# Health Economics Studies Assessment on Melanoma as Part of the 2ND China Rare Disease Catalogue: A Scoping Review

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## Abstract

**OBJECTIVES:** This scoping review aims to provide researchers insight into published economic evaluations, to support the market access and reimbursement decisions related to melanoma in the past decades.

**METHODS:** A scoping review based on PRISMA-ScR methodology was conducted. The search for articles was based on Pubmed from 2000 to June 2024. We assessed health care costs, cost-effectiveness, cost-utility, cost-benefit, budget impact analyses.

**RESULTS:** 88 studies were included of which 57 studies on systemic treatment, 13 on diagnosis, 6 on screening, 5 on surveillance, 4 on prevention, 1 on hospice, 1 on follow-up strategy, and 1 on psycho-educational intervention. Almost half of the studies (41/88) from North America, more than a quarter of the studies (29/88) from Europe, 2 studies from China. Since 2019 publications have highlighted advances in isolated or combined targeted therapy, immune checkpoint inhibitors, oncolytic viral immunotherapy, tumor infiltrating lymphocytes (TIL) therapy, bispecific fusion protein, radiotherapy and electrochemotherapy. Most CEA studies of treatments utilized Markov cohort - or partitioned survival models and adopted a healthcare system perspective. Diagnosis options included histopathologic-, various imaging technologies-, endoscopies and gene sequencing and expressions tests. Further evaluations on screening included: Type (skin examination, self-assessment tool); frequency; start time, whilst also considering the suspended situation during Covid-19. Reported surveillance interventions were all conducted in people with high risk. Prevention included personalized genomic risk provision, sun protection, public education program and banning the use of tanning bed by adolescents. Hospice for 4 or more days and reduced follow-up strategy were described as cost effective, whilst psycho-educational intervention targeted on fear of cancer recurrence was concluded as not cost effective.

**CONCLUSIONS:** This health economics studies assessment on melanoma has shown that screening, surveillance, and prevention interventions were most likely to be concluded as cost-effectiveness. Increased research in China is appreciated to promote rational drug use and enhance patient outcomes.

## Background & Objective

Melanoma is an aggressive form of skin cancer with fiercely increasing incidence and mortality. Previous studies showed that there has been a substantial increase in the burden of melanoma in China[1]. Among the Second Rare Disease Catalogue released by China, melanoma is one of the most investigated rare diseases[2]. This study aims to summarize the international progress in health economics studies regarding melanoma, and to further encourage related research in China.

## Methods

This study served as a sub-group analysis[2]. A scoping review based on PRISMA-ScR methodology was conducted. The search for articles was based on Pubmed from 2000 to May 2024 using a combination strategy of health economic related terms and rare disease related terms. The extracted data included study characteristics, populations, intervention, comparators and main conclusion. Two independent reviewers would first screen the article by title and abstract, and then the whole manuscript. The extracted data included study characteristics, populations, intervention, comparators and main conclusion.

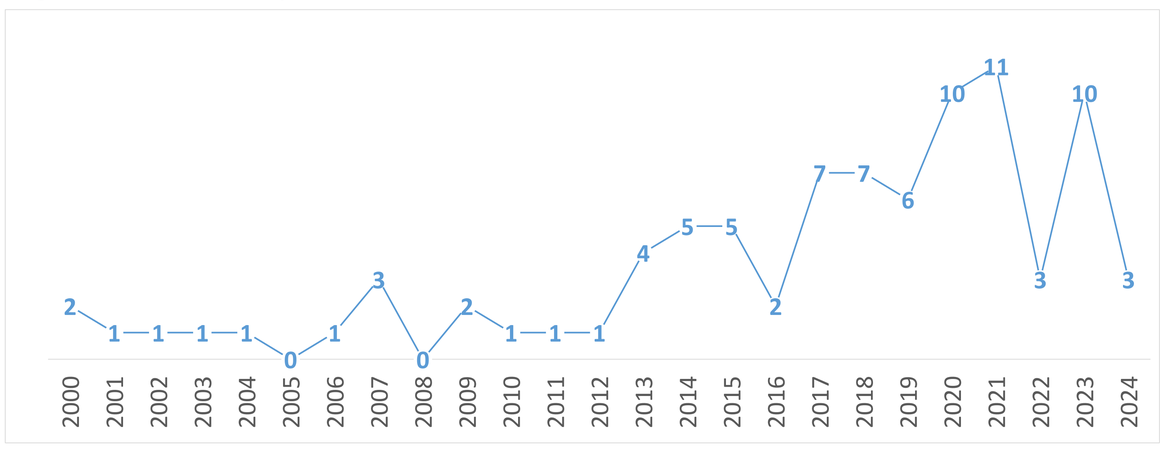
For the included articles, we conducted descriptive statistics on publication year, country, research type, interventions, and research conclusions.

## Results

A total of 88 articles were included for comprehensive analysis.

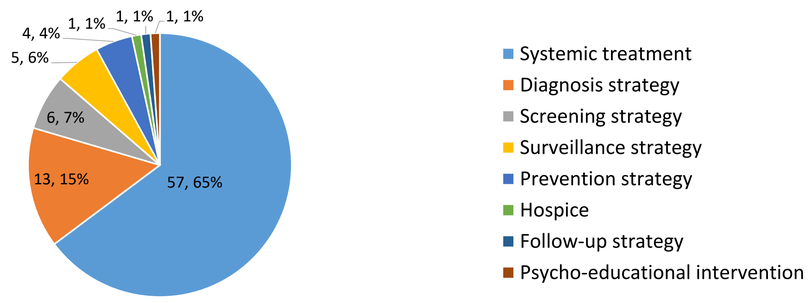
In terms of publication year, an increasing trend could be seen in the near decade compared to previous decade. However, the number of the publications fluctuate year by year.

Figure 1. Publication year distribution



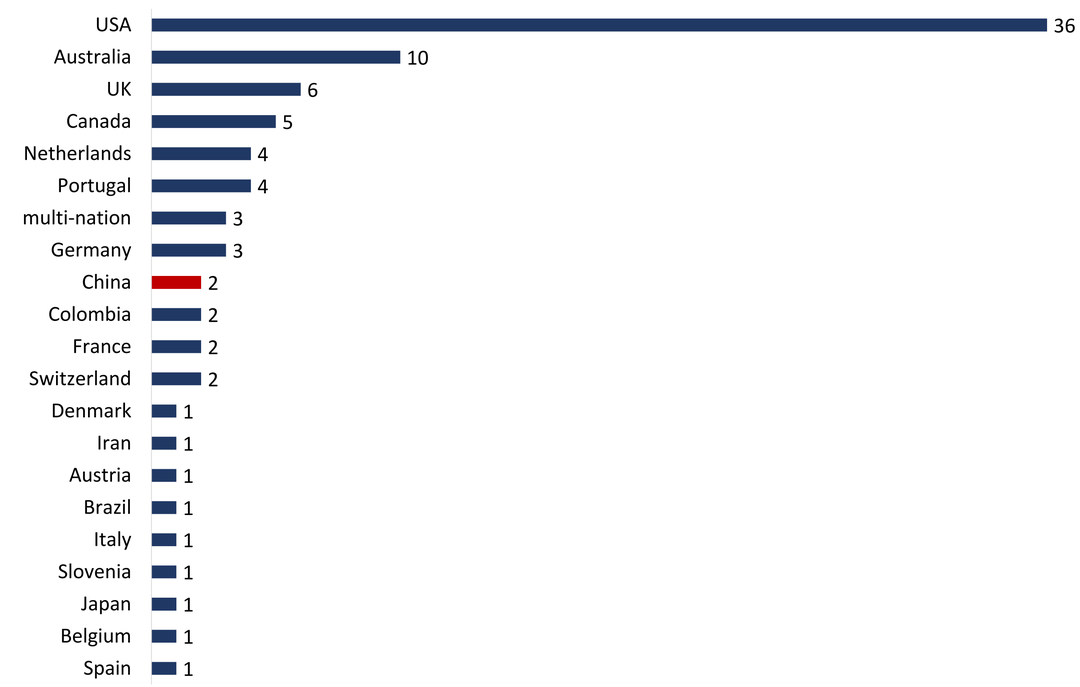

In all included studies, the evaluation topics pertained most often to systematic treatment (57, 65%), diagnosis strategy (13, 15%), and screening strategy (6, 7%).

Figure 3. Study intervention distribution




The top 3 countries with the highest number of publications are: USA (36, 40.9%), Australia (10, 11.4%) and the UK (6, 6.8%).

Figure 2. Study countries distribution




In systematic treatment, most of studies are CEA (54, 94.7%). Table 1 summarized the interventions and CEA conclusions derived from the studies. Most CEA studies of treatments utilized Markov cohort - or partitioned survival models and adopted a healthcare system perspective.

Table 1. Systematic treatment strategies evaluated by CEA

| Treatment strategies and Interventions | Cost-effectiveness to comparators? |
|---|---|
| • Bispecific fusion protein | No |
| Tebentafusp | |
| • Chemotherapy | Yes |
| Oral temozolomide (TEM) | |
| • Electrochemotherapy | Uncertain |
| • Adjuvant high-dose interferon (IFN) therapy | Mix |
| • Immune checkpoint inhibitors (ICI) | Yes, one reported uncertain |
| Anti-PD-1 anti-CTLA-4 | |
| • Radiotherapy | Yes and uncertain |
| Hippocampal-avoidant WBRT (HA-WBRT) and proton beam therapy | |
| • Surgery | Yes |
| • Targeted therapy | Mix of Yes and No |
| BRAF inhibitor and MEK inhibitor | |
| • Tumor-infiltrating lymphocytes (TIL) therapy | Yes |
| • Testing + IFN | Yes |
| • ICI + target therapy | Mix, most of report Yes |
| • ICI + oncolytic viral | No |
| • Sequential Treatment | Multiple results |

Evaluated diagnosis tools and reported CEA conclusions are summarized in Table 2.

Table 2. Study type distribution

| Treatment strategies and Interventions | Cost-effectiveness to comparators? |
|---|---|
| • histopathology | Mix of Yes and No |
| sentinel node biopsy (SNB), sentinel lymph node biopsy (SLNB), Fluorescence in situ hybridization (FISH) | |
| • Imaging technology | Yes |
| Radiography, computed tomography (CT), positron emission tomography (PET) | |
| • Endoscopy | Yes |
| Dermoscope | |
| • Gene expression and sequencing test | Yes |
| • non-invasive scanning technique | Yes |

Screening strategy included systematic screening skin examination, most of them are concluded as cost-effectiveness. Surveillance strategy included specialized surveillance through the high risk clinic, PET-CT, carriers of BAP1 germline variants and liver screening, all concluded as cost-effectiveness. Hospice for 4 or more days and reduced follow-up strategy were described as cost effective, whilst psycho-educational intervention targeted on fear of cancer recurrence was concluded as not cost effective.

## Conclusion & Discussion

There has been an increasing publication trend of over the past decade and research are mostly from developed countries. Most of the studies are CEA on various treatments and diagnosis tools. Increased research in China is much appreciated to promote rational drug use and enhance patient outcomes.

## References

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2. Fang J, Du H, Tjong A Hung I, Rai MK, Cameron C. Health Economics Studies Assessment of the 2ND China Rare Disease Catalogue: A Scoping Review. ISPOR 2024. Abstract#144172