ECONOMIC EVALUATIONS OF ORPHAN DRUGS FOR RARE KIDNEY DISEASES IN LOW- AND MIDDLE-INCOME COUNTRIES: A SYSTEMATIC REVIEW AND BIBLIOMETRIC ANALYSIS

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INTRODUCTION

- Rare kidney diseases (RKDs) comprise over 150 genetic, metabolic, autoimmune, and tumor-related conditions, with prevalence ~60–80 per 100,000 in Europe/US. Data from LMICs remain scarce, though RKDs cause high morbidity, mortality, and economic burden.
- Orphan drugs (e.g., sparsentan, lumasiran) have emerged as promising therapies but carry very high costs. This strains healthcare budgets, especially in LMICs with limited resources and weaker health systems.
- Most economic evaluations of orphan drugs come from high-income countries, limiting relevance for LMICs. Without region-specific analyses, policymakers cannot make evidence-based decisions on affordability, reimbursement, or access.

METHODS

Design & Registration

Systematic review (2014–2024), following PRISMA guidelines. Protocol registered in PROSPERO.

Search & Selection

- Databases: PubMed and Scopus.
- Inclusion: full economic evaluations (CEA, CUA, CBA, CMA) of orphan drugs for RKDs in LMICs.
- Exclusion: non-drug interventions, HIC-only, non-English, reviews/commentaries.
- Screening was done independently by two reviewers.

Data Extraction & Analysis

- Extracted study design, country, population, perspective, outcomes (QALYs, LYs, ICERs), costs, and funding.
- Tools: Excel (data management), Tableau (geographic mapping), VOSviewer (keyword analysis).
- Reporting quality assessed with CHEERS checklist (28 items; rated Excellent, Good, Moderate, Low).

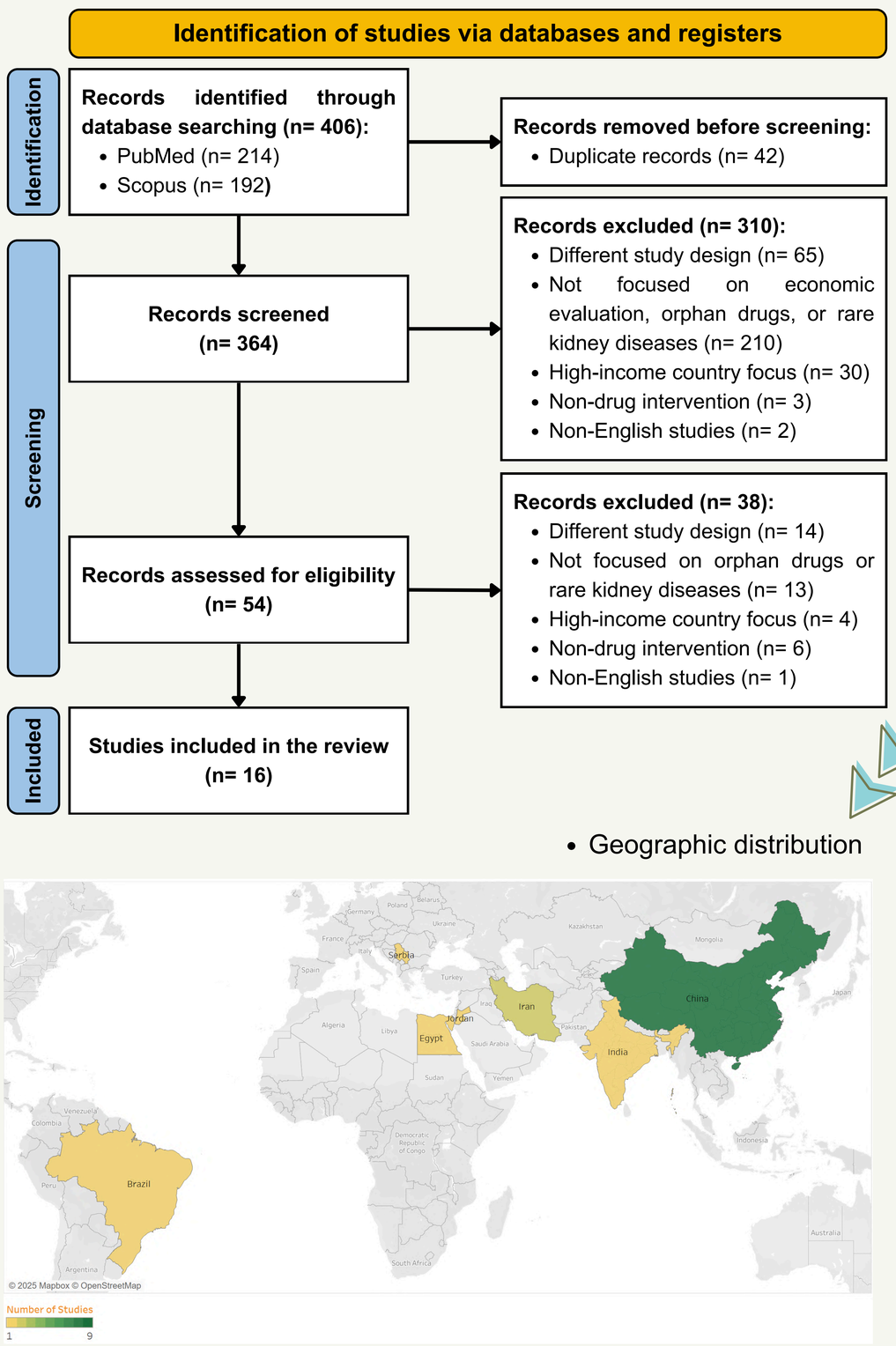
OBJECTIVE

To provide the first systematic review and bibliometric analysis of economic evaluations of orphan drugs for RKDs in LMICs, addressing gaps in cost-effectiveness evidence, reporting quality, and regional research trends.

RESULTS

1. Study Identification

- 406 records → 364 screened → 54 full-texts → 16 studies included



- Geographic distribution

2. Economic Evaluation Findings

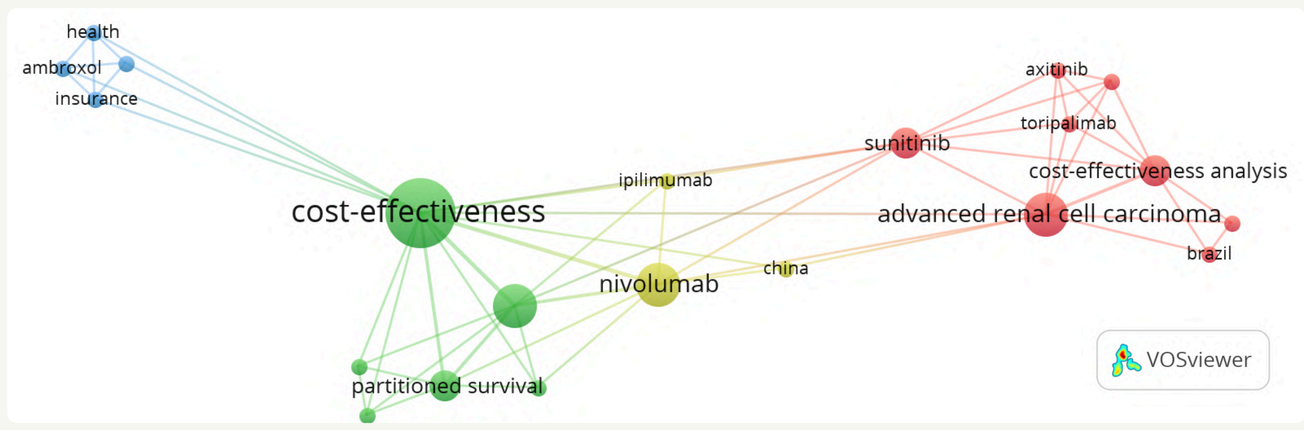
- 63% (10/16): orphan drugs for RKDs were cost-effective.
- Most CUA studies focused on advanced RCC (50%) or metastatic RCC (29%); others on Beta-Thalassemia and Gaucher disease type 2.
- Common costs: drug therapy, adverse event management, monitoring, follow-up, and hospitalizations.
- Sensitivity analyses: 71% used both PSA & DSA.

3. Quality of Reporting

- Reporting quality: 87.5% good, 12.5% moderate.
- Gaps: heterogeneity (37% not reported), distributional effects (31% not reported), funding disclosure (31% missing).

4. Bibliometric analysis:

- Keywords:



CONCLUSION

- **Orphan drugs for RKDs show promise as cost-effective options in LMICs, but more rigorous, transparent, and regionally diverse evaluations are needed to guide sustainable policy and reimbursement decisions.**
- **Evidence is geographically concentrated (mainly China), leaving large regions underrepresented.**
- **Strengthening the quality and breadth of economic evaluations is essential to support fair and sustainable access to orphan drugs in LMIC health systems.**

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