EDITORIAL

Guest Editorial: Articles selected from the 2020 International Conference on Health Policy Statistics

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Since 1995, the International Conference on Health Policy Statistics (ICHPS), a biennial conference organized by the Health Policy Statistics Section (HPSS) of the American Statistical Association, has served as a forum to share the latest innovations in statistical methods for health policy, health care and outcomes research. The 13th ICHPS was held January 6–8, 2020 in San Diego, California. With the theme "*Leveraging Data to Shape the Future*", the conference featured an ambitious program covering a broad range of topics (https://ww2.amstat.org/meetings/ichps/2020/onlineprogram/index.cfm). Conference speakers were invited to submit manuscripts for selection into a special issue of Health Services and Outcomes Research Methodology, with us (OH and CC) serving as guest co-editors. After a peer review process, fifteen articles were selected and are appearing in the December 2020 and March 2021 issues of the journal. These articles showcase the people, methods and applications that characterize the ICHPS conference series.

1 Conversations

The December 2020 issue includes conversations with three prominent members of the health policy statistics community: Thomas R. (Tom) Belin, Sherri Rose and Daniel Polsky.

Dr. Tom Belin, Professor of Biostatistics at the University of California Los Angeles, was the 2020 recipient of the Health Policy Statistics Section Long-Term Excellence Award, which recognizes significant contributions to the profession through mentoring and service, for a member who is 15 or more years from their terminal degree. In research, Dr. Belin is internationally known for methodological innovations to address practical problems in incomplete data. Through his many service contributions in academia and professional societies, he is known as a bridge builder across institutions, communities, and individuals. His service to HPSS includes co-chairing the 2005 installment of ICHPS with Dr. Arlene Ash, Professor of Biostatistics and Health Services Research at the University of Massachusetts Medical School. In this interview (Harel and Zigler 2020), Ofer Harel and

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Corwin (Cory) Zigler follow Belin's career trajectory to gain insight into his success and relate Belin's opinions on salient issues and anecdotes from his long career.

Dr. Sherri Rose, Associate Professor at Stanford University in the Center for Health Policy and Center for Primary Care and Outcomes Research and Co-Director of the Harvard–Stanford Health Policy Data Science Lab, received the 2020 Mid-Career Award from the Health Policy Statistics Section. This award recognizes leaders in health care policy and health services research who have made outstanding contributions through methodological or applied work and show promise of continued excellence. Dr. Rose is a renowned expert in machine learning methodology for causal inference and prediction. Her applied work has focused on risk adjustment, algorithmic fairness, health program evaluation, and comparative effectiveness research. The interview with Dr. Rose, conducted by her colleague Dr. Laura Hatfield (Hatfield and Rose 2020), follows Dr. Rose's upbringing and her path from the early stages of her career to becoming a leader in the field. The interview includes many suggestions for junior statisticians.

Dr. Daniel Polsky, Bloomberg Distinguished Professor of Health Economics at Johns Hopkins University, gave the opening keynote address at the conference. This interview (Mitra 2020), conducted by Nandita Mitra of University of Pennsylvania Department of Biostatistics, highlights the achievements in health economics and health policy that made Dr. Polsky uniquely qualified to give this address. The interview focuses on his ideas for bridging the gap between methodological health policy research and policy implementation. Dr. Polsky also has advice for both junior investigators and leaders in statistics on ways to ensure that their research is relevant, translatable, and impactful to policy makers.

2 Intensive longitudinal data

The use of mobile devices, wearables and other biosensors to collect and store huge amounts of health-related data has been rapidly accelerating. Many research studies are using ecological momentary assessment (EMA), in which participants report repeatedly on their symptoms, affect, behavior, and thoughts close in time to their experiences and in their current environment. Such intensive longitudinal data hold the promise of allowing us to answer important questions about the interaction between individuals' psychological states and their environment and social setting. However, analyzing such data requires the development of new, sophisticated statistical methods. In the special issue, we feature two articles tackling challenges of modeling intensive longitudinal data.

Hidden Markov models have proven useful for modeling longitudinal data that involve state-switching processes. In their contribution to the special issue, Lin et al (2020) propose a mixed location scale hidden Markov model for intensive longitudinal data that involve tracking individuals' states. The model allows heterogeneity for both the mean and the within-subject variance, which can achieve better model fit and improve model interpretability. They apply the model to intensive longitudinal data from an adolescent mood study to study the complex interplay between smoking behavior, social environment, and positive and negative mood states.

EMA studies often yield three-level longitudinal data, with assessments nested within days nested within participants. The assessments can also be unequally spaced, with varying time intervals between them. Such data can have complex within-subject correlation structures that need to be modeled correctly to avoid bias. Ma et al. (2020) propose a linear mixed effects model for such data that accounts for autocorrelation at both the within-day

and between-day levels, providing a better fit to the data. This approach represents a practical solution to the issue of modeling EMA data, which we expect to become increasingly common.

3　Real-world data and real-world evidence

While clinical trials are the traditional source of evidence on the safety and efficacy of medical products and drugs, real-world data (RWD) and real-world evidence (RWE) are playing an increasing role in health care decisions. RWD are data on patient health and health care delivery that are routinely collected from such sources as electronic health records and insurance claims activity. RWE is the clinical evidence regarding the potential benefits or risks of a medical product derived from analysis of RWD. The twenty-first Century Cures Act, passed in 2016, placed additional focus on the use of RWD and RWE to support regulatory decision making, including approval of new indications for approved drugs by the Food and Drug Administration. This has accelerated the use of RWD and RWE and the need for innovative statistical methods to handle it.

In their article, Zou et al. (2021) describe how to harness the power of RWD and RWE to help address the global burden of noncommunicable diseases. They note that RWE is essential for understanding the epidemiology of noncommunicable diseases, assisting with the early detection, and evaluating the effectiveness and cost-benefits of treatments, programs, and public policies. Effective development and utilization of RWE will require sustained partnerships between academic centers, governments, pharmaceutical companies, and other stakeholders.

An example of the vital role played by statisticians in harnessing the power of RWD and RWE is displayed in the work of Li et al. (2020), who discuss propensity score-based approaches for leveraging patients from a RWD source to construct a control group for a nonrandomized comparative study or to augment a single-arm or randomized prospective clinical study. The use of propensity score methodology is key here, since it separates the study design from the outcome analysis, which is essential in regulatory settings. The article includes many practical tips from the highly experienced authors.

4　The opioid crisis

Opioid-related mortality increased by nearly 400% between 2000 and 2018, and overdose deaths have accelerated during the COVID-19 pandemic (https://www.cdc.gov/media/releases/2020/p1218-overdose-deaths-covid-19.html), accentuating the need for policies and programs that are effective in reducing opioid-related harms. But which policies are effective? In their paper, Schuler et al. (2021) provide an overview of methodological challenges facing opioid policy researchers and propose ways forward. Their rigorous evaluation of the key challenges, ranging from obtaining high-quality policy and outcome data to addressing confounding and heterogeneity and overcoming limited statistical power, will be of interest not just to opioid policy researchers but also other researchers conducting policy evaluation using observational data.

An example of methodological innovation that address current data deficiencies is contributed by Kline et al. (2021), who propose a multivariate spatio-temporal model that leverages existing surveillance measures on opioid-associated deaths and treatment

admissions to learn about the underlying epidemic for counties in Ohio. Their method synthesizes information from death and hospital admission counts to estimate the common underlying risk, which can be interpreted as the burden of the epidemic. Such results can help policymakers identify the geographic areas that are most affected and allocate resources effectively, as well as facilitate policy evaluation.

5 Causal inference

Methods for causal inference using observational data is a popular topic at ICHPS, which tends to showcase the latest methods in this area. Recently, the arc of methodological development has moved from binary exposure variables to settings with continuous exposure metrics, and from propensity score-based methods to methods for generating weights for balancing samples more directly such as entropy balancing. In their paper, Vegetabile et al. (2021) join these two developments and explore entropy balancing methods in continuous exposure settings. They present methods for estimating population dose–response curves using nonparametric estimation combined with entropy balancing weights. Their presentation focuses on issues of importance to applied researchers.

6 Health care quality and costs

One of the principal objectives of the Health Policy Statistics Section is to focus on strategies for improving the quality and reducing the cost of healthcare in the United States and abroad through the systematic use of quantitative statistical methods (https://www.asahealthpolicy.org/mission-charter). The special issue includes several examples of research that speak to this core mission.

Chen et al. (2021) use Medicare fee-for-service claims data to estimate the effects of Medicare accountable care organization (ACO) programs on hospital admissions across hospital referral regions and provider groups. To accommodate complexities of the data, a model for a difference-in-difference study is articulated in creative ways. Of particular note, they propose a Gaussian mixture model to account for the inability to observe the practice group affiliation of physicians if the organization they worked for did not become an ACO. The results suggest that the ACO programs reduced the rate of hospital readmission, that the ACO program may have reduced heterogeneity in readmission rates, and that the effect of joining an ACO varied considerably across medical groups, findings that are important from policy evaluation.

Healthcare provider profiling attempts to assess the quality of healthcare providers using performance measures. The reliability of a performance measure is particularly important for provider profiling, as it indicates whether observed differences in patient outcomes can be attributed to genuine differences in quality across providers. In their article, Hwang et al. (2021) explore how to best assess the reliability of binary performance measures. They compare candidate definitions and estimators and find important differences across reliability estimators, particularly when the number of patients per provider is low, as expected for physician quality measures. They illustrate the practical implications by examining two process-of-care measures collected by the Centers for Medicare and Medicaid Services in the U.S. for physician quality reporting. Their work makes important contributions to achieving consensus for methods of assessing healthcare quality and value.

In clinical studies, there are benchmarks for defining minimally important differences in clinical outcomes. However, as noted by Dooley et al. (2021) in their special issue article, there is little existing guidance on what constitutes a "small" or "large" difference in costs. Such benchmarks are important for study design and sample size calculation for studies focused on cost comparisons. The authors explore three approaches for deriving minimally important differences in costs for hospital admissions (high cost) and clinic visits (low cost): anchor-based, distribution-based and expert consensus-based. They conclude that the anchor-based method, while logical and simple to implement, may be of limited value in cases where it is difficult to identify appropriate anchors. They recommend that in cases where acceptable anchors are not available, both the consensus-based and distribution-based methods be explored for convergence.

7 Health disparities and vulnerable populations

Addressing health disparities and vulnerable populations are an integral part of health policy research. The special issue includes several examples.

Rosenbaum and DiClemente (2020) evaluated whether the incidence of pregnancy is higher among women reporting reproductive coercion, that is, being forced or deceived into pregnancy by their male partners. They tested the hypothesis using longitudinal data from young adult African-American women recruited from community settings in Atlanta, Georgia. Selection bias was reduced by full matching on 22 baseline variables related to demographics, economic power, risky alcohol use, and gender-based power inequality. They found that women reporting reproductive coercion are at greater risk of unwanted or mistimed pregnancies. They also found that the semen exposure that caused the pregnancies could also transmit serious infections including HIV. Such findings are important for developing policies regarding screening and referral for patients experiencing reproductive coercion.

The homeless population experiences many barriers to health care. In order to study their health care needs and utilization, it is important to be able to accurately identify them in the large administrative data sets that are frequently used for health research. However, there is presently no uniform measure to identify homeless individuals in such datasets. In their contribution to the special issue, Bensken (2021) used state-wide hospital discharge data to identify homeless patient records using either an ICD-10 "Z-code", an administrative indicator, or both. The author found that not only was there little overlap between the two measures, but there were also important differences in the size, composition, and comorbidities of the groups identified by these two measures. This indicates that researchers, clinicians, and policy experts should be wary of current methods to identify homeless individuals and continue to improve capture of this determinant of health.

8 Discussion

Although many conferences involve the healthcare research community, most are not targeted to statisticians and data scientists. ICHPS engages this important segment of the health research community. A core mission of the conference is to link methodologists with other stakeholders to ensure that research answers questions important to patients and the community and produces impact. The 13th ICHPS involved numerous stakeholder

groups (early-career and late-career researchers, government agencies, patient care organizations) in the planning and delivery of the 3-day conference. In addition to many talks and panel discussion, it featured travel funds for students and numerous workshops on both technical topics and non-technical topics. Feedback from attendees was overwhelmingly positive feedback, with over 85% of participants agreeing that the conference had a suitable program, had good speakers, and was well organized.

The special issue is the culmination of the efforts of many individuals from the Health Policy Statistics Section and the American Statistical Association who worked tirelessly for the success of the conference. We have deep gratitude for all the help and support that we received. We hope you will enjoy the articles and we look forward to seeing you at a future International Conference on Health Policy Statistics.

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