Novel Value Measures and European HTA: Implications for Pharma/Device HEOR

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We must recognize that the term "value" may be more appropriately thought of as "benefit" which, in turn, triggers a discussion on the opportunity cost of other forgone benefits. New cost-effectiveness modeling methods are required to accommodate the novel sources of value.

MOVING BEYOND THE QALY

The recent ISPOR Special Task Force on U.S. Value Frameworks[1] identified 12 value elements for healthcare innovation. With the quality-adjusted life year (QALY) as the starting point and adding accepted sources of value (such as cost savings, productivity, and the adherence-improving factor), Lakdawalla et al suggested several more novel, uncertainty-related sources, such as the value of hope, option value, and the value of knowing. Informally referred to as the *"Value Flower,"* this offers an interesting starting point to consider how novel sources of value may apply to European health technology assessment (HTA).

There appears to be broad support for a wider perspective on value. For example, an informal audience poll (N~300) at the start of the ISPOR-EU 2018 panel discussion, "Do Novel Value Measures Have a Place in European HTA?" (Breakout #2, IP6) revealed that around three-quarters of respondents thought that the QALY inadequately captures the patient benefit and should be supplemented by other measures of value, and that the views of non-patients (also receiving benefits from the same payer) who may not receive the benefit of a new innovation — but will share the cost — should be included in HTA.

NOVEL VALUE MEASURES AND EUROPEAN HTA: KEY ISSUES UNDER DEBATE

From an HTA perspective, the inclusion of novel sources of value raises several issues:

Are we using the correct terminology?
– The much-used term "value" often conveys the benefits that a health technology offers, but in any collectively funded healthcare system, the term might more appropriately mean, "Do the benefits of a new intervention outweigh the opportunity cost?." So whatever gets included in the benefit function, eg, a concern for inequality in health as well as health gain itself, these need to be reflected in what the system doesn't fund as well as in the new investments in drugs and other interventions.

Who has the right to define benefit and how inclusive should this be?
For example, how do we differentiate direct medical care from care that supports the activities of daily living to promote independence, well-being from social interaction, and knowing versus just being informed? In simple terms, does the "Value Flower" require more petals?

Are novel benefits finite or infinite?
This provocative question addresses whether the benefits of a drug are finite, with each new study articulating different elements of a single, all-encompassing QALY or whether value elements X, Y, and Z are indeed incremental to the foundational QALY.

Do current CE modeling techniques accommodate novel benefits?
Acknowledging the foundational standing of the cost-per-QALY metric (also known as "cost-utility analysis") in HTA, is there a place to expand this to add the other elements of value as well as broaden the methods via such tools as multicriteria decision analysis? There is literature showing how this can be done – for example, distributional or augmented cost-effectiveness analysis.[2] However, there is a need for a wider discussion on other possible aspects of the benefits to include.

How do we reconcile "proof versus promise" for a health technology?
While the dynamic nature of a treatment's cost-effectiveness has been suggested,[3-4] a more fundamental tension faces society and HTA bodies in particular: "Given the resources available, do we invest in a new treatment with unproven effectiveness or continue to spend on existing, tried-and-tested treatments that have been on the market for a long time?" And, if the latter is chosen, what long-term societal benefits will be foregone if the scientific community does not continue to advance medical science that may find a future cure? The ISPOR Special Task Force labelled this element of value "scientific spillovers." Linked to this is the challenge of generating sufficient evidence to justify investment and establishing an iterative framework to determine how

much a system should pay for a product at launch, assessing whether additional evidence should be generated postlaunch, how this is incentivized and funded, and how the product's price can be adjusted as new evidence emerges.[5-6]

NOVEL VALUE MEASURES AND EUROPEAN HTA: IMPLICATIONS FOR PHARMA/DEVICE HEOR

Three implications are worth consideration by the HEOR researcher embedded within a pharmaceutical or medical device setting:

1. The commercial application of expanding a product's benefit profile – Within an existing treatment indication, recognize that emerging evidence on a new or existing benefit secures the drug's place in therapy and from a commercial perspective, may drive uptake and market share. In markets that allow a price increase, new trial or real-world data may help support a price adjustment.

2. Opportunity cost – In all collectively funded healthcare systems, consider describing the average foregone treatment opportunity and its associated benefits; ie, the things that a payer will not be able to do for the same amount of money. In simple terms, what unmet needs will be addressed and what are examples of unmet needs that will not be met? While acknowledging that the needs of the sick should not be ignored, preparing to debate the opportunity cost issue will focus the innovator on truly capturing the unmet need that is being addressed. Literature showing how opportunity costs can be estimated empirically is now emerging, with evidence from the United Kingdom, Sweden, Australia, and others.[7]

3. Capturing the value of disruptive innovation – In markets where access and reimbursement are driven by HTA relying on the cost per QALY, there is potential to mitigate this by demonstrating the product's other benefits (ie, elements of value) as long as the same aspects of value are assessed in measuring the opportunity costs.

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EMBRACING INNOVATION IN AN HTA WORLD

From an innovator's perspective, one could argue that while the current HTA approach was developed in an era when the focus was on high-prevalence, chronic diseases, it is now being applied in the era of precision medicine. Thus the cost-per-QALY approach presents limitations for some of the transformative treatments now in development such as curative gene therapies and highly anticipated treatments that society wants in the future, for example, in autism and Alzheimer's disease.

Alternatively, from an HTA perspective, perhaps the current critical issues are less around the methodological considerations that comprise an HTA, but instead about addressing the resource constraints facing collectively funded healthcare systems: (1) such public systems are limited in their ability to raise prices, (2) the supply of new health technologies, and (3) adoption of any new health technology in one deserving area of medical care implies less funding available for other services. Furthermore, although this is less explicit, the challenge of "opportunity cost" is central to resource allocation decisions in all healthcare systems funded collectively, with fixed budgets or not. Thus, the key point is that the focus of debate is usually about the merits of the new technology and rarely about the forgone benefits associated with alternative uses of resources; that is, we need societies to become more transparent and open about the opportunity cost of adopting innovation.

The insights for the pharma and device HEOR scientists are of real, practical use: (1) recognize the ongoing value of generating new real-world effectiveness evidence as the basis for describing the benefits to different patient groups and supporting a product's price; (2) be prepared to discuss the opportunity cost for adopting your treatment innovation versus maintaining the status quo; and (3) develop evidence of value beyond the QALY argument.

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REFERENCES

1. Lakdawalla DN, Doshi JA, Garrison LP, Phelps CE, Basu A, Danzon PM. Defining elements of value in health care – a health economics approach: An ISPOR Special Task Force Report. *Value Health.* 2018;21:131-139.

2. Asaria M, Griffin S, Cookson R, Whyte S, Tappenden P. Distributional cost-effectiveness analysis of health care programmes – a methodological case study of the UK bowel cancer screening programme. *Health Economics*. 2015; 24(6):742-754.

3. Garrison LP. Rewarding value creation to promote innovation in oncology: the importance of considering the global product life cycle. *Oncologist*. 2010; 15(suppl 1):49-57.

4. Lu Y, Penrod JR, Sood N, Woodby S, Philipson T. Dynamic cost-effectiveness of oncology drugs. *Am J Manag Care*. 2012;18:s249-s256.

5. Garrison LP, Neumann PJ, Willke RJ, et al. A health economics approach to US value assessment frameworks – summary and recommendations of the ISPOR Special Task Force Report {7}. *Value Health*. 2018; 21: 161-165.

6. Sculpher M. ISPOR's initiative on US value assessment frameworks: Seeking a role for health economics. *Value Health*. 2018; 21:171-172.

7. Sculpher M, Claxton K, Person SD. Developing a value framework: the need to reflect the opportunity cost of funding decisions. *Value Health*. 2017; 20:234-239.

ADDITIONAL INFORMATION

The preceeding article is based on am issue panel given at ISPOR Europe 2018. To view the presentations, go to https://www.ispor.org/conferences-education/conferences/past-conferences/europe-2018/conference-presentations.