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Application and future potential of Generative Artificial Intelligence (Gen AI) and Large Language Model (LLM) in Health economics and outcomes research (HEOR)

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KEY FINDINGS & CONCLUSIONS

- The integration of LLMs and GenAI is expected to be a critical innovation in the field of HEOR enhancing efficiency, accuracy, and adaptability of modeling and evidence synthesis approaches.
- Our review underscores the practical utility of AI-powered solutions and reporting within health economic research. However, the findings also highlight the importance of thoughtful prompt design, careful tool selection, and continuous human involvement to uphold quality standards.
- As AI continues to evolve, future research should aim to address current limitations, broaden database searches, and foster collaboration between technology and human expertise to ensure comprehensive and robust health economic outcomes.

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INTRODUCTION

Generative artificial intelligence (GenAI) refers to a deep learning model that can produce text, images, computer code, and audio-visual content in response to prompts and are powered by foundation models[1]. Large language models (LLM) serve as foundation models, providing a basis for a wide range of natural language processing (NLP) tasks[2]. LLM, as one specific application of GenAI, are specifically designed for tasks revolving around natural language generation and comprehension. LLM learn statistical patterns, grammar, and semantics from vast text datasets to predict relationships between words and phrases[1,2].

GenAI and LLMs have emerged as key areas of innovation and discussion within numerous industries and disciplines, including healthcare, economics, and research. In the domain of Health Economics and Outcomes Research (HEOR), current practices strongly depend on human expertise, including critical thinking, domain-specific knowledge, and nuanced decision-making to evaluate health interventions, policies, and outcomes effectively. GenAI and LLMs are being examined for potential uses in HEOR, including automating large dataset analysis, processing clinical outcomes, supporting decision modeling, and generating evidence-based insights. Their use is currently at an early stage, and standardization of applications has not yet been achieved[3,4].

While GenAI and LLMs show significant promise in automating large dataset analysis, synthesising evidence, and supporting modelling, their adoption in HEOR is hindered by concerns regarding accuracy and the necessity for expert oversight. Current HEOR practices rely heavily on human expertise, and GenAI/ LLM applications are still at a nascent stage, with no established frameworks or guidelines to ensure their responsible and effective integration. This highlights the need for robust validation methods, clearer standards, and collaborative efforts among researchers, health economists, artificial intelligence (AI) developers, and regulatory agencies to bridge the gap between technological potential and practical, reliable use in HEOR.

This lack of established guidelines or frameworks implies that these technologies are not yet seamlessly integrated into HEOR workflows. Understanding the current landscape of GenAI and LLM usage is warranted to gain an understanding of how the field of HEOR is leveraging these innovations. This can further help relevant decision makes and key opinion leaders to critically evaluate what are the current strengths and limitations of using GenAI and LLM and pave the way for how their usage in HEOR can be standardized.

OBJECTIVE

The objective of this study was to evaluate the current applications of GenAI and LLMs in HEOR focusing on strengths and limitations that were observed during their applications specifically in health economic modeling and evidence synthesis.

METHOD

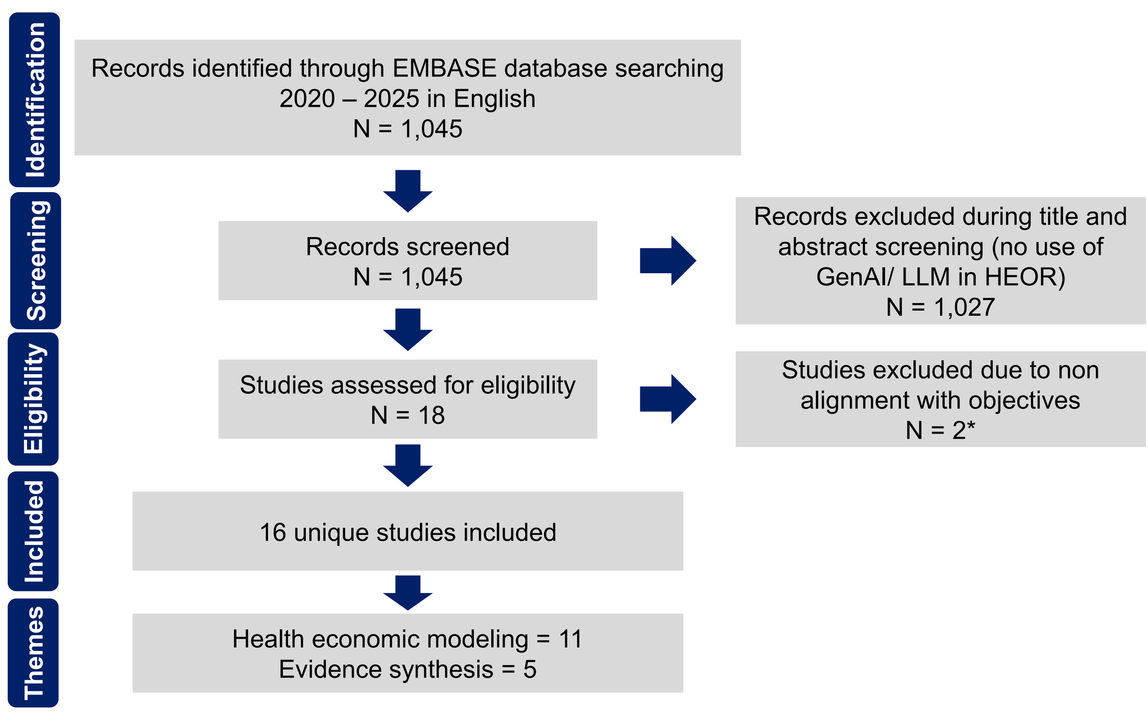
A targeted literature review (TLR) was undertaken using the EMBASE database to identify and analyze publications focusing on the application of GenAI and LLMs in HEOR. The review aimed to explore how these advanced technologies are being utilized, with a specific focus on their role in evidence synthesis and economic modeling. To carry out the review, a simple targeted search strategy was developed and implemented. This strategy involved the use of carefully chosen key terms and phrases associated with AI, LLMs, HEOR, and economic modeling. These terms were selected to ensure that all relevant literature from various subdomains of HEOR involving AI and LLMs would be captured. The search was conducted in April 2025. The search was restricted to publications from the last five years (2020-2025), English language. We included studies using GenAI and LLMs specifically for improving economic modeling or evidence synthesis processes in HEOR and excluded those that did not address these processes in this context for e.g. an HEOR study which would have used GenAI or LLM to enhance the screening process for the patients would be excluded for our purposes. However, if a cost-effectiveness model was built using the data from the same study and used GenAI or LLM to develop the model, that modeling study was included in our study. Among the total articles the title and abstracts were screened for against the selection criteria by three reviewers. All records that met the inclusion criteria were reassessed in full text independently by all three reviewers. Discrepancies were resolved by agreement or by input from another reviewer. Data extraction was done using a standardized data extraction template.

The studies identified in the review were classified into two principles themes according to the application areas of GenAI and LLMs: health economic modeling and evidence synthesis. Under the umbrella of health economic modelling, the key subthemes included model development, model adaptation, model conceptualization, and model optimisation. For evidence synthesis, the subthemes comprised data extraction and report summarisation.

RESULTS

The TLR yielded a total of 1,045 initial results, which underwent a rigorous screening process for titles and abstracts conducted independently by three reviewers to ensure consistency and quality. Following the initial screening, the reviewers performed a full-text assessment of the relevant articles, resulting in the inclusion of 16 publications. Of these, 5 were categorized under evidence synthesis, while the remaining 11 focused on applications in health economic modeling. The findings emphasized both the potential and challenges associated with using GenAI and LLMs in HEOR.

Figure 1: Flowchart of study selection

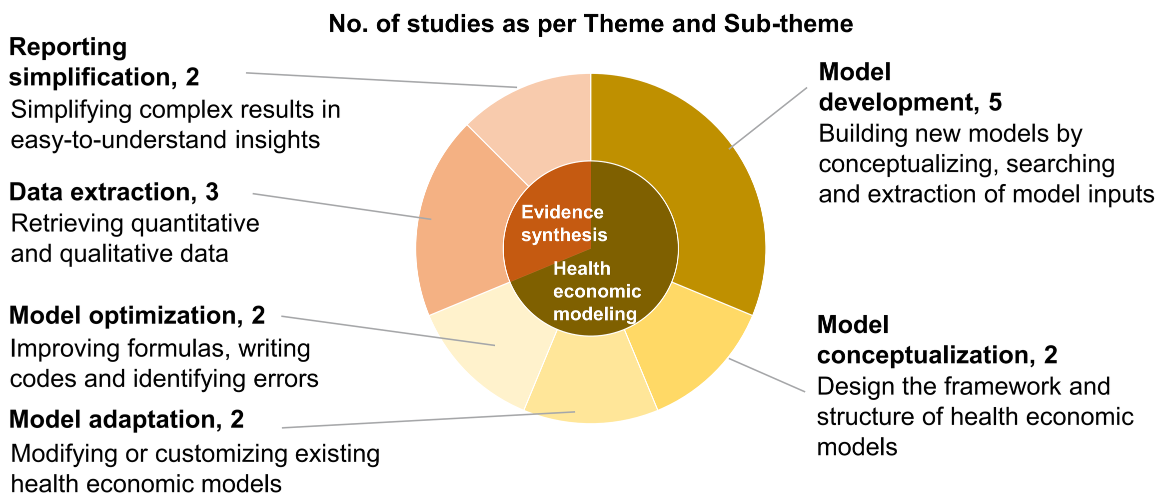


*Please note the 2 articles were removed from the initial approved abstract with 18 articles based on the inclusion criteria.

The subthemes emerged are based on following definitions:

1. **Model development:** Includes building new models by conceptualizing, searching and extraction of model inputs and replicating existing models
2. **Model adaptation:** Gathering inputs from published sources or input files to modify or customize existing health economic models to suit different markets or scenarios.
3. **Model conceptualisation:** Using GenAI to design the framework and structure of health economic models, such as defining health states, transitions, and key parameters.
4. **Model optimisation:** Applying GenAI to enhance, review, or streamline health economic models by improving formulas, writing codes and identifying errors.
5. **Data extraction:** Includes retrieving quantitative and qualitative data from publications, reports, or databases.
6. **Reporting simplification:** Condensing complex health economic model results or evidence into clear, accessible summaries tailored for different stakeholders.

Figure 2: Number of studies as per Theme and Sub-theme



Abbreviations: GenAI: Generative artificial intelligence, LLM: Large language model, NLP: Natural language processing, HEOR: Health Economics and Outcomes Research, AI: Artificial intelligence, TLR: Targeted literature review, GPT: Generative pre-trained transformers, HTA: Health technology assessment, NSCLC: Non-small cell lung cancer, VBA: Visual basic for applications, RCC: Renal cell carcinoma, ICER: Incremental cost-effectiveness ratio, MIUC: Muscle-invasive urothelial carcinoma, NICE: National Institute for Health and Care Excellence, FAD: Final Appraisal Document, BB: Briefing Book, ESA: Early Scientific Advice

#	Study title	Year	Objective of study	Result	Sub-theme
1	Fully Replicating Published Markov Health Economic Models Using Generative AI[5]	2024	Assesses GenAI's **feasibility and accuracy** in replicating health economic models against a well-established benchmark	GenAI **reliably extracted parameters**, yielding monotherapy costs (**8% error**), life years (**0.1% error**), and Incremental cost-effectiveness ratio (ICER) (**2% error**), with **consistent errors across 20 runs**	Model development
2	Development of De Novo Health Economic Models Using Generative AI[6]	2024	Evaluates the **feasibility and accuracy** of using Generative AI to **develop a de novo health economic model**	GenAI **built Markov model** (10–15 states, 22+ transitions), estimated parameters with cited sources, showed structural variability across five runs due to differing references, and **achieved overall face validity**	Model development
3	GPT: The Next Frontier in Health Economic Modeling?[7]	2024	Assess the **feasibility and accuracy** of Generative pre-trained transformers (GPT) in **developing early health economic models** and **predicting cost-effectiveness** for early-stage Health technology assessment (HTA) planning and pricing decisions.	GPT processed diverse datasets to **deliver nuanced early-stage economic assessments**. Scenarios closely matched those from detailed HTA models, and GPT **effectively identified key cost-effectiveness variables and evidence gaps** for HTA submission.	Model development
4	Automating Economic Modelling: A Case Study of AI's Potential With Large Language Models[8]	2023	To **validate** a GPT-4-generated **partitioned survival model** for non-small cell lung cancer (NSCLC) against a published model.	GPT-4 produced a 3-state model script with **results within 1–10% of published outcomes**; differences were due to input data, not model errors.	Model development
5	Artificial Intelligence to Automate Health Economic Modelling: A Case Study to Evaluate the Potential Application of Large Language Models[9]	2024	Assess if GPT-4 can automatically **program published health economic models** using textual input.	GPT-4 **accurately replicated** partitioned survival models for NSCLC and Renal cell carcinoma (RCC). Most AI-generated models were error-free or had only minor errors; **some human intervention was needed** for complex steps. **Error-free scripts matched published cost-effectiveness ratios to within 1%**	Model development
6	AI-Driven Virtual Assistance Interface for Excel-Based Economic Model[10]	2024	To develop an LLM-powered virtual assistant to **operate and customize** a bespoke excel-based cost-effectiveness model for different markets	AI interface **achieved perfect prompt processing** (10/10 retrieval, 20/20 updates) and **reliably applied multi-parameter changes** from uploaded input sheets across all cases	Model adaptation
7	Automating Economic Modelling: Potential of Generative AI for Updating Excel-Based Cost-Effectiveness Models[11]	2024	Assess GPT-4's **accuracy** and capability in **automating updates to Excel-based cost-effectiveness models** for muscle-invasive urothelial carcinoma (MIUC) across different country settings.	GPT-4 performed 62/64 required updates correctly, resulting in an overall **accuracy score of 97%**	Model adaptation
8	Leveraging Large Language Models for Conceptualizing Health Economic Models: A Feasibility Study in Oncology[12]	2024	Evaluates whether LLMs can feasibly and accurately **conceptualize** health economic models	LLM proposed, expert-aligned natural history and a four-state M**arkov model with key parameters** identified and gaps flagged	Model conceptualization
9	Can Large Language Models Generate Conceptual Health Economic Models?[13]	2024	Evaluate the **feasibility and accuracy** of Bing Chat and ChatGPT-4 in **developing conceptual health economic models** for chronic diseases with multiple health states	Both LLMs generated relevant summaries for hepatitis C models; **Bing Chat consistently provided high-quality parameters**, while **ChatGPT-4 sometimes produced implausible outputs**. Output quality and model structure varied by prompt and tool.	Model conceptualization
10	Can GenAI Plugins Work as a Savior for MS-Excel Based Health Economic Models?[14]	2024	Explores whether GenAI agent plugins can **enhance excel** by reviewing formulas, interpreting outputs, and suggesting optimizations while retaining the platform	GenAI plugin **read complex excel formulas, simplified them, suggested efficient alternatives, visualized dependencies, flagged potential errors, and improved model reliability and user comprehension** quickly	Model optimization
11	Man Versus Machine: Can AI-Assisted Technology be Used to Support the Development of Economic Models?[3]	2023	To explore if AI can **write and review Visual basic for applications (VBA) functions** for Excel-based health economic models.	AI-generated code was fast but sometimes had **errors or poor style**; it made valid suggestions, but some calculations were incorrect.	Model optimization

Based on the shortlisted publications, the summary of findings for health economic modeling is shown in Table 1. The publications identified the following applications in the below section:

Result segregation by Sub-themes - Health economic modelling

1. **Model Development:** In the study by Chhatwal et. al. 2024[5], a GenAI model was used to recreate a health economic model originally described in Briggs et al.'s book. The GenAI successfully extracted relevant parameters from the text, with estimated cost, life years, and ICER differing by 8%, 0.1%, and 2%, respectively, from published values. Srivastava et. al. 2024[7] study used GPT-4.0 to build an early health economic model by analyzing data from multiple sources, such as clinical trials, statistical plans, epidemiological research, and prior economic studies. GPT-4.0 effectively processed varied datasets, improved early-stage assessments, identified key cost-effectiveness factors, and highlighted evidence gaps for HTA submission. The study however underscored the need for expert oversight in interpreting and applying GPT outputs. In a study by Reason et. al. 2023[8], GPT-4 was tasked with adapting a generic R script to replicate a 3-state partitioned survival model for NSCLC using data, assumptions, and parametric model choices extracted from published tables. The AI-generated model produced total costs and QALYs within 1–10% of the original study, with any differences mainly due to input data quality rather than errors from GPT-4. Another study by Reason et.al. 2024[4], evaluated GPT-4's ability to reproduce partitioned survival models for NSCLC and RCC using R. GPT-4 replicated the NSCLC model with 100% of outputs either error-free or with only minor issues, and 93% completely error-free. For RCC, simplification of one calculation was needed; following this, 87% of models were accurate or contained a single minor error, with 60% entirely error-free. The error-free scripts matched published incremental cost-effectiveness ratios within 1%. The study noted that sensitive data may be retained by LLM providers, recommending the use of privately hosted instances. It also emphasized the need for trained health economists to validate LLM-generated scripts and called for further research into complex modelling and sensitivity analysis.

   Chhatwal et. al. 2024[6] found GenAI platform produced de novo Markov models for hepatitis C with 10–15 health states and over 22 transitions, citing published models and estimating transition probabilities, costs, and utilities from literature. Across five runs, structures varied—likely due to differences in references—but retained face validity.
2. **Model Adaptation:** Pandey et. al. 2024[10] developed a Claude-3-Opus–based interface with a Python backend and Jinja2 frontend to automate retrieval and updates in Excel health economic models. Across 20 input sheets and 30 prompts, the system achieved 100% accuracy for all single and multi-parameter changes, correctly processing 10 data retrieval and 20 update tasks. Multi-parameter updates succeeded in every tested scenario.

   Rawlinson et. al. 2024[11] evaluated GPT-4 for automating country-specific adaptation of an Excel cost-effectiveness model for MIUC, moving from the UK to the Czech Republic. GPT-4, using natural language instructions and tabular data, completed 62 of 64 required changes (97% accuracy), with all performed updates correct. Accuracy was highest for resource and adverse event costs (100%), and slightly lower for drug costs (82%).
3. **Model Conceptualization:** Chhatwal et. al. 2024[13] compared Bing Chat and ChatGPT-4, finding that Bing Chat's Retrieval Augmented Generation produced more accurate and relevant model structures and parameters. Output quality depended on prompt design, and Bing Chat consistently outperformed ChatGPT-4, though health states and transitions varied by run.

   The Srivastava et. al. 2024[12] proof-of-concept study used various LLM reasoning techniques and expert oversight to conceptualise a health economic model for advanced breast cancer. The LLM generated a Markov model with four health states that closely matched expert concepts and identified key data gaps, supporting the integration of LLMs with expert review for effective model conceptualization.

   Both studies however noticed that LLMs showed variability in their output quality and highlighted the importance of expert guidance in utilizing LLMs for HEOR.
4. **Model Optimisation:** Srivastava et. al. 2024[14] found that GenAI agent plugins enhanced Excel by reviewing and interpreting formulas, simplifying and suggesting efficient alternatives, visualizing dependencies, and flagging errors—rapidly improving model reliability and user comprehension. Medland et. al. 2023[3] explored whether AI could write and review VBA functions for Excel-based health economic models, showing that AI-generated code was fast and offered valid suggestions, but included errors, incorrect calculations, or poor style.

#	Study title	Year	Objective of study	Result	Sub-theme
1	Using Artificial Intelligence (AI) to Extract National Institute for Health and Care Excellence (NICE) Final Appraisal Documents (FAD): Evaluating the Potential Application of Large Language Models (LLM) vs Human Extraction[15]	2024	To **assess the potential use of LLM** in market access when **extraction of data** from NICE FAD is required.	The LLM demonstrated **high accuracy in extracting most data** type with perfect performance for quantitative and qualitative intervention data but struggled with qualitative economic modeling data.	Data extraction
2	Exploring the Development of Briefing Books for Early Scientific Advice Using Large Language Models: A Proof-of-Concept Study[16]	2024	To assess LLM-based **generation of Briefing Book (BBs) for Early Scientific Advice (ESA)**	While LLM responses matched the desired tone and format and summarized key knowledgebase points, they **lacked sufficient depth** (could not generate HTA grade BB) and detail in several sections, omitting critical analysis and specific information.	Data extraction
3	Artificial Intelligence to Automate Network Meta-Analyses: Four Case Studies to Evaluate the Potential Application of Large Language Models[9]	2024	Aim to assess use of a LLM, GPT-4 to automatically extract data from publications.	The LLM consistently delivered **accurate, comprehensive, and fully automated data extraction** and reporting across multiple case studies.	Data extraction
4	Can Gen-AI Assist in Interpreting the Health Economic Model Results as Per Target Audience?[17]	2024	To adapt the **dissemination of health economic model results** to the understanding levels of different target audiences.	GenAI made complex health economics results **more accessible and relevant** for both clinicians and non-technical stakeholders by simplifying key metrics and linking them to disease contexts.	Reporting simplification
5	Balancing Feasibility, Time, and Comprehensiveness: Approaches to Rapid Reviews of Health Economic Models[18]	2024	To deliver timely yet **robust evidence summaries**.	The AI approach was **highly comprehensive (≈97%) and time saving** compared with two other approaches, though the fourth approach was the most comprehensive (≈100%).	Reporting simplification

Based on the shortlisted publications, the summary of findings for evidence synthesis is shown in Table 2. The publications identified the following applications in the below section:

Result segregation by Sub-themes - Evidence synthesis

5. **Data Extraction** - Knott et. al. 2024[15] evaluated LLMs for market access by extracting data from NICE FADs. The LLM demonstrated high accuracy across most data types, achieved perfect performance for quantitative and qualitative intervention data, but struggled with qualitative economic modeling information. Thaliffdeen et. al. 2024[16], a proof-of-concept, assessed LLMs for generating BBs for ESA. The models produced responses with appropriate tone and format and summarized key knowledgebase points, but lacked depth, omitted critical analysis, and missed specific details in several sections, resulting in outputs that did not meet HTA-grade standards. Reason et. al. 2024[4] evaluated GPT-4 for automatic data extraction from publications and found that it consistently delivered accurate, comprehensive, and fully automated extraction and reporting across multiple case studies. However, assumed routine technical checks, as in human-led analyses. LLMs, though inconsistent, were expected to improve.
6. **Reporting Simplification** - Swami et. al. 2024[17] aimed to adapt dissemination of health economic model results to the understanding levels of different target audiences. GenAI made complex health economics findings more accessible and relevant for clinicians and non-technical stakeholders by simplifying key metrics and linking them to disease contexts. Smela et. al. 2024[18] sought to deliver timely yet robust evidence summaries. The AI method was nearly as thorough (≈97%) as the top approach (≈100%) and saved more time than the other two methods.

Discussion

1. Our study highlighted the growing use of GenAI and LLMs in HEOR. The application in health economic modelling outweighed the use in evidence synthesis as the primary application of GenAI and LLMs. GenAI and LLMs were used across the workflow for specific tasks. Health economic modelling applications involved data synthesis, model conceptualization, software implementation, and quality assurance—demonstrating the versatility and increasing significance of Gen AI and LLMs in the field. Usage of LLMs for evidence synthesis was primarily limited to data extraction and reporting simplification. It also involved processing large amounts of information for the purpose of extracting data and compiling reports for stakeholders based on synthesized content. AI tools were also utilized to convert technical HEOR concepts into outputs that were more accessible and easier to interpret for stakeholders. **2.** The utilisation of GenAI and LLMs presented multiple advantages. These technologies supported the optimisation of health economic models through comprehensive formula review and refinement, output interpretation, and the provision of targeted recommendations for improvement. Their capacity to evaluate code, including VBA scripts, further contributed to enhanced productivity and transparency within model development. Additionally, these tools demonstrated proficiency in proposing and structuring sophisticated models, as well as accurately extracting and validating essential parameters. The ability to replicate published models and generate new ones with minimal error rates illustrates their significant potential to streamline the modelling process. **3.** In terms of limitation, Gen AI and LLM tools require human experts to validate results, correct mistakes, and ensure quality. While these tools can efficiently extract structured data from scientific literature for reporting and evidence synthesis, they struggle with nuanced qualitative information in economic modeling. Expert review remains essential for maintaining quality and context in health economic research. **4.** The review had certain limitations. The use of a TLR rather than a systematic approach may have increased the risk of selection bias. The application of multiple reviewers, structured data extraction methods, and predefined themes for article classification helped reduce potential biases associated with the targeted literature review methodology. Another limitation was the short search time frame, which could have potentially excluded older studies. However, all selected studies were within the three-year window, indicating that the chosen time frame was appropriate for this review. **5.** Overall, the integration of GenAI and LLM into health economic modeling offers substantial benefits in terms of efficiency, accuracy, and adaptability. Yet, the success of these technologies depends on thoughtful prompt design, careful tool selection, and continuous human validation to maintain high standards of quality and reliability. This review could not find studies covering application of GenAI and LLMs in some other areas of HEOR. Future areas where these tools can be used may include other established economic modeling frameworks - budget impact analysis, cost minimization, and discrete event simulation and executing the full spectrum of evidence synthesis activities like systematic literature review but not limited to the ones mentioned.

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