IVI INNOVATION AND VALUE INITIATIVE

Summary
2024

5th Annual Methods Summit

The Future of Patient-Centered Health Technology Assessment

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The Innovation and Value Initiative (IVI) is a 501(c)(3), non-profit research organization dedicated to advancing the science, practice, and use of patient-centered health technology assessment to support decisions that make healthcare more meaningful and equitable.

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OVERVIEW

On March 14, 2024, the Innovation and Value Initiative (IVI) convened its 5th Annual Methods Summit, a pivotal event uniting diverse perspectives and industry leaders to chart the course for the future of patient-centered health technology assessment (HTA). With over 260 registrants for this year's hybrid event, the Summit served as a collaborative, inclusive forum where stakeholders heard the call to action and committed to work together to prioritize incorporating lived experiences into future HTA practices.

Patient-Centered Health Technology Assessment (HTA)

Health technology assessment (HTA) is defined as an analysis of the clinical and economic value of a particular healthcare intervention using various factors, such as cost, safety, and efficacy. It is used by employers, payers, manufacturers, and policymakers, among others, to help determine how patients are treated and at what cost.

Patient-Centered HTA is characterized by:

1. The core principles of patient-centricity, health equity, and transparency.
2. Authentic partnership with patients (and their families and caregivers) throughout the HTA process, including co-leadership in the process, strategy, and governance and partnership with decision-makers in reviewing and applying results.
3. Assurance that patient preferences, patient-preferred outcomes and goals, and lived experience inform HTA methodology (e.g., formation of research questions, model design, and incorporation of evidence) so that assessment models and results reflect what patients value.

THEMES

ENGAGING PATIENTS AS TRUE PARTNERS IN HEALTH TECHNOLOGY ASSESSMENT (HTA)

Patient-centered HTA requires comprehensive and intentional stakeholder engagement and the integration of patient perspectives throughout the assessment process, including patient journey mapping, involvement of patient advisory groups, and the need to reach out to underrepresented patients to ensure diverse input and experiences.

ADDRESSING EQUITY ACROSS HTA

Equity in HTA means ensuring that all HTA methods consider health equity implications. Noteworthy topics encompassed age and disability-related inequalities, the complexities in measuring equity for rare diseases, and the pressing need to quantify value within patient communities of smaller sizes. Additionally, disparities in affordability and the prevalent lack of trust in the medical community were highlighted as critical aspects to address as equitable healthcare outcomes are pursued.

EXPLORING INNOVATIVE METHODS IN PATIENT-CENTERED HTA

The Summit showcased innovative methodologies poised to reshape HTA practices, such as exploration of real option value (ROV), machine learning (ML), and other artificial intelligence (AI) technologies. There was consensus and emphasis regarding the need to be open to exploring new methodologies beyond traditional ones used in HTA. Panels delved into the practical implementation of these methodologies, highlighting opportunities and challenges for their integration into existing frameworks.

CONTINUING COLLABORATION FOR PATIENT-CENTERED HTA

Collaboration was a central theme during the Summit: the importance of fostering partnerships among patients, researchers, policymakers, and industry stakeholders was repeatedly lifted up as an essential undertaking. Discussions highlighted the need for open communication channels, continual engagement, and a shared commitment to advancing patient-centered HTA practices. The concept of "relationshipping" was introduced, emphasizing the need for active communication and ongoing connections between patients and researchers to ensure patient feedback is effectively integrated into HTA processes. Building trust and fostering relationships, especially with marginalized patient groups and those who have uncertainty about healthcare providers, emerged as critical components of patient-centered HTA.

RECOMMENDATIONS AND INSIGHTS

PANEL 1: OPENING KEYNOTE DISCUSSION: THE PATIENT AND THE MODELER AS FRIENDS, NOT FOES

This keynote discussion emphasized the intricate intersection of patient journey mapping and health technology assessment methodologies.

- **Closing the Communication Loop:** To integrate patient perspectives into HTA, it is vital to close the communication loop between patients and modelers. This involves incorporating patient input and providing feedback to foster stakeholder trust and collaboration. Modelers also have an important role to play in HTA-related efforts that patient organizations may be initiating and should be involved at the outset.

- **Incorporating the Patient Voice in HTA:** Diverse input and perspectives can be secured by advocating for the engagement of both empowered and less empowered patients, and the utilization of patient advisory groups (PAGs).

- **Laying the Groundwork:** Establishing collaboration between patients and modelers is important to promote a patient-centered approach to HTA.

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> **Allow patients to participate and make it possible for them to participate [in health technology assessment].**
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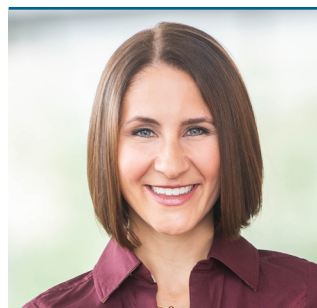> — Kistein Monkhouse (Patient Orator)

SPEAKERS

Ilisa Halpern Paul, MPP
Venable, LLP

Kistein Monkhouse, MPA
Patient Orator

Melanie Whittington, PhD, MS
CEVR, Tufts Medical Center

PANEL 2: PUTTING EQUITY INTO PRACTICE: PATIENT-CENTERED METHODOLOGIES AND FRAMEWORKS

This panel highlighted practical examples of how patients and researchers can partner to incorporate health equity into HTA and explore the research questions that need to come next.

- **Age-related and Disability Disparities in HTA:** The consideration of health equity must be inclusive, and include age – particularly in pediatric populations – and disabilities, in addition to race, ethnicity, gender, geography, etc.
- **Innovative Approaches to Addressing Data Gaps and Equity:** Innovative methods like machine learning (ML) and artificial intelligence (AI) have the potential to overcome data gaps and enhance equity considerations, particularly in the context of rare diseases.

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Social workers and other experts should be included in the HTA process. They have a unique way of understanding the whole person.

— Sneha Dave (Generation Patient)

SPEAKERS

Ilisa Halpern Paul, MPP
Venable, LLP

Sneha Dave
Generation Patient

Anton Avanceña, PhD
University of Texas at Austin

Bellinda King-Kallimanis, PhD
LUNGevity Foundation

PANEL 3: IS REAL OPTION VALUE REALLY AN OPTION FOR TODAY'S HEALTH TECHNOLOGY ASSESSMENT (HTA)

This panel discussed how patients and caregivers benefit from treatments that help them live longer and how to use real option value (ROV) in assessing health technologies practically.

- **Understanding Patient Perspectives on Value through the Lens of ROV:** As this particular method evolves, it is important to focus on how ROV offers patients valuable insights into the evolving landscape of treatment options, influencing their decision-making processes and perceptions of value in healthcare.

- **Future Integration of ROV in HTA:** It is important to integrate ROV into HTA methodologies, acknowledging the progress while emphasizing ongoing research and development necessary to optimize its utilization in future assessment frameworks.

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> **Surviving to future innovations or just survival alone has impact not only on health, but other factors such as caregivers and productivity.**
>
> — Meng Li (CEVR, Tufts Medical Center)

SPEAKERS

Rick Chapman, PhD
Innovation and Value Initiative

Zubair Ansari
Cancer Support Community

Meng Li, PhD
CEVR, Tufts Medical Center

Jon Campbell, PhD
National Pharmaceutical Council

PANEL 4: RARE DISEASE, UNIQUE NEEDS — ADAPTING HEALTH TECHNOLOGY ASSESSMENT (HTA) FOR TRUE PATIENT IMPACT

This panel explored what makes patient-centered HTA for rare disease different from more prevalent conditions and how that can inform HTA in general.

- **Being Holistic in Rare Disease HTA:** Panelists emphasized the importance of considering the relationships between mental and physical health while managing rare diseases. By embracing a holistic approach that integrates patient perspectives and soft skills, researchers can capture the extensive impact of rare diseases on patients' lives, going beyond their medical symptoms.

- **Unique Challenges of Rare Diseases in HTA:** The discussion highlighted the unique challenges that rare diseases present in the context of HTA, compared to more common diseases. It emphasized the need for specialized approaches that accurately assess and value the impact on patients, while considering factors such as disease severity, treatment timelines, and the small size of patient communities. The conversation stressed the significance of tailoring HTA methodologies to address the specific requirements and attributes associated with rare diseases.

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> **The rare disease space is vast and wide...There is so much work to do...There is power in numbers. If you continue to build relationships, that's where the work comes in. Their needs are different, but collectively, they are the same.**
>
> — Dionne Stalling (Rare And Black)

SPEAKERS

Ilisa Halpern Paul, MPP
Venable, LLP

Dionne Stalling
Rare And Black

Simu Thomas, PhD
Alexion, AstraZeneca Rare Disease

Annie Kennedy
EveryLife Foundation for Rare Diseases

PANEL 5: BEYOND BUZZWORDS: MAKING PATIENT-CENTERED HEALTH TECHNOLOGY ASSESSMENT (HTA) A REALITY

This closing fireside chat reflected on the day's discussions and identified concrete steps to put HTA into practice.

- **Educational Initiatives:** Promoting awareness and understanding of patient-centered HTA among stakeholders will help cultivate a culture of collaboration and engagement, facilitating the adoption of patient-centered HTA practices.
- **Hidden Barriers to Patient-Centered HTA:** Challenges hindering the adoption of patient-centered HTA include resource constraints, time limitations, and institutional barriers. Strategies, such as organizational commitment and resource allocation, were discussed to overcome these obstacles and effectively implement patient-centered HTA.
- **Practical Implementation for Patient-Centered HTA:** Actionable steps to actualize patient-centered HTA can be taken, starting with translating theoretical concepts into tangible practices to drive meaningful changes in healthcare decision-making processes.

> **One thing that is a great motivator is transparency. Asking for reporting of where that input took place and how it took place and how individuals were asked to participate...We need to be thinking about who is not at the table and set standards.**
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> — Eberechukwu Onukwugha (University of Maryland)

SPEAKERS

Jason Spangler, MD, MPH, FACPM
Innovation and Value Initiative

Eberechukwu Onukwugha, PhD
University of Maryland

CALL TO ACTION FOR PATIENT-CENTERED HTA

Throughout the Methods Summit, engaging and dynamic conversations unfolded across the panels, driving home critical insights for IVI and stakeholders focused on patient-centered HTA:

1. **Continual Engagement:** Continue to hold meetings and engagements involving all stakeholders to identify pain points and areas for improvement.

2. **Honest Conversations:** Foster honest conversations between stakeholders to address the tension between population-level considerations and individual needs.

3. **Exploration of New Methods:** Further develop and refine new methods such as ROV, acknowledging that while they may need additional work and refinement, it is essential to start implementing them and learn from practical application.

4. **Holistic Approach:** Embrace a holistic approach to patient-centered HTA, incorporating qualitative data and mixed methods to capture aspects of patient experiences that may not be quantifiable.

5. **Cultural Considerations:** Recognize and address cultural factors influencing healthcare decision-making and participation in HTA processes.

6. **Equitable Representation:** Ensure that all stakeholders, including those who may not typically have a seat at the table, are represented and involved in HTA processes throughout – and from the start.

7. **Building Trust:** Work towards building trust between patients, modelers, and other stakeholders, fostering bi-directional communication and collaboration.

8. **Integration of Patient and Modeler Perspectives, "Closing the Loop":** Encourage bi-directional involvement, where patients are involved in HTA processes, and modelers incorporate patient perspectives into their models, as well as patient groups initiating engagement with modelers and the research community.

9. **Resource Accessibility:** Recognize that not everyone has the same resources and strive to ensure all interested parties have access to participation opportunities and resources.

10. **Move Beyond Traditional Formulas:** Acknowledge the need to move beyond traditional HTA formulas and methodologies, embracing new approaches that better capture patient experiences and values.

11. **Consolidation of HTA and Patient-Centered HTA:** Advocate for consolidating HTA and patient-centered HTA into a unified approach, emphasizing the importance of equity and value.

12. **Commitment to Learning:** Emphasize a commitment to learning and adapting along the way, recognizing that progress may involve trial and error.

CLOSING REFLECTION: "YOU PAY EITHER WAY"

In reflection, the Methods Summit illuminated a fundamental truth: "You pay either way." This overarching theme underscores the significant cost of neglecting patient-centered healthcare practices, be it financial, societal, or personal. We must recognize the adverse outcomes and consequences stemming from failing to prioritize patient needs and perspectives. Beyond the individual level, as a society, we bear the burden of healthcare consequences when patients are not at the forefront of decision-making processes.

As we chart the path forward, it becomes increasingly evident that patient-centered approaches must be central to every aspect of healthcare, including HTA. The closing discussion iterated the need for ongoing engagement, candid conversations, and a commitment to learning and adaptation by emphasizing the importance of embracing a holistic approach that places trust, equity, and value at the core of HTA practices. Moving forward, it is essential for all stakeholders to ensure that patients remain the focal point, driving meaningful change and fostering a healthcare ecosystem that genuinely serves the needs of all.

APPENDIX

MEETING WORKSHEET

The IVI Methods Summit aims to bridge the gap between theory and practice by exploring actionable steps that stakeholders can take to implement patient-centered health technology assessment (HTA) effectively. This worksheet is provided to attendees as a place to add comments, questions, and recommendations. We will use your responses to inform our future work and the meeting summary, and we hope you will use it to foster discussion during each session.

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Session One – Keynote Discussion. What are examples of best practices to ensure that the perspectives of patients, family members, and caregivers are incorporated into HTA and modeling?

Session Two – Equity in Practice. What are the top steps that need to be taken to improve and promote equity-informed HTA? What role do you see for IVI? What role do you see in your own work?

Session Three – Real Option Value. What barriers or opportunities do you think exist to incorporate real option value into the HTA process?

> **Real Option Value (ROV):** Real option value calculates the benefit patients and caregivers receive from treatments that extend life, giving patients the chance to benefit from future treatment advances because of their greater longevity.

Session Four – Rare Disease. What makes patient-centered HTA for rare disease different, and how can that inform HTA in general?

Session Five – Closing Keynote. What is one t hing you would assign yourself or IVI to do to make patient-centered HTA a reality?

What other insights from today's Methods Summit would you like to share?

What stakeholder perspective do you most represent?

Please note your name and e-mail if you would like to connect on potential partnership opportunities.

Thank you!

Please return completed worksheets to IVI at the end of the event. Virtual attendees may send the worksheet to info@thevalueinitiative.org.

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Learn more on the IVI website.