LO Lynn O'Keeffe

Module 3 - Introduction to Health Economic Evaluation Methods

Module Three provides an introduction to some of the methods used in health economic evaluation.

The module has four units:

- Unit 1: Accessing and Appraising Published Economic Evaluation Evidence
- Unit 2: Experimental and Observational Designs
- Unit 3: Decision Analytic Models
- Unit 4: Preference elicitation

On successful completion, you will be able to:
Unit 1

- understand the rationale for obtaining and appraising published evidence
- identify sources of economic evaluation evidence

- use a framework for appraising the quality of economic evaluation evidence

Unit 2

- understand the rationale for conducting experimental or observational designs
- identify the key features of both trials and observational studies
- describe some of the approaches to analyzing uncertainty in trials and observational studies

Unit 3

- understand the rationale for developing decision analytic models
- describe the key features of a decision tree model
- be aware of the state transition type of model
- describe approaches to analyzing uncertainty in models

Unit 4

- understand the rationale for eliciting preferences
- identify alternative approaches to determining preferences
- describe key aspects of the discrete choice experiment stated preference technique

UNITS

Accessing and Appraising Published Economic Evaluation Evidence

Welcome to the first unit of Module Three, Accessing and Appraising Published Economic Evaluation Evidence.

Unit Objectives

The goals of this unit are to:

- Outline the rationale for obtaining and appraising published evidence
- Provide information on how to access economic evaluation evidence
- Introduce a framework for appraising evidence quality

Unit Topics

The topics that will be covered in this unit are:

1. Why look for published evidence?
2. Types of published evidence
3. Searchable databases
4. Appraising quality and transferability

Video Presentation

Here's the video presentation for this unit:

Click to play

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1. Why Look for Published Evidence?

There are many types of decision problems that can be informed by health economic evaluation. Examples include:

- [ ] Whether to make a new health information technology investment

- [ ] How to optimize a medicines management process in your organization
- [ ] Whether to switch from medication A to medication B

One of the first things to do when confronted with such problems is to take a look at the evidence that already exists. Investing the time to find and appraise economic evaluation evidence relevant to our decision problem can help us to answer:

- Do we need to do our own analysis or is there enough good quality evidence relevant to our decision problem already in existence that we can rely on?
- Based on the available evidence, what is the appropriate decision?

In many cases, looking at published evidence is going to be a sufficient step. It may provide good evidence relevant to a decision problem that would not be meaningfully improved by making the additional investments to conduct your own original analysis through trial, observational, or modelling studies.

However, even if the existing evidence base is not strong enough to help us make a decision with confidence, reviewing this evidence can help us plan and implement our own analysis:

- Reviewing existing evidence will help refine our search question and the methods we use
- If conducting a modeling study, the published evidence will be a major part of the data inputs into our models

2. Types of Published Evidence

The main types of published economic evaluation evidence that may be useful to us are below. Click each one to learn more:

Academic literature	Academic literature articles from a cost-effectiveness, cost-utility, cost benefit analysis published in either health economic or clinical journals (Cost-Minimization and Cost-Consequence Analyses may
Health Technology Assessment Reports	• Often produced or funded by government agencies (e.g. CADTH in Canada and NICE in UK) • May be peer reviewed or "grey literature"
Reimbursement decision documentation	From government agencies or commercial organizations

Other grey literature

Other grey literature (e.g. NGO reports)

3. Searchable Databases

The options available to you for searching for economic evaluations will depend on where you work and the sort of institutional support you have. If you work either in an academic institution or in an organization that subscribes to academic databases, then you will have a very rich potential evidence base at your fingertips. If you don't have access to that support, then you may find that you have ready access to some of the available evidence whilst other evidence will only be available to you for an additional fee.

When searching for economic evaluations, the three main options are to use a specialist economic evaluation/HTA database, use one of the academic medical databases, or use something like Google Scholar.

- Specialist economic evaluation or HTA databases:
    - http://www.crd.york.ac.uk/CRDWeb/ [NHS EED (excellent source of economic evaluation evidence published up to end of 2014)]

    - https://database.inahta.org/ [Canadian & International HTA]

- Academic medical databases:
    - EMBASE
    - MEDLINE/PUBMED
    - PsychINfo
    - CINAHL
    - Google scholar

The type of search terms you should use will vary according to the database you are using. In something like NHS EED, where you are searching articles that have already been identified as Economic Evaluations, your search terms don't need to include terms such as:

- economic evaluation
- cost-effectiveness
- cost-utility
- cost-benefit
- QALY

In other databases, such search terms can be helpful and should be combined with the topic area of interest (e.g. the name of the particular device or drug that you are evaluating or the illness group of the patients). For examples of how to construct search terms, view those used by the makers of NHS EED: http://www.crd.york.ac.uk/crdweb/searchstrategies.asp

4. Appraising Quality and Transferability

Having found a number of studies that are potentially relevant to your decision problem, the next task is to determine which, if any, of them are quality sources of evidence and which are the most relevant to the particular decision problem that you face.

Appraising quality

There are a number of different checklists that you can use to help you to critically appraise the quality of an economic evaluation study. Perhaps the most widely used is the Drummond checklist. By going through this checklist on a point-by-point basis, you will be able to determine whether the study is likely to adhere to all the criteria of a quality study or whether there may be some key gaps that leave serious question about whether or not this study is particularly useful to you.

Drummond Checklist

- [ ] was a well-defined question posed in answerable form? (e.g. specify the alternatives being examined and the analysis perspective)
- [ ] was a comprehensive description of the competing alternatives given? (i.e. can you tell who did what to whom, where, and how often?)
- [ ] was the effectiveness of the program or services established?
- [ ] were all the important and relevant costs and consequences for each alternative identified?
- [ ] were costs and consequences measured accurately in appropriate physical units? (e.g. hours of nursing time, number of physician visits, lost workdays, gained life years)
- [ ] were the cost and consequences valued credibly?
- [ ] were costs and consequences adjusted for differential timing?
- [ ] was an incremental analysis of costs and consequences of alternatives performed?
- [ ] was allowance made for uncertainty in the estimates of costs and consequences?
- [ ] did the presentation and discussion of study results include all issues of concern to users?

Transferability

Beyond the quality of the study, the other question is its its transferability to your particular decision problem.

It is unlikely a published study will be an exact match for the decision problem you are facing. For example, the study may have taken place in another jurisdiction, or in a different setting (e.g. in primary care as opposed to a hospital-based environment) or it may have taken place at a time when practice was very different to current practice. Such differences in jurisdiction/setting/practice may have a very significant relationship with a range of costs and outcomes that are **not** relevant to your decision problem. In these cases, the study under consideration may not be an appropriate evidence source for you to use.

Some of the things to consider when assessing how transferable study findings are to your decision problem, include:

- [ ] Country/region
- [ ] Health system
- [ ] Setting (e.g. primary vs tertiary care, type of organization)
- [ ] Time (e.g. have recent developments changed the context since the study was published?)

Exercises and Further Reading

References and Further Optional Reading

If you would like to do further optional reading about the topic, you may wish to consider the following resources:

- Department of General Practice, University of Glasgow. "Critical appraisal checklist for economic evaluations." University of Glasgow.
- M Drummond, M Sculpher, G Torrance, B O'Brien and G Stoddart: Methods for the economic evaluation of health care programmes. 3rd edition. Oxford University Press, New York; 2005.
- D Huserau et al. "Consolidated Health Economic Evaluation Reporting Standard (CHEERS)— Explanation and Elaboration: A Report of the ISPOR Health Economic Evaluation Publication Guidelines Good Reporting Practices Task Force." Value in Health 2013.
- L Niessen, J Bridges, B Lau, R Wilson, R Sharma, D Walker, K Frick and E Bass."Assessing the Impact of Economic Evidence on Policymakers in Health Care-A Systematic Review." 2012, Agency for Healthcare Research and Quality.
- D Walker, R Wilson, R Sharma, J Bridges, L Niessen, E Bass and K Frick. "Best Practices for Conducting Economic Evaluations in Health Care: A Systematic Review of Quality Assessment Tools. Methods Research Report". 2012, Agency for Healthcare Research and Quality.

Note on links: If you find that a hyperlink used in this module is out of date, please notify us at cdneducationlead@leadingedgegroup.com. You may also be able to find an out of date web resource by searching for the expired URL at http://archive.org/web/web.php.

Self-Assessment Exercise 4 - Searching for Economic Evaluation Papers

1. Task Summary

In this self-assessment task, you will practice searching for health economic evaluation papers. Specifically, your task is to see how many health economic evaluations of psoriasis-related interventions published since 1995 you can find using the NHS EED and MEDLINE databases.

In order to complete this task, you will need to create an account (registration is free) in each database. Registration for each is available at:

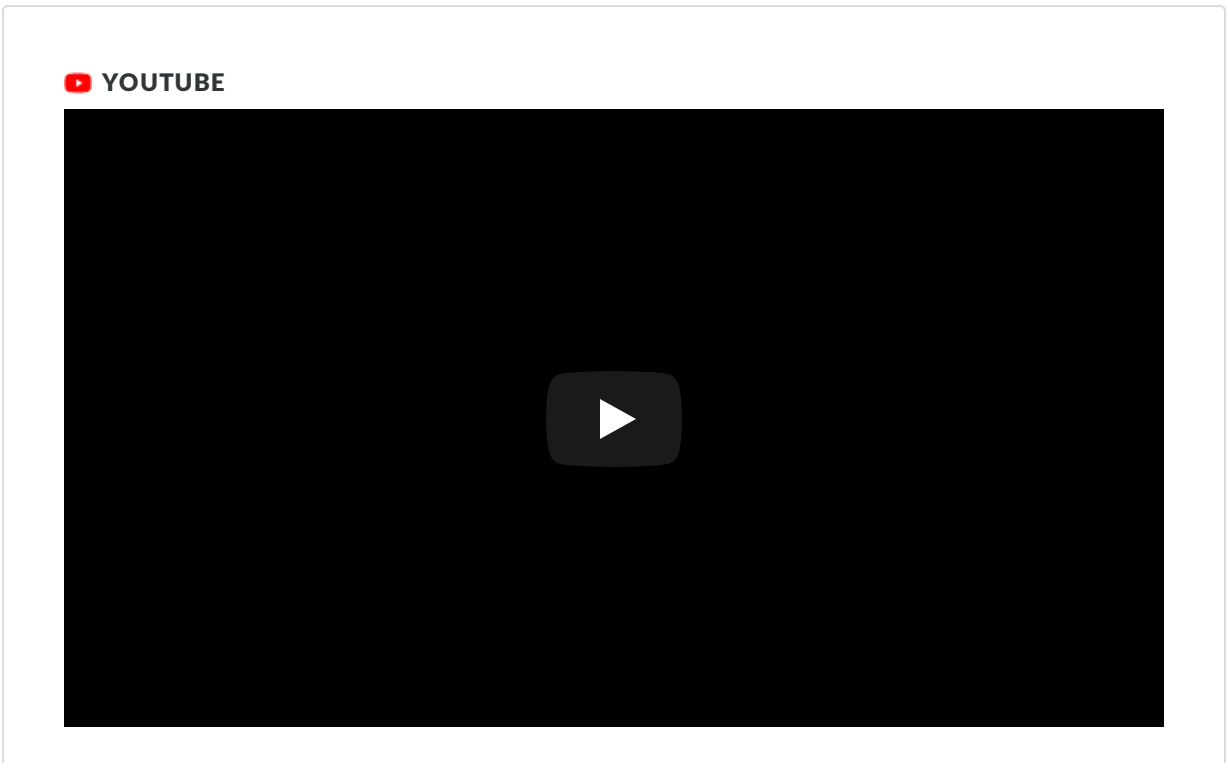
- For PubMed/MEDLINE searches sign up here
- For NHS EED searches sign up here

Please note that these are external databases with no affiliation to Leading Edge Group or linkage to this course. You may therefore wish to learn more about their publishers (the National Centre for Biotechnology Information [USA] and the University of York Centre for Reviews and Dissemination [UK] respectively) before choosing whether to register.

You should ensure that you are logged in to each account before attempting to build and save your search.

To search for psoriasis information, you will use MeSH (Medical Subject Headings) terms. More about what MeSH terms are and how to use them in your searches in PubMed/MEDLINE can be found in the following resources:

- Medical Subject Headings (MeSH®) in MEDLINE®/PubMed®: A Tutorial (Published by the US National Library of Medicine)
- PubMed Advanced Search Builder Video Tutorial (Published by the National Centre for Biotechnology Information):



PubMed Advanced Search Builder

A tutorial describing how to use the Advanced Search Builder to help refine your PubMed searches.

VIEW ON YOUTUBE >

A similar approach applies in NHS EED, although searches tend to be simpler as the entries in NHS EED are already preidentified as being economic evaluations; so it is not necessary to include the search terms related to economic evaluations that need to be used in PubMed/Medline. A guide to searching in NHS EED is provided here.

Task steps

1. Complete registration to both databases.
2. Review the tutorials and information provided in the links above.
3. Review the search strategies for each database provided in the example below.
4. Replicate the same search in each database using the advanced search tools (making sure to save your search).
5. Review the titles and the abstracts returned by your search in order to identify papers which are full economic evaluations of psoriasis (not partial economic evaluations or full economic evaluations of psoriatic arthritis). To get an idea about how much filtering you will need to do, see the example filtering flowchart below.
6. Compare the list of full economic evaluations of psoriasis interventions you generated with the master list below.

Search Strategy Using MeSH terms - Psoriasis Example

PDF

Self Assessment Exercise 4 -Sample Search Strategy.pdf

55.2 KB

Search Results Filtering Flowchart - Psoriasis Example

PDF

Self Assessment Exercise 4 -Search Strategy Results Flowchart.pdf

34.8 KB

Search Strategy Results - Psoriasis Example

PDF

Self Assessment Exercise 4 -Search Strategy Results.pdf

82.6 KB

Module 2 of 4

Experimental and Observational Designs

LO Lynn O'Keeffe

Welcome to the Unit Two of Module Three, which is: Experimental and Observational Designs.

Unit Objectives

The goals of this unit are to:

- Understand the rationale for conducting experimental or observational designs
- Identify key features of both trials and observational studies
- Describe some of the approaches to analyzing uncertainty in trials and observational studies

Unit Topics

The topics that will be covered in this unit are:

1. Rationale and key features
2. Randomized Control Trials
3. Observational studies

4. Data collection essentials
5. Sensitivity analysis
6. Bootstrapping

Video Presentation

Here's the video presentation for this unit:

1. Rationale and Key Features

In the last unit, we looked at reviewing the existing evidence base with a view to finding out whether or not it is strong enough to help you make a decision with confidence. In cases where the existing evidence is inadequate to your decision requirements, then the next potential step to consider is whether to:

- set up an experiment (a trial); or

- analyze what has previously happened (retrospective observation) or what we are about to do (prospective observation)

In both cases, our aim is to gather data from a sample or subset of the target population and infer from the cost and benefits we observe in that sample, the costs and benefits that will apply to the whole population.

The rationale for proceeding with an experimental or observational study is principally based on:

- [ ] **current evidence not being sufficient** (in quality, quantity or relevance) to base a decision on.

- [ ] the **decision problem being sufficiently important** to warrant an investment of resources in either a trial or observational study.

- [ ] the **feasibility / suitability** of answering our research question through a trial or observational design.

2. Randomized Control Trials (RCTs)

A Randomized Control Trial is an experiment that:

- is carefully designed to minimize potential sources of bias (e.g. arising from differences in study participants, how key data is collected, etc.)
- has at least one intervention group and a control group, participants being randomized between groups

Although regarded as the gold standard of evidence-generation methods, RCTs are often complex and expensive to plan and execute, requiring a lot of specialist expertise (e.g. from biostatisticians). For these reasons, an RCT should be considered only where the decision problem is sufficiently economically important to merit the potentially significant investment of time and resources required to undertake one.

3. Observational Studies

A normally less onerous alternative to the RCT is the observational study. Observational studies are not regarded as experiments, because subjects are not assigned to intervention or control groups in a manner that is designed to eliminate bias. Potential study data may in effect be limited in advance by what is available in the observational dataset being used (e.g. medical records from a service).

These limitations mean that results are normally not as internally valid as those from RCTs. However, an observational design may be a more appropriate choice than a RCT for your research question for a range of ethical (it would not be acceptable to randomize patients to a control group that is highly likely to produce poor outcomes), financial, or feasibility reasons. There are some other compensating benefits of observational studies, such as sample sizes often being higher than those commonly achieved in RCTs.

Observational studies can also be either retrospective (reviews records of what has already happened) or prospective (observes what is about to occur).

4. Data Collection Essentials

Whether an experimental or observational study design is chosen, there are some key data requirements to enable an economic evaluation. We need data on **resource** use and costs, and **outcomes**. Click each item to learn more:

Resource use and costs

Either from administrative data/health records or from surveys of resource use (e.g. a questionnaire administered to patients about which health services they used over the study period). The data collected

Outcomes

It is necessary to collect data on the outcomes of interest at all relevant time points (e.g. at baseline, at end of intervention, and at specified follow-up periods after the end of the intervention). The type of

5. Sensitivity Analysis

As highlighted in a previous unit, there will be some uncertainty attached to any results we produce from our study.

We can begin to explore this uncertainty by conducting sensitivity analyses. In sensitivity analyses, we see how sensitive our results would be (i.e. how much they would change) if we changed the values of some key parameters.

For example, common sensitivity analyses to undertake might include examining the impact of alternative unit cost for the resources, the amount of resources used per individual, or the size of treatment effects. By conducting these sensitivity analysis we can identify which parameters are most important to cost-effectiveness results, as well as threshold values for each parameter at which the intervention becomes or ceases to be cost-effective.

6. Bootstrapping

A common means of exploring uncertainty in trial or observational studies is to use a technique known as the bootstrap.

The essence of both trial and observational designs is that we are trying to estimate the mean incremental costs and effects (and hence ICERs) for our population of interest by examining a sample (subset) of that population.

However, the estimate we produce will depend on the study sample. If we select another sample, our estimate will probably be different. This means that an estimate from a sample is unlikely to be an exact match for the true population value.

Bootstrapping is a statistical technique to explore the uncertainty about the accuracy of the estimate derived from a sample.

As a simple example, imagine you had a sample of four with the following results:

- 1,2,3,4

In bootstrapping, we might create a number (in this example, five—in reality it will be much larger numbers) of alternative samples of the same size (four items) with results generated based on the frequency of occurrence in our original sample (in this case, 1,2,3, and 4 all have an equal 25% chance of being drawn). Your new results might be:

- 1,2,3,2
- 3,4,4,1
- 1,3,2,2
- 4,4,4,1
- 1,1,2,3
- 1,4,1,2
- 2,2,2,3
- 4,3,1,2
- 1,2,3,2
- 1,4,2,1

Each sample will have its own mean value (e.g. in the first bootstrapped sample the mean is 2=(1+2+3+2)/4). Having generated these bootstrapped samples, we are thus able to use their means to generate a confidence interval (e.g. 95% confidence interval) for the sample mean of the simulated population.

Self-assessment and critical review exercises

Your critical review task is as follows:

- Read the methods and sensitivity analysis sections of the trial/observation sample paper that was assigned to you. Are the methods used to minimize potential bias and to maximize study validity clearly outlined? Are sources of uncertainty identified and adequately addressed? Is bootstrapping used?

Health Economics Sample Economic Evaluation Paper 1.pdf
320.2 KB

Health Economics Sample Economic Evaluation Paper 2.pdf
346.6 KB

References and Further Optional Reading

If you would like to do further optional reading about the topic, you may wish to consider the following resources:

- M Cambell and D Torgensen. "Bootstrapping: estimating confidence intervals for costeffectiveness ratios." 1999, QJM.
- B Motheral, J Brooks J, M Clark, et al. "A checklist for retroactive database studies – Report of the ISPOR Task Force on Retrospective Databases." 2003, Value in Health.

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Self-Assessment Exercise 5 - Experimental and Observational Designs

1. Instructions for Self-Assessment Exercise 5

Your task in this exercise is to explore the uncertainty from a set of results from a trial by:

1. Bootstrapping ICER values and plotting them on a Cost-Effectiveness Plane (update cells in Part One Excel file).

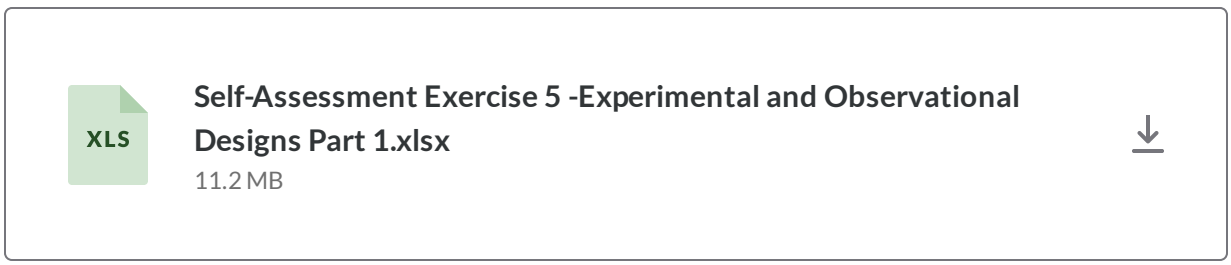
- As you had previously done as part of Self-Assessment Exercise 3, copy the cost and benefit results from Self-Assessment Exercise 3 into the appropriate dark orange or light orange cells on worksheets Study Sample - Effects and Study Sample - Costs
- On the Worksheet named Bootstrapped CIs, identify the 95% limits for the bootstrapped differences in effects and differences in costs (hint - use the PERCENTILE command on results outlined on the worksheet called "Differences - Bootstrap Samples".)
- On the worksheet named "Bootstrapped ICERS - CE Plane" complete the green cells (hint - use the TRANSPOSE command on results outlined on the worksheet called "Differences - Bootstrap Samples".)
- See how your results are plotted on the CE Plane

NOTE: When comparing results with the solution sheet, it is likely that there will be differences in the random draws. You should compare formulae, not results (though overall results should look similar, but not exactly matching).

2. Plotting a Cost-Effectiveness Acceptability Curve (update cells in Part Two Excel file).

- Cut and paste mean incremental effects, mean incremental costs, and ICER results from the bootstrapped results in Part One Excel file
- See the resulting CEAC curve

Part 1 of Self-Assessment Exercise 5: Experimental and Observational Designs



Part 2 of Self-Assessment Exercise 5: Experimental and Observational Designs

XLS

Self-Assessment Exercise 5 -Experimental and Observational Designs Part 2.xlsx

656.1 KB

Solution to Self-Assessment Exercise 5

XLS

Self-Assessment Exercise 5 -Experimental and Observational Designs Part 1 Solution.xlsx

13.2 MB

XLS

Self-Assessment Exercise 5 -Experimental and Observational Designs Part 2 Solution.xlsx

1.3 MB

Module 3 of 4

Decision Analytic Models

Welcome to Unit Three of Module Three, which is Decision Analytic Models.

Unit Objectives

The goals of this unit are to:

- Understand the rationale for developing decision analytic models
- Describe the key features of a decision tree model
- Be aware of the state transition type of model
- Describe approaches to analyzing uncertainty in models

Unit Topics

The topics that will be covered in this unit are:

1. Rationale for, and key features of, Decision Analytic Models
2. Decision Tree Models
3. State Transition Models
4. Sources of Uncertainty in Decision Analytic Models
5. Sensitivity Analysis

Video Presentation

Here's the video presentation for this unit:

1. Rationale for, and key features of, Decision Analytic Models

Rationale

The rationale for proceeding with a decision analytic model is principally based on three points.

- [ ] **Current evidence does not directly address our decision problem**

- [ ]

- [ ] However, **an appropriate synthesis of available evidence from multiple sources** (e.g. including assumptions and expert opinion) **may provide adequately strong evidence** to inform our decision problem

- [ ] There are practical (time, money, feasibility, ethical) reasons as to why **an experimental or observational design is not appropriate** to address evidence gaps. For example, it may be important to us to make a decision before deploying the intervention in even an experimental/observational context

Key features

Decision Analytic Models differ from trial or observational designs in that in an experimental or observational approach we are trying to address the research question by observing actual results from one primary data source, whereas within a decision analytic model framework we aim to synthesize different types of evidence from multiple different types of sources.

Some of the advantages of models include:

- Models can provide a global assessment of what the overall evidence base is saying about our decision problem
- Models can be less expensive and provide a quicker result than a trial or observational study

However, there are some weaknesses relating to the use of models to consider:

- Models provide a prediction rather than an observed result
- There may be a high degree of variability in terms of the quality or relevance of particular evidence sources, resulting in some parts of a model relying on shaky foundations (e.g. assumptions)

Types of decision analytic models

There are three common types of decision analytic models used in health economics:

- **Decision tree**
- **State transition** (e.g. Markov)
- **Discrete event simulation**

Furthermore, we can distinguish between

- a cohort model that is comprised of a group of identical individuals, and
- an individual patient/micro-simulation model comprised of a heterogeneous group of individuals where costs and outcomes can vary by individual characteristics

2. Decision Tree Models

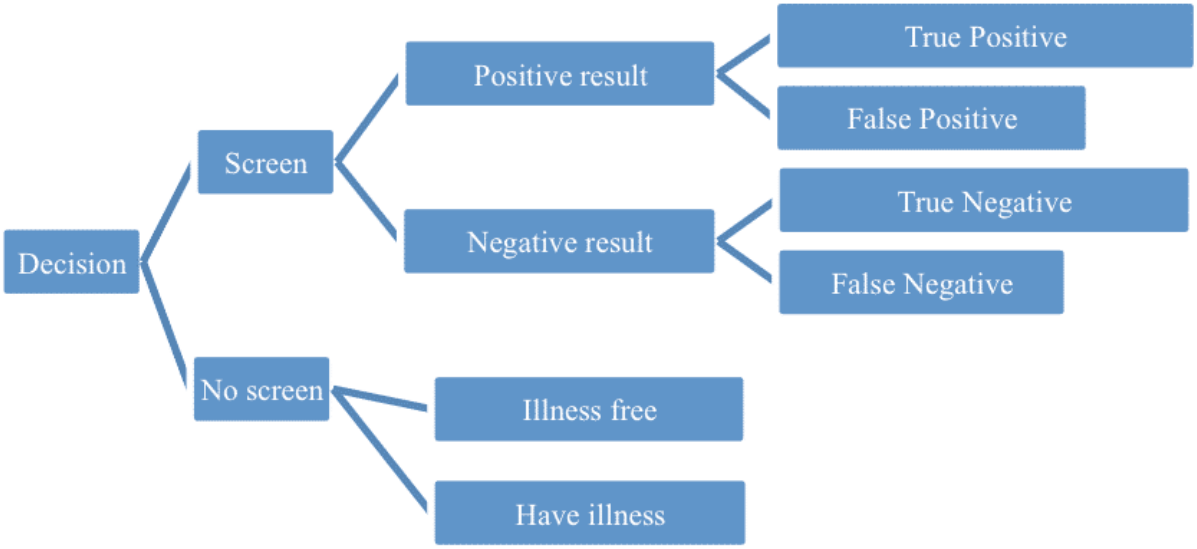
A decision tree is often a very useful way of exploring a decision problem. Building a decision tree involves describing all the potential outcomes of interest in a decision problem by means of a branching structure with probabilities associated to each branch.

As such, a decision tree model:

- includes a visual representation of all the possible decisions and the consequences that may follow each decision
- has a branching structure, with each branch representing an event that may take place in the future

Essential steps in building a decision tree model include identifying all alternatives and specifying the sequence and linkage of events.

Decision trees are particularly suited to exploring screening or preventative interventions or for interventions in acute care. An example of a decision tree relating to the decision whether to screen for an illness is described in the following graphic:

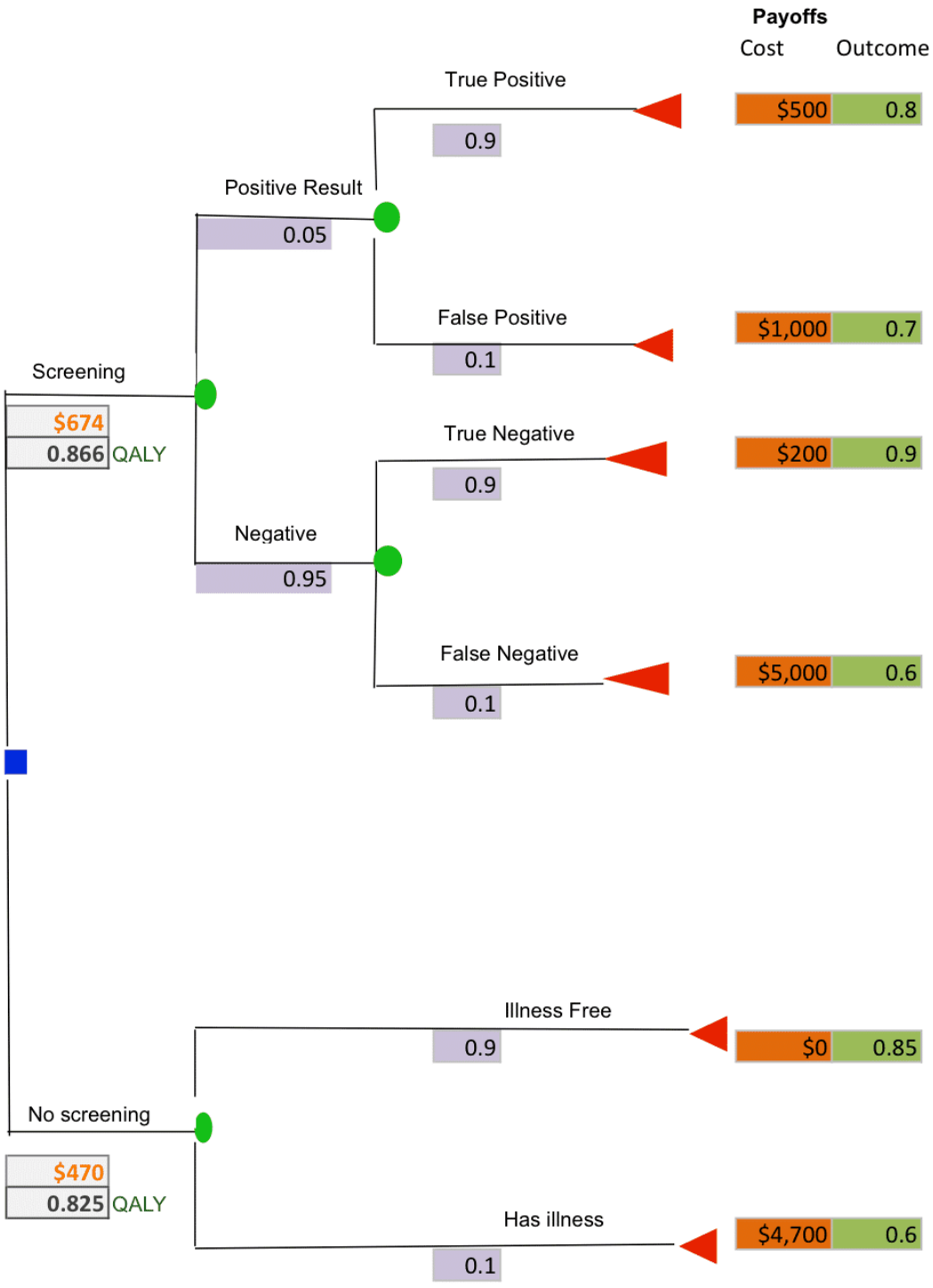


The above diagram describes both options—screening and no screening. If a screen is applied, there will either be a positive result or a negative result. If the result is positive, it will be either a false positive or a true positive. In the no screening branch, the individual screened will either have the illness or not have the illness.

Having described the logic, the next step is to input the probabilities of each event occurring and the payoffs in terms of costs and benefits of each outcome. Once these are inputted, we will be able to apply the probabilities to the payoffs to get an expected value for each decision option.

The example below outlines how the probabilities and payoffs can be represented in a decision tree diagram. The expected value of each arm is calculated by multiplying probabilities and payoffs together. For example, the expected value of the costs of the screening arm are:

$674 = 0.05*0.9*$500+0.05*0.1*$1000+0.95*0.9*$200+0.95*0.1*$5000


Payoffs
Cost
Outcome
True Positive
0.9
$500
0.8
Positive Result
0.05
False Positive
0.1
$1,000
0.7
Screening
$674
0.866 QALY
True Negative
0.9
$200
0.9
Negative
0.95
False Negative
0.1
$5,000
0.6
Illness Free
0.9
$0
0.85
No screening
$470
0.825 QALY
Has illness
0.1
$4,700
0.6

We would perform a similar exercise to calculate the expected value of costs and outcomes for the no screen branch. We can then compare the two results by calculating the ICER (incremental costs/incremental benefits) and comparing it to the WTP threshold that applies to our context.

3. State Transition Models

One area where a decision tree is not so appropriate is in modeling the management of chronic disease. In these circumstances, it is likely to be important to capture how an illness changes in nature and severity over time, as these may significantly impact cost and outcomes.

If capturing how costs and outcomes change over time is important to us, we might therefore choose to use a state transition model instead of a decision tree. An example of a state transition model is a Markov model, which shares some common features with a decision tree, but is distinctive in that a Markov model:

Jumps

Jumps forward in discrete steps of defined periods of time e.g. n = weekly cycles or monthly cycles or three monthly cycles.

Identifies

Identifies the health state, that the hypothetical patient is assumed to be in for the duration of each cycle (based on the health state of the patient during the preceding cycle, the potential health states that the patient would be allowed to transition to from that preceding health state and the probability of each allowable transition).

Calculates

Calculates the costs and benefits associated with each cycle based on the health state the patient is identified as being in for that cycle.

Continues

Continues the above process until the model time horizon is reached or the patient dies, and then calculates the (discounted) totals of costs and benefits for each patient for the model time horizon.

4 Sources of Uncertainty in Decision Analytic Models

One of the key steps in building and interpreting a decision analytic model is around the handling of uncertainty. There are many different ways uncertainty can be introduced into a model. Sources of uncertainty in decision analytic models include: (click each one to learn more)

Heterogeneity

The characteristics (e.g. gender, age, health behaviours, etc.) of different individuals may lead them to have different cost and benefit outcomes.

Structural

Decisions of analyst building the model will influence results e.g. model logic, the data sources selected (or not selected) to estimate key parameters. For example, you may have chosen a data source that estimates the treatment effect of the intervention as being greater than an alternative data source. Using the higher estimate will obviously make the cost-effectiveness results of the intervention look better than if the other data source was used.

Parameter

Estimates for each parameter (e.g. the size of treatment effect, unit cost) may be different than real value. Remember, estimates are derived from samples rather than the whole population. Each parameter estimate is thus best represented not as a one point estimate but as a measure that captures the uncertainty of the estimate (e.g. an estimated mean and the standard error of that estimate).

Stochastic

Random variation/luck of the draw. Even the same patient in exactly the same conditions may not get the same outcome every time.

5. Sensitivity Analysis

In order to explore the uncertainty in a decision analytic model, we run a number of different sensitivity analysis. The two main types of sensitivity analysis are:

Deterministic Sensitivity Analyses (DSA)

Frequently called one-way or two-way sensitivity analysis. Under DSA, we rerun the model, varying parameter values one or two at a time to see which parameters (or combination of parameters) contribute most to the model's uncertainty.

Probabilistic Sensitivity Analysis (PSA)

In a PSA, we will run the model many times (1000s). At each model run, every parameter is estimated from its underlying distribution (i.e. each parameter can take on many potential values from a fixed range). The range of results from all the model runs will give a picture for the underlying uncertainty from all parameters and—in individual patient simulations—from the heterogeneity of the patient population.

Although PSA gives us an overall picture of the underlying uncertainty in the model, one thing that deterministic sensitivity analysis is very useful for is identifying which specific parameters are driving the uncertainty in the model. This information is beneficial as it identifies the parameters that we may need to pay most attention to when doing additional analysis.

Exercises and Further Reading

Self-assessment and critical review exercises

Read the methods and sensitivity analysis sections of the modeling sample paper that was assigned to you. Is the model logic clear? Are all key data inputs to the model clearly outlined (e.g. by use of tables)? Was DSA, PSA, or both DSA and PSA approaches taken to sensitivity analysis?

Your self-assessment task is as follows:

- Undertake Self-Assessment Exercise 6: Decision Analytic Models

Self-Assessment Exercise 6: Decision Analytic Models

Your task in this exercise is to complete a decision tree model:

1. Calculate the point estimates associated with the data provided in the supporting Excel file (complete the green cells in the Excel file).

- What is the expected value of benefits in the screening arm of the model?
- What is the expected value of costs in the screening arm of the model?
- What is the expected value of benefits in the no screening arm of the model?

- What is the expected value of costs in the no screening arm of the model?
- What are the incremental costs, benefits, and ICER?

2. Calculate the value of the deterministic sensitivity analyses outlined in the sheet named "Sensitivity Analysis."

- For **each sensitivity analysis**, use the green cells (columns H to AM) to calculate Intervention Costs, Intervention Effects, Control Costs, Control Effects, Incremental Costs, Incremental Effects, and Incremental Cost-Effectiveness Ratio. (Hint: Look at the formulae used in column A47:A43 and copy them to corresponding cells for each sensitivity analysis, making sure that the formulae have updated to refer only to cells in its new column.)
- Examine the tornado diagram to identify which sensitivity analyses have highlighted the parameters with the most underlying uncertainty.

Excel file for Self-Assessment Exercise 6: Decision Analytic Models

XLS

Self-Assessment Exercise 6 - Decision Analytic Models.xlsx

25.6 KB

Solution for Self-Assessment Exercise 6

XLS

Self-Assessment Exercise 6 - Decision Analytic Models Solutions.xlsx

28.5 KB

References and Further Optional Reading

If you would like to do further optional reading about the topic, you may wish to consider the following resources:

- A Briggs, M Weinstein, E Fenwick, J Karnon, M Sculpher, and A Paltiel. "Model Parameter Estimation and Uncertainty: A Report of the ISPOR-SMDM Modeling Good Research Practices Task Force-6." 2012, Value in Health.
- A Briggs, M Weinstein, E Fenwick, et al. Model parameter estimation and uncertainty analysis: A report of the ISPOR-SMDM Modeling Good Research Practices Task Force-6. Value Health, 2012;15(6):835–842.

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Module 4 of 4

Preference Elicitation

LO Lynn O'Keeffe

Welcome to the Unit Four of Module Three, which is Preference Elicitation.

Unit Objectives

The goals of this unit are to:

- understand the rationale for eliciting preferences
- identify alternative approaches to determining preferences
- describe key aspects of the discrete choice experiment stated preference technique

Unit Topics

The topics that will be covered in this unit are:

1. Rationale for preference elicitation
2. Stated and revealed preference methods
3. Discrete choice experiments
4. Key steps in conducting discrete choice experiment
5. Contingent valuation

Video Presentation

Here's the video presentation for this unit:

1. Rationale for Preference Elicitation

Rationale

The rationale for eliciting preferences can include:

- Preferences may be required for the **derivation of the measure of benefit used in an economic evaluation.** Utility-based measures such as the QALY incorporate preference weights, i.e. the value placed on a health state by members of a defined population (normally patients, clinicians or society). If we don't assess the preferences of these populations, we are not able to derive utility weights.

- Preferences may need to be elicited in order **to derive the WTP values that are essential to the decision criteria in CEA/CUA studies** or for calculation of net benefit in CBA studies. Similarly, the willingness to pay threshold values for cost-effectiveness are preference measures. We need to capture the willingness to pay values from a relevant population (i.e. consistent with our study perspective) in order to be able to derive the threshold value we use to determine cost-effectiveness.
- We may wish to know more about the preferences of a target population in order **to ensure product/service development and/or pricing strategies better align with preferences of clients.**

2. Stated and Revealed Preference Methods

Ideally, we would derive data about preferences by studying people's actual choices. Such analysis of **revealed preferences** might be based on examining:

(Click each to learn more)

Consumption

How much of a product/service that is traded on the open market is purchased and at what price?

Travel	How much time are people prepared to spend traveling in order to avail of a service/amenity?
Risk premiums	The additional salaries that individuals demand for undertaking occupations with higher risks of injury or death.

However, as emphasized in the introductory unit, health is not a market-based good, so instead of using revealed preference techniques, we may need to rely on **stated preference** techniques, which essentially means asking people about their preferences.

Two common stated preference approaches are:

- discrete choice experiments
- contingent valuation

3. Discrete Choice Experiments

Economists tend to prefer to derive values through analyzing people's choices. In the absence of observing people's real world choices, we can use a discrete choice experiment (DCE) to present people with hypothetical choices and analyze these instead.

A discrete choice experiment:

- **assumes that good/services/outcomes can be described by their attributes** and that the value individuals place on the good/service/outcome is derived from the relative levels of these attributes.
- **provides participants with a series of hypothetical choices between options described as a collection of attributes** (e.g. location, service duration, effectiveness, price), with a range of potential levels (often measured on scales, e.g. completely satisfied to completely dissatisfied).
- shows by **varying the levels of attributes in each choice set**, it is possible to analyze participant's answers to **determine the relative strength of their preferences for each attribute.**

4. Key Steps in Conducting Discrete Choice Experiments

There are key steps in conducting a Discrete Choice Experiment:

1

Identify the attributes that are expected to contribute to the value derived from something. For example, the EQ5D identifies 5 attributes that comprise health-related quality of life (e.g. ability to participate in usual activities, anxiety/depression, mobility, pain, and self care). At least one of the attributes needs to be a quantitative attribute (e.g. time or money) as this will be used to calculate a marginal rate of substitution between attributes.

2

Identify the levels appropriate to each attribute. For example, a temperature attribute could be described by a binary choice (hot or cold) or with numeric values (0, 8, 15, 25, and 35 degrees).

3

Identify the appropriate number of choice sets to present to participants (e.g. should we ask 15 A or B questions or 25? Answering this either involves quite a lot of fancy mathematics or looking up values in DCE design catalogues).

4

Pilot the survey with a sample of potential users.

5

Refine on the basis of pilot feedback.

6

Administer survey to survey participants.

7

Analyze results (using a number of potential regression-based techniques) to determine preference weights.

5. Contingent Valuation

Another type of stated preference technique is contingent valuation. Contingent valuation is frequently used in areas such as environmental economics and takes the form of a highly structured survey in which participants are provided with a significant amount of information and asked to provide a willingness-to-pay value (WTP) for a particular good or service.

Contingent valuation has specific features:

- Unlike DCEs, contingent valuation asks participants to value the outcome/experience/product/service as a whole, not as the sum of its parts (attributes)
- Participants are given a description of a scenario (details about what they are being asked to value)
- Participants are also presented with how payment for the outcome/experience/product/service would be made (a new tax, a once off fee/donation, a recurring charge)
- Valuation can be elicited by an open-ended question, yes/no choices, picking from a list of options, or a simulated iterative bidding process

Exercises and Further Reading

Self-assessment and critical review exercises

There are no self-assessment or critical review exercises to undertake for this module.

References and Further Optional Reading

If you would like to do further optional reading about the topic, you may wish to consider the following resources:

- J Bridges, A Hauber, D Marshall, A Lloyd, L Prosser, D Regier, F Johnson, and J Mauskopf. "Conjoint Analysis Applications in Health—a Checklist: A Report of the ISPOR Good Research Practices for Conjoint Analysis Task Force. 2011", Value in Health.
- World Health Organization. "How to conduct a discrete choice experiment for Health Workforce Recruitment and Retention in Remote and Rural Areas: A User Guide with Case Studies" 2012, World Health Organization.

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