

ISPOR Taskforce Prospective Observational Studies for Comparative Effectiveness

When Seeing is Believing

European ISPOR Meeting

November 2010

Prague

Context

- Increased focus on comparative effectiveness for health policy decision-making
- US Health Care Reform – PCORI
- European High Level Pharmaceutical Form – Focus on Relative Effectiveness
- Comparative Effectiveness as critical part of Health Technology Assessment (HTA) used by HTA agencies around the world and P&T committee in US Managed Care

Build on Prior ISPOR Taskforce

Volume 18 • Number 1 • 2010
VALUE IN HEALTH

Good Research Practices for Comparative Effectiveness Research: Defining, Reporting and Interpreting Nonrandomized Studies of Treatment Effects Using Secondary Data Sources: The ISPOR Good Research Practices for Retrospective Database Analysis Task Force Report—Part I

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Why another taskforce?

- Retrospective Database
 - Secondary data
 - Issues:
 - Correlation vs causation
 - Analytic approaches to dealing with confounding
 - Utility to inform health policy decisions
- Good Research Practices to ensure quality, disclose potential conflicts of interest, etc.
- Prospective Observational Studies
 - Fit-for-purpose
 - Primary data collection
 - No treatment assignment
- Specific design issues
 - Sample size
 - Enrollment and study participation
 - Safety issues and AE reporting
 - Remuneration of subjects

Taskforce Members

Co-Chairs:

- Marc Berger MD, Vice President, Global Health Outcomes, Eli Lilly and Company, Indianapolis, IN, USA
- Sharon-Lise Normand PhD, Professor of Health Care Policy (Biostatistics), Harvard Medical School, Department of Health Care Policy, Boston, MA, USA

Members:

- Fred Anderson PhD, Research Professor of Surgery, Director, Center for Outcomes Research, University of Massachusetts Medical School, Worcester, MA, USA, Cambridge, MA, USA
- Nancy Dreyer PhD, MPH, Chief of Scientific Affairs & Sr. Vice President, Outcome, Cambridge, MA, USA
- Art Sedrakyan MD, PhD, Associate Professor, Director, Comparative Effectiveness Program at HSS and NYP, Weill Cornell Medical College, New York, NY, USA
- Thomas Ten Have PhD, MPH, Professor, Center for Clinical Epidemiology and Biostatistics, University of Pennsylvania School of Medicine, Philadelphia, PA, USA
- Adrian Towse MA, Director, Office of Health Economics, London, England, United Kingdom

Taskforce Timeline

- Identify and recruit Task Force leaders and leaders June 2010
- Identify and assign work streams July 2010
- Work stream groups produce report sections July–August 2010
- First draft of each section of the report completed September 2010
- Forum presentation at the ISPOR European Congress, Prague, Czech Republic November 2010
- Comments incorporated in draft report December 2010
- Face-to-face meeting to finalize draft report January/February 2011
- Draft report submitted to reviewer group for comments February 2011
- Draft report revised based on comments March 2011
- Final report presented at the ISPOR International Meeting, Baltimore, Maryland, USA May 2011
- Report submitted to *Value in Health* June 2011

Why prospective observational studies vs alternative naturalistic studies?

- Observational studies (i.e. no treatment assignment)
 - Registries
 - Prospective Observational Studies
 - Case Control Studies
 - Other
- Alternative naturalistic study designs
 - Pragmatic clinical studies (i.e. randomization to treatment)
 - Partial Patient Preference Studies (i.e. patients/providers select from limited number of treatment options)

Bias and Confounding as Issues for Observational Studies

Strategies to Address

- Design choices and Issues
 - New treatment vs existing treatments
 - Existence and strength of prior evidence
 - Drugs/biologics; Medical devices
 - Bayesian framework
 - Meta-analysis – direct/indirect
- Risk for confounding by indication
 - New treatment vs existing treatment
 - Evidence of confounding in alternative treatments
 - Standard of care
 - Perception/Preference
 - Alternative treatments – time-dependent confounding
 - Who applies the technology changes over time; How the technology is used changes over time; Acceptance as standard of care develops over time; Impact on confounding considerations
- Residual Confounding

Practical Challenges of Observational Research

- Safety reporting differs from trials
 - Reporting depends, in part, on sponsorship
 - Not all serious events are reportable
- Payments to physicians and patients must be commensurate with time and effort spent
- Data access practices for on-going, multicenter studies
- Creating and managing Advisory Boards
- To register...or not?

QUESTIONS?
COMMENTS?