Health Economics Studies Assessment of the 2ND China Rare Disease Catalogue: A Scoping Review

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Abstract

OBJECTIVES: China National Medical Products Administration recently released the Second Rare Disease Catalogue in 2023 which listed another 86 rare diseases. This catalogue provided strong support for the market access and reimbursement of relevant drugs. This study aims to summarize the international progress in health economics studies regarding these diseases, and further enhance related research in China.

METHODS: A scoping review based on PRISMA-ScR methodology was conducted. The search for articles was based on Pubmed from 2000 to May 2024. All the screening and data extraction steps were executed by two independent reviewers.

RESULTS: A total of 919 articles were identified, and eventually 189 articles were included for analysis. Of these articles, 40 articles (21.2%) and 77 articles (40.7%) were published in the previous three and five years. Of the 86 diseases listed, only 28 were found to have published studies. The top 5 diseases with the most published articles were as follows: melanoma (88, 46.6%), glioblastoma (15, 7.9%), thalassemia major (12, 6.3%), gastrointestinal mesenchymal stromal tumors (10, 5.3%), and retinopathy of prematurity (9, 4.8%). Cost-effectiveness analysis (CEA) was the main study type (172, 91.0%), whilst others were related to economic disease burden, budget impact analysis, society economic impact, etc. In all CEA studies, the evaluation topics pertained most often to medical therapies (128, 74.4%), screening strategies (19, 11.0%) and diagnose strategies (15, 8.7%). Most studies were conducted in the USA (65, 34.3%) and the UK (19, 10.0%), whereas China accounted for 6 (3.2%).

CONCLUSIONS: There has been an increasing publication trend of health economics studies on rare disease over the last couple of years. However, research on most diseases is still missing or scarce. Increased research, including in diverse geographic regions and especially in China, is much appreciated to promote rational drug use and enhance patient outcomes.

Background & Objective

In 2018, China issued the First Rare Disease Catalogue which included a total of 121 rare diseases[1]. With improving disease diagnosis, treatment level, and the expansion of national reimbursement directory list (NRDL) negotiations in China, the 2018 rare disease catalogue could no longer meet the clinical practice needs. Therefore, the Second Rare Disease Catalogue was released in 2023, including a total of 86 rare diseases[2]. These two catalogues will further strengthen the management of rare diseases in China and improve the level of rare disease diagnosis and treatment.

In reference to the 86 included diseases in the Second catalogue, there are currently 97 drugs involving 69 disease approved in foreign countries, whilst in China the number is at respectively 49 and 35[3]. In numerical terms, the number of approved drugs in China is only half of the global total, highlighting a significant gap in drug accessibility. This study aims to summarize international advancements in health economics research on these 86 rare diseases, with the goal of promoting similar research in China to help bridge this gap.

Methods

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. Articles regarding cost effectiveness analysis (CEA), budget impact analysis (BIA), cost analysis, economic impact and other economic related would be included. 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.

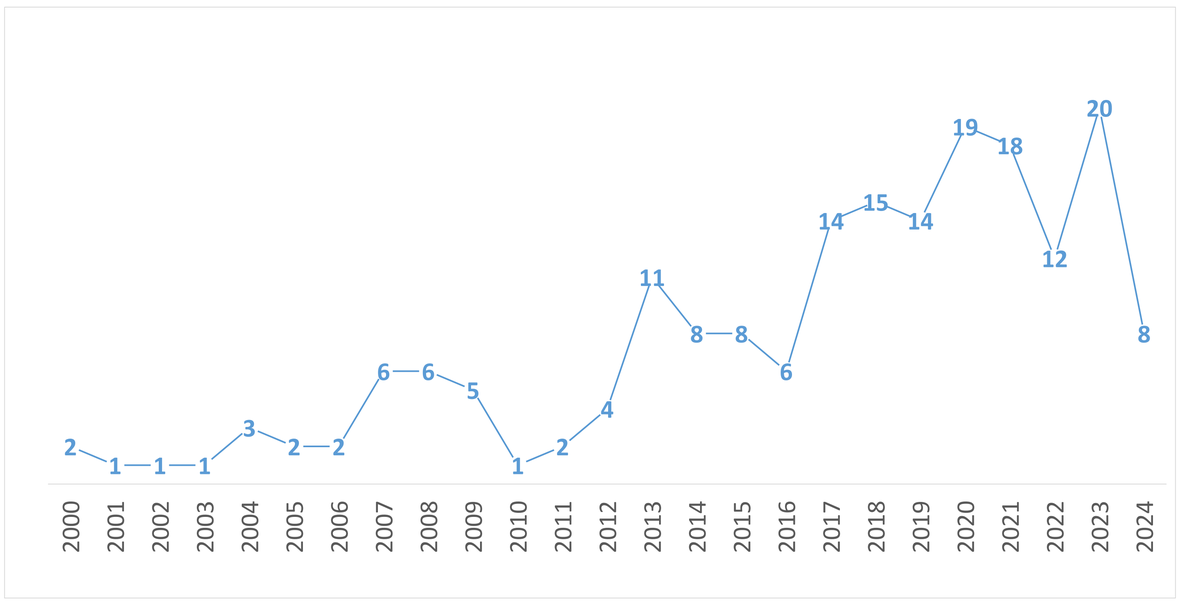
For the included articles, descriptive statistics will be conducted on their distribution in disease, publication year, country, research type, research perspective, and research conclusions.

Results

A total of 919 articles were identified using the combined search strategy, and eventually 189 articles were included for analysis. The 730 articles were excluded due to following reasons: not related to the defined diseases, not related to the defined economic studies, not primary research, no full text/abstract.

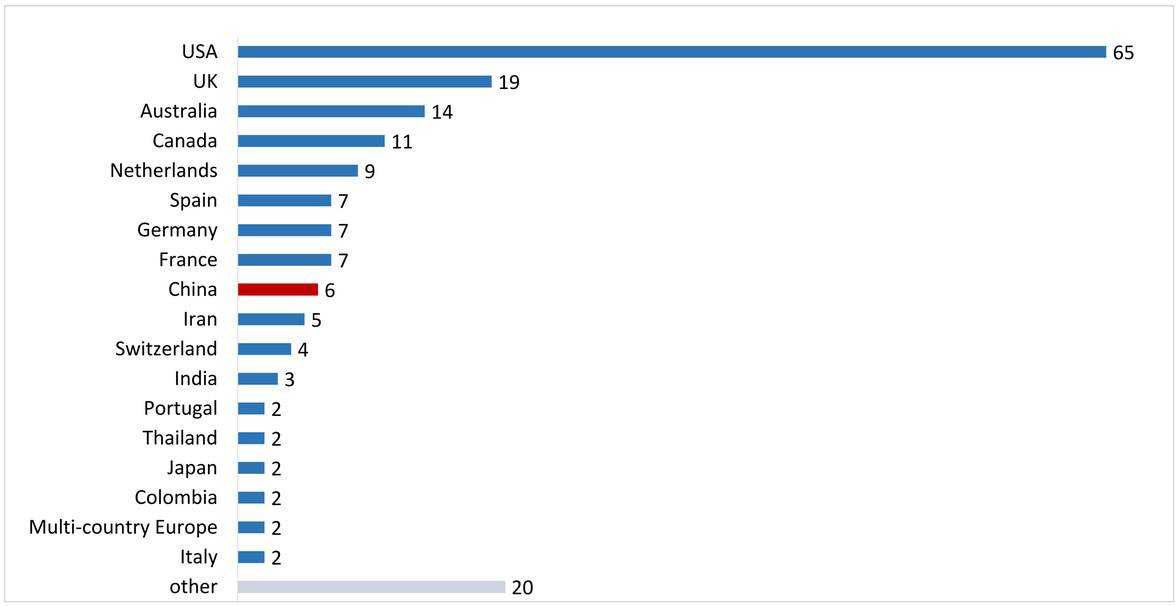
A clear trend is emerging, with the number of economics-related articles gradually increasing, particularly from 2017 to 2022. Out of the 189 articles included, 40 articles (21.2%) and 77 articles (40.7%) were published in the previous three and five years, see Figure.1.

Figure 1. Publication year distribution



As for the countries, most studies were conducted in the USA (65, 34.3%) and the UK (19, 10.0%), whereas China accounted for 6 (3.2%), see Figure 2.

Figure 2. Study countries distribution



Note: Other include countries with only one study, such as Egypt, Denmark, Finland.

Of the 86 diseases listed, only 28 were found to have published health economic related studies. The top 5 diseases with the most published articles were as follows: melanoma (88, 46.6%), glioblastoma (15, 7.9%), thalassemia major (12, 6.3%), gastrointestinal mesenchymal stromal tumors (10, 5.3%), and retinopathy of prematurity (9, 4.8%), see Table 1.

Table 1. Rare disease distribution

Disease	Included articles	Disease	Included articles
Melanoma	88	Persistent pulmonary hypertension of the newborn	2
Glioblastoma	15	Primary myelofibrosis	2
Thalassemia major	12	Gastroenteropancreatic neuroendocrine neoplasm	2
Gastrointestinal stromal tumor	10	Cutaneous neuroendocrine carcinoma(Merkel cell carcinoma)	2
Retinopathy of prematurity	9	Malignant hyperthermia	2
Hidradenitis suppurativa	5	Osteosarcoma	2
Malignant pleural mesothelioma	5	Waldenström macroglobulinemia/ Lymphoplasmacytic lymphoma	1
Familial adenomatous polyposis	4	Thrombotic thrombocytopenic purpura	1
Lennox-Gastaut syndrome	4	Giant cell arteritis	1
Short bowel syndrome	4	Neuroblastoma	1
Cutaneous T-cell lymphomas	4	Fragile X syndrome	1
Transthyretin amyloidosis	3	Polycythaemia vera	1
Primary sclerosing cholangitis	3	Systemic juvenile idiopathic arthritis	1
Pemphigus	3	Primary immunodeficiency	1

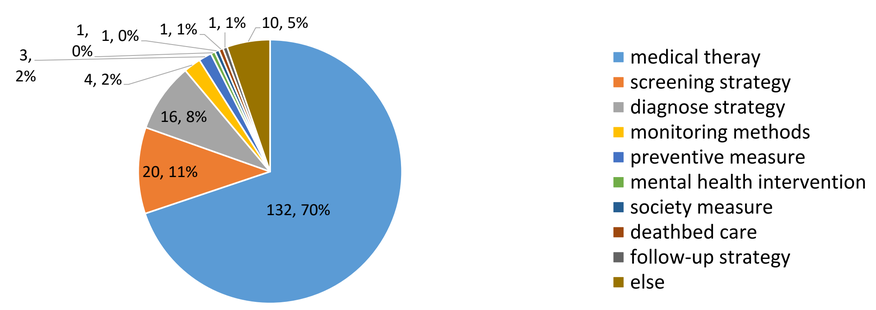
As for the study types, CEA was the main study type (172, 91.0%), whilst others were related to economic disease burden, BIA, society economic impact, etc. Same trend was also observed in the top 3 diseases, see Table 2.

Table 2. Study type distribution

Study type	No. of Included articles			
	all disease	Melanoma	Glioblastoma	Thalassemia major
CEA	172	80	13	11
economic disease burden	13	4	2	1
BIA	2	2	0	0
society economic impact	1	1	0	0
other	1	1	0	0

In all included studies, the evaluation topics pertained most often to medical therapies (132, 69.8%), screening strategies (20, 10.6%) and diagnose strategies (16, 8.5%), see Figure 3. In CEA articles，the proportional distribution is similar: medical therapies (128, 74.4%), screening strategies (19, 11.0%) and diagnose strategies (15, 8.7%).

Figure 3. Study intervention distribution



Conclusion & Discussion

There has been an increasing publication trend of health economic studies on rare disease over the last couple of years. However, research on most diseases is still missing or scarce. Most studies are related to the CEA and economic burden of medical therapies, possibly because these research could help with the reimbursement access and the clinical practice. Most studies were conducted in USA, UK, Australia and other developing countries. Increased research, including in diverse geographic regions and especially in China, is much appreciated to promote rational drug use and enhance patient outcomes.

Reference

1. National Health Commission, Ministry of Science and Technology, Ministry of Industry and Information Technology, etc. Notice on the Publication of the First Rare Disease Catalogue [EB/OL]. (2018-05-11) [2024-11-08]. https://www.gov.cn/zhengce/zhengceku/2018-12/31/content_5435167.htm
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