High costs and limited patient benefits – on evaluating interventions in health care

Mikael Svensson

SNS

Summary

MEDICAL TECHNOLOGY is constantly advancing, thus offering increased opportunities to prevent, alleviate, and cure illnesses. During the past 5–6 decades, life expectancy in Sweden has increased by almost ten years. The increased ability to treat and intervene also results in financial challenges. From 2011 to 2019, Swedish health care costs have increased from SEK 415 billion to SEK 549 billion in fixed prices.

Approximately 11% of Sweden's GDP is spent on health care. Considering a population that is growing older and the continued medical technological development, it is reasonable to believe that health care costs will continue to increase.

The question is how to fund future health care to enable the use of new and valuable innovations? The central premise is that an essential answer to this question is stronger priority setting based on scientific evidence – to ensure that interventions creating patient benefits while also being reasonably cost-effective are prioritized and reimbursed. This is due to the fact that most innovations create value for patients, but far from all.

Therefore, the purpose of the report is to analyze whether the current processes for priority setting and introducing new health care interventions in Sweden ensure that what is implemented offers real patient benefits at a reasonable level of cost-effectiveness.

Criteria for priority setting and the ethical platform

The legislated criteria for priority setting in Swedish health care, the so-called ethical platform, include: (1) the human dignity criterion, (2) the need and solidarity criterion, and (3) the cost-effectiveness criterion. These criteria are described

in very general terms by the legislators, and the operationalization of these criteria has been developed over time by government authorities, the health care regions, and bodies such as the Swedish Association of Local Authorities and Regions (SKR).

Priority setting regarding the implementation of new interventions based on the ethical platform requires scientific evidence regarding patient benefits and the cost-effectiveness of various interventions. Priority setting and treatment recommendations based on the best possible scientific knowledge form the basis of evidence-based medicine (EBM). EBM has had a significant impact over the past 30–40 years, and the basis is that the best available scientific evidence and relevant clinical experience should govern treatment recommendations.

Low-value health care

The most systematic use of EBM can be found in the regulatory processes for the approval and reimbursement of new pharmaceuticals. Despite this, new drugs are introduced at very high costs and based on highly uncertain scientific evidence regarding patient benefits and cost-effectiveness. A primary reason for this substantial uncertainty is that it has become a norm that regulatory approval is granted for showing beneficial effects on surrogate outcomes and using single-arm trials. A surrogate outcome is a measure that should serve as a marker for what is actually important for patients – how a drug affects clinically relevant outcomes such as quality of life and survival. An example of a common surrogate outcome is cholesterol levels, which may serve as a marker for the future risk of cardiovascular disease.

The problem with surrogate outcomes is that there is often a great deal of uncertainty as to whether they are reliable markers of the clinically relevant patient benefits. An example is the drug Bevacizumab used for treating advanced breast cancer. This drug was approved after it was shown to improve the most common surrogate outcome in relation to cancer (progression-free survival). However, when studies were later published evaluating the drug's effect on clinical outcomes, it was found that the treatment did not result in any benefits. The only apparent observed effect instead consisted of some severe side effects. Today, most new cancer drugs in Sweden are introduced at very high costs based on demonstrated benefits only on surrogate outcomes. Do they lead to increased survival and improved quality of life? This is uncertain, and pharmaceutical companies are rarely required to produce follow-up studies to confirm that benefits are also observed in the relevant clinical outcomes.

Costly investments in new interventions with no or unclear patient benefits are definitely not only seen for new drugs. An Australian study estimated that approximately 9,000 surgical interventions are performed annually with little or no benefit to patients. These interventions occupy 30,000 day beds per year in Australia. A British study identified 71 surgical interventions having been shown to have no positive patient benefits, but which are nevertheless performed on a regular basis in the UK National Health Service (NHS). The researchers estimated that if only the five most costly of these interventions were stopped, NHS could save around SEK 1.5 billion annually. There is no indication that things look different in Sweden. An example highlighted in the report is a surgical intervention for patients with intermittent claudication, a condition in which patients have a reduced blood supply to their legs and thus experience pain and difficulty walking. Although it is not recommended as a standard first-line treatment for this patient group, surgical interventions are performed to a large extent in Sweden at an annual cost of more than SEK 100 million. A Swedish randomized study with a long follow-up period showed that surgical intervention does not provide better patient outcomes compared to more uncomplicated medication and physical exercise. The use of surgery for this condition also varies significantly between Swedish regions. Such large regional differences in the use of an intervention also indicate an inefficient use of resources.

Optimistic policymakers and unreasonable cost-effectiveness

Why are costly interventions with no, or very uncertain, patient benefits introduced and funded? Several possible mechanisms are discussed in the report, and the focus is on what is referred to as regulatory optimism – too much (optimistic) trust is put on weak scientific evidence. There is thus an unfounded optimism and willingness to "say yes" to new interventions even when the evidence does not provide clear support for patient benefits and reasonable cost-effectiveness. This includes relying entirely on evidence from studies using surrogate outcomes or lacking a comparator ("single-arm trials").

Another factor is that the threshold for what is considered reasonable cost-effectiveness in Sweden lacks an empirical basis. Today, many new interventions being implemented have a cost per gained healthy life year (cost per QALY) of up to SEK 1 million. Since new cost-increasing interventions are typically funded within the framework of a given budget, this means that other interventions are displaced (the opportunity cost). Newly introduced interventions should thus have a lower cost

per QALY than the displaced interventions – otherwise, the fixed budget resources will produce worse health outcomes.

The question is thus whether new interventions are more cost-effective than interventions being displaced. This is an empirical question that is difficult to answer. Still, Swedish and international studies having addressed this issue find that if new interventions with a cost per QALY as high as SEK 1 million are accepted, it is quite likely that the health lost due to interventions is more significant than the health gained from the new interventions.

Policy proposals

The report provides a number of policy proposals that can reduce the risk of funding low-value health care.

STRENGTHEN THE REQUIREMENTS FOR SCIENTIFIC EVIDENCE

Too many new interventions are introduced involving substantial uncertainties regarding treatment effects and cost-effectiveness. A primary reason for this is that the scientific evidence is based on studies without comparators ("single-arm trials") and studies where treatment effects are assessed using only surrogate outcomes. A policy proposal is that this type of evidence should never lead to a general reimbursement or recommendation for introduction. In such cases, only limited reimbursement and temporary recommendations for routine health care implementation should be awarded. The limit may, for example, be set at five years, at which time there should be clear evidence of patient benefits based on studies of good scientific quality – otherwise, the intervention will be displaced. This policy could initially be implemented by national authorities and organizations such as TLV, the NT and MTP councils and later, hopefully, spread to the health care regions' processes for introducing new interventions in an orderly fashion.

EXPAND THE USE OF "NOT-TO-DO LISTS"

The National Board of Health and Welfare publishes "not-to-do" recommendations in national guidelines. Another proposal is that this work should be expanded and summarized in a comprehensive catalog of not-to-do interventions. Such interventions can be identified based on horizon scanning or approaches such as "choosing wisely". Each intervention listed as "not-to-do" should also be assigned a unique action or operation code. When such codes are available for each intervention, results on how frequently these interventions are performed per hospital and region may be compiled in an annual "not-to-do" catalog. Such open comparisons may

highlight low-value care that regions should target for defunding.

REVISE THE COST-EFFECTIVENESS THRESHOLD

A third proposal is to revise the threshold value for what is considered reasonable cost-effectiveness expressed in terms of the cost per gained QALY. Such a revision should be based on empirical work initiated by authorities such as TLV, the National Board of Health and Welfare, and SBU to create better evidence of what constitutes reasonable cost-effectiveness. The threshold value for typical priority setting decisions should be based on the opportunity cost approach given a relatively fixed budgetary framework – new costly interventions should generally have a cost per QALY that is lower than the interventions that are displaced.

STRENGTHEN THE HEALTH CARE SYSTEM AS A KNOWLEDGE-BASED ORGANIZATION

To address the problems and shortcomings described in the report, a fundamental task is to strengthen the health care system as a knowledge-based organization with a culture where implementation and priority setting are based on solid clinical evidence. An important aspect of such a culture is that these professions are able to take part in research and development work. There are some negative signals regarding such opportunities for Swedish health care professionals. For example, it is reported that between 2005 and 2017, the proportion of physicians with a PhD decreased from 20 to 17 percent. Important measures to reverse this trend include increasing the number of positions where health care workers can combine research and clinical work, financial incentives and career progression for health care workers with a PhD, and improving the quality and requirements concerning methodology training in PhD programs in medicine and health sciences.

About the author

Mikael Svensson is Professor in Applied Health Economics at the University of Gothenburg.