**i3 Innovus ISPOR Contributions**

**Issue Panel**

**IP11 ECONOMIC EVALUATION OF NEW CANCER TECHNOLOGIES: IS TRADING UNCERTAINTY FOR LAUNCH TIME WORTH IT?**  
**Moderator:** Joakim Ramsberg PhD, Director, i3 Innovus, Stockholm, Sweden  
**Panelists:** Michael Drummond PhD, Professor of Health Economics, Centre for Health Economics, University of York, Heslington, York, UK; Björn Wettermark PhD, Pharmacist and Senior Researcher, Center for Pharmaceutical Policy, Stockholm County Council, Stockholm, Sweden; Josephine Sollano PhD, Senior Director and Team Leader, Global Outcomes Research, Oncology Business Unit, Pfizer, New York, NY, USA

**Workshop**

**W12: RELATIVE EFFICACY: THE POSSIBLE NEW FRONTIER?**  
**Discussion Leaders:** Monique Martin MBA, MSc, Vice President, UK Operations, i3 Innovus, Uxbridge, Middlesex, UK; Benedikte Lensberg MSc, Project Leader, HEOR Uxbridge, i3 Innovus, Uxbridge, Middlesex, UK; Michael Drummond PhD, Professor of Health Economics, Centre for Health Economics, University of York, Heslington, York, UK  
**Purpose:** To discuss the implications of relative efficacy for manufacturers and HTA agencies  
**Description:** Currently, relative efficacy (and to some extent effectiveness) is evaluated by third party payers as part of the decision-making process leading to reimbursement (or not) of new medicines. Often, the decisions are based on relative cost-effectiveness. Marketing authorisations on the other hand have traditionally been given on the basis of safety, efficacy and quality relative to no treatment. Recently, European stakeholders have highlighted the need for an improved interaction between payers and regulators, as well as between individual Member States, and questions have been raised about whether relative efficacy should be evaluated as part of the drug licensing process. Relative efficacy involves the analysis of information on how a new drug compares to the gold standard available at launch, which would be obtained by conducting head-to-head trials. This possible new initiative raises many questions, one of the most important being how to establish which gold standard should be included when there are differences between countries. Requirements for establishing relative efficacy to gain reimbursement would present significant challenges to manufacturers who would have to re-think their market access strategies. On the other hand having richer data available at launch could improve, manufacturers’ chances for reimbursement. This workshop in three parts would first explain the concept of relative efficacy (not to be confused with relative effectiveness) and would then point out the potential short-comings of relative efficacy. The final section will deal with the possible advantages of this initiative from the perspective of the regulators, companies and technology assessment agencies. Audience participation will be ensured through the conduct of an exercise where a case would be presented and audience solutions solicited.
Podiums

AD2  THE SOCIETAL BURDEN OF POOR PERSISTENCE TO TREATMENT OF OSTEOPOROSIS IN SWEDEN
Landfeldt E\textsuperscript{1}, Lundkvist J\textsuperscript{2}, Strom O\textsuperscript{1}
\textsuperscript{1}i3 Innovus, Stockholm, Sweden, \textsuperscript{2}Amgen, Stockholm, Sweden

B11  ANALYZING OVERALL SURVIVAL IN RANDOMIZED CONTROLLED TRIALS WITH CROSS-OVER
Jonsson L\textsuperscript{1}, Sandin R\textsuperscript{2}, Ekman M\textsuperscript{1}, Ramsberg J\textsuperscript{1}, Charbonneau C\textsuperscript{3}, Huang X\textsuperscript{4}, Jonsson B\textsuperscript{5},
Weinstein MC\textsuperscript{6}, Drummond M\textsuperscript{7}
\textsuperscript{1}i3 Innovus, Stockholm, Sweden, \textsuperscript{2}Pfizer Oncology, Sollentuna, Stockholm, Sweden, \textsuperscript{3}Pfizer, Inc.,
New York, NY, USA, \textsuperscript{4}Pfizer Oncology, La Jolla, CA, USA, \textsuperscript{5}Stockholm School of Economics,
Stockholm, Sweden, \textsuperscript{6}Harvard School of Public Health, Boston, MA, USA, \textsuperscript{7}University of York, York, UK

Short Course

RETROSPECTIVE DATABASE ANALYSIS
Faculty: William H. Crown PhD, President, i3 Innovus, Waltham, MA, USA
Course Description: Large administrative claims databases provide a unique opportunity to examine retrospectively the effects of drug use on clinical and economic outcomes in "real world" settings. This course will cover a discussion of the ISPOR Checklist for Retrospective Database Studies - Report of the ISPOR Task Force on Retrospective Databases and selected topics related to estimators and sampling distributions, properties of sampling distributions (unbiasedness, efficiency, mean square error), and ordinary least squares (OLS) regression. OLS model assumptions and the implications of violations (e.g., heteroscedasticity, multicollinearity, autocorrelation) will also be discussed. More complex topics beginning with the problem of endogeneity, identification, instrumental variables, sample selection models, and propensity score models, maximum likelihood methods and the estimation of limited dependent variables models including logit, multinomial logit, count models, and survival models will be discussed. This course will assume participants have knowledge of statistical methods through OLS regression and experience in the analysis of administrative claims databases. This course is recommended as a precursor to the short course “Applications in Using Large Databases in Europe”
PCN13  EFFECT OF ZOLEDRONIC ACID AND PAMIDRONATE ON SKELETAL-RELATED EVENTS AND MORTALITY IN WOMEN WITH BONE METASTASES FROM BREAST CANCER IN A MANAGED CARE PLAN: A RETROSPECTIVE DATABASE ANALYSIS  
Henk HJ1, Kaura S2  
1i3 Innovus, Eden Prairie, MN, USA, 2Novartis Pharmaceuticals Corporation, Florham Park, NJ, USA

PCN14  CLINICAL CONSEQUENCES OF PRIMARY PROPHYLAXIS WITH PEGFILGRASTIM VERSUS FILGRASTIM FOR THE PREVENTION OF FEBRILE NEUTROPENIA IN NON-HODGKIN LYMPHOMA AND STAGE II BREAST CANCER PATIENTS IN GERMANY  
Ozer-Deniz S1, Taylor DC1, Hill G1, Skornicki M1, Danel A2, Kunz E3  
1i3 Innovus, Medford, MA, USA, 2Amgen Inc., Zug, Switzerland, 3Amgen (Europe) GmbH, München, Germany

PCN17  NO CONCLUSIVE EVIDENCE FROM RANDOMISED CONTROLLED TRIALS (RCTS) FOR IMPROVED SURVIVAL WITH SECOND-LINE TREATMENT OPTIONS, IN PATIENTS WITH METASTATIC HORMONE-REFRACTORY PROSTATE CANCER (MHRPC) PREVIOUSLY TREATED WITH DOCTAXEL  
Mason M1, Freemantle N2, Parnaby A3, Högberg D4  
1Cardiff Medical School, Cardiff, UK, 2University of Birmingham, Birmingham, UK, 3Sanofi-Aventis, Vitry-sur-Seine, France, 4i3 Innovus, Stockholm, Sweden

PCN18  RETROSPECTIVE DATABASE ANALYSIS OF THE EFFECT OF ZOLEDRONIC ACID ON SKELETAL-RELATED EVENTS IN MEN WITH PROSTATE CANCER AND BONE METASTASES  
Henk HJ1, Kaura S2  
1i3 Innovus, Eden Prairie, MN, USA, 2Novartis Pharmaceuticals Corporation, Florham Park, NJ, USA

PCN29  EPIDEMIOLOGY AND HEALTH CARE UTILIZATION FOR RESECTED SQUAMOUS CELL CARCINOMA OF HEAD AND NECK (SCCHN) IN SOUTH KOREA  
Kim K1, Amonkar M2, Lykopoulos K3, Kasteng F1, Högberg D1  
1i3 Innovus, Stockholm, Stockholm, Sweden, 2GlaxoSmithKline, Philadelphia, PA, USA, 3GlaxoSmithKline, London, London, UK

PCN45  DIFFERENCES IN HEALTH CARE COSTS FOR PATIENTS WITH CASTRATION-RESISTANT PROSTATE CANCER (CRPC) TREATED BY ONCOLOGISTS OR UROLOGISTS  
Engel-Nitz NM1, Alemayehu B2, Nathan F2, Parry D3, Kulakodlu M1  
1i3 Innovus, Eden Prairie, MN, USA, 2AstraZeneca, Wilmington, DE, USA, 3AstraZeneca, Macclesfield, Cheshire, UK

PCN68  COST-EFFECTIVENESS OF PRIMARY PROPHYLAXIS WITH PEGFILGRASTIM VERSUS FIlGRASTIM FOR THE PREVENTION OF FEBRILE NEUTROPENIA IN NON-HODGKIN LYMPHOMA AND STAGE II BREAST CANCER PATIENTS IN GERMANY  
Taylor DC1, Ozer-Deniz S1, Hill G1, Skornicki M1, Danel A2, Kunz E3  
1i3 Innovus, Medford, MA, USA, 2Amgen (Europe) GmbH, Zug, Switzerland, 3Amgen (Europe) GmbH, München, Germany
PCN83  A COST-EFFECTIVENESS ANALYSIS (CEA) FOR DENOSUMAB, A FULLY HUMAN MONOCLONAL ANTIBODY FOR CANCER-TREATMENT INDUCED BONE LOSS (CTIBL) IN NON-METASTATIC PROSTATE CANCER (PRCA): A SWEDISH PERSPECTIVE
Shroff S1, Martin M1, Kearney M2, Lothgren M2, Bracco A2
11i3 Innovus, Uxbridge, Middlesex, UK, 2Amgen (Europe) GmbH, Zug, Switzerland

PCN99  COST-EFFECTIVENESS OF IMATINIB AS ADJUVANT TREATMENT FOR RESECTED GASTROINTESTINAL STROMAL TUMORS (GIST) VERSUS BEST SUPPORTIVE CARE: CANADIAN PERSPECTIVE
El Ouagari K1, Pawar V2, Coombs J3, Rubin J2
1Novartis Pharmaceuticals Canada, Dorval, QC, Canada, 2i3 Innovus, Medford, MA, USA, 3Novartis, Florham Park, NJ, USA

PCN139  A REVIEW OF DATA COLLECTED ON NON-SMALL CELL LUNG CANCER (NSCLC) PATIENTS IN CANCER REGISTRIES, DATABASES, RETROSPECTIVE AND NON-RANDOMIZED PROSPECTIVE STUDIES
De Geer A1, Eriksson J1, Finnern HW2
1i3 Innovus, Stockholm, Sweden, 2Boehringer Ingelheim GmbH, Ingelheim, Germany

PCN162  EXTRAPOLATION IN TRIAL-BASED COST-EFFECTIVENESS MODELLING: IN SEARCH OF A STANDARD
Ekman M1, Charbonneau C2, Ramsberg J2, Jonsson L2, Sandin R3, Jonsson B4, Drummond M5, Weinstein MC6
1i3 Innovus, Stockholm, Sweden, 2Pfizer, Inc, New York, NY, USA, 3Pfizer Oncology, Sollentuna, Stockholm, Sweden, 4Stockholm School of Economics, Stockholm, Sweden, 5University of York, York, UK, 6Harvard School of Public Health, Boston, MA, USA

PDB64  TREATMENT PERSISTENCE AMONG PATIENTS INITIATING INSULIN THERAPY WITH INSULIN DETEMIR IN A FLEXPEN® VERSUS NPH INSULIN IN A VIAL. RETROSPECTIVE DATABASE ANALYSIS BASED ON A LARGE US MANAGED CARE ORGANIZATION
Conner C1, Buysman E2, Liu F2, Aagren M3, Bouchard J4
1Novo Nordisk, Seattle, WA, USA, 2i3 Innovus, Eden Prairie, MN, USA, 3Novo Nordisk Inc, Princeton, NJ, USA, 4Novo Nordisk, Plaistow, NH, USA

PDB87  STANDARDS FOR THE ASSESSMENT OF ANTIDIABETIC DRUGS – THE IQWIG PERSPECTIVE
Schwelkert B1, John J2, Ringborg Å3, Erhardt W4, Bleckmann A5, Neubauer AS6
1i3 Innovus, Aschheim, Germany, 2Helmholtz Zentrum München, Neuherberg, Germany, 3i3 Innovus, Stockholm, Sweden, 4Bristol-Myers Squibb, München, Germany, 5AstraZeneca, Wedel, Germany

PCV75  CANADIAN COST-EFFECTIVENESS ANALYSIS OF DRONEDARONE VERSUS OTHER ANTI-ARRHYTHMIC DRUGS IN PATIENTS WITH PAROXYSMAL AND PERSISTENT ATRIAL FIBRILLATION
Nilsson J1, Åkerborg Ö1, Lindgren P2
1i3 Innovus, Stockholm, Sweden, 2Sanofi-Aventis, Paris, Paris, France
PIH22 EVALUATING THE COST-EFFECTIVENESS OF CERVICAL CANCER SCREENING AND HUMAN PAPILLOMAVIRUS VACCINATION STRATEGIES USING A MATHEMATICAL MODEL
Taylor DC1, Pawar V1, Gilmore K1, Sanon M1, Kruzikas D2, Kohli M3, Arondekar B4, Demarteau N5, Weinstein M6
1i3 Innovus, Medford, MA, USA, 2Lovelace Respiratory Research Institute, Kannapolis, NC, USA, 3i3 Innovus, Burlington, ON, Canada, 4GlaxoSmithKline, Philadelphia, PA, USA, 5GSKbio, Wavre, Belgium, 6Harvard School of Public Health, Boston, MA, USA

PIH34 CONTENT VALIDITY OF THE BENIGN PROSTATIC HYPERPLASIA IMPACT INDEX (BII): RESULTS FROM CONCEPT ELICITATION AND COGNITIVE INTERVIEWS
Naegeli A1, Martin ML2, Kingery LR3, Viktrup L1
1Eli Lilly & Company, Indianapolis, IN, USA, 2Health Research Associates, Inc, Seattle, WA, USA, 3i3 Research, Basking Ridge, NJ, USA

PND34 A PSYCHOMETRIC EVALUATION OF THE REVISED SCOPA DIARY CARD IN PARKINSON'S DISEASE PATIENTS
Buck PO1, Castelli-Haley J1, White RE1, Rendas-Baum R2, White MK2
1Teva Neuroscience, Kansas City, MO, USA, 2QualityMetric Incorporated, Lincoln, RI, USA

PND36 HEALTH STATUS COMPARISON BETWEEN STABLE PARKINSON'S DISEASE PATIENTS AND THOSE EXPERIENCING OFF-TIME
Buck PO1, White RE1, Castelli-Haley J1, Rendas-Baum R2, White MK2
1Teva Neuroscience, Kansas City, MO, USA, 2QualityMetric Incorporated, Lincoln, RI, USA

PSS11 THE COST-EFFECTIVENESS OF A NEW GEL FORMULATION OF CALCIPOTRIOL/BETAMETHASONE DIPROPIONATE FOR THE TREATMENT OF SCALP PSORIASIS IN NORWAY
Holm MV1, Ekman M2, Ryttov J3
1LEO Pharma, Ballerup, Denmark, 2i3 Innovus, Stockholm, Sweden, 3LEO Pharma, Marlow, UK

PIN2 ESTIMATED IMPACT OF SUSTAINED VIROLOGICAL RESPONSE (SVR) ON LIFE EXPECTANCY, QUALITY-ADJUSTED LIFE-YEARS (QALYS) AND LIFETIME COSTS IN CHRONIC HEPATITIS C (CHC) PATIENTS
Čure S1, Bianic F1, Cawston H1, Dartois L1, Zhang H2
1i3 Innovus, Uxbridge, Middlesex, UK, 2Johnson & Johnson Pharmaceutical Services, LLC, Raritan, NJ, USA

PIN38 AN ECONOMIC EVALUATION OF THE PEDIATRIC VACCINATION SCHEDULE IN THE UNITED STATES
Clements KM1, Misurski DA2, Miller J3, Skornicki ME1, Hill GJ1, McGarry L1
1i3 Innovus, Medford, MA, USA, 2GlaxoSmithKline, Philadelphia, PA, USA, 3GlaxoSmithKline Biologicals, King of Prussia, PA, USA
PIN42 THE COST-EFFECTIVENESS OF 13-VALENT PNEUMOCOCCAL CONJUGATE VACCINE (PCV13) COMPARED WITH 10-VALENT PNEUMOCOCCAL CONJUGATE VACCINE (PCV10) IN TAIWAN
Chang CJ¹, Wu DBS², Wu CL¹, Strutton D³, Hwang VWH⁴, Rubin J⁵, Gilmore K⁵
¹Chang Gung University, Taoyuan, Taiwan, ²National Yang-Ming University, Taipei, Taiwan, ³Pfizer Limited, Collegeville, PA, USA, ⁴Pfizer Limited, Taipei, Taiwan, ⁵i3 Innovus, Medford, MA, USA

PMH21 SOCIETAL COSTS OF BIPOLAR DISORDER – THE CASE OF SWEDEN
Ekman M¹, Granström O², Omerov S³, Jacob J², Landén M⁴
¹i3 Innovus, Stockholm, Sweden, ²AstraZeneca Nordic MC, Södertälje, Sweden, ³Northern Stockholm Psychiatry, Stockholm, Sweden, ⁴Gothenburg University, Göteborg, Sweden

PSY51 STATED PREFERENCES OF PHYSICIANS AND CHRONIC PAIN SUFFERERS IN THE USE OF CLASSICAL STRONG OPIOIDS
Chancellor J¹, Martin M², Liedgens H³, Baker MG⁴, Müller-Schwefe GH⁵
¹Chancellor Health Economics Ltd, Beaconsfield, Buckinghamshire, UK, ²i3 Innovus, Uxbridge, Middlesex, UK, ³Gruenenthal GmbH, Aachen, Germany, ⁴European Federation of Neurological Associations, Helensburgh, UK, ⁵German Pain Association, Göppingen, Germany

PUK7 THE PHARMACY BUDGET IMPACT OF EXTENDING REIMBURSEMENT OF LANTHANUM CARBONATE TO TREATMENT OF HYPERPHOSPHATEMIA (>1.78MMOL/L) IN PATIENTS WITH CHRONIC KIDNEY DISEASE PRE-DIALYSIS IN FRANCE AND THE UNITED KINGDOM
Campbell J¹, Tao CY¹, Keith MS², Leahy KJ¹, Russo L²
¹i3 Innovus, Medford, MA, USA, ²Shire Pharmaceuticals, Wayne, PA, USA
Plenary Sessions

ASSESSMENT OF HEALTH TECHNOLOGY IN EUROPE: TAKING STOCK OF THE INEFFICIENCIES
Moderator/Speaker: Bengt Jonsson PhD, Professor of Health Economics, Centre for Health Economics, Stockholm School of Economics, Stockholm, Sweden.

KEY TRENDS AND DEVELOPMENTS IN HEALTH TECHNOLOGY DECISION-MAKING IN EUROPE
Speaker: Michael Drummond PhD, Professor of Health Economics, Centre for Health Economics, University of York, Heslington, York, UK.

Workshop

W20: MAXIMUM ACCEPTABLE RISK: ALTERNATIVE MEASURES FOR QUANTIFYING PATIENTS’ TOLERANCE FOR THERAPEUTIC RISKS
Discussion Leaders: A. Brett Hauber PhD, Global Head, Health Preference Assessment & Market Access, RTI Health Solutions, Research Triangle Park, NC, USA; F. Reed Johnson PhD, Distinguished Fellow and Principal Economist, RTI Health Solutions, Research Triangle Park, NC, USA; Axel Christian Mühlbacher PhD, Professor for Health Economics, IGM Institute Health Economics and Health Care Management, HS Neubrandenburg, Neubrandenburg, Germany; Deborah Marshall PhD, Canada Research Chair, Health Services and Systems Research, University of Calgary, Calgary, AB, Canada
Purpose: The purpose of this workshop is to review and appraise three alternative methods for estimating patients’ tolerance for therapeutic risks.
Description: Regulators in the EU and US currently are evaluating alternative approaches to quantitative benefit-risk assessments of pharmaceutical products. One approach under consideration is to elicit patients’ stated risk tolerance directly. There are three possible methods for quantifying patient preferences for benefit-risk tradeoffs: traditional health-state utility approaches, contingent-valuation using bidding games, and conjoint analysis. The workshop will include a presentation to describe the conceptual framework, practical implementation, and empirical examples of each of these methods. The workshop also will include an interactive segment in which audience members will answer survey questions based on a hypothetical case study. The discussion leaders will then debrief participants on their experience and elicit critical assessments of the validity and likely acceptability of the data produced by each method.