizes active involvement in research. For additional resources on implementing this approach, see page 13.

Value Principles and Key Areas of Work

Since 2019, the Center has served as a learning laboratory, leading the conversation around advancing methods for HTA and value research. This Blueprint reflects the culmination of the Center's research and stakeholder engagement efforts to date, and provides a roadmap for our future work, rooted in our value principles highlighted below.

CORE PRINCIPLES

Sustains Authentic Patient Centricity

Supports Health Equity

Advances Transparency

SUPPORTING PRINCIPLES

Focuses Value Discussion Across Treatment Interventions

Improves Clinical and Real-World Data

Facilitates Customizable Decision-Making

Adapts to and with Evolving Evidence

Cultivates Modernized Methods

Fosters Long-Run Innovation

These principles provide the foundation for the development of key projects and processes that underpin the Blueprint, including HTA Open-Source Value Models, Rare Disease Patient-Centered Outcomes Project, Patient-Centered Economic Impacts Project, and our Health Equity Initiative. As an ongoing process, the Blueprint also serves as a framework for new methods and approaches to patient-centered value research. We recognize that this is a continuous learning journey, and we remain committed to refining and improving our work.

Conceptual Blueprint

Defining Value Research

Value research focuses on understanding the value of healthcare services, interventions, treatments, and policies. The "value" is determined by assessing outcomes relative to the costs involved. Value research is an umbrella term that includes one or more of the following components:

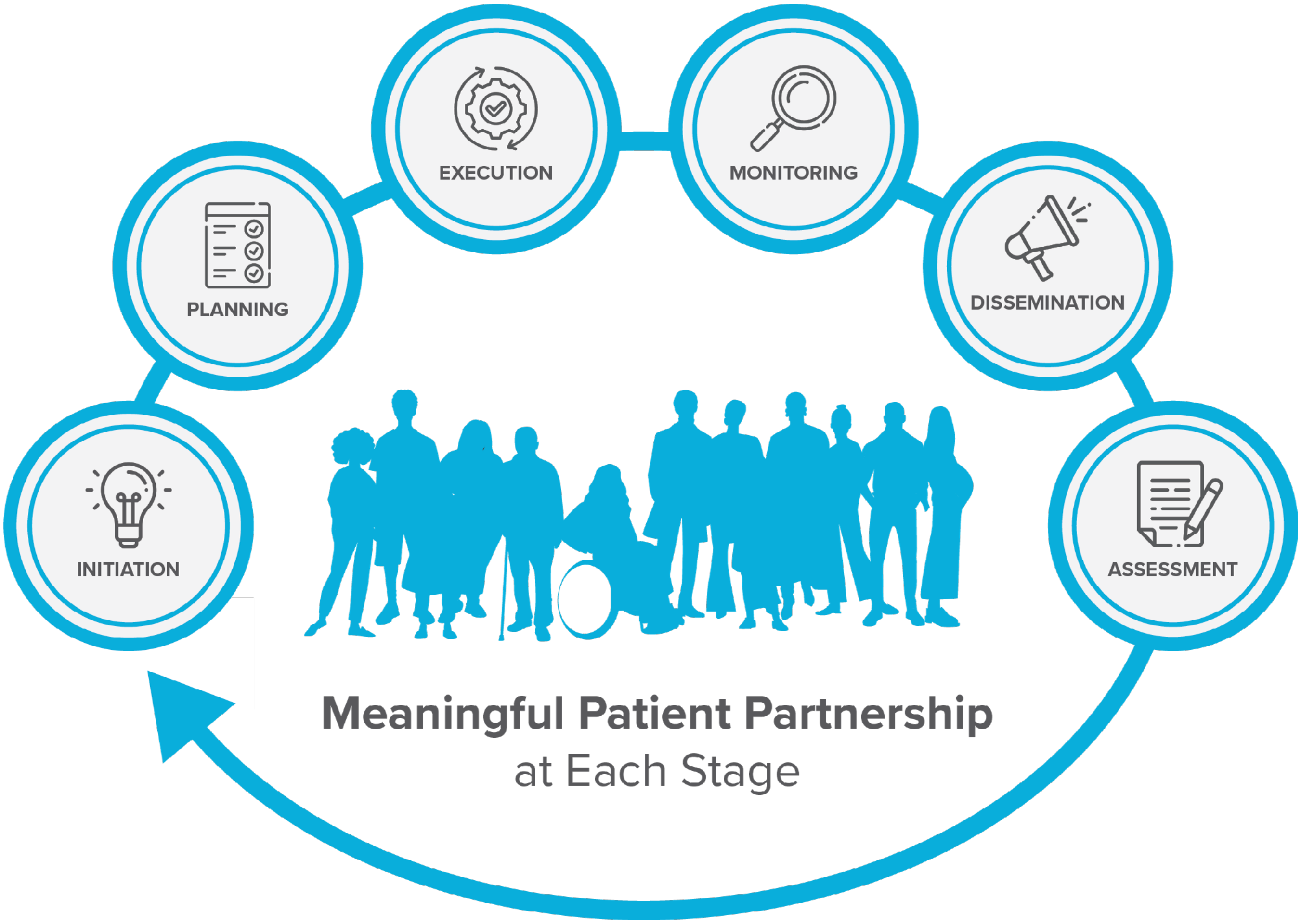
- **Comparative Clinical Effectiveness Research (CER)** is a type of clinical research that compares two or more medical treatments, services, or health practices to help patients and others make better-informed decisions.[1]
- **Patient-Centered Outcomes Research** "is the evaluation of questions and outcomes meaningful and important to patients and caregivers."[2]
- **Cost-Effectiveness Analysis** is "an analytic tool in which the costs and effects of a program and at least 1 alternative are calculated and presented in a ratio of incremental cost to incremental effect. Effects are health outcomes, such as cases of a disease prevented, years of life gained, or quality-adjusted life-years, rather than monetary measured as in cost-benefit analysis."[3]
- **Health Technology Assessment (HTA) or Value Assessment (VA)** is an analysis of the clinical and economic value of a particular healthcare intervention using various factors, such as cost, safety, and efficacy.[4] Employers, payers, life sciences manufacturers, and policymakers, among others, use it to help determine how patients are treated and at what cost.
- **Patient-Centered HTA or Patient-Centered VA** is where "patients are engaged, heard, understood, and respected throughout the entire VA process, and their input is incorporated and guides decision-making."[5]

How Patient-Centered Value Research Informs Healthcare Decision-Making

The Center conducts value research with an emphasis on one of our core principles, patient-centricity. **We define patient-centered value research as a meaningful and impactful partnership between patients and researchers in the design and conduct of research. Patients are engaged at every stage of the research process—from conceptualization and design through dissemination and implementation** (see Figure 1). This approach ensures that patient input directly shapes research priorities, methodologies, and outcomes, with the goal of generating insights that authentically reflect patient experiences, needs, and preferences.

On pages 7 through 12 of this document, we break down the six stages of patient-centered value research and highlight key activities that should be conducted at each stage, the applied value principles, and real-world examples of the Center's work in patient-centered value research.

Figure 1. A Blueprint for Patient-Centered Value Research



INITIATION

KEY ACTIVITY	APPLIED PRINCIPLE	REAL-WORLD EXAMPLE
Co-Develop Research Questions Collaborate with patients and caregivers to create research questions that directly address patients’ needs and priorities.	**Sustains Authentic Patient-Centricity** Includes diverse patient voices to reflect community and population needs.	**Health Equity Initiative** Developed research questions with input from community representatives to address economic and access disparities in healthcare.
Establish Multi-Stakeholder Advisory Groups Form advisory groups that include patients, caregivers, healthcare providers, and researchers to incorporate diverse perspectives.	**Supports Health Equity** Engage patients from diverse backgrounds to shape questions relevant to their experiences.	**Major Depressive Disorder Model** Created an advisory group involving patient advocates, employers, payers, life sciences companies, researchers, and clinicians to provide holistic input on model goals and applicability.
Explore the Patient Journey Map out the patient and caregiver journey to identify key points, challenges, and unmet needs related to treatment and care pathways over time.	**Improves Clinical and Real-World Data** Capture detailed patient perspectives to inform authentic journey mapping.	**Economic Impacts Projects** Developed report on how to use patient journey mapping to reveal the full spectrum of economic burdens faced by patients and caregivers.

PLANNING

KEY ACTIVITY	APPLIED PRINCIPLE	REAL-WORLD EXAMPLE
Seek Input on Patients' Priorities Engage patients and caregivers to define what outcomes are most meaningful, such as symptom relief, daily function, and overall quality of life.	**Facilitates Customizable Decision-Making** Align outcome priorities with what matters most to individual patients.	**Major Depressive Disorder Model** Focused on patient-defined priorities, such as daily functionality and symptom management, for treatment model outcomes.
Contribute to Data Exploration and Feasibility Work with patients and caregivers to assess what data should be collected, determine the best methods to ensure it accurately reflects their experiences, and ensure diverse populations are represented in data sources.	**Advances Transparency** Engage patients in data methods to ensure data integrity and transparency.	**Economic Impacts Project** Collaborated with patient representatives to understand a broad range of economic impacts that would best represent the cost burdens on patients and caregivers.
Review Study Protocols Develop and review study protocols with patients and caregivers to ensure that the study aligns with the needs and priorities of patients.	**Cultivates Modernized Methods** Patient feedback informs accessible innovative protocols that reflect real world needs.	**Major Depressive Disorder Model** Review study protocols with patient advocates to ensure inclusivity and accessibility in study design and execution.

EXECUTION

KEY ACTIVITY	APPLIED PRINCIPLE	REAL-WORLD EXAMPLE
Employ Mixed Methods Use both quantitative and qualitative methods, such as surveys and interviews, to provide a comprehensive view of patient outcomes and preferences.	**Improves Clinical and Real-World Data** Use a blend of data to accurately capture and respect diverse patient perspectives.	**Major Depressive Disorder Model** Utilized mixed methods, such as surveys and interviews, to understand a wide range of patient-reported treatment preferences and to design model's user interface.
Conduct Analyses Analyze data together with patient partners to evaluate outcomes that reflect the patient-centered goals established during planning, focusing on patient-defined priorities.	**Adapts to and with Evolving Evidence** Use analyses that can evolve to incorporate new findings and patient input.	**Rare Disease Project** Addressed outcomes data focusing on quality of life and healthcare access issues specific to rare disease patients.
Provide Feedback on Preliminary Results Share early results with patients and caregivers for feedback validation to ensure findings align with their experiences and perspectives.	**Advances Transparency** Early and open sharing fosters trust and public accountability.	**Economic Impacts Projects** Shared initial economic impact recommendations with patients and caregivers to validate results. Revised the framework based on their feedback.

MONITORING

KEY ACTIVITY	APPLIED PRINCIPLE	REAL-WORLD EXAMPLE
Determine Relevance of Study Results Assess how results apply to the broader patient community and identify potential implications for healthcare practice and policy.	**Supports Health Equity** Tailor findings to increase accessibility and relevance for diverse patient groups.	**Health Equity Initiative** Used findings to develop recommendations for equitable access to healthcare services in underserved communities.
Conduct Regular Feedback Host regular check-ins with patient partners to discuss progress, challenges, and any necessary adjustments to the research process.	**Sustains Authentic Patient-Centricity** Regular patient input ensures alignment with evolving patient needs.	**Major Depressive Disorder Model** Head quarterly sessions with mental health advocates to discuss model development and address any research barriers or areas needing adjustment.

DISSEMINATION

KEY ACTIVITY	APPLIED PRINCIPLE	REAL-WORLD EXAMPLE
Hold Public Comment Periods Provide an opportunity for public input and feedback, with a recommended minimum comment period of 30-60 days to ensure ample time for meaningful input.	**Advances Transparency** Enable community validation through public input and open access.	**Major Depressive Disorder Model** Shared preliminary findings with the public and held three public comment periods to gather diverse stakeholder feedback to ensure transparency.
Present Findings to Diverse Audiences Include a plain language summary for all reports that is easy to read and understand by all audiences, including patients, and provide a visual timeline or checklist to guide dissemination planning and stakeholder communications.	**Forces Value Discussion Across Treatment Interventions** Make findings accessible and clear for all stakeholders.	**Rare Disease Project** Presented findings on diagnostic challenges to patient advocacy groups and healthcare providers with accessible, plain language summaries.
Co-Author Reports and Publications Invite patients and caregivers to co-author reports and/or publications, giving them recognition for their valuable input and partnership, and incorporate a visual timeline or checklist to ensure clear and coordinated communication of findings across all stakeholders.	**Sustains Authentic Patient-Centricity** Give due credit to patient partners, reinforcing the value of patient collaboration in research.	**Economic Impacts Projects** Invited all steering committee members to serve as co-authors in poster presentation for the PCORI Annual Meeting.

ASSESSMENT

KEY ACTIVITY	APPLIED PRINCIPLE	REAL-WORLD EXAMPLE
Discuss Recommendations for Future Research Collaborate with patient partners to identify gaps in the current research and suggest directions for future studies that address remaining questions.	**Facilitates Customizable Decision-Making** Enable research to adapt and address patient-identified areas of need.	**Health Equity Initiative** Engaged patient advocates to identify additional research needs in policy applications for health equity.
Maintain Ongoing Feedback Loop Assess what worked well by asking for feedback from the team, including patient partners and caregivers.	**Adapts to and with Evolving Evidence** Foster adaptability and improvement based on real-time patient feedback.	**Major Depressive Disorder Model** Collected ongoing feedback from stakeholders on barriers and successes, ensuring an iterative improvement process for the model.
Evaluate Research, Policy, and Coverage Recommendations Assess the impact of research findings on policies and coverage decisions, ensuring they support patient needs and priorities, and assess equity impacts and relevance across patient subgroups.	**Fosters Long-Run Innovation** Align findings with policy changes that support patient-centered care and access.	**Inflation Reduction Act Initiative** Actively engaged with the Centers for Medicare & Medicaid Services, provided guidance comments, and organized a policy symposium to inform and shape health policy.

Resources for Patient Partnership in Research by Stage

The list below provides a selection of key resources for each stage of the Blueprint. Resources may apply to multiple stages of the Blueprint, but are not repeated. In addition, the list is not exhaustive, but is a starting point for organizations looking to partner with patients and caregivers on patient-centered value research.

Across All Stages

- Center for Innovation & Value Research Patient-Centered Economics Impacts Framework
- European Patients' Academy of Therapeutic Innovation (EUPATI) Open Classroom
- Institute for Patient- and Family-Centered Care: A Toolbox for Creating Sustainable Partnerships with Patients and Families in Research
- National Alliance for Caregiving (NAC) Caregiving in the U.S.
- National Health Council (NHC) Rubric to Capture the Patient Voice and Value Classroom
- Patient-Centered Outcomes Research Institute (PCORI) Foundational Expectations for Partnerships in Research
- Patient-Centered Outcomes Research Institute (PCORI) Measuring What Matters
- Patient-Focused Medicine Development (PFMD) Patient Engagement Management (PEM) Suite
- Society for Women's Health Research (SWHR) Policy Principles Report
- The Council of Medical Specialty Societies (CMSS) and Patient-Led Research Collaborative (PLRC) Patient-Led Research Scorecards
- U.S. Food and Drug Administration (FDA) Patient-Focused Drug Development Guidance

Initiation

- NHC Patient Compensation Tools
- NHC Patient Experience Mapping Tools
- Orphan Drug Development

Planning

- SPIRIT-PRO Extension: Protocol checklist for planning patient-reported outcomes in clinical trials

Execution

- Co-Design Methodologies: Guides on co-developing research protocols with patients
- The HTAi Patient and Citizen Involvement Group: Best practices for executing patient-centered HTA
- Tools like REDCap or Dedoose for managing qualitative data from patient input

Monitoring

- Council for International Organizations of Medical Sciences (CIOMS): International Ethical Guidelines for Health-related Research Involving Humans

Dissemination

- Agency for Healthcare Research and Quality (AHRQ) Publishing and Communication Guidelines
- Frameworks such as RE-AIM: Guidance for disseminating and scaling HTA findings effectively
- Plain Language Summary Resources: Templates and tools for creating accessible content

Assessment

- Evaluation of Patient Engagement in Medicine Development
- Frameworks for FDA’s Real-World Evidence Program: Guide assessing outcomes in real-world applications
- National Pharmaceutical Council Current Landscape: Value Assessment Frameworks

Glossary

Term	Definition
Caregiver	A person who provides physical, emotional or practical support to a patient, often a family member or friend, playing a critical role in managing health and treatment adherence.[6]
Comparative Effectiveness Research	Studies that compare the benefits and harms of two or more treatments, clinical strategies, or other approaches to healthcare.[7]
Cost-Effectiveness Research	Form of economic analysis that compares the relative costs and outcomes (effects) of different courses of action. In healthcare, this often takes the form of comparison of programs or interventions to a single comparator (generally the standard of care) in terms of incremental change in health benefits versus incremental changes in cost. CEA contains the basic elements needed to calculate a net monetary benefit of a new intervention versus the standard of care for the target population of interest.[8]
Health Equity	The attainment of the highest level of health for all people, where everyone has a fair and just opportunity to attain their optimal health regardless of race, ethnicity, disability, sexual orientation, gender identity, socioeconomic status, geography, preferred language, and other factors that affect access to care and health outcomes.[9]
Health Technology Assessment (HTA)	HTA is a process of looking at new medical treatments, devices, or procedures to see if they are safe, work well, and are worth the cost. It helps doctors and health systems decide which treatments should be offered to patients.[10]
Patient-Centered HTA	This approach focuses on making sure that the needs and experiences of patients are at the center of the decision-making process. It ensures that new treatments or technologies help improve the quality of life and health outcomes for patients.
Patient-Centered Value Research	Patient-centered value research is the meaningful and impactful partnership between patients and researchers in the design and conduct of value research, to ensure that every stage of the research process—from design to dissemination and implementation—is shaped by the experiences, needs, and preferences of those most impacted, aligning priorities and outcomes to generate meaningful and actionable insights.

Term	Definition
Patient Centricity	The meaningful and active involvement of patients as partners across all stages of research. Patient centricity ensures that patient needs, values, lived experiences, and preferences shape research priorities, design, conduct, and dissemination. This approach emphasizes co-creating evidence that is relevant, actionable, and aligned with what matters most to those directly affected by healthcare interventions.[11, 12]
Real-World Data	Data relating to patient health states and/or delivery of healthcare routinely collected from a variety of sources, such as electronic health records, claims databases, product and disease registries, and data gathered through personal devices and health applications.[13]
Transparency	The commitment to openly sharing research objectives, processes, and methods, data sources, and findings with all stakeholders, including patients, caregivers, and the public. This ensures trust, reproducibility, and accountability across all types of research and is essential for advancing knowledge, improving outcomes, and the responsible use of evidence.[14, 15, 16]

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- The Center's Scientific Advisory Panel
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- Anna Hyde, MA, Vice President of Advocacy and Access, Arthritis Foundation
- Kimberly Westrich, MA, Chief Strategy Officer, National Pharmaceutical Council

Their contributions were instrumental in shaping the Blueprint's content, ensuring that it reflects a wide range of perspectives and meets the needs of both researchers and patient communities. We appreciate their partnership with the Center and their commitment to advancing patient-centered value research.

Endnotes

1 https://www.pcori.org/about/about-pcori

2 https://jamanetwork.com/journals/jama/fullarticle/1901303. A more detailed definition can be found at: https://www.pcori.org/research-related-projects/about-our-research/research-we-fund/establishing-definition-patient-centered-outcomes-research/patient-centered-outcomes-research

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