Rational Allocation of Limited Health Care Resources: The Contributions of Epidemiological, Clinical, and Health Economic Studies

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Zusammenfassung der HABILITATIONSSCHRIFT

Rational Allocation of Limited Health Care Resources: The Contributions of Epidemiological, Clinical, and Health Economic Studies

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Zusammenfassung der Habilitationsschrift

For at least two decades, the health care expenditures of virtually all industrialised countries have been increasing faster than gross domestic products. In this situation, health care sectors are rarely perceived as contributors to the creation of societal value but are rather associated with a much debated cost problem. At the health system level, most countries have experimented with interventions in the fields of health care financing, health insurance, and reimbursement of health care providers. Managed care and patient co-payments were introduced or extended. The successes of these measures were highly variable and sometimes, dysfunctional effects (e.g. unintended rationing) were triggered. The field of macro-level health economics strives to address these issues.

In parallel, but more at the meso- and micro-levels, health economic evaluation aims at contributing to a more rational use of health care resources by analysing the costs and benefits of individual medical interventions, or groups of interventions, as a basis for decision making. According to the underlying paradigm, the final goal is to achieve an optimal use of limited resources, and thus maximise population health. Therefore, full-scale health economic evaluation studies (cost-effectiveness and cost-utility analyses) always imply two sides of a coin: an integration of clinical and economic evidence. Other health economic studies, such as budget impact analyses, cost-of-illness studies and studies of the medical resource use associated with disease entities or medical interventions, are more limited in scope but provide important auxiliary information. Epidemiological studies contribute background data on patterns of disease occurrence (incidence, prevalence, and geographical distribution). Moreover, studies with a clinical epidemiology or primarily clinical focus can at times yield important direct hints on how health care resources can be used more efficiently. This is for example the case where genetic or biological markers are identified that allow predict if an expensive treatment will be effective in a given patient, or where risk factors for diseases, clinical events or treatment side effects can be identified and used to efficiently target screening and preventive measures.

The following six publications selected for habilitation span most of the above-described range of possibilities. They all have, in a less or more direct manner, implications for the efficient use of medical resources. The first publication introduces and discusses risk models for the occurrence of febrile neutropenia in patients with non-Hodgkin lymphoma undergoing chemotherapy [1]. The second publication, a research letter, establishes a link between reduced chemotherapy delivery, a frequent consequence of myelosuppression, and long-term survival, in patients with the same disease [2]. In the third publication, the clinical and medical resource use implications of severe oral mucositis, another major side effect of anti-malignant chemotherapy, are assessed [3]. The forth publication reports a population-representative cross-sectional study of gastroesophageal reflux disease and its cost-of-illness implications [4]. The last two papers describe modelling studies of postmenopausal
osteoporosis. The first predicts future osteoporotic fracture occurrence in Switzerland and its economic consequences for the health system [5]. The second reports a full-scale health economic evaluation of the implications of adopting a population-based screening strategy for osteoporosis, with subsequent treatment where applicable, in Switzerland [6].

The febrile neutropenia risk model publication is based on a prospective observational study of patients from five European countries. The multivariate risk models proposed require further validation but appear to have the potential to predict febrile neutropenia with good precision. In particular, low risk patients are successfully identified. This may allow to better target, and hence improve the cost-effectiveness of, expensive prophylactic measures. A randomised clinical trial with an accompanying health economic sub-study should ideally be conducted to confirm this potential, if initial external validation of the risk models is successful.

As a side-finding, the analysis of febrile neutropenia occurrence demonstrates that patient risk is influenced by patient and treatment characteristics but also by clinical practice patterns, e.g. the tendency to delay chemotherapy cycles or reduce doses. In consequence, the administered chemotherapy dose intensity (a composite measure of dose and timeliness of delivery) can be substantially compromised. The second publication shows a negative impact of compromised chemotherapy delivery on long-term survival, based on data from two independent retrospective studies conducted in Belgium and Great Britain. Apparently, administering full chemotherapy dose intensity is important enough to justify expenditures on prophylactic measures. Again, further study is required and could be accompanied by a formal health economic assessment.

The oral mucositis publication is based on a pan-European prospective observational study of patients with non-Hodgkin lymphoma and multiple myeloma. By linking the clinical correlates of oral mucositis with medical resource use implications, it is shown that better management of this adverse condition would not only improve patient well-being but might also lead to savings. This notion is supported by a Poisson regression analysis of influences on duration of hospitalisation which includes severe oral mucositis occurrence as a highly significant predictor variable.

Telephone surveys are a valid and efficient alternative approach to gain information on the occurrence (prevalence, in the present case) of frequent medical conditions and the structure of associated medical resource use and costs, as is exemplified by the study on gastroesophageal reflux disease. The contributions of different cost components are shown and patient characteristics associated with increased total costs (e.g. urban versus rural dwelling) are identified. Such information supports the planning and development of health services and provides input data for subsequent health economic modelling.
The publications on osteoporosis are literature-based and make use of decision-analytic modelling techniques. A Markov model of osteoporotic fracture occurrence was developed and populated with demographic scenarios provided by the Swiss Federal Statistical Office, published epidemiological data, and publicly available Swiss data on duration of hospitalisation, nursing home residency and cost per day of stay. Fracture numbers and the burden to the Swiss health care budgets are projected until 2020.

The original Markov model was adapted to perform a cost-utility analysis of a population-based screen-and-treat strategy for osteoporosis (dual X-ray absorptiometry followed by bisphosphonate (alendronate) treatment if osteoporosis, or osteopenia and a fracture, were found to be present) from the perspective of the Swiss health care system. Population-based screening was found to be cost-effective in women aged 75 or older but not in men. Internationally, several other studies assessed the cost-effectiveness of alendronate but most of them used unrealistic compliance assumptions, disregarded the process of screening and diagnosis, or ignored disease-related events that occurred before a defined screening age. It is shown in the publication that the latter has led to too optimistic judgements on the benefits of screening later in life. The main limitation of the present analysis is that, due to a lack of data, it could not be assessed to what extent cost-effectiveness could be improved by targeting the screening to persons with known risk factors for osteoporosis or falls. Further work is currently being planned to address this issue.

In conclusion, independent of macro-level interventions, improving the efficiency of health care on the basis of emerging scientific evidence and rational decision making can contribute to patient well-being and cost-containment. In some cases, clinical epidemiology or primarily clinically oriented studies make direct contributions to this goal, by providing information that helps to better target screening, prevention, or treatment. In these situations, it is fundamental to achieve a thorough understanding of the underlying disease process and to ensure that any identified associations between predictors and risk factors on the one hand, and patient outcomes on the other hand, are valid. This typically implies the use of appropriate techniques of multivariate statistical analysis and independent validation. In other cases, explicitly health economic studies are needed to generate a sufficient knowledge base for improving the efficiency of health care delivery. Such studies often make use of decision-analytic modelling techniques, in order to integrate data from different sources and to extrapolate beyond the limited observation times of most randomised clinical trials. In these cases, careful selection of model input parameters, transparency, appropriate sensitivity analyses, and adherence to good modelling practice guidelines are of paramount importance. At the same time, oversimplification must be avoided. As is shown in the last of the publications selected for habilitation, health economic models must not ignore important elements of the disease and medical management process, such as disease occurrence before a given screening age. Otherwise, biased results may occur that cannot be detected by standard sensitivity analyses, and the goal of improving the efficiency of health care delivery may not be achieved.
Publications selected for habilitation


