Cost-effectiveness in nanomedicines: A small step for research, a giant leap in value

Putnam
Inizio Advisory

N Klimushkin, A Meunier, C Hardern, J Farrington
Putnam, London, United Kingdom

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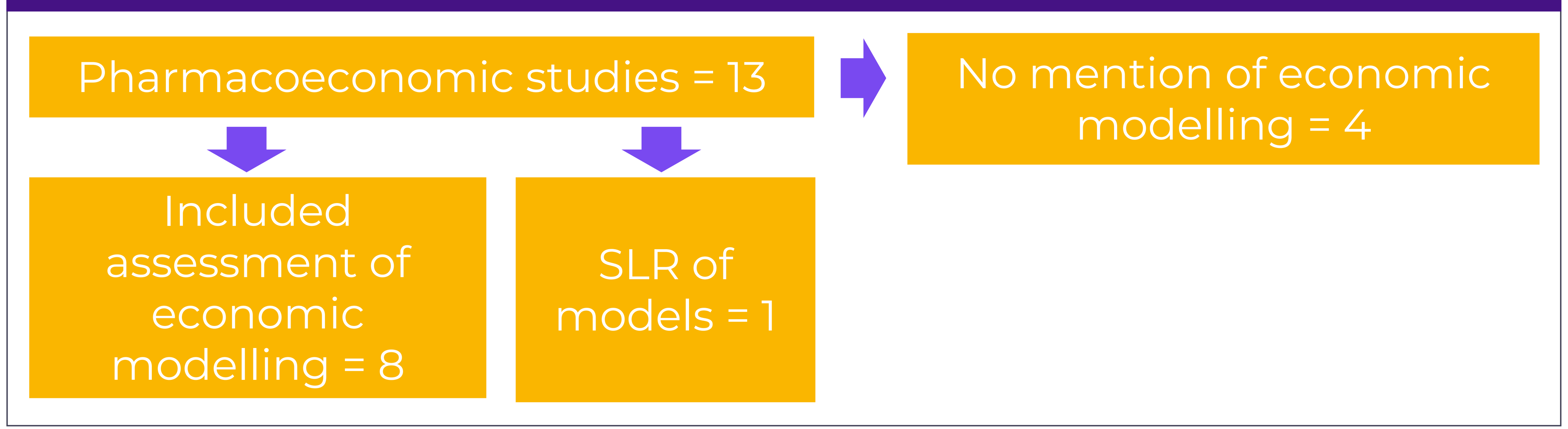
Background

- By encapsulating existing medicines with tailored nanomaterials, nanotechnology-enabled drug delivery addresses the limitations of conventional methods by improving bioavailability, targeting efficacy, and allowing controlled drug release. These advancements can lead to better patient outcomes, increased compliance, reduced dosing frequency, and fewer AEs, potentially lowering the overall healthcare costs
- The oncology segment is currently the largest and most advanced niche in the nanomedicine (NNM) market, and it is expected to continue driving market growth (1)
- Despite a decade of efforts towards commercialisation, the pharmacoeconomic value of NNMs in cancer has been demonstrated with limited success. This may be partially due to the limited data collected to support cost-effectiveness analysis, as evidenced by feedback from HTA agencies assessing NNMs:
  - Both Australian PBAC and the French HAS considered that the lack of data on QoL in the pivotal study of anticancer NNM Abraxane® was a significant limitation (2,3)
  - Private payers only reimbursed a nanotechnology-based photodynamic cancer therapy after a comprehensive CEA based on 2 Markov models was presented (4)

Methods

- A targeted search in PubMed was conducted using the search terms "nanomedicine", "oncology", "pharmacoeconomics", and "cost-effectiveness analysis"
- The inclusion criteria was focused on oncological indications from 2007 to present date. Reviews of economic analyses were also included and citation searched
- Duplicates, publications with outdated information, and publications with irrelevant focus were excluded from extraction
- Data were extracted on gaps in evidence generation, with the focus on analysing studies on economic models of NNMs used in cancer treatment

Figure 1. Targeted literature search of anticancer NNMs



Objective

The primary objective of this analysis is to investigate challenges in demonstrating the CEA evidence for applications of NNMs in cancer treatment.

Results

The targeted search yielded 13 pharmacoeconomic publications on the use of NNMs in cancer, 8 of these included mentions of economic models of NNMs in cancer, and 1 was a systematic review of 9 economic models (1,4-15) **(Figure 1)**. The extracted sources revealed that few studies exist on the cost effectiveness of NNMs in cancer, and those that do, contain methodological gaps.

Challenges

Insufficient evidence generation

Commercialisation of NNMs is driven by small enterprises, who, because of limited resources, primarily focus on proof of concept and clinical stages (1), with diminished emphasis on evidence generation for reimbursement. As a result, successfully reimbursed NNMs such as Abraxane® or PEGylated liposomal doxorubicin (Caelyx®), are primarily recognized by payers for the unmet needs they fulfil.

Data to support economic modelling

The systematic review of anticancer NNMs reported only 1 economic model which included QALYs, with utility data based on expert opinion rather than empirical evidence (5), demonstrating the lack of QoL data for NNMs. Without robust QoL data collected via clinical trials, there are substantial challenges for NNMs in countries that require models based on cost per QALY.

Lack of clarity for payers

Lack of strong economic evidence limits the value recognition of NNMs by payers. For PBAC, the generic status of the therapy being nano-encapsulated challenges the traditional distinction between off-patent drugs and new medicines. In Australia, NNMs may receive preferential F1 status because of their innovative value, and there are concerns that this could encourage anti-competitive practices and be in opposition to equitable access of generic medicines (13).

Discussion: Suggested solutions to challenges

Unmet needs and differentiation

The focus should be on developing NNMs for areas of unmet need or demonstrate superior efficacy to justify a price premium. For NNMs based on existing generics, it is crucial to differentiate them from less expensive generic drugs.

Enhanced trial design

To improve the demonstration of cost-effectiveness, trial designs should anticipate regulatory and HTA requirements, including endpoints that can be translated into clinical outcomes and QoL data from patients.

Early regulatory and HTA advice

Seeking early advice from regulatory bodies and HTA agencies can help ensure appropriate characterisation and clearer communication of the benefits of NNMs.

Conclusion

Oncology is the most promising sector in the NNM market; however, the lack of early focus on comprehensive evidence generation hinders reimbursement. The complex nature of NNMs complicates the process of evidence evaluation for payers because of the lack of early HTA engagement and optimal communication of the value of NNMs. Although there has been some progress, payers will likely continue to cite the lack of strong CEA evidence as a weakness until more forward-looking NNM trial designs are initiated by the industry.

Abbreviations: AE, adverse effect; CEA, cost-effectiveness analysis; FDA, Food and Drug Administration; HAS, Haute Autorité de santé (French National Authority for Health); HTA, health technology assessment; NNM, nanomedicine; PBAC, Pharmaceutical Benefits Advisory Committee; PEG, polyethylene glycol; QALY, quality-adjusted life year; QoL, quality of life; SLR, systematic literature review

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Contact
Nick Klimushkin
Nick.Klimushkin@putassoc.com

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