COST-EFFECTIVENESS ANALYSIS OF RESTRICTED PROTEIN DIET IN PATIENTS WITH PHENYLKETONURIA IN THE CZECH REPUBLIC

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BACKGROUND AND OBJECTIVES

Restricted protein diet (RPD) is the only effective and safe treatment in patients with phenylketonuria (PKU) along with amino acids supplementation excluding phenylalanine. If untreated, PKU leads to severe mental retardation and wide range of other health complications. Although specialized low-protein foods (LPF) are substantially more expensive than regular food, they are not reimbursed from public health insurance which may consequently lead to non-compliance with RPD. The main objective was to assess the cost-effectiveness of reimbursed RPD (i.e. basic LPF) versus non reimbursed RPD in patients with PKU from healthcare payer’s perspective.

METHODS

We developed a lifetime Markov cohort cost-utility model with yearly cycle length and 3 health states, i.e. on diet (normal health), all patients begin in this state, non-compliance to diet (mental retardation) and death. The model structure is shown in Figure 1 and model settings in Table 1. In order to estimate compliance, we conducted our own cost-of-illness study (COI) including approximately half of Czech PKU patients. We assumed that reimbursement of basic LPF (flour, pasta, milk (dried and liquid), rice and eggs) would decrease non-compliance from 34.1% (i.e. COI result) to 15.0% based on income distribution in society.

RESULTS

Over a lifetime horizon, reimbursement of RPD (i.e. basic LPF) compared to no reimbursement of RPD brings additional 0.81 QALY (23.15 vs. 22.35). The total life-time incremental costs are €16,235 (€15,295, €17,235) which is equal to €7,895 per QALY gained (Table 6). The results of PSA-show that reimbursement of basic LPF is cost-effective with probability of 95% at the WTP threshold and increase with increasing WTP (Figure 1 & 2). OWSA and SA consequently showed that probability of non-compliance has the biggest impact on the results along with the cost of RPD and utilities, other parameters had negligible impact (Figure 4). Sensitivity analyses confirmed high robustness of the base-case CE result.

CONCLUSIONS

Reimbursement of basic LPF proved its favourable cost-effectiveness in patients with PKU and therefore it represents good value for money. Further research is needed to assess the cost-effectiveness of alternative intervention strategies.

REFERENCES