Comment and Response Summary for Defining Appropriate Benefits for Economic Evaluation of Health Care Technologies

Brett McQueen*, Allan Wailoo*, Aki Tsuchiya*, Lauren Cipriano, Jason Robert Guertin, Gillian Schmidler, Lotte Steuten, Sean Sullivan, Kednapa Thavorn, Mark Sculpher*

**Denotes membership of the writing group*

This document summarizes the public and academic comments received on the draft report, published in October 2025, with responses from the HEMA working group. The summary presented is meant to cover the main themes of the comments that were responded to, rather than a reflection of all the comments received.

1. Clarifying points on the role of HEMA and current HTA practice

Comment: Commenters highlighted the experience and viewpoints of HEMA working group members, the role of HEMA in HTA, normative assumptions, how the final report may be used by HTA agencies, and perceptions of endorsing the status quo.

Response: We refer readers to the original objectives of the report, which included defining a set of guiding principles to support HTA organizations' decisions about potential changes in the benefit function used in economic evaluation and to apply the principles to additional benefits. It may be the case that current methods do not meet the proposed principles. In section 1.2 (Aims and Objectives), we added text addressing these comments:

HEMA's role is to provide independent guidance on methods for these different organizations. Those HTA organizations have previously selected normative frameworks for their decision-making, which reflect the remits and constraints of the health systems using their assessments. This includes decisions about the existing benefit functions they use in economic evaluation and aspects of how this is derived – for example, whose preferences are used as weights within the measure of lifetime health gain. These normative choices are open to debate, but they are not technically right or wrong, and the HTA organizations (and any organizations to which they report) are the appropriate sources of those judgments. As such, HEMA will take these normative positions as given and advise about the future methods developments needed to reflect the implications of these normative positions. However, it may also be the case that, despite these normative starting points, HEMA may identify weaknesses in current methods used or advocated by the HTA organizations, and it is not HEMA's responsibility to defend the methods decisions taken previously by these organizations.

Comment: Commenters expressed concerns that using the QALY as the default benefit function in the report may be perceived as endorsing a narrow view of health and/or HTA agencies often use an alternative benefit function as described in Box 2.

Response: We removed Box 2 from the report. Instead of attempting to include every alternative with each mention of the benefit function, we instead introduce the term "lifetime health gain" to refer to any measure of preference-weighted health over time. In section 1.1 (Background), the following text is introduced at the end of the section:

Alternatives to the QALY include the disability-adjusted life-year, the equal value life-year, and health years in total. In the remainder of this report, we refer generally to this measure of preference-weighted health as "lifetime health gain."

2. Whose preferences? And the role of patient-centeredness and patient experience

Comment: Commenters highlighted questions surrounding the preferences used in HTA evaluations, including the role of patient preferences and patient experience. Comments also highlighted a potential disconnect between the report and patients, emphasizing that HTA must represent the realities of the patient experience.

Response: Similar to our clarifying responses on HEMA's role in section 1, we provide additional text on assumptions surrounding the normative position that the value of health benefits should be drawn from the community (which by definition includes prior, current, and potential future patients). This normative position is also consistent with the US Second Panel on Cost-Effectiveness in Health and Medicine. We emphasize the role of patients in the measurement of health. It's important to note that HEMA does not rule out HTA organizations taking a normative position that preferences should come from patients with a specific disease for which the intervention under evaluation is indicated, but rather we recommend areas of additional research before implementing this in HTA practice. We have provided clarifying text in multiple areas, particularly in Sections 3.2 and 4.2, along with the final recommendations. Excerpts include, but are not limited to, the following:

Section 3.2:

The current normative position of HTA organizations reflects the collective nature of health system funding, whether tax- or insurance-funded. The community includes past, current, and potential future patients across all health conditions. This has been referred to as the "Insurance Principle." At the same time, limiting the source of values to patient preferences presents practical and conceptual challenges. Individuals living with conditions may adapt to changes in health over time. In addition, it may be difficult to identify patients who have experienced all relevant health states and adverse events or to ensure that valuations are not influenced by personal interests. For these reasons, community preferences are often used to support consistent and comparable valuation across different conditions and interventions. Importantly, this does not diminish the central role of patients. Patient experience is critical to identifying, describing, and measuring the health impacts of interventions that are subsequently valued within economic evaluation. Patient engagement to inform economic evaluation should be seen as complementary to the use of community preferences in valuation.

Section 4.2 (Recommendations):

• *A clear normative and practical case for routinely incorporating risk preferences into a measure of lifetime health gain is necessary. The normative case needs to consider both the theoretical merit of doing so and the importance of reflecting risk attitudes compared with other aspects of preferences that are simplified in standard methods.*

• *The practical case for routinely incorporating risk preferences into a measure of lifetime health gain should consider: whose preferences should be elicited; over which aspects of health they*

should apply; the need to produce robust evidence for the relevant jurisdictions; and whether such developments would significantly affect HTA decisions.

• HTA organizations that have adopted a normative position to use average community preferences to define benefits for economic evaluation should be aware that simultaneously incorporating aspects of patients' preferences would be a departure from this view and may be seen as inconsistent.

3. Societal Perspective

Comment: Commenters advocated for expanding the perspective used in current HTA.

Response: We refer commenters to existing text on spillover effects as already part of current HTA practice. We also reiterate that HTA decisions are very specific and do not necessarily involve broader policy decisions and overall funding levels across sectors. Much of this context is determined politically rather than by the HTA organizations. In other words, there is no single societal perspective that was the subject of much debate between Second Panel members, ultimately leading to the proposed impact inventory table.

4. Opportunity Cost

Comment: There was general acceptance that opportunity cost is an important principle. However, concern was expressed around a general lack of evidence in certain settings regarding the mechanisms and causal links leading to where opportunity costs are realized. Commenters further expressed concern that budgets are not fixed over time and allow for growth in health care funding.

Response: We added text to clarify the linkage between increasing health budgets overall and specific decisions to fund new technologies in Section 2. We expanded on this text in Section 3.3 to describe some of the challenges in establishing causal links with particular emphasis on the US, which may represent any system that may be funded through a mix of payers.

Section 2:

Although overall expenditure may increase over time in the health systems of high-income countries, there is no automatic linkage between a decision to fund a new technology and an increased funding envelope. Hence, opportunity costs mean decrements in health to real patients, although these individuals may not be easily identifiable, in contrast to those identifiable individuals who stand to benefit from new interventions for specific diseases. Across jurisdictions, evidence on the quantification of opportunity costs associated with increased expenditure is only now (and only partially) being reflected in economic evaluation in health. To ensure that the interests of all patients are reflected in HTA decisions, opportunity costs must be routinely considered in the methods used. Indeed, any analysis to inform HTA decisions without evidential consideration of opportunity costs cannot be defined as economic evaluation.

Section 3.3:

Given that evidence on opportunity costs (whether through cost-effectiveness thresholds or other means) is currently limited in HTA, it could be argued that ensuring any additional aspect of the benefit function is mirrored by an equivalent measure of estimated opportunity cost is unnecessary. The risk of failing to reflect evidence on opportunity costs is that less benefit is generated from available resources that could otherwise be achieved. There is also an equity concern, as some patients may end up with greater reductions in benefit compared to those who gain. This remains a current challenge for HTA, which should be addressed. The risks to public health of misallocating resources will be exacerbated if additional items of benefit are added to economic evaluation without symmetrical consideration of opportunity costs.

The US system, in particular, deviates from that in the UK and Canada, with no central insurance or funding system facilitating access to healthcare for all citizens. Identifying where opportunity costs fall in the US, or any system with mixed funding, is challenging for multiple reasons. For example, state-based Medicaid plans are statutorily required to balance budgets each year while covering the most vulnerable populations (e.g., approximately 25 percent of total Medicaid enrollment is through disability and aging eligibility pathways). Yet the Medicaid system is not closed for the duration of an individual's life, as members may rely on coverage at one point in time (e.g., over 50% of children rely on Medicaid coverage at one point in their lives) and later move to a private insurance plan through an employer. In this example, the lack of ability to follow and "track" health outcomes (e.g., as a function of restricted coverage), along with the mix of taxpayer funding and private funding, makes it difficult to identify causal links between marginal changes in health outcomes from marginal changes in expenditure. Research is necessary to understand further how opportunity costs manifest themselves in US systems and the implications for measurement. Despite this uncertainty in estimating opportunity costs, ignoring them in economic evaluations, with or without additional benefit measures, risks misallocating resources. Existing estimates, although uncertain, are the best available and need to be reflected in analyses to inform HTA decisions.