www.ispor.org

ISPOR
Improving healthcare decisions

ISPOR Special Interest Group

ISPOR STATISTICAL METHODS IN HEALTH ECONOMICS AND OUTCOMES RESEARCH SPECIAL INTEREST GROUP - WHERE HAVE WE BEEN? WHERE ARE WE GOING?

ISPOR Europe 2019 | Copenhagen, Denmark

Tuesday, 5 November 2019 | 12:30 – 13:45

ISPOR

WiFi Network: ISPOR2019 | Password: Avalere

Q&A

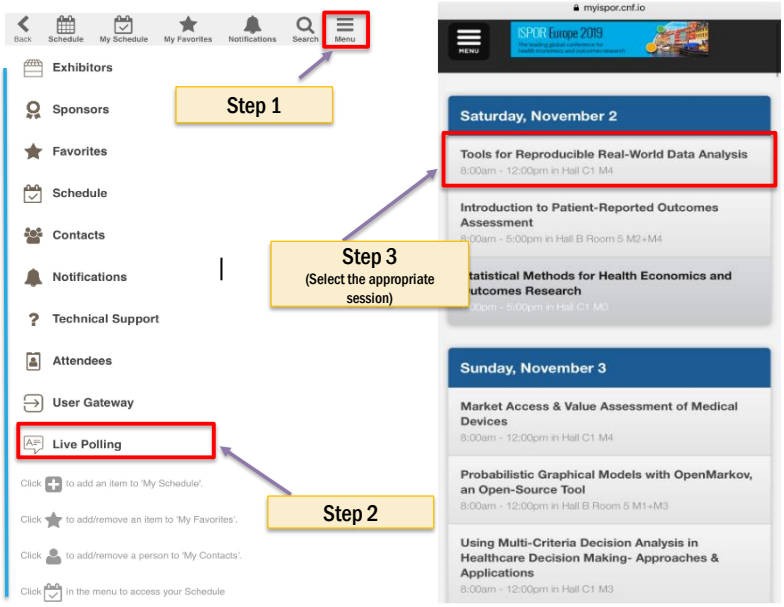
ISPOR **Conference Platform**

Web Platform

https://myispor.cnf.io/

Mobile App

Search "**ISPOR Europe 2019**" in the App Store or on Google Play!



ISPOR Statistical Methods in HEOR Special Interest Group (SIG)

Mission: To provide statistical leadership for strengthening the use of appropriate statistical methodology in health economics and outcomes research and improve the analytic techniques used in real world data analysis.

Co-Chairs of SIG

- **Rita M. Kristy, MS**, Senior Director, Medical Affairs Statistics, Astellas Pharma Global Development, Northbrook, IL, USA
- **Chair-Elect: Gian Luca Di Tanna, PhD MPhil MSc AStat Cstat,** Head, Statistics Australia, Statistics Division, The George Institute for Global Health, Newtown, Australia
- **David J. Vanness, PhD,** Professor, Health Policy and Administration, The Pennsylvania State University, State College, PA, USA

Co-Chairs of ISPOR Missing Data in HEOR Working Group

- **Necdet Gunsoy, PhD, MPH,** Director of Analytics and Innovation for Value Evidence and Outcomes at GlaxoSmithKline (GSK), England, United Kingdom
- **Gianluca Baio, PhD, MSc,** Professor of Statistics and Health Economics, University College London (UCL), England, United Kingdom

ISPOR Special Interest Group

3

ISPOR www.ispor.org

History of the SIG

- The Statistical Methods in HEOR SIG has existed since April 2017
- There are more than 300 ISPOR members in the SIG
- Our first Key project is the Missing Data in HEOR working group
  - Missing data is well addressed in clinical trials
  - There is little literature on missing data in HEOR

4

SIG Presentations and Other Engagements

- Issue panel presentation at ISPOR Europe 2018 in Glasgow
  - PRAGMATIC CLINICAL TRIALS TO ESTIMATE TREATMENT EFFECTS: ARE THEY WORTH THE EFFORT?
- Forum presentation at ISPOR Annual 2018 in Baltimore
  - HANDLING MISSING VALUES IN REAL-WORLD DATA: ARE THERE CHALLENGES FOR REGULATORY DECISIONS FOR MEDICAL PRODUCTS?
- Review of Guidance Document
  - Review of ICH E9 (R1) addendum on estimands and sensitivity analysis in clinical trials to the guideline on statistical principles for clinical trials

5

ISPOR www.ispor.org

SIG Presentations and Other Engagements

- Workshop presentation at ISPOR Europe 2018 in Barcelona
  - ARE MISSING DATA PROPERLY ACCOUNTED FOR IN HEALTH ECONOMICS AND OUTCOMES RESEARCH?
- Workshop presentation at ISPOR Europe 2018 in Barcelona
  - INDIRECT TREATMENT COMPARISONS: AN INTERACTIVE WORKSHOP ON CHOOSING THE RIGHT TOOL FOR THE AVAILABLE DATA
    - Value & Outcomes Spotlight article – coming soon (~December 2019)
- Workshop presentation at ISPOR 2019 in New Orleans
  - HOW TO TACKLE THE ESTIMATION OF TREATMENT IMPACT IN THE PRESENCE OF DIFFERENTIAL WITHDRAWAL AND MISSING DATA AMONG STUDY ARMS?

6

SIG Presentations and Other Engagements

- Workshop presented at ISPOR Europe 2019 in Copenhagen
  - DEMYSTIFYING MACHINE LEARNING- WHY WE SHOULD BE OPEN TO IT?
- Book of terms – 30 terms reviewed and monographs written by SIG members

SECTION

1

KEY PROJECT: Missing Data in Health Economics and Outcomes Research

Missing data in HEOR – Next steps

1. **Literature review and extraction**
2. **Evaluation and critical review**
3. **Classification of methods**
4. **Guidance and best practices - A framework on reporting and handling of missing data**

Complete / **In progress** / **Planned**

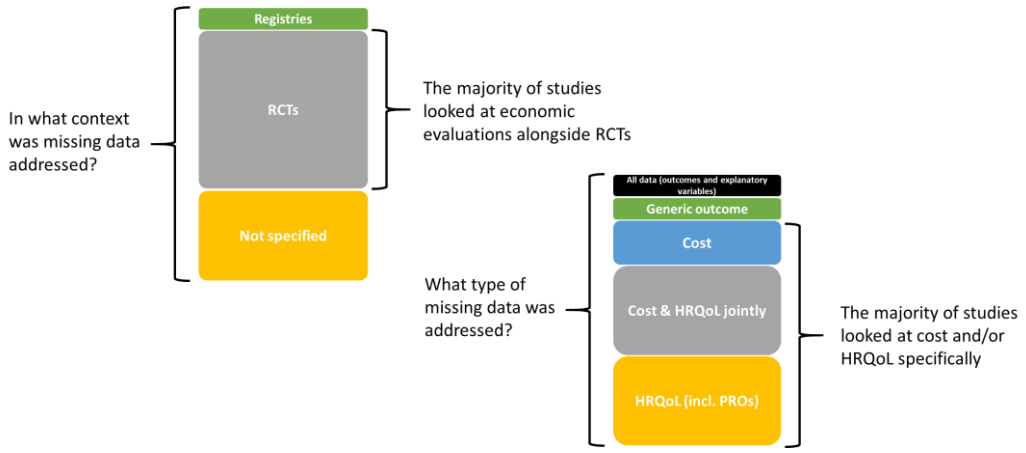
9

Literature review

- A literature review was conducted to understand methodological approaches used to account for missing data in cost-effectiveness analyses (CEA)

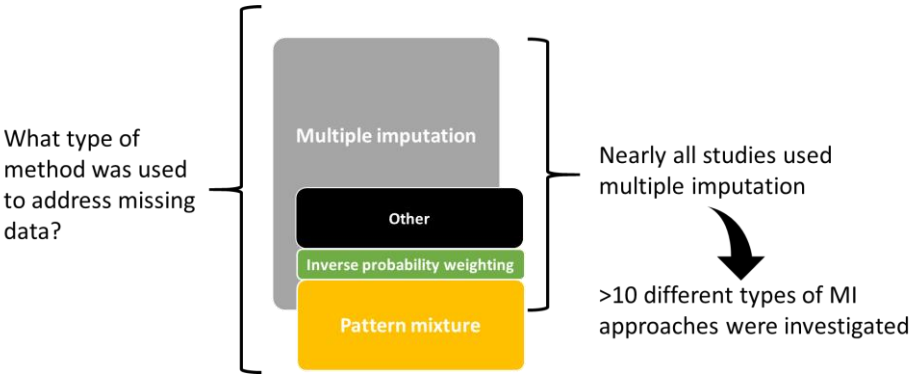
10

Literature review



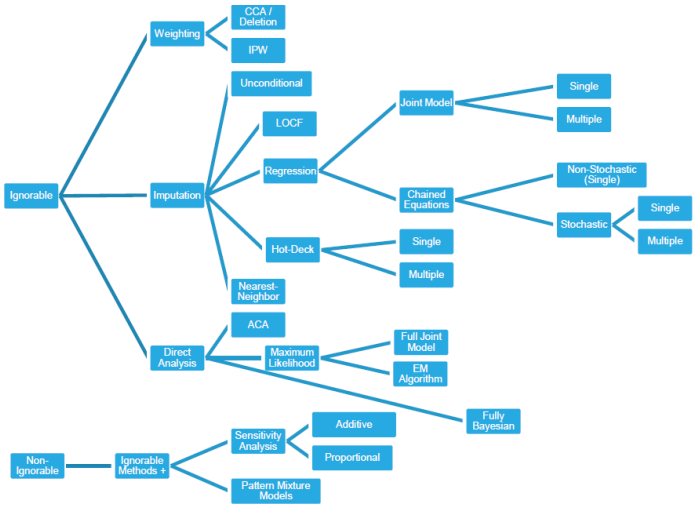
11

Literature review



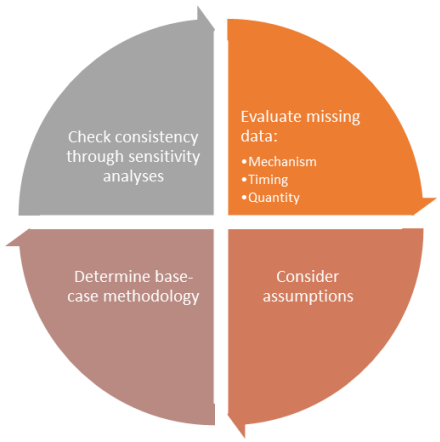
12

Classification of methods



13

Framework on reporting and handling of missing data in HEOR



14

www.ispor.org

Member Engagement Activities

RECENT WEBINAR – "Principles of Machine Learning for Prediction"

ISPOR Special Interest Group

- **Date:** July 30, 2019
- **Speaker:** David Vanness, PhD, Professor, Penn State University, USA
- **Description:** This webinar introduced learners to the basic principles of machine learning (ML) for prediction in health economics and outcomes research (HEOR). We began by framing the prediction problem and drawing both connections and contrasts with the related problems of explanation and causal inference. We then confronted the "Iron Law of Prediction" – which characterizes the goals of prediction in terms of "minimizing loss" – or the consequences of making poor predictions. We then explored the principles of "cross validation" and the "tuning" of algorithms to minimize loss. Using a simulated dataset, we applied several ML algorithms to illustrate the principles of "complexity penalization," "bagging," "random feature selection," and "boosting." We concluded the webinar by considering an empirical application of boosting to construct propensity scores – a method of prediction that is used to improve causal inference for other parameters of interest.
- **Learning Objectives:**
  - Describe the Iron Law of Prediction as a tradeoff between bias and variance of predictions to minimize the loss associated with making poor predictions.
  - Describe how cross-validation allows analysts to minimize loss.
  - Describe how the process of tuning ML algorithms is used to optimize their performance in minimizing loss.
  - Describe how the bagging, random feature selection and boosting can be used to minimize loss.
- **Recording link**: https://www.ispor.org/conferences-education/education-training/virtual/webinars

UPCOMING WEBINAR – "Machine Learning for HEOR: Prediction and Causal Inference"

ISPOR Special Interest Group

- **Date and Time:** Next Friday, November 15, 2019 at 12:00PM EST
- **Speaker:** Sherri Rose, PhD, Associate Professor, Harvard Medical School, USA
- **Description:** Health care research is moving toward analytic systems that take large health databases and estimate varying quantities of interest both quickly and robustly, incorporating advances from statistics, econometrics, and computer science. This workshop will discuss specific challenges related to developing and deploying statistical machine learning algorithms in prediction and causal inference for health policy research questions, including examples from the areas of health plan payment, mental health care, and medical devices. Considerations go beyond typical measures of statistical assessment, and include concepts such as dataset shift and algorithmic fairness.
- **Learning Objectives:**
  - Understand the shortcomings of standard parametric regression techniques for the estimation of prediction and causal effect quantities
  - Be introduced to the ideas behind machine learning approaches as tools for confronting high-dimensional data
  - Become familiar with the properties and basic conceptual implementation of machine learning for prediction and causal effect estimation
  - Recognize the centrality of fairness and generalizability considerations in quantitative health policy research
- **Registration link**: https://www.ispor.org/conferences-education/education-training/virtual/webinars/machine-learning-for-health-economics-and-outcomes-prediction-and-causal-inference

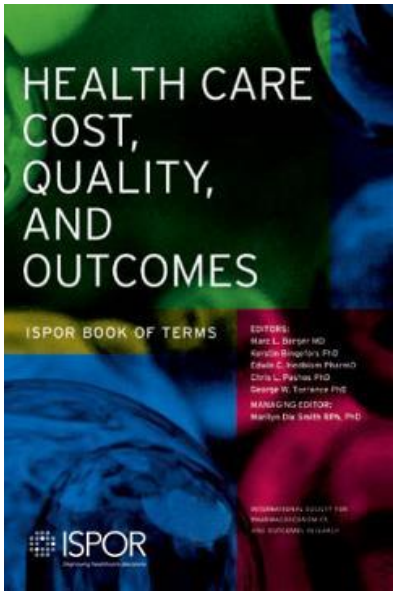
17

Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: What webinar topics would you the SIG to present on?

Member Engagement Project: ISPOR Book of Terms 2020



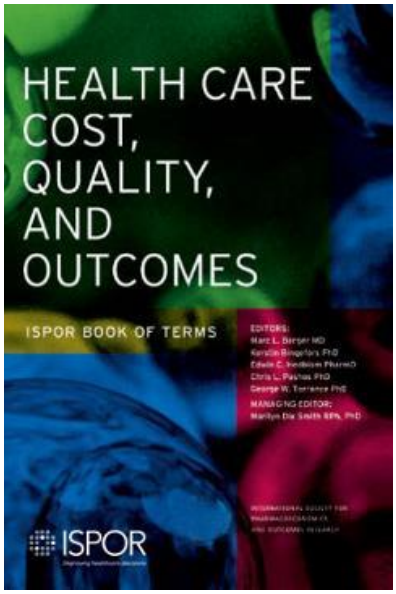
Section Editors

David J. Vanness, PhD, Professor, Health Policy and Administration, The Pennsylvania State University, State College, PA, USA

Gian Luca Di Tanna, PhD MPhil MSc AStat CStat
Head, Statistics Australia, Statistics Division, The George Institute for Global Health, Newtown, Australia

Kumar Mukherjee, MS, PhD Associate Professor of Pharmacy Practice, Philadelphia College of Osteopathic Medicine, Philadelphia, PA, USA

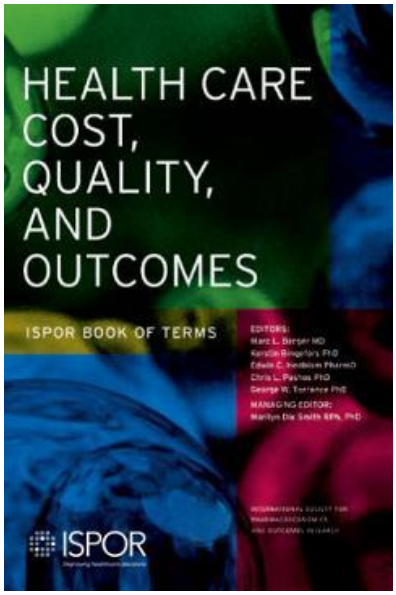
Juan-David Rueda, MD, MSc, Manager, Health Economics & Payer Evidence (Oncology), AstraZeneca, Gaithersburg, MD, USA



Terms Selected by the SIG:

Bayesian Analysis
Bootstrapping
Causal Inference
Confounding
Cumulative Distribution Function
Distribution-based Methods
Econometrics
Effect Modification
Experimental Design
Growth Curve Modeling
Incidence
Instrumental Variables
Likelihood Ratio
Linear Discriminant Analysis
Number Needed to Treat
Patient-level Data
Predictive Analytics
Predictive Value
Principal Component Analysis
Prognostic Models
Propensity Scores
Sample Selection Model
Sensitivity Analysis
Statistics in HEOR
Surrogate Outcome
Survival Analysis
Time Series Analysis
Time-to-Event Analysis
Time-to-Event Models

ISPOR www.ispor.org

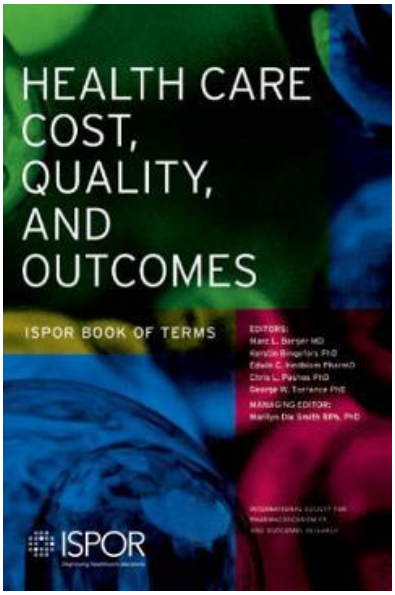


Terms Available for Volunteer Co-Authors

- Econometrics
- Incidence
- Number Needed to Treat
- Prognostic Models
- Sample Selection Model
- Survival Analysis

Please send an email to statisticalmethodssig@ISPOR.org with your choice.

Due date: Monday, November 25

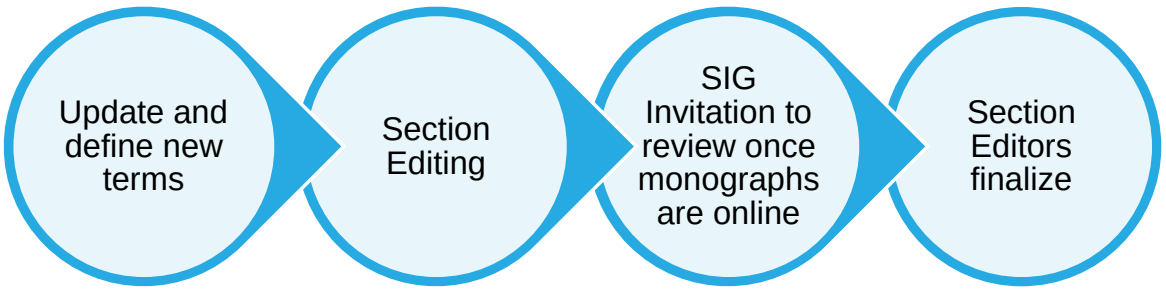


Monograph

Brief Definition: 1 – 2 sentences
Explanation: several paragraphs ~ 1/3 - 1/2 page
Value & Use: several paragraphs ~ 1/3 - 1/2 page
Issues: several paragraphs ~ 1/3 - 1/2 page
Bibliography: 5 current, seminal peer-reviewed references
Illustration (if needed**) :** A figure, table, or graph
Link to ISPOR-related sources: for example**,** an ISPOR Good Practices Task Force Report, SIG Report or PE Guideline, if applicable

Length: 1200 - 1500 words

Book of Terms 2020



Q2 – Q3 2019

Q4 2019 – Q1 2020

Q1 2020

Q2 2020

24

Membership Survey

Enter a number next to each topic below. The total across all topics should equal 100.

_____ Bayes Nets and Other Probabilistic Graphical Models
_____ Bayesian Methods: Individual Patient Data
_____ Bayesian Methods: Meta-Analysis
_____ Causal Inference with Directed Acyclic Graphs
_____ Continuous Multi State Models
_____ Cross-Design Methods (Combining Randomized Data with RWE)
_____ Discrete State Dynamic Models
_____ Dynamic Treatment Regimes
_____ Ensembles and Model Averaging
_____ Graph Analytics
_____ Heterogeneous Treatment Effects
_____ Hierarchical Modeling
_____ Instrumental Variables
_____ Latent Class Analysis
_____ Marginal Structural Models
_____ Matching Methods
_____ Multiplicity (Multiple Hypothesis Testing; Inference vs. Hypothesis Generation)
_____ Multivariate Analysis (Multiple Correlated Outcomes)
_____ Repeated Measures
_____ Spatial Analysis
_____ Structural Equation Modeling
_____ Survival Analysis
_____ Other: _____

25

Upcoming Conferences and Abstract Submissions

- **ISPOR 2020** [May 16-20, 2020] | Orlando, FL, USA
  - Abstract Submission Opens: **November 1, 2019**
  - Abstract Submission Deadline: **January 15, 2020**
  - Author Notification: **March 2, 2020**
- **ISPOR Asia Pacific 2020** [12-15 September 2020] \ Seoul, South Korea
  - Abstract Submission Opens: **2 December 2019**
  - Abstract Submission Deadline: **11 March 2020**
  - Author Notification: **1 May, 2020**
- **ISPOR Europe 2020** [14-18 November 2020] | Milan, Italy
  - Abstract Submission Opens: **2 March 2020**
  - Abstract Submission Deadline: **10 June 2020**
  - Author Notification: **3 August, 2020**

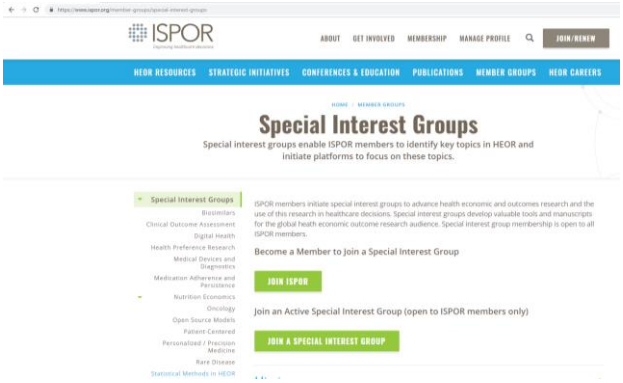
26

Sign up as a Special Interest Group Member

ISPOR Special Interest Group

1. Visit ISPOR webpage www.ispor.org
2. Select “Member Groups”
3. Select “Special Interest Groups”
4. On left side bar, click “Statistical Methods in HEOR”
5. Click button to “Join This Special Interest Group”

For more information, e-mail statisticalmethodssig@ispor.org



27

We Thank All of ISPOR’s Volunteer Leaders

#ISPOREurope

28