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# **OUT OF DATE OR BEST BEFORE? A COMMENTARY ON THE RELEVANCE OF ECONOMIC EVALUATIONS OVER TIME**

**Short title:** The expiration date of an economic evaluation

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## **Abstract**

The impact of time on the applicability and relevance of historical economic evaluations can be considerable. Ignoring this may lead to the use of weak or invalid evidence to inform important research questions or resource allocation decisions, as historical economic evaluations may have reached different conclusions compared to if a similar study been conducted more recently. There are multiple factors that contribute towards evidence becoming outdated including changes to the relevant decision problem (e.g., comparators), changes to parameters (such as costs, utilities, and resource use) and methodological updates (e.g., recommendations on uncertainty analysis). Researchers reviewing economic evaluations need to consider whether changes over time would influence the study design and results if the evaluation were repeated, to the extent that it is no longer helpful or informative. In this paper we summarise these key issues and make recommendations about how and whether researchers can future-proof their economic evaluations.

## **Key points**

- Economic evaluations invariably become less relevant to the present day over time, as data availability, preferred methods, clinical pathways, and costs continue to change.
- In this commentary we reflect on the range of issues which affect the timeliness of an economic evaluation and reflect on steps that individuals conducting economic evaluations could take to improve the future relevance of their studies.
- The shelf-life of an economic evaluation is highly dependent on a range of factors and will differ greatly across contexts (such as by disease areas and intervention type), reviewers and decision-makers need to carefully consider this when evaluating economic evaluation evidence.

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## 1. Introduction

Checklists for critically appraising the robustness/quality and transferability of economic evaluations, including the relevance of research questions and parameters, are commonly used [1–6]. What is less clear however, is for how long the results of a particular economic evaluation remain useful and relevant. Economic evaluations will inevitably become outdated due to various factors that can occur concurrently, including changes to methods and in parameters relating to baseline event rates, resource use, unit costs and effects. As this occurs the evidence may become less relevant to decision makers and might need to be disregarded. Systematic reviews of economic evaluations need to consider the impact of time yet, as far as we are aware, there is no guidance on appropriate timeframes for systematic reviews of economic evaluations or comprehensive summaries discussing how evidence may become outdated. Luhn et al., discussed that a common limitation noted in systematic reviews of economic evaluation is the time frame covered by searches, however, this was not discussed further [7]. Recently, an ISPOR task force report on systematic reviews of cost and cost-effectiveness studies noted that generalisability may increase when restricting by publication date, due to changes in standard care, methods and parameters [8]. However, it did not provide a comprehensive overview of these factors.

Health technology assessment (HTA) bodies increasingly compare cost-effectiveness estimates with a predefined value for money threshold, to determine whether an intervention is worth paying for. Therefore, economic evaluations have prime importance in how healthcare resources are allocated. However, evidence from economic evaluations can become outdated quickly. This commentary paper aims to summarise the factors contributing to economic evaluation evidence becoming out of date, how these can be considerable and why researchers and decision makers need to be aware of them. We provide a summary of the key issues and discuss ways in which individuals conducting economic evaluations can improve the future validity of their economic evaluations.

## 2. Factors to consider when judging whether evidence is current

This section details key factors (i.e. it may not be completely exhaustive) that may result in economic evaluation evidence becoming out of date. The likely relevance and importance of factors will differ across disease areas and intervention types.

### 2.1. *Is the research question still relevant?*

A key question with regards to whether an economic evaluation is out of date is whether the evaluation addresses the research question currently under investigation. Economic evaluations compare the costs and consequences of two or more alternative courses of action [9]. The research question of a study is commonly formulated from the components of the PICO acronym (population, intervention, comparator and outcome). Note that for economic evaluation, the outcome is the type of analysis which responds to the objective of the research (e.g. to assess value for money). If, due to the passage of time, any of these components of the existing economic evaluation do not align with the current research question then it is unlikely that the results of the economic evaluation will be useful.

The population targeted by a healthcare intervention may change over time, affecting baseline health and underlying rates (e.g., background mortality). For example, populations are ageing and living longer, with more comorbidities, which will likely affect underlying rates of health events for many conditions. Changes to diagnostic techniques or definitions will also affect the structure of the population. In recent years research in autism spectrum disorder has considered gender differences, as girls and women with autism might go undiagnosed until later in life as symptoms present differently [10,11]. Likewise, the introduction of precision medicine interventions, or changes to pathways of care, could result in cost-effectiveness analysis focusing on different subpopulations over time.

Changes to alternative interventions available and the delivery of healthcare will affect the relevance of previous economic evaluations. Most obviously, this could influence the choice of comparator(s) used within an analysis (i.e., the “standard of care” in current practice) [12]. Furthermore, these changes will affect other factors relevant to cost-effectiveness analysis, such as pathways of care, sequencing of treatments and concomitant medication, which will affect economic evaluation results. For example, over recent decades there have been substantial advancements related to treating human immunodeficiency virus (HIV) [13]. New treatments have become

available over time and some existing treatments have been removed from the market or are no longer used. This will affect the relevance of comparisons in older cost-effectiveness studies. Furthermore, changes to the treatment landscape may influence cost-effectiveness methodology. E.g., in HIV, early treatments sought to extend survival and reduce HIV-related morbidity, whereas contemporary treatments typically seek to reduce (and eliminate) viral load.

In recent years, there has been more focus given to the role of precision medicine, which can help to target treatments for maximum health gain and/or minimal harm. Introducing precision medicine interventions may alter treatment choices and subsequently impact cost-effectiveness results [14]. For example, in the last decade the treatment of advanced non-small cell lung cancer has evolved substantially from nearly universal systemic chemotherapy agents to include treatments targeted at *EGFR* mutations, *ALK* and *ROS1* alterations, and PD-L1 overexpression [15–17]. Furthermore, different targeted treatments are recommended for different stages, presenting an increasingly complex care pathway.

## **2.2. Does the analysis reflect the current evidence base?**

Even if the research question within an economic evaluation remains relevant, the data used to inform the analysis may be outdated. Updated information relating to costs and outcomes may warrant an update of key input parameters, which can have a marked impact on results.

Regular updating of some commonly cited cost sources may mean that as soon as a study is published, it is already outdated. For example, in the UK common unit costs are updated annually [18,19]. Costs from these published sources reflect data collected from the previous year, meaning that in most cases, even if the most up-to-date reference costs are used, they are reflective of costs derived from two years prior (accounting for the time taken to undertake, and subsequently publish the results of, an economic evaluation). In addition, the costs of medicines may change as drugs become available off patent, biosimilars enter the market, or monitoring requirements change over time. Several studies have updated previous economic evaluations to reflect pricing changes, resulting in changes to results, demonstrating the potential to change cost-effectiveness conclusions [20–23].

Updated clinical data may also affect results of an economic evaluation. A study by Tai *et al.*, (2020) found that 41% of cancer technology appraisals conducted by the National Institute for Health and Care Excellence (NICE) used immature data to inform reimbursement decisions [24]. In a case study, the authors found the incremental cost-effectiveness ratio (ICER) using the original data cut was £99,570; but when re-analysed using a later data cut, was substantially lower at £46,339 [24]. Updates of interim analyses feature frequently as part of NICE technology appraisals, particularly for medicines that are granted interim funding via the Cancer Drugs Fund (CDF) pending further data collection. Furthermore, the release of mature data from open label extension studies may also provide relevant data which changes the results of an evaluation.

It may also be the case that new evidence emerges which calls into question original findings. Economic evaluations of elective surgery versus endovascular treatment (EVT) for abdominal aortic aneurysm have been published, showing that EVT was cost-effective [25–27]. A key assumption made in these studies was an expectation of long-term survival gains since EVT reduced operative mortality; though as long-term data became available, no differences in long-term survival were observed. A recent NICE guideline reported the findings of a *de novo* cost-effectiveness analysis using new data and demonstrated that elective EVT is not cost-effective versus surgery [28].

Quality-adjusted life-years (QALYs) require health state utilities, which require preference weights. The EQ-5D is a commonly used measure of health status, though there are many different value sets available [29]. Using NICE as an example, the three-level (3L) crosswalk for the five-level (5L) version of the EQ-5D is currently recommended, though the methods guide consultation proposed a change this, and work is ongoing to develop a new value set [30,31]. Changes to preference weights applied will affect utilities and subsequently ICERs, as has been described in the literature [32].

Updated inputs could increase or decrease the estimated ICER. However, the combined effect of updating multiple model parameters could result in unchanged cost-effectiveness estimates (i.e., multiple changes may effectively cancel each other out). It may not be possible therefore to understand in which direction cost-effectiveness results may change without having performed the update, particularly for non-linear models. If only some sources can be updated with others left unchanged, the overall evidence base for the evaluation may

reflect only a partially updated analysis. E.g., if survival data are updated, but duration of treatment data are left unchanged, the resultant ICER may be misleading. HTA bodies seek to produce timely guidance shortly after marketing authorisation, which makes them more reliant on early and interim data cuts, with resulting parameters potentially becoming outdated quickly.

Whilst some of the changes noted may have negligible impact on cost-effectiveness results, the combined impact of multiple changes may have a more substantial impact over time. For reviewers to be able to consider and evaluate the impact of parameter changes, it is imperative that cost-effectiveness analyses are referenced well (with the original sources cited). Furthermore, it is worth considering that publication dates do not necessarily correspond to the dates of the data used. When applying a restriction based on publication date, this may neglect to consider that the data used within an analysis could be much older.

### **2.3. *If repeated, would the same methods be used?***

Methodological and computational advancements, and updates to methods guidelines and best-practice standards, may mean that if historical economic evaluations were repeated today, different techniques would be used. Over time, HTA bodies update their decision-making methods and criteria to reflect changes in methodology, social values or changing healthcare priorities. Since new interventions must often obtain positive HTA guidance to enable access to patients, the methods required by a HTA body are highly likely to influence those used by published economic evaluations for a particular setting. For example, over time, cost-minimisation analysis has fallen out of favour as it is rarely justifiable [33]. In 2012, NICE's methods guide for public health included cost-consequence analysis and cost-benefit analysis [34], whereas the unified guidelines manual (used from 2015) specifies cost-utility analysis should be used in the base case [35].

A HTA body might make a substantive change to its preferred reference case analysis, such as adopting a different perspective. For example, the Panel on Cost-Effectiveness in Health and Medicine in the US in 1996 recommended a societal perspective should be used, but updated this in 2016 to recommend the inclusion of both a health sector reference case and a societal perspective [36,37]. Permitting the inclusion of wider effects of interventions, for example by including the implications for informal caregiving, labour market productivity or non-health sectors, would almost certainly lead to very different cost and, potentially, QALY results [38,39]. In its draft proposals, NICE has indicated a new openness to the use of real-world evidence (RWE) to inform decision making and suggests increasing the primacy and robustness of probabilistic results [31]. Older economic evaluations might have obtained different cost-effectiveness estimates if they had been empowered to explore methods to incorporate alternative sources of evidence (such as RWE), or if they had been encouraged to conduct more thorough probabilistic analyses (for example, by minimising Monte Carlo error) [40]. Therefore, they might be less comparable with newer economic evaluations. Recommended discount rates have also changed over time. For example, the 3<sup>rd</sup> edition of the guidelines from the Canadian Agency for Drugs and Technologies in Health (2006) specified a discount rate of 5% in the reference case [41], which was changed to 1.5% in the 4<sup>th</sup> edition (2017) [42]. In Norway, guidelines were updated in 2018 to specify a discount rate of 4% for costs and benefits accrued up to 40 years, 3% between 40 and 75 years, and 2% after [43]. In some instances, updates to guidelines may change the recommended approach to various aspects of economic evaluation. In the Netherlands, a unification of HTA guidelines in 2016 changed various aspects of existing references cases including recommending longer time horizons, specifying cost-utility analysis as the recommended analysis type, and specifying that the EQ-5D 5L should be used with the Dutch valuation set [44].

Over the years there have been changes regarding the use of trial-based analysis and model designs. For example, it has been discussed that trial-based economic evaluations may not be sufficient for all research questions, which may have partially contributed to the increased use of decision analysis [45]. Further, there have been many advancements to modelling methodology. For example, version 4.0 of the guidelines from the Pharmaceutical Benefits Advisory Committee in Australia (2006) refers only to trial-based analyses and cohort models (decision-tree and state-transition), whereas version 5.0 (2016) also discusses individual-level/microsimulation approaches [46,47]. Developments in modelling methodologies may be particularly common in certain disease areas, such as the increasing use of individual models in rheumatoid arthritis [48], or the use of state-transition models instead of partitioned-survival models in cancer [49]. If the characteristics of the model structure mean that more sophisticated modelling approaches are required, it is unlikely that the results can be considered comparable to those generated by simpler models. A comparison of partitioned survival analysis and state-transition models in cancer found that the choice of model structure had a large

impact on cost-effectiveness results [50]. There have also been changes to the recommended methods for trial-based cost-effectiveness analysis, such as how missing data are addressed [51].

Cost-utility analyses may use different measures of utility. As noted in the previous section, the use of updated value sets can lead to changes in cost-effectiveness results. There have also been significant changes to the measures used; for example, the introduction of the 5L version of the EQ-5D which was made to overcome some of the limitations of the 3L version, but has been noted to result in differences to utility values and subsequently ICERs [52–55]. In mental health, the Recovering Quality of Life (ReQoL) measure has been introduced and has recently published preference weights, which may affect utility values in the future [56]. There is a growing literature on the use of capability measures in economic evaluations, which moves away from the QALY framework [57]. These examples demonstrate that there have been significant changes to methods used to measure health in economic evaluation and that the area is continually evolving.

Evidence synthesis techniques have also advanced over the years. For example, the growth in the use of population-adjusted indirect comparisons or the development of fractional polynomial (FP) network meta-analysis (NMA) for survival analysis [58,59]. Where standard NMA techniques require assumptions to be made, the application of more advanced techniques may be expected to lead to different effectiveness estimates and hence different cost-effectiveness results. In practice, this may depend to what extent this is a key driver of the ICER (e.g., in the NICE CDF review of TA593, the committee noted that the Bucher and FP NMAs had similar effects on the ICER [60]). Similarly, more flexible methods for extrapolation of survival data may lead to different long-term effectiveness estimates and therefore be expected to generate different ICERs, for example accounting for 'cured' patients drastically affected the ICER for ipilimumab [61–63].

While some of these elements may not lead to substantive changes in cost-effectiveness results, they may cause difficulty in determining how transferable the conclusions of older economic evaluations are (unless this structural uncertainty is explored in sensitivity analysis). Simply assuming different methods would lead to identical results is unlikely to be appropriate.

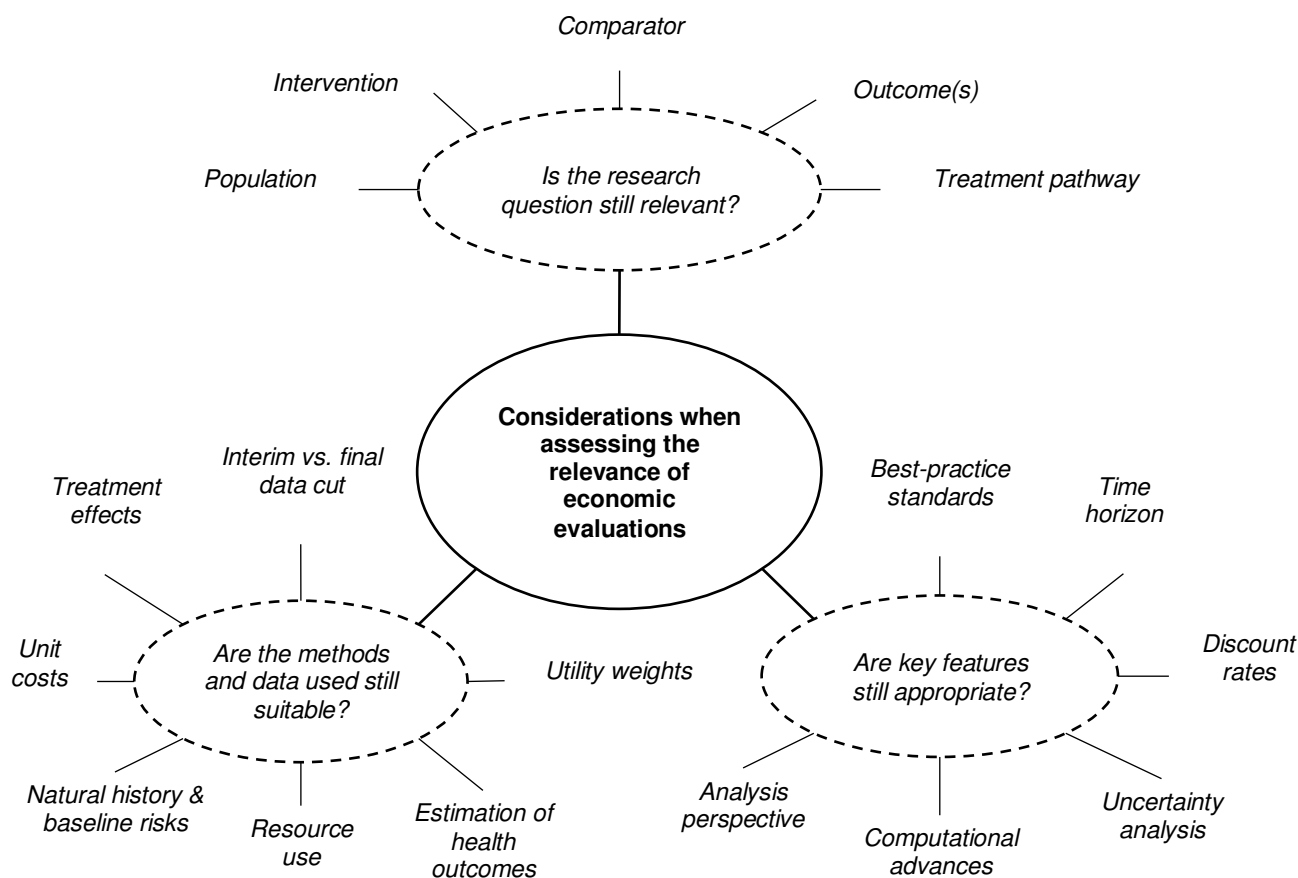
### **3. Assessing the timeliness of economic evaluation**

The factors outlined above all contribute to the results of economic evaluations losing validity over time, the impact of which can be substantial. High-quality economic evaluations should use the best evidence available at a point in time. Similarly, systematic reviews of economic evaluations provide a snapshot of analyses that have already been conducted. To some extent therefore, such studies might be considered out of date as soon as they are published. It is likely that the cost-effectiveness estimates, and review conclusions will become outdated over time, and studies may age particularly badly where the original research question has become obsolete, critical new information has become available, or methods guidance has changed substantially. However, this does not always mean their results are necessarily irrelevant or unhelpful.

Naturally, researchers and decision makers will assess the relevance of results of economic evaluations to their own decision problem or research question. Inevitably, some pragmatism will be required; for example, if there is a paucity of evidence and no feasibility of collecting new evidence, then it would be unhelpful to ignore a published economic evaluation or systematic review; rather, a judgement would need to be made regarding the applicability of the results. It is worth considering that creating new evidence and updating existing evidence will often be restricted by resource, cost and time constraints.

The assessment of whether the results of an existing economic evaluation are relevant to a current decision problem is highly dependent on the context. It is therefore not possible to give a set amount of time after which an economic evaluation would be deemed out of date or to state that one particular transferability issue is critical across time. The decision as to whether an evaluation is relevant will be a subjective one made by the researchers based on their perception on whether differences in the research question, evidence base, and methods used in the studies are critical or not. If researchers decide to omit evidence as they deem it to be out of date, it is important that they are transparent in their reasoning for doing so and reflective in their own role in this decision. Figure 1 presents a summary of the key issues presented in the text above. These elements, and their relative importance, are commonly reported in existing checklists to assess the quality and transferability of economic evaluations. **Figure 1**

**Figure 1 Key considerations in the assessment of the relevance of economic evaluations over time**



Whether an issue is deemed critical can be determined by considering the likely direction and magnitude of change, though this may not always be clear. Researchers need to consider whether the identified changes over time would potentially influence the study design and results, to the extent that it is no longer helpful for decision making. For example, a critical issue may occur when the comparator used in an evaluation is no longer part of clinical practice, or if a modelling study used historic utility weights that differ meaningfully from more recent weights obtained using more robust methods. Conversely, if an evaluation used interim trial data which has since been superseded by a final data cut, it may be considered a non-critical issue if the relative effectiveness estimates from the 2 data cuts are similar. Sometimes, the impact of issues will not be clear and predictable (e.g. if the evidence is affected by multiple changes, including changes to parameters and methods). In these cases, proposing comprehensive updates to existing evidence or future research is likely to be the best course of action. The figure above can help researchers make a judgement about whether evidence is still useful for decision making or helpful to include in a systematic review. Our intention is not to suggest that answers to the questions will provide the researcher with a relevant timeframe for their review or a dichotomous answer to the question of relevance. Rather, it should be a useful thinking aid to support researchers in reaching a judgement as to what is and is not appropriate.

#### **4. Can we future-proof economic evaluations?**

Rapidly changing treatment landscapes, evidence bases, and economic evaluation methods mean that it will never be possible to fully future-proof an economic evaluation. However, we recommended that researchers take steps where possible to present scenarios that may be applicable in future, and to enhance the feasibility of updates to studies.

There may be some instances in which researchers can anticipate changes that may be implemented which would affect their economic evaluation. For example, as previously noted, the NICE methods guide consultation proposed a change to the method for estimating utilities and researchers could present multiple sensitivity

analysis using different methods to mitigate the impact of this change on their economic evaluation [31]. Transparent and comprehensive reporting using existing checklists is recommended [1,2]. Ensuring economic evaluations are transparently reported in line with current guidelines will allow evidence users to more easily appraise the evidence, methods, and assumptions underlying an evaluation and to judge whether it is still relevant given temporal changes. A move towards making data and decision-analytic models openly available alongside published journal articles or in specialise repositories would aid the research process [64]. This would save researchers looking to update existing economic evidence a significant amount of time and resources in allowing them to adapt and update existing model structures and evidence rather than going through the process of creating new models.

## **5. Conclusion**

No guidance exists on appropriate timeframes for systematic reviews of economic evaluations, or when we can consider that an economic evaluation is no longer relevant, yet the impact of time on the applicability and relevance of a historical economic evaluation can be substantial. In this paper we have summarised those key issues and presented a simple framework to aid researchers and decision makers in considering whether the research question, parameters, methods, and therefore the results of a historical economic evaluation are relevant at the current point in time. While there is no easy answer that can be applicable across all areas, as the shelf-life of an economic evaluation will vary across contexts (disease and/or intervention), we hope this framework can serve as a useful guide.

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