

RESEARCH PROPOSAL FOR AN ECONOMIC EVALUATION STUDY

Objectives

This group assignment provides an opportunity for teamwork and reflection on material studied during the course. The objective is to develop a simplified proposal for an economic evaluation study of a chosen health technology and setting. This requires making choices regarding the study design and framework. Health professionals confronted with an economic evaluation, need to make such choices and the assignment demonstrates how this task can be approached. The application of material thought during the lectures is at the core of this assignment. There will be a possibility to consult a lecturer during the proposal preparation phase. The group will report the outcomes in the form of a presentation and will receive feedback from lecturers and other groups.

Background

The relevance of an economic evaluation for policymaking highly depends on the chosen 'framework' of that particular economic evaluation. The framing of an economic evaluation is an important step in the design phase and considers the making of a series of motivated choices which together define and describe the economic evaluation. In this phase, one has to think about the objective, audience, perspective, time horizon, technology of interest, target population, and comparative technologies. The choices made in framing an economic evaluation set the boundaries of the economic evaluation and determine which type of economic evaluation and which design (i.e. trial-based (TBEE) or model-based economic evaluation (MBEE)) is the most appropriate to address the decision problem. The framework of the economic evaluation determines which data should be collected and how these data should be analysed, presented and interpreted. Ultimately the framework of the economic evaluation determines if and to what degree results will be useful and relevant for policymaking. Choosing a 'wrong' framing may lead to inefficient decisions or not using cost-effectiveness results at all.

Group Task

Course participants work in groups of ca. 5 participants (max 6 students). The group chooses a health care setting and health technology to assess. It is possible to take a case from own practice. The group task is to develop a research proposal for a trial-based or model-based economic evaluation of the chosen health technology for the given setting. The proposed economic evaluation study should have a *societal perspective*, which contains at least a *cost-effectiveness analysis* and/or a *cost-utility analysis*. The final product of the group work is a PowerPoint presentation of the economic evaluation study proposed by the group. During the course lectures, different aspects of the design of an economic evaluation will be addressed. The group need to use this new knowledge to prepare the research proposal.

To frame the proposal, the group should first discuss and/or agree on: (1) study objective, (2) audience, (3) societal perspective, (4) time horizon, (5) technology of interest, (6) target population, (7) comparative technologies, (8) type of economic evaluation, (8) study design, (9) data collection, (10) data analysis.

The CHEC-list shown below can also be used by the group to ensure the completeness of the proposal. The CHEC-list is based on expert consensus. It consists of 19 yes-or-no questions, one for each category and only focuses on the methodological quality of economic evaluation studies. Appendix A provides instructions about the CHEC-list.

	CHEC-list	YES	NO
1.	Is the study population clearly described?		
2.	Are competing alternatives clearly described?		
3.	Is a well-defined research question posed in answerable form?		
4.	Is the economic study design appropriate to the stated objective?		
5.	Is the chosen time horizon appropriate in order to include relevant costs and consequences?		
6.	Is the actual perspective chosen appropriate?		
7.	Are all important and relevant costs for each alternative identified?		
8.	Are all costs measured appropriately in physical units?		
9.	Are costs valued appropriately?		
10.	Are all important and relevant outcomes for each alternative identified?		
11.	Are all outcomes measured appropriately?		
12.	Are outcomes valued appropriately?		
13.	Is an incremental analysis of costs and outcomes of alternatives performed?		
14.	Are all future costs and outcomes discounted appropriately?		
15.	Are all important variables, whose values are uncertain, appropriately subjected to sensitivity analysis?		
16.	Do the conclusions follow from the data reported?		
17.	Does the study discuss the generalizability of the results to other settings and patient/client groups?		
18.	Does the article indicate that there is no potential conflict of interest of study researcher(s) and funder(s)?		
19.	Are ethical and distributional issues discussed appropriately?		

Presentation Content

The number of slides below and their order and content are an indication and can be adapted if the group finds that appropriate.

TITLE SLIDE

Title and group members

- Make sure that the relevant elements are in the title (see e.g. CHEC checklist)
- Include the name of each group member

SLIDE 1: Characteristics of the disease

- Short explanation of the disease
- Incidence and prevalence
- Mortality, morbidity, quality of life
- Costs (in monetary value), the burden to society (in QALY)

SLIDE 2: Characteristics of patients

- Age, gender
- Cultural background

SLIDE 3: Interventions and comparators

- What is the background problem you want to solve
- Available interventions
- Important health and/or cost consequences of these interventions
- Which interventions will be compared in the economic evaluation and why?

SLIDE 4: Aim/research question

- Answerable research question (Use PICOT, include perspective, target group, and intervention and comparator, time horizon)
- Expected efficiency based on evidence (hypothesis)

SLIDE 5: Overall framing

- Objective of economic evaluation (e.g. reimbursement, standardisation of care)
- Relevant audience(s); to whom will the results be of importance?
- Time horizon and perspective
- Target group (in and exclusion criteria e.g. age, gender, disease severity, co-morbidities)
- Type of economic evaluation (CEA, CUA, and optional CMA, CBA)
- Definition of incremental cost-effectiveness ratio (ICER)
- Country and centres included (look at the relevant guidelines for that country)

- Analytical approach, i.e. TBEE/MBEE (and why) and specifics for the design
- Data sources and collection

SLIDE 6: Costs

- Identification of relevant cost categories (use a formal classification and societal perspective)
- Instrument used for measurement of cost categories
- Valuation; sources for of cost prices (guidelines for costing)
- Valuation; which method to use for paid work, unpaid work, informal care, etc.
- Chosen cost price year and why
- Discounting, look at guidelines

SLIDE 7: Outcome measurement

- Primary and secondary outcomes
- Selected outcomes for effectiveness and utilities, and why
- Instruments used for outcomes, and why
- Valuation method to derive utilities (instruments, population)

SLIDE 8: Synthesising costs and effects

- Calculation of ICER/ICUR
- Interpretation of ICER; what does it mean, comparable studies

SLIDE 9: Uncertainty

- Investigating uncertainty & diversity: (which sensitivity / scenario / subgroup analysis)
- CEA plane, CEAC curve
- Chosen threshold for the WTP of CUA and why

SLIDE 10: Discussion of the expected outcomes

- Expected most important outcome(s) of study, based on the literature
- Relationship of results with existing evidence
- Limitations of design economic evaluation (bias)
- Generalisability/transferability of the results to other settings and/or patient groups
- Other relevant considerations to decision-maker
- Issues of implementation
- Expected recommendations, and results based on this study

Other Requirements

The group presentation should be maximum 15 minutes. As a rule of thumb, ca.1.5 min per slide, about 10 slides, could be considered. After the presentation, there will be 10 minutes for discussion and questions. The group can decide who should give the presentation. No need for all group members to present. It is possible that the group chooses to appoint 1-2 members to give the presentation. Nevertheless, all group members should participate in the preparation. It is recommended to use a formal design, and make sure that the presentation is attractive, e.g. by using transitions and animations or using other visual aids to clarify and reinforce the spoken message. Interaction with the audience during the presentation is recommended. The presentation of a research proposal is an academic presentation, and references could be added as a footnote in the presentation slides.

Teamwork

It is essential that the group makes a list of tasks and fairly distribute those tasks among the group members. Group members should give and receive constructive feedback, listen and ask for clarification, and be open to change.

Support

The group works on the assignment during the “practical assignment” meetings indicated in the timetable. The group works independently but a lecturer is present to advise the group when needed and help with answering questions. The overall tasks per meeting can be found in the table below. Group members should also work on their individual tasks outside the scheduled hours.

	Practical assignment meeting 1 Mon 10-Oct 14.00-15.00 CEST	Practical assignment meeting 2 Tue 11-Oct 14.00-15.00 CEST	Practical assignment meeting 3 Thu 13-Oct 14.00-15.30 CEST
	Tasks: Discussion of the assignment, division of work, start preparing slides 1 to 5	Tasks: Reporting on progress, discussion of questions, start preparing slides 6 to 10	Tasks: Presentations and feedback
	Lecturers	Lecturers	Lecturers
Group 1	Bosiljka Djikanović	Bosiljka Djikanović	Bosiljka Djikanović Enkeleint Aggelos Mechili Wim Groot (online)
Group 2	Tomasz Bochenek	Tomasz Bochenek	
Group 3	Wim Groot (online)	Wim Groot (online)	
Group 4	Rositsa Koleva-Kolarova	Rositsa Koleva-Kolarova	Rositsa Koleva-Kolarova Kostas Athanasakis (online if possible) Silvia Evers (online if possible)
Group 5	Silvia Evers	Silvia Evers	
Group 6	Lorena Dini	Lorena Dini	Lorena Dini Elena Petelos Ciaran O'Neill (online if possible)
Group 7	Ciaran O'Neill	Ciaran O'Neill	

Assessment

Each presentation will be assessed by the lecturers and other groups present during the presentation. The assessment form is provided below.

ASSESSMENT FORM GROUP ASSIGNMENT

Group number: _____

Criteria	--/-/+/-/++	Remarks
1. Adequacy of the structure of the presentation		
2. Definition of the research questions		
3. Adequacy of framing <ul style="list-style-type: none"> • Fitting to research question and decision context • Definitions well supported with literature 		
4. Adequate identification, measurement and valuation of costs <ul style="list-style-type: none"> • Fitting to research question and framing • Argumentation well justified based on literature 		
5. Adequate identification, measurement and valuation of outcome <ul style="list-style-type: none"> • Fitting to research question and framing • Argumentation well justified based on literature 		
6. Combining of cost and effects <ul style="list-style-type: none"> • Fitting to the type of economic evaluation 		
7. Uncertainty and sensitivity analysis <ul style="list-style-type: none"> • Fitting to the type of economic evaluation and analytical approach 		
8. Discussion of the strengths and limitations and risk of bias		
9. Discussion of generalisability/transferability to other settings/patient groups.		
10. Adequate referencing style		

Appendix A: Assessment instruction for CHEC-list

The CHEC-list consists of 19 yes-or-no questions one for each category. In some cases insufficient information is available in the article, or in other published material. In those cases the assessor has to tick 'no'. The assessor should state 'yes' if they agree that the study paid sufficient attention to a certain aspect. To help the assessor when filling out the CHEC-list an explanation of the meaning of each item is given in below.

1. The relevant clinical characteristics, entry and eligibility criteria, as well as drop-out during follow-up should be stated explicitly.
2. A detailed description should be given of the competing interventions. This should encompass a clear and specific statement of the primary objective of each alternative, as well as relevant factors, such as intensity, duration, and frequency.
3. A research question has to identify clearly the alternatives being compared and the population for which the comparison is made.
4. An appropriate economic study design is a full economic evaluation (comparison of costs and effects of 2 or more interventions) based on primary research (cohort, case-control, randomised controlled trial).
5. The period of analysis of the study is the time horizon. This time horizon should always be equal for costs and outcomes if these are combined in a ratio. The time span should be long enough to include all relevant costs and outcomes relating the intervention. Ideally, the follow-up period should be extended till the situation is stabilised with reference to costs and effects.
6. 'Perspective' indicates from which point of view an economic evaluation study is performed. If the study is performed from a societal perspective tick 'yes', as all relevant costs and consequences of an interventions and disease are taken into account, if possible. Other narrower perspectives will only include certain components. The authors should motivate why a narrower perspective is valid.
7. A full identification of all important and relevant costs should be given in relation to the perspective and the research question.
8. The costs should be measured appropriately in physical units. The instrument by which the costs are measured should be valid and clearly stated (e.g. interview, questionnaire, cost-diary).
9. The sources of valuation should be clearly stated for each cost price of every volume parameter and their reference year. The main cost should be calculated based on depleted sources, no tariffs should be used.
10. A full identification of all important and relevant outcomes should be given in relation to the perspective and the research question.

11. The outcome measurement should result from the outcome identification and this should be straightforward (e.g. if mortality is a main outcome measure this should be taken into account in the analysis). The instrument by which the outcomes are measured should be valid and clearly stated.
12. The method of outcome valuation should be clearly stated. Examples of valuation methods are Discrete Choice Experiments (e.g. Conjoint analysis, Contingent valuation), Direct utility assessment (VAS, TTO, SG, etc.), Indirect utility assessment (HUI, EQ-5D, QWB, etc.), Person trade off, etc.
13. An incremental analysis should examine the additional costs from one intervention over another, compared to the additional outcomes that it delivers. The incremental costs-effectiveness ratio is obtained by dividing the costs differences ($C_2 - C_1$) by the outcome differences ($O_2 - O_1$) for the alternatives.
14. Discounting is done appropriately if all costs and outcomes are converted to one single year, based on a motivated discount rate.
15. All variables in the analysis are potential candidates for the sensitivity analysis. Only variables that are certain or which have a minimal impact on the study results (based on the preliminary analysis) can be excluded from the sensitivity analysis. Furthermore, a justification should be given over the range of the variables used in the sensitivity analysis.
16. Do the authors interpret their results cautiously and are their conclusions justified by the data.
17. This can be done by being explicit about the viewpoint of analysis and by indicating how particular costs and outcomes vary by location, setting, patient population, care provider, etc.
18. If an external agency finances the study, a statement should explicitly be given about who finances the study to guarantee transparency in the relationship between the sponsor and the researcher. Whenever a potential conflict of interest is possible a declaration should be given of 'competing interest'.
19. Does the article notes ethical aspects and elaborates on the characteristics of the population experiencing the disease or the intervention (young, old, poor, wealthy) and how this may have distributional implications.