**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 various rare diseases. Assessing the value of new medical technologies may require modified approach which takes into account other relevant criteria besides incremental cost per QALY (ICE). Multi-criteria 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, relate the importance to each criterion and present a single expression of value.

**OBJECTIVES**

The aim of this study was to find out how 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 new 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). 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.

**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). Policymakers 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, current need and safety (po.01), but in weights of other six criteria, there were differences among the groups (po.05). In multicriteria 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 4). Weights of following criteria were similar in all three stakeholder groups: disease survival prognosis, QoL, current need and safety (po.01), but in weights of other six criteria, there were differences among the groups (po.05). In multicriteria 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 4).

**CONCLUSIONS**

Multi-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-comparability and therefore should be adjusted in the assessment. Overall higher societal value of these drugs may not be fully appreciated by standard Czech (but not only Czech) reimbursement process when standard assessment is applied. Preferences varied markedly among stakeholders and therefore all should be ideally, to some extent, taken into account.