ABSTRACT

Charles University in Prague, Faculty of Pharmacy in Hradec Králové

Department of	Social and Clinical Pharmacy
Candidate	**Mgr. Eva Ornstová**
Consultant	**PharmDr. Jan Kostřiba, Ph.D**
Title of Thesis	**PARAMETERS OF DRUG ASSESSMENT IN THE THERAPY OF RARE DISEASES**

KEY WORDS:

rare diseases, health technology assessment, health policy, drug policy

OBJECTIVES

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

METHODS

The set of 10 criteria was selected by the expert panel and 3 model treatments were described. Two-round questionnaire was developed and distributed among highly qualified representatives of three stakeholder groups (patients or carers, clinicians and policy makers). In the first round participants provided weights for each criterion by answering on the scale from 1 to 7. In second round participants scored model treatments according to their performance in each criterion. Normalized weights were combined with scores and treatments were ranked based on the overall value. Differences between stakeholder`s preferences were observed. The Kruskal-Wallis test (two-tailed) was used to assess statistical differences among stakeholder groups.

Rankings of model treatments were compared to the one resulted from using the limited set of criteria. The change in the value of the orphan treatment based on the choice for the set of criteria for assessment was observed. Basic descriptive statistic methods were used (arithmetic mean, median, maximum and minimum value).

RESULTS

The study was completed by 27 (first round) and 13 participants (second round). Clinical effectiveness and quality of life were the most important criteria in all groups (relative weight $\square$ 11.5%). Policymakers gave markedly higher weight to cost-effectiveness and budget-impact. All three stakeholder groups agreed on these criteria: length of life, quality of life, unmet need and safety ($p>0.31$). There was disagreement in other six criteria ($p<0.03$). In multi-criteria assessment orphan drug showed the highest value of model treatments, but when using the limited set of criteria (only cost-effectiveness and budget-impact) it showed the opposite – the lowest value out of studied model treatments.

CONCLUSION

Multiple-criteria assessment can add a value to HTA in cases where standard cost-effectiveness analysis is not possible to construct or is not a key to appraise an entire value (e.g. in orphan drugs). Some classes 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 reimbursement process. Preferences varied markedly among stakeholders and therefore all should be ideally taken into account.