

#### **HEIDELBERG HEALTH ECONOMICS WINTER SCHOOL 2023**

#### **Program Outline:**

## **Heidelberg Health Economics Winter School 2023**

Studio Villa Bosch, February 06 – 10, 2023

## **International Faculty**

- ¬ Prof. Amanda Adler (London & Oxford)
- ¬ Dr. Richard Baird (Cambridge)
- ¬ Dr. Cinzia Brunelli (Milan)
- ¬ Dr. Andrew Bruce (Sydney, NSW)
- ¬ Prof. Heiner Bucher (Basel)
- ¬ Prof. Soren Holm (Manchester & Oslo)
- ¬ Dr. *Mohit Jain* (London)
- ¬ Dr. Gergő Merész (Budapest)
- ¬ Prof. Jorge Mestre-Ferrandiz (Madrid)
- ¬ Prof. Dan Ollendorf (Boston, MA)
- ¬ Prof. *Ulf Persson* (Lund)
- ¬ Prof. Maarten Postma (Groningen)
- ¬ Prof. Gérard de Pouvourville (Paris)
- ¬ Prof. Jeff Richardson (Melbourne, Vic)
- ¬ Prof. *Michael Schlander* (Heidelberg)
- Prof. Nils Wilking (Stockholm)

### **Scientific Program**

#### Module A

- Monday:
   Health Technology Assessment
   & Economic Evaluation
- Tuesday:
   Applied Health Economics:

   The Logic of Cost Effectiveness

#### ¬ Module B

- Wednesday:
   Objectives of Health Care
   Social Cost Value Analysis
- Thursday:
   Precision Medicine, Innovative
   R&D Strategies, & Evaluation
- ¬ Friday:
  The Perspective of Patients
  & Socioeconomic Impact Analysis

Please note that the present outline – although considered the "final program version" – may be subject to change and revision without notice. It represents the latest status of planning and preparation, as at January 27, 2023.

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#### **Program Overview**

#### Module A

A.1 Monday Health Technology Assessment (HTA) & Economic Evaluation:

Introduction to HTA and cost effectiveness analysis (CEA)

A.2 Tuesday Applied health economics: the logic of cost effectiveness

- a closer look

#### **Module B**

B.3 Wednesday Social objectives of health care, allocation and distribution

in economic evaluation, and Social Cost Value Analysis

B.4 Thursday New challenges in HTA and economic evaluation:

"precision medicine" and novel clinical trials designs

B.5 Friday Patients – still a broadly neglected stakeholder group?

The perspective of patients and Socioeconomic Impact Analysis

#### **Venue and Date**

Heidelberg, Studio Villa Bosch February 06 – February 10, 2023

## **Scientific Program Committee**

- ¬ Prof. Michael Schlander, University of Heidelberg and DKFZ, Germany (Chair)
- ¬ Prof. Jeffrey Richardson, Monash University, Melbourne, Australia
- Dr. Diego Hernandez Carreno, Anett Molnar, Muchandifunga Trust Muchadeyi, Ramon Schaefer, and Jasper Ubels, all University of Heidelberg and Division of Health Economics at the German Cancer Research Center (*Deutsches Krebsforschungszentrum*, DKFZ), Heidelberg, Germany

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## **Presenting Faculty**

- ¬ Prof. Amanda Adler,NICE, London, and Oxford University / UK
- Dr. Richard Baird,University of Cambridge / UK
- ¬ Dr. **Andrew Bruce**,
  Amgen, International Health Policy, Sydney, NSW / Australia
- Dr. Cinzia Brunelli,
   National Cancer Institute of Milan / Italy
- Prof. Heiner Bucher,
   University of Basel / Switzerland
- Dr. Diego Hernandez Carreno,
   DKFZ, Heidelberg / Germany
- Dr. Karla Hernandez-Villafuerte,
   wifor, Darmstadt, and DKFZ, Heidelberg / Germany
- ¬ Prof. **Søren Holm**,
  University of Manchester / UK and University of Oslo / Norway
- ¬ Dr. Mohit Jain / Dr. Thomas Butt,BioMarin, London / UK
- ¬ Dr. **Gergő Merész**,

  National Institute of Pharmacy and Nutrition, Budapest / Hungary
- Ass. Prof. Jorge Mestre-Ferrandiz,
   Universidad Carlos III de Madrid / Spain
- Anett Molnar,DKFZ & University of Heidelberg / Germany
- Muchandifunga Trust Muchadeyi,
   DKFZ & University of Heidelberg / Germany

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#### ¬ Prof. Daniel Ollendorf,

Tufts University School of Medicine, Boston / USA

¬ Prof. **Ulf Persson**,

Swedish Institute of Health Economics (IHE), Lund / Sweden

¬ Prof. Maarten Postma,

University of Groningen / The Netherlands

¬ Prof. Gérard de Pouvourville,

ESSEC Business School, Paris / France

¬ Prof. Jeffrey Richardson,

Monash University, Melbourne, Vic / Australia

¬ Ramon Schaefer,

DKFZ & University of Heidelberg / Germany

¬ Prof. Michael Schlander,

DKFZ & University of Heidelberg / Germany

¬ Jasper Ubels,

DKFZ & University of Heidelberg / Germany

¬ Prof. Nils Wilking,

Karolinska Institute, Stockholm / Sweden (online only)

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## **Objectives**

A major branch of health economics is concerned with the study of how scarce healthcare resources are allocated among competing health care programs and, by implication, among different groups in society.

The Winter School introduces to the theoretical underpinnings as well as the practical application and policy-relevance of conventional health economic evaluations, in particular in the context of Health Technology Assessments (HTAs) to inform health care decision makers in charge of allocating scarce resources.

Conventional cost effectiveness analyses (CEAs) are designed to help achieving the maximum potential health benefit given a health care resource (or budget) constraint. Some CEA results however are hard to reconcile with prevailing social norms and preferences. The Winter School program will address key underlying issues. Leading scholars of health economics will discuss with participants some of the major implications, potential solutions, and promising ways forward.

The Winter School will be comprised of two modules, which build upon each other but may also be attended separately. Together they will offer an overview of essential principles and methods, as well as limitations and some of the challenges posed by orphan medicines and novel cancer treatments.

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#### Module A – Days 1 and 2

Participants may also attend this module as a stand-alone subject.

The first two days provide an introduction to the discipline of health economics. This part of the Winter School will provide participants concerned with health care policy (e.g., academics, payers, industry, policy makers, and physicians) with an overview of the economics of health and health care. It will enable them to apply established analytical economic skills to problems of resource allocation in the health system.

After having attended the first two days, participants should

- understand the key objectives and methods of Health Technology
   Assessments (HTAs);
- be able to explain the fundamental principles and methods of economic analysis in health care;
- be familiar with the major concepts related to the identification and measurement of both health outcomes and costs;
- ¬ understand the use of willingness-to-pay, utility and capabilities in the valuation of health technologies;
- recognize the health economics techniques used to inform resource allocation and priority setting in the health system;
- be able to appreciate the role of economic evaluation in health care.

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#### Module B – Days 3 to 5

Participants may also attend this module as a stand-alone subject.

The program will be based on a mix of 'state of the art' lecture presentations and interactive discussion sessions with the faculty.

Days 3 - 5 will be dedicated to highlight current issues in the use of health economics to inform market access and reimbursement decisions, with a primary focus on

- ¬ the strengths and limitations of conventional CEAs, and potential alternatives such as extended cost per QALY calculations, multi-criteria decision analysis and social cost value analysis;
- the relevance and assessment of the perspective of patients, including the broader socioeconomic impact of diseases and the provision of medical care;
- sources of value and the importance of social norms and preferences, including their impact on the valuation of end-of-life treatments and orphan medicinal products;
- ¬ challenges posed by "precision medicine", including innovative clinical trial designs, biomarkers and companion diagnostics, advanced therapy medicinal products, and the "value of a cure";
- ¬ recent trends in medical research and development (R&D), including its economic dimension, implications for research prioritization, early value assessments and strategic R&D decision-making.

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#### **MODULE A**

## **INTRODUCTION**

**(1)** 

# **Health Technology Assessments** and Cost Effectiveness Analyses

Day 1:  Monday, February 06, 2023 [10:30am to 07:00pm]		
Welcome Reception and Registration	09:00am- 10:30am	
10. Introduction to Program & Objectives	[30 min] 10:30am- 11:00am	M. Schlander
11. Health Technology Assessments (HTAs) with and without health economic evaluation	[30 min] 11:00am – 11:30am	R. Schaefer
12. <b>The Logic of Health Economics (I)</b> The basics of cost effectiveness analysis (CEA)	[60 min] 11:30am – 12:30pm	D. Hernandez

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Lunch Break	1 hour 12:30pm - 01:30pm	
13. The Logic of Health Economics (II)  Modeling for cost effectiveness analysis (CEA)	60 min 01:30pm – 02:30pm	M.T. Muchadeyi & M. Schlander
14. <b>Group Work / Exercise</b> Conducting a stylized cost effectiveness analysis (CEA)	60 min 02:30pm – 03:30pm	All
15. <b>Group Work Presentations / Discussion</b> Interpretating the results of a CEA	<b>30 min</b> 03:30pm – 04:00pm	M.T. Muchadeyi & M. Schlander
Coffee Break	<b>30 min</b> 04:00pm – 04:30pm	
16. Value and Valuation in Health Economics  Cost benefit analysis (CBA) and CEA	<b>45 min</b> 04:30pm- 05:15pm	M. Schlander
17. <b>Group Work</b> What do you value? Case Study "The Kidney Machine"	60 min 5:15pm – 6:15pm	All
18. <b>Group Work Presentations / Discussion</b> Resource allocation decision-making	<b>45 min</b> 6:15pm – 7:00pm	All / M. Schlander

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### **MODULE A**

## **INTRODUCTION**

**(2)** 

### HTAs & Cost Effectiveness (CE):

### A Closer Look

Day 2: Tuesday, February 07, 2023 [09:00am to 07:00pm]		
21. The Logic of CE (III): Normative Foundations and its promise "a QALY is a QALY is a QALY"	60 min 09:00am – 10:00am	All / M. Schlander & J. Ubels
22. Exercise: Putting the "Q" into the QALY Utility measurement principles applied: TTO, SG, VAS	<b>30 min</b> 10:00am – 10:30am	All
Coffee Break	<b>30 min</b> 10:30am – 11:00am	
23. Exercise: Putting the "Q" into the QALY Lessons from TTO/SG/VAS exercise	<b>15 min</b> 11:00am – 11:15am	M. Schlander

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24. Utility	75 min	
Concepts, measurement, and problems	11:15am – 12:30pm	J. Richardson
Lunch Break	<b>60 min</b> 12:30pm – 01:30pm	
25. Alternative Health Outcome Measures HALYs; HYEs, Sen's "capability approach",	<b>45 min</b> 01:30pm – 02:15pm	J. Ubels
26. Costing in Theory and Practice Opportunity costs versus monetary flows	<b>60 min</b> 02:15pm – 03:15pm	D. Hernandez
27. <b>Group Exercise: Analyzing a Costing Study</b> Costing principles applied	<b>45 min</b> 03:15pm – 04:00pm	All
Coffee Break	<b>30 min</b> 04:00pm - 04:30pm	
28. Group Exercise: Analyzing a Costing Study Lessons from exercise	<b>45 min</b> 04:30pm – 05:15pm	D. Hernandez
29. <b>International Guidelines for CEA</b> Academic guidelines and application in HTA	<b>30 min</b> 05:15pm – 05:45pm	R. Schaefer
Break	<b>15 min</b> 05:45pm – 06:00pm	
Key Note Lecture  National Institute of Health and Care Excellence (NICE): Challenges from the perspective of decision-makers	<b>60 min</b> 06:00pm – 07:00pm	A. Adler

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#### **MODULE B**

## ADVANCED ISSUES IN APPLIED HEALTH ECONOMICS

## (3) ALLOCATIVE VS. DISTRIBUTIVE OBJECTIVES

Day 3: Wednesday, February 08, 2023 [10:00am to 07:00pm]		
Welcome Reception and Registration for New Participants	09:00am- 10:00am	
30. Introduction to Program & Objectives & Wrap-Up of Module A	<b>15 min</b> 10:00am- 10:15am	M. Schlander
31. Meaning(s) of "Efficiency" and the objectives of national health schemes	<b>30 min</b> 10:15am 10:45am	M. Schlander
32. <b>The Logic of Cost Effectiveness (IV)</b> Limitations from a theoretical economic perspective	<b>45 min</b> 10:45am - 11:30am	J. Richardson
Coffee Break	15 min 11:30am – 11:45am	
33. The Logic of Cost Effectiveness (V)  A normative perspective on limitations	<b>45 min</b> 11:45am - 12:30pm	S. Holm

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Lunch Break	<b>1 hour</b> 12:30pm - 01:30pm	
34. The Logic of Cost Effectiveness (VI)  Social preferences and their measurement	60 min 01:30pm - 02:30pm	J. Richardson
35. The Logic of Cost Effectiveness (VII): Some anomalies and assumptions (linearities, aggregation)	<b>30 min</b> 02:30pm- 03:00pm	M. Schlander
Coffee Break	<b>30 min</b> 03:00pm – 03:30pm	
36. <b>Orphan Medicinal Products (OMPs)</b> Orphan drug policies / impact and recent challenges	<b>40 min</b> 03:30pm - 04:10pm	T. Butt
37. <b>Industrial Economics: A Perspective on Costs</b> Costs, profits, and incentives – R&D, production, M&S	<b>40 min</b> 04:10pm- 04:50pm	J. Mestre-Ferrandiz
38. <b>Reflections on the "Value of a Cure"</b> Beyond cost effectiveness: what about budget impact?	<b>40 min</b> 04:50pm – 05:30pm	D. Ollendorf
Coffee Break	<b>30 min</b> 05:30pm – 06:00pm	
Moderated Panel Discussion with Audience Should We Value Rarity and Severity? (and if so, how?)	<b>60 min</b> 06:00pm – 07:00pm	A. Adler, S. Holm, J. Mestre-Ferrandiz, D. Ollendorf, U. Persson, J. Richardson, M. Schlander

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#### **MODULE B**

## ADVANCED ISSUES IN APPLIED HEALTH ECONOMICS

### (4) "PRECISION MEDICINE"

Day 4: Thursday, February 09, 2023 [09:00am to 10:00pm]		
41. The Promise of "Precision Medicine" & Wrap-Up of Day 3	<b>15 min</b> 09:00am – 09:15am	M. Schlander
42. Next-Generation (Cancer) Clinical Trial Designs Improving R&D efficiency and evidence of effectiveness	<b>30 min</b> 09:15am – 09:45am	R. Baird
43. <b>Real-World Evidence (RWE) and Effectiveness</b> Potential and limitations of RWE for the generation of high quality effectiveness data	<b>30 min</b> 09:45am – 10:15am	H. Bucher
44. <b>n=1 Trials – A Normative Perspective</b> Finding the right balance	<b>30 min</b> 10:15am - 10:45am	S. Holm
Coffee Break	<b>30 min</b> 10:45am – 11:15am	

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45. Cancer Combination Therapies (I)  An increasing challenge for cost effectiveness analyses	<b>30 min</b> 11:15am – 11:45am	N. Wilking
46. Cancer Combination Therapies (II) Economic analysis and value attribution	<b>45 min</b> 11:45am – 12:30pm	A. Bruce
Lunch Break	60 min 12:30pm – 01:30pm	
<ul> <li>47. HTA and Economic Evaluation in Practice – International Experience Focus on High-Cost Interventions and ATMPs for Rare and/or Severe Health Problems  <ul> <li>a. England: NICE</li> <li>b. Sweden: TLV</li> <li>c. France: HAS</li> <li>d. Netherlands: ZINL</li> <li>e. Hungary: OGYÉI</li> </ul> </li> <li>Useful Deliberative Processes: Integrating Clinical and Economic Evidence</li> </ul>	<b>120 min</b> 01:30pm - 03:30pm	M. Schlander A. Adler U. Persson* G. Pouvourville M. Postma G. Merész D. Ollendorf
Coffee Break	30 min 03:30pm – 04:00pm	
48. Moderated Panel Discussion with Audience What Is / What Should Be the Appropriate Role of Health Economics in HTA?	<b>90 min</b> 04:00pm – 05:30pm	A. Adler, A. Bruce, G. Merész, D. Ollendorf, U. Persson, M. Postma, G. Pouvourville, M. Schlander
Winter School Networking Dinner	7:00pm – 10:00pm	Palais Prinz Carl Heidelberg

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#### **MODULE B**

## ADVANCED ISSUES IN APPLIED HEALTH ECONOMICS

## (5) THE ROLE OF PATIENTS IN HTA

Day 5: Friday, February 10, 2023 [09:00am to 02:00pm]		
51. The Critical Role of the Patient Perspective & Wrap-Up of Day 4	15 min 09:00am – 09:15am	M. Schlander
52. Patients – Forgotten HTA Stakeholders?  The role of "Patient-Reported Outcomes" in HTA	<b>45 min</b> 09:15am – 10:00am	C. Brunelli
53. The Financial Impact of Health and Health Care "Financial toxicity" – does it matter outside the U.S.?	<b>45 min</b> 10:00am – 10:45am	K. Hernandez- Villafuerte
54. Socioeconomic Impact Analysis  An OECI consensus how to assess impact on patients	<b>45 min</b> 10:45am – 11:30am	M. Schlander
Coffee Break	<b>30 min</b> 11:30am- 12:00pm	
55. Which Way Forward?  Moderated Discussion – What Should be Done?  Potential Solutions from MCDA to SCVA	<b>60 min</b> 12:00pm – 01:00pm	All
Lunch & Departures	<b>60 min</b> as of 01:00pm	

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on behalf of the Winter School Faculty

Michael Schlander

Dr. Cledi

Heidelberg, January 27, 2023

#### Contact:

Michael Schlander Mail: m.schlander@dkfz.de Office: +49 6221 42 1910 Mobile phone: +49 170 296 54 01

Ramon Schaefer

Mail: ramon.schaefer@dkfz.de Office: +49 6221 42 1914

Mobile phone: +49 151 2344 71 60

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## **Appendix**

#### **Recommended Reading Materials**

Peter J. Neumann et al. (eds.):

Cost-Effectiveness in Health and Medicine (Second Edition)

Oxford University Press 2017

Marthe Gold et al.

Cost-Effectiveness in Health and Medicine

Oxford University Press 1996

Michael Drummond et al.

Methods for the Economic Evaluation of Health Care Programs

Oxford University Press 2015 (4th edition)

John Brazier et al.

Measuring and Valuing Health Benefits for Economic Evaluation

Oxford University Press 2017 (2nd edition)

Sherry Glied and Peter C. Smith (eds.)

The Oxford Handbook of Health Economics

Oxford University Press 2011

Morris L. Barer, Thomas E. Getzen, and Greg L-Stoddart (eds.)

Health, Health Care and Health Economics - Perspectives on Distribution

John Wiley & Sons 1998

Erik Nord

Cost-Value Analysis in Health Care. Making Sense out of QALYs

Cambridge University Press 1999

Gavin Mooney

Challenging Health Economics

Oxford University Press 2009

Thomas Rice

The Economics of Health Reconsidered

Health Administration Press 2002 (2nd edition)

Daniel M. Hausman and Michael S. McPherson

Economic Analysis, Moral Philosophy, and Public Philosophy

Cambridge University Press 2006 (2nd edition)

I. Glenn Coehn, Norman Daniels, and Nir Eyal:

Identified versus Statistical Lives. An Interdisciplinary Perspective

Oxford University Press 2015

Session-specific reading materials will be provided at the Winter School.