SPPH 581Q: Economic Evaluation in Health Care – part 2

The University of British Columbia
School of Population and Public Health

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Calendar Entry: 2015/16 Winter Term 2
Credits: 3

General Course Description: This course directly follows on from SPPH 541, and provides students with the opportunity to gain in-depth and practical skills in conducting economic evaluation of health technologies. In addition, the course considers, in detail, the policy context for the use of economic evaluation, drawing on experiences and evidence from both Canadian and international settings. Finally, current methodological and theoretical debates are presented, supplementing the topics already discussed in SPPH 541.

Prerequisites: SPPH 541 (or equivalent training)

Corequisites: None

Background: Biomedical science is progressing at a rapid pace and available health-care technologies to prevent, treat, or control diseases are becoming more numerous, complex, and expensive. Concordantly, decision makers face the challenge of maximizing the benefit obtained from the dollars spent in treatments, as well as in research. Over recent years, the theoretical and methodological framework underpinning economic evaluations has evolved to cope with such challenges and complexities. This course will cover, in depth, the methodological aspects of economic evaluation applied to health-care topics and provide opportunities for practical, hands-on skill development. This course is aimed at those who wish to be able to perform 'production level' economic evaluations suitable for publication in peer-reviewed journals or reports to decision makers at government agencies or stakeholders in the industry.

This course is a direct continuation of SPPH 541 and students are expected to be familiar with the content of that course. Knowledge of statistics, epidemiology, and some basics of health economics are also required.

Format: The course will be a combination of lecture presentations, interactive practical modeling sessions, assignments and individual projects. The topics for each session are provided below. Generally, the content of the course can be divided into three sections: (i) in-depth coverage of applied techniques of conducting economic evaluations; (ii) policy context for use of economic evaluation; and (iii) theory and current methodological debates.

Objectives:
As a result of this course, students will be able to:
- Develop a deep understanding of the available methodologies for conducting economic evaluation of health-care technologies, including an ability to distinguish clearly between decision modeling and data-driven paradigms

- Perform economic evaluation analyses, suitable for publication in peer-reviewed journals, using both decision modeling and data-driven approaches
- Evaluate and report on the extent of uncertainty in the analysis results
- Understand the challenges involved in the policy use of economic evaluation and identify strategies for overcoming the barriers, drawing on international experiences
- Take part in informed discussions of some of the key methodological issues currently being debated in the health economics literature

Evaluation:
Class participation (20%)
Midterm (40%)
Final assignment (40%)

Students will be assigned a mark for their willingness to participate in class and online discussion and the degree to which their participation enhances discussion in the class.

Text book: Briggs AH, Claxton K, Sculpher MJ. Decision Modeling for Health Economic Evaluation. Oxford University Press; 2006. ISBN: 0198526628, 9780198526629 (1)

Course Outline

Theory and recap of methods	
Session 1 <SB> 2016/01/07	The theoretical basis of economic evaluation in health care **Key references:** • (2) Hurley J (2010) Health Economics. First Edition. McGraw-Hill Ryerson. Chapter 3, ISBN-10: 0070916489, ISBN-13: 978-0070916487 • (3) Gold MR et al (1996) Cost-Effectiveness in Health and Medicine. OUP. Chapters 2, 6 & 7, ISBN-10: 0195108248, ISBN-13: 978-0195108248 **Objectives**: - To develop an understanding of the theory relevant to economic evaluation in health care - To be able to describe the strengths and weaknesses of economic analysis approaches from the perspective of theoretical underpinnings
Session 2 <SB> 2016/01/14	A review of the methods of economic evaluation in health care (recap of material covered in SPPH 541) **Key references:** • (4) Drummond MF et al (2005) Methods for the Economic Evaluation of Health Care Programmes. OUP. Third Edition. ISBN-10: 0198529457, ISBN-13: 978-0198529453 • (2) Hurley J (2010) Health Economics. First Edition. McGraw-Hill Ryerson. Chapter 4, ISBN-10: 0070916489, ISBN-13: 978-0070916487 **Objectives**: - To recap and refresh understanding of different approaches to economic evaluation in health care - To be able to identify when each approach should be employed and the benefits associated with each approach

Applied model-based economic evaluation methods

Model-based evaluations

| Session 2 (cont.) <SB>

2016/01/14 | Alternative modelling approaches (with focus on Markov models and discussion of discrete event simulation, individual sampling models, etc.) and model selection
Key references:
• (5) Modeling in the economic evaluation of health-care: selecting the appropriate approach. J Health Serv Res Policy. 2004 Apr;9(2):110-8. PMID 15099459
• (6) A taxonomy of model structures for economic evaluation of health technologies. Health Econ. 2006 Dec;15(12):1295-310. PMID 16941543
• (7) Conceptualizing a Model: A Report of the ISPOR-SMDM Modeling Good Research Practices Task Force–2. Value Health. 2012 Sep-Oct;15(6):804-11. PMID 22999129
Objectives:
- To develop a deep understanding of different modeling frameworks for decision analysis.
- To be able to categorize a modeling framework according to cohort/individual, interactive/non-interactive, and discrete/continuous time
- To enumerate the advantages and disadvantages of cohort versus individual models. |

	- To list some of the available software platforms for decision analysis.
Session 3, 4 and 5 <MS> 2016/01/21 2016/01/28 2016/02/04	Model building (with exercises) This will be a very practical session with presenting a case study and building a model from scratch. **Key references:** (1) Chapters 2 and 3 of the text book (8) Model Parameter Estimation and Uncertainty Analysis: A Report of the ISPOR-SMDM Modeling Good Research Practices Task Force Working Group–6. Med Decis Making. 2012 Sep-Oct;32(5):722-32. PMID 22990087 **Objectives**: - To create a real-world decision tree and Markov model in Microsoft Excel, and describe the strength and shortcomings of such a choice for modeling platform. - To develop an understanding of a model, input and output parameters, model structure, and the relation between modeling and real-world evidence (evidence synthesis) - To understand the key concepts underlying Markov models (state probabilities, transition matrices, payoffs, time-dependency) - To develop a deep understanding of the first-level, second-level, and modeling uncertainty - To learn key concepts of probability calculations (conditional vs. marginal probabilities, calculating probabilities from rates) - To learn about Monte Carlo simulation - To learn about typical and practical paradigms for evidence synthesis
Policy context for use of economic evaluation	
Session 6 <SB> 2016/02/11	The use of economic evaluation in policy making: an exploration of conceptual and practical issues, including the search for a cost-effectiveness threshold **Key references:** (4)Drummond MF et al (2005) Methods for the Economic Evaluation of Health Care Programmes. OUP. Third Edition. ISBN-10: 0198529457, ISBN-13: 978-0198529453. Chapter 10 (9)Bryan S, Williams I, McIver S. Seeing the NICE side of cost-effectiveness analysis: a qualitative investigation of the use of CEA in NICE technology appraisals. Health Econ. 2007 Feb; 16 (2): 179-93. PubMed PMID: 16960851 (10)Devlin N, Parkin D. Does NICE have a cost-effectiveness threshold and what other factors influence its decisions? A binary choice analysis. Health Econ. 2004 May;13(5):437-52. **Objectives**: - To develop understanding of alternative approaches to presentation and use of economic evaluations in health care - To be familiar with empirical work on the use of economic analyses to inform policy decision making
Reading week	**Mid-term exam**

	Evidence from international experience in the use of economic evaluation: the good, the bad and the ugly…
Session 7 <SB> 2016/02/25	**Key references:** • (11)Tengs,T.O. An evaluation of Oregon's Medicaid Rationing Algorithms. *Health Economics* 1996;7:171-181. • (12)Clement FM, Harris A, Li JJ, Yong K, Lee KM, Manns BJ. Using effectiveness and cost-effectiveness to make drug coverage decisions: a comparison of Britain, Australia, and Canada. JAMA. 2009 Oct 7;302(13):1437-43. doi: 10.1001/jama.2009.1409. • (13)McCabe C, Claxton K, Culyer AJ. The NICE cost-effectiveness threshold: what it is and what that means. *PharmacoEconomics* 2008;26(9):733-44 • (14)Gafni A, Birch S. Incremental cost-effectiveness ratios (ICERs): the silence of the lambda. *Social Science & Medicine* 2006;62(9):2091-100. **Objectives**: - To review international experiences in using economic evaluations in health care - To understand the conceptual and empirical approaches to determining the cost-effectiveness threshold - To appreciate the debates on the appropriateness of a cost-effectiveness threshold

Data-driven evaluations, and value of information

	Uncertainty, probabilistic sensitivity analysis (PSA), and Expected Value of Information (EVI)
Session 8 <MS> 2016/03/03	**Key references:** • (15) Representing uncertainty: the role of cost-effectiveness acceptability curves. Health Econ. 2001 Dec;10(8):779-87. PMID 11747057 • (16) The irrelevance of inference: a decision-making approach to the stochastic evaluation of health care technologies. J Health Econ. 1999 Jun;18(3):341-64. PMID 10537899 • (17) Sensitivity analysis and the expected value of perfect information. Med Decis Making. 1998 Jan-Mar;18(1):95-109. PMID 9456214 **Objectives** - To learn how to estimate and interpret ICER under uncertainty - To learn how to construct and interpret credible intervals for the ICER - To learn how to construct and interpret cost-effectiveness plain and cost-effectiveness acceptability curve (CEAC) - To learn how to do PSA in Excel - To understand the concept and important metrics in EVI - To learn how to calculate EVPI from PSA data
Session 9 <MS>	Economic evaluation data sets: components, data collection, etc. **Key references:** • (18) Good Research Practices for Cost-Effectiveness Analysis Alongside Clinical Trials: The ISPOR RCT-CEA Task Force Report. Value Health. 2005 Sep-Oct;8(5):521-33. PMID 16176491 • (19) Experimental versus observational data in the economic evaluation of pharmaceuticals. Med Decis Making. 1998 Apr-Jun;18(2 Suppl):S12-8. PMID 9566462 • (20) Whither trial-based economic evaluation for health care decision making? Health Econ. 2006 Jul;15(7):677-87. PMID 16491461

2016/03/10	**Objectives:** - To understand the theoretical concepts underlying data-driven economic evaluations. - To understand the processed required for measuring cost and effectiveness outcomes alongside an RCT. - To understand the potential role of observational studies in economic evaluations. - To understand the challenges posed by non-random treatment assignments and the potential remedies in economic evaluations based on observational data. - To understand the trade-off between internal and external validity in data-driven economic evaluations.
Session 10 <MS> 2016/03/17	Analyses: Missing data, Bootstrapping, Bayesian perspective, incorporating external evidence **Key references:** (21) Pulling cost-effectiveness analysis up by its bootstraps: a non-parametric approach to confidence interval estimation. Health Econ. 1997 Jul-Aug;6(4):327-40. PMID 9285227 (22) Estimating the cost-effectiveness of fluticasone propionate for treating chronic obstructive pulmonary disease in the presence of missing data. Value Health. 2006 Jul-Aug;9(4):227-35. PMID 16903992 (23) Incorporating external evidence in trial-based cost-effectiveness analyses: the use of resampling methods. Trials. 2014 Jun 3;15:201. PMID:24888356 **Objectives** - To understand the theoretical challenges in obtaining unbiased estimate for cost and effectiveness outcomes in the presence of missing data and incomplete follow-up. - To compare and contrast the parametric and non-parametric approaches in inference on outcomes in an RCT-based economic evaluation - To understand the theoretical basis and practical aspects of the bootstrap for inference in data-driven CEAs.

Current debates (suggested topics but these will be selected each year to ensure that 'current' debates are reflected in the course)

Session 11 <SB> 2016/03/24	Measuring health-related quality of life for use in economic evaluation Critiques of the QALY; Capabilities theory and its implications for health economic evaluation
Session 12 <SB> 2016/03/31	'Indirect costs' and Productivity measurement and valuation
Session 13 <SB> 2016/04/07	Debates on discounting and time preference in health evaluation
Final assignment	

References

1. Briggs AH, Claxton K, Sculpher MJ. Decision Modelling for Health Economic Evaluation. Oxford University Press; 2006. 251 p.

2. Hurley J. Health Economics. Canadian edition. Toronto: McGraw-Hill Ryerson Higher Education; 2010. 448 p.

3. Gold MR, Siegel JE, Russell LB, Weinstein MC. Cost-Effectiveness in Health and Medicine. Oxford University Press; 1996. 462 p.

4. Drummond M, O'Brien B, Stoddart G, Torrance G. Methods for the Economic Evaluation of Health Care Programmes. United Kingdom: Oxford University Press; 2005.

5. Barton P, Bryan S, Robinson S. Modelling in the economic evaluation of health care: selecting the appropriate approach. J Health Serv Res Policy. 2004 Apr;9(2):110–8.

6. Brennan A, Chick SE, Davies R. A taxonomy of model structures for economic evaluation of health technologies. Health Economics. 2006;15(12):1295–310.

7. Roberts M, Russell LB, Paltiel AD, Chambers M, McEwan P, Krahn M, et al. Conceptualizing a model: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force--2. Value Health. 2012 Oct;15(6):804–11.

8. Briggs AH, Weinstein MC, Fenwick EAL, Karnon J, Sculpher MJ, Paltiel AD, et al. Model parameter estimation and uncertainty: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force--6. Value Health. 2012 Oct;15(6):835–42.

9. Bryan S, Williams I, McIver S. Seeing the NICE side of cost-effectiveness analysis: a qualitative investigation of the use of CEA in NICE technology appraisals. Health Econ. 2007 Feb;16(2):179–93.

10. Devlin N, Parkin D. Does NICE have a cost-effectiveness threshold and what other factors influence its decisions? A binary choice analysis. Health Econ. 2004 May;13(5):437–52.

11. Tengs TO. An evaluation of Oregon's Medicaid rationing algorithms. Health Econ. 1996 Jun;5(3):171–81.

12. Clement FM, Harris A, Li JJ, Yong K, Lee KM, Manns BJ. Using effectiveness and cost-effectiveness to make drug coverage decisions: a comparison of Britain, Australia, and Canada. JAMA. 2009 Oct 7;302(13):1437–43.

13. McCabe C, Claxton K, Culyer AJ. The NICE cost-effectiveness threshold: what it is and what that means. Pharmacoeconomics. 2008;26(9):733–44.

14. Gafni A, Birch S. Incremental cost-effectiveness ratios (ICERs): the silence of the lambda. Soc Sci Med. 2006 May;62(9):2091–100.

15. Fenwick E, Claxton K, Sculpher M. Representing uncertainty: the role of cost-effectiveness acceptability curves. Health Economics. 2001;10(8):779–87.

16. Claxton K. The irrelevance of inference: a decision-making approach to the stochastic evaluation of health care technologies. J Health Econ. 1999 Jun;18(3):341–64.

17. Felli J, Hazen G. Sensitivity analysis and the expected value of perfect information. Med Decis Making. 1998;18(1):95–109.

18. Ramsey S, Willke R, Briggs A, Brown R, Buxton M, Chawla A, et al. Good research practices for cost-effectiveness analysis alongside clinical trials; the ISPOR RCT-CEA Task Force report. Value in health. 2005;8(5):521–33.

19. Drummond MF. Experimental versus observational data in the economic evaluation of pharmaceuticals. Med Decis Making. 1998 Jun;18(2 Suppl):S12–8.

20. Sculpher M, Claxton K, Drummond M, McCabe C. Whither trial-based economic evaluation for health care decision making? Health Econ. 2006;15(7):677–87.

21. Briggs A, Wonderling D, Mooney C. Pulling cost-effectiveness analysis up by its bootstraps: a non-parametric approach to confidence interval estimation. Health Econ. 1997;6(4):327–40.

22. Briggs A, Lozano-Ortega G, Spencer S, Bale G, Spencer M, Burge P. Estimating the cost-effectiveness of fluticasone propionate for treating chronic obstructive pulmonary disease in the presence of missing data. Value in health. 2006;9(4):227–35.

23. Sadatsafavi M, Marra C, Aaron S, Bryan S. Incorporating external evidence in trial-based cost-effectiveness analyses: the use of resampling methods. Trials. 2014 Jun 3;15(1):201.