

Partnering with Patients

Principles and Commitments

Version: 1.0

April 12, 2018



This work is licensed under the Creative Commons Attribution 4.0 International License. To view a copy of this license, visit <http://creativecommons.org/licenses/by/4.0/>.



Table of Contents

Table of Contents.....	i
List of Tables and Figures.....	ii
List of Abbreviations.....	iii
Executive Summary.....	1
1. Patient-Centered Value Assessment.....	2
1.1. Objectives.....	3
1.2. Document Development.....	3
2. Pillars of Patient Partnership.....	3
2.1. Core stages of partnership.....	5
2.2. IVI Principles of Patient Partnership.....	5
Principle 1: IVI will involve multiple patient stakeholders in value assessment.....	5
Principle 2: IVI will employ robust methods to identify and involve patient stakeholders.....	6
Principle 3: IVI will partner with stakeholders at all stages of research.....	6
Principle 4: IVI will continuously evaluate and refine patient partnership action.....	8
Principle 5: IVI will commit resources for continuous engagement.....	9
3. The Road Ahead.....	9
4. Acknowledgements.....	10
4.1. Working Group Membership.....	10
4.2. Funding.....	10
5. About the Innovation and Value Initiative (IVI).....	10
6. References.....	12
7. Appendices.....	13
Appendix A: Literature Review.....	13
Appendix B: Alignment with NHC Patient-Centered Value Model Rubrics.....	14
Appendix C: Surveys to Evaluate the Impact of Patient Partnership.....	17



List of Tables and Figures

Table 1. Stakeholders in Patient-Centered Outcomes Research: The 7Ps Framework	4
Table 2. OSVP IVI RA Model alignment with NHC Patient Centered Value Model Rubrics	14
Table 3. Evaluating the impact of patient partnership in IVI – <i>Researcher Survey</i>	17
Table 4. Evaluating the impact of patient partnership in IVI – <i>Patient Stakeholder Survey</i>	19



List of Abbreviations

Abbreviation	Description
CEA	Cost effectiveness analysis
CER	Comparative effectiveness research
IVI	Innovation and Value Initiative
NHC	National Health Council
OSVP	Open-Source Value Project
PCOR	Patient centered outcomes research
PCORI	Patient Centered Outcomes Research Institute



Executive Summary

IVI Partnering with Patients: Principles and Commitments

Background

There is enormous pressure in the US healthcare system to find the equilibrium of the Triple Aim: high-quality, accessible care at an efficient cost. Accordingly, the US healthcare system is undergoing a transformation as costs are being aligned to the perceived value of care and treatment. Numerous organizations have developed value frameworks to assess and compare the value of new therapies. However, to date, these value frameworks have largely been developed using clinical trial data, and often do not include data reflecting real-world patient experiences or outcomes. In addition, despite calls to include patients at all stages of value assessment, the patient perspective is often limited, or incorporated at later stages of value assessments.

IVI's mission includes a commitment to developing scientifically-rigorous, patient-centered value assessment tools. Our approach aims to take into consideration the most relevant possible forms of patient input and data, and incorporate consistent methods for involving patient input from the earliest stages of research design.

Perspective

To date, efforts to meaningfully engage patients in value assessment have been uneven, but there is increasing focus on addressing these challenges. We intend to contribute further to this dialogue by outlining our own principles and practices for partnering with patients in our value assessment work. We believe that collaborating with patient stakeholders – both individual patients and patient advocacy organizations – at the formative stages of value assessment will increase the likelihood that our decision-making and strategic initiatives are aligned with what patients' value about treatment. In particular, we hope to establish patient-centricity in IVI projects on an ongoing basis by ensuring that our work:

- Addresses the questions and issues most meaningful to patients by engaging patients and stakeholders from diverse communities to understand what is most relevant and meaningful for them in value assessment
- Integrates the patient voice throughout our development of value assessment processes and tools
- Solidifies the role of patient stakeholders in continuing public dialogue around value

Principles of Patient Partnership

- **Principle 1:** IVI will involve multiple patient stakeholders in value assessment.
- **Principle 2:** IVI will employ robust methods to identify and involve patient stakeholders.
- **Principle 3:** IVI will partner with patient stakeholders at all stages of research.
- **Principle 4:** IVI will continuously evaluate and refine patient partnership action.
- **Principle 5:** IVI will commit resources for continuous patient engagement.



1. Patient-Centered Value Assessment

The US healthcare system is undergoing a transformation as costs are being aligned to the perceived value of care and treatment. Numerous organizations have developed value frameworks to assess and compare the value of new therapies. However, to date, these value frameworks have largely been developed using clinical trial data, and often do not include data reflecting real-world patient experiences or outcomes. In addition, despite calls to include patients at all stages of value assessment, the patient perspective is often limited, or incorporated at later stages of value assessments.

IVI's mission includes a commitment to developing scientifically-rigorous, patient-centered value assessment tools. Our approach aims to take into consideration the most relevant possible forms of patient input and data, and incorporate consistent methods for involving patient input from the earliest stages of research design.

We recognize that there are many obstacles to engaging patients in value assessment. These include the use of technical language, which prevents patients not steeped in medical or reimbursement terminology from understanding and participating in conversations about value. Other barriers may include tight project timelines and limited resources allocated to accommodate interaction with patient communities. Beyond the technical aspects, however, perhaps the most significant obstacle to authentic patient engagement is the lack of sufficient trust. Historically, patients have neither been prioritized nor supported as active participants in the research enterprise. This effort seeks to help address the trust issue, and take it into account in our work.

There has been widespread acknowledgment that this must change.[1-4] We intend to contribute further to this ongoing dialogue by outlining our own principles and practices for partnering with patients in our value assessment work. We are guided in this by the notion that *“Existence of trust is key to motivating partners and communities to participate and enrolling patients in patient-centered outcomes research (PCOR). Building trust is not a step in the patient engagement process but rather an overarching paradigm that needs to be ubiquitous throughout PCOR efforts.”*[5]

We recognize the heterogeneity of patients, their experiences, and their values and preferences. Our intent is to integrate the diversity of patient preferences into all aspects of our work. We believe that collaborating with patient stakeholders – both individual patients and patient advocacy organizations – at the formative stages of value assessment will increase the likelihood that our decision-making and strategic initiatives are aligned with what patients value about treatment. This involvement by patients and the resulting relevance of our research will contribute to the ongoing dialogue taking place among patients, payers, providers and innovators about how to define and evaluate value. In particular, we hope to establish patient-centricity in IVI projects on an ongoing basis by ensuring that our work:

- Addresses the questions and issues most meaningful to patients by engaging patients and stakeholders from diverse communities to understand what is most relevant and meaningful for them in value assessment;
- Increases transparency about IVI research and trust between patient communities and IVI researchers to achieve increased usefulness of IVI output.
- Integrates the patient voice throughout our development of value assessment processes and tools;
- Encourages the role of patient stakeholders in continuing public dialogue around value.



1.1 Objectives

In setting forth its principles and practices for partnering with patients, IVI aims to achieve the following objectives:

- Outline guiding principles and actions that will facilitate meaningful patient collaboration in IVI work;
- Ensure that the patient perspective is present throughout all of our work;
- Establish a basis for assessing patient-centricity in IVI projects on an ongoing basis.

This document is dynamic, and we anticipate ongoing input and refinement to reflect key learnings and priorities as IVI grows, builds alliances, and carries out its mission.

1.2 Document Development

On September 13, 2017, IVI convened a Working Group on Patient Engagement. The Working Group met to identify and explore issues and approaches to patient partnership that could then be used to inform the development of a guidance document for how best to collaborate with patients in IVI research projects and initiatives. The Working Group represented a collaboration among members of the patient, medical, healthcare policy, health care delivery, life sciences, and payer communities. A roster of participants appears in Section 4.1.

Members of the IVI Working Group on Patient Engagement shared their experiences, perspectives, and opinions about the most effective strategies and approaches for building patient partnerships in research and in value assessment. They reviewed IVI patient engagement efforts to date, and provided input into the content that should be included in the guidance document as well as the best mechanisms for dissemination and for eliciting feedback from the patient communities.

While effort was made to include members of patient communities in the Working Group, we recognize that broader patient participation is still needed. Accordingly, as part of the meeting agenda, the members outlined a plan for public dissemination and comment before the guidance document is to be finalized. Finally, to inform guidance document content, IVI staff conducted a review and synthesis of the literature (both published and unpublished) disseminated by the Patient-Centered Outcomes Research Institute (PCORI) and other established organizations and researchers on engaging patients in healthcare and healthcare research.

2. Pillars of Patient Partnership

To be patient centered, value assessment needs to consider and incorporate the preferences, values, and experiences of patients and patient groups (patient stakeholders) when determining treatment value. This is because the questions and outcomes that matter most to other types of healthcare stakeholders are not always the questions and considerations that are most important to patients.

IVI is aware that historically, the research enterprise has not consistently included patient stakeholders and thus their perspectives. Although strides have been made in establishing the methods and expectations for patient participation in research (most notably championed by PCORI), all stakeholders acknowledge that such work continues to evolve and that the focus must remain on ensuring structured



involvement at the earliest stages of research design. Moreover, we must recognize that achieving balanced stakeholder contribution to the research enterprise requires greater focus on:

- Patient education and empowerment to participate in research design, execution and evaluation,
- Prioritizing patient perspectives and evaluating their impact on final value assessment.
-

The IVI approach to patient collaboration is guided by the principles outlined by the Patient-Centered Outcomes Research Institute (PCORI) and the National Health Council (NHC). Each prioritize ensuring that diverse patient voices and perspectives drive every step of their research and initiatives.[6, 7] Specifically, the NHC defines the term “patient centered” as broadly meaning “*any process, program, or decision that is focused on patients, in which patients play an active role as participants, and with a central focus on optimizing the use of patient-provided information.*”[8]

By using these important frameworks as our “true north,” IVI will ensure that patients are our active partners and participants in setting research priorities and implementing patient-centered research studies and value assessment initiatives.[9]

Finally, IVI efforts to understand and measure value must take a multidisciplinary approach with input from various stakeholders in patient centered outcomes research, as described in a taxonomy developed by Concannon and colleagues and depicted in Table 1.[10]

Table 1. Stakeholders in Patient-Centered Outcomes Research: The 7Ps Framework

Stakeholder Category	Description
Patients and the Public	Current and potential consumers of patient-centered health care and population-focused public health, their caregivers, families, and patient and consumer advocacy organizations
Providers	Individuals (e.g., nurses, physicians, mental health counselors, pharmacists, and other providers of care and support services) and organizations (e.g., hospitals, clinics, community health centers, community-based organizations, pharmacies, EMS agencies, skilled nursing facilities, schools) that provide care to patients and populations
Purchasers	Employers, the self-insured, government and other entities responsible for underwriting the costs of health care
Payers	Insurers, Medicare and Medicaid, state insurance exchanges, individuals with deductibles, and others responsible for reimbursement for interventions and episodes of care
Policy Makers	The White House, Department of Health and Human Services, Congress, states, professional associations, intermediaries, and other policy-making entities
Product Makers	Drug and device manufacturers
Principal Investigators	Other researchers and their funders



Through its governance structure (Strategic Advisory Panel) and specific research approach, IVI seeks input and collaborative engagement from these stakeholder sectors. We do so throughout the lifecycle of our work, from forecasting new project opportunities to delivering final tools for use in value assessment. As this document focuses on our partnership with patients, our delineation of specific stages and actions concentrates on that stakeholder segment in ensuing sections.

2.1 Core Stages of Partnership

IVI wants to ensure that patients are included as partners in the design and execution of value assessment tools at all stages of a project or initiative life cycle. We envision three primary realms where we will partner with patient stakeholders:

Open-Source Value Project (OSVP) Model Development

- Explore and assist IVI in prioritizing research questions for developing value assessment tools and economic model development
- Contribute to understanding of patient experience, determinants of value, and preferences that should inform model design
- Review research study procedures as appropriate, and provide recommendations
- Inform and refine user interface development
- Model testing, user comment and public feedback

IVI Research Projects

- Explore and prioritize research questions with IVI researchers during project planning phase
- Guide development of procedures and data collection instruments
- Review and comment on papers; reports, abstracts, articles, as appropriate

Strategic Decision Making

- Patient representatives invited to the Strategic Advisory Panel (SAP) offer input and support to IVI strategic goal setting
- Patient stakeholders contribute perspective that can inform IVI decision making and goal setting
- Ensure that decisions consider and acknowledge the perspectives of patients
- Provide feedback on patient partnership activities and IVI performance for process improvement

2.2 IVI Principles of Patient Partnership

Principle 1: IVI will involve multiple patient stakeholders in value assessment.

Consistent, thoughtful and respectful partnership with patient stakeholders helps IVI to conduct research that accounts for diverse patients and patient experiences and is relevant at both the individual and population levels. As a guiding principle, IVI is committed to establishing processes that involve patient stakeholders and contributors at the exploration phase of research design, and considering the involvement of multidisciplinary stakeholders for each project undertaken.



Actions:

- **Document IVI processes for patient partnership.**
- **Involve individual and group stakeholders at the inception of a research project idea.**

Principle 2: IVI will employ robust methods to identify and involve patient stakeholders.

At IVI, we identify patient partners for potential collaboration in multiple and deliberate ways.

Actions:

- **Involve patient stakeholders who are as similar as possible to the community or population in which the project results are intended to be applied.**
Engaging these stakeholders and caregivers or support partners who have personal experience and familiarity with the therapeutic area is critical to successful collaboration.[6, 11]
- **Partner with patient groups that have an interest in learning about value assessment.**
We are actively working to build broad alliances with patient organizations and relevant professional associations who are interested in learning about IVI's approach and processes, engaging with us on design and application of methods for future engagement, and establishing a wide base of dialogue about value assessment.
- **Identify stakeholders using multiple methods.**
We will actively conduct outreach with disease disease-focused stakeholder organizations and with patient-focused organizations representing the values and experiences of patients, as well as families, caregivers and other relevant stakeholders. We will also conduct outreach to IVI member organizations, researchers, and provider communities for connection to patient organizations. We will reconnect with collaborators or partner organizations with whom we've previously worked to access their networks. Finally, we will provide public notification for exploratory dialogue about a disease category to solicit interested parties and conduct social media outreach via websites and other mechanisms to seek contributor candidates.

Early outreach and identification of diverse voices from the patient and patient/family/caregiver community is essential to IVI achieving inclusion that is not only based on number of patient stakeholders, but also a reflection of a given community in terms of spectrum of disease severity, socio-demographic considerations and geographic representation. In addition, IVI's focus is on developing lasting relationships with multiple patient organizations to ensure that we build on best practices in patient collaboration, that we continually evaluate and improve these processes and that we demonstrate our mission through our actions.

Principle 3: IVI will partner with stakeholders at all stages of research.

Actions:

- **Preparatory Stage**
Prior to each strategic initiative or project, we will incorporate a period of pre-engagement with patient stakeholders as articulated by Mullins et al.[5] During the pre-engagement stage, patient stakeholders will be invited to provide input on the relevance and priority of topics for the patient population.

Once a topic or research project has been identified, the pre-engagement stage may also include formulating questions, identifying and selecting interventions, outcomes, and questions of high priority for patients, and identifying new patient partners.

- **Research Execution Stage**

As an initiative or project is launched, patient stakeholders will be involved in project design and implementation. Ideally, this engagement will span the life cycle of the project and include framing of the questions and study design, input into a protocol and data collection approaches, model development, and the review, interpretation, and translation of results.

Following is a list of the many different ways patient stakeholders may participate as partners, collaborators, and consultants, contributing to IVI work. Specific activities in this phase may vary with the project, but will include one or more of the following:

- Review protocol and technical design documents relevant to the project
- Provide insight into disease experience, treatment priorities and preferences
- Refine study questions, methods, protocols
- Inform selection of data elements, comparators, attributes by participating as moderators, subjects or respondents in research studies (, e.g. focus groups, in-depth interviews, online surveys) and advising on best approaches for patient recruitment and data collection
- Assist with recruitment of patient research partners and research subjects/respondents through social media
- Assist with dissemination of research results via social media
- Provide input through advisory councils
- Provide input through online patient forums to provide feedback
- Analyze and interpret findings
- Determine particular relevance or applicability of findings for patients

- **Translational Stage**

In the translational stage, patient stakeholders will provide guidance on dissemination and real-world application of project or initiative findings. Specifically, patient stakeholders may participate as partners, collaborators, or consultants in the following translational activities:

- Provide guidance on plans for results dissemination
- Dissemination of results
 - Share process and outcomes with patient audiences
 - Write articles or similar aimed towards patient group(s) and for use in social media communications
 - Write statements or deliver presentation via conferences, social media, etc.
- Communication of results to stakeholder communities
 - Facilitate engagement of other patients
 - Help patient communities to understand findings.

In summary, through the three primary stages, from pre-engagement to translation, patient stakeholders will help IVI to include important elements of value that reflect patient priorities in the science of value assessment with the beliefs, goals, and perspectives of patients.



Principle 4: IVI will continuously evaluate and refine patient partnership action

As a first step towards measuring the degree to which our processes are patient centric, we will explicitly document patient engagement and patient centricity in two ways.

Actions:

- **We will align our work as closely as possible to the NHC Patient Centered Value Model Rubric and will work towards alignment by continuously assessing our progress against the rubric benchmarks.**

The rubric describes six domains of a patient-centered value framework, and within each, how patient engagement and patient centeredness can be operationalized in value framework processes.^[12]

We will map our activities against the rubric in order to measure indicators of engagement. Mapping activities to date have been conducted internally, however for future assessments we will explore the feasibility of ongoing third party program evaluation. For example, Table 2 in Appendix B details how patient engagement activities for the OSVP IVI-Rheumatoid Arthritis model activities aligned with the NHC Patient Centered Value Model Rubric.

- **We will conduct post engagement assessments and evaluations (to be made public) to measure the impact of patient collaboration on IVI output.**

We will survey IVI researchers, IVI leadership, and patient stakeholders on their perspectives and experiences of collaboration. These assessments will aid in the identification and development of a key set of outcomes and the impact of incorporating patient centric approaches in IVI.

These assessments will also help to explicitly document in what ways IVI collaborated with patients, how patient stakeholders viewed the collaborations, and most importantly the impact of patient collaboration on IVI initiatives.

For example:

- Were patient preferences successfully integrated into the model or project?
- What worked or did not work about the collaboration?

Essentially, we seek to build in a feedback loop for not only how we do patient engagement in IVI, but also how we monitor and evaluate our collaborations and partnerships with patient stakeholders. This will be a dynamic and ongoing process.

- **To evaluate our patient partnership activities, we will implement a self-report instrument developed by PCORI and Academy Health based on PCORI's conceptual model for patient-centered outcomes research [13, 14], and adapted for use in IVI.**

The sample instruments will be completed by IVI researchers and patient stakeholders, and are detailed in Appendix C, Tables 3 and 4.

Evaluation results will be reviewed internally and with the Strategic Advisory Panel to inform continuous improvement of outreach and research project processes and to measure fidelity to the NHC rubric. In addition, we will disseminate aggregate results on a semi-annual basis to



demonstrate transparency of our process and to contribute to wider education and engagement with patients in future IVI work. Results will be shared with all stakeholders and posted on the IVI website.

Principle 5: IVI will commit resources for continuous engagement

Both a review of the literature and recommendations by the Working Group underscored the importance of continuous stakeholder engagement. Maintaining continuous engagement requires a resource commitment on the part of IVI.

Actions:

- **IVI will devote the resources necessary to ensure engagement with patient partners.**
IVI recognizes that consistent and robust patient engagement is resource and time intensive. We are committed to demonstrating commitment and investment through the following actions:
 - Articulate and offer partnership opportunities early in the initiative development,
 - Clarify desired inputs, contributions and expectations of patient partners
 - Identify supports, resources and technologies that will be provided to support patient involvement in IVI work;
 - Foster equality by ensuring that compensation rates for patient stakeholders are equitable and of fair market value, e.g. similar to compensation provided by other organizations or research entities.
 - Provide materials to patient stakeholders well in advance of any activity and allow sufficient time for review and comment, with deadlines for feedback
 - Accept and document all contributions and provide rationale for the use of such input or its omission from final work product (if applicable)
 - Be transparent and create transparent processes

3. The Road Ahead

IVI recognizes that our work represents a new frontier in value assessment. We acknowledge that early efforts, while well intentioned, have demonstrated a need for more formal processes and structure to operationalize our vision for patient partnership. Using established frameworks and metrics as our guide, we are establishing our processes to fully incorporate the patient perspective in our work. Based on this document, we plan to develop the following components of our patient partnership program:

- One page statement of IVI Partnering with Patients: Principles and Commitments
- Statement of Patient Roles in IVI Research Projects (Types of Contributions, Expectations, Supports Provided)
- Process for Identifying and Selecting Patient Participants
- Checklist of Patient Partnership Actions – for Internal Project management
- Process Documents for each project, including
 - Goals Statement for Project
 - Defining Patient Roles and Contributions
 - Partnership Agreement
 - Patient Participation Activities (focus groups, interviews, surveys, activities)



- Timelines, including Comment Periods (definitions, draft protocol, findings)
- Model Development/Research Process
- Public Comment Period and Feedback
- Dissemination

4. Acknowledgements

4.1 Working Group Membership

These Principles were developed by IVI staff, with oversight by the IVI Strategic Advisory Panel and based on critical input and guidance provided by the following members of the Working Group for Patient Engagement:

- Alan Balch, PhD Chair (Patient Advocate Foundation)
- Randy Burkholder (PhRMA)
- Thomas Concannon, PhD (RAND)
- Sachin Kamal-Bahl, PhD (Pfizer)
- Sarah Krug (Cancer101)
- Michael Millenson (Health Quality Advisors LLC)
- W. Benjamin Nowell PhD, MSW (Global Healthy Living Foundation)
- Neil Weissman, MD (MedStar Health Research Institute)
- Carole Wiedmeyer (Patient Advocate)
- Stephen Yates, PhD (UCB Biosciences)

We gratefully acknowledge the Working Group Members for sharing their expertise and perspectives in the development of this framework.

4.2 Funding

This activity was funded by the Innovation and Value Initiative, a collaboration among thought leaders in academia, patient advocacy organizations, payers, life science firms and pharmaceutical manufacturers, providers, delivery systems and other organizations dedicated to preserving innovation, value, and choice in the health care system. IVI receives funding from multiple sources, including membership organizations representing the life sciences industry. More details about IVI's funding can be found on the IVI website at www.thevalueinitiative.org.

5. About the Innovation and Value Initiative (IVI)

The Innovation and Value Initiative (IVI) was created to raise the level of discussion regarding value in healthcare and find common ground in the approach to measuring value. Our mission is to improve the way value is measured and rewarded in the healthcare system. Our goal is to promote the development and use of high value interventions that advance health. In pursuit of our goal, IVI designs, conducts, and disseminates research reflecting five key principles:

SCIENTIFIC RIGOR: IVI uses contemporary scientific methods to rigorously derive actionable insights directly from both clinical and real-world evidence.



TRANSPARENCY: IVI develops open-source tools to promote value-based decision making in healthcare. To facilitate the evaluation and replication of our work, IVI will publicly release research methods, assumptions, results, and external reviews.

PATIENT-CENTERED APPROACH: IVI engages patients as active participants in defining priorities for and developing models of value assessment. Our research attends to diversity across patients in their clinical characteristics, preferences, and other circumstances.

INNOVATION: IVI examines how new treatment interventions and government policies affect the well-being of society and its stakeholders. We promote approaches that rapidly incorporate the latest scientific evidence in a changing healthcare landscape.

MARKET-BASED SOLUTIONS: IVI aims to promote better value-based decision making in the decentralized U.S. healthcare marketplace.

IVI was established in 2016 and is organized and staffed by Precision Health Economics, a health economics consultancy. Precision Health Economics provides IVI's direction and research agenda are determined in collaboration with its Strategic Advisory Panel, which includes representatives from patient advocacy organizations, pharmaceutical firms, academia, insurers, and health systems. All funding supports IVI's overall activities, with no funding or funder tied to specific activities or research projects.

6. References

1. *Partnering with Patients to Drive Shared Decisions, Better Value, and Care Improvement: Workshop Proceedings*. 2013, Institute of Medicine Washington, D.C.
2. Newcomer, L.N., et al., *Changing physician incentives for affordable, quality cancer care: results of an episode payment model*. *J Oncol Pract*, 2014. **10**(5): p. 322-6.
3. *Memorial Sloan Kettering Drug Abacus*. 2016 [cited 2016; Available from: <http://www.drugabacus.org/>].
4. Carman, K., et al., *A Roadmap for Patient and Family Engagement in Healthcare Practice and Research*. 2014, American Institutes for Research.
5. Mullins, C.D., A.M. Abdulhalim, and D.C. Lavalley, *Continuous patient engagement in comparative effectiveness research*. *Jama*, 2012. **307**(15): p. 1587-1588.
6. Garces, J.P.D., et al., *Eliciting Patient Perspective in Patient-Centered Outcomes Research: A Meta Narrative Systematic Review*. . 2012, Mayo Clinic: Rochester, MD.
7. Frank, L., et al., *The pcori perspective on patient-centered outcomes research*. *JAMA*, 2014. **312**(15): p. 1513-1514.
8. Gill, S., et al., *PANCREOX: A Randomized Phase III Study of Fluorouracil/Leucovorin With or Without Oxaliplatin for Second-Line Advanced Pancreatic Cancer in Patients Who Have Received Gemcitabine-Based Chemotherapy*. *J Clin Oncol*, 2016. **34**(32): p. 3914-3920.
9. Davidoff, A.J., et al., *Out-of-pocket health care expenditure burden for Medicare beneficiaries with cancer*. *Cancer*, 2013. **119**(6): p. 1257-1265.
10. Concannon, T.W., et al., *A new taxonomy for stakeholder engagement in patient-centered outcomes research*. *J Gen Intern Med*, 2012. **27**(8): p. 985-91.
11. *Workshop on Incorporating the Patient Perspective into Patient-Centered Outcomes Research Executive Summary*. 2012, Patient-Centered Outcomes Research Institute: Baltimore, MD.
12. Peretto, E.M., et al., *Value to Whom? The Patient Voice in the Value Discussion*. *Value in Health*, 2017. **20**(2): p. 286-291.
13. Frank, L., et al., *Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute*. *Quality of Life Research*, 2015. **24**(5): p. 1033-1041.
14. Forsythe, L.P., et al., *Patient and Stakeholder Engagement in the PCORI Pilot Projects: Description and Lessons Learned*. *Journal of General Internal Medicine*, 2016. **31**(1): p. 13-21.
15. Vinik, A., et al., *Patient-Reported Outcomes and Quality of Life with Sunitinib Versus Placebo for Pancreatic Neuroendocrine Tumors: Results From an International Phase III Trial*. *Target Oncol*, 2016. **11**(6): p. 815-824.
16. Seidman, J., et al., *Patient-Perspective Value Framework*. 2017.



7. Appendices

Appendix A: Literature Review

Overview

In developing our principles for patient partnership, IVI staff reviewed the literature detailing case studies and examples of patient engagement in PCOR and value assessment. These data, along with recommendations and guidance by Working Group members, were reviewed and adapted for use in this guidance document. [1, 2, 4, 5, 7-20] In addition to informing the development of content for the guidance document, we also sought to establish an understanding of the evidence in the literature to-date about patient engagement in research, including the identification of gaps in collaboration.

IVI staff conducted a targeted literature search using different search databases (PubMed, Google Scholar, and Google Search Engine) to capture key publications and reports of interest in both the peer-reviewed literature (i.e., observational studies, systematic reviews, and commentaries), and grey literature (industry publications). Additional publications were identified from the references cited in the publications and reports selected for review. In addition, reverse citation searches of articles and reports identified in the targeted literature search and their references were conducted. We excluded in our final literature review list publications and reports that were written in a language other than English, had no full-text article available (peer-reviewed journals only), or were published more than a decade prior to the search date. Thirty-one publications were selected for review, and where relevant are cited and detailed in the list of references.

Summary of Findings

In conducting the literature review, we identified peer reviewed publications and guidance describing best practices for partnering with patients in healthcare, from care delivery to research. In the healthcare research realm, much of what has been published has been spearheaded by PCORI-funded research and academic scholars who have been working on patient centered approaches in healthcare research. These publications outline what patient-centeredness in research really means and why it is important to implement and conduct patient-centered research. These studies also detail various approaches to patient engagement in research and how best to assess the impact of engagement. Augmenting this work, several organizations representing patient and industry perspectives have published frameworks and guidance for engaging patients in research. These include guidance by the National Health Council, the National Pharmaceutical Council, Avalere Health, and the American Institutes for Research in collaboration with the Gordon and Betty Moore Foundation.[33, 4, 15, 16]



Appendix B: Alignment with NHC Patient-Centered Value Model Rubrics

Table 2. OSVP IVI RA Model alignment with NHC Patient Centered Value Model Rubrics

Rubric	Domain	Subcomponent/Characteristics	Addressed in OSVP IVI-RA model	
Meaningful Patient Engagement in the Value Development Process	Patient Partnerships	Patients are recognized as partners in all aspects of model development process.	X	
		Patients are supported to enhance participation and capacity to engage.	X	
		Direct input is collected from wide range of patients through mixed methods.	X	
		Tools are available to patients to help them understand all of the model and to communicate the model to other patients.	Not currently applicable	
	Transparency to Patients	The process for selection of patient representatives is transparent.	To be developed	
		The patient community has early opportunities for review of and comment on model inputs, methods, and drafts through multiple venues (public meetings, online comments).	X	
	Patient-Centered Considerations in General	Inclusiveness of patients	Representatives from the patient community are involved throughout the process, as required or expected given the condition / population.	X
			Model results (data) are translated into usable and meaningful information for patients.	N/A
		Diversity of patients / populations	Diversity of the patient population is acknowledged and considered.	X
		Outcomes patients care about	Outcomes important to patients are identified and incorporated into the model (e.g., functional status, HRQoL, well-being, clinical measures, survival, productivity, goals/expectations/aspirations, financial stress).	X
Patient-centered data sources	Existing sources of patient-generated health data (e.g., patient registries or patient reported outcomes) are identified and considered.	To be developed		
Patient-Centered Considerations in General	Patient Partnerships	Rationale to substantiate the adequacy of the ratio of patients to non-patient participants is provided.	To be developed	
		Patients are engaged in pilot testing and refinement of the model.	X	
		Patients are engaged in providing technical assistance to model end-users on implementation.	X	



Rubric	Domain	Subcomponent/Characteristics	Addressed in OSVP IVI-RA model
		Patient engagement in the development process is evaluated, including an assessment of whether patient expectations have been met and if patients realize/see the impact of their engagement.	X
	Transparency to patients	The purpose and goals of the model are made clear to patients (including the intended audience and use) and are well-defined (includes caution on how the model should not be used).	X
		The desired outcome of using the model and its implications for patients are made clear.	X
		The methodology is made transparent to patients in a timely manner.	X
		All assumptions and inputs used are articulated in an understandable, patient-friendly way.	To be developed
		Inputs considered but not used are described with the rationale for exclusion that patients can understand.	To be developed
		Results of the model pilot test(s) are disclosed and subsequent refinements are clearly indicated so patients can understand the sequence.	X
		A clear distinction is made in public communications that are accessible to patients about the model development stage (e.g., undergoing pilot testing versus finalized).	X
		Developer responses to public comments are made public to allow the patient community to understand how its input has or has not been used.	X
		Processes for evaluating that the model performs as intended are transparent and patients can understand them.	To be developed
		Model evaluation considers if it is being used as intended, achieving intended outcomes, and assesses unintended consequences for patients.	To be developed
		Results of any evaluation are made public.	X
		Processes for updating and maintaining the model are clear and accessible to the patient community.	X
		Patient partners are acknowledged as contributors/authors to the process.	X
		All potential conflicts of interest are disclosed, including those of patient partners.	X
		All funding sources are publicly disclosed.	X

Rubric	Domain	Subcomponent/Characteristics	Addressed in OSVP IVI-RA model
	Inclusiveness of patients	A role for a medical ethicist is considered.	To be developed
		The draft model is vetted with a broad coalition of stakeholders, including patients.	X
		Patient partners are engaged to support the dissemination and implementation of the model.	X
	Diversity of patients/populations	Differences in patient perceptions of value, that shift over time as patient circumstances change, are acknowledged and considered (reflects expected stages over time).	X
		Applicability and limitations across patient subpopulations and disease trajectory are acknowledged and considered.	X
		Processes are included for identifying and incorporating new knowledge regarding patient subpopulations and disease trajectory.	X
	Outcomes patients care about	Economic inputs are considered in the context of a patient's experience.	To be developed
		Processes are in place for identifying and incorporating emerging information on outcomes of importance to patients.	X
	Patient-centered data sources	Data beyond randomized controlled trials are considered (e.g., natural history of disease, outcomes and/or treatments, patient preferences regarding outcome or treatment characteristics).	X
		Rationale for the inclusion or exclusion of available data sources is provided and information is provided in a patient friendly way.	X
		Processes are in place for identifying and incorporating emerging data sources, in particular patient-generated health data.	X

Appendix C: Surveys to Evaluate the Impact of Patient Partnership

Table 3. Evaluating the impact of patient partnership in IVI – *Researcher Survey*

Researcher Survey Questions	Response Options
Have you engaged patients or other stakeholders (e.g. advocates, caregivers) in your project in ways other than as research subjects?	<ul style="list-style-type: none"> • Yes • No
[IF YES] Which of the following patient stakeholder communities have you engaged in your project in ways other than as research subjects?	<ul style="list-style-type: none"> • Patient/Consumer • Caregiver/Family Member of Patient • Patient/Caregiver Advocacy Organization • Other (Please describe)
Please indicate the nature of their involvement.	<ul style="list-style-type: none"> • Stakeholder led • Collaborator • Consultant • Other
How many patient stakeholders have you engaged in your project?	Response options delineated by type of patient stakeholders as determined by project needs.
Please describe what worked well in establishing the relationship with this/these	Open
What challenges did you encounter in establishing the relationship with this/these patient stakeholder(s)?	Open
What was your primary motivation for engaging this/these patient stakeholder(s) in your project?	Open
What stage(s) in the project have you worked with this/these patient stakeholder(s)? CHECK ALL THAT APPLY	<ul style="list-style-type: none"> • topic solicitation/agenda setting • question development/framing • proposal development • methods/study design • data collection • data analysis • results review/interpretation/translation • dissemination • other (please specify)
How frequently did you engage with this/these patient stakeholder(s) throughout the project?	<ul style="list-style-type: none"> • fewer than 4 times a year • once every 2-3 months • approximately once a month • 2-3 times a month • more than 3 times a month • once a week
Based on your interactions with patient stakeholder(s), what learnings can you offer to others about collaborating with patient stakeholders in IVI work?	Open

Researcher Survey Questions	Response Options
What have been the most significant contribution(s) made by patient stakeholder(s) to your work?	Open
What challenges have members of your team experienced in collaborating with patient stakeholder(s), if any?	Open
Is there anything else you would like to share or think we can do to improve our collaborations with patient stakeholders?	Open

*Adapted from Forsythe et al

Table 4. Evaluating the impact of patient partnership in IVI – *Patient Stakeholder Survey*

Patient Stakeholder Survey Questions	Response Options
Please indicate the nature of your involvement in the IVI initiative or project.	<ul style="list-style-type: none"> • Stakeholder led • Collaborator • Consultant • Other
How many IVI projects or initiatives have you been involved with to date? Please specify which projects you have been involved in.	Open
From your perspective, what went well in your collaboration(s) with IVI staff and/or leadership?	Open
What challenges did you encounter in establishing a collaboration with this/these individual(s)?	Open
What was your primary motivation for participating in the IVI project/initiative?	Open
What stage(s) in the project/initiative were you involved in?	<ul style="list-style-type: none"> • topic solicitation/agenda setting • question development/framing • proposal development • methods/study design • data collection • data analysis • results review/interpretation/translation • dissemination • other (please specify)
How frequently did you engage with this/these individual(s) throughout the project?	<ul style="list-style-type: none"> • fewer than 4 times a year • once every 2-3 months • approximately once a month • 2-3 times a month • more than 3 times a month • once per week
Based on your interactions with IVI, what recommendations can you offer to IVI about collaborating with patients on IVI work?	Open
What do you think were your most significant contribution(s) to IVI work?	Open
What challenges have you experienced in collaborating with IVI researchers, if any?	Open
Is there anything else you would like to share or think we can do to improve our collaborations with patient stakeholders?	Open
Would you recommend working on IVI future projects to other patients like you or to patient organizations interested in value assessment?	Yes/No

*Adapted from Forsythe et al