ISPOR 15TH ANNUAL EUROPEAN CONGRESS

3-7 NOVEMBER 2012
BERLIN, GERMANY

SHORT COURSE PROGRAM

SHORT COURSES

SATURDAY, 3 NOVEMBER
(ALL DAY COURSE) 9:00-18:00

INTRODUCTION TO HEALTH ECONOMIC / PHARMACOECONOMIC EVALUATIONS *Roof Garden*


Annemans

TRACK: **Economic Methods**

LEVEL: **Introductory.** *This course is suitable for those with little or no experience with pharmacoeconomics.*

FACULTY: **Lieven Annemans, PhD, MMan, MSc,** Professor of Health Economics, ICHER (Interuniversity Center for Health Economics Research), Ghent University – Brussels University, Ghent, Belgium

COURSE DESCRIPTION: This course is designed to teach clinicians and new researchers how to incorporate pharmacoeconomics into study design and data analysis. Participants will first review the basic principles and concepts of health economic evaluations, then discuss how to collect and calculate the costs of different alternatives, determine the economic impact of clinical outcomes, and how to identify, track and assign costs to different types of health care resources used. Different pharmacoeconomic models and techniques will be demonstrated, including cost-minimization, cost-effectiveness, cost-benefit, cost-utility and budget impact analysis. Decision analysis, sensitivity analysis, and discounting will all be demonstrated and practiced.

SATURDAY, 3 NOVEMBER
(MORNING COURSES) 9:00-13:00

INTRODUCTION TO RETROSPECTIVE DATABASE ANALYSIS *Hall 10*


Martin

TRACK: **Observational Data Methods**

LEVEL: **Introductory**

FACULTY: **Bradley C. Martin, PhD, RPh, PharmD,** Professor and Head, Division of Pharmaceutical Evaluation and Policy, University of Arkansas for Medical Sciences College of Pharmacy, Little Rock, AR, USA

COURSE DESCRIPTION: Retrospective studies require strong principles of epidemiologic study design and complex analytical methods to adjust for bias and confounding. This course will provide an overview of analytic techniques and specific best practices to improve causal inference in studies using retrospective databases. Specific topics to be covered include: measurement of exposure and outcome, causal graphs, the use of stratification analysis before multivariable modeling, multivariable regression including Cox proportional hazards survival analysis, propensity scoring, instrumental variable, and structural modeling techniques including marginal structural models.

INTRODUCTION TO PATIENT-REPORTED OUTCOMES ASSESSMENT: INSTRUMENT DEVELOPMENT & EVALUATION *Hall 8*


Lloyd


Acaster


Nixon

TRACK: **Patient-Reported Outcomes / Preference-Based Methods**

LEVEL: **Introductory.** *This is an entry level course which assumes only a passing familiarity with patient-reported outcomes.*

FACULTY: **Andrew Lloyd, DPhil,** Vice President (Practice Lead), Oxford Outcomes, Oxford, UK; **Sarah Acaster, MSc,** Director, Patient Reported Outcomes, Oxford Outcomes, Oxford, UK; **Annabel Nixon, PhD,** Director, Patient Reported Outcomes, Oxford Outcomes, Oxford, UK

COURSE DESCRIPTION: Patient-reported outcomes (PROs) are widely used to evaluate the impact of health technologies, practice innovations or changes in health policy from the patients' perspective. This course is designed to familiarize people with the range and scope of what PROs are used for, how they are developed and evaluated, what they measure and how PRO data can be used to support licensing and reimbursement applications. This includes generic and disease-specific measures of health-related quality of life (HRQL) as well as measures of patient preference, systems, functioning, utility, and treatment satisfaction. We will describe the steps that researchers generally go through in order to develop and test a new PRO. This will include qualitative work, item generation and testing and then validation. Finally, in the last hour, we will frame this in terms of what the FDA and EMEA expect to see when PROs form an important part of a licensing submission. In addition, we will describe the approach of bodies such as NICE and how they review PRO data and use it to guide reimbursement decisions.

INTRODUCTION TO MODELING *Hall 7*


Siebert

TRACK: **Modeling Methods**

LEVEL: **Introductory.** *This course is designed for those with some familiarity with modeling techniques.*

FACULTY: **Uwe Siebert, MD, MPH, MSc, ScD,** Professor and Chair, Department of Public Health, Medical Decision Making and Health Technology Assessment, UMIT - University of Health Sciences, Medical Informatics and Technology, Hall/Innsbruck, Austria and Adjunct Professor of Health Policy and Management, Center of Health Decision Science, Harvard School of Public Health, Boston, MA, USA

COURSE DESCRIPTION: This course gives a brief overview on different decision-analytic model types and provides an introduction to Markov modeling techniques and their practical application in economic evaluation and outcomes research. We present analytic approaches including deterministic cohort simulation and Monte Carlo simulation and we give some technical instructions for modelers. We discuss the concepts of variability, uncertainty, probabilistic sensitivity analysis (PSA), and cost-effectiveness acceptability curves (CEAC). We review the *ISPOR Principles of Good Practice for Decision Analytic Modeling in Health Care Evaluations* and explore, when and how modeling should be used in economic evaluation and which are suitable model types.

STATISTICAL METHODS FOR PHARMACOECONOMICS & OUTCOMES RESEARCH *Hall 6*


Hawkins


Govan

TRACK: **Economic Methods**

LEVEL: **Introductory.** *This course is intended for participants with little (or rusty!) statistical training.*

FACULTY: **Neil Hawkins, PhD, CStat,** Research Fellow, University of York and Director of Health Outcomes, Oxford Outcomes, Oxford, UK; **Lindsay Govan, PhD,** Research Associate, Health Economics and Health Technology Assessment, Institute of Health and Wellbeing, University of Glasgow, Glasgow, UK

COURSE DESCRIPTION: This course will provide an introduction to statistical concepts with an emphasis on the use of techniques commonly employed in pharmacoeconomics and outcomes research. We will begin by introducing the concept of random variables and will then proceed to discuss the foundations of statistical estimation and testing of hypotheses. We will go on to discuss the importance of correlation between variables and the use of regression techniques. The differences between a classical (frequentist) approach to statistics and a Bayesian view of probability will also be outlined.

COST-EFFECTIVENESS ANALYSIS ALONGSIDE CLINICAL TRIALS *Hall 4/5*


Ramsey


Willke

TRACK: **Economic Methods**

LEVEL: **Introductory/intermediate.** *Familiarity with economic evaluations will be helpful.*

FACULTY: **Scott D. Ramsey, MD, PhD,** Member, Fred Hutchinson Cancer Research Center and Professor, Department of Medicine, University of Washington, Seattle, WA, USA; **Richard J. Willke, PhD,** Head, Global Health Economics & Outcomes Research, Global Market Access, Primary Care Business Unit, Pfizer Inc., Peapack, NJ, USA

COURSE DESCRIPTION: The growing number of prospective clinical/economic trials reflects both widespread interest in economic information for new technologies and the regulatory and reimbursement requirements of many countries that now consider evidence of economic value along with clinical efficacy. This course will present the design, conduct, and reporting of cost-effectiveness analyses alongside clinical trials based on, in part, the *Good Research Practices for Cost-Effectiveness Analysis alongside Clinical Trials: The ISPOR RCT-CEA Task Force Report.* Trial design, selecting

SHORT COURSES: SATURDAY, 3 NOVEMBER CONTINUED

data elements (measures of cost and outcomes), database design and management, analysis, and reporting of results will be presented. Trials designed to evaluate effectiveness (rather than efficacy) as well as clinical outcome measures will be discussed. How to obtain health resource use and health state utilities directly from study subjects and economic data collection fully integrated into the study will also be discussed. Analyses guided by an analysis plan and hypotheses, an incremental analysis using an intention to treat approach, characterization of uncertainty, and standards for reporting results will be presented. Various case studies will be employed to guide participants through the elements listed above.

ELEMENTS OF PHARMACEUTICAL/BIOTECH PRICING *Hall 9*


Mycka


Dellamano

TRACK: Use of Pharmacoeconomics / Economic / Outcomes Research Information

LEVEL: Introductory. *This course is designed for those with limited experience in the area of pharmaceutical pricing and will cover topics within a global context.*

FACULTY: Jack M. Mycka, Global President and CEO, MME LLC, Montclair, NJ, USA; **Renato Dellamano, PhD,** President, MME Europe & ValueVector (Value Added Business Strategies), Milan, Italy

COURSE DESCRIPTION: This course will give participants a basic understanding of the key terminology and issues involved in pharmaceutical pricing decisions. It will cover the tools to build and document product value including issues, information and processes employed (including pricing research), the role of pharmacoeconomics and the differences in payment systems that help to shape pricing decisions. These tools will be further explored through a series of interactive exercises.

Saturday Morning Coffee Break
Coffee sponsored by Alliance Life Sciences

Alliance Life Sciences Information Rules.™

SATURDAY, 3 NOVEMBER
(AFTERNOON COURSES) 14:00-18:00

INTRODUCTION TO HEALTH TECHNOLOGY ASSESSMENT (HTA) *Hall 4/5*


Siebert

TRACK: Use of Pharmacoeconomics / Economic / Outcomes Research Information

LEVEL: Introductory. *This course is suitable for those with little or no experience with HTA.*

FACULTY: Uwe Siebert, MD, MPH, MSc, ScD, Professor and Chair, Department of Public Health, Medical Decision Making and Health Technology Assessment, UMIT - University of Health Sciences, Medical Informatics and Technology, Hall/Innsbruck, Austria, and Adjunct Professor of Health Policy and Management, Center of Health Decision Science, Harvard School of Public Health, Boston, MA, USA

COURSE DESCRIPTION: This introductory course is designed to teach academic researchers, health policy decision makers, manufacturers and clinicians about the key elements, methods and language of health technology assessment (HTA). The course provides an overview of basic HTA disciplines including benefit assessment (biostatistics, clinical epidemiology, patient-relevant outcomes, risk-benefit assessment), economic evaluation (costing, cost-effectiveness analysis, pharmacoeconomic modeling, budget impact analysis, resource allocation), and ELSI (ethical, legal and social implications). Using real world HTA examples of drugs and devices, the course will review the practical steps involved in developing and using HTA reports in different countries and health care systems. Group discussion will focus on the perspectives of different stakeholders and the implementation of HTA in decision making.

META-ANALYSIS & SYSTEMATIC LITERATURE REVIEW *Hall 9*

TRACK: Outcomes Research

LEVEL: Intermediate.

PREREQUISITE: *The short course "Statistical Methods for Pharmacoeconomics & Outcomes Research" is recommended as a precursor to this course.*


Hawkins


Wu

FACULTY: Neil Hawkins, PhD, CStat, Research Fellow, University of York and Director of Health Outcomes, Oxford Outcomes, Oxford, UK; **Olivia Wu, PhD, MSc,** Reader, Public Health and Health Policy, University of Glasgow, Glasgow, UK

COURSE DESCRIPTION: Meta-analysis may be defined as the statistical analysis of data from multiple studies for the purpose of synthesizing and summarizing results, as well as for quantitatively evaluating sources of heterogeneity and bias. A systematic literature review often includes meta-analysis and involves an explicit, detailed description of how a review was conducted. This course highlights and expounds upon four key areas: 1) impetus for meta-analysis and systematic reviews; 2) basic steps to perform a quantitative systematic review; 3) statistical methods of combining data; and 4) an introduction to methods for indirect comparisons. The material includes practical examples from the published literature relevant to pharmacoeconomic and PRO research. This course is designed for those with little experience with meta-analysis and includes interactive exercises.

NEW! PROPENSITY SCORES AND OBSERVATIONAL STUDIES OF TREATMENT EFFECT *Hall 7*


Seeger

TRACK: Observational Data Methods

LEVEL: Intermediate. *This course is designed for those with little experience with this methodology but some knowledge of observational databases.*

PREREQUISITE: *Previous attendance at the short course "Introduction to Analysis of Retrospective Database Studies" – or equivalent knowledge – is recommended.*

FACULTY: John Seeger, PharmD, DrPH, Lecturer in Medicine, Division of Pharmacoepidemiology and Pharmacoeconomics, Harvard Medical School/Brigham and Women's Hospital, Boston, MA, USA

COURSE DESCRIPTION: In observational research, issues of bias and confounding relate to study design and analysis in the setting of non-random treatment assignment where compared subjects might differ substantially with respect to comorbidities. No control over the treatment assignment and the lack of balance in the covariates between the treatment and control groups can produce confounded estimates of treatment effect. We will explain how propensity scores can be used to mitigate confounding through standard observational approaches (restriction, stratification, modeling, matching, regression, or weighting). The advantages and disadvantages of standard adjustment relative to propensity score-based methods will be discussed. Details of propensity score methodology (variable selection, use, and diagnostics) and issues surrounding validation will also be discussed.

INTRODUCTION TO PATIENT PREFERENCE METHODS USED FOR QALYS *Hall 6*


Busschbach

TRACK: Patient-Reported Outcomes / Preference-Based Methods

LEVEL: Introductory/Intermediate. *This course is for those with some experience with quality of life measures in health economic evaluation.*

FACULTY: Jan Busschbach, PhD, Interim Director, Department of Medical Psychology & Psychotherapy, Erasmus MC, Rotterdam, The Netherlands

COURSE DESCRIPTION: During this course, faculty will evaluate the relevant aspects of validity and sensitivity of utility (QALY) assessments, review indirect utility measurement (EQ-5D, HUI and SF-36), direct utility measurement (standard gamble, time trade-off, and visual analogue scale) and disease-specific utility measurement. Utility measurement, however, is not only about mastering these techniques; it is about using them in such a way that health care decision-makers can apply the results, for instance in QALY-analyses. For this purpose, one needs to be aware of shortcomings of the available utility measures and potential solutions. Furthermore, one should be aware of the decision-making context and the way results are interpreted. To equip participants with expertise in the field of utility measurement, the most important issues will be discussed. For instance, we will consider potential insensitivity of generic instruments for particular disease-specific problems and discuss to what extent adaptation of generic- or disease-specific quality of life instruments may offer a solution. This will be demonstrated with practical exercises. Also, the issue of "whose values count: patient values or values from the general public?" will be discussed. Finally, we turn to the interpretation in the context of resource allocation.

SHORT COURSES: SATURDAY, 3 CONTINUED AND SUNDAY, 4 NOVEMBER

PHARMACOECONOMIC MODELING – APPLICATIONS
Hall 10


Roberts


Corman

TRACK: **Modeling Methods**

LEVEL: **Intermediate.**

PREREQUISITE: *Attendance at (or familiarity with the topics discussed in) the short course "Introduction to Modeling" is required.*

FACULTY: **Mark S. Roberts, MD, MPP,** Professor and Chair, Department of Health Policy and Management, University of Pittsburgh Graduate School of Public Health, Pittsburgh, PA, USA; **Shelby Corman, PharmD, MS, BCPS,** Senior Clinical Outcomes Scientist, Pharmerit International, Bethesda, MD, USA

COURSE DESCRIPTION: During this course, students will have hands-on experience in constructing and analyzing a decision analysis tree using TreeAge Pro software including Markov models and one-way, two-way and probabilistic sensitivity analysis. Instructors will provide a series of short lecture-based sessions followed by model-building exercises in the software. Sessions will demonstrate how to build a simple decision tree; extend a decision model to incorporate costs and utilities; and replace terminal nodes with state-transition (Markov) models to represent time-varying events. Other more advanced topics will be covered if time permits. **Participants are required to bring laptops equipped with software as provided when registering for the course.**

Saturday Afternoon Coffee Break

Coffee sponsored by PharmaQuest

PHARMAQUEST
SPECIALISTS IN TRANSLATION & LINGUISTIC VALIDATION

SUNDAY, 4 NOVEMBER
(MORNING COURSES) 8:00-12:00

BAYESIAN METHODS IN ECONOMIC EVALUATIONS – INTRODUCTION *Hall 9*


Hollenbeak

TRACK: **Modeling Methods**

LEVEL: **Introductory.** *This course is for those with a basic appreciation of statistics and probability.*

FACULTY: **Christopher S. Hollenbeak, PhD,** Associate Professor, Surgery and Public Health Sciences, Penn State College of Medicine, Hershey, PA, USA

COURSE DESCRIPTION: This course is designed to provide an overview of the Bayesian approach and its application to health economics and outcomes research. The course will cover basic elements of Bayesian statistics, discuss differences between Bayesian and classical (frequentist) approaches, and demonstrate how to apply the Bayesian approach to clinical trials and cost-effectiveness analyses. Available software will be discussed and examples of studies will be presented.

DISCRETE EVENT SIMULATION FOR ECONOMIC ANALYSES – CONCEPTS *Hall 4/5*


Caro


Möller

TRACK: **Modeling Methods**

LEVEL: **Introductory.** *This course is designed for those with some familiarity with modeling.*

FACULTY: **J. Jaime Caro, MDCM, FRCPC, FACP,** Adjunct Professor of Medicine, Adjunct Professor of Epidemiology and Biostatistics, McGill University, Montreal PQ and Senior Vice-President, Research, United BioSource Corporation, Lexington, MA, USA; **Jörgen Möller, MSc Mech Eng,** Vice-President, Modeling, United BioSource Corporation, Eslov, Sweden

COURSE DESCRIPTION: This course will provide a basic understanding of the key concepts of discrete event simulation. Topics to be covered are: how does it work; what are the components; where is it used; for which problems is DES well suited; what are the advantages and disadvantages of DES; PSA as a simple task. The focus will be on the use of these simulation models to address pharmacoeconomic (and device-related) problems. Faculty will also discuss the recently published ISPOR-SMDM guidelines on DES.

PATIENT REGISTRIES *Hall 10*


Larson


Parry

TRACK: **Observational Data Methods**

LEVEL: **Introductory.** *This course is designed for those with some or no experience with patient registries.*

FACULTY: **Leanne Larson, MHA,** Vice President, Evidence Development, PAREXEL International, Waltham, MA, USA; **Caroline Parry,** Project Director, PACE, PAREXEL International, Uxbridge, UK

COURSE DESCRIPTION: This course is designed to provide an overview of patient registries and its applications in identifying real world clinical, safety, and patient-perspective issues. The advantages and disadvantages of patient registry versus other real world data collection will be presented. The course will address safety and clinical objectives as well as regulatory trends and requirements. Key operational components, challenges and measures of program success will be discussed. Management issues - including creating effective partnerships with patient-oriented organizations and facilitating long-term program operations within a changing organizational structure - will be addressed.

NEW! INSTRUMENTAL VARIABLES *Hall 8*


Craig


El Khoury


Martin

TRACK: **Observational Data Methods**

LEVEL: **Intermediate.** *This course is suitable for those with some knowledge of econometrics.*

PREREQUISITE: *Previous attendance at the short course "Introduction to Analysis of Retrospective Database Studies" – or equivalent knowledge – is recommended.*

FACULTY: **Benjamin M. Craig, PhD,** Assistant Member, Health Outcomes and Behavior, Moffitt Cancer Center & Associate Professor, Department of Economics, University of South Florida, Tampa, FL, USA; **Antoine C. El Khoury, PhD, MS,** Director, Market Access and Health Economics, Johnson and Johnson, Horsham, PA, USA and Adjunct Assistant Professor, Division of Pharmaceutical Evaluation and Policy, University of Arkansas for Medical Sciences College of Pharmacy, Little Rock, AR, USA; **Bradley C. Martin, PharmD, RPh, PhD,** Professor and Head, Division of Pharmaceutical Evaluation and Policy, University of Arkansas for Medical Sciences College of Pharmacy, Little Rock, AR, USA

COURSE DESCRIPTION: In any non-randomized study, selection bias is a potential threat to the validity of conclusions reached. Failure to account for sample selection bias can lead to conclusions about treatment effectiveness or treatment cost that are not really due to the treatment at all, but rather to the unobserved factors that are correlated with both treatment and outcomes. Sample selection models provide a test for the presence of selection bias. These models also provide a correction for selection bias, enabling an investigator to obtain unbiased estimates of treatment effects. This course will discuss the various models and their applications and, in particular, will address instrument variables (two-stage least squares, intuition, RCTs), including an overview of examples from the current literature. Participants will benefit from interactive exercises using instrumental variables and sample selection techniques using STATA.

For those who have STATA loaded on their laptops, you are encouraged to bring your laptop.

REIMBURSEMENT SYSTEMS IN EUROPE *Roof Garden*


Furniss

TRACK: **Use of Pharmacoeconomics / Economic / Outcomes Research Information**

LEVEL: **Intermediate.** *This course is designed for individuals with intermediate experience within a single health care system wishing to broaden their appreciation of other reimbursement systems.*

FACULTY: **James Furniss,** Vice President, GfK Bridgehead, Melton Mowbray, UK

COURSE DESCRIPTION: Unlike market access which is mostly regulated at the European level by either EMA for pharmaceuticals or EC for devices, reimbursement decisions in Europe is the responsibility of each country. European health care systems are primarily government payer models. Therefore, based on each country's set of laws and values, wide variations exist in health technologies (drugs, diagnostics, devices). Using the ISPOR Global Health Care Systems Roadmap, this course will discuss health

SHORT COURSES: SUNDAY, 4 NOVEMBER CONTINUED

technology decision-making processes for coverage and reimbursement decisions in various European countries. Faculty will systematically describe the reimbursement systems across Western, Central & Eastern Europe and compare and contrast the key characteristics among them.

TRANSFERABILITY OF COST-EFFECTIVENESS DATA BETWEEN COUNTRIES *Hall 7*


Severens

Evers

Joore

Knies

TRACK: **Economic Methods**

LEVEL: **Advanced.** *This course is for those with advanced understanding of economic evaluations of health care programs and experience in the critical assessment of cost-effectiveness studies.*

FACULTY: **JL (Hans) Severens, PhD,** Professor of Evaluation in Health Care, Institute of Health Policy and Management, Erasmus University Rotterdam and Department of Health Organization, Policy, and Economics, CAPHRI, Maastricht University, Maastricht, The Netherlands; **Silvia Evers, PhD, LLM,** Chair, Public Health Technology, Maastricht University, Department of Health Services Research, Faculty of Health, Medicine and Life Science and at the School for Public Health and Primary Care (Caphri) and the Netherlands School of Primary Care Research (CaRe) Maastricht, The Netherlands; **Manuela Joore, PhD,** Associate Professor, Department of Clinical Epidemiology and Medical Technology Assessment, Maastricht University Medical Centre, Maastricht, The Netherlands; **Saskia Knies, MPhil, PhD,** Advisor, Pharmacoeconomics/HTA, Dutch Healthcare Insurance Board, Diemen, The Netherlands.

COURSE DESCRIPTION: Although the number of countries requiring an economic dossier as part of the submission dossier for public reimbursement of new drugs is growing, the pharmaceutical industry cannot conduct economic evaluations in every potential market. However, national decision makers require country-specific or region-specific data or estimates on health care costs and patient outcomes. More and more, they are only willing to accept foreign or international data when they are transferable to their own specific decision making context. However, little guidance on how to do this exists. This course starts with a discussion of factors that make economic data more difficult to transfer from one country to other countries than clinical data, and will focus on the report of the ISPOR Good Practices on Economic Data Transferability Task Force. Then we will review the methods that have been presented to assess the transferability of foreign cost, effects and cost-effectiveness estimates and their pros and cons. This topic will be practically covered in a case (working in small groups), that will be discussed subsequently. Methods available focus on trial-based economic evaluation. However, we will present transferring issues encountered when assessing model-based economic evaluations. Finally, we will discuss the transferability of health state valuation based on the EQ-5D instrument. The statistical methods to analyze multinational trial data and to transfer these data to a specific country are beyond the scope of this course.

CONJOINT ANALYSIS – THEORY & METHODS *Hall 6*


Hauber

Bridges

TRACK: **Patient-Reported Outcomes / Preference-Based Methods**

LEVEL: **Intermediate.** *This course is designed for clinicians, policymakers, researchers, and patient advocates/researchers with some familiarity with conjoint analysis or other stated-preference methods.*

FACULTY: **A. Brett Hauber, PhD,** Senior Economist and Vice President, Health Preference Assessment, RTI Health Solutions, Research Triangle Park, NC, USA; **John F.P. Bridges, PhD,** Assistant Professor, Johns Hopkins Bloomberg School of Public Health, Baltimore, MD, USA

COURSE DESCRIPTION: Course participants will learn the conceptual and empirical basis for using conjoint analysis to elicit preferences in outcomes research. The course will introduce participants to both the conceptual basis for quantifying decision-maker preferences for medical interventions and the practical design and analytical issues that must be addressed in order to obtain valid empirical preference estimates. The course will be structured following the good research practice guidelines and discussion being prepared by the ISPOR Good Research Practices for the Application of Conjoint Analysis in Health Task Force. The course will include lectures and interactive group exercises and group discussion.

Sunday Morning Coffee Break
Coffee sponsored by BaseCase Software

BASECASE

SUNDAY, 4 NOVEMBER

(AFTERNOON COURSES) 13:00-17:00

BAYESIAN METHODS IN ECONOMIC EVALUATIONS – ADVANCED *Hall 9*


Abrams

TRACK: **Modeling Methods**

LEVEL: **Advanced.**

PREREQUISITE: *Basic knowledge of a Bayesian approach will be assumed equivalent to attendance at the "Bayesian Methods in Economic Evaluations – Introduction" short course.*

FACULTY: **Keith R. Abrams, PhD,** Professor of Medical Statistics, Department of Health Sciences, University of Leicester, Leicester, UK

COURSE DESCRIPTION: This course considers the use of a Bayesian approach to both with-trial and model-based economic evaluation. The specific use and advantages of a Bayesian approach to subgroup analyses and missing data analyses in trial-based economic evaluations is considered. The use of a Bayesian approach to probabilistic decision tree and Markov models is also presented, including the use of comprehensive decision modeling (involving the seamless integration of meta-analyses within an economic decision model). All methods are illustrated using examples implemented within the freely available WinBUGS software. [Details and downloads at www.mrc-bsu.cam.ac.uk/bugs]. **Patricipants who wish to have hands-on experience must bring their laptops with software installed.**

DISCRETE EVENT SIMULATION FOR ECONOMIC ANALYSES – APPLICATIONS *Hall 10*


Caro
PHOTO NOT AVAILABLE
Lanitis

Möller

TRACK: **Modeling Methods**

LEVEL: **Intermediate.** *This course is designed for those with some understanding of discrete event simulation (equivalent to attendance at the short course "Discrete Event Simulation for Economic Analyses – Concepts") and who wish to have more practical modeling experience.*

PREREQUISITE: *Attendance at the short course "Discrete Event Simulation for Economic Analysis – Concepts" - or equivalent knowledge - is required.*

FACULTY: **J. Jaime Caro, MDCM, FRCPC, FACP,** Adjunct Professor of Medicine, Adjunct Professor of Epidemiology and Biostatistics, McGill University, Montreal PQ and Senior Vice-President, Research, United BioSource Corporation, Lexington, MA, USA; **Jörgen Möller, MSc Mech Eng,** Vice-President, Modeling, United BioSource Corporation, Hammersmith, UK; **Tereza Lanitis, MSC,** Research Associate, Modeling and Simulation, Health Economics, United BioSource Corporation, London, UK

COURSE DESCRIPTION: This course is structured around practical discrete event simulation exercises. Topics to be covered are: components of a DES; how do you build a model; modeling of processes and resource use; modeling of variables and decisions. Simple animation will be demonstrated. We will use Arena to build entry level models. Instructions for downloading training version of Arena will be distributed prior to the course. **Participants who wish to have hands-on experience should bring their personal laptops with Arena installed.**

ADVANCED RETROSPECTIVE DATABASE ANALYSIS *Hall 6*


Crown

TRACK: **Observational Data Methods**

LEVEL: **Advanced.**

PREREQUISITE: *The short course "Introduction to Retrospective Database Analysis" is a prerequisite for this course. Participants must have knowledge of statistical methods through OLS regression and experience in the analysis of administrative claims databases.*

FACULTY: **William H. Crown, PhD,** President, HEOR and Late Phase Research, OptumInsight Life Sciences, Waltham, MA, USA

COURSE DESCRIPTION: Large administrative claims databases provide an opportunity to examine retrospectively the effects of drug use on clinical and economic outcomes in

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real world settings. This course will describe analytic techniques for estimation of treatment effects and statistical properties of estimators including bias, efficiency, and mean square error. It will briefly review the assumptions underlying ordinary least squares regression (OLS) and the implications of violations (e.g., heteroscedasticity, multicollinearity, autocorrelation). Particular emphasis will be placed on model specification including structural equation models and alternative statistical estimators when OLS is not the appropriate methodology. Maximum likelihood estimation will be discussed along with the concepts of endogeneity and instrumental variables estimation.

CONTENT VALIDITY OF PRO, CLINRO AND OBSRO ASSESSMENTS *Hall 8*

TRACK: **Patient-Reported Outcomes / Preference-Based Methods**

LEVEL: **Advanced.** *This course assumes attendees have a basic understanding of qualitative interviewing methods and measurement properties of PRO instruments.*


Patrick


Martin


Gwaltney


Leidy

FACULTY: **Donald L. Patrick, PhD, MSPH,** Professor, University of Washington, Seattle Quality of Life, Seattle, WA, USA; **Mona L. Martin, RN, MPA,** Executive Director, Health Research Associates, Inc, Seattle, WA, USA; **Chad Gwaltney, PhD,** Senior Scientist, PRO Consulting, Pittsburgh, PA, USA and Assistant Professor (Research), Department of Community Health, Brown University, Providence, RI, USA; **Nancy Kline Leidy, PhD,** Senior Vice President, Scientific Affairs, United BioSource Corporation, Bethesda, MD, USA

COURSE DESCRIPTION: This course will focus on establishing the content validity of patient-reported outcomes, clinical outcomes and caregiver reported outcomes assessments that are intended for use as the basis for medical product claims in the US and Europe. The evidences for supporting content validity take into account the recommendations of the *FDA Guidance for Industry – Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims,* and the *EMA Reflection Paper on the Regulatory Guidance on the Use of Health-Related Quality of Life (HRQL) Measures in the Evaluation of Medicinal Products.* After this course, participants will be able to: define the essential evidences needed for establishing and documenting content validity of assessments planned for use in applications for regulatory approval of desired medical product claims. Examples will be given throughout on each evidence requirement. Participants will take part in several practical exercises that are part of the iterative process for determining and establishing evidence of content validity for instruments. Faculty will also reference The *ISPOR Good Research Practices for Evaluating and Documenting Content Validity for the Use of Existing Instruments and Their Modifications PRO Task Force Report.*

NETWORK META-ANALYSIS IN RELATIVE EFFECTIVENESS RESEARCH *Hall 7*

TRACK: **Outcomes Research Methods**

LEVEL: **Intermediate**


Jansen

FACULTY: **Jeroen P. Jansen, PhD, MSc,** Vice President, Health Economics & Outcomes Research, MAPI Consultancy, Boston, MA, USA

COURSE DESCRIPTION: For several medical questions of interest, many treatment options exist for the same indication. These treatments may have been compared against placebo or against each other in clinical trials. Knowing whether one specific treatment is better than placebo or some other specific comparator is only a fragment of the big picture, which should incorporate all available information. Ideally, one would like to know how all the different treatment options rank against each other and how big the differences are in effect size between all the available options. Network meta-analysis offers a quantitative method of integrating all the data from all the available comparisons. Based in part on two ISPOR Task Force reports, the fundamentals and concepts of network meta-analysis will be presented, which is especially useful when there is little or no evidence from direct comparisons. Network meta-analysis provides an integrated and unified analysis that incorporates all direct and indirect comparative evidence about treatments. The material in this course is motivated by instructive and real examples. Instructors will highlight the value of network meta-analysis and indirect treatment comparisons for decision-making; the concepts and assumptions of network meta-analysis (indirect and missed treatment comparisons); and the statistical models for network meta-analysis of different types of outcomes (i.e. dichotomous, continuous and time-to-event) and how heterogeneity and inconsistency can be captured.

RISK-SHARING/PERFORMANCE-BASED ARRANGEMENTS FOR DRUGS AND OTHER MEDICAL PRODUCTS *Roof Garden*

TRACK: **Use of Pharmacoeconomics / Economic / Outcomes Research Information**

LEVEL: **Intermediate**


Garrison


Towse


Carlson

PREREQUISITE: *It would be helpful for individuals taking this course to have completed the short course "Elements of Pharmaceutical/Biotech Pricing I – Introduction" or to be familiar with both the key determinants of pharmaceutical pricing and the main international health systems.*

FACULTY: **Lou Garrison, PhD,** Professor, Pharmaceutical Outcomes Research & Policy Program, Department of Pharmacy, University of Washington, Seattle, WA, USA; **Adrian Towse, MA, MPhil,** Director, Office of Health Economics, London, UK; **Josh Carlson, PhD,** Research Assistant Professor, Pharmaceutical Outcomes Research & Policy Program, Department of Pharmacy, University of Washington, Seattle, WA, USA

COURSE DESCRIPTION: There is significant and growing interest among both the payers and producers of medical products for arrangements that involve a "pay-for-performance" or "risk-sharing" element. These payment schemes involve a plan by which the performance of the product is tracked in a defined patient population over a specified period of time and the level of reimbursement is tied by formula to the outcomes achieved. Although these agreements have an intrinsic appeal, there can be substantial barriers to their implementation. The theory and practice, including incentives and barriers, will be analyzed along with several examples of performance-based schemes from Europe, the United States, and Australia. A hypothetical case study will be used in an interactive session to illustrate a systematic approach to weighing their applicability and feasibility.

COST ESTIMATION AND ASSESSING FINANCIAL (BUDGET) IMPACT OF NEW HEALTH CARE TECHNOLOGIES *Hall 4/5*

TRACK: **Economic Methods**

LEVEL: **Intermediate.** *This course is designed for those with some experience with pharmacoeconomic analysis.*


Mauskopf


Mullins


Earnshaw

PREREQUISITE: *The short course "Statistical Methods for Pharmacoeconomics & Outcomes Research" is recommended as a precursor to this course.*

FACULTY: **Josephine Mauskopf, PhD,** Vice President, Health Economics, RTI Health Solutions, Research Triangle Park, NC, USA; **C. Daniel Mullins, PhD,** Professor and Chair of Pharmaceutical Health Services Research, University of Maryland, School of Pharmacy, Baltimore, MD, USA; **Stephanie R. Earnshaw, PhD, MS,** Vice President, Health Economics, RTI Health Solutions, Research Triangle Park, NC, USA

COURSE DESCRIPTION: This course will guide participants through a 6-step process for budget impact analysis, describing the methods to determine costs associated with a health condition and the budget impact of new technologies for that condition. Instructors will also present incidence- and prevalence-based costing strategies. Treatment algorithms and event-based approaches will be demonstrated for disease-specific costs from different decision-maker perspectives. Both static and dynamic methods for estimating the budget impact of adding a new drug to a health plan formulary will be presented, and issues related to imputing missing data will also be discussed. Discussions will conclude with a summary of the *ISPOR Principles of Good Practice for Budget Impact Analysis Report.*

Sunday Afternoon Coffee Break
Coffee sponsored by ZRx Outcomes Research

Outcomes Research