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EDITORIAL

Incorporating Quantitative Patient Preference Data into Healthcare Decision Making Processes: Is HTA Falling Behind?

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1 Introduction

Health economists regularly elicit quantitative preference data from samples of patients and the general public [1–4]. Quantitative preference data, such as estimates of trade-offs and valuations, can influence healthcare decision making by being provided as supplementary information or by being used within other analyses. A common example of the latter is health state valuation, using exercises such as time trade-off or standard gamble [5]. These exercises enable researchers to create value sets for multi-attribute utility instruments (MAUIs), such as the EuroQol Group's EQ-5D [6], that are routinely applied in health technology assessments (HTAs) that use cost-utility analyses (CUAs) where the quality-adjusted life year (QALY) is the measure of benefit [7, 8].

However, this is not the only way in which such data are elicited. Increasingly, methodologies such as discrete-choice experiments (DCEs) and best-worst scaling (BWS) are being utilized [9]. These exercises require individuals to make hypothetical choices between different options such as public health interventions [10], vaccinations [11], financial incentives for health behaviors [12], drugs [13], or even health states [14]. The exercises force respondents to make trade-offs between the different attributes that make up a healthcare intervention (for example) and the results could be used to inform decision making. With respect to HTA, data from such exercises can be particularly useful in highlighting the value of an intervention when the QALY is considered too narrow, e.g., when non-health outcomes are relevant, such as improvements in treatment administration.

Despite the potential, the use of quantitative *patient* preference data ("patient preferences" hereafter) in healthcare decision making is still relatively minimal in HTA. This article briefly reviews the recent advancements in the use of patient preferences in the regulatory space and puts forward possible explanations as to why HTA appears to be lagging. Thoughts on how HTA could "catch up" conclude.

2 The Use of Patient Preferences in a Regulatory Setting

Over the past 5 years, the US FDA Center for Devices and Radiological Health (CDRH) has taken a major role in encouraging the use of patient preferences in regulatory decision making [15]. A 2015 publication detailed how data from a DCE were directly incorporated into a benefit-risk assessment (BRA) for the CDRH [16]. Since this case study, the CDRH, along with another FDA center, the Center for Biologics Evaluation and Research (CBER), have finalized guidance on incorporating patient preferences into BRAs, acknowledging the merits of DCEs [17]. In addition, two notable pieces of legislation have been passed in the USA. The 21st Century Cures Act [18] and the Prescription Drug User Fee Act, Revision VI (PDUFA VI) of the FDA Reauthorization Act [19] both highlight the importance of considering the patient experience during the drug-development process, specifically referring to patient preferences. The legislation has led to the FDA producing a plan for "issuance of patient-focused drug development guidance" [20], which will lead to a series of new guidance

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documentation being finalized by 2021. Therefore, it is likely there will be a rise in the incorporation of patient preference data in regulatory decision making in the USA, and a related research agenda has already been put forward to support this [21].

There is also related activity in Europe [22]. A collaborative project called The Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium (PROTECT) under the Innovative Medicines Initiative (IMI) explored, in part, how preferences on benefits and risks might feed into the decision making process [23]. Their recommendations put forward DCEs as the "utility survey technique" that warrants further testing [23]. Another, ongoing, IMI project called PREFER is looking into this area by taking a broader perspective. The project aims to "develop a systematic approach for considering the use of patient preferences across the medical treatment life cycle" and will consider both BRA and HTA, with final recommendations due in 2021 [24].

3 The Use of Patient Preferences in Health Technology Assessment

In contrast to what is happening in the regulatory space, consideration of how to formally incorporate patient preference data into HTAs appears to be in its infancy [22], despite persistent calls for patient-centric HTA [25, 26]. There are several potential reasons for why this might be the case, which are outlined in turn.

3.1 The Dominance of Cost-Utility Analysis

The use, and focus on, CUA may limit opportunities for patient preferences to be incorporated into HTA processes. Beyond eliciting health state utilities from patients (which can be challenging [27]), no other quantitative preference information is required for a CUA. Existing guidance that has encouraged the use of, and focus on, CUA and reference cases in relation to its conduct [28] may inadvertently stifle attempts to incorporate patient preferences. In contrast, it is clearer to see how such evidence could be incorporated into BRAs as these require information on the likes of "maximum acceptable risk" and "minimum acceptable benefit." A DCE or BWS study that contains the relevant risk and benefit attributes can readily provide this information using marginal rates of substitution [29].

3.2 Normative Arguments

There are well known arguments against the use of patient preferences with respect to health state valuation [30], and some of these arguments can be applied to other types of preference studies [31]. HTAs assess whether an intervention should be financed. In a publicly funded system, it is arguably more appropriate to consider the preferences of the general population. In a privately funded system, one might argue that payees' preferences are more relevant than those of the broader patient population. These issues are not relevant in the regulatory space, where patient preferences are highly likely to be preferred over other stakeholders' preferences.

3.3 Preference Studies are Often Condition Specific

Several influential HTA agencies in countries such as the UK, Australia, the Netherlands, and Canada recommend the use of generic MAUIs such as the EQ-5D rather than condition-specific measures [32]. A major benefit of these generic measures is that they help improve the consistency of HTA decisions. In contrast, many preference studies, especially DCEs eliciting preferences for healthcare services (the most common application [9]), are condition specific. This means that studies are not always comparable and study quality could differ drastically from one HTA to another. This is not likely to be desirable in the eyes of HTA decision makers but is unavoidable in the regulatory space.

3.4 HTAs Often Incorporate Qualitative Preference Information

Patient involvement in HTA is well documented [33], and its importance is regularly stressed. Patient representatives are often included in decision making committees, allowing the "patient voice" to be heard [34]. Therefore, while it could be informative, it is not essential that patient involvement in HTA is done in a quantitative manner. The challenges associated with incorporating additional quantitative preference information into HTA provide a fairly convincing argument for a qualitative focus.

4 Looking Forward: Can Health Technology Assessment Catch Up?

Incorporating the "patient voice" into HTA is desirable [25], but doing so quantitatively is clearly a challenge given the current landscape. What changes might be needed for it to be possible for HTA to catch up with the regulatory space in this regard?

4.1 A Change of Stance from Influential Agencies

Key HTA agencies in countries such as the UK, Australia, the Netherlands, and Canada recommend that the general

population are the source for health state utilities [32]. If this stance changes, patient preferences could be incorporated within the QALY. This is only a minor change, and the challenges associated with eliciting experienced health state utilities are already being explored [27, 35]. However, the QALY is not free from criticism. One issue with the QALY is that it is not easy to incorporate broader outcomes or the "patient experience" into health state utilities, which is why projects such as "Extending the QALY" are currently being conducted [36]. Regardless, patients have been shown to adapt to their conditions [37, 38], and a movement away from using general population preferences would represent an ideological change relating to the perspective of CUAs (i.e., movement away from extra-welfarism [39–41]). Therefore, using patient preferences within a CUA could be controversial irrespective of the benefit measure. An alternative would be for more HTA agencies to provide clear guidance stating that condition-specific preference studies using the likes of DCE and BWS methodologies would be accepted as supplementary evidence when appropriate (e.g., where the benefits of an intervention extend to non-health outcomes), as is the case in Australia [42].

4.2 A New Methodological Approach to Health Technology Assessment

A new approach to HTA could provide greater opportunities for the incorporation of patient preferences. Multi-criteria decision analysis (MCDA), despite its own challenges, has been proposed as an alternative to CUA in HTA [22, 43, 44]. In an MCDA, the different criteria that are important for the decision making process are weighted in terms of their relative importance. This weighting could be done by patients using the likes of a DCE [45], allowing patient preferences to be incorporated into HTA decisions. Additionally, the criteria could be kept relatively generic and therefore consistent across HTAs, which could improve the acceptability of this approach amongst decision makers. However, it should be noted that advocates of an MCDA approach for HTA have not specified that patients in particular should be the source of weightings [46], nor have the practicalities of having patients undertake this task been thoroughly explored.

Most of these changes are substantive. Whether they are realistic in the long term is currently unclear, but the appetite for incorporating the "patient voice" into HTA is unlikely to subside. For now, it seems that patient preferences will play a bigger role in the regulatory space and, if no changes are made, HTA will be left playing catch-up.

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