Article:
Ara, R.M., Peasgood, T., Mukuria, C. et al. (2017) Sourcing and Using Appropriate Health State Utility Values in Economic Models in Health Care. PharmacoEconomics, 35 (Suppl 1). pp. 7-9. ISSN: 1170-7690

https://doi.org/10.1007/s40273-017-0543-z

The final publication is available at Springer via http://dx.doi.org/10.1007/s40273-017-0543-z

Sourcing and using appropriate health state utility values in economic models in health care

Running header: sourcing and using health state utility values

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1. Background

Decision analytic models (DAM) in health care are generally used to assess different interventions to determine which provides the best value for money [1]. These models explore both the costs and the benefits accrued by patients receiving the interventions and results are presented in terms of the incremental cost per benefit of the intervention under evaluation [2]. Whilst not all policy decision makers and reimbursement authorities conform to the same methodology [3], there has been a substantial growth in the use of incremental cost per quality adjusted life year (QALY) over the last two decades [4]. As the incremental QALY is the denominator in the outputs of the model, results can be sensitive to both the health state utility values (HSUV) used and the methods and techniques used to deploy these values within the model [5].

The different components or stages involved in sourcing appropriate HSUVs (Figure 1) are rarely independent and the process is generally iterative and often challenging [6]. In addition to identifying an appropriate preference-based measure for the particular condition (Figure 1), and satisfying any associated reimbursement agency requirements, factors such as: the advantages (or disadvantages) of collecting utility evidence in randomised clinical trials and other sources [7], the potential need to use a mapping function to predict the required HSUVs [8], and satisfying the exact and often evolving health states used within the DAM all require consideration.

Once the most appropriate HSUVs available have been identified, these may not match the requirements of the DAM exactly and analysts may then need to make a series of methodological decisions or assumptions related to the practicalities of using this evidence within the DAM. These may include adjustments to account for age, gender or adverse events, evidence for the baseline or counterfactual (i.e. the trajectory of HSUVs for people who do not have a particular health condition or clinical event), estimate HSUVs for comorbidities (when a second health condition is present concurrently with the primary health condition), and characterising uncertainty appropriately (when using summary statistics sourced from different studies) (Figure 1).

2. Existing recommendations on best practice

2.1 Recommendations for decision analytic models

Guidance on appropriate DAM approaches are available in the literature [1;2;9;10;11] and cover: the choice of model structure [12], measuring and estimating costs [13;14]; identifying, reviewing and synthesising evidence on HRQoL [6;15], and methods for characterising uncertainty [16]. In addition, a

number of checklist have been published over the years to encourage good practice in this area [1;2;9;17;18].

It is worth noting that there are issues where best practice can be defined and issues for which countries may have legitimately different preferences (such as the use of public or patient preferences). Both the US Panel on Cost-effectiveness in Health and Medicine, and the UK National Institute for Health and Care Excellence (NICE) have developed guides with a view to standardising the methodological approaches in health technology appraisals to facilitate comparability of submissions [19;20]. However, these and similar guidelines in other settings do not provide detailed guidance on good practice for HSUVs other than stating a preference for a particular preference-based measure (e.g. EQ-5D), source of evidence (e.g. patient), methodology (e.g. time trade-off) and source of preference-weights (e.g. general population or patient), or specifying a specific output form the models (e.g. cost per QALY).

2.2 Recommendations for health state utility values

A series of technical support documents commissioned via NICE's Decision Support Unit in 2010 provide some recommendations of good practice when using HSUVs [6;21;22;23] and a recent textbook provides an up to date review of preference-based measures of health [24]. More recently, their use in DAMs has formed the focus of two ISPOR task force reports [7;8]. The first describes a framework for collecting HSUVs within clinical studies and covers a broad spectrum from early planning of requirements, inclusion in clinical trial protocols, the advantages and design of alternative sources such as prospective and cross-sectional observational studies, statistical analyses and reporting standards [7]. The second provides recommendations relating to conducting high quality mapping studies involving HSUVs and includes: good practices for the selection of datasets used to obtain the mapping function, model selection and performance assessment, the use of results (including characterising variability and uncertainty appropriately), and reporting standards [8].

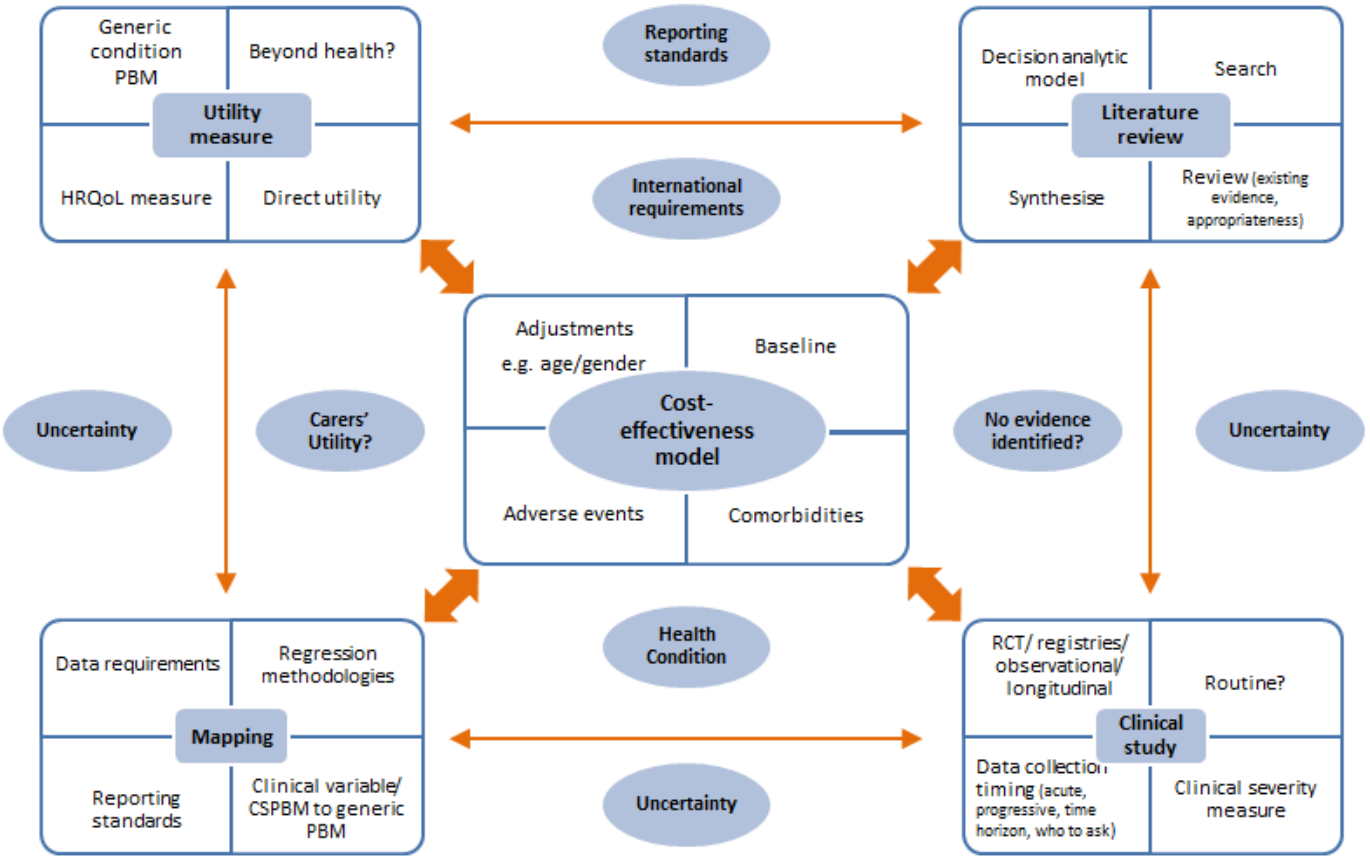
3. Standardising good practices

Good practice recommendations and checklists have evolved over time to address the developments in methodological research and the needs associated with the increasing complexity of DAMs. Consequently there is no common source that covers the state of the art recommendations across the full spectrum of considerations associated with the use of HSUVs informing DAMs and there are still areas where recommendations for good practice are absent. The

result of this is there is no standardisation across current practice and policy decisions informed by the results from these models may be suboptimal.

The articles in this special issue provide a comprehensive compilation of the most robust recommendations in this area and are intended as a reference source for state of the art evidence on good practice across the full spectrum of needs associated with HSUVs that inform DAMs.

Figure 1: The iterative process involved in identifying, analysing and using health state utility values used in decision analytic models



CSPBM – condition specific preference-based measure; **PBM** – preference-based measure; **RCT** – randomised controlled trial; **HSUV** – health state utility value

Disclosure statement

This article is published in a special edition journal supplement wholly funded by Takeda Pharmaceutical International AG, Zurich, Switzerland.

Acknowledgement

The authors would like to thank Prof Jon Karnon, PhD of The University of Adelaide and Dr Andrew Lloyd, Phd of Bladen Associates Ltd for their editorial review.

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