Aim: Sorafenib (S), a multitargeted tyrosine kinase inhibitor, has become standard of care for first-line systemic treatment of advanced HCC. Everolimus (E) is a potent inhibitor of the mTOR. In preclinical HCC models, S + E has additive effects compared to S. The objective of the health economic analysis (HEA) alongside the SAKK77/08 trial was to investigate the incremental cost utility ratio (ICER) of the treatment with S + E.

Methods: Patients (pts) with unresectable or metastatic HCC and Child-Pugh ≤7 liver dysfunction were randomly assigned to receive daily S 800 mg (n = 46) or S 800 mg + E 5 mg (n = 60) until progression or unacceptable toxicity. The primary endpoint of the HEA was the ICER for Swiss pts (n = 60). The HEA adopted a health system perspective including all substantial direct medical costs incurred in the treatment of the pts. Health-related quality of life was measured by means of the EQ-5D utility instrument. A generalized linear model (glm) was used to analyze utilities and the costs (using a gamma distribution and a logarithmic cost transformation) for the two arms, controlling for gender and age.

Results: In January 2014 (time point of data extraction) 2 pts in the S (n = 26) and 8 pts in the S + E (n = 34) arm were still alive. The clinical study results will be presented at ASCO 2014. Mean observation time for the HEA was 1.0 years in arm S (95%CI 0.7–1.3) versus 1.1 years (95%CI 0.8–1.4) in arm S + E. Utilities were slightly but not significantly higher in the S + E arm (glm). Total incurred mean costs per patient were CHF 48'396 (95%CI 35'857–60'934; overall cost per year (cpy) CHF 48'564) versus CHF 56'425 (95%CI 43'745–69'104; cpy CHF 52'669) in arm S + E. The costs for S accounted for 42%, S + E for 56% of the total costs in the respective arms. In the glm, total costs were not significantly different between treatment arms and age and sex had no significant effect on the results. The ICER was CHF 58'160/QALY.

Conclusions: Addition of a reduced dose of everolimus to full doses of sorafenib is slightly more costly in Swiss patients. In view of the clinical results the HEA does not favor the S + E strategy.

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