



REVIEW OF METHODOLOGICAL QUALITY OF BUDGET IMPACT ANALYSES FOR ORPHAN DRUGS*

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BACKGROUND & OBJECTIVE

Given current concerns with the growing expenditure on orphan drugs, **budget impact analysis** has become indispensable for health authorities to **regulate** long term **affordability** of orphan medicinal products. To date, **little is known about budget impact analyses for orphan drugs.**

This study aims to **(1) assess the methodological quality of budget impact analysis of orphan drugs** with a view to **(2) determine** how the accuracy and reliability of such studies can be enhanced.

METHODS

- **Review** of peer-reviewed **literature** & ISPOR conference abstracts
 - Literature retrieval up to October 2019
 - Databases: Pubmed, Embase
- **Quality** assessment based on **ISPOR good practice guidelines** for budget impact analysis
 - Criteria: intervention/comparator, perspective, time horizon, market effects, scope of costs, sensitivity analyses, data sources, discounting, validation

RESULTS

Number of studies per ISPOR criterion for combination of and individual orphan drugs ^x

Target population		Perspective			Time horizon			Intervention/comparator			Market effects		
Static	16	Third-party	31	7	1 year	4	-	None	1	Substitution	11		
Dynamic	6	Patient	1	-	2 year	2	-	Current	29	Combination	2		
Unspecified	15	Hospital	-	1	3 year	13	-	Unspecified	6	Expansion	26		
		Unspecified	5	-	4 year	1	2						
					5 year	10	2						
					> 5 year	-	5						
					Unspecified	7	-						

Scope of costs		Sensitivity analyses			Data sources			Discounting			Validation			
Drug-only	35	7	One-way	10	3	Literature	16	4	No	7	1	Yes	2	7
Drug-related	24	-	Probabilistic	1	-	Expert	9	1	Unspecified	29	6	No	5	-
Other	1	-	Scenario	15	4	Assumptions	26	6				Unspecified	30	-
			Unspecified	14	3	Market	6	5						
						Survey	2	1						
						Historical	7	5						
						Claims	14	5						
						Pharma	13	4						
						Registry	15	5						
						Other	9	1						

= individual orphan drugs
 = combination of orphan drugs
 = highest number of studies

^x Preliminary results are depicted

CONCLUSION

(1) Few published studies **meet the standard** of good practice for budget impact analysis. **(2)** The methodological quality of existing budget impact analyses of orphan drugs varies, making **comparative evaluations difficult**. **(3)** In **future** budget impact analyses, stakeholders should include accurate comparative analysis **with and without orphan drug(s)**, determine a defined **time horizon**, calculate the budget impact of **costs other than** related to **drugs** and perform extensive **scenario analyses**.