Health Technology Assessment Handbook

Danish Institute for
Health Technology Assessment

DIHTA

Health Technology Assessment Handbook

1st edition

Finn Børlum Kristensen, Mogens Hørder and Peter Bo Poulsen (Editors)

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Foreword

As part of the fulfilment of the 4th strategy element in The National Strategy for Health Technology Assessment – *the need for research and development of methods for HTA* – the ”Health Technology Assessment Handbook” has been prepared by The Scientific Advisory Board at the Danish Institute for Health Technology Assessment.

The purpose of the handbook is to *provide an introduction to the scientific methods and instruments* in HTA and in particular to the four main elements of an HTA analysis – *The Technology, The Patient, The Organisation and The Economy*. In relation to an HTA project, the Health Technology Assessment Handbook may help to provide some answers to common questions such as how decision-makers' questions are specified in an HTA, how literature is searched and collected, how studies could be designed and how data can be collected and analysed within these four main elements in order to answer the HTA questions posed.

As HTA is often an interdisciplinary activity involving many scientific fields, a further objective of the Health Technology Assessment Handbook is to give the various scientific fields involved in HTA a broader perception of what HTA is, what the conduct of HTA consists of, and which qualifications a project group should have.

The target groups of the handbook are participants in HTA projects and/or initiators of an HTA, that is to say health professionals, political and administrative decisions-makers, interest groups, researchers, etc. It is thereby an advantage to use the Health Technology Assessment Handbook when planning actual HTA projects, as well as when qualifying or assessing project applications.

The handbook may also be used for teaching as well as for courses in HTA. Thus, the Health Technology Assessment Handbook was used with success at the HTA summer school arranged by The Danish Institute for Health Technology Assessment in co-operation with University of Southern Denmark in the summer of year 2000.

The six chapters of the Health Technology Assessment Handbook have been written by specialists educated and experienced in the specific areas, who at the same time remain in touch with and connected to HTA. This should secure the highest possible professional quality and, conversely, also secure that the concepts can be applied in specific HTA projects. Furthermore, an editorial committee has, in collaboration with The Scientific Advisory Board, monitored the preparation of the Health Technology Assessment Handbook.

The present edition of the Health Technology Assessment Handbook is the first one. It is intended that the handbook should be up-dated continuously (every year) and among other include the latest knowledge, which may have been gathered in the previous year through on-going HTA projects.
It is our hope that the Health Technology Assessment Handbook may contribute to further improving the quality of HTA work in Denmark.

Contents

Chapter 1: Stating the Problem.....……....page 6-13
by Director, professor, Ph.D. ***Finn Børlum Kristensen****,*
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Chapter 2: Information Searching And Critical Appraisal…….page 14-21
by Information Specialist, Librarian, ***Malene Fabricius Jensen****,*
Danish Institute for Health Technology Assessment

Chapter 3: The Technology…….page 22-35
by Professor ***Leiv Bakketeig****,*
Epidemiology, Institute of Public Health, University of Southern Denmark

Chapter 4: The Patient…….……...page 36-65

Chapter 4a: Field Research, Interview and Questionnaire Studies …..……page 37-55
by Senior Researcher, Ph.D. ***Helle Timm***
The University Hospitals Centre for Nursing and Care Research, Copenhagen
Associate Professor, Ph.D. ***Helle Ploug Hansen***
The Faculty of Health Sciences, University of Southern Denmark
Associate Professor, Ph.D. ***Janine Morgall***
Department for Social Pharmacy, The Danish School of Pharmacy
Advisor, M.Sc. (Tech.Soc.) ***Helga Sigmund***
Danish Institute for Health Technology Assessment

Chapter 4b: Measurement of Health Status…….…….….page 56-65
by Assistant Professor, Ph.D., M.Sc. (Econ) ***Peter Bo Poulsen***
Health Economics, Institute of Public Health, University of Southern Denmark

Chapter 5: The Organisation……..page 66-95

Chapter 5a: HTA Administration and Organisation …….page 67-84
by Assistant Professor, Ph.D. ***Karsten Vrangbæk***
Institute of Political Science and Institute of Public Health
University of Copenhagen

Chapter 5b: The Organisation…… …page 85-95
by Assistant Professor, Ph.D. ***Kjell Tryggestad***
Professor, Ph.D. ***Finn Borum***
Department of Organization and Industrial Sociology (IOA)
Copenhagen Business School

Chapter 6: The Economy……..page 96-121
by Assistant Professor, Ph.D., M.Sc. (Econ) ***Peter Bo Poulsen***
Health Economics, Institute of Public Health, University of Southern Denmark

Chapter 1: Stating the Problem

by

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Contents for chapter 1:

1.1 IS IT HTA OR A DIFFERENT APPROACH WHICH IS NEEDED? 7

1.2 IS ENOUGH DOCUMENTATION AVAILABLE TO PERFORM AN HTA? 8

1.3 PRIORITY-SETTING: IS THE SUBJECT IMPORTANT ENOUGH TO USE RESOURCES FOR AN HTA? 8

1.4 WHO IS THE TARGET GROUP? 8

1.5 WHO SELECTS THE SUBJECTS FOR HTA? 8

1.6 WHAT IS THE ORGANISATIONAL STARTING POINT FOR THE HTA? 9

1.7 SPECIFIC QUESTIONS THAT CAN BE ANSWERED 9

1.8 DESIGN OF PLANNING AND POLICY QUESTIONS 10

1.9 DESIGN OF THE HTA QUESTIONS 10

1.10 ESTABLISHING A PROJECT GROUP 11

1.11 REFERENCE GROUP 11

1.12 THE COMMON WORK FIELD OF THE PROJECT GROUP 12

1.13 CORRELATION BETWEEN PROBLEM, SYNTHESIS AND ABSTRACT 12

LITERATURE FOR CHAPTER 1 12

A health technology assessment (HTA) is a research based, applied assessment of relevant available knowledge of problems, when applying technology in relation to health and disease. HTA is related to research due to its methods, but is also related to planning, administration, and management due to its focus on decision-making. HTA can thus be seen as a bridge between a science paradigm and a policy paradigm. (Battista & Hodge 1995). To fulfil their aim, the central problem statements in an HTA must take their starting point in the needs of the decision-makers (and their advisers) for a documented basis for deciding whether or not to apply health technology and to what extent.

The problem could be the introduction of a technological innovation, which needs to be studied in relation to other already presently applied technologies in the area (e.g. positron emissionstomography (PET-scanning) or computerised MR- and CT-scans of the large intestine (virtual colonoscopy)). Or the problem could be that there are uncertainties about the basis of the use of commonly applied technologies in connection with a certain clinical problem (e.g. diagnostics and treatment of low back pain and hormone stimulation used in in-vitro fertilisation (IVF-treatment)).

HTA is a comprehensive, systematic assessment of the conditions for, and the consequences of using health technology. HTA includes analysis and assessment of a number of areas, where use of the health technology may have consequences. These can be divided into four main elements: the technology, the patient, the organisation and the economy (Health Technology Assessment 2000). The main elements partly overlap each other, Ethics, for example, which is placed under the element “the patient”, cannot be separated from the analysis of the technology, as ethics may form the framework for analysis cross the elements (Andersen 2000).

1.1 Is it HTA or a Different Approach Which is Needed?

It is useful to clarify whether HTA is the right instrument to use for the particular problem. It may conceivably be more beneficial to apply a different approach.

Alternative procedures to clarify the problem

- An HTA
- A quality-assurance project (if one knows what should be done in the particular organisational situation, but what is presently done, is not the right approach (!))
- A basis for decision-making is developed in the usual administrative framework (if, for instance, a national HTA or an HTA from a region is available.)
- A traditional expert and/or stakeholder committee (if the aspect of stakeholders is very important, or if the opinion of particular expertsis desired, or if only little time is available).
- Exclusively a systematic literature review, possibly a meta analysis, to determine the clinical effects and efficiency of the technology.
- An economic analysis (if sufficient knowledge of the effect and efficiency of the technology is available, and if there are no specific organisational questions) e.g. of drugs.
- a (primary-) research project (if documented research is simply not available, especially of the clinical effects).

None the less, it is a good idea to apply the HTA line of thought, if it is decided to use one of the above approaches, which is not HTA. This will so to say force the considerations into the relevant clinical, organisational, economic and ethical areas. Thereby, diversity will be maintained and ensure that decisions are not made on a restricted basis.

1.2 Is Enough Documentation Available to Perform an HTA?

It only makes sense to make an HTA, if there is sufficient – but not necessarily complete - documented information present. Is this not the case, it may be necessary to initiate primary research in order to collect this information, and decision-making must be based on expert evaluations until a more documented basis is established. Far too often, however, there is not put enough effort into a systematical search for and assessment of available results of research already made. This could result in the initiation of primary research that may be redundant (Chalmers 2000) – or expert assessments, which are not based on a systematic literature review, is used. The latter is the most common. The experts, who can assure that their advice is based on a thorough literature review of the relevant literature, should be the ones who have the largest say . However, there is quite often a need for data collection, e.g. to establish the epidemiology, the actual diffusion, etc. (see chapter 3 about the **technology**).

Only seldom a complete, thoroughly documented set of data will be available at the beginning of an HTA – often something will be missing – for instance examination of the patient/user aspects or organisational aspects. To clarify these aspects one should, to start with, consult the experts of these aspects, and at the same time make a preliminary literature search for HTA reports, review articles and health economic analyses (see chapter 2 about **information search and assessment**).

1.3 Priority-setting: Is the Subject Important Enough to use Resources for an HTA?

In a formal HTA organisation, there will often be principles for choosing between potential HTA's (Goodman 1998). This is the case at the Danish Institute for Health Technology Assessment, DIHTA (Strategy plan, DIHTA, 2000) for instance. Also in departments, in hospitals and in the county administrations the resources used for projects should be prioritised. Therefore, it is important to have a solid basis for assessing the importance of an HTA.

1.4 Who is the Target Group?

An essential opening question is, who is the primary target group of a possible HTA? This has great importance for the wording of the problem, and for the presentation of the final report.

Primary target groups

- Politicians and civil servants at national or regional levels
- Planners at county or hospital levels
- Administrations in hospitals or clinical departments
- Organisations and companies
- Citizens in general

1.5 Who Selects the Subjects for HTAs?

The fact that HTA is aimed at decision-makers does not necessarily mean that these persons demand an HTA of a given problem, even though this may also be the case – and hopefully this will increase in the future. If an HTA is demanded, the ones giving the assignment are more likely to be

policymakers, who are expected to provide a possible basis for decision, should a policy be called for in the area.

In most cases, it is the institution or the local experts, assigned to make HTAs, who put subjects on the agenda, based on a more or less formal analysis of demand and opportunities for HTA. The institution or local experts will expect that there is or will be a need for assessments before decision-making. Some HTA institutions or programmeslike the HTA institution in the UK., have formal systems for continuous collection of suggestions for HTA subjects.

1.6 What is the Organisational Starting Point for the HTA?

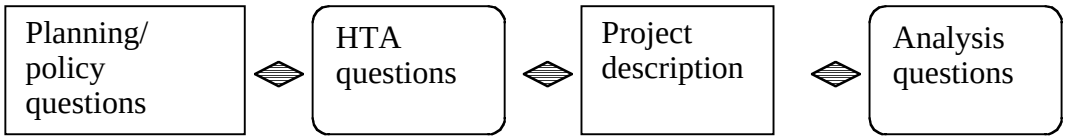
Organisational basis for the HTA

- Policy/planning
- Daily running
- Research/development

The organisational starting point can determine who the target group is. Regardless of whether or not someone is in fact giving the assignment, it should be determined, who the primary users of an HTA will be. If the HTA is made in a hospital unit or in a clinical department, it will be management who is the primary target group. In the same way, the planners in the county are the primary target group, if the HTA is made in the framework of a county. When the HTA is made in a research/development institution, thorough consideration of the target group is called for, especially when there is no external party asking for the HTA. An HTA should not be made without a policy purpose in mind!

1.7 Specific Questions that can be Answered

The wording of the problem is an iterative process, which may consume quite some resources, but which is worthwhile to spend time on. A group that includes a combination of persons who have clinical knowledge of the field, administrative knowledge and knowledge of the HTA method, will have the best chances of reaching a suitable problem definition. Schematically, the actual project definition can be described in three steps.



In accordance with the nature of HTA as an applied method, the formulation of the problem must be specific and only aim at existing problems. For instance, if there are no special ethical or organisational problems, one should not ask questions or spend resources finding and examining these. However, one must early on make a broad, open examination of where the main questions are – one or more HTA elements should not be excluded beforehand, the choices must be explained specifically. One should - to ensure the applicability of the final work – focus on which information the decision-makers need.

1.8 Design of Planning and Policy Questions

Instead of going directly to the formulation of the project description and literature search, one should, in accordance with the idea of HTA, seek to determine the planning/policy questions which an HTA can contribute to answering. Put simply, the policy questions could be: Is there a substantial effect of improved health or other beneficial effects? Which demands are there with respect to staff and administration? What are the costs? And are there other factors one should pay attention to? If one works within the framework of a single organisational unit, e.g. a department, it is the questions of the management of this department, which must be answered. In such a framework, clearly the policy questions should be posed in close collaboration with management, based on the HTA line of thinking. But the final formulation is the repsonsibility of the HTA persons, as part of a systematic HTA process.

Examples of planning/policy questions prior to an HTA

- Should there be a wish to introduce a public offer of influenza vaccination of the elderly, how should this be organised and what would the effects and the costs be?
- How can the health care sector's **diagnosis of colo-rectal cancer** be organised around suited clinical strategies – with what effects and at what cost?

The content – the core of the HTA - what is it the HTA should examine?

1.9 Design of the HTA Questions

To fulfil the purpose of HTA, the work that answers the planning/policy questions should examine the HTA questions, which can be derived from the decision-makers' need for information. In that way, the HTA can contribute to a documented basis for decision-making.

The HTA questions must

- Be clearly worded
- Be clearly defined
- Be answerable
- Be limited in number

In order to keep track of things the questions can be classified within the framework of the usual main elements; **the technology, the patient, the organisation and the economy**, but that is more of an internal HTA matter to secure that one, in all phases of the HTA, examines the problems thoroughly, than it is something the users need to know of.

Often new questions arise during the HTA process, but usually it is advisable to keep to and answer the questions agreed upon originally, and only thereafter examine new questions. If not, the change may cause confusion and thereby result in lack of commitment. It is advisable to engage an HTA expert for clarification.

Examples of derived HTA questions – influenza vaccination

- How effective is the vaccine in preventing the disease and its effects?
- Which health benefits can be expected from a general offer of inoculation of the elderly?
- Are there relevant alternatives to the influenza inoculation?
- How may the inoculation be offered?
- How do the elderly view the offer of inoculation, and what role does the question of payment play?
- What will the inoculation cost in various models of provision – for the individual, for the government and for society?

Examples of derived HTA questions – colo-rectal cancer

- What is the status of present diagnostic methods and strategies?
- Are there alternative methods and strategies?
- What are the organisational consequences for the alternative strategies?
- What are the patient related aspects of the problem?
- What are the resource consequences?
- How will a possible screening affect the organisation and the economy?

When conducting the particular HTA analyses, the HTA questions will perhaps have to be specified in further detail, in the form of analytical or descriptive research questions cf. the following chapters.

1.10 Establishing a Project Group

It is the specification of the problem that determines which areas, authorities and staff groups should be included in a project group – and not the tradition of who is usually included. One should pay attention to the fact that HTA does not necessarily limit itself to one department. The whole sequence in which the technology is involved must be included – both across work fields and across sectors. To ensure commitment, strategy and planning of the project course, it is important that all relevant authorities are included at the beginning of the project.

If the project group tends to become very large, it could be an advantage to divide this into sub-groups, which will each work with elements of the HTA. However, if one chooses this work form, it demands that the project group meetings are controlled strictly, in order for the group to discuss all relevant issues. Thus, it will demand more of the project management, when dividing the project group into sub-groups.

1.11 Reference Group

In some circumstances it would be better to supplement the project group with a reference group, which during the project can provide review, advice and guidance. The participants in reference groups will often be stakeholders related to the health technology, e.g. administration,

representatives from the administration and representatives from staff unions and patient organisations.

1.12 The Common Work Field of the Project Group

The project group should be organised with a project leader and agree on a mandate for the task.

To create the basis for a good and fruitful collaboration, one must first reach agreement on the following:

- Wording and demarcation of the problem - what is the question and what is it one wants to examine?
- Clarification of the alternatives, which should be examined?
- Strategy for information search – what has been done previously and with what evidence?
- Planning of time schedule/meeting schedule for the project period
- Planning of the work phase – who does what and when?
- Planning of the final stage – how, in which format and to whom should the result be presented?
- Planning of implementation in health care seem to be called for, what options are there?
- Strategy for follow-up and solving of derived questions.

1.13 Correlation Between Problem, Synthesis and Abstract

At the end of the HTA project, it is important to make a link back to the original planning questions of the problem. The analytical HTA questions of the project, and the sub-analyses, which have been made, must be captured and synthesised to the product, which is passed on to the decision-makers, in order for this material to be part of the basis for decisions that may have to be made on the issue.

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Chapter 2: Information Searching And Critical Appraisal

by

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Contents for chapter 2:

2.1 INTRODUCTION 15

2.2 BACKGROUND AND HTA PROBLEM 17

2.3 INCLUSION AND EXCLUSION CRITERIA 17

2.4 STRATEGY FOR COLLECTING THE LITERATURE 17

2.4.1 WHICH INFORMATION SOURCES SHOULD BE APPLIED? 17
2.4.2 HOW ARE THE VARIOUS SOURCES SEARCHED? 18
2.4.3 EVALUATION OF THE SEARCH 18
2.4.4 FOLLOW-UP ON THE SEARCH 19

2.5 STRATEGY FOR CRITICAL SELECTION AND APPRAISAL OF THE LITERATURE 19

LITERATURE FOR CHAPTER 2 20

SELECTED INFORMATION SOURCES 20

2.1 Introduction

A health technology assessment is to a large extent based on available evidence. Therefore, a literature review should always be performed rather early in the HTA process.
It is advantageous to use the five principles of Evidence Based Medicine in the literature review.

1. Formulate answerable questions
2. Find the best available evidence
3. Select and appraise the evidence
4. Apply in practice
5. Evaluate the process

(Sackett 2000)

A literature review must be planned in detail. Here, it is a good idea to work out a **search protocol** (see example in Figure 2.1), which should include the following components:

- **Background and problem** (including the derived HTA questions)
- **Inclusion and exclusion criteria**
- **Strategy for collecting the literature** (which information sources should be consulted, and how are the different sources searched?)
- **Strategy for critical selection and appraisal of the literature**

The search protocol has to be sufficiently detailed, so that by following the description, the search can be performed again with the same result. That is to say, it should include information about not only how you *intend* to perform the literature search, but also how you in fact *did search* for it.

Problem: Influenza vaccination of the elderly	
HTA question – the technology: What is the expected survival of the elderly, who are vaccinated against influenza, compared to elderly, who are not vaccinated ? **Inclusion criteria:** persons aged 65 years or older; randomised controlled trials **Exclusion criteria:** experimental studies	
Information sources/databases –*examples*: The Cochrane Library Medline (PubMed)	**Search strategy:** 1. influenza*:me OR influenza 2. mortality*:me OR mortality 3. 1 AND 2 4. influenza NEXT vaccine 5. influenza-vaccine*:me 6. 4 OR 5 7. 3 OR 6 8. aged*:me 9. 7 AND 8 1. "influenza/mortality"[MeSH Terms] 2. "influenza vaccine"[MeSH Terms] 3. 1 OR 2 4. "aged"[MeSH Terms] OR "aged, 80 and over"[MeSH Terms] 5. 3 AND 4 6. "human"[MeSH Terms] 7. 5 AND 6 8. "randomised controlled trial"[publication type] 9. 7 AND 8
HTA question – the patient: What do the elderly think of influenza vaccination ? **Inclusion criteria:** persons aged 65 years or older	
Information sources/databases - *examples*: Medline (PubMed) PsychInfo (Ovid)	**Search strategy:** 1. "influenza vaccine"[MeSH Terms] 2. "attitude to health"[MeSH Terms] 3. 1 AND 2 4. "aged"[MeSH Terms] OR "aged, 80 and over"[MeSH Terms] 5. 3 AND 4 1. exp influenza/ 2. exp vaccination/ 3. 1 AND 2 4. attitude 5. 3 AND 4

Figure 2.1: Extract from search protocol - example

2.2 Background and HTA Problem

The systematic information search will be formed by the individual HTA problem. Here, it is important to remember that an information search must include all relevant areas, and that the sources will differ from sub-area to sub-area.
The questions examined in the search should be clarified – including specification of the starting point of the search (e.g. disease, diagnosis, method, treatment), and in which contexts the issue should be examined. Often, an initial search will be able to help specify the problem in the particular HTA. The following searches take their starting point in the final problem, and could also clarify the sub-elements of the HTA.

2.3 Inclusion and Exclusion Criteria

From the selected studies, what demands are made in order for them to be included in the HTA? Here one should consider which of the following should. be included: e.g only a particular age group, both clinical and experimental studies, only patients with a certain course of disease, only male or female individuals, only literature from a certain time-period, and which study designs should be included (e.g. randomised clinical trials , meta analyses, cohort studies).

2.4 Strategy for Collecting the Literature

2.4.1 Which information sources should besearched?

A good starting point is to investigate whether HTAs regarding the particular question have already been performed. The database "The Health Technology Assessment (HTA) Database" contains information about completed and on-going HTA projects worldwide. Information about the Danish HTA projects can be sought in The Danish HTA Project Database on DIHTA's homepage.

<u>The technology</u>
For the technology, The Cochrane Library, Medline and EMBASE are central databases. The Cochrane Library consists of six databases including systematic reviews done by Cochrane Review Groups, references to controlled clinic trials, references to economic evaluations, and a number of methodological studies. Medline is produced by the National Library of Medicine and is based on articles from more than 4,000 journals, primarily within the medical area. EMBASE covers basically the same areas as Medline, but has a larger coverage of pharmacological literature and includes more European journals than Medline.
In addition it is necessary to search in other databases - e.g. if nursing, physiotherapeutic or ergotherapeutic problems need to be investigated.

<u>The Patient</u>
Literature that clarifies questions on the patient/user perspective can be found in databases like Sociological Abstracts, PsycInfo and Medline, but often it will be necessary to supplement this information with literature and information from other sources. This might be found on the patient organisations' websites, The Danish Council of Ethics, or others.

The Organisation
Literature about questions on the organisation can be found in the databases DSI-Bib (DSI •Institut for Sundhedsvæsen), and HealthStar, just as the Library of the Copenhagen Business School has access to a number of databases concerning organisation.

The Economy
The NHS Economic Evaluation Database includes assessments of already published health economics analyses. References concerning health economics can also be found in the DSI-Bib database. The Institute of Public Health at University of Southern Denmark has also published a number of reports that include health economics analyses.

In the end of this chapter is a list of some of the most important information sources within the field of health technology assessment.

2.4.2 How should the various sources be searched?

Based on the problem (or the derived HTA questions) a search strategy is established (see Figure 2.1). The various sources require separate search strategies. Searching bibliographic databases should be based on a detailed search strategy, where it is noted beforehand how searches are to be performed in the particular database, which search words are used, and how these words are combined in order to make the search as precise and exhaustive as possible. Apart from search words which describe the subject (subject filter), the search strategy should also include considerations as to which research design should be included in the search (methodological filter). When searching for research designs (e.g. randomised controlled trials and meta analyses), a number of search filters have been established, which can be used when searching e.g. Medline. At the end of this chapter there is a list of examples of such search filters.

A search of databases must frequently be supplemented with information found in untraditional sources e.g. via the Internet (pharmaceutical companies, patient organisations), in reports, in conference literature, and by handsearching journals etc. A number of databases are available free of charge via the Internet, while others require subscription. Many hospital and university libraries have access to databases on a pay-per-view basis. Selecting information sources will often benefit from the assistance of a librarian or an information specialist, who has knowledge of the various databases, and of how to search these.

2.4.3 Evaluation of the search

If prior to the search one has a well-known article (and preferably a more recent one) on the subject, this article can be used as a "control" of whether or not one has found what one was looking for. If the article is not part of the search result, it could be because the article is too new, or that the journal is not represented in the particular database, and not necessarily because the search was not sufficiently precise. However, if the article is indeed included in the database (but not in the search result) the “good” article’s subject headings can be used to adjust the search strategy in the database.

2.4.4 Follow-up on the search

At what point in the process the search is repeated depends, among other things, on the magnitude of the project (assessment of a broad or narrow, an established or an emerging technology) and the time schedule of the project (does the project run for several years or a few months). In some projects it is sufficient to repeat the search at the end of the data collection period, while in other projects the search must be performed with an interval of a few months. Regardless of how often the search is repeated during the project period, it is important that at each search, information regarding the following is stored:

- Which sources have been consulted (databases, journals, Internet-addresses etc.).
- Which years did the performed search cover?
- How the search was performed (which search words were applied, in which fields and with which combination of words)?
- Date of the search.

Documentation on searches in sources that provided useful information, as well as the ones that did not, must be kept and included in the search protocol. The criteria used for assessment of which information should be included and which should be excluded in the HTA should also be apparent.

2.5 Strategy for Critical Selection and Appraisal of the Literature

The literature found should be reviewed and appraised systematicallyon the basis of relevance to the problem.

The titles and abstracts should then be reviewed, references of no interest excluded and the chosen articles obtained. Often there will be access through the libraries of the institutions/hospitals or the university libraries to full-text articles on the Internet. The remaining literature is ordered via the libraries.

Each article is assessed based on the following questions:

1) Is the article **relevant** to the subject?
2) Are the article's results **valid**?
3) Are the article's results **important** for answering the question?

In order to facilitate the appraisal, check lists have been made with a number of questions to be answered when reviewing the article. There are a number of check lists which can be used for appraising studies of the various HTA elements, and of studies with different research designs (e.g. systematic reviews, cohort studies, qualitative studies). Examples of these check lists can be found at the end of this chapter.

In addition, descriptions of methods to be used for literature appraisal within the particular HTA elements can be found in following chapters.

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ISTAHC database (http://www.istahc.org)
The Cochrane Library* (http://www.update-software.com/cochrane/cochrane-frame.html)
HealthStar (http://www.nlm.nih.gov/pubs/factsheets/healthstar.html)

Technology
The Cochrane Library * (http://www.update-software.com/cochrane/cochrane-frame.html)
Best Evidence* - consists of ACP Journal Club and Evidence Based Medicine (http://www.bmjpg.com/data/ebm.htm)
DARE – Data base of Abstracts of Reviews of Effectiveness (http://agatha.york.ac.uk/darehp.htm)
Medline (PubMed) (http://www.ncbi.nlm.gov/entrez/query.fcgi)
Embase* (http://www.elsevier.nl/inca/publications/store/5/2/3/3/2/8/)
Science Citation Index*
Subject specific data bases, as e.g. CINAHL (nursing), PsychInfo (physiology), AMED (physio- & ergotherapy, alternative treatment) – payment for the use of these is required
Websites of pharmaceutical companies

The Patient
PsycInfo*
Sociological Abstracts*
Medline (PubMed) (http://www.ncbi.nlm.gov/entrez/query.fcgi)
Embase* (http://www.elsevier.nl/inca/publications/store/5/2/3/3/2/8/)
Social Science Citation Index*
The Campbell Collaboration (http://campbell.gse.upenn.edu/index.html)
Websites of the various patient organisations in Denmark (lists are located at http://www.dadl.dk and http://www.netdoktor.dk)
The Danish Council of Ethics (http://www.etiskraad.dk)
Department of Social Pharmacy, DFH (http://www.dfh.dk/indexinsts.html)
Danbib*

The Organisation
HealthStar (http://www.nlm.nih.gov/pubs/factsheets/healthstar.html)
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Search filters and check lists for literature appraisal

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Chapter 3: The Technology

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Contents for chapter 3:

3.1 HEALTH TECHNOLOGY 23

3.2 ASSESSMENT OF THE TECHNOLOGY 23

3.3 LOOKING CLOSER AT THE INDIVIDUAL ASPECTS OF HEALTH ASSESSMENT 24

3.3.1 THE FIELD OF APPLICATION OF THE TECHNOLOGY 24
3.3.2 HOW IS THE EFFECT OF THE USE OF A HEALTH TECHNOLOGY THEN MEASURED? 24
3.3.3 RISK ASSESSMENT 26

3.4 THE NEED FOR PERFORMING ONE'S OWN STUDY OF THE EFFECT OF HEALTH TECHNOLOGY 27

3.4.1 RANDOMISED CONTROLLED TRIALS 27
3.4.2 OBSERVATIONAL EPIDEMIOLOGICAL STUDIES 29

3.5 VALIDITY 31

3.6 OTHER SOURCES OF ERROR 33

LITERATURE FOR CHAPTER 3 35

3.1 Health Technology

Health technology is a term which includes procedures and methods for examination, treatment, nursing and rehabilitation of patients, including equipment and drugs. The term also includes procedures used for preventive care.

3.2 Assessment of the Technology

The following main aspects needs to be assessed:

- Field of application
- Effectiveness
- Risks

Field of application

The aim of the technology can roughly be divided into the following:
- **Preventive care**, aimed at preventing diseases from occurring (primary prevention).
- **Screening**, aimed at detecting early signs of diseases or risk factors, with the aim to slow down the development of the disease (secondary prevention).
- **Diagnosis**, aimed at identifying diseases in patients with clinical signs and symptoms.
- **Treatment**, seeking to maintain health status, cure the patient or provide palliation.
- **Rehabilitation**, which takes its staring point in the treated, but still ill patient and seeks to restore the functioning or minimise the consequences of dysfunction or defects (tertiary prevention).

The Field of application is then assessed further based on:
- What the indication is, including whether there is a reasonable consensus among the specialists.;
- How widespread the use of the technology is and how many patients does it affects;
- Whether there are alternative treatments or technologies;
- Or whether the technology is a supplement to established technologies.

Effectiveness is assessed by answering the following questions:
- Is the effect of the technology scientifically documented?
- Is the effect better than alternative technologies?
- Is it realistic that the documented effect can be achieved in actual treatment situations?

Risk assessment is made by:
- Focussing on undesired side effects;
- Assessing whether the possible risk of side effects is reasonable, considered what can be gained by using the technology.

3.3 Looking Closer at the Individual Aspects of Health Assessment

3.3.1 The field of application of the Technology

With the health problem as the starting point it should be assessed as to whetherthe technology is presumed to treat or prevent. The health problems should then be described in detail. For example, ultrasound equipment and the examination procedures related to this could be a useful technology applied to a number of diseases, partly on the therapy side, and partly when used for diagnosis, including being part of screening procedures.

Description of the separate diseases should be based on common medical usage, being the aetiology of the disease, incidence, symptomatology, development, prognosis and consequences, including how life threatening the disease is, and what the advantages are of using the technology for its diagnosis and treatment, and during the further course of the disease.

This information is presumed to be available from scientific literature (see below). For the particular health problem (disease) an analysis should be made as to what the technology can contribute, how the technology is used and when in the course of disease the technology is useful.

Here, it is important to examine the particular areas in which the technology is used, both the areas which are scientifically supported, and the areas for which the technology is assumed to have an effect, but where the scientific basis is not yet well documented. It is fairly common that health technology is developed for a particular problem, but that its use is expanded to other areas over time.

Again, the use of ultrasound technology is a good example. However, at a given health technology assessment the indication area is sought to be revealed beforehand, given the decisions the assessment should lead to.

Having described the health technology in question, one then has to examine the alternative technologies available, both established ones and new alternative ways to solve the health problem.

A rational solution to health problem presumes that the effect and the use of the resources in connection with the different ones is examined. This implies that the particular technology must be compared to alternative technologies for assessment of effectiveness. Advantages and disadvantages of the technologies should the be compared.

3.3.2 How is the effect of the use of a health technology then measured?

Firstly, what is effectiveness? Commonly, the term effectiveness describes how effective the treatment (or the use of the technology) is. If the effect of the technology is measured under ideal circumstances, the term "efficacy" is used. When the effect is measured in a more realistic setting or under normal circumstances, the term “effectiveness” is used.

When referring to the effect of a technology in connection with an HTA, one normally discusses the “effectiveness". However, the effect measured in a controlled randomised survey is considered to be ”efficacy”.
When assessing the effect of a health technology, one first seeks to systematise the knowledge which is to be found in the scientific literature (Magnus & Bakketeig 2000).

Empirical research literature includes three types of data: data from original articles, from review articles and from meta-analyses. In an original article, for example, a particular trial, problem, method, results and discussion are described. A review article gathers the results from selected original literature. A meta-analysis takes this a step further – here, data from a selected number of original articles are gathered and a statistical pooled analysis of the results is made.

In many cases there is an exiisting a literature review of the effectiveness of the technology in question which can be used right away. But often one has to make ones own critical literature review based on the empirical medical literature (Guyatt 1994).

Empirical literature in medicine can be divided into four main categories by theme:

- Basic research, the experimental medicine, where one studies molecules, cells or organs. The objective is to understand the mechanism behind diseases and their development and perhaps reactions to intervention.

- Epidemiological research, or cause-and-effect research, which poses the question : why are some people stricken with disease while others stay healthy? Focus is on potential explanatory factors or causes of diseases. This research is population-based. It includes health supervision and the description of the incidence of disease, as well as the effect of intervention in population groups to prevent disease or promote health.

- Clinical research, where the focus is on effects of treatment, but also on diagnosis, patient understanding, care and nursing.

- Health services research, where the health services, their organisation and their activities are described regarding effectiveness and resource-use.

When assessing a health technology it is especially epidemiological or clinical studies which are most commonly applied. However, health services research often also contributes important knowledge.

When assessing the effect of a health technology the gathered literature which meets the requirements of the inclusion criteria is systematised. A traditional scientific article contains four main elements. The first element is introduction, which must contain the background and the problem. The second part contains information on the population (the material), the variables, the design and the methods (including statistical method). The third part contains the results and the last part contains a discussion of the results and a attempt to answer the problem posed. The literature can also be systematised by going through the relevant abstracts . A good abstract contains four statementswhich answer the following questions:

- Why was the study performed? (Introduction)
- What was done? (Material and method)
- What was found? (Results)
- What do the results mean? (Discussion)

Certain characteristics of every study are summed up, preferably in table form. The articles are examined for systematic errors, and those which do not meet the quality criteria are excluded. Finally, a report is made based on the gathered literature review, with a description of the

background, the problem, the inclusion and the exclusion criteria, the discussion of the results, and a conclusion (Magnus & Bakketeig 2000).

When going through and assessing the particular articles, it must be realised, that the reviewer's assessments are subjective and reflect the reviewer's insight and analytical abilities. Thus it should be pointed out that scientific evidence depends on the observer. Guidelines for analysis and review exist, however, but many elements cannot be subject to fixed rules. Often, it is a relatively newly qualified person who performs the literature review. This is not necessarily the best solution as much of the review depends on the knowledge and experience of the reviewer.

In a large systematical literature review it has become standard procedure to use an expert panel of reviewers who first make a primary sorting of the result of the original literature search and thereafter, go through the chosen articles more thoroughly, working in small groups of two (perhaps with a third "judge").

See also the section on literature search in chapter 2.

During the last two decades a professional movement has developed which attaches great importance to so-called evidence based medicine. The activity is named after Archie Cochrane, a British epidemiologist, who in 1979 suggested that systematic up-to-date reviews should be made of all controlled randomised trials with relevance for medical activities. These reviews are located in a database called The Cochrane Library, and can be reached directly via MEDLINE. (The Cochrane Library 2000) (see chapter 2). The Cochrane reviews are exemplary because they document criteria for choice of articles and they explicitly show which articles were excluded because they did not meet the inclusion criteria. Often meta-analysis of the results is performed based on individual articles and these reviews are regularly up-dated with new studies (Sutton et al 1998, Moher et al 1999, Chalmers 1999).

3.3.3 Risk Assessment

Risk can be defined as the probability of an adverse event. The probability of a positive event (for instance being cured of a disease) would therefore be referred to as the chance of being cured and not the risk of it. The word risk origins from the Greek word *rhiza* which is linked to the risk of sailing round a rock.

The historian Bernstein's main thesis in the book "Against the Gods: The remarkable story of risk" is that the development of the modern world from 1600 century rests on our ability to handle risk, which is in turn ascribed to the development of calculation of probability introduced by Pascal and others (Bernstein 1998).
In health technology assessment the risk concept is an important issue. Risk assessment or the perception of risk is fundamental, and is used as the background and the reason for a health technology assessment.

Risk analysis is a method of revealing areas of high risk and suggesting initiatives to reduce the risk of undesired results of a technology (Bakketeig & Magnus 1998).

Risk assessment consists of four elements:

- Identification of the problem (the risk)
- Description of the exposure

- Assessment of the relation between exposure and outcome
- Summary of the risk and its implications

When introducing a new health technology for the treatment of a group of patients, the first step in the risk assessment would for example be whether drug A should be replaced by drug B. The exposure is then described. How many patients need this treatment, for how long and in how large doses?

Thereafter the effect of the new drug is assessed. Which degree of improvement can be expected if one uses drug B compared to the old drug A? What is the difference in survival? Which side effects are related to the two technologies?

Uncertainty is often related to both positive and negative effects of treatment (desired and undesired effects of the use of a health technology).

It could then be of interest to establish different risk perceptions linked to the different estimates of desired and undesired effects of the technology. When assessing the risk perception it must be taken into consideration which short-term and long-term side effects can be expected and how serious these are. Furthermore, one must consider to which degree side effects are acceptable. In less serious diseases the tolerance for undesired side effects of the technology is low. But when dealing with serious diseases one would possibly tolerate some side effects. Often side effects are not recognised until after the technology has been used for a long time, or until the patient has been observed for a period of time.,It is therefore very important that the results of the technology are monitored carefully for a period of time. For this, clinical data bases can be a very important source of information.

3.4 The Need for Performing One's own Study of the Effect of Health Technology

A lot of health challenges are dealt with on a poorly-founded basis withinadequate scientific documentation. When assessing health technology there is, therefore, often a need for conducting own research. However, it shpould be pointed out that before such studies are carried out a thorough search must be made to ascertain whether systematic reviews (of the Cochrane reviews type) have been made. All to often new research is initiated, despite the fact that documentation of the scientific effects can be found in the literature (Chalmers 1999, and Moher et al 1999).
If one plans own studies, there is every reason to make oneself familiar with similar studies, including ongoing ones, performed by others. Especially when dealing with randomised clinical trials a rising number of registers of on-going randomised studies can be found. By consulting such registers spending time on unnecessary studies is avoided, or the information can be used to enhance the quality of ones own study (Chalmers 1999, Moher et al 1999).

3.4.1 Randomised controlled trials

From a scientific point of view, the strongest design when evaluating the effect of health technology is undoubtedly the randomised controlled trial or randomised controlled clinical trial (RCT – randomised controlled trial).

The principle in RCT is that the patient is chosen randomly for two different technologies where the effect of these is to be compared. It could be the comparison of a technology to a placebo treatment,

or between two different technologies (e.g. when a new technology is compared to a technology currently in use).

Dividing the patients randomly ensures that the patient groups are directly comparable and that one controls for both well-known and, not least importantly, for so far unknown risk factors among the patients. Ideally, where such an experiment is controlled – for example – for the differences in the initial patient populations, the effect of the technologies can be estimated by interpreting the differences between treatment groups as differences in the effect of the examined technologies.

Ideally, the trials should be blinded for both the patients and the researchers (double blinded). This is not always possible. It can be impossible to hide from the patients and/or the researchers to which group the patients belong (in other words which technology they are exposed to), with the risk of bias thisentails.

The randomised trial implies that the patients give their informed consent. However, those patients who choose not to participate are often different from the ones who do. Furthermore, the inclusion criteria in these trials are often strict and thereby large proportions of the target population are excluded from the study. All this implies that the effects which are documented in studies are often not representative for the effects which would be seen if the technologies were used on all of the target population.

This represents a substantial dilemma for many randomised trials. On one hand a relatively homogenous study population is desirable, but on the other hand the results should preferably be applicable for a population which has not been selected. This dilemma is difficult to solve. One is often left with the feeling that the result of a study represents an experimental laboratory result, which can not necessary be seen or reproduced in a ”normal” patient population. To this is added the difficulties encountered when applying the results from one area in another.

The randomised controlled trials are intervention studies. One intervenes with a health technology, e.g. a drug treatment. There are also other forms of intervention studies which are not controlled in the same effective way. There are, for instance, the typical before/after studies, where a technology is used for one period of time and a different technology is used for a later period in the same population. A large weakness of this design is that in the time between the two periods changes in both the populations and in the intervention itself may have occurred (factors which can influence the result of the intervention may have changed over time).

Of other interventions one could mention studies where the patients are their own controls from one intervention to the other. Here, a large weakness is the possible interactions between the interventions which is referred to as ”carry over effect” (a late effect of one intervention which then affects a subsequent intervention). There exist various ”cross-over” designs which try to control for this effect.

However, it can be concluded that the randomised controlled trial is superior to the other intervention studies.

3.4.2 Observational epidemiological studies

Cohort studies

In some observational epidemiological studies one follows different exposed groups of the population for a period of time and compares the results in the exposed groups. These are called cohort studies.

		effect	
		+	-
exposure	+	a	b
	-	c	d

where a + b = index cohort and c + d = control cohort

For example, when testing a drug treatment A for a particular disease (exposure +) *a* patients survive 5 years, whereas *b* patients do not survive 5 years. Among the control cohort who are treated with drug b, *c* patients survive 5 years, whereas *d* patients do not survive 5 years. (Effect + in the diagram indicates survival more than 5 years, while effect - indicates survival less than 5 years).

The results of a such cohort study are based on a comparison of $\frac{a}{a+b}$ and $\frac{c}{c+d}$.

If there is no statistical significant difference between the two fractions this indicates that there is no difference in the effect of the two technologies.

However, if $\frac{a}{a+b} > \frac{c}{c+d}$ then drug A is more effective than B, and if $\frac{a}{a+b} < \frac{c}{c+d}$ then drug A is less effective than B.

This interpretation of the result implies that the two populations are homogenous from the beginning, though this is seldom the case. However, methods exist which make these populations more comparable in the analysis (so-called *matching*). A cohort study is called a *prospective* study. Beginning with two comparable populations, these are divided according to exposure and are thereafter monitored for a period of time and one or more outcomes is/are measured.

Such a study can also be performed on historic material and is then called a historic prospective study (Bakketeig & Magnus 1998, Olsen et al 1994).

Case-control study

An alternative observational study design starts with a group of individuals with a specific outcome and compares the exposure of the group with that of a control group without the specific outcome. This is called a case-control study.

		effect	
		+	-
exposure	+	a	b
	-	c	d

a + c = cases and b + d = controllers

Here, the analysis will focus on a comparison of $\frac{a}{a+c}$ and $\frac{b}{b+d}$.

In this example one lets the exposure + mean treatment of the same disease as in the previous example with drug A and exposure - means treatment with drug B, and lets effect + and effect - mean survival after 5 years or non-survival after 5 years, respectively.

If $\frac{a}{a+c}$ and $\frac{b}{b+d}$ are equal (statistically), then this means that there is no difference in the effect of treatment with the two drug A and B. If drug A is more efficient than drug B $\frac{a}{a+c}$ will be larger than $\frac{b}{b+d}$ and vice versa if drug B is more effective than A as regards survival after 5 years.

Observational studies are often based on one's own data collection, but they can also be based on routinely collected register data. Thus, cancer register data is suitable for cohort studies of survival after alternative treatments in various types of cancer. Data from the Medical Birth Register, the National Patient Register and the Cause of Death Register are other examples of valuable data sources for both cohort and case-control studies in Denmark.

Advantages and disadvantages in different experimental and observational studies are demonstrated in Table 3.1.

Table 3.1 Attributes of Different Types of Research Design*

Attributes	Types of design			
	Controlled randomised trial	Cohort Studies	Case-control studies	Before/after Studies
Easy to perform	(-)	-	+	(-)
Affordable	-	-	+	+
Good control of interfering factors	+	(-)	-	-
Results to be generalised	(+)	+	+	-
Good control of exposure	+	+	-	-
Good control of outcome	+	+	+	(+)

*Qualitative studies are not included in the table

All studies mentioned in Table 3.1 are of the quantitative type. Qualitative studies, which also can be of interest when evaluating health technology are not included in the table, as they are not immediately comparable to quantitative studies. Qualitative studies are often based on in-depth interviews. See chapter 4.

The ranking of the studies displayed in Figure 3.1, introduced by Preventive Services Task Force in the US, is the one which has become the most commonly used (Concato 2000). . But a similar ranking is also used by the Cochrane Review Centre.

Figure 3.1 Degrees of evidence after quality of study design

I	Evidence obtained from at least one properly designed randomised controlled trial
II-1	Evidence obtained from well-designed controlled trials without randomisation
II-2	Evidence from well-designed cohort or case-control studies, preferrably from more than one center or research group
II-3	Evidence from multiple time series with or without intervention
III	Opinions of respected authoroties based on clinical experience (deskriptive studies, case reports or statements from expert committees)

The above mentioned studies (randomised and observational) aredependent on reliable measurements of outcome. Measurement of outcome and disease are dealt with in chapter 4.b - Measuring the Health Status.

3.5 Validity

Reliability of measurement of outcome are preferably estimated as precision and validity (Bakketeig & Magnus 1998). One speaks of measurement precision - or whether it is reproducible (or repeatable) - and of measurement validity (validity or accuracy) – is one in fact measuring what one wants to measure? A measuring equipment at the laboratory can provide precise measurements, but the measurements can deviate from the correct value (standard sample). The measurements can then be said to have high precision but low accuracy. On the other hand, the measurements may show some dispersion (low precision), but they can, however, on average be close to the correct value, that is, the accuracy is relatively high.

Within epidemiological research and in large population studies, the term precision is used as it is in a laboratory. Validity is, however, expressed in two different measures: sensitivity and specificity. The sensitivity is a measure for how good a study is at identifying the people suffering from a particular disease. The specificy, on the other hand, is a measure for how good a study is at identifying those not suffering from a particular disease.

		Disease		
		+	-	
Test	+	TP	FP	TP+FP
	-	FN	TN	FN+TN
		TP+FN	FP+TN	

From the above diagram, it can be seen that those who have the disease and where the test is positive, are called true positive (TP). However, for some people the test is negative but they do in fact have the disease, these are called false negative (FN). Among the persons where the test is positive, some may in fact not have the disease, these are called false positive (FP), but most of the people who were found to be negative in the test, do in fact not have the disease, and these are called true negative (TN).

Sensitivity can be expressed as

$$\frac{TP}{TP+FN}$$

And this measure is often expressed in percent (multiplied by100).

Specificity is expressed as

$$\frac{TN}{FP+TN}$$

When, for instance, a screening programme is to be evaluated, one is interested in two other measures which can also be read directly from the above diagram. First, how large a proportion of those tested positive do, in fact, have the disease? On one hand, one talks about the **predicative value of a positive test finding**, and this is expressed as

$$P_{v+} = \frac{TP}{TP+FP}$$

That is to say, the number of true positive as a proportion of all persons with a positive test result (the sum of the true positive and the false positive). On the other hand, one is also interested in a measure for how large a proportion of the test negative, who, in fact, do not have the disease. Or put differently, how large a proportion of the test negative who could, in fact, turn out to have the disease (false negative, FN).

The expression used is **predictive value of a negative test finding**

$$P_{v-} = \frac{TN}{FN+TN}$$

The predictive values are expressed as proportions in percentages.

The validity term is used in two different ways: internal validity and external validity. Validity in connection with a clinical study, as described above, is called internal validity.

External validity means for example how valid a study result is in general circumstances. This could, for instance, concern the use of a particular method in another context than the one existing in a given project. Or it could concern the validity of a result used on a totally different population or in a different location.

Whether or not the results of a study can be transferred or used in general is a critical issue, not least when it comes to the results of a randomised controlled trial. Such trials are often performed under extremely well-controlled conditions and with detailed specifications for inclusion and exclusion and for the study and follow-up procedures. When the results of such trials (RCT) are to be used in an everyday, realistic setting, it will often be difficult to transfer or reproduce the effect indicated previously. As mentioned earlier, a distintion is made between these two situations when the benefit of a technology is assessed. The benefit which can be measured under more ideal conditions, as in a randomised controlled study, is called “efficacy”, while the benefit of a technology used under more ”normal” conditions is named ”effectiveness”. These two measures will, in many cases, show important differences.

3.6 Other Sources of Error

When assessing one’s own and others’ research results (through literature review) it is important also to focus on other sources of bias than just the inaccuracy of the measurements.

It is said that the material of the study is biased when it is not what it is supposed to be. It is important to be attentive to the most important types of bias, as studies often have biases of some type. Biases can be divided into two main groups: selection bias and information bias. Selection bias means that the material of the study is skewed due to an inappropriate selection. In large population surveys randomly selected material (e.g. samples from the Danish CPR-register) is often used. Such random selection can be stratified, that is, different groups of the population are represented with different fractions. The use of such randomly selected sample is recommendable and helps avoiding selection biases. However, skewed study material is often seen in case-control studies, where it is often difficult to find suitable control groups. Another factor which can affect the study material is selective defection or drop-out. This applies both for inclusion and for the implementation of the study itself. Generally, the persons who leave the study prematurely are said to be different from the persons who participate in and complete the study.

Information bias is the phenomenon there there areuncertainties linked to the information the study is based on. This is often seen in case-control studies, where the participants tend to search their memory to find the explanation for their disease, or they may, consciously or subconsciously, under report important events. This is often called recall bias. Such forms of information bias often lead to mis-classification. If the uncertainty linked to this is equally high regarding the presumed causal factor and the effect, this mis-classification is called ”non-differentiated”. Such a mis-classification will lead to a weakening of a possible associations. In a differentiated mis-classification, however, a systematic deviation from the real association will occur due to incorrect information, for example an under reporting of an exposure in a case-control study due to recall bias. Differentiated mis-classification can lead to "false" associations, or incorrect disconfirming associations.

A third form of bias is publication bias, something one must keep in mind during the literature review. Which results are being reported, and which results are being published in the journals? Negative results tend, probably, to be under reported.

Confounding is another term which is often discussed when research results are revealed. Confounding means mixture. Unlike bias, confounding does not express an error in the material, but can be seen as a result of the causal structure in the data material. A confounder variable is characterised by being associated with both the exposures and the effect variables in the study.

It is a confounder if:

- The factor is associated with the disease (the effect)
- The factor is associated with the presumed causal factor (exposure)
- The factor is not part of the chain of causes from exposure to effect.

All three conditions must be fulfilled before one speaks of a confounder. The confounder term plays an important role in analytic epidemiology, and it can lead to that the relation between cause and effect becomes skewed. In analysis there are, however, advanced methods to control for confounders (or co-variables), e.g. multivariate mathematical models (for instance, logistic regression and Cox-regression analysis).

It is recommended to seek the advice of experts when performing some of these analyses.

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Chapter 4: The Patient

by Helle Ploug Hansen, Janine Morgall, Helle Timm, Peter Bo Poulsen and Helga Sigmund

In order to interest other than experts to participate in HTA, there is a need for development of methods based on the visions, desires, experiences of people in general and methods which take into consideration the users' expectations and demands of the health care sector.

Expert opinions, that is to say reports/studies performed by experts based on their own knowledge and understanding of a given technology/treatment, are still dominant in today's Denmark. These reports aim at creating a basis for political or administrative action. But why are the users/patients in question not asked about their opinion, and how can they, in the future, be included more actively in the studies and the creation of the basis for decision-making in relation to HTA?

Even if the desire to include the users is present, resource constraints often limit the choice of method and the extent of the study. However, regardless of the approach, the examining the patient/citizen aspect in HTA will almost always be work intensive. Commitment is essential, and a knowledge of methods and action are demanded, if one wishes to discover, influence or prevent possible consequences which a technology/treatment may lead to. In the following a set of methods is presented, which either alone or in combination could be useful for examining the patient element in HTA, cf. Figure 4.1. The methods are, as regards theoretical basis and application, very different from each other, and include both completely "open", non-controlled, dialogue oriented designs, and completely "closed" designs with a rigid structure.

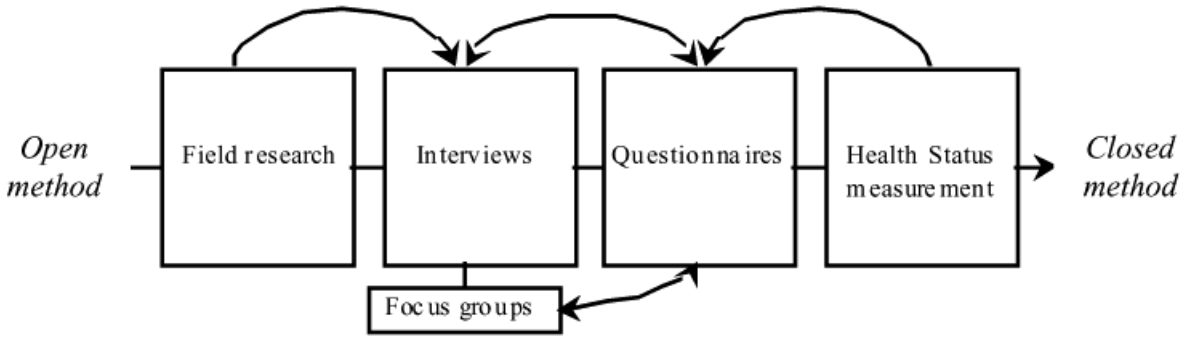

Figure 4.1: Research methods for examining the patient element

4a. Field Research, Interview and Questionnaire Studies

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Contents ofchapter 4a:

4.1 INDIVIDUAL, GROUP AND SOCIETY 38

4.2 ABOUT METHODS FOR EXAMINATION OF THE PATIENT ELEMENT 38

4.3 FIELD RESEARCH 39

4.3.1 PARTICIPANT OBSERVATION 40

4.4 INTERVIEWS 42

4.4.1 UNSTRUCTURED INTERVIEW 42
4.4.2 INTERVIEWS BASED ON A THEME GUIDE 44

4.5 FOCUS GROUP INTERVIEWS 45

4.6 QUESTIONNAIRE STUDIES AND SURVEYS 47

4.7 PROSPECTIVE METHODS 50

4.7.1 THE DELPHI METHOD 50
4.7.2 THE FUTURE WORKSHOP 51

LITERATURE FOR CHAPTER 4A 53

The patient element can be understood as the element which describes the technology in question from the users' perspective. That is to say in relation to the particular person's life history and present life situation, including knowledge, life experience, opinions, skills and actions (Timm 1997). This chapter consists of two parts. The first part deals with the *user* as:

- *An individual human being* with unique experiences, thoughts, feelings and life experience
- *A member of a group/groups who share the same experience*
- *A member of society* and the related institutions, e.g. the health care sector, the family, consumption, education.

There follows a description of a number of methods for studying the patient element. These methods are based on various possible research approaches such as ethnology, psychology, sociology, communication science etc., which can be used in scientific studies of the patient element. Methods for measurement of health status is described separately (Chapter 4.b).

4.1 Individual, Group and Society

It is our assumption that the perspective of the patient/user will often be different from the perspectives of the health professionals (Kleinman 1988, Hansen 1995, Timm 1997, Jensen et al. 1987). The patient's/user's knowledge and experience of disease, suffering and treatment is first and foremost based on their own and/or close relations' actual experience and is linked to everyday life. It is often based on 'common-sense' knowledge and experience. The patient's/user's perspective is different from that of the health professional's. In various ways, the patient perspective is linked to the individual and to the body. The starting point is the disease, suffering, cure or relief of the individual. For the patient/user the body is first and foremost a personal body. One 'exists' in one's own body, one has a body and with this one functions in life. Disease, suffering, cure or relief is, therefore, linked to the extent to which the individual is "put out of the running", and to what extent the disease or the suffering affects the life of the individual.

With regard to the introduction of new technologies in the health care sector, including evaluation of existing examinations, treatment and rehabilitation measures, it could be useful to acquire knowledge about the effects the they have/could have for the patient/user based on:

- *An individual perspective:* "Is it the individual patient's/user's description of how a given technology works in specific situations?"
- *A group perspective:* "Is it their experiences and assessments of the effect a given technology has on everyday life and existence?"
- *A citizen perspective:* "Is it the citizens' assessment of which criteria should be the basis for development or implementation a particular technology, and how different technologies should be prioritised?"

Thus, it is important that one reflects on and decides whether one needs information based on an individual, group and/or society perspective, and if – and to what extent - the life story and present life situation of the patient/user should be included.

4.2 About Methods for Examination of the Patient Element

In the following, various research methods for studying the patient element are presented:

- Field research, including participant observation

- Interviews, including focus group interviews
- Questionnaire surveys
- Prospective methods

It should be kept in mind that this is a presentation with emphasis on the methods which can be used for generating data. When it comes to analysis, synthesis and interpretation of data, reference is made to relevant literature (Kvale 1997, Fog 1994, Spradley 1980, Hansen 1995, Timm 1997).

We have chosen not to use the 'common' division into qualitative and quantitative research methods, as we regard quantity and quality not as opposites, but as part of a hierarchy (Figure 4.2). Quantity is part of quality, whereas quality is not a part of quantity (Wilden 1987).

Figure 4.2 Quality
/Quantity\

Put simply, one can say that every time a researcher wishes to weigh, measure or count something, it is necessary first to decide what must be weighed, measured and counted. The process of deciding the object of study is qualitative. Which research approaches one employs to study the patient element thus depends on the field in which one would like to acquire new knowledge and insight. This decision is based on an individual, group or society perspective.

Common for all methods is that they cannot be understood as value free tools, which the individual researcher uses at will. *Methods always imply theoretical considerations.* If the research methods are regarded as mere tools, it is the methods that control the researcher, and thereby the research process, and not the researcher who controls the methods. A strong and rigid commitment to method primarily serves to reduce the apprehension of the researcher (Devereaux 1967:97). Thus, choice of methods always includes reflections on the relation between theory, method and data.

General requirements to the researcher:
- Stay focussed, have analytical and creative abilities
- Have knowledge of the methods in question and their theoretical basis, and insight and skills in their analysis and synthesis.
- Be critical towards own prejudices and preliminary assumptions (Hansen 1996)
- Gain the confidence of the interviewed persons
- Have insatiable curiosity

4.3 Field Research

The term field research should be understood as a framework for research methods, the most important being participant observation and interview (the latter is not described under field research, but in a special section on interview methods). Field research is the individual researchers encounter with 'the other' (patients, health professionals etc.). Field research is a central element in anthropology, which is *the science of mankind.* Here, the researcher has traditionally done field research in foreign 'exotic' cultures, but from the beginning of the 1980s the anthropologists' interest in exploring their own culture grew. Danish anthropologists have studied the health care sector with focus on patients/users and health professionals (Hansen 1995, Jensen et al. 1987).

4.3.1 Participant observation

Participant observation has, during the past century, been given differing significance. Early versions emphasised *observing participation*. The researcher participated in the life of the society beingstudied, in order to observe it as closely as possible. The researcher was considered a neutral person, for whom it was necessary to be 'in situation' to be able to observe. The observed persons were regarded as objects, from which it was possible to collect objective and true statements. The problem with this approach was the assumption that the observed persons existed in an external and objective world. Today, the emphasis is put on *participating observation*, which recognises that the researcher is also participating when he/she is observing. If, for example, the researcher is observing an initiation ritual or a conversation between a doctor and a patient, the acts (including acts of speech), which take place between the parties, cannot be seen from an objective and neutral position. Consciously or subconsciously, the parties will see the researcher as an audience who, in various ways, has and will have influence on the messages which are exchanged and thereby on the data which is generated. The researcher thus participates in and observes the life which is lived in the research field, and through participation he/she also observes his/her own participation.

The researcher is not a data collector, because data does not exist before the researcher generates it. It is only at the moment the researcher realises: *"This is something significant"* that statements, gestures and behaviour - e.g. in a conversation between a doctor and the patient - becomes data. Data does not *exist* in the field beforehand, waiting for the researcher to come and collect it. Data is structured within a scientific discourse. It is not until something is recognised and defined (as data), that it becomes data. It is through theoretical reflection that the researcher decides what should have the status of data, and what should not. Thus, the analytical process begins at the instant something is discovered and defined, and receives the label "data", and does not begin behind the desk after the field research is over-.

Field research is time-consuming. Often participant observations will be fairly unfocussed in the beginning. Even though the researcher has done thorough theoretical preliminary work, reality has qualities which cannot be determined beforehand. The researcher seldom knows what makes sense to the people who are studied, including which questions would be relevant to ask. Therefore, the researcher will, in the beginning of the field research, often be reluctant to conduct an interview. *If one does not know what makes sense for the people one studies, one cannot know which questions are central to ask.* Gradually, the participant observation becomes more and more focussed, and the interviews become more specific, while at the same time the unfocussed view is maintained.

Field research in an HTA context. Field research is useful in a HTA context when one is interested in a thorough analysis of the technology in question, seen from the patients/users perspective. Field research with participant observation and interview can be used in relation to studies of individual, group and society, but as this is a time-consuming research approach it is important that the researcher/researchers carefully consider whether this is the right approach, and not least whether they have the requisite scientific expertise to carry it out.
One of the advantages of using field research is that it offers an opportunity to gain insight into how the patients/users *verbally* express their thoughts, knowledge of technology, experience etc. and *non-verbally*, that is to say, how contemplations, experience etc. influence their everyday life. Thoroughly planned and performed field research gives the opportunity to spot new and unknown sides of the patient's/user's contemplations, experiences etc. The output of field research, however, is closely related to the skills of the researcher in analysing and interpreting the material cogently and transparently. Data generated through participant observation can be more difficult to analyse than data generated through interviews, solely for the reason that participant observation is not recorded on tape in the same way as an interview (even though one may use video recording). This

makes demands on the researcher that he/she is able to approach the material in a very reflexive way, to realise which written observations are trustworthy, or if they, consciously and/or sub-consciously, are the product of the researcher's own presuppositions.

Practical application. Field research is resource-consuming. It takes time to plan, to generate data, and to process, analyse and interpret it. Field research and analysis (depending, of course, on the complexity of the problem) can easily take up to two years. There is, however, often no need for expensive statistics programmes or assistance of a statistician, mailing of questionnaires etc. Apart from their own salary, most field researchers require a dictaphone and a transcriber, a secretary during certain periods of the project and transport expenses.

Example of field research. It has not been possible to find examples of field research done specifically in relation to the patient element in HTA in the literature. However, field research among patients in another context can be found, for example 'The Danish Cancer Society', - the publication "What is the Meaning of Cancer" (in Danish) (Jensen 1987).

Summary – field research

Is applied when the goal is:
- To make thorough descriptions and analysis of a given technology from a user perspective
- To study the experience, thoughts, attitudes, behaviour and actions of the patients/users
- To study the perception of the patients/users regarding health and disease in relation to a specific technology
- To study the patient's/user's wishes for information, participation etc.
- To study both what the patients/users say and what they do

Specific demands to the researcher:
- Have a thorough theoretical and methodological knowledge, insight and skills
- Have the personal qualities needed for field research, since the researcher will often risk becoming involved in the lives of the patients/users
- Be critical and reflective towards own prejudices and presuppositions

Advantages of field research:
- Close to the everyday life of the patients/users
- Based on both verbal and non-verbal statements
- Follows the patients/users for a period of time
- The interview is based on what will make sense for the participants in the study
- Can study the individual, group and society perspective, respectively
- The researcher experiences the life that is lived in the field
- Provides the opportunity to discover unknown sides of the contemplations, thoughts, experiences and actions of the patients/users
- Is often inexpensive

Difficulties of field research:
- It is time-consuming
- Often produces large quantities of unsystematic data (field notes, unstructured interviews), that must be analysed and systematised

4.4 Interviews

As a research method, interviews cover a spectrum of enquiry from closed and rigidly structured questionnaires to the open and unstructured (open) conversation. Common for these various forms of interviews is the desire to explore the interviewee's knowledge, experience, perception, opinion etc. of a specific topic, theme, life circumstance etc. It is important to keep in mind that, regardless of which method of questioning is chosen, the *answer to the question is not necessarily the same as information*. Many people who agree to participate in a study will also willingly answer questions that do not make sense to them. Regardless of what kind of interview one chooses, it is necessary to reflect on one's own presuppositions, and to consider one's own skills as an interviewer (Hansen 1996).
In the following section the methods *unstructured interviews, interviews according to theme guide* and *focus group interviews* are presented. "Interviews based on a questionnaire" is described later under the heading *questionnaire surveys*.

4.4.1 Unstructured interview

Open and unstructured interviews or informal conversations are often used in an early phase of the field research or interview survey. An unstructured interview is useful when one does not know

what is relevant to ask about e.g., which choice of wording patients/users use about a subject or in which correlation they imagine a subject. It is also a way of ensuring that the interviewee has a say in what the interview should include. Often the unstructured interview is then replaced by interview based on a theme guide. Unstructured interviews are also useful during the entire field research combined with participant observation and interview based on a theme guide, because this always provides the possibility of including new themes which turn out to be important.

Unstructured interviews are also used in connection with:
- Narratives on the course of disease and treatment
- Life histories

Both forms of interview are informal conversations taking place during a period of time (weeks, months, years) with selected interview persons, who tell about their life or of certain periods in their life (Horsdal 1999).

It has not been possible to find an example of an unstructured interview in relation to the patient element in HTA in the literature, see also under ”Field research”.

Summary – unstructured interviews

Relevant when one wishes:
- To examine the perception of life of a patient/user, with emphasis on subjective experiences and life story experiences

Important:
- That data is generated during the examination process which begins by identifying the problem, the question, thought or the hypothesis
- That study, analysis and interpretation are related to the theoretical framework

Advantages:
- The intervieee’s perspective decides the direction of the interview
- The interview is open, which easily allows new themes to be introduced
- The interviewed person is given the opportunity to tell their story without interruption

Difficulties:
- It can be very difficult to process the information because of the large quantities of unstructured data

4.4.2 Interviews based on a theme guide

An interview based on a theme guide can be regarded as an open interview. It is situated somewhere between the questionnaire interview (closed and with a strict structure) and the unstructured conversation. Often an interview based on a theme guide is described as a *semi-structured interview, ethnographical interview*[1] *or a qualitative interview*[2]. An interview based on a theme guide is a conversation between the researcher doing the interview and the person being interviewed. In advance, the researcher has thought through and described a number of themes and formulated some questions which the interview should address: that is to say the *interview has a purpose, and some form of guidance*. At the same time, the interview is characterised by being open to new themes which both parties, due to the dynamics of the conversation, may uncover. The interview is thus based on *confidence*, and it is important that the interviewer is responsive and does not force specific answers on the interviewed person.

HTA-example of interviews based on a theme guide. An interview based on a theme guide is well suited for HTA use, when the goal is to uncover the patient/users perspective, that is to say their experiences, views and subjective understanding. An example of this can be seen in the HTA project ”Between Hope and Despair - About ALS-patients and the drug riluzole” (in Danish) (Jensen et al. 1997). ALS (Amyotrophic Lateral Sclerosis) is a severe, chronic disease which leads to death within a few years. An interview study of 12 ALS-patients and 6 health professionals was carried out. The purpose was to obtain a nuanced understanding of the decision-making process of the patients, their views and thoughts in regard to their choice of a new drug, which did not cure but could prolong life by about 2 to 3 months. The patient interviews were based on a theme guide containing the three following main themes, which the patients were familiarised with at the beginning of the interview:

- Perception and experience of the disease
- Personal experiences with riluzole
- Level of information in regard to the disease and the treatment

The interviews/conversations were recorded on tape and later transcribed verbatim. During the processing of the material the researcher aimed at studying the differences and similarities across the material, partly to establish essential subject fields and partly to uncover common experiences and opinions (Jensen et al. 1997:37-38).

[1] The American anthropologist James Spradley has written a very thorough book on ethnographical interviews, including how the interview should be structured and ways to process and analyse the interviews (Spradley 1979).
[2] In particular Steinar Kvale and Jette Fog aim at describing ’the qualitative research interview’ as a scientific method (Kvale 1997, Fog 1994).

Summary – interviews based on a theme guide

When to use interviews based on a theme guide:
- When one has specific themes one would like to study
- When at the same time one wishes to be open to other potential themes

Advantages:
- The interview is open and new themes can be introduced by both parties
- It may take the form of an intimate conversation
- It can be used to explore the knowledge and experiences of the patients/users

Difficulties:
- The interviewer must not loose sight of the aim
- The border between interview and therapy can be subtle

4.5 Focus Group Interviews

Focus group interviews date back to the 1930s, when social researchers in the United States recognised the limitations of structured survey studies. The goal was to develop a method which would allow the respondents to structure their own thoughts and statements - in other words to gather ”more authentic” data, which was not guided by the researcher. Focus group interviews have been used with increasing frequency in social research - for example in programme evaluation, policy analysis, organisation development and applied social research in general. Today, one can find several examples of the use of focus group interviews in public health research.

A focus group interview can be defined as a semi-structured group interview, usually with 6 to10 participants, who concentrate on a specific subject. A characteristic of the method is that it has a group dynamic element, which makes it possible to collect information/perspectives on a detailed level from several persons at the same time. Apart from this, the method has the following advantages:

- Formation of groups develops a more natural ”discussion” between a group of people instead of a more formal interview situation
- The interaction in the group stimulates subtle statements
- It is possible to get relatively under-privileged persons to express themselves in a safe environment surrounded by similar persons (as compared to an interview conducted by experts/professional researchers/authorities, who ask questions about a particular subject)
- The mutual inspiration generated in the group is more likely to promote rather than inhibit exposure of interesting information
- The process can uncover complex motivation, opinion and response factors

Compared to single-person-interviews it can be a weakness that, when working with focus groups, one must limit oneself to only a few questions (themes) due to the number of participants. Not all subjects are well-suited for focus group interviews, for example intimate topics.

The method demands an experienced leader (moderator), who can guide the group, can prevent sub-groups from forming, or an opinion leader from dominating the interview. One uncertainty in the method is that one never knows exactly which direction the discussion will take. In reality the focus group interview may end up as a combination of an experiment and an interview.

Selection of participants and composition of the group. Participants should be selected based on their ability to contribute to the present problem (topic to be assessed). Practical as well as resource factors must be taken into consideration. The goal of the selection process must be to obtain a balance between differences and similarities in each group as regards gender, age and other characteristics such as diagnosis etc.

Identifying themes. As when constructing other forms of interviews and theme guides the themes/problems should contain the relevant aspects of the technology/treatment which one wishes to study. The goal of a focus group interview is to elicit the opinion of the participants regarding the subject, in their own words. Structure and order are necessary even if this demands open questions. There are two general rules regarding the order of the questions: 1) Begin with general questions and proceed to more specific ones. 2) Begin with those questions most central for the problem, and proceed to less central ones. In this way one avoids the possibility that the discussion will become too narrow too soon.

The role of the interviewer (moderator). The role of the interviewer is to be a sort of chairman and catalyst for the group process. The moderator encourages the participants to put forward their opinions and stimulates interaction in the group. Most important of all it is important to keep the discussion going, to keep the discussion on course, and to make sure everyone has their say, and that no one person dominates the discussion.

Data administration/analysis. This involves two procedures, one is data administration, where one physically organises one's data, and the other is the interpretation of data, which is an on-going process that includes reflection and adjustment of the problem posed. There is a difference between the participants stating "facts" and statements which represent a perspective (such as that of a patient), and it is the task of the researcher to make a thorough assessment.

Focus group interviews. Specific examples of use in HTA are not available, but focus groups have been used for evaluation studies. In a Canadian study on a cholesterol lowering drug, several focus group interviews were conducted whereby 63 patients participated. The objective was to study which factors have influence on whether or not the patient stays in treatment (Thompson 1997). Another example is a Danish/Icelandic study, which examines the effect of a new law concerning pharmaceutical distribution. Here, focus group interviews were used to obtain the user perspective and their experiences with the new legislation (Almarsdóttir et al. 2000). The study included seven focus group interviews - three of them were held in or near Reykjavik, and the four remaining were held in various rural districts.

Summary – focus group interviews

Basic uses for focus group interviews
- When one has an overall theme, but wish to identify (potential) problems
- When the aim is to collect knowledge, experience, statements and assessments

Important:
- The topic must be relevant to the participants
- The participants must feel comfortable with each other

Advantages of focus groups:
- Mutual inspiration, group dynamic effect

Difficulties:
- A built-in element of uncertainty in that the discussion can take an unintended course
- Confrontations can arise in the group

4.6 Questionnaire Studies and Surveys

Questionnaire or survey methods are tools which provide data by use of questionnaires. The questions are posed to a group of persons either verbally or by using *standard interview forms* (often as telephone interviews) or in written form as *postal questionnaires*, where the persons read the questionnaire and give a written answer. Studies of a limited extent are often referred to as ”questionnaire studies”, while the term ”survey” is used for studies which includes a large number of participants. However, the terms are often used interchangeably.

Data from questionnaires is not information which already exists and thus can be collected, but is information which requires processing before it can be analysed - as opposed to studies based on information from public or other registers (and not verbal or written answers to questionnaires), which are not survey studies, but register studies (Olsen 1998).

Studies conducted as surveys or questionnaires are most often carried out by research institutions, public institutions and private opinion poll institutes. In the health care sector the use of questionnaires for learning about the patients’ view of health services is a widespread (and much debated) method, best known in connection with studies of patient satisfaction as part of quality improvement.

When are questionnaire and survey studies used? These methods are a form of measurement tool, which are suited to mapping measurable phenomena in a *representative* way. This is achieved by putting the answers of the respondents together in order to make quantitative statements about the distributionof the phenomena. For example, it could be knowledge and abilities, actual behaviour or characteristics of the respondent which are measured. It could also be questions which aim at uncovering opinions, assessments, prejudices, preferences, satisfaction etc., as well as possible motives for actions or attitudes.
Some of the criticism of using quantitative questionnaires is that complex problems and diversified opinions are reduced to a mere x in a box, in a questionnaire with a limited number of choices. It is therefore important to realise that questionnaires and survey methods *are not* suited for uncovering more complex and diverse phenomena. Here, one needs open and flexible interview methods and a *general understanding* of the phenomena instead of representivity.

In practice one often combines methods. In fairly small studies one questionnaire could for example. comprise both a standardised part which is processed quantitatively, and a number of open questions for amplification, which require separate processing. Other typical combinations are, 1) to perform some relatively unstructured interviews/focus group interviews with a number of key persons prior to a questionnaire study, in order to refine the design of the questionnaire or 2) that a questionnaire study is followed by a separate, in depth interview study in chosen areas.

What is important when designing the questionnaire? The requirements for designing a questionnaire are not very different from those of requirements for other types of measurement tools:

- The questionnaire should measure the subject it attempts to measure (validity) and achieve this as accurately as possible (reliability)
- The measurement must be reproducible (that is to say, the person will give the same answer if he/she is asked the question repeatedly)
- The measurement should preferably be comparable with other studies.

Validity and/or reliability problems are often expressed as inaccuracy in measurement and can be prevented in the design of the questionnaires by thorough preparation and pilot testing of the individual questions and questionnaires.

It is imperative that the questions are comprehensible and unambiguous in order for the questionnaire to provide reliable data. This is extremely important in the case of postal questionnaires, where the respondent (the citizen/user) is entirely on his/her own. In general, it is advisable to have relatively short questions, and to use short instead of long words . The meaning of each word should be evaluated, as well as the relation between the words, in order to avoid unnecessary words and terms. At the same time, the questions must be worded so that they are clear to the person who should answer them. One way to get started with designing the questions, is to think of which sort of answers one wants.

As to the categories of answers, the main rule is that these should fit the questions in a logical way, be exhaustive, exclusive and seem natural for the person being asked. When using opinion questions, one must consider that the respondent may have *an inconclusive* opinion or perhaps have *no* opinion regarding the subject of the question. This could mean that the question has no relevance for the individual respondent. It is therefore important to include answer categories such as ”I do not know” or ”I have not thought about this”. When one includes these categories, information is not lost. On the contrary, a clear distinction between what is opinion and what is ‘no opinion’, helps increase the quality and limit the errors.

The problems of selection, drop out rate and interpretation. Decisions about the target group and random sample design (but also about the data collection method and the analysis strategy) should be made early in the process. It is the theme/problem which is decisive for who and how many should participate in the study. Selection of a group of participants who represent the entire study field is usually based on specific statistical methods, which in principle are the same as the ones used in clinical controlled studies. That the selection of the participants is representative has an important impact on the quality on the results. Therefore, it is of paramount importance to ensure that the participants, in fact, represent the area they are supposed to.
The question of representation is not only relevant in the selection phase, but must also be controlled later with regard to that part of the original participant group who actually answered the questionnaire. This is done by *drop-out analysis,* which aims at studying why some persons do not want to participate in a study. Motivation of the respondents (for example through a thorough introduction to the study) can limit the drop out rate, thereby enhancing the response rate and the

quality of the study. Another factor which influences the response rate is how relevant the subject of the study is to the individual respondent.

As far as checking for possible errors, one must (besides the problem already mentioned) be aware of cases where the respondents do not wish to reveal inappropriate social behaviour (e.g. smoking or excessive use of alcohol). Experience has shown that circumstances viewed as negative can lead to under reporting whereas socially accepted behaviourmay lead to over reporting.

For processing the survey data a number of analysis programmes exist, of which one of the best-known is SPSS (Software Package for Social Science).

HTA-example of surveys/questionnaire studies. Questionnaire or survey studies can be relevant in connection with revealing the opinions and perceptions of patients or citizens. An example of this is the HTA-project ”Influenza Vaccination of the Elderly” (in Danish) (Sigmund 2000), which consisted of an evaluation of four different organisational models for influenza inoculation. The project included comparative studies (standardised telephone interviews) in the municipality of Copenhagen, and in the rest of Denmark. Such a comparison requires use of standardised methodology. Part of the study focussed on the opinions and behaviour of the elderly over a period of time, as the same target group was asked the same questions in three successive years. Another part of the study compared different regions - that is to say various groups were asked the same questions at the same time. The studies included 700 and 1,000 persons respectively.

Summary – questionnaire and survey studies

When are questionnaires used:
- When one is clear about what one would like to have the answer to
- For factual questions
- To study opinions

Important:
- The issue is to map phenomena in a representative way

Advantages:
- One can collect answers from small as well as very large groups

Difficulties:
- Uncontrolled drop out rate, e.g. when selected persons do not participate or respondents do not answer, and the reason for this is unknown

4.7 Prospective Methods

Prospective (proactive) studies aim at predicting the users' attitudes and preferences to a new (not yet available) medical treatment/technology or trend. Prospective studies cannot be expected to give a thorough understanding of the participants' present way of thinking or acting. The aim is, through proactive methods, to create a situation which promotes or renders it possible to make predictions, based on collected opinions and preferences. The time aspect is important. Prospective methods are best used in a very early stage of technology development.

4.7.1 The Delphi method

The Delphi method dates back to the 1950s and was developed by the RAND Corporation for use by the American military, as a forecasting tool to predict the effects of warfare for the U.S. Since then, the method has been used in many variations and connections. While the method was originally used to collect and integrate expert opinions towards a final decision on a future situation, it is currently used to collect the views and perspectives of users.

The Delphi method is often used to predict and gather thoughts and opinions about new trends, in particular within areas where empirical data are unavailable or uncertain. The method is also used to generate "forecasts" and plans in connection with the design of new programmes and policies. The Delphi method includes a summation of present knowledge and suggestions for alternatives. For health care issues the Delphi method is used to obtain estimates where existing empirical data is insufficient or where the occurrence of disease or side effects of a technology is unknown.

How does the Delphi method work? It is characteristic for the method that agroup (researchers or the like) collaborates with a decentralised panel of chosen individuals (e.g. users) to iteratively formulate their knowledge on a particular subject. The participants must have experience in an area which is defined beforehand, and the persons must be able to assess development tendencies and directions, and have the ability to extrapolate into the future. They are part of a process where they must be inspired to think through a problem and provide relevant, carefully prepared solutions and new ideas and visions.

It is also important that the research team, as far as possible, remains neutral during the debate, and lets the opinions and statements of the panel guide the outcome of the process. The result is thus the expression of the pooled responses of panel participants about a particular subject.

The method is a structured, interactive survey process, which is based on controlled feedback-rounds among the participants, who are anonymous to each other. The Delphi method is a multi-stage technique, where each new stage builds on the previous stage. Using a questionnaire, followed by feedback-rounds, the aim is to reach convergence of opinion. The method consists of the following three steps: 1) Collection of anonymous written responses of opinions/attitudes from respondents (that is to say the first round of questionnaires which introduces the subject). 2) Several rounds of systematic modification/criticism of the collected, anonymous feedback 3) Through "aggregation" one reaches the answer/response of the group which represents the opinion of all the individuals in the group. The reliability of this method rises with the number of participants.

Strengths and weaknesses. The Delphi method is a relatively quick and inexpensive way to collect data, and is suited for use through Internet and e-mail. As the participants have no direct contact with each other, the method allows persons who find it difficult to voice their opinion verbally in a

group to present their views and opinions. It allows the inclusion of people who live in remote areas. The participants can respond at any hour they prefer, and use as much time as they need.

A limitation is that not everyone has access to the Internet and e-mail. Furthermore, the implicit expectation that the process under all circumstances must end up as some sort of consensus may limit the commitment of the individual. The method does not allow for debate of ideas in a direct discussion, as is the case in face-to-face interaction. Moreover, the possibility of obtaining insight into conflicts or the views of minorities in connection with processing and ranking of the answers is limited. The consensus achieved may be criticised for being forced or artificial.

Example of the Delphi method. At the Department of Social Pharmacy at the Danish School of Pharmacy studies have been done using the Delphi method as a tool for expert as well as citizen participation in connection with decisions regarding new drugs and new trends in treatment (Møldrup et al. 2000, Møldrup et al. 2001). The Internet and WWW were used for data collection in these studies.

Summary – the Delphi method

When is the Delphi method used?
- When one wishes to contribute to the prediction of technological development
- For mapping ”trends” (but does not reflect ”objective” views)

Important:
- Identifies common features of people from different areas based on the respondents’ own perception of future development and political possibilities

Advantages:
- Persons with different opinions, who would not normally meet each other, are confronted with the various views in an interactive process (though without face to face contact)

Difficulties:
- Conflicts are difficult to uncover and the consensus achieved may be artificial

4.7.2 The future workshop

The future workshop is a forum where persons who are affected by particular conditions or problems meet in order to find solutions.The participants work together to formulate and present their own predictions of what the future of the field could look like, set up requirements and test arguments. The objective of the future workshop is to include interested citizens in the decision making process.
The method was developed towards the end of the 1970s by two German sociologists in order to strengthen locally based democracy. The method is based on the idea that people generally are capable of acting in a visionary and energetic way. The objective is, through new thinking, to reach constructive solutions to problems. Points of criticism of the future workshop are similar to those which are sometimes raised regarding other methods using open statements. To this one might add that the future workshop is developed to deal with themes/solutions before there is any possibility of establishing a scientific basis for this.

The participants are – according to the original concept – ordinary citizens and laymen (local population, user groups or other interest groups). However, in Denmark the method is often used for problem-solving or planning among health professionals in the health care sector. As regards HTA, the future workshop is useful, in particular when making progressive (proactive) assessments which focus on discovering the needs of the citizen/patient.

The method consists of a group process in three phases with approximately 20 participants. The process will last 1-3 days and will be controlled by two facilitators. The facilitators' "control of the process" must, however, be next to invisible and is mainly to ensure that the process complies strictly with a few rules. The content of the workshop is created by the participants themselves through energetic participation (in relation to the theme in question). During the process all statements are written down. These statements act as tools during the process and as documentation in the subsequent processing and follow-up

The future workshop is divided into distinct phases:

- First is the *phase of criticism*. Here, the participants must consistently think of the subject in a *negative* way and 1) criticise the subject from different angles, 2) prioritise core problems, 3) analyse cause and effects.
- This is followed by a *fantasy phase* where the participants must consistently think of the subject in a *positive* way 1) "forgetting" the limitations of everyday life, 2) embellishing, but not going against the ideas of others and 3) finding imaginative solutions for the problems in question.
- In the last phase, the *strategy phase*, the participants must consistently think in a *realistic* way and 1) analyse barriers to the imaginative solutions, 2) point out realistic elements in the solutions, 3) outline plans to overcome the barriers and implement the solutions.

The preparatory work done prior to the workshop may have an important impact on the results which are obtained. The core of this is the wording of the theme (in interaction with initiator and facilatator), which covers all of the problems associated with the theme of the workshop. At the same time, the theme must be feasible to work with.

In short, the characteristics of the future workshop are:

- Identification of problems/needs
- Production of plan outlines and solution strategies
- Emphasis on the process/commitment/acceptance
- Active inclusion of users/citizens in the decision and planning processes.

HTA example of a future workshop. As part of the proactive technology assessment "Communication in the health care sector" (in Danish) (Lund 1986) a number of future workshops were conducted in order to discover the needs of administrators, health professionals and not least users prior to a decision to implement new information technology in domestic care. The workshops had a promoting effect and as result demands for reorganisation of the community health care work and alteration in communication (not necessarily though IT solutions) were discovered.

Summary – future workshop

When are future workshops used?

- When the goal is to have creative suggestions for future solutions
- When a detailed examination of a diffuse subject is required within a short period of time

Important:

- The content must be formulated through active participation of the parties involved

Advantages:
- Inspiring and activating process, democratic approach
- Few rules, simple structure

Difficulties:
- When the participants have hidden agendas

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4b. Measurement of Health Status

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Contents for chapter 4b:

4.8 TYPES OF INSTRUMENTS 57

4.9 CRITERIA FOR SELECTION OF INSTRUMENT FOR MEASUREMENT OF HEALTH STATUS 58

4.10 GENERIC INSTRUMENTS FOR MEASUREMENT OF HEALTH STATUS – SOME EXAMPLES 59

4.10.1 SF-36 61
4.10.2 SICKNESS IMPACT PROFILE 61
4.10.3 NOTTINGHAM HEALTH PROFILE 62
4.10.4 COOP/WONCA 63
4.10.5 PSYCHOLOGICAL GENERAL WELL-BEING 63
4.10.6 EUROQOL-5D 64
4.10.7 15D 64

LITERATURE FOR CHAPTER 4B 65

In clinical studies the efficiency of the studied health technologies is usually measured through changes in mortality and/or morbidity, as the primary aim of the treatment. This could for example. be as survival rates, risk reductions, and elimination or reduction of symptoms and disease. But the disease and treatment of a patient may also influence the health-related quality of life for the individual, as well as for family, friends, employers and others who are affected, and this is not necessarily reflected in measures of mortality and morbidity (McColl et al. 1996, Goodman 1998). This is why the primary objectives of clinical studies are increasingly complemented by secondary objectives, which focus on changes in the health status of patients due to a particular treatment. In HTA focus on the patient's health status other than mortality and morbidity is extremely relevant seen in the light of the broadness of the HTA analysis compared to that of a clinical study. Unlike the quantitative data of the primary mortality or morbidity measurements, the assessment of the health status of the patients include a subjective experience in relation to health and the consequences of disease, simply because it is the patients themselves who are asked about this (Fitzpatrick et al. 1998). Health status, and health-related quality of life, affects the health and well-being of a patient, where health includes both clinical, functional and psychosocial dimensions - dimensions which a health status instrument is designed to include. A health status measurement in an HTA therefore measures the health and well-being of the patients.

4.8 Types of Instruments

Condition and disease specific instruments are developed to uncover the subjective opinion of the patients having a specific disease or health problem (Fitzpatrick et al. 1998). The advantage of these instruments is, of course, their particular focus on, and therefore high sensitivity towards, a specific disease and changes in the disease when treated. On the other hand, it is a disadvantage that they can only be used on the specific disease, and not on patients who do not have that disease. A number of disease specific instruments have been developed (see examples in Brooks 1995, Hutchinson et al. 1998).

Generic (or general) *instruments* are, on the other hand, more universal and able to uncover a number of aspects of health status apart from the disease-specific one, which make them relevant for a more widespread part of the population and for more health problems, and enables comparison across these aspects. A disadvantage of generic instruments could, however, be that these instruments are not sufficiently sensitive in relation to a specific disease, which is why it is advisable to include both generic and disease specific instruments in a study (Fitzpatrick et al. 1998)-where this can be done without over-burdening the patient, of course. Generic instruments include a number of dimensions, e.g. functioning, symptoms, psychological well-being, role activity etc. (see Table 4.1), and are thus multi-dimensional. Additionally, each dimension will be divided into items, with more specific questions regarding the item in question. Sickness Impact Profile (SIP), for example, includes a total of 136 items. The generic instruments can furthermore be divided into profiles and aggregated indexes (McColl et al. 1996). The difference is that a generic profile measure only measures the score for each separate dimension, e.g. SF-36, whereas an index measure is able to aggregate the dimensions of the instrument to a joint index score by giving the dimensions in the instrument individual weights. Some generic instruments also have a sort of numeric weighting or valuation of health status that enables these to be further characterised as utility measures, and thereby be applied directly in connection with an economic evaluation, e.g. EuroQol-5D. But how must one then select the appropriate health status instrument for a study?

4.9 Criteria for Selection of Instrument for Measurement of Health Status

The generic instrument for measurement of health status selected for the HTA analysis must first and foremost be relevant. But apart from this, there are also a number of criteria, both psychometric and more practical ones, which the chosen instrument should fulfil.

The first criterion for the choice of generic instrument is whether the tools of the instrument are *suited* for the objective, the perspective and the target group of the HTA analysis. Is the instrument designed for and/or has it been used before for purposes similar to the one it is to be used for in the study in question? (McColl et al.1996). Has it been used for the disease and the patient group before? The second criterion is the question of *reliability*. How reproducible and internally consistent are the results which a given instrument produces? (McColl et al. 1996, Fitzpatrick et al. 1998). The reliability of an instrument is important in pre- and post- test designs, where the measurement is repeated after some time, after the population has been exposed to an intervention. The difference in the pre- and post- test situation should be exclusively attributable to the intervention and not to the instrument applied. A measure of the reproducibility of an instrument is test-retest reliability, which is recommended should be higher than 0,7 (Fitzpatrick et al. 1998). A third important criterion is whether the instrument is, in fact, measuring what it claims to be measuring – *the validity* (McColl et al. 1996, Fitzpatrick et al. 1998). The validity can be measured in a number of ways and will, of course, in each case depend on the objectives and the area studied. Some of the types of validity one should be aware of is criterion validity, face and content validity. A fourth criterion concerns the *sensitivity* of the instrument - that is to say, how sensitive is it for measuring changes in health status of importance to the patient during a period of time (Fitzpatrick et al. 1998). To be useful an instrument must be sensitive to changes appearing over a period of time due to an intervention (Bentzen et al. 1998). A fifth criterion relates to the *precision* in relation to its scoring (Fitzpatrick et al. 1998). The precision is affected by the format of the answer categories and their numeric values, the scaling etc. The sixth criterion – *interpretation* – concerns the possibilities of interpretation of the scores of the instrument, and whether this makes sense (Fitzpatrick et al. 1998). This must be assessed compared to the context of the study. As a seventh criterion, the *acceptance* of an instrument must be examined, as this is essential for the success of the measurement (McColl et al. 1996, Bentzen et al. 1998, Fitzpatrick et al. 1998). The acceptability of an instrument can, for instance, be uncovered by studying the percentage of questions answered in earlier studies, where the same instrument was used. The burden for the patient is affected by factors such as the time it takes to fill in the questionnaire, how easy it is to manage, size and language. The last criterion which must be considered when choosing an instrument is the *feasibility*, for example, what are the costs and how easy is it to manage and to use (Fitzpatrick et al. 1998).

One must thus choose a generic instrument which fulfils these eight basic criteria, perhaps with aid from an expert in the field. Whether disease specific instruments should be added must also be considered. For further information see McColl et al. 1996 and Fitzpatrick et al. 1998.

4.10 Generic Instruments for Measurement of Health Status – Some Examples

This section includes a short description of seven generic instruments which are widely disseminated and used for the measurement of health status in connection with clinical studies, economic analyses and HTA. There are validated Danish versions of all these instruments available, and for some of these permission for, or notification of, use is mandatory. The objective of this section is not to give detailed knowledge, but on the contrary to give a general introduction to the instruments. The reader who is interested in further information about health status measurement may consult the references in the end of this chapter for general literature on the subject, and more specific literature on the instruments.

Table 4.1 below shows the extent and the dimensions in the seven generic instruments.

Table 4.1: Generic Health Status Instruments – Dimensions And Items

Dimensions	SF-36	SIP	NHP	COOP	PGWB	EQ-5D	15D
general health perception	5			1	3	1 [c]	
physical health function	10		8	1			4 [e]
Mobility		22 [a]				1	1
ADL/IADL		23				1	1 [f]
energy/vitality	4		3		4		1
Eating		9					1
Sleep		7	5				1
Pain	2		8			1	1 [g]
mental health/well-being	5	9	9	1	4		1
cognitive functions		10					
Communication		9					1 [h]
Anxiety					5	1 [d]	1 [i]
Depression					3	1 [d]	1
self esteem							
self control					3		
social health/functioning	2	20	5	1			1 [j]
role function	7	27 [b]		1		1	
health change	1			1			

Notes:

a. The SIP categories ”mobility” and ”agility” are merged in the category ”mobility”.
b. The role function under SIP consists of the categories ”house work”, ”work” and ”recreational and leisure activities”.
c. The respondents in EQ-5D are asked to state their general, present health status on a rating scale (thermometer).
d. In the EQ-5D this item is categorised jointy under Anxiety /Depression
e. 1n 5D the dimensions ”eyesight”, ”hearing”, ”respiration”, and ”urination” are merged in the category ”physical health function”.
f. Equals the 15D dimension ”usual activities”.
g. Equals the 15D dimension ”discomfort and symptoms”.
h. Equals the 15D dimension ”speech”.
i. Equals the 15D dimension ”suffering”.
j. Equals the 15D dimension ”sexual activity”.

Source: Some parts are taken from Hutchinson et al. (1996): Cross cultural health outcome assessment. A user’s guide. European Research Group on Health Outcomes (ERGHO). 1996.

4.10.1 SF-36

The SF-36 (MOS SF-36 or RAND 36-Item Health Survey 1.0) is a generic profile measure, which measures health-related quality of life (health status) in large populations or in different patient groups. As a multi-dimensional instrument SF-36 is capable of measuring both negative health states (e.g. disease) and positive health states (e.g. well-being). SF-36 consists of 8 dimensions, which are sub-divided into a total of 36 items (questions), cf. the list in Table 4.1. The answers to these 36 questions rank from a dichotome (yes/no) to 6 point Likert scales. Sum-scores for each of the 8 dimensions are calculated and transformed on a scale from 0 to 100 by summing up the answers for each dimension. The highest score indicates the best health. An aggregated index score (utility value) of the individual dimensions cannot be calculated. However, research is on-going in this area. SF-36 includes an extra question, where the respondent is asked about the change in health in the course of a year. This question is, however, not scaled. SF-36 allows changes in health status (before and after intervention) as well as between different interventions to be calculated. As SF-36 is short and easy to fill in (approximately 10 minutes) it can be filled in by the patients themselves or through interviews (possibly by telephone). It is an extensively tested instrument with a satisfactory reliability (test-retest 0.60-0.81). SF-36 has been used internationally in clinical practice and research in a number of areas and for different patient populations, to measure differences and changes in health status due to treatment. SF-36 has also been used in HTA. A Danish version of SF-36 has been translated and validated. There is copyright on the use of SF-36. Attempts have been made to further reduce SF-36 to include only 20 units (MOS SF-20), 12 units (SF-12), or 6 units (SF-6).

Further information on SF-36 (examples):

Bro S, Bjorner JB, Tofte-Jensen P, Klem S, Amltoft B, Danielsen H, Meincke M, Friedberg M, Feldt-Rasmussen B. A prospective, randomised multi center study comparing APD and CAPD treatment. Perit Dial Int. 1999;19(6):526-33.

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4.10.2 Sickness Impact Profile

The Sickness Impact Profile (SIP) is a multi-dimensional general health status instrument which measures the patient's perception of the effect of a disease on common everyday activities. SIP is based on an advanced model for the behaviour of the disease, and is in particular useful for measuring behavioural changes during a period of time in different patient groups. The SIP instrument is divided into 12 dimensions, which are further sub-divided in 136 units (questions), cf. the list in Table 4.1. In SIP, the respondent is asked to state which statements are related to the person's situation and perception of health status. Then each dimension is weighted in order to reflect the difference in the severity of the behavioural limitation. The sum-scores are calculated for each dimension based on values, which are determined beforehand, for each statement. In SIP it is possible to merge dimensions into a physical dimension (*ambulation, mobility, motion*) and a psychosocial dimension (*social interaction, emotional behaviour, alertness/intellectual behaviour, communication*). Despite the name, the SIP instrument can be used as an index, as it is possible to calculate a general SIP-score, where the highest score indicates the worst possible functioning ability. The test-retest reliability of this has proven to be high (>0.79), while the individual questions have a lower reliability. SIP can be filled in by the patients themselves or through interview (25-30 minutes). The instrument is used for general populations and a vast number of

patient groups (general practice, hospitals, etc.) and disease areas. As SIP focuses on the behaviour of the patients, SIP is not suited for use when assessing treatments which are not likely to influence the behaviour of the patients. A Danish translation and validation of the SIP instrument exists.

Further information on SIP (examples):

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4.10.3 Nottingham Health Profile

The Nottingham Health Profile (NHP) is a short, generic, multi-dimensional profile measure for the assessment of perceived suffering in connection with serious or potentially handicapping health states. The objective of the instrument is to uncover the layman's opinion of health rather than the opinion of the health professionals. NHP consists of two separate questionnaires. Part 1 includes 6 dimensions divided into 38 items (questions), cf. Table 4.1. The questions are negatively put and the respondent confirms or denies these by answering yes/no. Within each dimension the statements are weighted, and an aggregated score can be calculated. Yes-answers to all questions (value 100) indicates the worst possible health status for the given dimension, while the best health status is indicated when the answer is no (value 0) to all questions. The separate domain scores cannot be aggregated and an overall NHP score cannot be calculated. In part 2 of NHP, the patient must answer (yes/no) to if the experienced health status limits seven everyday activities (*job, housework, home life, social life, sexual activity, hobbies and interests, holidays*). Part 2 is not weighted. The advantages of NHP are that it has been applied for many patient groups, health areas and countries, is simple and easy to manage (can be filled in by the patients themselves in 10 minutes), and validity and reliability (test-retest 0.52-0.85) are satisfactory. The disadvantage of the instrument is, on the other hand, that it is less sensitive to less severe health problems (temporary conditions), and that the negative wording has been criticised. A Danish translation and validation of the NHP questionnaire exists.

Further information on NHP (examples):

Bucquet D (editor). European Guide to the Nottingham Health Profile. Montpellier, 1992.

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Thorsen H. Helbredsstatusmål. Erfaringer med Edinburgh Rehabilitation Status Scale og Nottingham Health Profile. Foreningen af Danske Lægestuderendes Forlag. København, 1995.

4.10.4 COOP/WONCA

The COOP/WONCA (Dartmouth coop Functional Health Assessment Chart/WONCA) is a short generic, multi-dimensional profile measure for assessment of functional health status of patients in general practice. However, the instrument has also been used for example for patients in hospitals, as well as for patients with specific diseases, e.g. asthma. COOP/WONCA aims at assessing the actual performance for a number of physical, social and work related activities which are considered normal for healthy persons. COOP/WONCA focuses on 6 dimensions of health, each measured by one item (question), cf. Table 4.1. COOP/WONCA is a visual questionnaire, where the questions are illustrated in simple drawings, where the respondent must assess his/her own health status on a 5 point ordinal scale (optimal to poor capability/well-being) A high score equals a bad health status. The individual dimensions can not be aggregated in COOP/WONCA, thus an overall score can not be calculated. The questionnaire can be filled in by the patient him/herself, with assistance from health professionals, or can be filled in by health professionals as proxy (<5 minutes). Validity and reliability are satisfactory (test-retest 0.64-0.83 after two weeks). The advantages of the COOP/WONCA questionnaire is that it is short and easy to manage, and that it is accepted by patients and health professionals. A Danish, validated version exists.

Further information on COOP/WONCA (examples):

Lindegaard PM, Bentzen N, Christiansen T. Reliability of the COOP/WONCA charts. Test-retest completed by patients presenting psychosocial health problems to their general practitioner. Scandinavian Journal of Primary Health Care, 1999;17(3):145-8.

Nelson E, Wasson J, Kirk J, et al. Assessment of function in routine clinical practice: description of the COOP Chart method and preliminary findings. Journal of Chronical Diseases, 1987;40(Suppl 1:55S-63S.

Weel C van, König-Zahn C, Touw-Otten FWMM, et al. Measuring functional health status with the COOP/WONCA charts. A manual. NCH series no. 7. Groningen: Northern Centre for Health Care Research, 1995.

4.10.5 Psychological General Well-Being

The Psychological General Well-Being Index (also called General Well-Being Index) is a generic, multi-dimensional index measure, which is used to assess the patients' self-representation of intrapersonal affective or emotional stage reflections, as sense of subjective well-being or distress in large populations or different patient groups. The instrument consists of 6 dimensions, sub-divided into 22 items (questions), cf. Table 4.1. Each question has 6 possible answers and is scored on a 5 point scale, where the lowest score equals the worst state of health. For each dimension a sum-score can be calculated, and for the entire PGWB index an overall score can be computed. PGWB is usually completed by the respondents themselves (8-15 minutes) due to the intrapersonal starting point, but it has also been used in interview form. The test-retest reliability in PGWB has been 0.50-0.86 in various in samples. The advantages of PGWB are that it is easy to manage and score, and at the same time it has clearly defined objectives for emotional well-being. PGWB has been used for example in clinical studies with both inpatients and outpatients with physical as well as mental health problems. The PGWB questionnaire exists in a Danish version.

Further information on PGWB (examples):

Becker N, Sjogren P, Bech P, et al. Treatment outcome of chronic non-malignant pain patients managed in a danish multidisciplinary pain centre compared to general practice: a randomised controlled trial. Pain, 2000; 84(2-3):203-11.

Dupuy HJ. The psycological general well-being (PWBG) index. In: Wenger N, Mattson ME, Furberg CD, et al., eds.. Assessment of quality of life in clinical trials of cardiovascular therapy. Washington DC: Le Jacq, 1984: 170-83.

4.10.6 EuroQol-5D

The EuroQol-5D (EQ-5D), which is a short, generic, multi-dimensional index measure, is designed as an instrument that is not specific to any disease, to describe and valuate health states. In the EQ-5D questionnaire patients describe their present health-related quality of life in five dimensions, cf. Table 4.1. Each dimension is then sub-divided into three levels of severity (*no problems*, *some problems* or *extreme problems*), where a low score equals good health. EQ-5D incorporates a total of 243 health states, plus the states unconsciousness and death. Furthermore, an assessment is made by the patients themselves of their present health-related quality of life on a visual, analogue scale (thermometer 0-100). A high score on this equals a perception of a high health status (general health perception). EQ-5D is performed by the patients themselves or through an interview and takes only short time to fill in (2 minutes). Other ways of using EQ-5D are: 1) comparison of the patients on the particular dimensions and for a certain time span, 2) analysis of the EQ-5D health states, 3) analysis of the patients' direct assessment of their own health status (the VAS score), and 4) EQ-5D health states can be converted into an aggregated single-numeric index score for each patient by using derived population-based preference weights for the 245 health states. The latter application makes it possible to use the EG-5D as utility measure, and thereby calculate the number of QALYs which have been obtained by the various treatments that are used in connection with economic evaluation (cost-utility analyse – see the chapter on economy). Danish preference weights are under construction for this purpose. EQ-5D is a widely recognised and validated instrument with a high test-retest reliability (0.90). It has been used for both general populations and for specific patient groups in many different disease areas. EQ-5D can, however, be supplemented with other generic health status measures to obtain further description of the individual dimensions. A Danish version of EQ-5D has been translated and validated.

Further information on EuroQol-5D (examples):

Brooks R with the EuroQol Group. EuroQol: the current state of play. Health Policy 1996;37:53-72.

EQ-5D. User guide. 1999.

Gudex C, Sørensen J. EuroQol: Et generisk mål for helbredstilstand. Månedsskrift for Praktisk Lægegerning 1998, oktober:1339-45.

4.10.7 15D

The 15D questionnaire, which is developed by Harri Sintonen, Kuopio University in Finland, is a generic, multi-dimensional measure, managed by the respondents themselves, for health-related quality of life. The measure can be used both as a profile and to give a single index score. As the name indicates, 15D covers 15 dimensions, cf. Table 4.1, where each dimension is divided in a 5 point scale reflecting the severity of the dimension. One of the characteristics of 15D is that it includes specific dimensions regarding eyesight, hearing, respiration, urination and sexual activity, and it will therefore be relevant to diseases which affect these functions. With its 15 dimensions and 5 outcomes 15D incorporates more than 30 billion possible, different health states, making it, at least in theory, an extremely sensitive instrument. Other advantages of 15D are that it is easy for the patient to fill in (5-10 minutes), and that it is well accepted. The test-retest reliability has also proven to be high (0.92-1.0). Furthermore, 15D has been developed to be used for children. 15D is based on multi-attribute utility measures, and weights between the individual dimensions exist. This means that for 15D, as utility measure, a single overall utility score (index score) for health-related quality of life can be calculated for different patient groups and for comparison of treatment alternatives. This information can be used in economic analyses (cost-utility analysis) to calculate

the QALYs which have been obtained by using a treatment, see the chapter on economy. A Danish version of the 15D questionnaire has been translated and validated.

Further information on EuroQol-5D (examples):

Kauppinen R, Sintonen H, Vilkka V, et al. Quality-of-life measures and clinical parameters in asthmatics during three year follow-up. Monaldi-Arch-Chest-Dis. 1998 Aug; 53(4): 400-4.

Kannisto M, Merikanto J, Alaranta H, et al. Comparison of health-related quality of life in three subgroups of spinal cord injury patients. Spinal Cord., 1998;36(3):193-9.

Rissanen P, Aro S, Sintonen H, et al. Quality of life and functional ability in hip and knee replacements: a prospective study. Quality of Life Research, 1996;5(1):56-64.

Literature for Chapter 4b

Bentzen N, Christiansen T, McColl E, Meadows K. Selection and cross-cultural adaption of health outcomes. European Journal of General Practice 1998;4:27-33.

Bowling A. Measuring health: review of Quality of Life Measurement Scales. Oxford Univ. Press, 1997.

Brooks RG. Health Status Measurement. A Perspective on Change. Economic Issue in Health Care. 1995.

Fitzpatrick R, Davey C, Buxton MJ, Jones. Evaluating patient-based outcome measures for use in clinical trials. Health Technology Assessment 1998;**2**(14). En rapport fra det engelske nationale MTV-program, hvis rapporter kan downloades på internet-adressen: http://www.hta.nhsweb.nhs.uk/htapubs.htm.

Goodman CS. TA101. Introduction to Health Care Technology Assessment. National Library of Medicine. 1998.

Hutchinson A, Bentzen N, König-Zahn C, on behalf of the European Research Group on Health Outcomes (ERGHO). Cross cultural health outcome assessment. A user's guide. 1996.

McColl E, Christiansen T, König-Zahn C. Making the right choice of outcome measure. Kapitel 2 Hutchinson A, Bentzen N, König-Zahn C, on behalf of the European Research Group on Health Outcomes (ERGHO). Cross cultural health outcome assessment. A user's guide. 1996.

Spilker B. Quality of life and pharmaeconomics in clinical trials. Lippincott-Raven Publishers, 1996.

Chapter 5: The Organization *by Karsten Vrangbæk, Kjell Tryggestad and Finn Borum*

Chapter 5a: HTA Administration and Organization *by Karsten Vrangbæk*

5.1 Introduction

5.1.1 Health Technology Assessment and political/administrative science

A common starting point for political science and administrative analyses is that conflicting interests exist. Organisational arrangements typically favor certain actors and interests over others. Similarly some objectives will be easier to accept for some actors than others Objectives within organization are therefore often compromises - many and different views of both objectives and criteria may exist. Similarly it can be argued that administrative and organisational structures are expressions of compromises and developments over time. They may not be optimal structures, but are structures that have developed through compromises and conflicting interests. One can say that the understanding of administration, organization, objectives and means in the health care sector is undergoing constant development, and that there are on-going discussions of how they should be defined. HTA analyses will be part of such discussions, because in an HTA one chooses particular criteria and focus areas. Therefore, one must keep in mind that no matter which set of criteria one chooses for an administrative/organisational HTA, it will represent one of several possible interpretations and, equally, the related choice of measuring methods will be one choice out of many possible. This means that an administrative/organisational HTA cannot be defined once and for all. Focus areas and dimensions of analysis have to chosen from case to case, and one must be able to argue for these choices.

It should also be kept in mind, that in relation to administration/organization there will usually be more than one way to reach the objective. Furthermore, it must be kept in mind that administrative organizations often have several concurrent objectives. Health care organizations are often expected to pursue a number of different goals such as quality of treatment and economic efficiency, flexibility and control of activities, development of knowledge and efficient management, staff participation, political legitimacy and acceptance in the general public etc.

Due to the multiplicity of objectives and perceptions administrative/organisational HTA analyses will be less pre-determined and more complicated than economic analyses and clinical-medical analyses. The methodology is more contested and results are generally more ambiguous. It is therefore very important that one makes explicit choices when planning the analysis. It is also wise to be more cautious about making firm conclusions.

Usually, it will be difficult to isolate and measure <u>output</u> effects of given organisational initiatives. A more realistic and not less important ambition must be to use administrative/organisational HTA analyses to describe various <u>process</u> dimensions in the relationship between technology and organisational behavior.

In any case, one must consider HTA analysis to be more an input into a political and interest-driven decision-making process, than an exact science. However, seen as such the administrative/organisational HTA is an important tool. The impact of a technology can only be evaluated by looking at how it is used, and it is in the interaction between the technology and the organization that both the economic and clinical consequences are determined.

The ambiguities of administrative and organisational HTA should therefore not lead to abandoning the evaluation of organisational aspects of medical technology. On the contrary it can be argued that many decisions in both politics and business are taken on basis of incomplete information. The ambition of the administrative/organisational HTA analysis must be to create a more informed basis for assessing various choices and the consequences they may impose on the clinical, economic and patient-related dimensions, while keeping the uncertainties of such analyses in mind.

5.1.2 Definition of Health Technology in an administrative/organisational perspective

Health Technology consists of a combination of techniques (drugs, diagnosis, treatment, nursing) and a set of behavioral patterns around the techniques. A technology only exists through the way it is applied, and it is through the interaction between technology, staff and patient that the technology can create changes. The organization is based on making behavioral patterns formal and part of the routine, and this will depend on various elements in the individual environment (existing structures, processes, basis for understanding, parties in and around the organization etc.).

How large a proportion of the complete health technology the technical component comprises may vary but there are organisational and behavioral elements in all technologies. In some cases, the assessed technology is synonymous with a change in organization/behavioral patterns. Here, it is especially important to focus on the organisational dimensions.

The interaction between technology, structure, parties/culture and activities will be adjusted in an on-going process, and it is difficult to determine beforehand, which course this adjustment will take. It is likely that there will be more courses than one, and there are various models, which can be applied, depending on the organisational context.

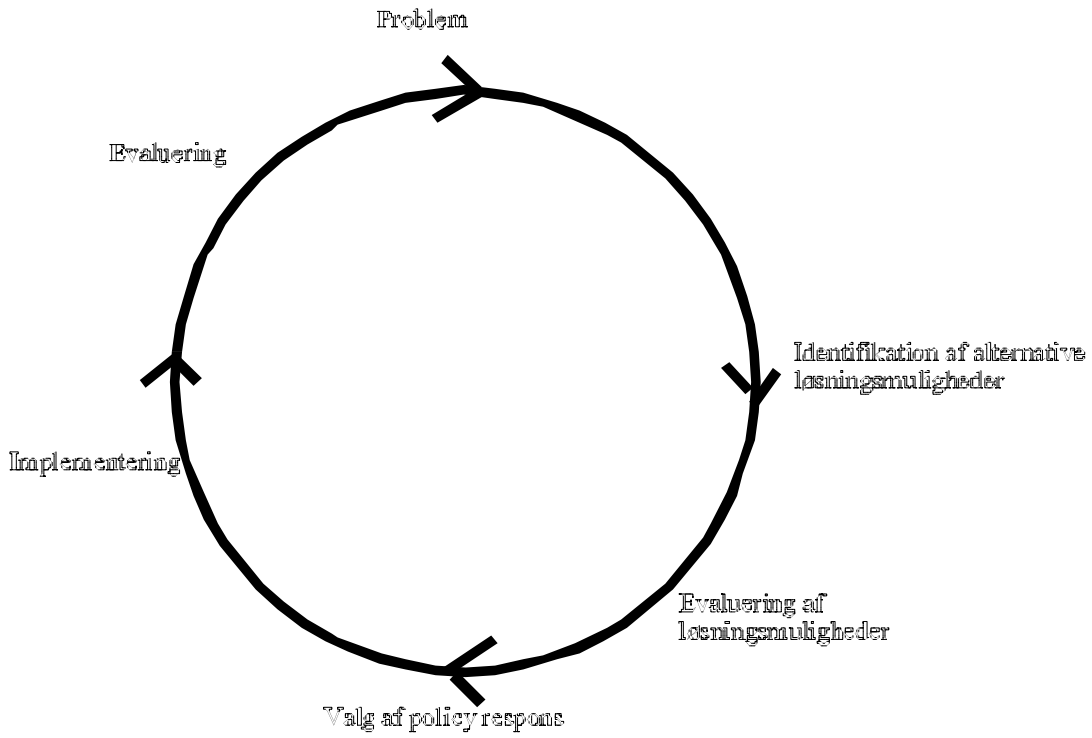
Administrative structures are to be found in and around organizations. These mechanisms are supposed to influence the organisational behavior in certain directions based on political or administrative choices. Administrative structures are usually aimed at control, co-ordination, allocation, evaluation, communication, information etc. There are particular norms and traditions for how these dimensions are handled in different areas and at certain times. The actual structure is the result of compromises and tradition. Global administrative structures can set up the framework for all parties in the system, but often there will be differences in the local interpretation of the general rules (Czarniawska 1997, Bentsen et al 1999).

The aim of administrative/organisational analyses is to pinpoint some of the dimensions, which can be of importance for how the interaction between technology, organization and administration develops. That is to say, to describe some of the elements, which could play a part in the interaction between the behavioral patterns around the technology, and point out possible consequences of different directions, well aware that there are substantial uncertainties in the assessment.

5.2 Dimensions of Political-Administrative HTA Analysis

The perspective in this section is to describe HTA as a tool for making political or administrative decisions on whether to recommend or reject certain technologies. The perspective is thus a top down perspective where decisions to introduce new technologies are taken at a political or administrative level and where one should consider barriers and opportunities concerning the new technology, particularly regarding the existing organisational structure and the various actors and interests connected to the current workflow.

The process of making such political-administrative decisions may conceptually be divided into several different phases as illustrated by the following figure.



The stages in the model are the following:

1) Problem identification
2) Problem definition
3) Identification of solutions
4) Analysis of consequences of different solutions
5) Choice of solution
6) Implementation of solution
7) Evaluation of solution

As regards the HTA analysis the model can be described as follows

5.2.1 Problem identification

The first task of the HTA analysis is to describe if there, in the situation at hand, is a problem, or perhaps more problems, to which the new technology may represent a solution. Sometimes the need is not yet realized. In other cases the technology in itself may lead to identification of not yet realized needs. Depending on the type of technology the problems identified may be related to treatment, resources, organization or management. Problems can be defined narrowly or more broadly. Usually, how one defines the problem will have implications when selecting solution models.

Evaluation of problems and possible solutions can be done through literature studies and by way of interview/questionnaires to key persons in the sector. Important questions are: Have others chosen to use the technology and in relation to which problems? How was the solution implemented?
It is useful to include a broad segment of actors/agents in the data collection process, in order to avoid bias in the evaluation. One may try to quantify the size of the problem in comparison with other problems in the sector.

>> Example: It has been decided to perform an HTA analysis of a new type of endovascular treatment technique. In this part of the analysis one must map which problems this type of technology can solve. How many patients are suffering from the given problems, and how much are they suffering? One should establish if there are existing solutions, which reduce the problem, and to what extent existing technologies reduce the problem. One could ask the question, can the given technology perhaps solve other types of problems?, or reveal new problems? It is important to gather information from different parties, as the understanding of (and interests in) presenting the nature of the problems may differ.

5.2.2 Solutions and consequences of solutions

The next step is to analyse alternative solutions to the defined problem/s. There are at least two dimensions in this analysis namely the assessment of alternative technologies and the assessment of alternative ways of organizing the use of a particular technology.

a) Alternative technologies
There can be different competing technologies, which aim at solving the same type of problem. One of the competing technologies can already be implemented in the organization, which means that one should compare an old technology with a new one. In other cases the alternative solutions are all new.
The above mentioned definition of health technology includes that the proposed change can be of a purely organisational nature. The new technology could consist of reorganization of treatment or nursing routines, or perhaps be a new way to apply existing technology. The approach is, however, the same. The purpose is to map the different alternative solutions and assess the possible consequences they may have (cf. below).

b) Alternative ways of organizing the use of a particular technology
Typically, technologies can be introduced in various ways. The aim of this part of the analysis is to describe the various possible organisational forms surrounding a given technology and evaluate the consequences of the various forms in relation to the parameters one chooses to look at in the analysis. Different elements for description of form of organization are described below.

As criteria for assessing the consequences one can start with the official objectives for the area, or one can define ones own objectives (cf. point d. below). It is important to be explicit about the applied criteria.

It is important, both for point a and b, that one performs a broad search for possible alternatives. In many areas there will be both medical and surgical solutions. There can be both organisational, preventive and treatment approaches. This can be illustrated by, for example, choice of treatment for type 2 diabetes. Here, one can imagine, apart from the pharmacological solutions, a vast number of measures available, such as prevention and changes in lifestyle (diet, smoking, exercise) diabetes schools, motivating talks, courses, interaction between sectors etc. Ideally, one should look at all alternative solutions and combinations hereof. Realistically, one could perhaps list the various initiatives/possibilities, and then choose some of these for further examination.

For both point a and b it is important to seek the opinions of several agents/actors when assessing the relevance and effect of the solution. Some parties will hold specific interests in promoting the solution at hand, while others will seek to restrain the technology. Part of the analysis is to uncover who gains and who loses if the new technology is introduced (cf. next point).

c) Interests. Who wins and who loses?
An element of this analysis is to evaluate how the different actors and interest groups might react to the various solutions. Who wins and who loses given the design of the various technologies/organizations? It is important to analyse parties both inside and outside the primary treatment relations. Main groups of actors and interested parties are:

Actors and interests groups regarding new health technology.
Various staff at hospital level (groups of physicians, nursing staff etc.) General practitioners and specialist Physiotherapist, occupational therapists etc. Various social and nursing functions Municipalities Other counties and the Association of County Councils in Denmark National authorities (National Board of Health, Ministry of Health) Various patient groups Various departments/department administrations Various hospitals/hospital administrations Administrative parties Political parties (county politicians, national politicians) Interest groups (patient associations, staff clubs) Pharmaceutical firms (suppliers and sub-suppliers etc.) Pharmacies/pharmacists

>> Example: Introduction of the previously mentioned endovascular treatment. Obviously, some patients will benefit more from the technology than others. This is true across treatment groups, but perhaps also inside the group, as some patients will react more positively to treatment than others (or patients who were previously more difficult to treat could now be treated). The technology also moves competence and responsibilities from the surgical area to the X-ray diagnosis area. Surgeons will therefore loose work areas and expertise, while radiologist will gain from this. Correspondingly, one could imagine that small hospitals or local departments could use the technology to maintain their footing. A centralized implementation on the other hand may lead to further weakening of departments at the local level. Suppliers of the technology will gain from the implementation. Suppliers of the alternative technology used so far will loose. On the political level, it must be assessed which global consequences the new technology could have. Will it result in pressure on other regions/counties if the technology is implemented? Will the technology be accepted or rejected centrally and among the political parties?

How can questions a, b and c be investigated? Generally it is useful to perform a combination of literature study, perhaps field research, interviews, description of work-flow etc (see the section on methodology at the end of this chapter). A useful method is to start with key informants, who then can point out other important informants in the field. On the background of this "cob-web method" one can map some of the important parties and opinion networks around the given technology field.

d) Criteria
Which criteria should then be applied for assessing the various solutions? The following table illustrates some possible criteria.

Criteria for political/administrative evaluation of new health technology
Financial control Transparency and administrative possible solution Politically legitimate/accepted by the public Equity (use and access). Justice Effectiveness (various measurement dimensions) Co-ordination and interaction (internally and externally) Quality (various measurement dimensions) Development of knowledge/research Job satisfaction and staff participation Staff courses, attraction and adherence of staff. Management competence Service (waiting, information etc.) Balance between preventive actions and treatment. Primary, secondary and tertiary sector. Robustness (Will the system be able to endure different degrees of workload? Any weak links in the chain?) Freedom of choice. Flexibility for patients and staff. Planning capacity.

The list represents a number of typical political/administrative criteria. One approach could be to start from the top, and systematically consider the given intervention in relation to the listed criteria/problem fields. Another approach would be to choose the criteria, which seem the most suited for the particular study.

A central point of the discussion of criteria is that particular technology-organization combinations may have conflicting effects, in relation to different criteria. It is important to uncover such conflicting effects, in order to avoid recommending a technology based on one-sided effect assessments. Typical conflicts could be between a user-orientation and dimensions of effectiveness; expenditure control and promotion of activity/reduction of waiting time; co-ordination/planing and the possibility of choice for the patients/autonomy for attending staff; prevention and treatment etc. This is why one must be very conscious of and open to the choices of criteria, which are made in the analysis.

5.2.3 Choice of solution

This is essentially a "political" problem, where one must consider the various solution models in relation to different interests. Often the solution models will have different consequences for different dimensions and in relation to various objectives (cf. above). The decision process involves considering the various dimensions on the background of the information which isprovided in part by the HTA analysis, and then make the choice which supports the desired mix of results. Usually, there will be uncertainties connected to the organisational-administrative and patient-related analyses (cf. above), but also the clinical and economic consequences will vary with the particular organization and behavior surrounding the technology. This is why decision-making is not an easy or technical discipline. It is about considering various interests on the basis of uncertain information. The aim of the HTA analysis is to examine the various alternatives a little closer, but it will seldom be possible to reach definite answers.

The implementation dimension should be included in the decision phase (cf. below). In the same way, a dimension in this analysis should be to assess the various possibilities and barriers for support of the various solutions. It may not be the technically most suited solution, which will be able to gain the most support in the decision process. Part of this evaluation is the consideration of where the decision is to be made. For example, it could be made on department level, hospital administration level, county administration level, county political level, or national level. The choice of decision arena must depend on the particular project and the possible implications of implementing the technology. The choice of arena influences which parties will be involved and thereby the assessment of possibilities and barriers.

5.2.4 Implementation

It is of little use to have a clear definition of the problem and a technically optimal solution if it is unlikely that it will be implemented. Problems of implementation can arise for many reasons. It could be because of resistance from the practising levels (due to culture or interests). It could be due to lack of clarity or complexity in the decision basis and the decision itself. It could be due to conflicts with existing routines. It could be due to resource bottlenecks (personnel, supporting technology, time etc.) or there might be knowledge and competence barriers. The dimensions for the organisational analysis listed below aims, among other things, at uncovering such possible implementation barriers.

A main element in the implementation analysis is a broader assessment of the administrative structure surrounding the arrangement. Relevant questions could be how is it decided where the particular technology should be placed? Should all counties and hospitals have access to the technology? How much should be paid for use of the technology? Can these decisions be made on a decentralized level or are there reasons for favouring a central co-ordination? Is the co-ordination between primary, secondary and tertiary sector affected? How about co-ordination with social care, housing, environmental and other relevant administration areas? How and who does the follow-up on results? As mentioned, the answers to these questions may vary from area to area (and from technology to technology), but it is useful to analyse differences and similarities. Generally, this will enable an assessment of both the conditions for implementation of the technology and of the possible processes of change, which the technology will introduce.

The following table lists the main dimensions.

Dimensions of implementation. Administration.
Structures for decision-making and planning. Co-ordination of adoption between administration levels and across units. Centralisation/decentralisation of decision-making about technology. Co-ordination with other sectors and administration areas. Structure for financial control of technology. Structure for communication and co-ordination of the use of technology. Structures for control and evaluation. Resistance. Cultural and interest based barriers Competence or knowledge barriers Bottlenecks (staff, equipment, time etc.) Unclear rules and guidelines Main question: How is the use of the technology controlled? Is it necessary to establish particular structures for co-ordination, payment, control etc.? Who makes the final decision of implementing the technology? Is it necessary/desirable to have co-ordination across administrative units and levels? How is the follow-up on the results made? Can special administrative barriers or bottlenecks for implementation be identified?

As regards the method it can be useful to perform the above detailed analysis of the treatment flow both before and after a possible implementation of the new technology. Furthermore, it may be relevant to look at the literature for previous implementation processes to see which factors seem to promote or limit the implementation processes. Literature studies, interviews and participant observation are additional methods. Other possibilities are description of structure/process and "testing" of the description through interaction with parties in the fields.

5.2.5 Evaluation

Thoughts of how, when and according to which criteria the technology can be evaluated should be built into the HTA analysis. The effect of the technology-organization interaction will, typically, develop in the course of time. This can lead to variation and both poorer and better results. Therefore, it is essential for the assessment of a technology to perform follow-up on the HTA analysis, when the technology has been implemented for some time. Such evaluations will also provide useful knowledge of conditions in the implementation process, variation in use, local interpretation etc. For further discussion, see the section below on "HTA as an on-going process".

5.3 Organisational Analysis

The organisational analysis can be said to form part of the mapping of solutions/consequences of the implementation, as described above. In the following the aim is to expand the above points by describing a number of different organisational dimensions, which can be important in relation to assessment of the particular technology. When performing this analysis, one must be attentive to the fact that it will seldom be possible to point to a single optimal organization model for the new technology (see also chapter 5b in this book). The formulation of the problem is complicated by the fact that organizations usually have more, and sometimes conflicting objectives, and that there in and around the organization may be various opinions of success criteria and effects. Furthermore, many processes in health care organizations are complex and there can be various underlying and contributory factors for outcome effects of a given organization/technology change. Yet another point is that implementation of technology will often be an on-going and progressive adjustment. This raises the question of when effects should be measured. Typically, ex ante assessments will be uncertain, while continuous follow-up of the development in a given organisational context can give indications of the development in the course of time.

As a consequence of these complexities, it is usually impossible to predict the consequences of a given organization-technology combination in connection to output measures. A more fruitful approach is to focus on the interaction between the organization-technology and the organisational processes in order to describe the possible impact and the alternative options. The natural starting point of an analysis of change in processes will be to map the current work-flow/patient-flow. Then one may expand the analysis to include various structural and management dimensions linked to the work processes.

The main approach in the following is to list a number of dimensions aimed at the analysis of organisational processes, which should be considered when implementing a given technology. The answers to these dimensions may vary from organization to organization, but there are also cases, where one may see common features. The assessment of the particular organization in relation to general dimensions and indications can form the basis for reflection as to the organisational arrangement of the technology and perhaps, after some time, to “soft” comparisons across organizations, for example, by establishing exchange of experiences, bench marking, discussion of ”best practice” etc.

5.3.1 Description of technology and organisational change

The starting point for assessment of a particular technology should be a description of the technology, which looks closely at the particular work process and describes the possible organisational choices in different parts of the process. Of course, the description will not be complete and the particular interpretation can vary from place to place, but the description may clarify some of the conditions, choices and consequences, which are immediately visible. The data collection for the assessment can be done through literature study and collection of experiences from other organizations, which have worked with similar technologies. Making workflow and patient-flow diagrams in relation to the existing production can make up a useful basis for assessment of the changes the new technology may introduce.

The following tables list the dimensions for this analysis.

a. Organisational processes

Dimensions for description and ex ante assessment of organisational adjustments.	
Process	Description of the technology e.g. via work-flow diagrams. A) Work-flow How is the technology applied? Describe patient-flow and work processes. How are the existing patient-flow and work processes influenced? How does one secure continuous control and evaluation? B) Staff, education and resources. Which parties and resources are needed to use the new technology? Is extra staff needed? Is different staff or further education needed? Who decides which patients are to undergo treatment? On what basis? C) Interaction and communication Interaction with other parts of the structure (other treatment units and shared functions such as financial control). Explain the consequences for other treatments and other treatment units inside and outside the department. Interaction and communication with patients and relatives. Explain the changes and new demands. Interaction with external parties (other hospitals, general practitioners, pharmacies, technical consultants etc.). Explain the changes. Are changes in economic reporting and payment structure needed? Can potential bottlenecks (staff, money, knowledge/information) in the work process be identified?

The first point is a description of work processes and changes in work processes as a result of the new technology. How does the technology work in detail and which resources are needed to start? Which choices can be made regarding the technology and how is it done in other places? Is the current work-flow affected and will there be consequences for the patients going through the system?

The next point covers various dimensions related to staffing, education and resources. Main issues are questions of whether new staff must be brought in or existing staff must be trained to perform new functions. Other questions concern the decision structure regarding the technology. Which kinds of staff can make decisions regarding when and how to use the technology? Should it be available to all types of patients or only to some? Should there be guidelines for the use of the technology?

The third set of issues deal with interaction and communication. In every organization there is a need to coordinate and communicate around the work processes. The question is how this coordination should take place in regards to the new technology. How should the various parties interact? There are several pertinent dimensions such as relationships between patient-professional, professional-professional, department-department, professional-management, external-internal professionals etc. An important question is whether it is necessary to create new structures for information exchange or monitoring of activities?

A major part of this analysis is to evaluate the potential for bottlenecks in terms of staff, resources, information etc. which may create problems in the work flow and general organisational processes around the new technology.

Methods for investigating the issues could be literature studies and description of work processes. In addition one may apply various qualitative data collection methods such as interviews and participation studies (see section on methods below).

b. Organisational structure
The next set of dimensions relate to the organisational structure

Structure	A) Centralisation/decentralisation. Diffusion of technology. Where is the treatment placed, as regards organisation? Who handles management and responsibility? Where is the treatment placed, physically? Who makes the decisions of diffusion and organisational arrangement? Who runs the control and evaluation? B) Economy Are changes in payment arrangements, rates, etc. necessary ? Does the technology impose a substantial extra cost, which must be covered by giving lower priority to other types of treatment? Which incentive structures (financial, carrier-wise, work process-wise, treatment-wise etc.) are established with the new technology for staff, patients, and others (general practitioners)?

The first set of issues under this headline concerns the placement of the technology in the organisation. Should the technology be present in all locations or should you build a structure with particular units having responsibility for the technology. Which parts of the organization should be responsible for managing the technology and making sure that the optimal results are obtained. Who should be in charge of evaluating the implementation and use of the technology over time?

The next set of issues relate to the structural dimensions of the economy. Decisions must be made regarding payment arrangements, rates between departments etc. Similarly it should be discussed whether the technology should be implemented with particular incentive structures or sanctions for the various actors in the system. Finally the economic reporting structure must be considered.

c. Cultural elements.

Culture	Attitude and norms among staff and patients. Is it likely that the treatment will be accepted or will it meet resistance? How does the treatment suit the existing routines and traditions in the organization? Are changes in perception and understanding of the treatment needed? Is the technology perceived as having advantages or disadvantages of different staff groups (work conditions and work environment).

The last set of dimensions for organisational assessment of the new technology deal with issues of attitude, norms and traditions among those involved with the technology. If staff or patients are strongly opposed to the new technology for various reasons it is unlikely to become a success. It should

therefore be evaluated if there are particular barriers in terms of perception or traditions in the system, which might hinder the implementation.

Choice of dimensions

The table represents a "check list" with possible dimensions, but one can adjust the list to one's immediate needs. The assessment could start with a literature study and knowledge of the existing organization. Possibly, one could do interviews with different parties inside and outside the organization to get an overall view of the different organisational conditions.

In real life, there will probably be some overlap as "structural" and "cultural" dimensions are closely linked to "process" dimensions. For example, it will be difficult to describe work-flow/patient-flow without describing elements of the organisational and administrative structure of the technology. But it could be an advantage to separate the dimensions in one's mind beforehand.

The table aims at creating dimensions for ex ante description and assessment. However, it is obviously problematic to assess the consequences of a technology before it has been implemented in a real organisational context. Especially when considering that there may be different adjustment possibilities and ways of alteration.

Different strategies can be used to deal with such problems. The most extensive and probably the most productive will be to regard the HTA as a process, where organisations continuously assess adjustment and consequences themselves. Another and less extensive model is to use pilot studies as a basis for assessing possible adjustment patterns before choosing a general implementation of the given technology. Pilot studies provide an opportunity to study various alternative ways of implementation and to some extend to test various models of organizations, though one has to pay attention to the fact that it is the organisational context in interaction with the technology, which create the change. This may render comparison difficult, because one in fact risks comparing different objects. On the other hand, comparison can provide some general indications of possibilities and barriers, even if one may not necessarily be certain of the exact cause-and-effect relations. In the following the advantages and disadvantages of pilot studies are described.

5.4 Pilot Studies

In some cases it will be possible to perform pilot studies before assessing the new technology. Pilot studies provide the opportunity to expand the above ex ante assessments with more thorough descriptions of various organizations. Furthermore, pilot studies can in some cases be used as limited assessments of outcome in relation to different dimensions and objectives. Pilot studies also provide the opportunity to gather more pluralistic perspectives, which can capture differences in the different parties' understanding of objective and importance of the given technology.

If it is possible to perform pilot studies in several different organizations, it will be possible to describe, and perhaps compare, the different organisational adjustment patterns for the technology. However, one must keep in mind that it can be difficult to isolate the effect of one single technology. Thus, it is difficult to know whether exactly the same phenomena in different organizations are being compared. But having a description of different organisational adjustment models canin itself be useful.

Another important possibility of pilot studies is that one can look closer at organisational conditions of the effects, which are uncovered in the medical/clinical and economic parts of the HTA analysis. When analysing the actual behavior and stated perceptions among various parties in depth, one will be able to point out possible barriers and bottlenecks, which lie outside the scope of the clinical and economic

study designs. In particular, one will be able to identify deviation in the behavior of the parties in relation to the set conditions, and one will be able to look at effects of interaction relations in the organization. Finally, one can put the importance of standards, habits and traditions into focus, which in an organisational context will affect the possibilities of reaching the expected results.Through pilot studies one will be able to expand the methodical approach with participant observation, interviews, survey studies (e.g. among patients). This will give the opportunity to form a more detailed view of different parties' perception of the change dimensions and outcome in relation to various parameters.

5.5 HTA as an on-going process

Pilot studies can thus give a better basis for decision-making before full implementation of a technology. As the adjustment of technology takes place over a period of time, and with a continuous development of the organization it will give a more true and complete view to regard HTA as an on-going evaluation process, where the individual organizations describe adjustment and perhaps, on the background of indications, seek to assess whether the development seems to be going in the desired direction. Such evaluations may lead organizations to be more conscious of the results. Analysis of different measurement indicators can be part of the on-going self evaluation. Despite measurement problems it can thus be useful to establish a quantitative body of figures as a supplement to more qualitative descriptions. Joined qualitative and quantitative elements will be part of the gathering of experience and in the varied/interpreted comparison across organizations.

An obvious opportunity in this connection, is to establish fora where representatives from the different organizations can exchange experiences on the background of their various results and descriptions. This can be developed to more formal "bench-marking" exercises and descriptions of "best practice(s)", even though the large gains probably lie in the continuous exchange of experience.

5.6 Methods for administrative/organisational HTA

In organisational-administrative analysis the way to gather information is closely linked to the perspective of the analysis one chooses. Therefore, there are various ways in social science of analyzing and there are a number of actual tools, which can be applied, depending on the chosen perspective of the analysis. The following table displays some of the more important ones.

Methods of structuring	
1) Description of the formal organization structure and the relations between the organization and important external partners, as regards the given technology.	Use of formal diagrams etc. Mapping of functions and relations as regards the given technology.
2) Produce work-flow charts and description of interactive relations (within and across organizations), which the new technology may affect.	An iterative process with involvement of parties from the area and use of interview, participants observations and studies of formal organization elements (organization diagrams, work process descriptions etc.).
3) Produce resource flow tables. Where are the resources used and how will the new technology affect the use of resources.	Use of formal budgets/ statements of accounts. This overlap with "budget analysis" in economic HTA.
4) Cob web method	Starts with a few key informants. Let them point out other important parties. Through systematic expansion of the circles, a web of parties, networks, and opinion structures is formed.

Methods for data collection	
1) Participant observations and field studies. Detailed description of organisational processes.	Establish information to 1, 2 and 3 above. Point out strengths, weaknesses, opportunities and challenges
2) Interview	Establish information to 1, 2 and 3 above. Point out strengths, weaknesses, opportunities and challenges
3) Questionnaires	Useful for gathering a large volume of data on particular questions, but can seldom stand alone.
4) Literature studies and analyses of organisational processes of change in other organizations/systems/networks.	One must be cautious not to generalize across organizations, but one can gather inspiration from reading other analyses.
5) Pilot studies	See closing section.

Regardless of which methods one chooses, one must keep in mind that the study objects (parties, groups) have their own interests, and are capable of modifying both interpretation and behavior along the way. The HTA measurement can thus in itself affect the development, and it may be difficult to see to which degree the conclusion one draws, are affected by interests and the specific social situation around the measurement. You are always dealing with second interpretation in relation to social phenomena (Gilje and Grimen, 1989; Flyvbjerg 1994).

One must remember, as mentioned above, that there are often several possible interpretations of the observations. Organisational realities are constructed in an on-going interaction between various parties, who will take an interest in establishing certain views and perceptions. As an HTA party, it should be taken into consideration that the investigator necessarily becomes part of the interpretative processes by designing and performing the study.

5.7 General remarks on the relationship between political-administrative and organisational HTA analyses

As a general rule, one could say that the objective of administrative HTA analyses is to assess the technology in relation to the political and administrative context consisting of the structure of the administration, control measures, objectives and priorities.The main question in political-administrative HTA analyses is thus, *whether the technology will assist in reaching objectives and priority-setting*. This includes both the formal political objectives (if these exist) and the objectives of various parties. When assuming a pluralistic point of view of measuring functions and assessment criteria, a broad picture of the importance of the technology on various dimensions is displayed. A technology can have positive effects on some dimensions and for some parties, while it has negative effects for others.

Other main questions are *whether the technology can be implemented within the given political and administrative context?* and then *how will the technology affect this context?* Thus, there is a duality between looking at the conditions for implementing the technology, and at the same time looking at how the technology will supposedly be implemented in a process that will affect existing structures, and where various interests will seek influence.

When described this way, there is a certain overlap between administrative analyses and organisational analyses, but as a general rule the administrative analyses apply a top down public management perspective, while the organisational analyses look at changes in and around the production process itself. In the organisational perspective impulses for change may come from other organisations or through professional networks and not necessarily top down.

Typically, the administrative analysis looks at the structures for decision-making and co-ordination across all levels (e.g. government, county, municipality, hospital management, departmental management) and at which control measures (planing, financial control, communication and control) are used to perform political decisions, secure and control the operation of the health services. Often, the organisational analysis focuses more narrowly on organisational conditions and processes of change linked to the particular organisational setting and the individual health services. The overlap appears because the administrative structures are an important part of the surrounding conditions for providing the treatment.
From an administrative perspective the general objective of the organisational analysis is to *describe* the organisational dimensions of the new technology and some of the important conditions for implementation, and possible consequences for the organisational structure, based on the available material, and under consideration of uncertainties and various possibilities for interpretation.

5.8 General remarks on the levels of analysis and importance of administrative/organisational HTA

Typically, the form of the analysis and the possibilities for analysis will vary in relation to which type of intervention/technology one is examining. Is the issue a *particular treatment* (e.g. a new type of drug or a new type of surgical procedure)? Is the issue a *new treatment procedure* (e.g. a new type of cancer treatment, consisting of various treatment elements)? Is it a *change in the organisational procedures in the health care sector* (re-organization to ambulatory treatment, establishing units with

particular functions)? Or is it a broader *structural reform* (establishing free price formation on treatment, transition to activity-based payment, changes in the role of the counties)? The following table illustrates the various aspects of analysis as regards the type of technology. Generally it can be argued that the complexity of analysis rises as you move from top to bottom in the table. At the same time the relevance of political/administrative/organisational analysis increases because these approaches are developed to deal with complexities and because the clinical and economic consequences of the technology will be strongly influenced by the organisational choices.

	Clinical	**Financial**	**Organisational-administrative**
Individual Element of treatment	Experimental controlled randomized trials. Double blinded test etc.	Cost-benefit. Resources/ Output	Conditions and organisational context. Conditions in structure, process and culture
Treatment Program	Experimental controlled randomized trials. Double blinded test	Cost-benefit. Resources/ Output	Political and administrative context. Structure, co-ordination, control. Objectives and priorities. Changes in structure, process and culture
Organisational/ Structural change of the treatment area.	?	Cost-benefit. Resources/ Output	Political and administrative context. Structure, co-ordination, control. Objectives and priorities. Changes in structure, process and culture
Structural reform of important parts of the sector	?	?	Political and administrative context. Structure, co-ordination, control. Objectives and priorities. Changes in structure, process and culture

When assessing individual treatment elements it would be natural to start with the clinical assessments. Here, it is fairly simple to use traditional, medical experimental designs. Even in this fairly simple assessment it will, however, be important to assess the organisational context of the treatment element. How do staff and patients regard the treatment? Are sufficient routines established to secure correct dosage etc.? Is the communication sufficient? How does this treatment element interact with other routines and general understanding of the treatment situation? Correspondingly, it can be relevant to analyse the political and administrative context. Are changes in administrative structure called for? Will the technology promote general objectives and priorities? Which interests are linked to the technology?

As one proceeds further down the table, the assessed technological change becomes more complex. It becomes more difficult to establish isolated experimental designs, and it becomes more difficult to perform economic evaluations based on “all other things being equal” conditions. Thereby the organisational and political-administrative elements will become more important, and it will become increasingly relevant to use descriptive, interpretative and qualitative ways of assessment, to supplement or replace the other HTA dimensions. Only then it will be possible to establish a more realistic basis for decision-making.

Another distinction is whether the HTA can be performed as a global HTA for all parts of the system (all organizations or networks), which are to implement a given technology, or if one has to look at each individual organization's choice of and adaptation to the technology as unique. The tendency in clinical and economic analyses is to see the implementation as independent of the specific organization. It is usually assumed that results can be used globally.

As for political and administrative analyses the result of this will be a mixture. Some dimensions will be of a general nature (assessment according to global objectives, co-ordination on national level, general financial control principles etc.). However, in many other cases the general procedures are translated and interpreted in order for them to be adapted to the procedures in various parts of the system (Czarniawska 1997, Røvik 1998). Thereby one will have structures and topics, which are specific for some parts of the administration, for certain counties or regions. This could be the case for settling of accounts, agreements etc., which are based on particular procedures, geographic or other conditions in different regions. What is preferred in one country or region may not be applicable in another country or region. Thus, one could say that the objective is to describe possible ways of adaptation as basis for comparison.

As for organisational analyses it is preferred to see each adaptation as a unique result of the particular organisations history, development, relation to the surroundings etc. This is why many organisation theorists are sceptical with regard to generalisation and comparative assessments of various utilisation of technology-combinations of organisation (see the chapter 5b in this book).

The perspective in this contribution is that there may be various possibilities, but that at the same time, there are a number of dimensions which are worth considering for all organisations, and which can be the basis for assessments of the process of development surrounding the particular technology, and in the course of time perhaps also for "soft" comparisons such as exchange of experiences, benchmarking etc. The degree to which the results can be generalised, depends on, among other things, the type of technology in question. Some technologies will be easily adapted to all parts of the organisation, while others to a higher degree will induce changes in the existing organisation.

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Chapter 5b: The Organisation

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Contents for chapter 5b:

5.5 REVIEW: THE TECHNOLOGY-ORGANISATION PROBLEM:
IS TECHNOLOGY A NON-ORGANISATIONAL ENTITY? 86

5.5.1 SUMMARY 87

5.6 THE TWO METHODOLOGICAL APPROACHES
- TECHNOLOGY SEEN AS "DIFFUSION" OR AS "TRANSLATION" – EXEMPLIFIED 88

5.6.1 CASE 88
5.6.2 CONCLUSIONS BASED ON THE LINEAR MODEL 88
5.6.3 CONCLUSIONS BASED ON THE TRANSLATION MODEL 89
5.6.4 SUMMARY 90
5.6.5 METHODS FOR ANALYSIS OF THE ORGANISATIONAL ELEMENT AS USED IN THE TWO MODELS 91

5.7 PERSPECTIVES OF EVALUATION 92

5.7.1 A SHORT STORY 93
5.7.2 TOWARDS A FIFTH GENERATION EVALUATION? 94

LITERATURE FOR CHAPTER 5B 94

5.5 Review: The technology-organisation problem: Is technology a non-organisational entity?

With this contribution, central aspects of the current theoretical debate of the technology that isrelevant for HTA matters are described. The essential matter is the question of whether technology is considered to be exogenous or endogenous, and whether technologies are disseminated through diffusion or translation processes. However, no definite answer to how the organisational aspect should be analysed in connection with an HTA is given. The article has different purposes instead:

1. to give input to consideration of which principal assumption of the technology-organisation relationship should form the basis of an HTA
2. to point out the methodological repertoire, which would be relevant for conducting an HTA from an exogenous and an endogenous view of technology, respectively.

Different views of the technology-organisation relation are already expressed in existing HTAs. Thus, the HTA report "Interferon Beta Treatment for Multiple Sclerosis" sees the technology (interferon beta as injection treatment for patients with relapsing multiple sclerosis) as exogenous. Hereafter, the organisational aspects become a matter of the resource strain, which an implementation of the technology will trigger in the health care system. Two other HTA reports "Low Back Pain: Frequency, management and prevention – from an HTA perspective" and "Influenza Vaccination of the Elderly" view the technology as endogenous. "Low Back Pain" includes the organisation of the treatment system in relation to a broad diagnosis group in the assessed technology. The HTA of influenza vaccination does not assess the effects of the vaccine, but four different models for organisation of programmes and their suitability to reach a certain client target group.

The separation between technology and organisation is for example expressed in an often applied starting point for technology assessment "the impact of technology on society" (Miettinen 1999) [3]. This expresses not only a distinction between technology and organisation (society), but also expresses the assumption of a causal order between variables: an independent variable 'technology' with 'effects' ('impact') on 'organisation' (the non-technological matters). This fundamental perspective is also expressed in the Danish HTA model, where the organisational dimensions of HTA are usually defined as structure, staff and environment within a model, where the technology is viewed as *exogenous* in relation to the organisational aspect (Borum 2000). To see the technology as an exogenous, determining variable will lead to identification and measurement of effects, for which there, for example within the socio-technical school, are developed a number of checklists and measurement methods (see for example Borum 1977).

An organisational-theoretical perspective would, however, question the logic of making a distinction between technology and organisation. Instead, technology can be seen as part of an organisational process – as an entity which is *endogenous* to the organisational element. This is expressed in common definitions of the term within organisational theory:
"… most organisation theorists have embraced the broader view that technology includes not only the hardware used in performing work, but also the skills and knowledge of workers, and even the characteristics of the objects on which work is performed." (Scott 1998).

[3] See also Poulsen (1997, especially p 23,25,47) International comparison of 124 technology assessments in the medical field. This study more than suggests the "impact" perspectives dominantes in HTA.

In the table below some consequences of performing HTA, based on the technology's exogenous or endogenous relation to the organisational element, have been identified.

Table 5.1: Methodological consequences of two different views on technology

Methodological Dimensions:	**Assumption 1: Technology as exogenous and stable entity**	**Assumption 2:Technology as endogenous and variable entity**
Definition of the technology	The technology is defined and assessed as independently defined in relation to an organisational practice (essentially)	The technology is defined and assessed through an organisational practice (performatively)
Definition of the organisation	The organisation is defined and assessed as the 'non-technological': 'structure', 'staff', 'environment'. The borderline is the distinction between technology and organisation.	The organisation is defined and assessed through interaction between humans and non-humans (technology). The borderline is not fixed but can be re-defined through practice.
The dynamics of the technology	The technology history is external to organisational practice. Development of it can be explained through exogenously instigated technological leaps andtraces.	The technology history is created though organisational practice. The dynamics of the technology can be assessed and explained through organisational practice.
The dynamics of the organisation	The dynamics of the organisation can be assessed and explained through the "impact" of the technology.	The dynamics of the organisation can be assessed and explained through interaction between people and technology.
Basic model for Technology-assessment	The assessment and diffusion of technologies **The innovation-diffusion model**	The analysis of the technologies creation in organisational practise **The translation model**

5.5.1 Summary

- There are at least two different assumptions about technology, which an evaluator can use as a basis, when HTA is to be performed: technology as exogenous and constant, versus technology as endogenous and variable. The two different assumptions can be found in the literature as: 1) the linear innovation- diffusion model and 2) the translation model.
- The two different assumptions have methodological consequences: they will tend to control the definition of the study object in question in different ways, which will lead to different empirical studies and conclusions. This can be seen from the very diversified treatment of the organisational element in HTAs performed so far.
- As for now, it seems as if HTA is closer to the linear innovation-diffusion model ("Assumption 1" in Table 1), while European technology and organisation research increasingly uses the translation model ("Assumption 2" in the table).

5.6 The Two Methodological Approaches - Technology Seen as "Diffusion" or as "Translation" – Exemplified

In this section a situation relevant for an HTA is discussed. When new technology is introduced in clinical practise, the evaluator will be confronted with a number of questions: Will the new technology work as intended? Does the technology have advantages and/or disadvantages compared to existing treatments? Should the technological innovation be used in other clinics, so more patients will benefit from it? Does the technology set a new national standard for treatment of specific diseases? In such a situation HTA can be conducted using one of two models: 1) The traditional *linear innovation- diffusion model*[4] and 2) The *translation model*[5].

The *linear* model assumes that the technology stays c*onstant* from the moment it is introduced until it is implemented. Implicit in the model is also the assumption that it is the same technology which is diffused from the innovator, for example from an Information Technology Company (ITC), to different users, for example the clinic (C). The *Translation model* presumes that the technology *does not stay constant* during the introduction process – that something will happen to the technology, when it is applied. The term "translation" does, in fact, try to capture the qualitative change during the process.

Depending on which one of the two models that is used as a basis, the health technology assessment will tend to produce different results and conclusions. In the following, using a fictitious example, it is illustrated how the choice of a different basic model for technology assessment has decisive importance for the conclusions of the assessment.

5.6.1 Case

Imagine the following situation: an HTA of the electronic patient (case) records (EPR) is to be made through observation of the results in the clinics K1 and K2. After the observation, it was established that the results of the two clinics were significantly different. In one clinic (K1) a reduction of wrongful administration of drugs of 20% and of the admission days of 10% was established for patients, when EPR was incorporated in the treatment in comparison with the control group, which was treated according to the traditional procedures. In the other clinic (K2) no changes were established due to the use of EPR. The two clinics had comparable patient groups. There were remarkable differences as regards how demanding the use of EPR was: K2 had, on average, allocated approximately 15% more staff time per treated patient compared to K1. For the clinic K1 it was only marginally more resource intensive to use EPR. Now, the question is what conclusion can be drawn from the observations.

5.6.2 Conclusions based on the linear model

If the HTA is performed based on this model, it is obvious to draw the conclusion that the difference in the results between K1 and K2 must be related to the "non-technological" dimensions, such as the different organisational structure and management of the two clinics, or the competence of the staff. Thus, it is these organisational dimensions, which can explain the differences. Such a

[4] See (Rogers 1962/1983), and (Pinch1987/1990) for a closer account of og review of the linear model.
[5] The translation model is due to (Callon 1986 and Latour 1986). For adaption within the organisation theory, see in particular (Czarniawska and Sevón 1996). For adaption with focus on introduction of computerbased technology, see Tryggestad (1995).

conclusion seems logical given the assumption that it is the same technology which is introduced in the two clinics. In this perspective the ”organisation” of the clinics are in focus, when the results are explained. The possible changed importance of the technology in the two clinics is at the same time put out of focus, as the technology is presumed to be stable and constant for all user and client categories. Based on this, the following policy conclusion can be worded: ”As the good results in clinic K1 has proven, EPR has an inherent potential, which can be implemented in other clinics. Furthermore, when applied in the right way, the technology is only marginally more resourceintensive. The implementation of EPR in other clinics should thus be intensified. This can be done through various competence enhancing measures, such as courses to further educate clinical staff in the use of EPR, seminars, study visits to K1, etc.”.

5.6.3 Conclusions based on the translation model

From a translation perspective the task of an HTA is to investigate, how the process evolves and explain why it looks the way it does in the two clinics, K1 and K2. A relevant question, from this perspective, is: how many resources in the form of staff, time, methods for administration of medicine, treatment and follow-up on patients etc. should be mobilised and allocated in order to produce satisfactory results of EPR? Here, one reconstructs the process of introduction for the two clinics in the empirical study. Maybe it turns out, that K1 and K2 have different relations to both EPR and the IT Company (ITC), which produces and supplies EPR. A closer analysis shows that the doctors in K1 for several years have been invited to participate in annual conferences arranged by the consultant department of ITC. Also, an earlier version of the technology, EPI, was used as a pilot study in K1. When EPR was introduced some years later in both K1 and K2, the consultants from ITC were active in the introduction process in K1, this time as well. The representatives helped out in many ways, including introduction to Windows, data recording, medication, documentation of course of disease etc. As ITC did not have the resources to be equally active in K2, the main part of the consultant resources were allocated to the introduction in K1, where good contacts had already been established[6].

Is EPR then the same technology in K1 as in K2? The answer to this is a pragmatic no – because there are two different results from the two clinics. Perhaps more important is to emphasise that the explanation of the difference between the results of the clinics is different from that of the linear model. When using the translation model, it becomes possible to take the explanation of the difference between the results a step further, by including the *technology history*. For EPR is not the same technology for K1 as it is for K2. Considering the relations K1 had previously and still have to ITC, this difference becomes considerable: ITC and K1 create technology history in the interaction taking place during the introduction of EPR. For K2 it is a different technology history, which is created in interaction with EPR: ITC primarily participates via K1, because K2 has not become familiar with EPI, and because EPR does not have the same history in the two clinics. In the translation model the HTA study thus continues to reconstruct the technology history. The linear model, however, sees the organisational boundary of the introduction process as fixed and taken for granted – limited to K1 and K2 – and draws the logical conclusions from this.

At first glance, it seems as if EPR works with a relatively good result in clinic K1, because the staff has acquired knowledge of EPI previously. But these learning processes are not limited to K1s own staff. Also, the representatives from ITC should, therefore, be included in the history, as such interorganisational relations at the same time redefines organisational boundaries. Previous to the

[6] The presence of such interorganisational and interdisciplinary relations between research and different practises is one of the themes in Rosenberg et al. (1994) and Tryggestad (1995). Furthermore, a substantial amount of information is published under the same theme under the titles “technology transfer” and “diffusion of innovation”.

good result of K1 lies several years of contact with consultants from the IT supplier (ITC), which was followed-up during the introduction of EPR with assistance from consultants. K1 was in a way transformed to be an extension of ITC's own development department during the introduction of EPR. This is an important reason for the good clinical and economic results. In other words, EPR could be considered as a health technology, which seems to work satisfactorily under such *laboratory conditions*. For other clinics such laboratory-like conditions are less realistic to establish as organisational standard: for one, the results from K2 suggests that it is not certain that the satisfactory results of EPR in K1 can be copied in general just like that, and secondly, free consultant assistance is not available for all. The fact that ITC has such extraordinary research and development resources available for a clinic (K1) may have various causes: the extra resources can be seen as an expression of an objective for learning more about how EPR works in clinical practice. But it is not unreasonable to consider the extra research and development resources as part of a broader marketing of EPR – it is, no doubt, good for marketing to be able to refer to well documented results.

Furthermore, it is possible to imagine that EPR, if the technology is established as standard for other clinics, may induce increased costs. The observations available, suggest that the use of EPR tends to increase use of staff resources compared to traditional case records. Also, the cost issue has not been dealt with in detail: The total costs in the long run have not been assessed. It would seem reasonable to presume, that the relatively low use of staff resources in K1 would have been higher had the clinic not had access to the support of ITC.

The conclusion using a translation perspective is the following: "The present HTA study has no unambigious results. The same can be said for the results concerning the costs of introducing EPR. Both these circumstances may very well be related to the fact that the two clinics har quite different technology histories concerning EPR – in particular the special laboratory conditions of K1 may have had a decisive effect for both the good result and the marginal rise in staff time spent per treated patient. These ambiguous results lead to the conclusion that EPR should not be recommended as a new standard today, but should be assessed again with normalised laboratory conditions for the clinics participating in the next round. In such an assessment a comparison with established patient (case) records as regards costs for diagnosis methods, administration of drugs, and an assessment of the development of the costs in the long run should also be included".

5.6.4 Summary

Depending on which assumptions about technology and the corresponding models one uses in an HTA, different results and conclusions will be produced. This is illustrated through a case, where the exogenous status of technology is problematized. In relation to the present case, we have argued for an HTA perspective and an HTA model that sees the technology as an organisational event – as an endogenous and variable entity. We will claim that HTA can be further developed through detailed studies of organisational processes, through which the results of the technology are produced.

The Innovation-diffusion model has, according to Rogers (1962/1983), a tendency to accuse the potential user if the diffusion is not going well enough, and it also has the risk of leading to policy-conclusions with a 'pro-innovation' bias. From the innovation-diffusion perspective EPR was assessed to be relevant for more clinics than K1, but in the case it was stated how this perspective did not put enough weight on the organisational dimension of the technology.

From a translation perspective the evaluation will, instead of looking critically at potential users, look into how the technology and its various results are created in interaction with different categories of users, and put emphasis on the organisational processes.

5.6.5 Methods for analysis of the organisational element as used in the two models

In connection with an HTA one must make some basic choices of method with regard to how the analysis of the organisational element is to bedone. A research design should be chosen that fits the model, which forms the basis for the HTA. In the table below the possible methods which are consistent with the two basic models are listed. The references listed after the table are meant as an inspiration for adjusting the methods to the particular case, which is necessary in every HTA project, regardless of that the fact that the model will usually be based on case studies.

If the HTA is performed according to the linear innovation-diffusion model, Yin's understanding of the case-method will be useful. Yin (1994) contains a number of useful considerations as regards choice of single or multiple case studies, embedded case studies and the possibility to make general assumptions based on cases. The analysis of the selected organisational dimensions will demand further adjustments. Mintzberg (1979) is an important source regarding specification of the structural dimensions. The actor dimension within the socio-technic analysis tradition has various different specifications. Most important are the ones regarding job content and change of this as the core (see Borum, 1977), while the culture dimension of Schultz (1990) is cast in two variations: the functionalistic and the symbolic. For an overall view of the basic different strategies for organizational change, see Borum (1995).

If the HTA is performed according to the translation model Latour (1987) has formulated a concrete methodological principle: Follow the actors! Andersen et al. (1992) give a specific guide as to how to make actor based field research following an open ethnographic method. The task of the evaluator is, within this methodological framework, to reconstruct the chain of events which is related to the introduction of the health technology in question. The written report will be in the form of a case, which tells the reader, how the process of introduction took place, including how technology and people interacted during the process – how the symbolic, material and political features of the technology are created and recreated in the process of organising.

Table 5.2: Methods and models for HTA: a summary and specification

Methods and models for HTA	Innovation-diffusion model	Translation model
Assumptions about the technology	Technology as exogenous and stable entity	Technology as endogenous and variable entity
Central HTA questions	Which organisational impact does a health technology have? What resistance will the use of the technology meet? Which strategy of change should be adopted to diffuse the technology?	Which organisational potential does a health technology have? How many resources (material entities, time, money, people, etc.) must be mobilised and organised in order to produce satisfactory results from a health technology?
Primary analytical approach	Measurement of the consequences of the technology as regards the chosen aspects of one or more of the dimensions, structure, party and culture. Identification of possible sources of resistance against change. Argumentation for which strategy for change will most likely promote the use of the technology in order to reach the desired results.	Analysis of the processes in which the resources are mobilised and organised. Identification of which possibilities are used and which are discarded during the process. Analysis of the shaping of the technology and the consequences of this during the process. Discussion of the possibility to transfer knowledge or to reach conclusions that can be generalised to other contexts.
Primary empirical approach	Case studies or surveys. Interviews, collection of quantitative data, analysis of secondary data. Identification of relevant studies.	Interviews with and observational studies of the relevant actors according to the principle 'Follow the actors!' Studies of practice, of interaction between people and technology. The result is a case, which reconstructs the process of introduction of the technology as a series of events.

5.7 Perspectives of evaluation

Evaluation methodology, including technology assessment, has a history. This history should be written, in order to contextualise what technology assessment is, and can be. Therefore, this chapter ends with a short story and the argument for using the translation perspective in technology assessment.

5.7.1 A short story

In the book " Fourth Generation Evaluation" Guba & Lincoln (1990) give a fine introduction to the change of the paradigm of the methodology on evaluation in the course of time. The authors have identified three such change of paradigm [7]:

-Quantitative evaluation aimed at measuring the performance of individuals and/or fulfilment of objectives. Examples: IQ test of students. The evaluation of a student's performance in relation to the objectives and intentions the teacher formulated for the student.
-Descriptive evaluation aimed at reaching the objectives of the groups. Qualitative techniques are now considered as one of more tools in the methodological arsenal. Example: evaluation of an education program's fulfilment of objectives. The students influence the result of the evaluation and thereby the future outline of the programme.
-Evaluation aimed at assessment. This aims at evaluating the fulfilment of objectives through descriptive, quantitative techniques, supplemented by assessment. At the same time, the role of the evaluator becomes more complex: the role as a neutral messenger must now be combined with the role as involved judge. The evaluator assesses fulfilment of objectives as before, but must now also assess the objectives.

Guba & Lincoln also point out three shortcomings related to the three paradigms: a normative bias related to the management/policy-maker ('managerialism'), the problem of incorporation of value pluralism, and an excessive belief in the kind of science, which is known as 'scientific method' (positivism). The ambition is to create an evaluation process open to negotiation, where value pluralism is seen as legitimate. This is seen in contrast to the 'scientific method', which tends to view value pluralism as a problem. In this connection, the authors make a point of giving relevant parties – including those who could be negatively affected of the result of the evaluation – a possibility to have their say. Also conclusions and recommendations should be the result of negotiations between the relevant parties in order to secure value pluralism in the process. Thus, evaluation becomes an integrated part of a continuous organisational process. Evaluation will not be reduced to an instrumental technique with a clear border to the issue being evaluated – an individual, a program, a project, an organisation. Such definite borders are, as all other borders, not naturally defined – but artificial. The evaluations of the earlier generations took part in organising and reproducing such definite borders by taking them for granted. The evaluations of the fourth generation regard the borders as problematic, this means that the result of the evaluation can be integrated as part of the organisational processes, which take place, whether or not the evaluator recognises them as such (read: "Fourth Generation Evaluation") or not (read: earlier generations of evaluation) .

[7] There are, of course, other criteria of division (read: History documentaion) than the one Guba & Lincoln suggest. (Morgall 1991) indicates, for example, that evaluation, and more specifically, technology assessment has undergone two phases. The first lasted until 1970 with "industry" as the main party. After this came the second phase, where "public concern"dominated. The more technological-economic oriented technology assessment, which characterised the first phase was thus replaced with a technology assessment, which incorporated more relevant parties with the objective, to show the social consequences of the technological development. A similar division is made by Miettinen (1999), when distinguishing between "the traditional or instrumental model" and "the model of participatory or "discoursive" technology assessment".

5.7.2 Towards fifth generation evaluations?

Is there something to add to Guba & Lincolns suggested ”Fourth generation evaluation”? What one would like to add is what the authors have excluded. There is a dynamic entity, which they (and many with them) have not included – the technology and its changing characteristics. Guba & Lincoln (1990) assume implicitly, that the ‘social aspect’ constitutes itself in the interaction between people. The authors do not consider the social aspect as constituted in the interaction between people *and* material entities of various sorts. Thereby, the technology itself and its characteristics are excluded from the organisational processes, which constitutes the social aspect. The traditional border between the technological and the social matters, between technology and organisation, will implicitly be reproduced by the authors.

Through the introduction of the “translation perspective” it is intended to contribute to specifying such borders and to review the perspective in a critical way. This is both relevant and important, considering that the technology assessment, which is performed today also tends to take the boundaries of the technology for granted. By formulating the problem of the technology assessment as an assessment of the “effects of the technology” (read: the “impact” perspective) the evaluator has already externalised the technology and defined it as a given object, which stands outside or above the society and its organisations. The “translation perspective” does not externalise and stabilise the technology as a given object, but regards the technology as part of the social aspect, including society and its organisations.

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Chapter 6: The Economy

by

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Contents of chapter 6:

6.1 INTRODUCTION 97

6.2 ECONOMIC ANALYSES OPPOSED TO BUDGET OR BUSINESS ANALYSES 97

6.3 ECONOMIC ANALYSIS 98

6.3.1 THE PURPOSE OF THE ECONOMIC ANALYSIS 98
6.3.2 TYPES OF ECONOMIC ANALYSIS 98
6.3.3 CHOICE OF ALTERNATIVES 100
6.3.4 PERSPECTIVE OF THE ANALYSIS 101
6.3.5 COSTS 101
6.3.5.1 What is a cost? *101*
6.3.5.2 Types of costs *102*
6.3.5.3 Costing in practice *105*
6.3.6 CONSEQUENCES 108
6.3.6.1 Measure of effectiveness in natural units *108*
6.3.6.2 Quality-adjusted life years *109*
6.3.6.3 Willingness-to-pay *111*
6.3.7 DISCOUNTING 114
6.3.8 MODELLING IN THE ECONOMIC ANALYSIS 114
6.3.9 HANDLING OF UNCERTAINTY IN THE ECONOMIC ANALYSIS 115
6.3.10 WHEN IS A HEALTH TECHNOLOGY THEN COST-EFFECTIVE? 116

6.4 BUDGETARY AND BUSINESS ECONOMIC CONSIDERATIONS 119

LITERATURE USED IN CHAPTER 6 119

6.1 Introduction

There is a vast demand for health care services, and thereby preferences for the use of different health technologies. However, some limits in the form of resource restraints with respect to time, staff, and money do also exist, which is why the supply of health care services does not always correspond to this demand. A rapid technological development in the health care sector also implies a conflict between technological possibilities and economic possibilities. Newhouse (1992) has shown that the use of new technologies made the largest contribution to the growth in the health care costs. These conflicts make the setting of priorities between different health care services, including health technologies, relevant and indispensable. In a free market the demand will be defined by the price of the supplied commodity. However, the market for health care services is not based on this simple price mechanism, which is why priority setting in this type of market becomes a bit more complicated.

Economic analyses can assist the priority-setting process in the health care sector in deciding on the best use of resources. The basis for economic thinking and economic analysis is the concept of *opportunity costs*, which states that *the real cost of a health care programme's implementation is not the number of dollars appearing on the programme's budget, but rather the health outcomes achievable in some other health care programme which have been forgone by committing the resources to the first programme* (Drummond et al. 1997). What is therefore very important is that choices betweenalternatives have to be made (priority-setting) and that these choices have consequences in terms of resource-use. The questions that have to be answered are what should be produced in the health care sector? How should this be produced? And how should the produced health benefit be allocated among the citizens in society? This is the economic problem.

In a health technology assessment (HTA) the purpose of the economic analysis, together with the other relevant questions, e.g. clinical ones, is to provide information to improve decision-making in the health care sector with respect to priority-setting between different health technologies, both emerging, new and existing ones. The overall role of the economic analysis in the HTA study is therefore to provide information about the necessary resource consumption from the use of health technologies compared with the health outcome obtained. In this chapter the methods for the conduct of economic analysis within an HTA are introduced. Where necessary, reference is made to literature sources where further information can be obtained on a specific subject. When performing the analysis, it may be useful to have assistance available for the analysis from a health economist with a background either in research or, alternatively, in health care administration.

6.2 Economic analyses opposed to budget or business analyses

Health technology assessment is made for the purpose of decision-making, both at the clinical level and at the political-administrative level. In the health care sector there are a number of levels of decision-making for the priority-setting of health technologies, e.g. decisions made at the overall societal level, decisions made by the county, or decisions made by a clinical department in the hospital. The broadest perspective is obtained at the societal level.

An HTA ought to include an economic analysis conducted at the societal level, where the economic consequences for society, which means everyone who is directly or indirectly affected by technology, are assessed and included. This will provide information about which health technologies should be preferred and prioritised. If the economic analysis in an HTA is not conducted at the societal level, then there is a risk of that the recommendations put forward based

the HTA study about which technology to choose will not necessarily be the optimal ones for the society.

Besides considering whether a health technology is optimal from the societal level, it is often appropriate in an HTA also to investigate who carries the burden in terms of expenditures and who will benefit from the use of the technology. Is it the government's budget, the counties' budgets, or the municipalities' budgets that are affected, and should this lead to reallocation of resources between these *budgets* to ensure the introduction of the technology, e.g. in Denmark by raising the block grant from the government to the counties? This can be investigated in what we can call a *budget* analysis, which can be conducted as a supplement to the full economic analysis. Finally, it can be of value for the single hospital department, hospital, county or general practice to provide information about the needs for investment and the running costs with respect to a new technology. This information can be obtained by the conduct of a *business analysis*.

It should be pointed out, however, before considering carrying out budget- or business analyses of a new health technology that it is first of all necessary to investigate whether a new health technology is attractive from a societal perspective, and therefore whether or not it should be introduced. The introduction of a new technology can in the simple budget analysis show savings for one specific budget, e.g. the county's, whereas the conduct of an economic analysis may find that there is no benefit at all, and that society is perhaps even worse off having the new technology. Making decisions based exclusively on limited budget analyses may not result in an optimal priority setting in society. The focus for the economic question in a HTA is, therefore, on economic analysis. This is also the focus of the rest of this chapter.

6.3 Economic analysis

6.3.1 The purpose of the economic analysis

An economic analysis is the comparative analysis of alternative courses of action in terms of both their costs and consequences (Drummond et al. 1997). Having a more specific focus on the health care sector, the purpose of the economic analysis is to investigate the relationship between the costs and the consequences of a (new) health technology compared to one or more relevant existing alternatives for screening, diagnosis, treatment or rehabilitation purposes. This will contribute information about whether the technology is cost-effective from a societal perspective.

As opposed to existing clinical study designs, there is no existing (true) standard for the conduct of economic analyses, and for which methodological requirements should be fulfilled in the running of a good analysis. Part of the method is also still under debate or development. Some European countries have introduced or are about to introduce guidelines for economic analyses in the pharmaceutical area (Drummond et al. 1999). In Denmark this is also the case. The Ministry of Health and the Danish Medicines Agency have produced a Danish guideline for economic analyses of pharmaceuticals. Whenever relevant in this chapter, reference is made to these guidelines (Alban et al. 1998).

6.3.2 Types of economic analysis

There exit four types of economic analysis that can be relevant to consider as part of an HTA: cost-minimization analysis, cost-effectiveness analysis, cost-utility analysis and cost-benefit analysis. The identification of various types of costs, and the subsequent measurement and monetary

valuation, is in principle similar across these four types (Drummond et al. 1997). On the other hand, these four types differ in the way the consequences (health outcome) are estimated (see section 6.2.6), as well as in the conclusions and recommendations for decision-making that can be made based on these analyses.

Cost-minimization analysis (CMA)
Using the most simple type of economic analysis – cost-minimization analysis – implies that the consequences (health outcomes) of the use of the compared health technologies are identical. In this type of analysis it is therefore only necessary to estimate the costs.

Cost-effectiveness analysis (CEA)
In the cost-effectiveness analysis it is necessary to identify, measure and value both costs and consequences of the use of the compared health technologies. In this type of analysis the consequences are measured in natural units, e.g. life years gained (see e.g. Gyrd-Hansen et al. 1998). Apart from the cost-minimization analysis, the cost-effectiveness analysis is the most limited type of analysis used in decision-making. From this analysis it is only possible to conclude which of the compared technologies are cost-effective to obtain a specified goal and how great this cost-effectiveness is (Drummond et al. 1997).

Cost-utility analysis (CUA)
The cost-utility analysis differs from the cost-effectiveness analysis in the way the consequences are measured and valued as utilities in the form of quality-adjusted life-years (QALY). Besides a gain in life years (reduction in mortality) the quality of these gained life years, and therefore the morbidity, will also be important, especially in the case of chronic diseases. In the cost-utility analysis the gained life years are quality-adjusted with this health-related quality of life. It will be relevant to choose the cost-utility analysis in situations where the health-related quality of life is important for the treatment and its outcome. Furthermore, it will be relevant to consider cost-utility analysis, when there is a desire for an overall measure of effectiveness enabling comparisons to be made accross the health care sector.
Cost-benefit analysis (CBA)
Finally, as the broadest type of economic analysis, the cost-benefit analysis measures and values the consequences in monetary units. This could be done by asking about the willingness-to-pay for a specific treatment and treating the response as an expression of the preferences for, and the value of, the treatment. The clear advantage of this analysis is that the costs and consequences are now both measured in monetary units (e.g. Danish kroner - DKK), from which the net benefit can immediately be calculated. Furthermore, in a cost-benefit analysis it is possible to conclude overall whether the technology is wanted, i.e. are the benefits higher than the costs, and also to include issues about allocation (to whom shall the technology be offered?).
Factors like the disease area, the compared technologies, the measurement and valuation of the consequences, as well as the use to which the economic analysis will be put, are important in decidinghave in each case importance for which type of economic analysis should be chosen in any given case. Table 6.1 sums up, for each type of economic analysis, when one type should be chosen over another.

Table 6.1: Choice of type of economic analysis

Type of economic analysis	When should the specific type of analysis be chosen?
Cost-minimization analysis ⇓	1. When the compared technologies are equally effective - then it is only necessary to collect data about costs
Cost-effectiveness analysis ⇓	1. When the effectiveness of the compared technologies are different - i.e. the difference in costs have to be weighted against the difference in effectiveness 2. When a technology dominates the other technologies* - i.e. is more effective and cheaper than the compared technology 3. When activities with the same aim and measure of effectiveness are compared
Cost-utility analysis ⇓	1. When health-related quality of life is an important health outcome 2. When activities across specialities or departments in the health care sector have to be compared
Cost-benefit analysis	1. When non-health effects are also important - e.g. the treatment process itself, utility of information. 2. When only one technology is assessed (net-benefit) 3. When there is a wish that individual lives are valued in monetary units 4. When activities across society have to be compared

* For further information about dominance see section 6.2.10.

There is another type of analysis - *cost of illness analysis (COI)* – that aims at describing the costs of a disease to society, e.g. the back pain problem in Denmark (a *positive use*). However, as the COI-analysis only calculates the total costs for society of one disease, and does not compare alternative technologies, , it cannot be regarded as an economic analysis (Drummond et al. 1997). The COI-analysis does not provide any information about the opportunity costs from the use of a technology, and it can therefore not be used as part of an HTA-study, where the starting point is in a *normative use* in the setting of priorities.

6.3.3 Choice of alternatives

To be able to perform an economic analysis of the technology in question, there has to be at least one alternative technology to compare with, i.e. as a comparative analysis. The cost-benefit analysis can, however, be conducted for only one technology. An economic analysis aims to answer the questions as to whether a new health technology is cost-effective compared to current practice which it is supposed to replace, and whether the technology is cost-effective in general compared to other optimally cost-effective technologies (CCOHTA 1997). To answer these questions the new health technology has ideally to be compared with both current practice and all other relevant alternatives (including a zero-option or placebo alternative), including the cheapest alternative that is more effective than the zero- or placebo alternative. To be relevant to decision-making the chosen alternative for the economic analysis should at least represent the current health technology or practice which the new health technology is expected to replace. If the placebo alternative is the only alternative for comparison, it may threaten the ability to generalise the conclusions of the study (external validity). This may result in a lack of relevance of the economic analysis for decision-making, because no patients in normal practice will be left untreated, as is the case with placebo (Drummond et al. 1997). The placebo-alternative can, however, be used when the compared technology is additional to the existing therapy, such as medical prophylaxis together with shock-wave lithotripter treatment of kidney stones. The alternatives and the new health technology can belong to different areas of medicine; e.g. medical treatment might, if relevant, be compared with

surgery. However, the chosen alternatives for the economic analysis have to be described and their choice justified. Finally it should be decided whether other relevant, but not analysed, alternatives should be included in the discussion section.

6.3.4 Perspective of the analysis

An economic analysis can be performed from different perspectives. The choice of perspective is important, because it determines the width of the economic analysis, i.e. which costs and consequences are to be included, and what use can be made of the analysis and its result in decision making. The most comprehensive perspective is the societal one, where all relevant costs and consequences of the considered health technologies have to be identified, measured and valued, no matter whom these costs and consequences fall on (Drummond et al. 1997). With this perspective it is of equal importance to include costs for the patient as well as costs for the hospital. Often, however, economic analyses are conducted from more narrow perspectives, such as health care sector perspectives, hospital perspectives or patient perspectives. The chosen perspective influences the extent of the costs which have to be estimated (see table 6.2 in the following section). Furthermore, the analyses can, in a budget analysis, be conducted based on different budgets or decision-maker perspectives - for example the government or the national health insurance.

In general, it is recommended that the economic analysis be conducted from the broadest possible perspective, and that an analysis having a budget perspective is not the only analysis performed. If an economic analysis is to be used for priority-setting at the societal level, the analysis has to be conducted from the societal perspective. Otherwise, there is a risk that the priority setting will not be optimal. This is the situation where a technology is shown to be cost-effective from more narrow perspectives, but not cost-effective from a societal perspective. Both Danish and Canadian guidelines for economic analysis recommend economic analyses conducted from a societal perspective (Alban et al. 1998, CCOHTA 1997). However, independently of which perspective is chosen for the specific economic analysis, it has to be clear-cut in the analysis which costs and consequences are included. These costs ought to correspond with the chosen perspective. This will in the end ensure that economic analyses are comparable.

6.3.5 Costs

6.3.5.1 What is a cost?

To carry out an activity in the health care sector, e.g. a treatment, and thereby obtain a health outcome, a number resource inputs are required. By definition a cost is understood as a consumption of resources (the value of the resources consumed). The consumption of resources with respect to an activity in the health care sector concerns both use of *health resources* (e.g. manpower, drugs, equipment), *non-health resources* (e.g. the patients travel to treatment), *the informal caregivers time* (e.g. use of time for (unpaid) caregiving provided by family and friends), *the patient's own use of time* in connection with the activity, as well as the *lost production* as a result of disease and dead (Luce et al. 1996, Drummond et al. 1997). If relevant for the considered technologies, all these resource-uses have to be measured in an economic analysis with a societal perspective. In analyses with narrower perspectives fewer of these resource-uses may be identified. For example will an analysis with a hospital perspective only focus upon resource-use in the hospital? The perspective chosen for the economic analysis is, therefore, crucial with respect to which resource-uses, and thereby costs, need to be identified and measured.

When resources are used for the treatment of one patient, the same resources are not available for other patients and for other uses in society (Dranove 1995). In order to be able to assess the benefit of introducing a new health technology one has to judge what has to be given up at the same time. This cost is expressed by the term *opportunity cost.* By the opportunity cost is understood the (lost) health gains that could have been achieved from an alternative technology, which, however, cannot be introduced or retained, because the resources, e.g. manpower, are used on the new technology (Drummond et al. 1997).

When one performs an economic analysis, it is important to distinguish between what is a cost and what is not. A cost is the use of a resource that would otherwise have been available for alternative purposes. Everything which does not imply use of resources is not a cost and should, therefore, not be included in an economic analysis.

Transfer payments, by which is understood a reallocation of money between groups in society, is not a real cost to society (Luce et al. 1996). These only involve a reallocation, for example through taxation, but do not involve any opportunity cost and use of resources. Transfer payments should thus not be included in an economic analysis. Typical examples of transfer payments are sickness benefit, pensions, value-added tax, etc. It can, however, be argued that the administration of transfer payments should be included in the analysis, although these often do not have any importance (Drummond et al. 1997). On the other hand it may be relevant in the budget analysis to include transfer payments to try to determine which budgets win and which lose from a possible change in, for example, sickness benefit.

Neither are expenditures the same as a cost in the terminology of the economic analysis, because they are usually related to a budget and are not expressing the opportunity cost of an activity. A typical example is charges. The charge for an inpatient-day in a hospital department is an average estimate that is calculated based on information about the department's annual expenditures and the total number of inpatient days in the department (Alban et al. 1995). Such an average charge does not usually reflect the actual resource use related to different activities and types of diseases in the department, which means that it can seldom be considered as a real cost. An average charge for an inpatient day ignores the fact that the costs usually vary during a stay in hospital, where the last days often are the cheapest (Brooks 1996). When introducing new technologies, conclusions about cost savings due to saved inpatient days (the last days) - for example from a shift to day-surgery - are therefore often overestimated if the inpatient days are only based on average charges instead of an actual measurement of costs. One hastherefore to be careful about equating expenditures with costs, and about the use of charges as an approximation of costs in economic analyses.

6.3.5.2 Types of costs

Depending on the chosen perspective all relevant costs have to be included in the economic analysis, although with the focus upon changes in resource consumption between the compared technologies. An economic evaluation includes both the direct costs (health care sector, other sectors, and patient and family), as well as the lost production in society (Drummond et al. 1997). Table 6.2 shows these different types of costs for three selected perspectives.

Table 6.2: Types of costs in an economic analysis

Perspectives			Types of costs	Examples
Societal Perspective	Health care sector	Hospital	Direct costs: -in the hospital	*Health care staff, medicine, tests, capital costs (equipment and buildings), inpatient stay (hotel), outpatient visits, overhead costs (e.g. food, light, heat), (research and education)*
			Direct costs: -in the primary HC-sector	*Visits to the general practitioner, private specialist, physiotherapist, etc., prescription drugs (the share paid by the public health care insurance), screening programmes*
			Direct costs: -in other sectors	*Home care and nursing care at home, social arrangements (financial support for medicine from the municipality, aids and appliances)*
			Direct costs: -for the patient and family	*User payment (medicine, dentist), cost for travelling, time costs due to patients time used for the treatment, family or friends (unpaid) use of time of the patient*
			Lost production in society	*The patient's temporary absence from work due to illness, reduced working capacity due to illness and disablement, or lost production due to an early death*
			Future health care costs	*Future unrelated health care costs caused by the cure of the patient with the present treatment*

Lost production is a cost for society due to morbidity (lost or reduced working capacity due to disease and disability) and mortality (lost production due to an early death), as shown in Table 6.2. Luce et al. (1996) argues, however, that mortality is already included in the measurement of effectiveness, for example in the calculation of life years or QALYs. The lost production can be measured either by *the human capital method* or *the friction cost method*. The human capital method estimates the value today (the present value) of all future potential earnings that are lost due to absence from work (Johannesson et al. 1997). It is normally measured by the gross income. The friction costs method, on the other hand, assumes that there will be unemployment in society in the long run, and that a sick person thereby can be replaced by another person after a short friction period (Koopmanschap et al. 1992). This means that the loss will only be the (lost) working costs of the sick person during the friction period. Supporters of the human capital method have emphasised that this method is the theoretically correct one, whereas the friction cost method is ascribed the highest realism and results in considerably lower estimates. Because of the disagreement and the uncertainty whether lost production should be included in economic analyses at all, the Danish guidelines recommends that *lost production is reported separately and that valuation be made only in situations, where it is judged to be relevant* (Alban et al. 1998). Finally, it is repeated that the concept of lost production should not be confused with a transfer payment like sickness benefit. For further information the reader is referred to Drummond et al. (1997), page103-107.

Inclusion of future unrelated health care costs in life years obtained due to the success of the treatment is a contentious subject (Luce et al. 1997). If lost production is included in the analysis, it can also be argued that future costs, if of any importance, should be included, or at least that the impact of future costs upon the result is assessed in sensitivity analyses.

Intangible costs such as anxiety, nervousness and the like, for example those caused by participation in a screening programme, should not be included on the cost-side in an economic evaluation, but can alternatively enter the effect-side by the estimation of willingness-to-pay or QALYs (Drummond et al. 1997). Although intangible costs can be included on the effect-side, they do not deprive resources from alternative consumption (opportunity cost), which is why they cannot be considered as costs.

Average costs or marginal costs

When performing the economic analysis it is important that one decides how the costs should be calculated with respect to the compared health technologies. The difference between calculation of average costs and marginal costs is that the fixed costs will be included in the average costs, as seen in table 6.3. However, when a programme is extended, e.g. includes less symptomatic patients, the costs will likely increase more than proportionally to the extension, while the effects (health gains) will increase less than proportionally to the extension (Alban et al., 1995). With the focus in the economic analysis upon changes in activity it will be of interest to answer the question, *what would be the costs (and consequences) of having a little more or a little less?* (Drummond et al., 1997). In this case a calculation of the marginal costs – *the extra costs of producing one extra unit of output* (see Table 6.3) – will be the relevant for decision-making.

Table 6.3: Different cost concepts

Total Costs	*The costs of producing a particular quantity of output q, TC = FC + VC*
Fixed Costs	*The costs which accrue to the production regardless of its level (FC), e.g. investments.*
Variable Costs	*The costs that vary with the level of output (VC), e.g. running costs.*
Average Costs	*The costs per unit of output produced q, AC = TC / q.* *Can also be calculated as the average variable costs,* $AC_{var} = VC / q$.
Marginal Costs	*The extra costs of producing one extra unit of output q, MC* *MC = (TC for q + 1 units) - (TC for q units) = _TC / _q.*
Incremental Costs	*The difference in costs between two technologies (difference costs),* IC_{A-B}

(Drummond et al. 1997)

An example presented by Gyrd-Hansen et al. (1998) concerning the cost-effectiveness of screening programmes for colorectal cancer illustrate the difference between average costs and marginal costs. Let us assume that it is present practice to screen people aged between 65-74 years every second year to find those who are predisposed for colorectal cancer. Doing this will result in a gain of 974 life years, compared with no screening, at a cost of 16.5 million DKK. The average cost per life year gained is then DKK 17,000. If the programme is extended to screen people aged 50-74 years, and to do it every year, the gain will be 3,081 life years, or an extra 2,107 life years, for a total cost of 80 million DKK. This extension of the screening programme seems at first to be attractive, because the average cost per life year only increases to 26,000 DKK. However, only considering the average will not provide the relevant input for decision-making regarding an extension of the screening programme. Instead one needs information about the extra cost that is required to obtain the extra benefit, i.e. the extra life years, - the marginal costs. This extra cost per extra life year gained from choosing to screen people of the age between 50-74 years every year can be calculated to 42,500 DKK by comparing with the second most effective screening programme (at the age between 55-74 years, screening every year). This extra cost per extra life year gained is thereby more than twice as high compared to the cost per life year of the screening programme assumed to exist today (at the age between 65-74 years, screening every second year). Or expressed in another way, one can decide to spend an extra 64 million DKK on a screening programme for colorectal cancer to gain extra 2,107 life years. With the focus then upon changes in activity, considering the margin and marginal costs will therefore often be relevant for economic analyses.

The time horizon of the decision problem also influences whether average costs or marginal cost should be calculated. In the long term it will be possible to make changes in "production", i.e. to open or close hospital departments, so that fixed costs become variable costs (Andersen et al. 1992). In the long run the marginal costs will then be closer to the average costs. Conversely, the consequence of this is that some (saved) resources, such as saved inpatient days, cannot in reality be

realised in the short run, because beds in a hospital department cannot be removed from one day to the next(Brooks 1996). Ideally, it is therefore important that the time horizon of the decision problem be considered before it is decided which types of costs need to be estimated.

Finally, incremental costs can be calculated. Incremental costs are defined as the difference between two programmes (the difference costs), as stated in Table 6.3. These are often interpreted synonymously with marginal costs, as the extra cost of having an extra unit of output produced.

6.3.5.3 Costing in practice

The strategy for costing in practice can be either *deterministic* or *stochastic* (Johnston et al. 1999). In the deterministic costing approach the resource-use data are non-patient specific, which implies that the resource-use in practice is assumed to be of equal size for all patients in the analysis. This was the typical strategy for costing chosen in earlier economic analyses. However, economic evaluations are increasingly carried out prospectively within the context of on-going clinical trials, which makes it possible to collect stochastic resource-use data (De Graeve et al. 1996). The advantage of a stochastic strategy is that patient-specific data is obtained for each patient and that statistical analysis of resource-use and costs can be performed, as is the case in the clinical study. The Danish guidelines also recommend the use of prospective designs for the collection of data about resource-use (Alban et al. 1998).

Whether a deterministic strategy or a stochastic strategy has been chosen, the costing procedure can be divided in three phases: *identification, measurement* and *valuation.* First of all the relevant resource-use has to be identified, and this resource-use has next to be measured. Because a cost is defined as a *quantity* multiplied by a *price*, prices in the form of unit costs then have to be valued and attributed to the measured resource-use. The nurse's hourly wage rate could for example be used as the unit cost for one hour of nursing care.

Identification of resource-use

The first step in the costing procedure will be to identify the relevant resource-use involved in the use of the health technologies to be compared. This should, however, fit with the chosen perspective for the analysis. Ideally it is only the resource-use that is expected to vary between the compared technologies or between the patients (marginal analysis), or resource-use data that are expensive or impossible to collect retrospectively after the clinical trial (e.g. from patient journals), that needs to be collected prospectively (Poulsen 1999). This is also recommended to minimize the necessary data collection within the clinical trial (Johnston et al. 1999). Before the start of the clinical trial it has to be decided which relevant economic data needs to be collected.. Methods such as review of earlier studies in the area, pilot studies, modelling exercises or expert advice can help to identify the relevant resource-use (Johnston et al. 1999). However, resource-use only caused by the clinical study itself, for example to randomise the patients, should not be measured, as it is not a cost in daily clinical practice. This is called a protocol-driven cost (Drummond et al. 1997).

Measurement of resource-use

In a prospective economic analysis the patient-specific resource-use is often measured within the clinical study. The typical units for measuring resource-use are physical units like time consumption of health professionals, inpatient stay, medicine (type of drug and dosage levels), number of tests,

number of operations, number of visits to a general practitioner and duration, days of absence from work, etc. The importance of each resource-use for the specific activity determines how detailed the collected data has to be. There are a number of different methods or data sources that can be used to collect and measure patient-specific resource-use, as shown in the upper part of Table 6.4. Additionally, the reader is referred to Johnston et al. (1999).

Table 6.4: Methods and sources for the collection and measurement of resource-use

Patient-specific (stochastic) data			
Sources	**P/R**	**Description**	**Typical data** (examples)
Case Record Forms	P	Filling in formulary. A supplement about economic data to the clinical CRF. Filled in prospectively by the clinician or the monitor person. The most detailed data collection.	Detailed measurement of all resource-uses in a hospital or outpatient setting within a clinical study. E.g. number of procedures, time, use of materials, hospitalizations.
Cost diaries	P	Concerns data on non-hospital activities used in the study. Can be used in the hospital too. Filled in prospectively by patients or by staff.	Primary sector (e.g. GP visits), the patients own expenditures, travelling expenditures and time, work absence.
Questionnaires (or interview)	R	Filled in by patient or staff personally for the whole sequence or part of it. Or interview.	Primary sector, patients own expenditures, travel expenditures and time, work absence.
Registers and data files	R	1) National Patient Register and administrative files, 2) National Health Insurance Board, 3) Municipalities data files.	1) Time of inpatient stay, diagnosis- and operation codes, 2) primary sector data, 3) social services.
Patient records	R	Review of patient record files.	Inpatient stay, procedures, outpatient visits.
Non-patient-specific (deterministic) data			
Sources	**P/R**	**Description**	**Typical data** (examples)
Pilot studies	P/R	On a representative sub-sample of the study patients. Time studies, counting of materials.	Work input (time) from health professionals, medicine & material, length of inpatient stay
Clinical databases	R	Existing clinical databases in the disease area.	Length of inpatient stays, number of treated patients, rates of complications.
Earlier studies	R	Resource-use data from published studies.	Number of procedures, tests, unit costs.
Expert advice	--	Experts' assessment of the resource-use in the specific case. E.g. in the form of expert panels.	In principle all data. However, the validity and the reliability have to be considered.

Note: P refers to a prospective method and R refers to a retrospective method.

In the specific economic analysis it is often a good idea to combine some of the methods or sources shown in Table 6.4 above, because the burden for the patient and the staff caused by the collection of data also has to be considered. However, the advantage of the primary data collection methods, such as Case Record Forms (CRF) and cost diaries, is that they can be included directly in the data collection going on in the clinical study and thereby be collected simultaneously (Mauskopf et al. 1996, Poulsen 1999). Cost diaries and questionnaires or interviews might be especially relevant as a supplement to the CRF for the measurement of resource-use in the primary sector (e.g. GP visits) as well as the patient's own use of resources for health care services and goods (e.g. over-the-counter drugs). Besides a prospective collection of data, existing sources like registers and patient record files can also be used (Johnston et al. 1999). Among the relevant registers in Denmark are the hospitals' administrative files and the National Patient Register, both of which can provide information about hospital activities. With respect to the provision of data from the primary sector the National Health Insurance Board might be a possibility, while it can be relevant to use data from the municipalities if data is needed on social services like home care. In general it should be noted, however, that these files or registers are often not made for the purpose of collecting resource-use data and they may not be detailed enough. As seen in Table 6.4, a number of sources for the collection of deterministic and non-patient-specific data also exist, e.g. pilot studies and clinical databases. Some clinical databases might even include patient-specific data. Existing sources of

patient-specific data (administrative files, records, etc.), as well as the sources for the collection of deterministic resource-use data can, furthermore, be used in economic analyses having a retrospective design.

Valuation of unit costs

Because a cost is defined by a quantity multiplied by a price, the third phase in the costing process is to assign prices in the form of unit costs. The price of a resource-use should ideally be that of its opportunity cost (Boardman et al. 1996). This means that the price, and therefore the unit cost, for a limited resource like radiologists have to be valued higher than their salary, because the value of their alternative use is high when the supply of radiologists cannot meet the demand. Furthermore, due to the lack of a normal market for health care services, market prices equivalent to the opportunity cost do not exist - only charges (e.g. for an inpatient day). However, opportunity costs are difficult to measure in practice, which is why market prices are used whenever possible (e.g. drug prices). Some practical examples of valuation of unit costs are shown. below in Table 6.5 s

Table 6.5: Examples of unit costs in practice for different resource inputs

Type of resource	**Suggestion for a valuation in practice**
Labour	The average wage rate for the specific group of health professionals (adjusted for seniority)
Medicine	Hospital: the cost prices. Primary sector: the sale prices in the pharmacy as well as the user payment share and the subsidies from the health insurance board (societal perspective)
Materials	Hospital: the cost prices. Primary sector: possible user payment (plus health insurance board)
Capital cost (equipment, buildings)	Operating costs + depreciation. The depreciation per year for equipment is calculated using the investment price, depreciation period and discount rate (see Drummond et al., 1997, pp. 88-95)
Overhead-activities	The total costs in the hospital for overhead activities (e.g. cleaning, food, etc.) allocated with the "*step-down*" method to a specific activity in a department, for example in terms of overhead per inpatient day (additionally, see Drummond et a., 1997, pp. 74-81).
Inpatient stay (hotel)	The daily hotel cost in e.g. a patient hotel. Alternatively allocated by the "*step-down*" method.
Lost production and patient use of time	Wage rates adjusted by age and gender (average). Perhaps divided into different sectors of industry. The CCOHTA guidelines (1997) recommends: *average wage rates in the industry.*

These unit costs will vary with factors like geography, type of institution, category of patients, etc., which is why caution is recommended when considering the use of costs from foreign countries in Danish analyses, because of differences in health care systems, practices and labour market conditions. A systematic literature review of economic analyses reported in the international literature will, therefore, never be a satisfactory way of dealing with and answering the economic questions in an HTA. The primary use of a literature review for the economic part of the HTA is, therefore, to get inspiration for the design and data collection in the specific economic analysis (see chapter 2 about literature searching and assessment of literature). Transfer payments like taxes and duties (value added tax) also disturb the costing. Depending on the perspective of the analysis, it is often chosen in practice not to include value added tax in the measurement and valuation of the costs for medicine (a transfer payment argument). However, disagreement exists on this issue. When the unit costs have been valued, every patient's resource-use can then be multiplied by the corresponding unit costs. After this, the total costs can be revealed by summing up across all patients, and average total costs for each study arm in the trial can then be calculated (Johnston et al. 1999).

Overall the strategies for costing in practice can be divided in two approaches - *micro-costing* and *macro-costing approaches*, which vary in level of detail and precision (Luce et al. 1996, CCOHTA 1996). The micro-costing approach is the direct measurement (e.g. time studies) and estimation of unit costs for every single resource-input in the treatment of a specific type of patient. This approach is used for costs, and thereby resource-use, that are both of central importance for the analysis. On the other hand, by using the more aggregated macro-costing approaches the costs of a treatment can be arrived at by ascribing a national average unit for large units of inputs and outputs, for example DRG-charges or inpatient day charges. If the costs are only expected to have a limited variation, this approach can be used, because the issue of precision is then less important. However, both approaches are often used in the same analysis. The choice of strategy has, nevertheless, to be decided in the initial phase of the design of the HTA study and the economic analysis.

6.3.6 Consequences

As a comparative analysis the other part of an economic analysis, besides the costing exercise, is to estimate the consequences or benefits from the use of the compared health technologies. The only exception is the cost-minimization analysis, where only costs are of interest.

The expected outcome of the use of a health technology is that the health status of the patient will be improved. This can be measured in a *measure of effectiveness in natural units*, or be valued in terms of *quality-adjusted life years (QALY)* or *willingness-to-pay* (Drummond et al. 1997). Another benefit of a technology's use can be in the value of the information it yields, e.g. from a screening programme. This type of benefit can be incorporated in QALYs or willingness-to-pay. Finally, the use of a health technology may result in cost savings, i.e. costs that are no longer used on alternative health technologies, which can therefore be withdrawn (a benefit in the cost-benefit analysis). With respect to cost savings one should remember the time horizon for the decision problem and whether these resources are in reality freed up (see section 6.2.5). There are, in general, three ways that (health) consequences can be estimated in economic analyses – either as measures of effectiveness in natural units, QALYs or willingness-to-pay, depending on the type of analysis chosen. These three general ways are explained further in the following sections.

6.3.6.1 Measure of effectiveness in natural units

According to section 6.2.2 the effectiveness of health technologies is in the cost-effectiveness analysis measured in natural units and is presented as costs per unit of effectiveness. This is relevant, when the compared technologies have the same objective and measure of effectiveness, and when health-related quality of life is not important.

Identification
To be able to identify the relevant measure of effectiveness it has to be clarified, what the objectives of the compared health technologies and the economic analysis are (Drummond et al. 1997). If the objective is clear, then there is a clear dimension, whereby the effectiveness can be measured. If there, however, are many objectives that the technologies fulfil to the same extent, then there is no difference in effectiveness between the technologies and a cost-minimization analysis is sufficient. Initially it can, however, be difficult to know, when there is no difference in the effectiveness. Therefore, the only possibility is often, especially in prospective studies, to start by designing a cost-effectiveness analysis, which then possibly later, after collecting the data on effectiveness, may end up being a cost-minimization analysis.

The measure of effectiveness can be divided into two main groups – intermediate measures and final measures. *Intermediate measures of effectiveness* concerns changes in a health-related variable from the use of a health technology (Alban et al. 1998). Intermediate measures of effectiveness can be further divided into surrogate measures, e.g. reduction in mmHG blood pressure, per cent serum cholesterol; or into (avoided) events, e.g. number of correct diagnosed patients, number of heart attacks. The intermediate measures of effectiveness are close to or equal to the output measures chosen in the clinical studies, which is why they are often relevant for clinical decision-making. On the other hand are the *final measures of effectiveness* indicators of survival that reflect the likelihood or frequency of survival a in defined time interval (Alban et al. 1998). Examples of final measures of effectiveness are gained life years or lives saved. Final measures of effectiveness are related to the end result of using the technology and not just a clinical output, which is why they are more relevant for the patient and in priority setting in general (Drummond et al. 1997). Using final measures of effectiveness makes comparison of different types of health technologies possible. Finally, the effectiveness can be covered by a health status measure or health status profile, if only health status, and not survival, is affected by the technology, which was the topic of the last part of chapter 3. Health status measures can, hvowever, not be directly included as a measure of effectiveness in the cost-effectiveness analysis.

Measurement

A source for data about the effectiveness of health technologies is the medical literature, e.g. in the form of systematic reviews or meta-analyses (see chapter 2 and 3) or clinical databases. The effectiveness can, however, also be measured directly, if the cost-effectiveness analysis is conducted prospectively alongside a clinical trial. The measure of effectiveness is then often the same as the clinical output measures in the clinical trial. Whether literature reviews, meta-analyses or primary studies are chosen as the source for effectiveness data, the same requirements to the data as in the clinical studies apply (see chapter 3). The design of the clinical studies needs to have a high quality, be randomised and controlled and possess the highest possible internal validity. At the same time, however, it is also required that the effectiveness data in the economic analysis is relevant for decision-making (Drummond et al. 1997). Randomised controlled clinical trials provide data on the efficacy of the technology in the study sample. However, with the focus upon decision-making, on data about the effectiveness in daily practice will be needed to increase the external validity and generalisability of the economic analysis. Then in the choice of data on effectiveness a trade-off between internal and external validity exists. It will often be a good idea to choose the best clinical design and effectiveness data, and, if necessary, then later analyse the effectiveness in daily practice using a model. If data on intermediate measures of effectiveness is collected, then one should be cautious with respect to extrapolate directly from these intermediate measures to final measures, unless a clear biological or medical reason exists. A short study period may make it impossible to collect data about gained life years in the clinical study directly. Extrapolation with data from survival tables or analyses, as well as Cox proportional regression analysis (proportional hazard) are possibilities in these situations.

6.3.6.2 Quality-adjusted life years

Health care services will often encompass more dimensions than can be included, if a measure of effectiveness in natural units is chosen. If the use of the health technology is expected to imply changes in the health-related quality of life of the patients, then a cost-utility analysis should be undertaken. In the cost-utility analysis the consequences are measured and valued in the utility measure *quality-adjusted life-years* (QALY). QALYs take into account that health care services and

health technologies for one thing can affect the duration of life years (mortality) and for another can affect the quality of life in these life years (morbidity) among the patients/users who the service is aimed at (Christiansen et al. 1995). Compared to the measures in natural units, the QALY approach is often more useful in decision-making, and thereby also in HTA, because it is a completely general approach that can be applied to any population, any disease, any intervention, and can be used to compare across quite diverse programs in the health care sector (CCOHTA 1997).

Quality-adjusted life-years (QALY) is a result of a quality adjustment of each gained life year from an intervention. The quality adjustment is made with a QALY-weight that reflects the utility in a health state in the form of health-related quality of life during the time considered. The QALY-weight can have values between 0 and 1. The one extremity with a QALY-weight of zero is the worst possible health state, where the patient is dead, while the other extremity, with a QALY-weight of one, indicates that the patient is in a state of perfect health (healthy). Between these extremities, an infinite number of health states reflecting different degrees of sickness and handicap appears. Health states then would have a QALY-weight higher than zero, but lower than one. One can think about the utility measure 15D, which covers more than 30 billions of different health states, as described earlier in chapter 4b. The QALY-weights have to be based upon preferences and assumes rationality, i.e. that a person always will prefer a condition of perfect health with the highest utility (1) instead of conditions with disease, which again is preferred instead of the condition dead (0). A better health state will therefore have a higher QALY-weight compared to an inferior state. However, it is a fundamental value judgement in the QALY-measure that a QALY has the same weight, no matter who receives it. Furthermore, the QALY-weights need to be measured on an interval scale, so the difference in QALYs between two health states can be measured as the distance between their corresponding QALY-weights.

Measurement

To be able to calculate the QALYs gained using one health technology compared with another the number of QALYs have to be measured both before the intervention and after the intervention. As an example, having home dialysis might result in a slightly higher health-related quality of life with a QALY weight of 0.7 compared with a QALY-weight of 0.6 having dialysis in the hospital. Hospital dialysis, however, is more effective compared with home dialysis, because the gain in life years is a bit higher, e.g. 9 years instead of 8 years having home dialysis. The number of QALYs obtained having home dialysis can, therefore, be estimated to 8*0.7=5.6 compared with 9*0.6=5.4 having hospital dialysis. This means that every patient can gain of 0.2 QALYs having home dialysis. All QALYs gained from a health technology can be calculated by aggregating the QALYs gained for every single patient. Finally, in a cost-utility analysis the gained QALYs from home dialysis has to be compared with the difference in costs between home and hospital dialysis.

QALY weights are measured by asking the relevant individuals (a group of patients or the population), which consequences or states they prefer and how much, thereby reflecting the value people place on different health outcomes (CCOHTA 1997). This measurement can either be direct or indirect. In a direct measurement of QALYs the preferences for different health states is measured within an on-going clinical trial. Usually one of three methods are used to reveal these preferences: *the standard gamble method, the time trade-off method or the rating scale method* (see Torrance (1986) and Drummond et al. (1997) for further about these methods). However, today this direct measurement of QALY weights is seldom done. Instead, indirectly measured QALY weights for different health states are used. These QALY weights are obtained indirectly from the use of existing multi-dimensional utility instruments, where QALY weights have been elicited from a sample of the general population. The EQ-5D and the 15D instruments, both described in chapter 4,

are examples of multi-dimensional utility instruments that can be chosen for the measurement of QALYs. Then the only thing that has to be remembered is that the patients at different times in the clinical trial (e.g. before and after the intervention) have to fill in the chosen instrument concerning their health state and the included dimensions. After this is done, the instrument's preference score (QALY weights) and a score algorithm for each dimension can be used, whereby the number of QALYs gained from a health technology can be calculated. The chosen instrument should fit the purpose of the study and its target group, as well as the requirements of the instrument and the measurement, as previously described in chapter 4b.

The advantages of measuring quality-adjusted life-years in an economic analysis, and thereby in an HTA, are that more than one dimension can be included in the measurement of the effectiveness of a given intervention, and that QALYs is a more global measure than the measure of effectiveness in natural units, as well as QALYs are based upon preferences. Being a more global measure, QALYs makes now can be compared and prioritised it possible that different areas and specialities within the health care sector, and thereby different health technologies. This has been done in the QALY-League Tables that compare different health technologies and programmes with respect to their cost per QALYs gained (Drummond et al. 1997). It has also been suggested what a fair cost per QALY is for the society in the choice among different health technologies. Laupacis et al. (1992) have, for example, suggested that adoption of new technologies up to a maximum limit of 20,000 CAD $ per QALYs gained is unproblematic. However, one should be careful with the direct and uncritical use of such simple criteria for decision-making as these, because QALYs in the different studies may be estimated by the use of different methods, and because other criteria than economic also are important for decision-making.

The use of QALYs has also been critized. One typical point of criticism is that QALYs favour younger patients compared to older patients (ageism bias). However, the answer to this type of criticism is that both existing life years and health status declines with age, which is the only thing that the QALY-measure is trying to capture. In the calculation of QALYs, a small gain for many people is just as preferred as a large gain for a few people, as long as the total number of QALYs gained is the same. This assumption has also been critized for QALYs not taking the severity of the disease into account. Finally, the QALY approach has been critised for not measuring quality of life, which cannot be measured on a scale from 0 to 1. This is true, but is not at all the purpose of the QALY-measure. QALYs measure the patients' preferences for different health states, and are thereby a measure of the patients' health-related quality of life (health status) resulting from interventions in the health care sector, and are not trying to measure quality of life in general. Additional description and discussion of the QALY measure can be found in Poulsen (1994) and in chapter 6 in Drummond et al. (1997).

6.3.6.3 Willingness-to-pay

Often "saved costs" from a treatment are used as a measure of benefit in a cost-benefit analysis. This is reasonable, if the saved costs express the substantial part of the benefit of the treatment. However, in many cases the total benefit is more than just the saved costs. An example of this is a cost-benefit analysis of nutritional support for hospitalised patients (Levnedmiddelstyrelsen 1997). In this analysis the benefits from providing nutritional support to the patients is only calculated as the saved inpatient days in hospital, and thereby saved costs, due to the patients expected earlier discharge. Such a simple analysis has, however, some obvious limitations. First, it is not for certain that the saved costs are realised at all (see section 6.2.5.1) and second, saved costs are a very narrow measure for the benefit that does not, for example, take the patients experience of the process and their preferences for a faster recovery into consideration. Overall, in many cases there is no relation at all between saved treatment costs and the individual consumer or patients valuation of the benefit from a programme, as for example nutritional support or prevention of traffic accidents (Kidholm

1995). Valuation of benefits is, therefore, often more than just the costs that potentially can be saved.

Instead of saved costs, the individuals or patients valuation and preferences for a treatment can be obtained by investigating, what each of them is willing to pay for the treatment, i.e. willingness-to-pay (WTP). In a normally functioning market the value of a good is equal to, what the consumer is willing to pay and is given by the market price. However, in health care markets, e.g. the Danish one, this market does not exist, among other things because most of the health care services are tax-financed. If people's value (benefit) in monetary terms regarding the use of a health technology has to be revealed, alternative methods has to be used, because the value cannot be found in the market.

A group of willingness-to-pay methods to reveal individuals' willingness-to-pay use observation of behaviour to identify the value that the individuals indirectly attribute to a good, with the purpose of revealing their preferences (e.g. the hedonic pricing method or the travel cost method). These methods have, however, almost never been used for studies in the health care area.

Another group of willingness-to-pay methods (contingent valuation and conjoint analysis) is survey methods, where the individuals are asked directly about their willingness-to-pay for a treatment with the aim of measuring the benefits (consequences) in a cost-benefit analysis. The focus is on the contingent valuation methods in the remaining part of section 6.2.6.3. In the contingent valuation methods one asks, on behalf of a thoroughly described hypothetical scenario, people directly about their maximum willingness-to-pay for the benefits of the use of a technology (Drummond et al. 1997). The total benefit is then the maximum amount that people are willing to pay for a wanted output (Boardman et al. 1996). This could for example be the willingness-to-pay for *in-vitro fertilisation* (IVF) and thereby to obtain a pregnancy or a child with some probability (Neumann et al. 1994). In the cost-benefit analysis the total costs of a health technology are subtracted from the total benefits, whereby the net-benefit can be express in monetary terms (e.g. in Danish kroner - DKK). A positive net-benefit (or zero) means that the technology should be chosen, because the benefits are higher than the costs. If the net-benefit, on the other hand, is negative then the technology should not be introduced at all.

In a willingness-to-pay study, and thereby in a cost-benefit analysis, consequences other than those related to the saved costs and a change in the individuals' health state due to the use of the health technology, can be included. Having information, e.g. in a screening programme, can for example have a value in itself, as mentioned earlier. The process, which the health gain derives from, may also influence on the value of the technology. In the case of IVF one could for example imagine that a process of severe hormone stimulation of the woman, as well as its associated side effects and risk of hyperstimulation influence the valuation of IVF and its output. It might also have a value that one, as a potential patient, has the possibility to have treatment in the future, if sickness appears (an option value). Finally, the introduction of a new health technology could have a value for healthy people as well; knowing that the people in need for treatment actually are treated (altruism). These consequences, other than those directly related to health status, can be incorporated in the willingness-to-pay study, and the cost-benefit analysis, depending on the way that the questions are framed and who is asked.

Measurement

Willingness-to-pay surveys can be conducted either as interviews or (postal) survey using questionnaire. The former method is recommended because of the high degree of severity and level of abstraction of the questions (hypothetical scenario). However, in the health care area the survey method is also used. No matter whether an interview or questionnaire is used, it has to be decided

who is going to be asked. In reality, all individuals, who have a benefit (utility) from the use of a health technology, either directly or indirectly, should be asked (O'Brien et al. 1996). If the focus is upon improvements in health status, the most obvious group to ask is the patients themselves (a direct use-value). The advantage of asking this group is that the patients are familiar with the specific situation, which is why they do not need to have a detailed description, as well as the situation is less hypothetical. However, to have a broader impression of the preferences and willingness-to-pay in the society for a health technology, a comprehensive group has to be asked. This will also make it possible to include other (indirect) values, such as a future use value (option) and altruism. There is no general agreement in the literature about who should be asked. In some studies patients are asked, while other studies ask samples representing the general population.

Generally, the willingness-to-pay questions can be framed in two ways (O'Brien et al. 1996). The question can be asked from a user perspective, where the respondent has to assume that he/she has a direct need for treatment. Afterwards the respondent is asked to express his/her maximum willingness-to-pay for this treatment (ex post). Alternatively the question can be framed as an insurance-based question, where persons in risk of disease, in principle everyone, is asked about their maximum willingness-to-pay in the form of an insurance package that makes the treatment free from charge, if it becomes necessary (ex ante). The ex post formulation of the WTP question is the most frequently used, while the ex ante formulation of the WTP question is argued to lead to more realistic answers, especially in tax-financed health care systems like in the Nordic countries. The choice of the type of framing will, although, depend on, who is going to be asked, and what type of benefits that are going to be revealed.

The willingness-to-pay question can furthermore have different question formats depending upon the value elicitation method chosen. The simplest of the elicitation method and WTP question, but also the most difficult one to answer, is the *open-ended question.* An example of an open-ended question could be "imagine that you are incapable of having children, how much are you willing to pay for participating in an IVF programme that provides you a 25% chance of success for being pregnant?". WTP questions can also be formulated as *closed-ended questions*, where the respondents state their willingness-to-pay within pre-specified money value intervals. An example is the bidding game, which begins with a small money value and continues with higher bid values, until the respondent answers "no" to a bid. The highest money value with a "yes" response will then represent the respondent's willingness-to-pay. Due to the guidance with the pre-specified money values this elicitation method is easier for the respondent than open-ended questions. Alternatively, different samples of respondents could instead be asked about different willingness-to-pay values. For information about other valuation-elicitation methods the reader is referred to Boardman et al. (1996) and O'Brien et al. (1996). In the formulation of the willingness-to-pay questions one should be aware of the existence of a number of potential problems, such as protest answers (zero answers or non-response), hypothetical bias, strategic bias and judgement bias (i.e. probabilities), as well as ordinary method problems entailed in the survey or interview methods. It is expected that willingness-to-pay depend on income levels. Socio-economic data, such as income, education and employment will be asked for as well, to be able to explain each individual's willingness-to-pay values. The advantage of the method is that the value of an improvement in health status can be compared directly with the associated costs, and that benefits broader than those related to changes in health status can be included.

The status for the willingness-to-pay methods today is that they are at a very experimental and incomplete stage of development (CCOHTA 1997). The precision of their estimates is uncertain and it is, for example, not clear whether the willingness-to-pay vary with the amount of the considered good. The willingness-to-pay estimates can be used to point out the preferences in the population for specific health technologies. One should, however, be careful with the direct

interpretation of the specific size of the willingness-to-pay value found in a study. The Danish guidelines also recommend that the willingness-to-pay methods are not used alone, but only as a supplement (Alban et al. 1998). Therefore, the WTP methods have, until now, only been used in a few situations in health technology assessments.

6.3.7 Discounting

Costs and consequences in the economic analysis that will not occur until one year after the beginning of the trial have to be *discounted*. Future costs and future health consequences are discounted to reflect the fact that, in general, individuals and thereby society have a positive rate of time preference, which means that people prefer desirable consequences (like health benefits) to occur earlier and undesirable consequences (like costs) to occur later (CCOHTA 1997). Future health benefits are discounted to reflect that they are worth less in the future, while future costs are discounted, because it is desirable if these are postponed. If these do not occur at the same time, it will be necessary to discount costs and consequences by calculating the present values of future costs and consequences to be able to compare health technologies in an economic analysis. For example, there will be costs from running a screening programme immediately, while the expected gain in life years first will be experienced later. Discounting should not be confused with inflation. However, when data on prices used in the economic analysis come from different time periods, the past prices has to be brought into current terms so that they reflect the opportunity cost of the resources in the same present value monetary terms (Luce et al. 1996).

When discounting, the value of the costs and consequences that occur in t years are reduced, as they are multiplied with the factor $1/(1+r)^t$, where r is the chosen discount rate (Alban et al. 1995). A cost of 5,000 DKK that has to be paid after three years, will with a discount rate at 5 percent have a present value of $5{,}000/(1 + 0.05)^3 = 4{,}319$ DKK. The present value of a health benefit of 250 QALYs that is gained after five years, will with a 5 percent discount rate be the same as a gain of 196 QALYs today ($250/(1+0.05)^5$). As the examples show, the choice of discount rate is crucial. There is no recommendation in the Danish guidelines with respect to a specific discount rate to use, but internationally rates between 3 to 6 percents have been recommended. It is, however, recommended that the discount rate is varied in the sensitivity analysis to investigate its influence on the result of the analysis.

6.3.8 Modelling in the economic analysis

In some situations it is necessary to model the economic analysis – entirely or partially. There are several reasons to this (Buxton et al. 1997). Extrapolation of short-term clinical data with the purpose of predicting these data in the long run or the extrapolation of intermediate measures of effectiveness to final measures of effectiveness might be reasons for the use of modelling in economic analysis. The conduct of a clinical trial in a controlled and randomised setting, which secures a high internal validity, imply on the other hand that the study has a low external validity. In these situations it might be necessary to model the economic analysis to be able to generalise to the routine practice setting or between regions in a country. As mentioned previously, the new technology can be compared with a placebo in the clinical trial. In this case, the use of models can be the only solution for investigating the cost-effectiveness of the new technology compared with routine practice. Finally, economic and clinical data can be missing, especially in the early development phase of a health technology. In such a situation the economic analysis may be entirely modelled and based upon the best evidence available. *Decision trees* and *Markov models* are some of the most frequently used types of models. In a Markov model a hypothetical cohort is

typically simulated until the individuals in the cohort are exhausted, because the patients recover or are dead (for more information on Markov models see Briggs et al. (1998)). However, independently of whether modelling is necessary or the economic analysis is based on a clinical trial, an overview of the possible treatment paths can be obtained by drawing a decision tree.

6.3.9 Handling of uncertainty in the economic analysis

To various degrees uncertainty with respect to specific parameter values, assumptions and models will affect the results and conclusions made at in economic analyses. One reason to this is that the economic analysis is often based upon deterministic point-estimates without any distribution and variance. However, it is important that this uncertainty is handled systematically and it's impact is quantified, because it will affect the decisions that can be made, based on the economic analysis. Table 6.6 shows the different forms of uncertainty that are possible in an economic analysis.

Table 6.6: Forms of uncertainty in an economic analysis

Parameter uncertainty	1. Variability in data input	Does the point-estimates reflect the parameters' true values? The method used for the data collection can be a reason to the uncertainty, e.g. expert advices on resource-use.
	2. Sample data variability	Variability in the sample data. Different samples drawn from the same population may result in different data concerning resource-use and health consequences.
	3. Extrapolation	Uncertainty caused by the extrapolation of intermediate measures of effectiveness to final measures of effectiveness, as well as uncertainty caused by extrapolation beyond the time horizon of the study.
	4. Generalisability	Can the results found in the study population and in the study location be generalised to other populations and locations? E.g. the generalisability of results to routine practice.
Model uncertainty	5. Analytical methods	Disagreement about the use of different methods in the economic analyses, where the choice of method leads to uncertainty about the results and conclusions from the analysis. E.g. two methods to estimate lost production.
	6. Model structure	Uncertainty about the specific method to combine the parameters in a model –the relation between the parameters. Putting forward an alternative model can perhaps test this.

A systematic quantification of the uncertainty implies that sensitivity analyses and/or statistical analyses have to be undertaken after the base-case analysis is made. This will show the decision-maker, how *robust* (trustworthy) the results and conclusions of the economic analysis are, or perhaps how uncertain these are. In sensitivity analyses the sensitivity of the result from changes in assumptions and values are investigated. Sensitivity analyses should always be a part of an economic analysis, and possibly conducted together with statistical analyses. To perform a statistical analysis stochastic sample data is required. Statistical analysis is not sufficient alone, because it will only investigate the uncertainty caused by variation in the population, as seen in Table 6.6 and Table 6.7. The reader is furthermore referred to Briggs et al. (1994, 1999).

Table 6.7: Different methods to handle uncertainty (based upon Briggs et al. (1994))

Sensitivity analyses	Simple "one-way" sensitivity analysis	Each parameter (e.g. the wage rate) is varied individually and the effect upon the result can be seen. This is the most used analysis, but also the most simple. A good start for the investigation of uncertainty.	Type 1-5
	Simple "multi-way" sensitivity analysis	The difference from the "one-way" analysis is that two or more parameters are varied simultaneously. Has a higher degree of realism, but is more difficult to conduct.	Type 1-5
	Analysis of extremes	After the base-case analysis two analyses are performed focussing on the most optimistic and the most pessimistic parameters. This sensitivity analysis will show the scope of the result.	Type 1,2,3
	Threshold analysis	Identifies the critical value (threshold) of parameter(s) above or below which the conclusion of a study will change. Can only be used if the variables are continuous.	Type 1-4
	Probabilistic sensitivity analysis	The uncertain variables are assigned ranges and distributions, and a Monte Carlo simulation selects values at random. This simulation is undertaken many times, whereby a distribution is obtained from which the variance can be estimated.	Type 1-2
Statistical analysis - if stochastic sample data		The uncertainty (as a result of the variation in the population) is indicated with distributions, ranges, variances and confidence intervals for each variable. Eventually a test of hypothesis can be made.	Type 2

6.3.10 When is a health technology then cost-effective?

To be able to conclude which health technology that is cost-effective, and thereby should be adopted, the total costs have to be compared with the effectiveness (health consequences) of each health technology. In the cost-effectiveness analysis and the cost-utility analysis this is done with the calculation and comparison of ***cost-effectiveness ratios*** for each technology investigated using the formula below.

$$\frac{C}{E} = \frac{C_{\text{(health care sector)}} + C_{\text{(patient and family)}} + C_{\text{(lost production)}} - \text{saved costs}}{\text{Effects}}$$

The effectiveness (consequences) can be measured in natural units or in quality-adjusted life years (QALY). In the comparison of the cost-effectiveness between two health technologies (a new technology and an old existing technology), there appears to be nine possible outcomes for the recommendation to be provided to the decision-makers based on an economic analysis. These outcomes are shown in Table 6.5 below.

Table 6.5: The cost-effectiveness decision matrix

A new technology compared with an old technology	**Less effectiveness** $E_{new} < E_{old}$	**Same effectiveness** $E_{new} = E_{old}$	**More effectiveness** $E_{new} > E_{old}$
Less costs $C_{new} < C_{old}$	1. No clear decision - *non-dominance*	4. Adopt the new technology - *the new dominates the old*	7. Adopt the new technology - *the new dominates the old*
Same costs $C_{new} = C_{old}$	2. Keep the old technology - *the old dominates the new*	5. The technologies are equal	8. Adopt the new technology - *the new dominates the old*
More costs $C_{new} > C_{old}$	3. Keep the old technology - *the old dominates the new*	6. Keep the old technology - *the old dominates the new*	9. No clear decision - *non-dominance*

The decision that can be made, based on the cost-effectiveness and cost-utility analyses is most obvious in two situations. The first situation is, where the new technology is more effective (e.g. a gain in life years) and at the same time is less costly, than the existing (old) technology that is used today. If this is the case, then it seems obvious that the recommendation should be to adopt the new health technology and replace it for the old one, as more effectiveness is gained for less (or the same) resources. In such a situation, the new health technology is cost-effective, because the cost per unit of effectiveness (e.g. life years) for the new technology is lower compared with the old technology ($C_{new}/E_{new} < C_{old}/E_{old}$). The new technology is then said to *dominate* the old technology. The second situation, where a clear recommendation can be made on behalf of the economic analysis, is on the other hand, when the new technology is less effective and is more costly compared with the existing old technology. In this situation the old technology dominates the new technology, because it is both more effective and less costly, and should therefore still be used as the most cost-effective one ($C_{new}/E_{new} > C_{old}/E_{old}$). These two situations are equal to the decision outcome 4,7,8 and 2,3,6 in the matrix. If the conclusion from a cost-effectiveness analysis falls in one of these dominating outcomes, then it is sufficient just to conduct a cost-effectiveness analysis, because dominance implies cost-effectiveness (Alban et al. 1995). However, the issue of dominance can sometimes be difficult to know at the early stage, when the economic analysis is designed.

If the economic analysis, on the other hand, ends up in the decision outcomes 1 and 9 in the matrix, it will be more difficult to conclude and recommend. Here, the new technology is either more costly and more effective, or less costly and less effective compared with the existing old technology. There is no dominance between the two technologies (non-dominance). We do not know whether we want to spend some extra resources to obtain the extra effect. It is now no longer possible to make any clear recommendation for decision-making about the choice of technology by only comparing the cost-effectiveness ratios of the two technologies. Instead, it must be assessed, whether this extra effectiveness that can be gained from the use of a new technology (or perhaps the old technology) is worth the extra costs that it requires (Briggs et al. 1999). To assist in this assessment the ***incremental cost-effectiveness ratio*** (ICER) can be calculated and reported. ICER expresses the cost of an extra unit of effectiveness produced with the new technology, e.g. the price to gain an extra life year. The formula for ICER is:

$$\mathrm{ICER} = \frac{\mathrm{C}_{(\mathrm{new})} - \mathrm{C}_{(\mathrm{old})}}{\mathrm{E}_{(\mathrm{new})} - \mathrm{E}_{(\mathrm{old})}}$$

Whether the new (or alternatively the old) technology should be accepted as being cost-effective in a situation, where it is more costly but also more effective, will depend on the maximum price that one is willing to pay for this extra effect (Drummond et al. 1997). Those able to judge this could be

both decision-makers as well as patients. In a situation with non-dominance between the compared technologies it is not possible to make any further conclusions in the cost-effectiveness analysis.

If a cost-utility analysis is performed, then the *cost per QALY*, in case of non-dominance, can be compared with QALY-prices in previously performed cost-utility analyses. This comparison may give an idea about the "size" of the cost per QALY gained from the new technology compared with other technologies and areas. As previously mentioned, no specific guidelines exist concerning what is regarded as a reasonable price per QALY, although suggestions have been put forward, e.g. in Laupacis et al. (1992).

Another solution having a situation of non-dominance is to enlarge the cost-effectiveness analysis with a cost-benefit analysis. Then it is possible to answer the question, whether one is willing to pay for the extra effectiveness that is obtained using the more costly new technology. However, as previously explained the cost-benefit analysis imply that the consequences (health benefit) are valued in monetary units, for example in the form of willingness-to-pay estimates. To be able to make any conclusions from the cost-benefit analysis the (present value) net benefit has to be calculated following the formula shown below.

$$\text{Net benefits} = \text{Benefits}_{(new)} - \text{Costs}_{(new)} \ ? \ 0$$

If the benefits are higher than the costs (or equal to zero) then there will be a total benefit for the society by adopting the new technology. The decision rule in the cost-benefit analysis can be based on just one technology, because both consequences and costs are valued in monetary units. Further information about the cost-benefit analysis can be found in Boardman et al. (1996).

When cost-effectiveness ratios (or net benefits) are calculated and compared for the analysed health technologies, and a conclusion concerning cost-effectiveness is made, then the results and the recommendations must be reported in the HTA-analysis. It is important to be as explicit as possible, when an economic analysis is reported. To make it easier for the reader and user of the economic analysis the results, design, input data and assumptions made have to be thoroughly described. Assumptions and sensitive data and results must be varied and tested in sensitivity analyses to give an impression of how robust the analysis and its results are. Because of economic analyses and HTA focus on use in decision-making, the reporting of the economic analysis, its results and recommendations need at the same time to be described and explained as clearly as possible.

The check-list for economic analyses, presented below, can be used as a list for what should be remembered in the conduct of an economic analysis as part of an HTA, as well as to provide the reader with an impression of the quality of published economic analyses.

A check-list for economic analyses (inspired by Drummond et al. (1997))**:**

1. Is a well-defined question posed? This will be one or more of the HTA-questions.
2. Is the perspective of the analysis clearly stated?
3. Are the relevant competing alternatives included and described in the analysis? Are any perhaps omitted?
4. Is the effectiveness of the compared technologies documented? What is the source?
5. Are all relevant costs and consequences, corresponding to the perspective, identified?
6. Are costs and consequences of the technologies measured in appropriate (physical) units?
7. Are costs and consequences valued credibly?
8. Are differential timing of costs and consequences handled? Discounting.
9. Are sensitivity analyses carried out to test for uncertainty in the economic analysis and to investigate how robust this analysis and its conclusion are?
10. Are the conclusions in the analysis presented as a ratio of costs and consequences?
11. Are the conclusions valid and able to generalise, and are all interested parties considered?
12. Is it considered to conduct a budget analysis to identify who gains and who loses in budgetary terms, as well as a business analysis, as supplements?

6.4 Budgetary and business economic considerations

In budget- and business analyses the perspective is more narrow than in the economic analysis. The aim of a budget analysis is to investigate, how different "budgets"; e.g. county councils or municipalities are affected (positively and negatively), if a new health technology is adopted. Due to the organising and financing of the health care sector, the economic consequences for the different budgets will be different, and savings for one budget may lead to expenditures for another. Savings for a county council by implementing day surgery in hospitals might, for example, cause some additional expenditure for nursing care at home in the municipalities to the earlier discharged patients. This is a phenomenon of "maximising the budgets". It is, therefore, important that the consequences for each affected budget are considered. Concepts like expenditures and revenues are used in budget analyses instead of costs and utilities from the economic analysis. Expenditures can, besides the actual resource-use for manpower, equipment, etc., incorporate transfer payments like sickness benefit. For any disease these may have a significant importance for the specific budget. In an economic analysis transfer payments are not included, because they do not imply an extra use of resources in society, but only a transfer of income between individuals in society. There are a few examples of HTA analyses in Denmark, where budget analyses are incorporated (Sundhedsstyrelsen 1985, Statens Institut for Medicinsk Teknologivurdering 1999).

A business analysis includes an assessment of the expenditures and revenues from the establishment and use of a health technology in the specific department, hospital or county council. The analysis can be divided in three phases: 1) the purchase and establishment of the technology (the need for investments), 2) the operation and use of the technology, and 3) the costs and savings derived from the establishment and use of the technology. Naturally, this information about the need for resources for investments and running costs throughout the lifetime of the technology is essential for the specific department or hospital, which considers adopting the new health technology.

However, as stressed in the beginning of the chapter, it is not sufficient in a health technology assessment just to conduct the more narrow budget or business analyses to cover the economic aspect of the adoption of a health technology. To answer the economic questions, this part of the HTA should first of all include an economic analysis, e.g. conducted from a societal perspective. This will reduce the likelihood of only "thinking in terms of maximising the budgets" with respect to the adoption and use of health technologies, which is often not optimal from a societal perspective.

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