

# HEALTH ECONOMICS FOR NON-ECONOMISTS

Principles, methods and pitfalls of  
health economic evaluations

**Lieven Annemans**

Pelckmans Pro

© 2018, Lieven Annemans and Pelckmans Pro  
Pelckmans Pro is part of Pelckmans uitgevers nv  
[www.pelckmansuitgevers.be](http://www.pelckmansuitgevers.be)  
Brasschaatsteenweg 308, 2920 Kalmthout, België

All rights reserved. No part of this book may be reproduced, stored or made public by any means whatsoever, whether electronic or mechanical, without prior permission in writing from the publisher.

Cover design: Filip Coopman ([www.tinxz.be](http://www.tinxz.be))  
Book design: Crius Group

D/2018/13955/18  
ISBN 978 94 6337 113 1  
NUR 801/882

[pelckmanspro.be](http://pelckmanspro.be)  
[pelckmanspro.nl](http://pelckmanspro.nl)

 **Pelckmans Pro**

# CONTENTS

<b>Foreword</b>	<b>5</b>
<b>Introduction</b>	<b>7</b>
<b>Chapter 1</b>	
<b>Why health economic evaluations?</b>	<b>13</b>
1.1 The purpose of health policy	13
1.2 In search of efficiency	15
1.3 Encouraging efficiency	20
<b>Chapter 2</b>	
<b>Definitions and principles</b>	<b>25</b>
2.1 Some definitions	25
2.2 Net saving	27
2.3 Cost-effectiveness	34
2.4 Expressing health effects	37
2.4.1 The 'unit' of health effect	37
2.4.2 The QALY	39
2.4.3 How to measure the index?	41
2.4 The threshold value	48
<b>Chapter 3</b>	
<b>The methods used in health economic evaluations</b>	<b>52</b>
3.1 Decision trees and Markov models	53
3.1.1 General mathematical principles of the decision tree	53
3.1.2 A more realistic decision tree	58
3.1.3 The Markov model: basic principles	64
3.1.4 The Markov model: a real example for the treatment of breast cancer	68

3.2	The validity of health economic models	78
3.3	Sensitivity analysis	80
3.4	Prospective health economic evaluations	88

**Chapter 4**

**Guidelines for health economic evaluations** 96

4.1	Why guidelines?	96
4.2	Summary of the most important guidelines	98

**Chapter 5**

**Health economic evaluations in policy. Problems and trends** 112

5.1	A QALY is not always equal to a QALY	112
5.2	The impact on the health budget	116
5.2.1	An exercise: choosing between seven different strategies	117
5.2.2	Problems with budget impact	120
5.2.3	Different levels of budget impact	121
5.2.4	A new concept: value informed and affordable prices	122
5.3	Dealing with uncertainty	124

**Closing thoughts** 129

**Endnotes** 133

# FOREWORD

When the first edition of this book appeared in 2008, I could only hope that many people who were active (or who wished to become active) in the health sector would show interest in a basic handbook about health economic evaluations. The success of the book demonstrated that this hope was well justified and that there was indeed a need for a practical introduction focused specifically at non-economists.

Like the first edition, this revised version is intended for doctors, dentists, pharmacists, nursing staff, physiotherapists, dieticians, psychologists and other clinicians, as well as employees of the health industry (pharmaceuticals, med-tech, diagnostics, etc.), policy makers, and patient advocates. It can also serve as useful reading for students studying in the wider field of health care.

The most important purpose of the book is to set out the principles and methods for conducting health economic evaluations in a clear and simple manner, illustrated with numerous recent examples. Reading the book must make it possible for health professionals and others to understand the underlying rationale of health economic evaluations and enable them to interpret the results they produce, whilst at the same time allowing them to make a distinction between good and less good evaluations by identifying the potential pitfalls. Hence, the findings of these evaluations can be implemented in health policy and daily practice in the best possible way.

In comparison with the first edition a significant number of changes have been made. The new text not only includes more (and more recent) practical examples, but also devotes attention to new methods (such as those to measure quality of life) and obviously to a number of trends (such as the use of data from real practice), all of which have had a considerable impact on health economic evaluations. The processes and criteria used to support decisions relating to health investments (such as health outcomes-based agreements) are also covered in detail, as is the new concept of value informed & affordable prices.

Of course, the book continues to be very much a first introduction to the subject of health economic evaluations and in many places I provide the reader with literature references for further and deeper study.

I am very grateful to Bart-Jan Van Hasselt (Vintura – The Netherlands) and Hugo Robays (Belgian Commission for the Reimbursement of Medicines) for their relevant comments and suggested improvements to this second edition. They have made the book more readable and more accessible. I would also like to thank all the people with whom I have worked in recent years, both at Ghent University and elsewhere. They were often a source of inspiration and have undoubtedly helped me to improve the manner in which I seek to explain and clarify health economic evaluations.

In particular, I would like to thank Christel, my wife and soul mate, for always being there and also my fantastic children, Silke, Mayke, Toon and Daan, who fortunately have not become health economists but have all found their own unique ways to help make the world a better place.

# INTRODUCTION

The economic evaluation of programmes and investments in health care is a discipline that is enjoying increasing attention throughout the world. In many countries, the providers of health technologies, such as medicines, medical devices, diagnostics, etc., are now obliged to conduct this kind of evaluation, so that policy-makers can have a clearer insight into the costs and benefits of these technologies.

Take, for example, a new medicine. It is logical to assume that the organization paying for this medicine (a hospital, a health insurer or a national or regional health service) wants to know that it is getting *value for money*. It needs to be sure that the money it spends is a proper and worthwhile use of its finite resources. In the health sector, you can only spend your money once, so you need to be as certain as possible that you are spending it wisely. This kind of 'value for money' judgement is being made on an increasing scale in many countries, not only with regard to medicines but also for investments relating to medical devices and diagnostic tools, prevention and screening programmes, programmes for integrated care, etc.

---

## The organization paying for a medicine wants to know that it is getting value for money

---

But what exactly does the term 'spending your money wisely' mean in the context of health care? To answer this question, we need to look more closely at the purpose and usefulness of health economic evaluations.

Chapter 1 puts forward arguments to explain why these evaluations are important and how they can contribute towards better decision-making in the health sector and therefore lead to better health policy and better health care. That being said, at this point I wish to make clear that 'better health care' does not necessarily mean 'cheaper health care'. The primary purpose of health policy is not to save money but to *produce* health, by the prevention, curing or controlling

of illness, so that people can live longer and healthier lives. Or as a report from the European Commission recently put it: *"Health expenditure is recognised as a growth-friendly expenditure. Cost-effective and efficient health expenditure can increase the quantity and the productivity of labour by increasing healthy life expectancy"*.<sup>1</sup> Using European data, researchers from Oxford have shown that when governments spend more on health care, each euro invested in the nation's health ultimately yields an additional 2 euros for the nation's economy over time.<sup>2</sup>

---

## What exactly does the term 'spending your money wisely' mean in the context of health care?

---

The main aim of health spending is therefore to realize health gains. Of course, these gains cannot be 'bought' at any price. Financial resources are not limitless. The benefits must therefore be commensurate with the costs. Consequently, it is becoming clearer (and more widely accepted) that it is no longer possible, for example, for society to pay for extremely expensive treatments for cancer when the health gains they produce are only minimal. This does not automatically mean that we need to close the door on these forms of treatment; it can sometimes be a signal to the company providing the treatment that the price of the treatment needs to come down.

After all, it is a question of ensuring that a health system makes the best possible use of the resources available to it. Or to put it in slightly different terms: that the money society invests in the health system is spent in a way that results in the greatest possible return on investment in terms of the production of additional health.

Of course, this same principle applies in every sector where something is produced: the objective is either to achieve the highest possible level of quality output with the available resources or to achieve the same level of quality output with fewer resources. In other words, savings in the health sector are possible and can of course be useful, providing that they do not result in any loss of quality that would lead to a loss of health.

This 'health economic' way of thinking, this desire to achieve 'cost-effectiveness', obviously becomes more explicit when budgets for health (and health care) are tight. It is self-evident that when resources are unlimited there is a much greater likelihood that society will be willing to pay for all possible preventative, curative and care treatments, providing they yield a health gain of some kind. In practice, however, we see that the available budgets do not allow each and every health investment to be made by the (public or private) health insurance or the national health service. And even if budgets grow, this growth is only likely to be modest, and the limitations remain.

In chapter 1 we will look at the increased importance of health economic evaluations in the health sector. This will also be linked to the concept of equity, which in terms of health care means that two people with the same health needs must receive the same quality of care and that a person with greater health needs must receive a correspondingly higher intensity of care. We will see that striving to achieve both equity and cost-effectiveness is by no means self-evident.

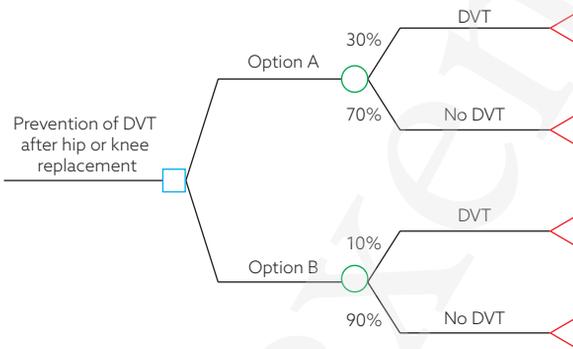
Chapters 2 and 3 look at the core of the matter. It will be demonstrated how health economic evaluations involve the use of different fields of scientific expertise, such as medicine, pharmacology, statistics, epidemiology, psychology, ethics, economics, etc. When an economic evaluation is applied to a medicine, we often speak of a pharmaco-economic evaluation. However, not all evaluations relate to medicines. Evaluations can be carried out for every form of preventive, diagnostic, curative or support technology, programme or device. In other words, it is possible to apply the same evaluation method to care programmes, health promotion campaigns, interventions to improve therapy compliance, for the delivery of pharmaceutical care, integrated care programmes, etc. For this reason, I will often refer in the pages ahead to a *health intervention*, which can mean any of the above.

Using a number of examples, chapter 2 will explain the basic principles of health economic evaluation. Reference will be made, amongst other things, to the incremental cost-effectiveness ratio (ICER) and the quality adjusted life year (QALY). Once we are familiar with the meaning of these concepts, we will move on to look more closely at how 'value for money' can be interpreted in health care.

The principles in chapter 2 are followed by the methods in chapter 3. Attention will be focused on two types of method in particular. The first method relates to decision-making models, which make it possible to simulate what will happen if

a particular decision – for example, adopting a new procedure B in preference to standard approach A – is taken. The interesting thing about these models is the fact that they are based on a medical decision-making logic: “If we do this, the chance of a complication arising is equivalent to  $x$ , and, if the complication arises, the likelihood that the patient will need to be admitted to hospital is equivalent to  $y$ , etc.” An example. Imagine that we want to evaluate the costs and benefits of a new product B (which might be a new drug or a new medical device) for the prevention of deep vein thrombosis (DVT) in patients with a total knee or hip replacement. This option B is then compared with current approach A, based on the results of a clinical study. The study suggests that the chance of DVT with option A is 30%, whereas with option B it is only 10%. These results would then be depicted in a decision tree (figure1):

**Figure 1.** A simple decision tree for the prevention of DVT



Note: the square at the ‘root’ of the tree (at the left side) is known as a decision node and indicates that a choice must be made between the options shown.

So far, so good. But let us now further imagine that option B is more expensive than option A. This does not automatically mean that the use of option B will increase overall expenditure, since the reduced number of DVTs will also save money. To quantify this, however, we need to know how much an individual DVT costs, so we can calculate the potential savings with option B. This requires us to consult other external sources (for example, a database that gives reliable statistical information about the average cost of various medical conditions). Moreover, our calculation needs to take account not only of potential financial savings, but also of possible health gains. A DVT inevitably results in a health loss (and in rare cases can even lead to a fatal lung embolism), which means that if DVTs can be avoided, health loss is also avoided. In other words, avoiding DVTs leads to health gains.

A decision tree therefore allows us to estimate the possible consequences of new health interventions, both in terms of health care expenditure and health gain. The calculation of these potential benefits demands access to financial data and data from other clinical, epidemiological and quality of life studies.

This method, which will be extensively examined (with further practical examples) in chapter 3, is highly popular with health economists. In particular, the so-called Markov model is widely used, and results based on its computations are being published with increasing frequency in clinical journals. We shall see that these models are more than just 'black boxes', but can be developed in a logical, consistent and transparent manner. Admittedly, this demands a degree of mathematical effort on the part of the reader, but it is an effort that will be well rewarded with greater understanding!

In addition to the approach based on decision-making models, there is also a second method that carries out a health economic evaluation within the framework of a comparative clinical trial. Let us again imagine that we want to compare option B with option A. This time we do not make use of a theoretical model, but instead seek to measure in a comparative clinical trial everything that happens to every patient within the study. Traditionally, such a trial only collects health-related data: what complications arise, are there any side-effects, etc. However, the health economic approach also necessitates the collection of all data relating to all aspects of the medical consumption of the patients in question: consultations, (re)admissions, examinations, medicines, surgical interventions, etc. At the end of the study, this yields not only a set of clinical results, but also a set of economic results. At first glance, this appears to be a more attractive option than the decision-making model, because we achieve two things with one effort, in the sense that a single study gives us immediate insight into both the health and the economic consequences of any new therapy. Unfortunately, this method also has a number of drawbacks, mainly related to the artificial character of a comparative clinical trial. These drawbacks are extensively discussed in chapter 3 and we will see how they might be overcome.

Chapter 4 discusses the available guidelines for making health economic evaluations. These guidelines have been applied over the years by health economists so that they can conduct their evaluations as accurately and as reliably as possible. But they are also used by policy-makers and care-providers, so that they can distinguish good evaluations from bad ones and thereby make choices that are well grounded. The professional literature contains a variety of different

guidelines, which I have attempted to synthesize, thereby using examples of studies that did or did not comply with the guidelines. This chapter involves the use of much of the theory mentioned earlier in the book, so that it also serves as a useful test of your knowledge about the basic principles and methods of health economic evaluations.

---

**I believe strongly in the power of these evaluations as an instrument to meet the challenges currently facing health systems**

---

The fifth and final chapter looks more closely at how health economic evaluations can be applied to make policy choices. This is far from straightforward. In addition to cost-effectiveness, several other factors play a role. How serious is the health problem the new intervention is supposed to treat? What is its impact on the health budget? How certain are we about the results? It will become clear that health economic evaluations are just one element within the complex totality of a health technology assessment (HTA). Current trends in HTA will be reviewed, such as the increasing interest in ‘health outcomes-based agreements’, where the final price that the policy maker or health insurer is willing to pay for a particular health intervention is dependent on the performance of that intervention in daily practice.

The book closes with some last thoughts about the use of health economic evaluations. I believe strongly in the power of these evaluations as an instrument to meet the challenges currently faced by health systems. It is a belief I hope the readers of the following pages will come to share.

# CHAPTER 1

## Why health economic evaluations?

### 1.1 The purpose of health policy

Health is one of the most important aspects of our lives. If we are healthy, we function better and we have more chance of being happy. Many countries around the world have developed a health care system that is based in part (some more than others) on solidarity. Society as a whole collects money through a taxation system or through income-related social contributions, which it then invests in the health of its people. However, the financial viability of this solidarity-based model is increasingly under pressure. In general, this is attributable to three factors: the ageing of the population, the non-stop introduction of new (and often expensive) health technologies, and the ever-increasing expectations of people with regard to the quality of health care.<sup>3</sup> For many years, in most Western countries and also in the new growth countries an increase in health care expenditure was observed that was consistently higher than the growth rate of the national economies. It was realized that this trend could not continue indefinitely, but no-one knew – or knows – just how long this situation could last or how large the healthcare growth rate could realistically be. Since the financial-economic crisis that started in 2007, there has been a marked slow-down in the annual increase in health expenditure in many parts of the world. Before the crisis, the average annual healthcare growth rate in OECD (Organization for Economic Cooperation and Development) countries was in excess of 4%. At the height of the crisis, as governments struggled to make financial ends meet, it fell to roughly 0%, although it has now climbed its way back (not without difficulty) to somewhere around 2%.