

2021 METHODS SUMMIT

PROCEEDINGS REPORT



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

March 2022

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

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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.



OVERVIEW

Inclusion of experiences 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 convened the 2021 Methods Summit as a multi-day, virtual event (on October 27th, October 28th, and November 4th) to drive consensus on what matters most to patients in their health and health care and how to measure it. The Methods Summit public meeting brought rich discussion from more than 170 stakeholders, including patient leaders, payers, researchers, and providers through panel conversations and stakeholder comments.

As the data generated by our complex health care system rapidly increases, it is important to understand what matters to patients and what data we need to truly inform patient-centered decision-making. The 2021 Methods Summit explored current gaps in patient experiences and decision-making processes to further our understanding of why incorporating patient perspectives is important.

Key points:

- **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 healthcare 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:** Comparative effectiveness research (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.



METHODS SUMMIT FOCUS

To transition to an equitable value-driven health care system, patient perspectives — including patient preferences, patient-reported outcomes, and impacts — must be incorporated in patient-centered outcomes research and value assessments.

EVENTS

IVI convened the 2021 Methods Summit as a multi-day, virtual event to drive consensus on what matters most to patients in their health care and explore how to measure value from the patient perspective. Inclusion of inputs 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 yields credible and relevant insights to inform decisions.

We talk about value-based payment and value assessment of interventions as different things, but where do they overlap? How should we think about tying the two together in measure development and application? - IVI

Steering Committee Meetings

A 22-member Steering Committee held its first (five total) meeting on June 23, 2021 to inform the planning and analysis of feedback from stakeholders. Members of the committee included patient representatives, employers, researchers, payers, and health system representatives. A roster of the Steering Committee members is included in the Appendix of this report. The diverse perspectives included in the group resulted in highly engaged discussions about the key issues to be addressed, areas for consensus building to prioritize in agenda design, and strategies to engage stakeholders.

We can collect data from all these sources, but still not be collecting the right things if we don't ask patients what is important to them. We could still end up in a sea of meaningless data if we don't get that upfront piece right.
- Patient Leader

Public Meeting

IVI held the first virtual event of the Methods Summit on October 27, 2021, which brought rich discussions from over 170 participants through panel conversations and stakeholder reactions. In total, 25 patient organizations were represented, as well as national and federal partners including Centers for Medicare and Medicaid Services, FDA, and National Quality Forum, among others (see Page 7 of this report for the full list of panelists).



Jennifer Bright

Innovation and Value Initiative



Rick Chapman

Innovation and Value Initiative

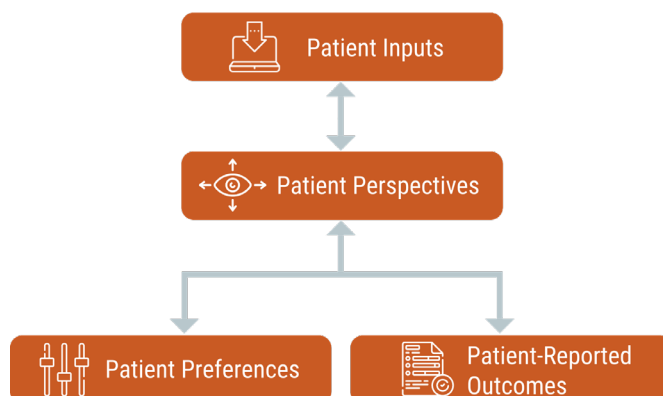
Opening Sessions Include IVI and NQF Leadership

The Public Meeting event included an opening session by IVI leadership — Jennifer Bright, Executive Director (above), and Rick Chapman, Chief Science Officer (above) — that offered an overview of the purpose and intended outcomes of the events of the Methods Summit. This was followed by a keynote address by Dr. Dana Gelb Safran, Chief Executive Officer of National Quality Forum (NQF). Dr. Safran offered a perspective on the imperative of incorporating patient-centered outcomes data for use in healthcare decision-making. She also provided comments on the strategic direction of NQF in driving change in healthcare measurement.

Panels Highlight Patient Leaders, Payers, and Methods Experts

Three panel presentations followed that incorporated the perspectives of patients and patient leaders, public and private payer groups, employers, researchers, and value assessors on the current gaps and challenges in capturing patient perspectives and. Highlights of these panel discussions are included in the next section of this report.

Figure 1. Hierarchy of Patient Inputs.



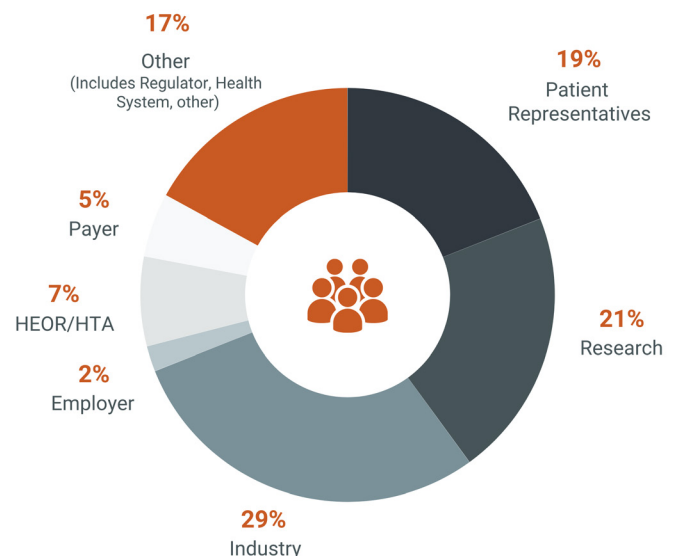
FDA Final Patient Preference Guidance Document. [Available Online](#). (adapted)

Small and Large Group Dialogues

In order to seed robust discussion and analysis of the feedback shared by panelists during the event, three breakout group discussions were held on October 28, 2021. These small group conversations used key themes from the prior day to prioritize areas for impact. The small diverse groups included patient and caregiver representatives, employers, payers, health systems, and researchers.

IVI processed the feedback gained over both days of events and prepared the final stakeholder discussion on November 4, 2021. Over 45 experts gathered to develop Key Principles to better incorporate patient perspectives in health care decision-making.

Figure 2. Profile of IVI Methods Summit Participants



The success of the patient often depends on the family caregivers, but often they're not even identified. Plus the family caregivers' health and employment is often at risk... Is this being considered for inclusion in these measures?
- Patient Leader

Key Takeaways

Patient Panel

Patient representatives shared the following key takeaways:

- Patients and caregivers are at the center of managing their diseases and care, but are often left out of the entire data reporting and value determination process.
- Patient community-led surveys are demonstrating approaches to capturing patient-centered impacts that are not being captured now, including loss of work productivity or income due to treatment, costs of treatment, and time spent navigating complex systems.
- Claims data is an incomplete data set for assessing value in healthcare because what matters to patients is what is happening at home, at work, or in the community.
- The burden of participation is being placed on patients and patient advocates at this time to ensure the patient experience is integrated into measurement and value assessment.

"The cost of at-home treatments, non-prescription treatments (including holistic therapy), and lack of productivity including enjoyment of life TRULY add up, but are not adequately considered as part of the 'value' of therapy. I see a disconnect as well between what patients say matters and what is perceived as the value of therapy...How do we close that gap to ensure regulators are more willing to measure effectiveness based on how that intervention improves life?" - Patient Leader

Payer Panel

Panelists shared innovations they are leading to build the data ecosystem and discussed the challenges they face in obtaining access to comprehensive data, beyond just claims data. Comments from payer representatives reflected that data currently used for decision-making does not reflect the full range of impact to patients.

"Digital therapeutics, as we look to cover them more and understand their value, I'm looking at that piece of the puzzle is actually solving maybe the puzzle of collecting patient-reported outcomes in a way that we can get that information into the healthcare space." - Payer

Methods Panel

Discussions across stakeholders reflected the critical need to define patient impacts (such as financial burden) and measures to capture them in patient-centered outcomes research/comparative effectiveness research (PCOR/CER) and value assessment, but also highlighted the research that is needed to move this into practice. One researcher commented in response to the question on priority patient inputs:

"...My first reaction to this question is, these things don't exist. They haven't been found. And there are a lot of people out there who say they do exist, but they haven't actually been properly tested to do what it is that we're expecting these measures to do...And so I just think that there's a lot of proper development and testing that really needs to be done before we can just say, 'Oh, let's use these three and run with them.'" - Researcher

In addition, comments shared the need for stakeholder discussion about the balance between the need for rigor of research methods and patient-centricity.

Methods Summit Panel Speakers



Hugo Campos

Stanford Medicine X



Nathaniel Counts

Mental Health America



Pat Gleason

Prime Therapeutics



Juan Marcos Gonzalez

Duke University



Erin Holve

Washington D.C. Department of
Health Care Finance



Maggie Jalowsky

Sick Cells



Annie Kennedy

EveryLife Foundation



Marjana Marinac

JDRF



Cara Nikolajski

UPMC Center for High-Value
Health Care



Ilisa Halpern Paul

District Policy Group



Margaret Rehayem

National Alliance of Healthcare
Coalition Purchasers



Upal Basu Roy

LUNgevity



Dana Gelb Safran

National Quality Forum



Shana Traina

Janssen

KEY PRINCIPLES

Incorporating Patient Priorities in Patient-Centered Outcomes Research/Comparative Effectiveness Research (PCOR/CER) and Value Assessment

IVI has drafted nine Key Principles based on input from the committee. These principles are intended to inform action by IVI, PCORI, and other stakeholders in how patient communities are engaged in prioritizing patient-centered impacts to measure and develop methods that incorporate patient perspectives into decision-making.

The key principles defined by the invited discussants fell into two domains:

1. Raising the leadership profile of patients and patient communities in defining meaningful impacts that must be measured early and throughout an intervention's life-cycle; and
2. Improving methods and metrics that can be specifically included in developing PCOR/CER and value assessment resources to guide regulatory and payer decision-making.

Under these two domains, nine Key Principles were defined and form the primary outcome of the convening. The Key Principles outline how researchers and value assessors can raise the leadership profile of patients and patient communities in defining meaningful impacts that should be measured, and how these entities can improve methods and metrics for use in PCOR/CER research and value assessment to inform decision-making.



DOMAIN 1

Elevate Visibility and Policy Support for Patient-Centered Impacts as an Essential Element in Assessing Value and Facilitating Access



DOMAIN 2

Incorporate Patient Impacts and Perspectives in Regulatory and Payer Decision-Making by Improving Measures and Methods

Value Added



1



Elevate Visibility and Policy Support for Patient-Centered Impacts as an Essential Element in Assessing Value and Facilitating Access



Prioritize and define patient-centered impacts and research questions as early as possible in comparative effectiveness research and value assessment. Patients' lived experiences, goals, unmet needs, and patient-important impacts (including clinical outcomes and quality-of-life implications) must be primary drivers of key research at all phases: pre-clinical, clinical efficacy and safety, risk-benefit analysis, post-market comparative effectiveness, and patient-centered outcomes research.



Share data and synthesis of such data collected as part of comparative effectiveness research or value assessment back with patients in a timely and actionable manner. Whether in clinical trials, patient-reported outcome measurement, patient preference research, patient goal definition and attainment, or through patient experience surveys and wearables, the capture of patient data is increasingly robust across the healthcare system. These data stand to benefit patients themselves, providing insights into their health and quality-of-life impacts individually and in the context of others. A learning health system will ensure that patient-derived data and the insights they generate are shared back with contributing patients.



Comprehensively include and fairly compensate patient leadership, perspectives, and expertise (scientific, data collection, lived experience) in PCOR/CER and value assessment projects. Research at every stage of an intervention's development should involve patient collaborative leadership at inception, including in research question development, design, recruitment, and analysis. This ensures that the patient community's lived experience is embedded in the development and collection of evidence. Following on PCORI's model and other examples of best practices in patient engagement, researchers should demonstrate shared leadership and also evidence of compensation for such contributions.



Establish standards and accountability that increase transparency for how patient inputs are considered and incorporated in decision-making. Until explicit metrics and accountability exist for researchers, innovators, healthcare systems, and value assessors in the healthcare industry, the achievement of patient-centricity and inclusion of patient data across decision-making will continue to be inconsistent, under-resourced, and undervalued. Moreover, increased transparency and accountability metrics will reduce the considerable burden currently placed on patients and patient communities to design research, build funding for, and advocate for use of the experience and expertise of patients and their families. Increased consideration of patient input can encourage the direction of more resources towards research for the elicitation of patient information, for example.

We should not confuse patient-reported data with patient-important data. Just because patients report it doesn't mean it's meaningful to them. We have to move beyond thinking about patient-centered value assessment ONLY in terms of data. - IVI

2



Incorporate Patient Impacts and Perspectives in Regulatory and Payer Decision-Making by Improving Measures and Methods



Create measures and methods to capture costs and other burdens to patients/families as a result of their health or healthcare. Patients, families, and caregivers experience impacts associated with one or co-occurring health conditions, and the treatments for those conditions, that are often not measured in clinical research or PCOR/CER. Consistent measures for these patient impacts must be identified and developed so that an evidence base can be built, adopted, and disseminated. Examples include, but are not limited to, financial impacts related to transportation, non-clinical treatments, wraparound services and supports, lost wages/lost days of work, and similar impacts on caregivers.



Identify the purpose of measurement within comparative effectiveness research or value assessments for specific decision contexts and address the tension between the utility of condition-specific and cross-cutting measures by investing in both areas of measurement development. Improvement in measuring patient-centered impacts in specific disease conditions (e.g., rare diseases or cancer) and more general impacts across health conditions (e.g., pain, fatigue, functioning) are both important to improving relevance of data that informs decision-making about access, value, and use. Prioritizing advancement in both areas is challenging given resource scarcity and the burden on patient communities, as well as the ability to define metrics that meet the purposes of cross-disease comparison. Finding a balance fit for different objectives and identifying priority areas for development can lay the groundwork for developing the methods, engagement, and resources needed for implementation.



Increase transparency in data collection and consistency in data management with sharing of patient-centered data as a “public good.” To create mutual benefit and trust in the co-development of patient-centered data and its uses for decision-making on clinical efficacy and safety, access, and outcomes measurement, such data should be shared and made accessible in a manner that is relevant to the end user, including patients and caregivers, employers, regulators, and payers. For these data to be broadly applicable and provide the most value, standards for transparent and consistent measurement and data collection must be developed and applied across contexts.



Define and standardize meaningful measures of health-related social needs that impact patient-centered outcomes and address health disparities. Stakeholders should recognize that factors outside the healthcare system may interact with healthcare interventions and delivery to have important impacts on patients. Ongoing efforts to define and standardize meaningful measures of patient-centered outcomes must also include development of best practices in identifying and measuring these social needs (e.g., food and housing security).



Invest in the science of decision analytic methods and workforce training to support the research, patient, regulatory, and payer communities in collecting, interpreting, and incorporating patient-important impacts into decision-making. In addition to investment in patient leadership to define impacts and experience important to healthcare decision-making, policy-makers, healthcare systems, and other stakeholders should direct resources to invest in the skills and expertise to collect, analyze and incorporate such inputs so that they can be used in real-world decision contexts.

People don't think of themselves as diseases, they see themselves as a whole person with life goals and preferences and interests and passions and needs. So disease-specific outcomes and measures have always been an ill-fit from the patients' perspective. Lived experience can help show the commonalities in preferences and meaningful outcomes regardless of health conditions. - Researcher

Acknowledgments

The Innovation and Value Initiative gratefully acknowledges the contributions of the Steering Committee members and our facilitator, Ms. Ilisa Halpern Paul, President of the District Policy Group at Faegre Drinker Biddle & Reath LLP, in the success of the IVI Methods Summit. IVI appreciates the support provided for the project through a Patient-Centered Outcomes Research Institute® (PCORI) Eugene Washington PCORI Engagement Award (#EAIN-21156).

Appendices

- A. Steering Committee Roster
- B. Large and Small Group Participant List
- C. Agenda

Appendix A

Steering Committee Roster

2021 IVI Methods Summit Steering Committee

| Steering Committee Members | | |
|--|--|---|
| Name | Title | Organization |
| Arturo Cabra, MsC, Bec | Associate Director, Scientific and Health Policy Initiatives | ISPOR |
| Ashley Valentine, MRes | Co-Founder & President | Sick Cells |
| Barry R. Liden, JD | Vice President, Patient Engagement | Edwards Lifesciences |
| Eleanor Perfetto, PhD, MS | Executive Vice President, Strategic Initiatives | National Health Council |
| Erin Holve, PhD, MS | Director, Health Care Reform and Innovation Administration (HCRIA) | DC Department of Healthcare Finance (DHCF) |
| Jessica Brooks Woods, CEO, MPM, PHR | President and CEO | Pittsburgh Business Group on Health |
| Jon Campbell, PhD, MS | Senior Vice President, Health Economics | Institute for Clinical and Economic Review (ICER) |
| Juan Marcos Gonzalez, PhD | Associate Professor, Population Health Sciences Assistant Professor, Medicine Member, Duke Clinical Research Institute | Department of Medicine, Duke University School of Medicine |
| Kristi Mitchell, MPH, AB | Senior Advisor | Avalere |
| Lizheng Shi, PhD, MsPharm, MA | Endowed Regents Professor, Interim Chair Director, Health Systems Analytics Research Center | Tulane University School of Public Health and Tropical Medicine |
| Maggie Jalowsky | Director, Advocacy | Sick Cells |
| Margaret Rehayem | Vice President | National Alliance of Healthcare Purchaser Coalitions |
| Margo Edmunds, PhD | Vice President, Evidence Generation and Translation | AcademyHealth |
| Michael Stancil, MPS, ASCA | Chief of Staff Director, Marketing | Pittsburgh Business Group on Health |
| Patrick Gleason, PharmD, FCCP, FAMCP, BCPS | Assistant Vice President, Health Outcomes | Prime Therapeutics |
| Simu Thomas, PhD, MS | Vice President, Global Head Health Economics & Outcomes Research | Alexion Pharmaceuticals |

| IVI Staff and Partners | | |
|---------------------------|--|--|
| Name | Title | Organization |
| Jennifer Bright, MPA | Executive Director | Innovation and Value Initiative |
| Rick Chapman, PhD | Chief Science Officer | Innovation and Value Initiative |
| Melanie Ridley | Director, Development and Partnerships | Innovation and Value Initiative |
| Judy Thomas | Operations Manager | Innovation and Value Initiative |
| Ilisa Halpern Paul, MPP | President, District Policy Group | Faegre Drinker Biddle & Reath LLP |
| Andrew Hu, MPP (observer) | Director, Public Policy and Government Relations | Patient-Centered Outcomes Research Institute (PCORI) |

Appendix B

Large and Small Group Participant List

| Participant Name | Organization |
|----------------------|--|
| Morenike Ayo-Vaughan | Commonwealth Fund |
| Lindsey Bandini | National Comprehensive Cancer Network |
| Linda Blount | Black Women's Health Imperative |
| John Bridges | Ohio State College of Medicine |
| Jennifer Bright | Innovation and Value Initiative |
| Arutro Cabra | ISPOR |
| Jon Campbell | ICER |
| Hugo Campos | All of Us Research Program Stanford Medicine X |
| Kristin Carman | PCORI |
| Rick Chapman | IVI |
| Joseph Clift | Centers for Medicare and Medicaid Services (CMS) |
| Nathaniel Counts | Mental Health America |
| Erica de fur Malik | Innovation and Value Initiative |
| Margo Edmunds | AcademyHealth |
| Patrick Gleason | Prime Therapeutics |
| Juan Marcos Gonzalez | Duke University School of Medicine |
| Jennifer Graff | Association for Managed Care Pharmacy |
| Erin Holve | Washington, D.C. Department of Health Care Finance |
| Leah Howard | Psoriasis Foundation |
| Andrew Hu | PCORI |
| Tom Hubbard | NEHI |
| Maggie Jalowsky | Sick Cells |
| Anusha Kheir | Amgen |
| Barry Liden | Edwards Lifesciences |
| Mark Linthicum | Innovation and Value Initiative |
| Marjana Marinac | JDRF International |
| Kristi Mitchell | Avalere / Health Equity Outcomes |

| Participant Name | Organization |
|-----------------------|--|
| Jonathan Moore | PCORI |
| Cheryl Neslusan | Janssen Pharmaceuticals |
| Cara Nikolajski | UPMC |
| Ilisa Halpern Paul | District Policy Group |
| Eleanor Perfetto | National Health Council |
| Eva Powell | Alliance of Community Health Plans |
| Margaret Rehayem | National Alliance of Healthcare Purchaser Coalitions |
| Melanie Ridley | Innovation and Value Initiative |
| Murray Ross | Kaiser Permanente |
| Upal Roy | LUNgevity |
| Julia Slejko | University of Maryland, PAVE Center |
| Simu Thomas | Alexion Pharmaceuticals |
| Judy Thomas | Innovation and Value Initiative |
| Shana Traina | Johnson & Johnson |
| Aaron Turner-Pfieffer | JDRF International |
| Danny van Leeuwen | Health Hats |
| Kimberly Westrich | National Pharmaceutical Council |
| Ruth Wetta | Cerner |
| Tatia Woodard | Pfizer |
| Richard Xie | Innovation and Value Initiative |
| Chuck Yonan | Neurocrine Biosciences |

Appendix C

Agenda

2021 IVI Annual
METHODS SUMMIT

FALL 2021 | VIRTUAL EVENT

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

October 27, 2021 | IVI Methods Summit - Public Meeting

[Registration Information](#)

Purpose: Level knowledge and framing of the key questions, as well as establishing awareness of initiatives and progress made to date in the incorporation of patient perspectives in comparative effectiveness research and value assessment. **This session is open to the public.**

10:00 am ET **Introduction and Framing of the IVI Methods Summit**

Jennifer Bright, Executive Director and Rick Chapman, Chief Science Officer, Innovation & Value Initiative (IVI)

10:10 am **Keynote Address**

Dana Gelb Safran, Sc.D., President & CEO, National Quality Forum

Offers perspective on the central role of outcomes measurement to the sustainability and success of value based payment, and will share a model for accelerating progress toward the next generation of measures to advance improved outcomes, equity, patient-centeredness and affordability.

10:25 am **Taking the Next Leap: What Have We Learned and Where Can Including Patient Perspectives Have Impact?**

In a fireside chat, patient representatives will discuss real-world case examples as a reference point for demonstrating the complexity and impact of patient perspectives in healthcare design and decision-making.

Moderator: Annie Kennedy, EveryLife Foundation for Rare Disease

Panelists:

- Maggie Jalowsky, Sick Cells
- Nathaniel Counts, Mental Health America
- Marjana Marinac, JDRF International
- Hugo Campos, All of Us Research Program Stanford Medicine X

11:15 am **Question & Answer Session**

11:25 am **Break**

11:35 am **How Payers, Purchasers, and Policymakers Can Incorporate Patient Priorities Into Research and Value Assessment**

The reactor panel will feature a facilitated discussion from stakeholders that develop and use patient-generated data and perspectives. Using the case studies as a reference point, panelists will explore both barriers and opportunities for alignment and change.

Moderator: Ilisa Halpern Paul, District Policy Group, Faegre Drinker Biddle & Reath, LLP

Panelists:

- Erin Holve, DC Department of Healthcare Finance
- Patrick Gleason, Prime Therapeutics

12:15 pm **Question & Answer Session**

12:25 pm **Break**

2021 IVI Annual
METHODS SUMMIT

FALL 2021 | VIRTUAL EVENT

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

October 27, 2021 | IVI Methods Summit - Public Meeting (continued)

12:55 pm

How Do Methods Align with Patients?

This panel will feature a facilitated discussion with stakeholders who conduct and use research on patient perspectives to emphasize areas of work in methods, including issues of rigor, novel methods, and fit for purpose in decision making.

Moderator: Rick Chapman, Innovation & Value Initiative (IVI)

Panelists:

- Cara Nikolajski, UPMC Center for High-Value Health Care
- Juan Marcos Gonzalez, Duke University School of Medicine
- Shana Traina, Johnson & Johnson
- Upal Basu Roy, LUNgevity Foundation

1:30 pm

Question & Answer Session

1:40pm

Reactions, Wrap-up and Next Steps

Ilisa Halpern Paul, District Policy Group, Faegre Drinker Biddle & Reath, LLP

Reactors:

- Margaret Rehayem, National Alliance of Healthcare Purchaser Coalitions
- Annie Kennedy, EveryLife Foundation for Rare Disease

2:00 pm ET

Adjourn

About the Innovation and Value Initiative

IVI is a 501(c)(3) nonprofit research organization committed to advancing the science and improving the practice of value assessment in healthcare through collaboration among thought leaders in academia, patient organizations, payers, life science firms, providers, delivery systems and other organizations.



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