Economic Evaluations of Technologies for the Treatment of Rare Diseases in Latin America and Southeast Asia: A Scoping Review

Gil-Vaca, EN [1], Valderrama-Rios, MC [1], Vargas-Peláez, CM [2], Hernández, F [1].

[1] IQVIA Solutions, Bogotá, Colombia, [2], Universidad Nacional de Colombia, Bogotá, Colombia

Introduction and objectives

Rare diseases are often neglected due to their low prevalence (1). However, their economic burden can be very significant in low and middle-income countries due to their high-cost treatment. Economic evaluations for rare diseases are important to evaluate the costs generated by the acquisition of one type of technology instead of another, as well as the potential repercussions in terms of health outcomes (2). However, this scenario poses challenges for the elaboration of health technology assessments specially in economic evaluations due to the lack of data on these diseases, their therapeutic options and related costs (3, 4).

The aim of this scoping review is to map out how the economic evaluations of technologies for the treatment of rare diseases are being carried out, in terms of data sources, methods, perspective of analysis and costs used, in Latin America and Southeast Asia.

Methods

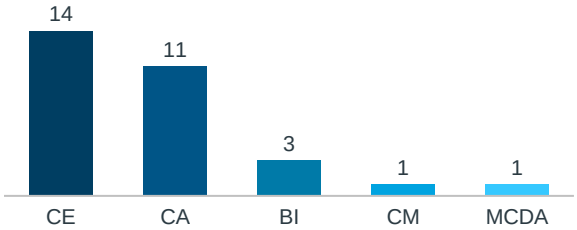
Population	Concept	Context
Patients diagnosed with rare diseases	Economic evaluations of health technologies	Latin America and Southeast Asia

This review was conducted in accordance with Joanna Briggs Institute's methodology for scoping reviews (5). We developed a literature search through the databases Medline, Embase, LILACS, and Scopus. Two reviewers carried out the evidence screening and selection. One reviewer conducted the data extraction process in a form designed for this review and it was backed up with quality control by a second reviewer. The findings were synthesized and summarized through a narrative description.

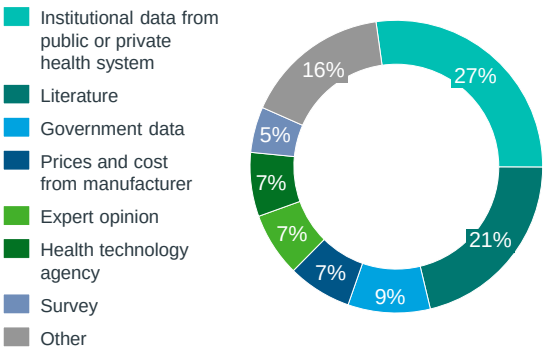
Literature search → Screening and selection → Data extraction → Narrative description

Results

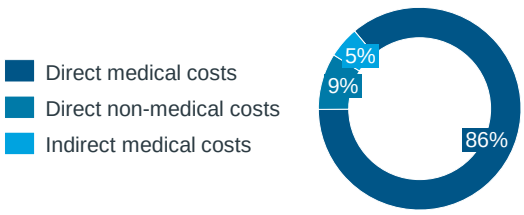
A total of 31 references were included. More information was found in Latin America (88%), specifically in Brazil (33%). The most used methods for economic evaluations were cost-effectiveness analyses (45%) and cost analysis (35%), the most used perspective of analysis was from the public buyer (61%), direct medical costs were the most frequently used (86%) and the most consulted data sources were from institutions of the national healthcare system (27%) and literature (21%). We found limitations in the economic evaluations concerning lack of information such as epidemiological data on the rare disease, either local or worldwide.



Graphic 1. Methods of economic evaluation. CE: cost-effectiveness, CA: cost analysis, BI: budget impact, CM: cost-minimization, MCDA: multi-criteria decision analysis



Graphic 2. Data sources for economic evaluation including epidemiology, effectiveness and costs.



Graphic 3. Costs used for economic evaluation.

Rare diseases	
Melanoma with BRAF positive mutation	Mucopolysaccharidosis II
Hereditary angioedema	Acromegaly
Myelofibrosis	Acute myeloid leukemia
Hodgkin lymphoma CD30+	Ankylosing spondylitis
Pompe disease	Gaucher disease
Myelodysplastic syndrome	Fabry disease
Hemophilia	Hemolytic uremic syndrome
Stevens-Johnson syndrome	Osteogenesis imperfecta
Neuromyelitis optica	Thromboembolic pulmonary hypertension
Cystic fibrosis	Bronchiectasis
Mucopolysaccharidosis II	Rare diseases in general

Table 1. Rare diseases found in the revision.

Sector	Analytical prespective	Number of studies
Public	User	2
	Payer	5
	Buyer	12
	Provider	1
Private	Payer	1
	Buyer	2
Social security	Payer	1

Table 2. Analytical perspectives used in economic evaluations. *This adds up to more than 31 references because one study presented results for 2 types of costs.

CONCLUSIONS

Economic evaluations of rare disease treatment technologies in Latin America and Southeast Asia are scarce; however, some trends were identified across different countries; for example, we identified that the most used method was the cost-effectiveness analysis. The trends found were based on recommendations by different authors, organizations, and international guides in order to guarantee reliable results when conducting economic evaluations. Latin America and Southeast Asia could benefit if research on rare diseases increased, especially in the field of economic evaluations, but to achieve this, joint efforts need to be exerted with governments, medical centers, universities, and other research institutes.

LIMITATIONS: Limitations are related to the lack of available information. First, most of the included studies (n=69%) were published as abstracts; these studies were not excluded given that that the purpose of this scoping review was to map the largest amount of information; however, some abstracts did not provide us with the necessary information. Second, due to the nature of the data, these were not presented in a homogeneous manner, which required us to categorize the information for a better understanding

1. World Health Organization. Priority Medicines for Europe and the World 2013 update: Rare diseases. 2013.
2. Drummond MF, Sculpher MJ, Claxton K, Stoddart GL, Torrance GW. Methods for the Economic Evaluation of Health Care Programmes. 4th ed. Oxford: Oxford Medical Publications.; 1997. 1–13 p.
3. ISPOR. Rare Diseases in Latin America: Challenges and opportunities for the equitable attention and proposal of Patients Organizations. 2017.
4. Shafie AA, Chaiyakunapruk N, Supian A, Lim J, Zafra M, Hassali MAA. State of rare disease management in Southeast Asia. Orphanet J Rare Dis. 2016 Dec 2;11(1):107.
5. Peters M, Godfrey C, McInerney P, Munn Z, Trico A, Khalil H. Chapter 11: Scoping Reviews. In: JBI Manual for Evidence Synthesis. JBI; 2020.

ISPOR Europe 2022, Austria