HPSS Newsletter

Summer 2021

Greetings from our 2021 HPSS Chair

Cory Zigler Cory.Zigler@austin.utexas.edu

Dear colleagues,

As we settle into another summer without the privilege of gathering in-person at JSM, I invite our membership to use this newsletter as a reminder that our HPSS community is and has been continually active in health policy research (COVID-related and otherwise), and has proceeded to plan many exciting upcoming events during the virtual JSM and beyond.

I also want to remind everyone that there are many opportunities to get involved in our section, through both official and informal roles. Please contact me directly if you are interested in getting involved – even if you don't even know exactly how! I personally feel very lucky to remain connected to colleagues within HPSS, and participation in section activities is a rewarding way to both contribute to the community and grown your own professional network.

Enjoy the newsletter, and hope to see everyone (virtually, of course) at JSM!

Cory Zigler

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2021/2 HPSS Executive Committee

Chair – Cory Zigler
Chair-Elect 2021– Yuanjia Wang
Past Chair 2020 – Laura Hatfield

Secretary – Lane Burgette
Treasurer – Jason Brinkley
Publications – Miguel Marino & Hwanhee Hong
Council of Sections Rep – Frank Yoon

JSM Program Chair 2021 – Hui Xie
JSM Program Chair Elect 2021 – Mousumi Banerjee
JSM Program Chair Elect 2022 – Summer Han
JSM Program Past Chair 2020 – Lisa Lix

ICHPS Co-Chair: Mike Baiocchi & Ruth Etzioni

JSM 2021
August 8-12, Virtual Conference

Program Chair: **Hui Xie** huixie@uic.edu
Program Chair-Elect: **Mousumi Banerjee** mousumib@umich.edu

JSM 2021 will be again virtual. It is our pleasure to have scheduled a full slate of engaging activities, including invited sessions, our famous HPSS and MHSS joint mixer, student paper competition winners, topic-contributed and speed sessions, round tables and our speaker with lunch.

Four HPSS-sponsored **invited sessions**

- Statistical Research in Rapid Response to COVID-19 Pandemic: Forecasts, Risk Factors, Therapeutics and Vaccine Trials
- Machine Learning Methods for Better-Informed Decision-Making in Heath Care
- Population Diversity Considerations in Clinical Trials
- Using EHRs to Run Pragmatic Trials: Opportunities and Challenges

Four HPSS-sponsored **topic-contributed sessions**

- Presentations from Health Policy Statistics Section Student Paper Winners
- Case Studies in Bayesian Methods for Health Policy Research
- New methods and diagnostics for propensity score matching
- Learning Individualized/Sub-group Treatment Rules in Complex Settings

Two HPSS-sponsored **speed sessions**

- Novel Statistical Methods for COVID Pandemic and Other Current Health Policy Issues
- Causal Inference and Statistical Learning of Intervention and Policy Effects

Three **round table discussion sessions** will provide fantastic opportunities for sharing expertise and experience in a small group setting

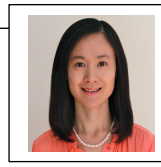
- Anti-racism in the Ivory tower (with Laura Hatfield); Selecting methods to adjust for observed confounders (with Luke Keele)
- Innovative use of EHR to construct family health history for chronic disease risk prediction (with Lisa Lix); Selling the narrative ethically, technically (with Frank Yoon)
- Health policy statistics: stories of adventure, intrigue and speaking truth to power (with Ruth Etzioni)

Finally, we hope to see you at some of our HPSS-sponsored engaging and fun events!

- Aug 11, 12-1 pm EDT. **Lunch with Speaker** "***A Seat at the Table: The Key Role of Statistics and Data Science in the COVID-19 Pandemic***" (with Professor Jeffrey Morris, University of Pennsylvania)
- Aug 9, 5:30-7 pm EDT. **HPSS and MHSS joint mixer** featuring 2021 Student Paper Award Ceremony, Announcement of the 2021 ASA Fellows, Breakout Sessions, and Fun Stat Trivia!

Perspectives on the COVID-19 Pandemic, Part 1

An interview with Dr. Dean Follmann, Chief, Biostatistics Research Branch, National Institute of Allergy and Infectious Diseases (NIAID)



By Yuanjia Wang yw2016@cumc.columbia.edu

COVID-19 pandemic has dominated our lives in the past year. As the pandemic evolved around the world and within the US, it has becoming clear that "science is the only exit strategy" – Sir Jeremy Farrar. Statisticians have been called upon to provide their expertise at the forefront of the fight against the greatest health challenge in recent history. To reveal essential roles statisticians have played in response to the pandemic, the HPSS has interviewed Dr. Dean Follmann, Chief of the Biostatistics Research Branch at the NIAID. Here, we share Dr. Follmann and his team's stories and lessons learned as we look forward to resuming a normal life.

Yuanjia : **What are the most significant challenges you and your team have encountered as statisticians responding to the crisis in the COVID-19 pandemic? How to ensure rigor under the urgency of a rapidly evolving pandemic?**

Dean : *At first, the biggest challenge was to quickly design studies when we didn't know much about the disease. Finding the right study endpoints was critical. For treatment studies we modified an ordinal score used for influenza that had been proposed by the World Health Organization and chose day 15 as the primary time of analysis. As data emerged, we realized a single day might miss a treatment effect. This led to using time to recovery as the endpoint which obviates choice of a specific day for the effect of treatment. After the results of ACTT-1 were announced there was interest and perhaps suspicion why the endpoint had been changed. This required some press statements explaining our rationale. For vaccine trials endpoint discussions focused on whether to use any infection, COVID-19 disease, or severe COVID-19 disease. For the individual, severe disease is most important but reducing infections should reduce transmissions and have a public health benefit. A great vaccine should do it all, but we didn't know if these vaccines would work selectively—or at all. We ended up using any disease as a middle ground—it's meaningful and could achieve an answer much faster than severe disease and we could collect data on infection. Thankfully the vaccines work incredibly well against mild and severe disease.*

Rigor is always important and certainly scrutinized in the fishbowl environment of COVID-19 response. I worked with incredible teams of statisticians and there was a kind of camaraderie and support in rapidly designing trials and then dealing with the next new thing and then the next new thing, all so rapid fire. When things are moving rapidly it greatly helps to have experience and the associated comfort to fly by instinct to tee up solutions, evaluate quickly, and then move on. There was no time to fret. It was kind of liberating, actually.

Yuanjia : **Most vaccine trials use COVID symptoms as the primary endpoint. What are the barriers to track infections? How can different variants be handled in trials?**

Dean : *We were quite concerned early on about infections and the possibility that the vaccines might not eliminate infections but rather shift the distribution of infection severity to mostly infection without symptoms which could increase transmission risk. But to directly measure infection you need to swab everyone in the trial frequently, weekly or twice weekly, and it just wasn't possible. Being practical we chose to measure infection indirectly by infrequent blood draws to check if people had seroconverted, i.e. developed antibodies to SARS-CoV-2 proteins not in the vaccines.*

Continued on next page…

Dean : *Variants are both fascinating and inevitable. I think we hoped they might take longer to emerge, but they are the central issue for vaccines now. Within the trials, we sequence the infecting strain for each infected individual and thus can look at vaccine efficacy by each strain or collection of strains by basically defining the endpoint as disease by a given strain. It's basically a competing risks approach. Beyond that, there are exciting approaches to give a score for each infected individual that measures how well the vaccine worked against that individual's viral sequence in a personalized neutralization assay. We can thus form models where vaccine efficacy depends smoothly on this assay score.*

Yuanjia : **Long term vaccine effectiveness is central to health policy decisions such as when to administer a booster shot. Do you have insights on how to evaluate long term vaccine effectiveness?**

Dean : *The vaccine trials were designed to follow people for up to two years as knowing the durability of vaccine effect is incredibly important. Shortly after the trials were launched we could think about the next set of things, including what to do with the placebo volunteers if efficacy was established. Well placebo volunteers should receive the benefit of an effective vaccine. So there was a flurry of work about what could be learned about durability once the placebo group was vaccinated. Kind of magically, you can continue to estimate time-varying placebo-controlled vaccine efficacy long after the placebo group is vaccinated. As in a trial with a placebo group, this result requires similarity of the trial environment over time. The only additional assumption is that the newly vaccinated get the same benefit of vaccine whether in May or October. Emerging variants can be straightforwardly handled provided they occur both before and after the placebo group is vaccinated by looking at variant specific time-varying vaccine efficacy. For variants that emerge after the placebo group is vaccinated, we can still assess if vaccine efficacy is waning—although you can't easily estimate what the efficacy actually is.*

Yuanjia : **What should statisticians do differently now in order to prepare for the next pandemic (e.g., learning the best ways to communicate to policy makers and the public)?**

Dean : *Train to be familiar with immunology, infectious diseases, clinical trials, and the theory underpinning statistical methods for design and analysis. Be in a place where you can trust your instincts. And then quickly verify you were right.*

At the start of a pandemic, there is uncertainty and fear. Communicating what you do know and what you don't know in a clear and simple way is needed. I think if you understand issues in a conceptual way, it is easier to communicate and I always try for conceptual understanding. Dr. Fauci has a nice dictum—precision of thought, economy of expression. I take it to heart and it's on my whiteboard, next to the equations.

Perspectives on the COVID-19 Pandemic, Part 2

Where Science Meets Humanity: A Story of Suffering and Love in India

By Mousumi Banerjee mousumib@umich.edu

In March, I went to Kolkata India, my hometown, to get my 84-year old mother vaccinated. My mother suffers from progressive dementia and multiple other comorbidities. **On March 15, India recorded 24,437 new cases and 130 deaths**.

Three days after I arrived, my mother and her two caregivers received their first shots of Covishield, the Indian version of the Astrazeneca-Oxford vaccine. My plan was off to a good start.

Things began changing rapidly a week later. India experienced a sharp spike in cases. *It was the beginning of a storm.* **On March 27, there were 62,632 new cases of COVID-19, the highest since October 2020**.

The storm continued as India celebrated festivals and five states held elections. Amid this perfect storm of careless crowds, loose government guidelines, and viral mutations, the Indian health care system collapsed. People of all ages are dying outside hospitals and on the streets.

On April 30, India recorded 402,014 new cases and 3,525 deaths. Even as India tries to produce and supply vital vaccines to the rest of the world, COVID-19 vaccines are in short supply in India. People queue up at 4am for a shot and vaccination centers thronged with people become a breeding ground for fresh infections.

At times like these humanity rises. It has to. You might live on the other side of the world from India, but this is not India's problem alone. This pandemic has reminded us again how interconnected the human race is. We cannot fail to act on the responsibilities we have as a global community.

Prior to the return flight I had booked, I had a mandatory COVID-19 test. It came back COVID positive. I was asymptomatic and shocked at the result, given how careful I had been throughout my stay. Still, I isolated myself. I cancelled my flight to the US, not knowing when I would get another booking. My children, at our home in Michigan, awaited their mother's return. I feared the US borders closing down to travelers from India.

As a data scientist, I know all about uncertainty. But I couldn't handle this uncertainty in my own life.

Six days later, fresh tests at two different labs both came back negative. The emotional wreckage from the earlier false positive was huge. But I was cared for and nurtured by friends and family in India. I returned to the US in early May and quarantined again. I got another test result—negative.

I carry with me a lot of hurt and pain. My hometown is burning. People I love are living under dangerous conditions, in fear of losing their lives. India—and the whole world—continues to suffer.

But I knew I had to leave. My on-the-ground job in India was done. *It is time now to mobilize my energy and expertise to advance public health in the ways only I can.* I research human disease, and support health care professionals and policy makers to develop ways of preventing and treating the suffering that comes from disease.

With a prayer for each of you, I ask you to practice safety guidelines. Protect yourself, your family, and your community. Protect India, protect your own nation, protect humanity, because at the end we are one world.

I shall see you all at dawn. That's a promise.

Reprinted and edited with permission based on the original article: https://sph.umich.edu/pursuit/2021posts/where-science-meets-humanity-a-story-of-suffering-and-love-in-india.html

Call for Submissions: HPSS Discussions on Health Policy and Trainee Mentoring Studios

HPSS is seeking submissions from student and postdoctoral trainees to participate in informal research discussions and mentorship studios with the chance to have your work highlighted in a virtual HPSS-wide discussion event!

Each applicant will be assigned to a group of at most 4 other trainees and a single senior mentor, with groups formed according to research topic. The senior mentor will facilitate a ***virtual studio event*** with the group that will provide an opportunity to:

- Meet other trainees working on similar topics in an informal setting
- Receive informal mentorship from a senior colleague working in a similar area
- Obtain feedback on your completed or ongoing research project

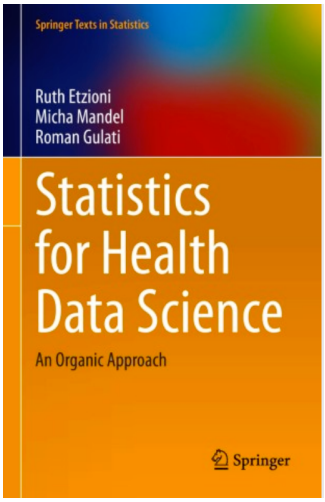
After the mentorship studios, ***selected participants will have their work featured in an HPSS-sponsored virtual event*** consisting of brief research talks and discussion.

Application Requirements: A 1-page .pdf "digest" containing: (1) Title of research project; (2) Name affiliation of trainee and all collaborators; (3) Research abstract describing objectives, methodology, and key (anticipated) results; (4) A statement describing impact and relevance for health policy; (5) Names of up to 5 suggested senior colleagues from whom you might like feedback/mentorship. Submit applications via email to: Professor Cory Zigler Cory.Zigler@austin.utexas.edu

Timeline: Applications must be received by September 1, 2021. Research studios are expected to be held during October 2021, with HPSS-wide virtual events held to feature selected participants in January 2022.

New Textbook about Health Data Science

We are excited to share news about the publication of a new textbook that our readers may want to check out. The book is, "**Statistics for Health Data Science: An Organic Approach**." According to co-author Ruth Etzioni, a past HPSS chair and co-chair of ICHPS 2023, book was a "labor of love" that grew out of a methods course she developed for non-biostats PhD students at the University of Washington. The book differs from the competition in its blend of methods and its determination to ensure that readers gain an understanding of how, when, and why to apply them. Powered by topical examples and stories, the chapters empower readers to take charge of their own health data studies using traditional statistical models, health econometrics methods, and predictive algorithms. The book is available on Amazon and directly from Springer Nature.



- Endorsement by the University of Washington: https://www.biostat.washington.edu/news/stories/biostatistics-and-health-services-faculty-member-ruth-etzioni-co-authors-data-science
- Endorsement by Andrew Gelman: https://statmodeling.stat.columbia.edu/2021/01/21/new-textbook-statistics-for-health-data-science-by-etzioni-mandel-and-gulati/

Perspectives on the COVID-19 Pandemic, Part 3
Behind the Scenes of COVID-19 Vaccine Review – Interview with Butch Tsiatis

By Christy Sadler

Since last year, Anastasios "Butch" Tsiatis, professor emeritus of statistics, has served on the Data and Safety Monitoring Board (DSMB) for the U.S. government-sponsored clinical trials evaluating COVID-19 vaccines such as those developed by Moderna, AstraZeneca, Johnson & Johnson and Novavax. He talked to us about the work of the Data and Safety Monitoring Board and how statistics expertise can help in evaluating the COVID-19 vaccines.

Describe the charge and composition of the Data and Safety Monitoring Board.

The purpose of the DSMB is to ensure the safety of study participants and the rigor and integrity of the clinical trials that it monitors. The COVID-19 vaccine DSMB is responsible for reviewing and monitoring all U.S. government-supported clinical trials of candidate COVID-19 vaccines.

The board consists of 11 members from the United States, Brazil, South Africa and the United Kingdom, and includes experts in infectious disease, vaccinology, immunology, biostatistics, pharmacoepidemiology, public health and biostatistics.

When did you begin serving on the board, and how were you appointed?

On June 9, 2020, I received a letter from Dr. Anthony Fauci inviting me to serve on the COVID-19 vaccine DSMB. I was honored to be asked and immediately accepted to serve.

What has been your role on the board?

When a clinical trial is first proposed, the other statisticians on the board and I carefully review the statistical analysis plan and check all the calculations for the sample and the analyses.

How does statistics expertise apply to this project?

At each meeting, we are presented with a plethora of data on both vaccine efficacy and adverse events. We are trained to understand uncertainty and can help guide the DSMB to distinguish between real effects and those that may have occurred by chance.

Outline a typical review process for a vaccine. What are some factors you and your colleagues consider in evaluating them?

Once a trial begins enrolling, our reviews focus on three main elements: trial conduct, safety and vaccine efficacy. The DSMB examines metrics related to trial conduct such as proportions of participants in relevant subgroups and the quality of data. When a vaccine recipient experiences an adverse reaction, the DSMB must assess the likelihood that it was related to the vaccine and, if so, whether it recommends changes to the protocol or informed consent documents or whether the trial should be paused pending further investigation. The DSMB also reviews two types of efficacy analyses to determine whether accumulating data suggest that it is highly unlikely that a vaccine will meet specified criteria for effectiveness or whether they show convincing evidence of efficacy by surpassing stringent, prespecified criteria.

In what ways does the work of this group add to the evidence that the approved vaccines are safe?

The COVID-19 vaccine trials have been perhaps the most politicized trials in history, but the DSMB has focused throughout on its primary goals, the safety of study participants and the integrity and scientific validity of the trials.

Reprinted and edited with permission based on the original article: https://sciences.ncsu.edu/news/behind-the-scenes-of-covid-19-vaccine-review

ICHPS News

Special issues from the ICHPS 2020

The 13th International Conference on Health Policy Statistics (ICHPS) held in January 2020 has been summarized into fifteen peer-reviewed articles in 2 special issues of Health Services and Outcomes Research Methodology

- December 2020: https://link.springer.com/journal/10742/volumes-and-issues/20-4
- March 2021: https://link.springer.com/journal/10742/volumes-and-issues/21-1

Catherine Crespi and Ofer Harel, past ICHPS co-chairs, overviewed the work, people, methods and applications in this editorial: https://link.springer.com/article/10.1007/s10742-021-00240-0

ICHPS 2022 is Happening – in 2023!

The Health Policy Statistics Section together with ASA are planning the 14th International Conference on Health Policy Statistics! Originally planned for January 2022, this conference will now be held in January 2023 with high hopes that we will be able to meet in person somewhere sunny. Conference co-chairs are Mike Baiocchi (mike.baiocchi@gmail.com) and Ruth Etzioni (retzioni@fredhutch.org). In the coming months we will settle on a conference theme and location, and will be soliciting suggestions for keynote speakers, workshops and invited sessions. ICHPS is a smaller meeting (typically 300-350 attendees) that provides an excellent opportunity to get to know a community of statisticians and other professionals working in the health policy and health services arena. We look forward to being able to gather and share science and community at ICHPS 2023!

Events! Meetings! Dates! Deadlines!

JSM 2021 – Joint Statistical Meetings: Virtual, August 8-12, 2021
https://ww2.amstat.org/meetings/jsm/2021/index.cfm
Early registration: May 17 - June 15, 2021
Regular registration (with increased fees): June 16 – July 15, 2021
Late registration (with increased fees): July 16 – August 12, 2021

WSDS – Women in Statistics and Data Science: Virtual October 6-8, 2021
https://www.amstat.org/ASA/Meetings/Women-in-Statistics-and-Data-Science.aspx
Early registration: May 27 - August 19, 2021
Regular registration (with increased fees): August 20 – October 9, 2021

SMDM – Society for Medical Decision Making: Virtual October 18-20, 2021
https://smdm.org/meeting/43rd-annual-north-american-meeting

ENAR 2022 – Eastern North American Region: Houston, TX March 27-30, 2022