

Frameworks for the use of AI within HEOR: A targeted literature review and thematic analysis

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KEY TAKEAWAYS



80% noted the importance of **transparency and reporting** when AI is used but only 50% of these explicitly stated what should be reported

Recommendations to consider the rationale for using AI was identified in 35% of records:

1. Other options should be considered as AI may not be the most suitable solution
2. Multiple stakeholders should be involved in the decision to use AI
3. The need to follow a framework is itself dependent on risk



70% stated that users are **ultimately accountable** for all outputs but only 40% of these provided detail on the level of human oversight



65% discussed **validation** of AI algorithms and testing of outputs but only 20% of these gave recommendations for how this should be done

CONCLUSIONS

- Core principles are useful to align frameworks, and clear task-driven guidelines are also beneficial
- Lack of practical guidelines is a manifestation of the varied applications and fast-evolution of AI systems
- Emphasis on reporting is driven by the scientific principle of reproducibility, but this is inherently more challenging with the lack of explainability of AI algorithms
- Organisations should take a case-by-case approach in line with risk-based assessment frameworks
- Learning pathways are equally important to frameworks: they are essential to support appropriate use and integration of AI systems

INTRODUCTION

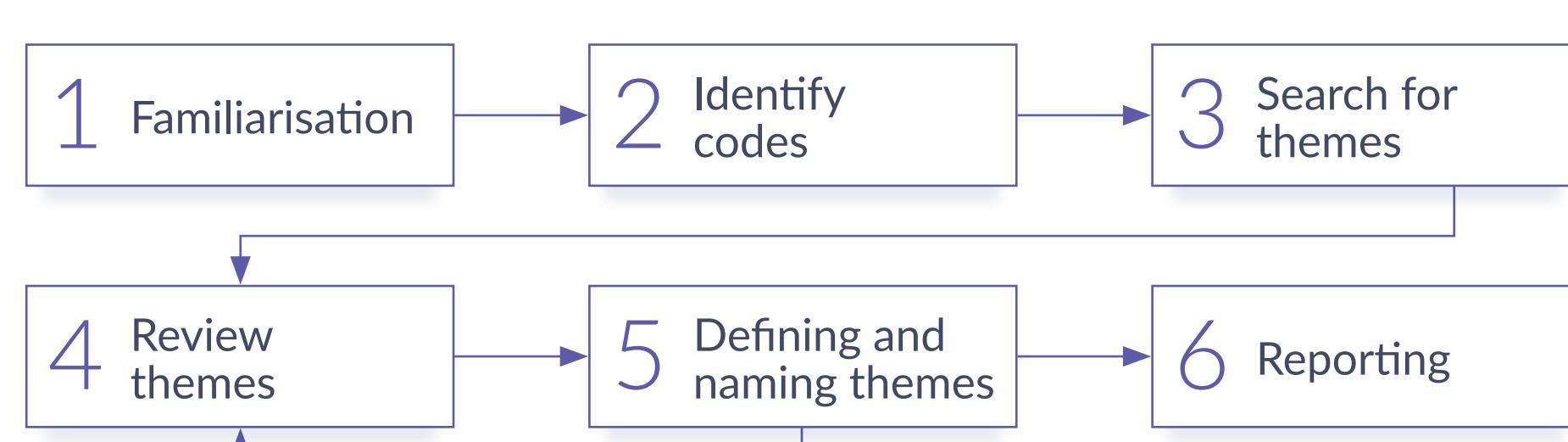
- AI is any computer system that can perform complex tasks that would typically require human intelligence, such as pattern recognition, decision-making, and problem-solving¹
- Many tasks arise from the HEOR and market access industry where AI has the potential to assist and improve on traditional methods. A few key examples include evidence review (sourcing and summarising evidence), code generation (VBA, R, Python), data analysis, and pricing strategy development

OBJECTIVES

- To understand what guidance is available on the use of AI within HEOR, and to evaluate what guidance is necessary

METHODS

- A targeted literature review (TLR) was conducted to find evidence of guidelines, recommendations, or critiques, of the use of the AI within HEOR and market access
- Structured searches were conducted on 26th June 2025 in MEDLINE, with supplementary searches of Semantic Scholar, WHO, FDA, EU parliament, UK gov, NICE, ISPOR, and Cochrane
- A single reviewer screened all records with a 10% validation check from a senior reviewer; enough clarity was provided in the abstracts that a second pass screen of the full text articles was not deemed necessary
- Thematic analysis was conducted on the relevant records according to Braun and Clarke guidelines²



• TLR identified 34 records (Figure 1)

Figure 1. Flow of literature



LIMITATIONS

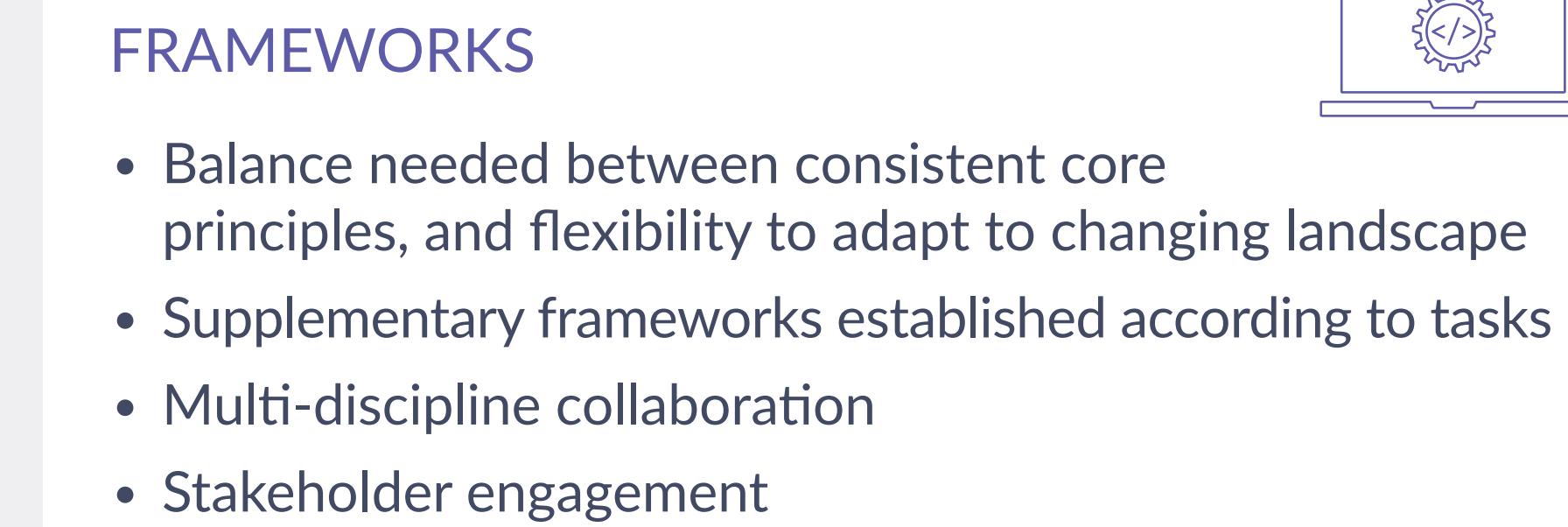
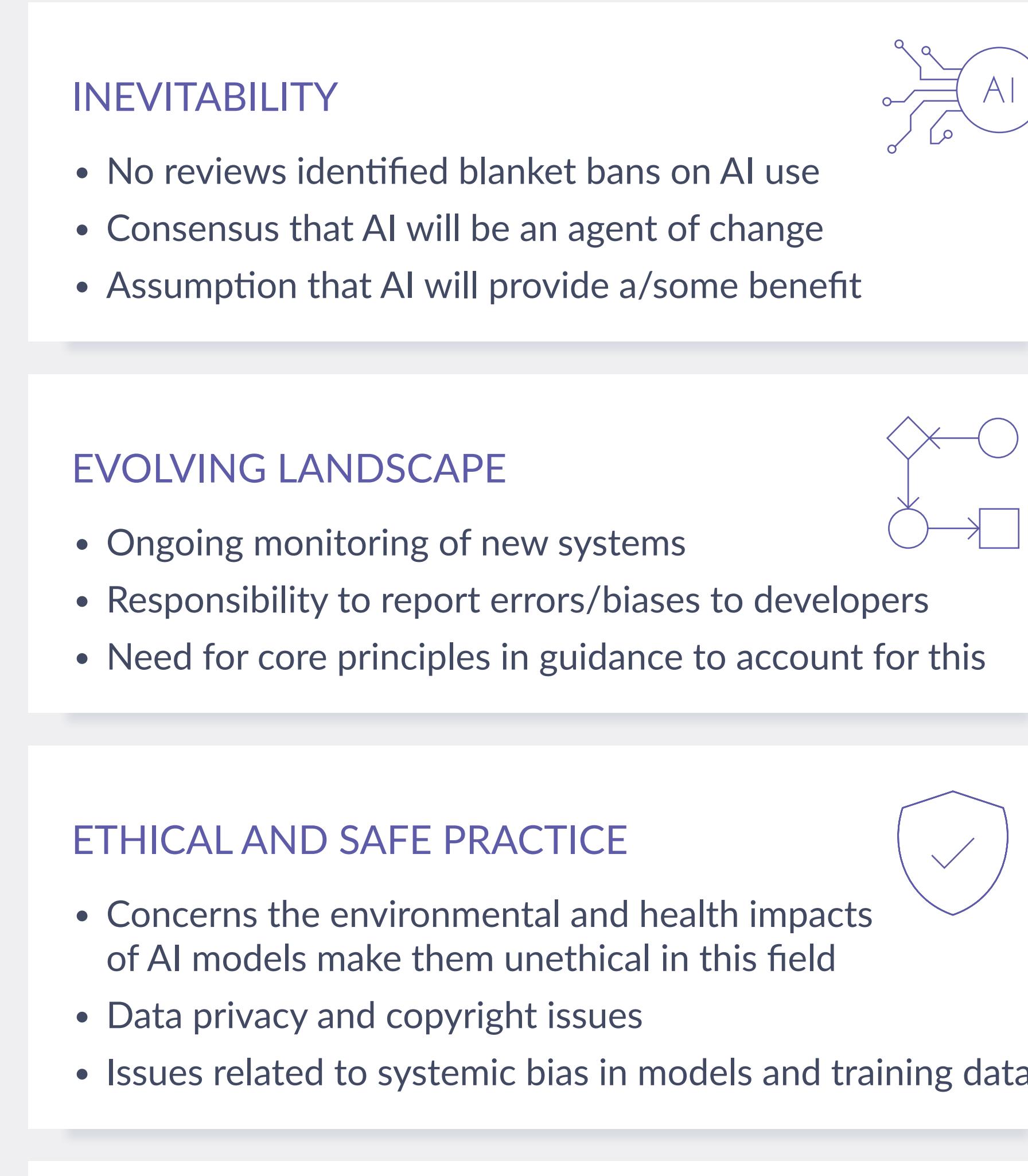
- Not a comprehensive review. Citation lists of articles revealed several missed guidelines and frameworks.
- Heterogeneity in the objectives and methods of the articles reviewed limit the synthesis

RESULTS

- 65% records were secondary reviews or commentaries on guidelines: more theoretical than practical
- Primary frameworks/guidelines identified (35% records) included:
 - Broader guidelines with elements relevant to HEOR, e.g., EU³, UK⁴, FDA⁵, WHO⁶
 - HEOR related policies, and task-specific frameworks, e.g., NICE⁷, ISPOR⁸, RAISE⁹, Hamel¹⁰, Fleurence¹¹, Hasan¹²

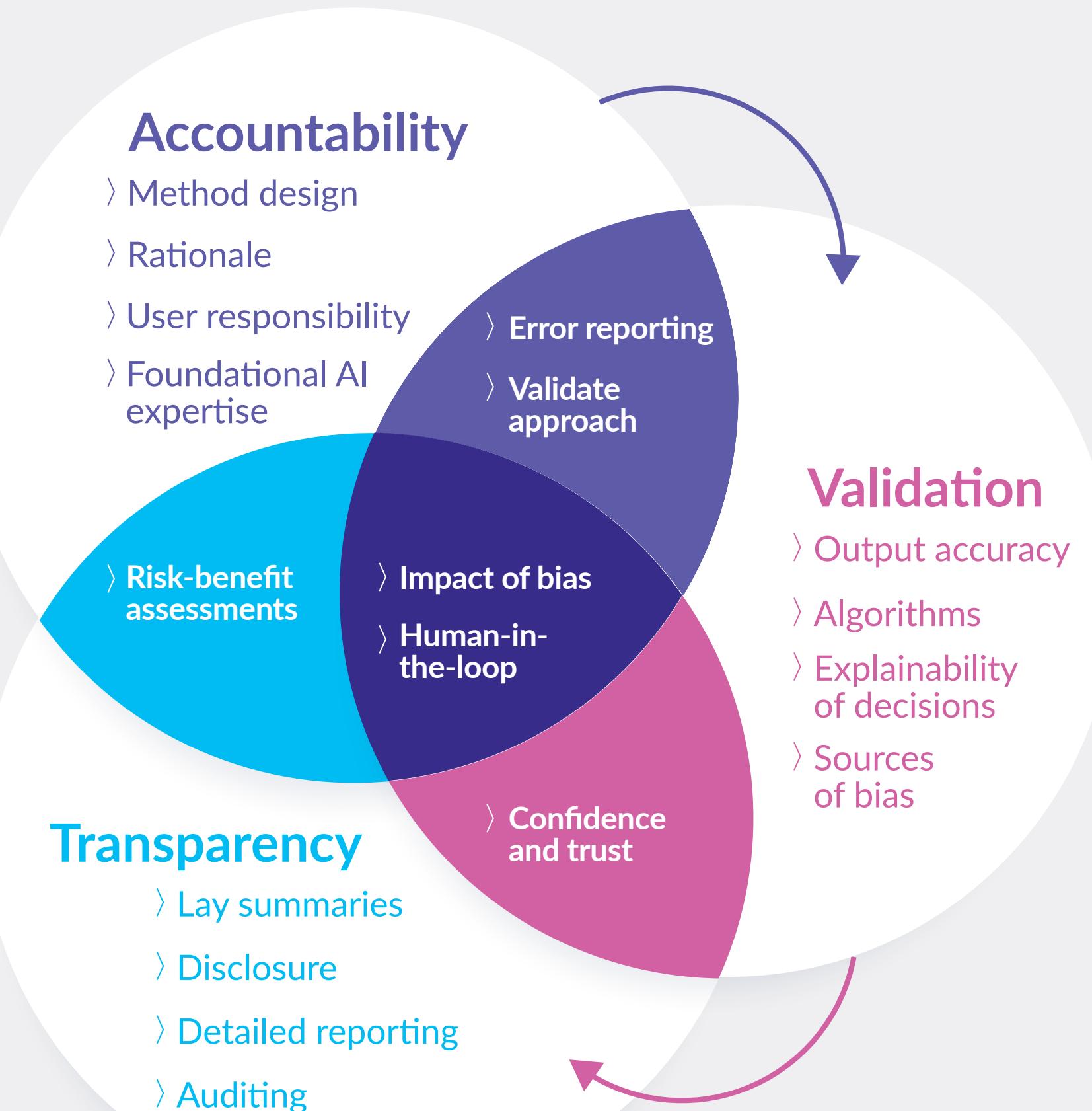
Relevant themes and sub-themes, derived from 29 records, fell into two overarching categories:

1 Underlying principles on which interactions with AI should be based



- Several sub-themes discussed overarching considerations and principles that should be considered at all stages of interacting with AI
- Limitations and challenges in existing guidance/frameworks:
 - Lack of specificity
 - Inconsistent expectations
 - Heterogeneity of AI models and applications
 - Lack of consensus on what defines a trustworthy AI system

2 Practical steps and considerations to be followed in the use of AI within HEOR and MA



- Practical steps and considerations spanned all stages from project conceptualization through to external review
- Consensus that users are accountable, with suggestion that developers are too, and an onus on users to report errors back and contribute to improved performance
- The various sources of bias were a key concern
- Validation needs to be specific to the use: consider the algorithm, training data, and implementation

“ no consensus on what defines a trustworthy AI, let alone how to measure it ”

Hartung.¹³

“ preservation of critical thinking abilities and ethical writing practices must be balanced with the advantages of efficiency to maintain the integrity of academic research ”

Alnaimat et al.¹⁴

ABBREVIATIONS

AI, artificial intelligence; EU, European Union; FDA, Food and Drug Administration; HEOR, health economics and outcomes research; ISPOR, International Society for Pharmacoeconomics and Outcomes Research; MA, market access; NICE, National Institute for Health and Care Excellence; RAISE, Responsible AI Governance and System Engineering; TLR, targeted literature review; UK, United Kingdom; WHO, World Health Organisation

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