

Cost-Effectiveness analysis of Precision Medicine: The MetAction Study

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Precision cancer medicine (PCM) is currently being implemented into the Norwegian health care system. However, the framework needed to regulate PCM is lacking. In a strategy document by the Directorate of Health, the major bottlenecks for implementation of PCM are pointed out: Identifying the health benefit, data security, regulatory factors and estimation of costs.

Currently, reimbursement of pharmaceuticals is based on cost-effectiveness analysis, where the efficacy typically is based on a meta-analysis or a single randomised controlled trial. Uncertainty is in these analyses represented by assigning distributions from published standard errors to the input factors, such as adverse events, progression free survival and overall survival. Hence, uncertainty is an important output in the documentation at the point of the reimbursement decision. The introduction of precision medicine implies several challenges for the recommended framework: Firstly, what should be considered as a relevant comparator and secondly, how should uncertainty be accounted for and reported as samples sizes are very small.

We will illustrate the first challenge with data from a single-arm study, MetAction. In this study end-stage cancer patient, where screened for actionable target agents. The sample consisted of 26 individuals, where 10 received target treatment (test positives). As MetAction was a single-arm study, we considered several control groups: Firstly, we comparing those receiving target therapy with those not receiving treatment. Secondly, we compare the total patient group in MetAction ($n=26$) with two external comparators. Costs were estimated from a health care perspective and health outcomes in both QALYs and life-years.

The results indicate that the cost-effectiveness depends on the choice of comparator, and when comparing MetAction to an external control, the ICER (incremental cost-effectiveness ratio) is between NOK600 000 and NOK750 000.