The Who, When and How of Model Validation: A Targeted Literature Review of Roles and Opportunities for Improving Credibility Through All Phases of Model Development



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A targeted literature review was conducted via an electronic search in PubMed followed by citation searching of key papers.

Methods

- Additionally, model validation documents published by national HTA bodies were igated
- terms. dation

 to trust model results. While most models undergo some level of validation, recommendations around how, when, and by whom it should be conducted vary widely. Objective: To assess current literature and summarise published guidance on HE model validation. 	 Additionally, model validation documents published by national HTA bodies were considered. The national bodies were identified from a study which investigated international HTA systems from 32 countries.¹ The PubMed search included HE and modelling terminology alongside validation terms. Exclusion criteria included non-HE model types and missing discussion of validation methodology.
Re	sults
 Search and screening A total of 346 studies were identified following the PubMed, citation and national HTA body searches. 	 The 22 sources covered 21 unique approaches, which were categorised as conceptual guidance (n=14) and specific validity tools (n=7)²⁻⁸ (Figure 2). Study authors referenced external data sources including structured literature reviews (n=7) or non-author HTA experts (n=12) (Figure 3).

- After title and abstract screening, 317 studies were excluded. Following full-text review, eight articles were identified from the PubMed search, eight from citation searches and six documents from HTA bodies (Figure 1).
 - Most approaches (n=15) reported technical validation methods that required a modeller

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Across all studies, key reasons for exclusion were non-English (n=8), no validation methods (n=30) and unsuitable study design (n=16).

Introduction

Health economic (HE) decision-analytic models are a critical component of health technology

assessments (HTAs), and evaluation of model validity can support a decision-maker's ability

Figure 1. PRISMA diagram							
	Identification of studies via PubMed			Identification of studies via other methods			
Identification	Records identified from PubMed electronic database searches (n=346)	Records removed before screening: Duplicate records removed (n=1)		Records identified from: Citation searches (n=16) HTA bodies			
	Records screened (n=345)	Records excluded (n=317)		(n=32)			
screening	Reports sought for retrieval (n=28)	Reports not retrieved (n=0)		Reports sought for retrieval (n=48)		Reports not retrieved (n=0)	
	Reports assessed for eligibility (n=28)	Reports excluded No validation (n=12) Study design (n=8)		Reports assessed for eligibility (n=48)		Reports excluded (n=34): Non-English (n=8)	
ncluded	Studies included in review	4				No validation (n=18) Study design (n=8)	
E	(n=22)						

Abbreviations: HTA, health technology assessment.

- with expertise to conduct.
- Six provided guidance for decision-makers or model users to check face validity and reporting. Two described the role of clinical experts in validation and how to assess models based on clinical understanding (Figure 4).



	portional						
Assess fit for decision problem	To compare the model components to the proposed research question and ensure all aspects are answered.	11 studies	Model developer				External assessor, model user, decision maker
Black box testing	To test the model behaviour through the changing of external input values and observing the impact on the	12 studies		Model developer	Model developer External assessor		External assessor
Grey-box testing	To assess the data imputation methods using actions such as duplicate programming.	9 studies		Model developer	Model developer External assessor	Model developer External assessor	External assessor
White box testing	The testing of internal model coding and programming, conducted through methods such as cell by cell formula checks in Excel.	9 studies		Model developer	Model developer External assessor	Model developer External assessor	External assessor
Cross-model comparisons	Structural and results validation through comparison to available evidence and clinical evidence.	11 studies	Model Model developer External assessor		Model developer External assessor		External assessor, decision maker, model user
Transparency actions	To test model transparency though observing the use of input sources, justification of assumptions, uncertainty analysis and the highlighting of potential bias.	6 studies	Model developer			External assessor, decision maker	
Post- completion testing	To assess the model by an independent expert modeller(s), clinical expert(s), and sometimes a decision maker (e.g., NICE). Key action by model developer	17 studies • Kev	v action by external party (non-developer)			External assessor, decision maker pert	



Abbreviations: HTA, health technology assessment.

Conducting a validation

Building a HE model can typically be broken down into five distinct stages, with different validation methods recommended for each one (Figure 5).

Figure 5. Stages of validating an HE model						
 The assessment of the decision problem Observation of the model by a clinical expert Cross model-comparison of structure Outcomes compared to available clinical data for 	or relevance checks	 Black-box testing White-box testing Grey-box testing Cross-model comparison of result 	S	All previous tests repeated by an independent expert modeller, clinical expert, and decision maker.		
Conceptualisation The research and planning of the model structure and design	Programming The model is built in a software (e.g. Excel) by applying calculations to inputs to generate results	Finalisation The model is producing results which can be validated	Reporting Happens alongside finalisation, but with a focus on model transparency	Post-completion All work by the model developer is complete, including any modifications following feedback		
 Key action by model developer Key action by external party (non-developer) Key action by clinical expert 	 Black-box testing White-box testing Grey-box testing 		 Clear referencing Justification of assumptions Uncertainty analysis 			

Conclusions

- Current literature provides a range of theoretical frameworks and assistive tools to guide the critical assessment of HE model validity.
- Most validation approaches were found to be suitable for the final stages or after the full completion of the economic model, with a notably lower number of publications in methods applicable during the model conceptualisation and development phases.
- A key consideration of this study is that it was conducted as a targeted review rather than a full systematic literature review. Consequently, it may not capture every study in the literature.
- This study can help modellers, assessors, and model users better understand role-specific validation recommendations across different stages of model development.

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