IVI INNOVATION AND VALUE INITIATIVE

VALUE BLUEPRINTS
Research Brief Series

Prioritized Attributes to Better Define and Measure Innovation in Health Technology Assessment

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Introduction

Formalized health technology assessment (HTA) provides a framework to assess the value of health interventions to different stakeholders in our society, for example, whether prices of treatments reflect their benefits to patients. HTA is increasingly used to inform decisions that will impact patient access to novel health technologies worldwide.[1] However, as biomedical innovations accelerate, novel health technologies with potentially transformative health benefits, such as cell and gene therapies[2], have posed unique challenges in applying our existing practice and methods in HTA (e.g., uncertainty in long-term clinical outcomes). These challenges could lead to decisions that would hinder access to innovative therapies among those in need of treatments and prevent the full societal benefits from being realized.

As drugs, medical devices, digital health applications, and other types of biomedical innovations evolve, it is critical to refine our practice and methods to better define, measure, and reward innovations to ensure the long-term sustainability of our innovation ecosystem and to maximize welfare for those that receive care.

In collaboration with multi-stakeholder partners, IVI launched the Valuing Innovation Project (VIP), a multi-phased research initiative, to identify creative solutions with cross-stakeholder buy-in to improve existing practices and methods in HTA. During the first phase, the 2022 IVI Methods Summit gathered patients, clinicians, health system manufacturers, employers, payers, and researchers to discuss the current approaches to how innovations are considered in healthcare decision-making, and the potential opportunities and challenges specific to HTA. Based on the synthesis from the Methods Summit, IVI organized an Expert Roundtable in October 2022 to prioritize specific attributes for additional research in data and methods. These prioritized attributes have informed the next phase of the initiative, a Call for Papers, to explore creative solutions for the data and methods gaps.

This Value Blueprint documents the major discussion topics from the Expert Roundtable and summarizes the key findings.

Expert Roundtable

The Expert Roundtable brought together eleven patient, clinician, health system, manufacturer, employer, payer, and researcher expert participants[3]. The session consisted of two facilitated discussion sessions, structured in a stepwise approach to discover the most important areas requiring additional HTA research[4].

During the first session, participants reviewed a list of 23 attributes used to define innovative properties of health technologies across six key domains and identified from a targeted literature review of existing HTA frameworks and guidelines (see Table 1). The review was followed by a discussion to identify missing or unnecessary attributes.

The participants continued to a second session, using the first session's revised list to prioritize the most important attributes for incorporation into HTA. Next, they refined the list further for additional discussion about available data and methods. The experts participated in a group exercise to plot the attributes on a two-dimensional scatter plot, illustrating the available data and readiness of appropriate methods.

The roundtable ended with a voting exercise and each expert participant ranked their top three prioritized attributes for the Call for Papers research questions.

1 Source: World Health Organization. https://www.who.int/health-topics/health-technology-assessment#tab=tab_1

2 Besley, S., Henderson, N., Towse, A. & Cole, A. (2022) Health Technology Assessment of Gene Therapies: Are Our Methods Fit for Purpose?. OHE Consulting Report. Available from https://www.ohe.org/publications/health-technology-assessment-gene-therapies-are-our-methods-fit-purpose/

3 Link to complete list of participants: https://thevalueinitiative.org/wp-content/uploads/2022/10/Expert-Roundtable-List-of-Participants.pdf

4 Link to the pre-read and discussion guide: https://thevalueinitiative.org/wp-content/uploads/2022/10/Expert-Roundtable-Pre-Read-Packet.pdf

Table 1. List of Attributes to Define and Measure Innovation in HTA

Therapeutic Properties

- Comparative efficacy over existing treatments (whether it is incremental or substantial)
  - Example: Time to treatment effect
- Improved diagnostic accuracy and therapeutic targeting (e.g., from genetic testing)
- Cure (vs. symptom improvement)
- ~~Strength/uncertainty of clinical evidence~~

Side Effects and Convenience

- Tolerability/reduced side effects
- Patient convenience
  - Example: Route of administration
- Patient preference
- Impacts on adherence
- Caregiver convenience (e.g., transportation; work absence, burden)
- Family spillover
  - Impacts on those that do not directly provide care
  - Not just absenteeism and presenteeism

Underlying Health Conditions of the Patients and Current Care

- Severity of underlying disease
  - Comorbid disease conditions
- Impact on the health of the broader population (e.g., spread of infectious diseases)
  - Not just health sectors, but also non-health sectors
- Unmet needs
- Availability of existing treatments
- Improve the ability of health system to deliver acre more efficiently
- Ability to personalize care for individual patients
- Ability to help novel treatments reach full potential

Long-Term Dynamic Effects (i.e., Impacts on Future Generations)

- Novelty of the technology (e.g., a new mechanism of action)
  - Incremental vs. radical
- Scientific spillover effects
  - Novel knowledge/learning
  - Impacts on the costs for next innovation
- Real option value

Economic and Social Impacts

- ~~Cost of treatments~~
- Cost of non-healthcare resources (e.g., costs to caregivers and family members)
- Budget impact for a health system
- Productivity
- Employment
- Education
- Environmental impacts

Health Equity Impacts

- Access/delivery of treatments to different subgroups within a population
- Addressing unmet needs that disproportionately affect certain subgroups
- Disparities in key clinical and economic outcomes (specified above) across subpopulations
- Representation in clinical trial

Black Font: Attributes in the initial list Orange Font: Attributes that should be added to the list Blue Font: Attributes that should be removed from the list

Summary of Discussion

Missing Attributes

Eight attributes were added to the initial list. These attributes, highlighted in orange in Table 1, included patient preference, impacts on adherence, family spillover, ability to personalize care for individual patients, ability to help novel treatments reach full potential, impacts on costs for future innovation, environmental impacts, and representation in clinical trials.

Participants gave both specific and nuanced examples of attributes included in the initial list. For instance, in applying "severity of underlying disease," HTA practitioners should think about the impacts on comorbid conditions from a novel health technology aspect, not just the impacts on the intended disease area. Participants shared that a highly comorbid condition, such as depression, was a case where improved symptoms could result in improved management of other conditions, such as diabetes and cardiovascular diseases.

Attributes to Remove

Participants removed two attributes from the initial list, highlighted in blue in Table 1. Experts noted that, instead of being a standalone attribute, "strength/uncertainty of clinical evidence" should be considered for all listed attributes. "Cost of treatments" was removed because it depended on factors other than the innovative properties of the technology, including reimbursement decisions made by payers.

Prioritized Attributes for Data and Method Readiness Assessment

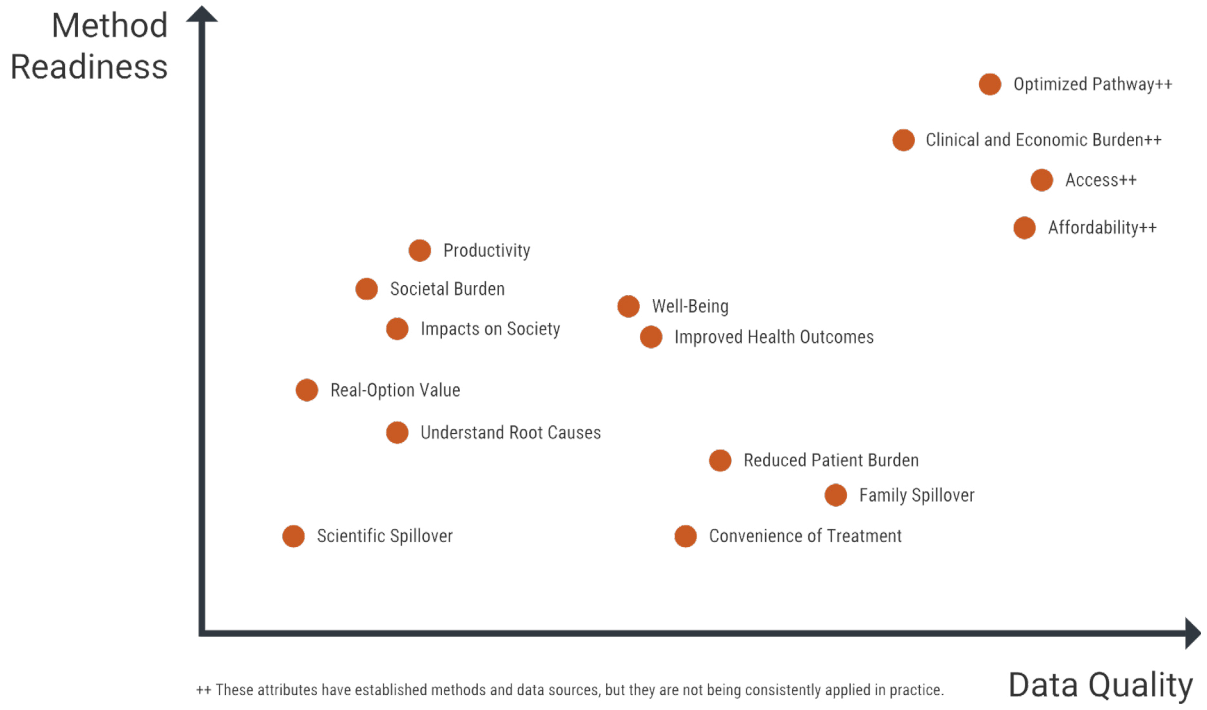
In the second discussion session, participants began by identifying 21 essential attributes to consider in valuing innovation in HTA and put them in three domains: quality of life, long-term dynamic effects, and unmet needs. Table 2 lists these attributes.

Table 2. List of Attributes Most Important to Consider in Valuing Innovation in HTA

Quality of Life	Long-Term Dynamic Effects	Unmet Needs and Wants

• Ease
• Convenience
• Productivity
• Well-being/wellness
• Family spillover/family burden
• Reduced patient burden
• Impacts on the broader society
• Health outcomes
	• Novelty of the technology (e.g., a new mechanism of action)
	  • Incremental vs. radical
	• Scientific spillover effects
	  • Novel knowledge/learning
	• Real options value
	• Impacts on the costs for next innovation
		• Access
		  • Availability of existing treatments
		  • Accessibility of existing treatments
		  • Affordability
		  • Timely access/optimized treatment pathway
		• Diagnostics and its potential to improve health through the targeted and related diseases (spillover)
		• Removing clinical and economic burden
		• Available treatments (curative, disease modifying, treatment exists, exists but not great)
		• Therapeutic options
		• Undiagnosed patients and population
		• Understanding the "root cause" of a disease
		• Disease severity
		• Lack of market incentives

Figure 1. Attributes Categorized by Data and Methods Readiness



Fifteen of the 21 attributes were prioritized for additional discussion to assess whether there are existing methods and data sources to measure them in HTA. Experts conducted a group exercise to plot these 15 attributes based on an assessment of data and method readiness. This scatter plot is featured in Figure 1.

Access, affordability, optimized treatment pathway, and clinical and economic burden were categorized as attributes with good data and methods available, but the use of quality data and validated methods was inconsistent across different stakeholders and diseases.

The well-being of patients and improved health outcomes were attributes judged to have some emerging data sources and methods.

Family spillover[5], reduced patient burden, and convenience of treatments were attributes judged to have some emerging data sources but lacking methods.

Impacts on broader society[6], real-option value[7], productivity, understanding the root causes of a disease, and societal burden were attributes that were judged as having established methods but requiring additional data collection efforts in applying such methods.

Lastly, scientific spillover[8] was considered an attribute that lacked both established data and methods.

Top Three Attributes Prioritized for Call for Papers

Based on committee votes, scientific spillover, real-option value, and impacts on broader society were identified as the top attributes prioritized as themes for the Phase 3 Call for Papers.

5 Family spillover can occur when a disease and its management impact not only the person diagnosed with the disease condition, but also family members who may or may not directly provide care.

6 Examples of impacts of innovation on broader society beyond the healthcare sector include education, employment, and overall GDP.

7 Real-option value applies when an existing treatment option prolongs survival or reduces disease severity of patients, which might subsequently enable them to benefit from future innovations that they are concurrently eligible for.

8 In the innovation system, scientific spillover usually arises when knowledge or learning from an existing R&D effort benefits other concurrent or future R&D activities. Such benefits are usually not entirely appropriated by the innovator that generated such knowledge.

Conclusion

The Expert Roundtable concluded with cross-stakeholder consensus on prioritized attributes for further research, so we can better define and measure innovations in HTA. Advancing research in these areas through a Call for Papers initiative will help us identify innovative solutions that will address these gaps, investing in the long-term sustainability of our R&D ecosystem and improving social welfare.

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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 health technology assessment through collaboration among thought leaders in academia, patient organizations, payers, life science firms, providers, delivery systems, and other organizations.

About IVI

The Innovation and Value Initiative's mission is to advance 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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