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Guest editorial summary on articles selected from the 2018 International Conference on Health Policy Statistics

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1 Introduction

Throughout the past 21 years, the International Conference on Health Policy Statistics (ICHPS), organized by the Health Policy Statistics Section of the American Statistical Association (ASA), has played a vital role in the dissemination process of health policy (and health services) statistics. ICHPS provides a unique forum for discussing research needs and solutions to the methodological challenges in the design of studies and analysis of data for health policy research. The aim of ICHPS is to create interactions among practitioners, methodologists, health service researchers, health economists, and policy analysts so they can exchange and build on ideas they will disseminate to the broader health policy community.

The 12th International Conference on Health Policy Statistics (ICHPS 2018) was held in Charleston, South Carolina, January 10–12, 2018. With its theme "Health Statistical Science Care, Policy, Outcomes", the conference featured an ambitious program (http://ww2.amstat.org/meetings/ichps/2018/onlineprogram/index.cfm). Conference speakers were invited to submit manuscripts of their presentations for consideration in a special issue in *Health Services and Outcomes Research Methodology*, with us (JC and JB) serving as guest co-editors. Four articles appear in the December 2018 issue of the journal and five in the March 2019 issue. Extending upon the aim of the conference, the articles are grounded in innovative and timely research on advances in quantitative methodology regarding health services, policy, and outcomes research.

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2 December 2018 issue

2.1 Informative missingness in ecological momentary assessments

In the December 2018 of the journal, Lin, Mermelstein and Hedeker address the problem of accounting for informative missing in the context of ecological momentary assessment (EMA) studies where each study unit gets measured intensively over time and intermittent missing is usually presented.

In their article, "A shared parameter location scale mixed effect model for EMA data subject to informative missing", a shared parameter modeling framework is presented that links a normally distributed, intensively measured primary longitudinal outcome with potentially informative missingness by a common set of random effects that summarize a subjects' specific traits in terms of their mean (location) and variability (scale). Unlike previous methods, which largely rely on numerical integration or approximation, the authors' estimate the model by a full Bayesian approach using Markov Chain Monte Carlo. An adolescent mood study example is illustrated together with a series of simulation studies. Relative to more conventional approaches, the approach used here is shown to significantly improve model fit and, in addition, to provide benefit in understanding how missingness can affect the inference for the primary outcome.

2.2 Bivariate subgroup analysis

Subgroup analysis is a frequently used tool for evaluating both heterogeneity of treatment effects and heterogeneity in treatment harm across observed baseline patient characteristics. While primary treatment efficacy and adverse event measures are often reported separately for each subgroup, analyzing their within-subgroup joint distribution is critical for better informed patient decision-making.

In their paper, "Bayesian bivariate subgroup analysis for risk-benefit evaluation", Henderson and Varadhan describe a Bayesian approach for performing subgroup analysis so that benefit-safety endpoints are modeled jointly, rather than used to perform separate subgroup analyses of benefit and safety, between two treatment arms and, by doing so, generate relevant within-subgroup risk–benefit measures. Their approach emphasizes estimation of heterogeneity in this joint distribution of the primary efficacy endpoint and an adverse event across subgroups and, furthermore, directly accommodates subgroups with small numbers of observed primary efficacy and adverse event combinations. In addition, the authors describe several ways in which their models may be used to generate interpretable summary measures of benefit-risk tradeoffs for each subgroup. The methods described here are illustrated throughout using a large cardiovascular trial (with 9361 individuals) on the efficacy of an intervention for reducing systolic blood pressure to a lower-than-usual target.

2.3 Missing data in confounders when estimating propensity scores

Propensity score models are frequently used to estimate causal effects in observational studies. One unresolved issue in fitting these models is handling missing values in covariates, which are potential confounders, in the propensity score model. As these models may contain a large set of covariates, using only individuals with complete data (ignoring the missing data) can significantly decrease sample size and statistical power and may increase bias. Several missing data imputation approaches have been proposed, including multiple

imputation (MI), MI with missingness pattern (MIMP), and treatment mean imputation. Generalized boosted modeling (GBM), a nonparametric approach to estimate propensity scores, can automatically handle missingness in the covariates by including an additional node for the missingness. Although the performance of GBM and other approaches (such as MI, MIMP and treatment mean imputation) have been previously evaluated for binary treatments, they have not been compared for continuous exposures.

In their publication, "Addressing missing data in confounders when estimating propensity scores for continuous exposures", Coffman, Zhou, Cai and Graham compare GBM and other approaches in estimating the generalized propensity score for a continuous exposure using both a simulation study and an empirical dataset. Using GBM with the incomplete data to estimate the GPS did not perform well in the simulation (even under the assumption of missing completely at random). The authors recommend that missing values should be imputed before estimating propensity scores using GBM or any other approach for estimating the GPS. If GBM is used following imputation, then the bias due to the missingness disappears—that is, single imputation or multiple imputation by GBM is no more biased than GBM on the complete data prior to introducing the missingness.

2.4 A conversation with Anirban Basu

As a health economist and a statistician who specializes in research on comparative and cost-effectiveness analyses, causal inference methods, program evaluation and outcomes research, Anirban Basu is currently the Stergachis Family Endowed Director of the Comparative Health Outcomes, Policy, and Outcomes (CHOICE) Institute and Professor of Health Economics at the University of Washington. He is also a Faculty Research Fellow at the National Bureau of Economic Research and a Fellow of the ASA. In addition, he served as one of the panelists for the Second Panel on the Cost-Effectiveness Analysis in Health and Medicine. At ICHPS 2018, Basu was awarded the Mid-Career Excellence Award from the ASA Section on Health Policy Statistics. He was exceptionally and uniquely qualified for this award. Highlights include his providing outstanding service to the Health Policy Statistics Section of the ASA, advancing statistical methodology, advancing methodology in health policy, and performing extensive and highly impactful applied work in medicine and health care.

In this interview, entitled "A conversation including 39 questions with Anirban Basu", A. James O'Malley and Aasthaa Bansal trace Basu's upbringing, schooling, early career, and mid-career phases in order to gain insights into his success. These interviewers also sought Basu's opinions on salient topics or issues.

3 March 2019 issue

3.1 Effect of asthma on subsequent physical activity and obesity in a nationally representative sample

Asthma and obesity are both prevalent conditions that appear related, but the etiology for this association remains unclear. Past findings of associations between asthma and subsequent obesity were not conducted in nationally representative samples and, moreover, relied on regression to reduce confounding.

In the March 2019 issue, the article "Asthma at mid-life is associated with physical activity limits but not obesity after 10 years using matched sampling in a nationally representative sample", by Islam, Rosenbaum and Cataletto evaluates whether asthma precedes obesity and limited physical activity in a nationally representative sample of middle-aged United States adults after adjustment for potential confounding using matching samples. Specifically, the study examines whether asthma is associated with obesity and physical activity limits 10 years later among a subsample from the National Longitudinal Survey of Youth 1979 from individuals who were age 40 at baseline. They address selection bias using inverse-propensity score weighting and confirmed the results with full matching and, with both methods, they estimated new sampling weights (while also incorporating complex sampling weights) so that the sample would remain nationally representative. The results indicate that asthma was not associated with obesity 10 years later, but it was associated with limited physical activity due to health. The authors conclude that asthma disease management programs that encourage physical activity and weight loss as separate goals may encourage people with asthma to maintain physical activity even in the absence of weight loss.

3.2 Pragmatic randomized trials

Randomized clinical trials often serve the purpose of assessing the efficacy and safety of a therapeutic compound. Differing from classical randomized clinical trials, *pragmatic* randomized clinical trials (PrCTs) answer the important question of the effectiveness of a therapy in the "real world", rather than efficacy in a pre-specified patient population. One of the main advantages in conducting PrCTs is the importance on the use of randomization, which cannot be implemented in real-world observational studies. By combining real-world evidence and randomization, PrCTs can be used to inform treatment effectiveness and healthcare decisions. PrCTs include several practical elements regarding eligibility, endpoints and follow-up and, by doing so, pose unique challenges.

In their article, "Pragmatic randomized clinical trials: best practices and statistical guidance", Gamerman, Cai and Elsaesser use a literature review to propose a definition of PrCT and discuss strategies to overcome some of its challenges. The authors note that the use of alternative data collection approaches may lead to uncertainties. In addition, the absence of blinding could potentially lead to non-random missing data at study endpoints, such that randomization is no longer protected by an intent-to-treat strategy. Therefore, the authors recommend more complex randomization strategies may be needed to minimize bias. The article also notes that additional data sources could be used to synthesize information and create a more accurate endpoint definition, which may require tools such as natural language processing. The authors conclude that the statistician must become familiar with the challenges and strengths of PrCTs, ranging from design to analysis to interpretation, in order to transform data into valid and reliable evidence.

3.3 Average causal effect in observational clustered data

Bias reduction of the average causal effect in non-randomized studies is generally thought as the primary concern in causal inference. One of the primary sources of bias is the confounding effect that differs between the treatment and the control group due to non-randomization in observational studies, including for clustered data.

In their publication, "Model-based inference on average causal effect in observational clustered data", Wu and Yucel examine causal inference in clustered data settings where observational units are clustered in naturally occurring groups (e.g., patients within hospitals). To incorporate the correlated nature of the data, they applied mixed-effects models and a sandwich estimator to make inferences on the average causal effect (ACE). Their methods apply the concept of potential outcomes from the Rubin Causal Model and extend Schafer and Kang's methods of estimating the variance of ACE. On particular, the authors develop two model-based approaches to estimate ACE and its variance under a dual-modeling strategy that adjusts for the confounding effect through inverse probability weighting; these two approaches use linear mixed-effects models for the estimation of potential outcomes, but differ in how clustering is handled in the treatment assignment model. A summary of their comprehensive simulation study is given in assessing the repetitive sampling properties of the two approaches in a pseudo-random simulation environment. In addition, findings are reported from an application to quantify ACE of inadequate prenatal care on birth weight among low-income women in New York State.

3.4 Difference-in-differences estimation for semi-continuous dose treatments

As a popular method in the empirical literature, the difference-in-differences (DID) approach has been increasingly applied to observational and quasi-experimental data. However, its requirement for discrete treatment statuses may not be met in large-scale policy implementations where the treatment levels are more similar to a continuous dosage. The ongoing efforts to promote the medical home model across the nation aim to improve health care outcomes by increasing "medical homeness" with a range of numerical dosages. With these efforts comes the need to develop novel DID methods by relaxing the discrete treatment assumption for analyzing the effects of interventions with semi-continuous dosages, where the treatment can have a range of dosage or exposure levels.

In their paper, "Causal difference-in-differences estimation for evaluating the impact of semi-continuous medical home scores on health care for children", Han and Yu focus on "medical homeness" measured as a semi-continuous score ranging from 0 to 100 to indicate the extent to which a patient-centered medical home model is achieved. The authors develop a causal DID approach to estimating the effects of a treatment with semi-continuous dosages; the approach allows for mixed-type designs and different propensity models. They applied the proposed approach to evaluate the dosage effect of medical homeness scores on the utilization and quality of children's health care. The results indicate a roughly linear effect of medical homeness scores on the annual number of visits to doctor offices when medical homeness scores were below 60 points. The number of office visits did not further increase when medical homeness scores were above 60. A similar relationship was found between medical homeness scores and ratings for health care quality.

3.5 A conversation with Sally C. Morton

Dr. Sally Morton is internationally recognized in the use of statistics and data science to help patients make better healthcare decisions. She has been involved in projects across a wide range of clinical and societal topics such as back pain, healthcare quality, homelessness, mental health, and substance abuse. Holding the Lay Nam Chang Dean's Chair, Morton joined the College of Science at Virginia Tech as Dean in 2016 and is also a professor in its Department of Statistics. Previously, she was chair of Biostatistics at the University

of Pittsburgh, vice president for statistics and epidemiology at RTI International, and head of the RAND Corporation Statistics Group. Morton served as president of the ASA and is a Fellow of the ASA and the American Association for the Advancement of Science. She received the Janet L. Norwood Award for Outstanding Achievement by a Woman in the Statistical Sciences in 2017.

In this interview, entitled "A Conversation with Sally C. Morton: Excellence in Health Policy Statistics", Bonnie Ghosh traces Morton's family and schooling and her leadership and influence on statistics, public policy, comparative effectiveness, health policy, and meta-analytic research. As highlighted in the interview, Morton was awarded the ASA Health Policy Statistics Section's Long-Term Excellence Award in January 2018 at ICHPS. This award recognizes Morton for her "outstanding contributions to the development of statistical methods and innovative statistical applications to health care policy and health services research, for increasing the awareness of health policy statistics in the statistical community, and for significant mentoring and service that advances the aims of the Health Policy Statistics Section."

4 Articles from ICHPS 2018 in the December 2018 issue

Lin, X., Mermelstein, R., Hedeker, D.: A shared parameter location scale mixed effect model for EMA data subject to informative missing. Health Serv Outcomes Res Method. **18**(4), 227–243 (2018).

Henderson, N.C., Varadhan, R.: Bayesian bivariate subgroup analysis for risk-benefit evaluation. Health Serv Outcomes Res Method. **18**(4), 244–264 (2018).

Coffman, D.L., Zhou, J., Cai, X., Graham, J.W.: Addressing missing data in confounders when estimating propensity scores for continuous exposures. Health Serv Outcomes Res Method. **18**(4), 265–286 (2018).

O'Malley, A.J. Bansal, A.: A conversation including 39 questions with Anirban Basu. A shared parameter location scale mixed effect model for EMA data subject to informative missing. Health Serv Outcomes Res Method. **18**(4), 287–297 (2018).

5 Articles from ICHPS 2018 in the March 2019 issue

Islam, S., Rosenbaum, J., Cataletto, M.: Asthma at mid-life is associated with physical activity limits but not obesity after 10 years using matched sampling in a nationally representative sample. Health Serv Outcomes Res Method. (2019). https://doi.org/10.1007/s10742-019-00197-1.

Gamerman, V., Cai, T., Elsäßer, A.: Pragmatic randomized clinical trials: best practices and statistical guidance. Health Serv Outcomes Res Method. (2019). https://doi.org/10.1007/s10742-018-0192-5.

Wu, M., Yucel, R.M.: Model-based inference on average causal effect in observational clustered data. Health Serv Outcomes Res Method. (2019). https://doi.org/10.1007/s10742-019-00196-2.

Han, B., Yu, H.: Causal difference-in-differences estimation for evaluating the impact of semi-continuous medical home scores on health care for children. Health Serv Outcomes Res Method. (2019). https://doi.org/10.1007/s10742-018-00195-9.

Ghosh, B.: A conversation with Sally C. Morton: Excellence in health policy statistics. Health Serv Outcomes Res Method. (2019). https://doi.org/10.1007/s10742-018-0193-4.

Compliance with ethical standards

Conflict of interest Joseph C. Cappelleri is an employee and stockholder of Pfizer Inc. Jason Brinkley is an employee of Abt Associates Inc.

Ethical approval This article does not contain any studies with human participants or animals performed by any of the authors.