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Establishment of a value assessment framework for orphan medicinal products in China

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Background:

Multi-criteria decision analysis (MCDA)[1] is increasingly used as an applicable method for evaluating the value of orphan drugs. It is a collection of analytical techniques employed to support decision making on multiple and conflicting criteria. It can decompose a decision into many criteria, rank the individual criteria by importance, and define the influence of each criterion on the decision and its relative importance. Finally, the existing information is used to evaluate the decision-making plan and assist in improving the consistency, transparency, and rationality of decision making.

Objective:

Based on MCDA method, the study aims to establish a value assessment framework suitable for orphan drugs in China and explores the ideas and feasibility of comprehensively evaluating the value of orphan drugs and medical insurance access decisions from multiple dimensions.

Methods:

1. Building the initial framework of the criteria

A draft framework of the MCDA criteria was built based on systematic literature evaluation and EVIDEM(Evidence and Value: Impact on Decision-Making) framework tools[2].

2. Forming of the criteria framework

Stakeholder groups were formed by inviting clinicians, clinical pharmacists, health economics experts, policymakers, and patient representatives and collecting expert opinions through the brainstorming method of centralized workshops and expert consultation methods.

3. Weighting empowerment for orphan medicinal products based on MCDA

To compare the weight differences of the criteria under different perspectives, we conducted the weighting empowerment from the perspective of stakeholder and the public, respectively.

From the perspective of stakeholders, the five-point weighting and two-step percentile distribution methods were employed to weight the quantitative criteria in the framework for orphan drug value evaluation.

① Five-point weighting method: Each stakeholder expert assigned a relative weight to each criterion using a simple 5-point scale (1 = lowest relative importance; 5 = highest relative importance).

② Two-step percentile distribution method: The stakeholder experts first assigned 100 points to the five first-level domains of the quantitative framework, followed by 100 points between the second-level criteria under each domain, thus obtaining the relative weight of each criterion.

From the view of the public, a questionnaire survey of 70 sample people was conducted to obtain the scoring scale of the framework criteria for orphan drugs through two-step percentile distribution method. Finally, Based on the synthetization and comparison of all evidence and methods, we determined the framework criteria and scoring scale for the orphan medicinal products.

4. Data analysis

All data analyses were performed using Microsoft Excel 2019 and SPSS17.0. The sum of the criteria weights was normalised to 1. For the five-point weighting method, the relative weight of each criterion was divided by the sum of all the criteria. For the two-step percentile distribution method, the relative weight of each criterion was its domain score multiplied by the score of its local criterion and normalised to 1. The coefficient of variation (CV) of the weight and The Kendall W coefficient (W) were used indicate the coordination degree of the criteria and the extent of agreement among raters in the ranking of items, respectively.

Results:

1.Building results of the value assessment framework for orphan medicinal products based on MCDA

Combined with the stakeholder experts' opinions in the first workshop and the expert letter review, a revised orphan drug value evaluation criteria framework was formed. It consists of 11 quantitative(including "disease severity", "unmet needs", "comparative effectiveness", "comparative safety/tolerability", "comparative patient-perceived health/patient-reported outcomes", "type of therapeutic benefit", "comparative cost consequences–cost of drugs", "comparative cost consequences–other medical costs", "comparative cost consequences–non-medical costs", "quality of evidence", "expert consensus/clinical practice guidelines") and 8 qualitative criteria("mandate and scope of the healthcare system", "population priorities and access", "common goal and specific interests", "system capacity and appropriate use of the intervention", "government objectives and policy priorities", "aid program sustainability", "technological innovation", "affordability of medical insurance funds").

2.Weight empowerment results of the value assessment framework for orphan medicinal products based on MCDA

Figure 1. Weight empowerment results based on the five-point weighting method

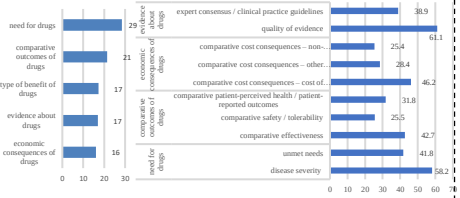

Figure 2. Weight empowerment results based on the two-step percentile distribution method

3.Coordination analysis of the stakeholder experts' opinions

Types of the stakeholder member	Number	Coordination coefficient within the expert grou (five-point weighting method)	Coordination coefficient within the expert group (two-step percentile distribution method)
CN	3	0.49	0.47
CP	2	0.59	0.8
PE	4	0.46	0.32
PR	2	0.67	0.74
DM	2	0.27	0.61
The Kendall coordination coefficient(W)		0.21	0.37
The asymptotic significance		0.002	0.002

4.Results of weighting empowerment of the value evaluation criteria for orphan medicinal products from the perspective of stakeholders

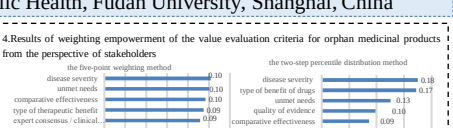

Figure 3. Contrast results of the five-point weighting and two-step percentile distribution method

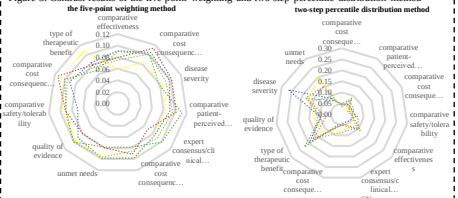

Figure 4. Contrast results of criteria empowerment by stakeholder experts between the five-point weighting and two-step percentile distribution method

5.Results of weighting empowerment for orphan medicinal products based on MCDA from the perspective of the public

A total of 76 questionnaires on the importance preference of criteria were issued, with 71 valid questionnaires recovered, and the recovery rate of the valid questionnaires was 89.9%. The statistic results showed that the standard deviation of the weight of each criterion was 12-19, and the coefficient of variation was 0.3-0.6, indicating the importance preference among respondents were different, and the weight of "type of benefit of drug" had the highest weight among the 11 criteria.

Conclusions:

MCDA is feasible for the value evaluation of orphan drugs in China and can be used as a supplementary tool for drug access decisions in medical insurance. It is suggested to further improve the value assessment framework of orphan medicinal products, scientifically evaluate the MCDA framework empowerment method, explore a framework empowerment system suitable for China's national conditions, refine the scoring criteria to enhance operability, and open the MCDA process to increase decision-making transparency. Broadening the application of the MCDA value assessment framework to orphan medicinal products is recommended.

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1.BELTON V, STEWART T. Multiple guideline decision analysis: an integrated approach [M]. 2.GOETGHEBEUR M M, WAGNER M, et al. Combining multiguideline decision analysis, ethics and health technology assessment: applying the EVIDEM decisionmaking framework to growth hormone for Turner syndrome patients [J] and etc