

MULTI-CRITERIA ASSESSMENT IN HTA PROCESS IN THE CZECH REPUBLIC: THE ROLE OF DIFFERENT STAKEHOLDERS INVOLVEMENT AND POTENTIAL ALTERNATIVE APPRAISAL OF DRUGS INCLUDING ORPHANS

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BACKGROUND

Decision making about reimbursement is based on health technology assessment (HTA), which informs decision maker about the value of technology. There are concerns whether standard methods of HTA adequately reflect societal preferences for the treatment of serious rare diseases.¹ Assessing the value of new medical technologies may require modified approach which takes into account other relevant criteria besides incremental cost per QALY (ICER).² Multi-criterial approach and multi-stakeholder involvement might prevent specific types of drugs being preferred or handicapped when assessing their value. All relevant criteria should be included in order to assess the overall value and reflect different interests. Techniques of multi-criteria assessment aggregate the information on relevant criteria, attach the relative importance to each criterion and present a single expression of value.

OBJECTIVES

The aim of this study was to find out how can the use of multiple criteria in HTA influence the outcome of the assessment in different drug categories, with main focus on orphan medicinal products. Next objective was to explore which criteria are the most important in the assessment of value and to measure the difference in the preferences among three main stakeholder groups in the Czech Republic.

METHODS

The set of 10 criteria was selected by the expert panel and 3 model treatments were described (cancer, chronic and rare disease treatment). Two-round questionnaire was developed and distributed among highly qualified representatives of three stakeholder groups (patients or carers, clinicians and authorities). In the first round, participants were asked to provide weights for each criterion by answering on the scale from 1 to 7. In second round they were asked to score (scale 1–7) model treatments according to their performance in each criterion. Normalized weights were combined with scores and treatments were ranked based on the overall value (Table 1).² Stakeholders' preferences were observed.

Rankings of model treatments were compared to one resulted from using only the limited set of criteria of standard assessment (i.e. cost-effectiveness (CE), budget impact (BI)). The value of orphan treatment based on the choice of the set of criteria for assessment was observed.

Basic descriptive statistic methods were used (arithmetic mean, median, maximum and minimum value). The Kruskal-Wallis test (two-tailed) was used to assess statistical differences among stakeholder groups

Table 1. Rating of treatments using multi-criteria assessment

CRITERION	WEIGHT (%)	CANCER TREATMENT		CHRONIC DISEASE TREATMENT		RARE DISEASE TREATMENT	
		SCORE	WEIGHTED SCORE	SCORE	WEIGHTED SCORE	SCORE	WEIGHTED SCORE
1A DISEASE SURVIVAL PROGNOSIS	10.4	6.20	64.7	4.40	45.91	6.10	63.6
2A QUALITY OF LIFE	11.5	5.70	65.5	5.90	67.8	6.60	75.8
3A UNMET NEED	9.2	5.50	50.8	5.10	47.1	6.80	62.9
4A DISEASE SOCIAL IMPACT	9.7	5.30	51.2	5.70	55.1	6.20	59.9
5B CLINICAL EFFICACY	11.8	5.00	59.2	4.50	53.3	4.30	50.9
6B PATIENT REPORTED OUTCOME	9.0	5.60	50.6	5.50	49.7	5.10	46.1
7B SAFETY	10.5	4.30	45.2	3.80	39.9	4.00	42.0
8B COST-EFFECTIVENESS	9.5	3.70	35.0	4.80	45.4	3.00	28.4
9B BUDGET IMPACT	8.7	3.40	29.5	2.60	22.6	3.50	30.4
10B TREATMENT SOCIAL IMPACT	9.7	4.90	47.4	5.40	52.2	4.70	45.4
TOTAL VALUE*			499.0		478.9		505.4

*Best possible value = 700

Table 2. Assessment of model treatments using multi-criteria vs. standard assessment (only CE, BI)

		Multi-criteria assessment	Standard criteria assessment
RANK BY BEST VALUE*	CANCER TREATMENT	2	2
	CHRONIC TREATMENT	3	1
	ORPHAN TREATMENT	1	3

*1 is the best value

Table 3. Results of multi-criteria assessment in stakeholder groups

		Multi-criteria assessment by		
		Authorities	Clinicians	Patients/carers
RANK BY BEST VALUE*	CANCER TREATMENT	2	1	3
	CHRONIC TREATMENT	3	2	1
	ORPHAN TREATMENT	1	3	2

*1 is the best value

RESULTS

The study was completed by 27 (first round) and 14 participants (second round). Clinical effectiveness and quality of life (QoL) were the most important criteria in all groups (relative weight > 11.5%; see Figure 1). Policy-makers gave markedly higher weight to cost-effectiveness and budget-impact compared to other stakeholder groups (Figure 2). Weights of following criteria were similar in all three stakeholder groups: disease survival prognosis, QoL, unmet need and safety (p>0.31), but in weights of other six criteria there were differences among the groups (p<0.03). In multi-criteria assessment, orphan drug showed the highest value (best weighted score) of model treatments, but when using the standard assessment with limited set of criteria (only CE and BI) it showed the opposite – the lowest value out of studied model treatments (Table 1 & Table 2). Each stakeholder group put different weights/scores and consequent ranking of model treatments was different (Table 3). When using the limited set of criteria, weighted score total value dropped the most (by 26%) in orphan treatment (Table 4).

Table 4. Comparison of value measured using different set of criteria

		TOTAL VALUE weighted score		Value lost	
		Multi-criteria	Standard criteria	Absolute difference	Relative difference
CANCER TREATMENT	All stakeholders	499.0	355.6	143.4	20%
	Authorities	458.6	330.0	128.6	18%
	Clinicians	555.9	400.0	155.9	22%
	Patients	499.5	350.0	149.5	21%
CHRONIC TREATMENT	All stakeholders	478.9	374.7	104.2	15%
	Authorities	431.9	320.0	111.9	16%
	Clinicians	545.7	409.4	136.3	19%
	Patients	484.8	461.1	23.7	3%
ORPHAN TREATMENT	All stakeholders	505.4	323.9	181.4	26%
	Authorities	471.0	290.0	181.0	26%
	Clinicians	533.7	322.5	211.2	30%
	Patients	534.7	411.1	123.5	18%

*Best possible value = 700

Figure 1. Average weights of all considered criteria

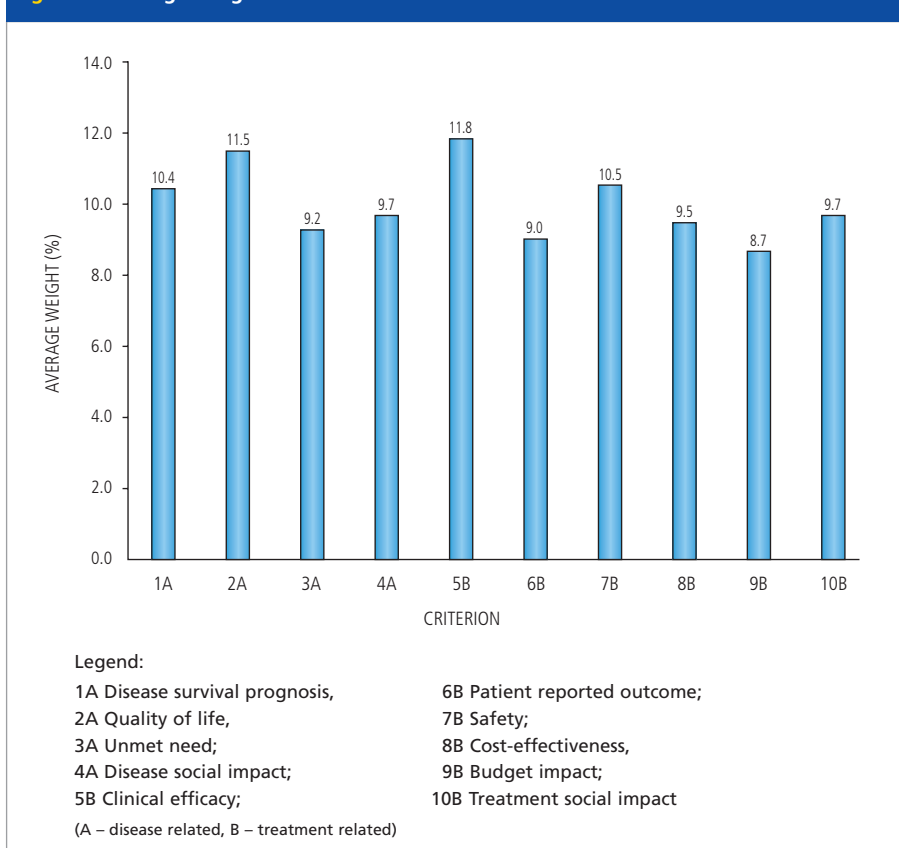
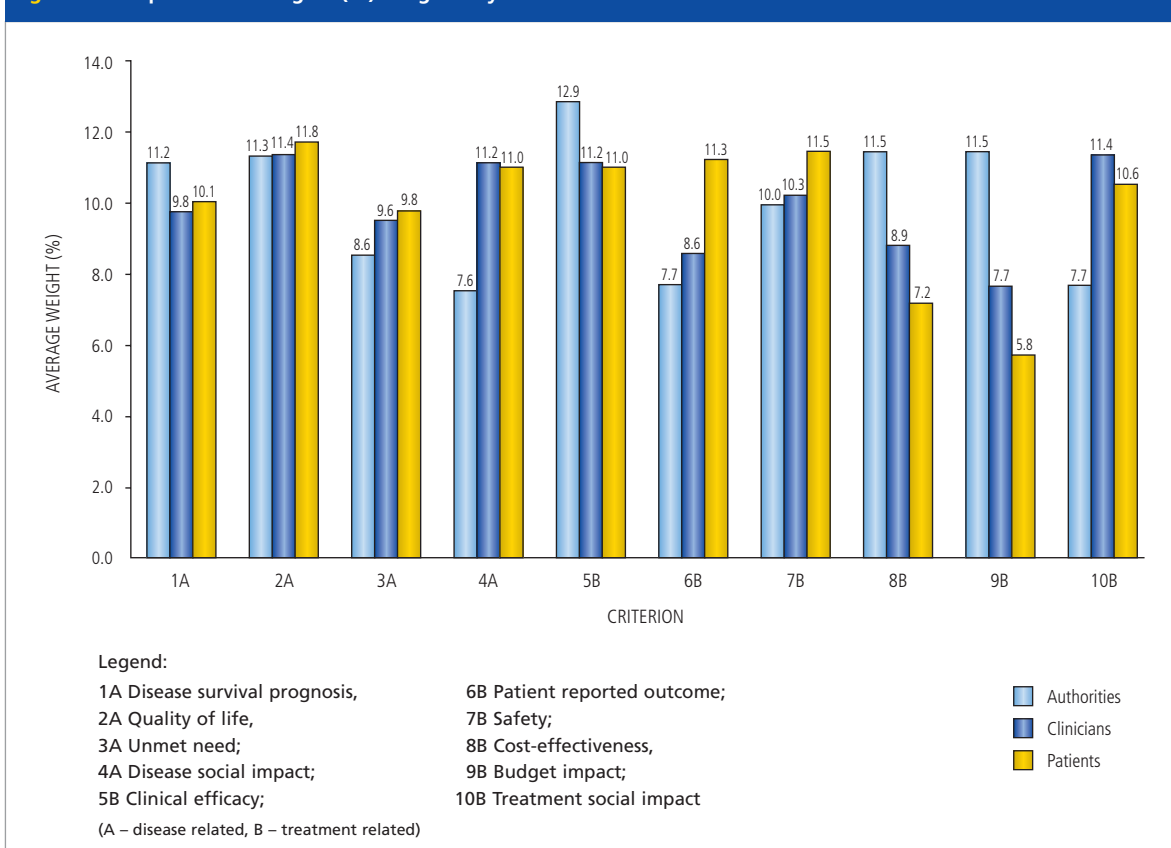


Figure 2. Comparison of weights (%) assigned by different stakeholders



CONCLUSIONS

Multiple-criteria assessment can add a value to HTA in cases where standard cost-effectiveness analysis is not possible to apply or cannot appraise an entire value (e.g. in orphan drugs). Some categories of drugs can be fundamentally affected by non-complexity of criteria used for the assessment. Overall higher societal value of these drugs may not be fully appreciated via standard Czech (but not only Czech) reimbursement process where standard assessment is applied. Preferences varied markedly among stakeholders and therefore all should be ideally, to some extent, taken into account.

REFERENCES

1 Drummond MF1 et al. Assessing the economic challenges posed by orphan drugs. *Int J Technol Assess Health Care.* 2007 Winter;23(1):36–42. • 2 Sussex, Jon et al. A Pilot Study of Multicriteria Decision Analysis for Valuing Orphan Medicines. *Value in Health*, Volume 16, Issue 8, 1163–1169. • 3 Panos Kavanos and Iris Angelis. Multicriteria decision analysis for Value Based Assessment of New Medicinal Technologies: A conceptual Framework. *LSE Health Working Paper No:33/2013* March 2013 • 4 Praveen Thokala et al. Multiple Criteria Decision Analysis for Health Care Decision Making—An Introduction: Report 1 of the ISPOR MCDA Emerging Good Practices Task Force/Value in Health, Volume 19, Issue 1, 1–13.