Quantitative methods for the early assessment of health technologies. A review of the evidence and policy implications

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Background: Traditionally, regulatory and coverage decisions around novel medical technologies have been based on analyses that are performed late in the product development stage, usually at the end of large clinical studies. More recently, the advantages of anticipating the evaluation process to earlier phases of the technology life-cycle have raised increasing attention among payers and regulators. The basic concept is that an earlier assessment will help to improve clinical development pathways, allow more rapid decision-making, and ultimately reduce the time to access to effective and cost-effective technologies. It may also inform early dialogues between manufacturers, regulators and payers as well as conditional reimbursement schemes such as coverage with evidence development agreements. However, production of early assessments is challenged by a systemic lack of solid evidence on the potential costs and effects of the new technology and other structural uncertainties around future developments of the technology and its competitors.

Objective: to critically review available quantitative tools that may be used for the early assessment of health technologies and their use within an early dialogue or conditional reimbursement schemes.

Methods: early health technology assessment is a relatively novel research area, that is still lacking precise definitions, methods and tools. Therefore, a scoping review was performed to map the existing literature. Electronic databases, including Pubmed, Scopus, Google Scholar, and Embase were searched from January 1995 to December 2017. References have been identified using a *snowball method*, that allows to create a comprehensive network of studies and ultimately to map the available literature in the area.

Results: The review has identified a series of potentially relevant methods, although empirical applications are still limited. These methods aimed to inform decisions relevant to either manufactuers (e.g. stopping rules for product development, maximum reimbursement prices) or payers (cost-effectiveness, value of research). Most of the methods were focused around conducting early economic evaluation models; estimating effectiveness before clinical evidence, and characterizing models parameter uncertainty. Particularly, value of information analysis allows estimating the value of resolving all or part of the uncertainty around the value of a certain technology. This type of analysis can inform what specific parameters in an early cost-effectiveness model contribute the most to the overall uncertainty, and by how much this uncertainty would be reduced after performing a further study with a specified sample size and design. Consistent use of this method may constitute an explicit negotiating base between manufacturers and payers during early dialogues and when defining reimbursement schemes that are conditional to the collection of further evidence such as “only in the context of research” or “accept with research”.