BASS XXXIII

Thirty-third Annual Biopharmaceutical Applied Statistics Symposium

November 2-4, 2026
Savannah, GA

Schedule of Events

Sunday, November 1, 2026

Registration 4:00–6:00 p.m.

Monday, November 2, 2026

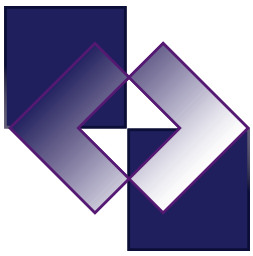
Registration 8:00--9:00 a.m.
Keynote Address 9:00–10:00 a.m.
Morning Break 10:00–10:30 a.m.
Tutorial 10:30–12:00 p.m.
Lunch Break 12:00–1:00 p.m
Tutorials 1:00–3:00 p.m.
Afternoon Break 3:00–3:30 p.m.
Tutorial 3:30–4:30 p.m.
Banquet Dinner 7:00–8:30 p.m.

Tuesday, November 3, 2026

Tutorial 9:00–10:00 a.m.
Morning Break 10:00–10:30 a.m.
Tutorial 10:30–11:30 p.m.
Lunch Break 11:30–12:30 p.m.
Tutorials 12:30–2:30 p.m.
Afternoon Break 2:30–3:00 p.m.
Poster Session: 3:00–4:00 p.m.

Wednesday, November 4, 2026

FDA Session 8:30–10:30 a.m.
Morning Break 10:30–11:00 a.m.
Panel Discussion 11–12:00 a.m.



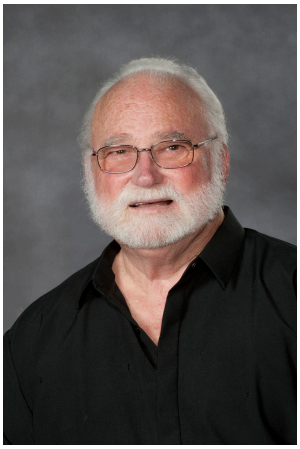
Monday, November 2

Keynote Address

9:00 a.m.

Title: Notable Events in a Purpose Driven Life – Filling Gaps

Presenter: Dr. Karl Peace, BASS Founder, Senior Research Scientist and Professor of Biostatistics in the Jiann-Ping Hsu College of Public Health at Georgia Southern University



Dr. Karl E. Peace is currently the Georgia Cancer Coalition Distinguished Cancer Scholar (GCCDCS), Senior Research Scientist and Professor of Biostatistics in the Jiann-Ping Hsu College of Public Health (JPHCOPH) at Georgia Southern University. He is the architect of the MPH in Biostatistics, Founding Director of the KEP Center for Biostatistics and Founder of the JPHCOPH. Dr. Peace holds the Ph.D. in Biostatistics from the Medical College of Virginia, the M.S. in Mathematics from Clemson University, the B.S. in Chemistry from Georgia Southern College, and a Health Science Certificate from Vanderbilt University. He was awarded an Honorary Doctorate Degree from Ismail Qemali University of Vlora, Albania.

Dr. Peace's first career was that of teaching and research at the University level. He previously taught at Georgia Southern College, Clemson University, Virginia Commonwealth University, and Randolph-Macon College, where he was a tenured professor of Mathematics. He holds or has held numerous adjunct professorships: at the Medical College of Virginia, the University of Michigan, Temple University, the University of North Carolina, and Duke University.

Dr. Peace's second career was in research, technical support and management in the pharmaceutical industry. He held the positions of Senior Statistician at Burroughs-Wellcome, Manager of Clinical Statistics at A.H. Robins, Director of Research Statistics at SmithKline and French Labs, Senior Director of GI Clinical Studies, Data Management and Analysis, at G.D. Searle, and Vice President of World-Wide Technical Operations at Warner Lambert/Parke-Davis. He then founded Biopharmaceutical Research Consultants, Inc. (BRCI), where he held the positions of President, Chief Executive Officer, and Chief Scientific Officer. BRCI was a contract research organization (CRO) providing a full line of services attendant to the research and development of pharmaceutical compounds, particularly with regard to the conduct of clinical (trial) investigations.

Dr. Peace has made pivotal contributions in the development and approval of drugs to treat Alzheimer's disease, to prevent and treat gastrointestinal ulcers, to reduce the risk of myocardial infarction, to treat anxiety, depression and panic attacks, to treat hypertension and arthritis, and several antibiotics.

Dr. Peace is or has been a member of several professional and honorary societies, including the Drug Information Association (DIA), the Regulatory Affairs Professional Society (RAPS), the Biometric Society, Technometrics, the American Society for Quality Control (ASQC), Biometrika, the American Statistical Association (ASA), and Kappa Phi Kappa (KPK). He is a past member of the Committee on Applied and Theoretical Statistics, National Research Council, National Academy of Science.

He is the recipient of numerous citations and awards: (1) Georgia Cancer Coalition Distinguished Cancer Scholar, (2) Fellow of the American Statistical Association, (3) the Distinguished Service Award of the DIA, (4) Deen Day Smith, Statesboro Herald Humanitarian of the Year, (5) Recipient of the GPHA Sellers-McCroan Award for Improving Public Health through academic and laboratory advancement, (6) College of Science and Technology Alumnus of the year, Alumnus of the year in private enterprise, Presidential Fellowship Award, the First Recipient of the prestigious President's Medal for outstanding service and extraordinary contributions, and the President's Visionary Award, all from Georgia Southern University, (7) Star and Featured Alumnus, School of Basic Sciences, Founder's Society Medal, and the Inaugural Public Health Fellow, all from the Medical College of Virginia, and (8) several meritorious service awards from the ASQC and BASS. He is or has been Chair of the Biostatistics Subsection of the Pharmaceutical Manufacturers Association (PMA), the Biopharmaceutical Section of the ASA, the Training Committee of the PMA Biostatistics Subsection, and is Founder and Chair of the Biopharmaceutical Applied Statistics Symposium (BASS).

Dr. Peace founded BASS in 1994, to provide an opportunity for biopharmaceutical professionals to share insight to common biopharmaceutical problems in a timely manner, and to provide a source of funding to support graduate work in Biostatistics. Attendees and presenters at BASS derive from academia, the pharmaceutical industry and the US government, particularly the Food and Drug Administration. BASS is one to the premier conferences in the world and represents a first-rate educational opportunity. As of 2006, BASS has raised approximately $2,000,000.00, with which endowments at the Medical College of Virginia and Georgia Southern University have been created. These endowments have supported over 100 students in attaining the Ph.D. or DrPH in Biostatistics, the MPH in Biostatistics, or the MD.

Dr. Peace has a lengthy record of philanthropy to education. He has endowed eighteen scholarships at five institutions. Eleven (11) of these are at his undergraduate alma mater, Georgia Southern University including 5 for students from his native Baker County, Georgia (two of these honor his high school mathematics teacher, Mr. Bill Tom Reeves, and principal, Mr. Joe Vines, and three honor his son, sister and brother). Three (3) are at his Ph.D. alma mater, the Medical College of Virginia. Two (2) are at the Ph.D. alma mater of his late wife, the University of California at Berkeley (UCB). One (1) is at Randolph-Macon College. And one (1) is under the umbrella of the International Chinese Statistical Association.



In addition, he endowed the first eminent scholar chair at Georgia Southern, and his gift of 2.5 million dollars to honor his late wife, effectively created the Jiann-Ping Hsu College of Public Health at Georgia Southern – the first school of public health in the University System of Georgia. He also established the first Endowed Chair in Biostatistics at UCB: the Jiann-Ping Hsu/ Karl E. Peace Chair. As of 2026, these endowments have supported more than a 200 students in various degree seeking programs.

Dr. Peace has also given generously in terms of time and money to various organizations in their fight against the war on cancer. These include the American Cancer Society, the Georgia Cancer Coalition, the Southeast Georgia Cancer Alliance and the Southwest Georgia Cancer Coalition.

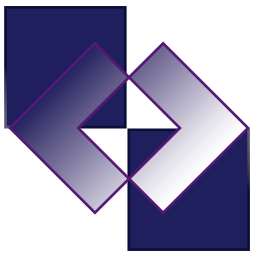
Dr. Peace has published extensively in the pharmaceutical, statistical, medical and scientific literature. He is the author or co-author of over 150 articles and 15 books and the editor or reviewer of several peer reviewed journals, including the founding editor of the Journal of Biopharmaceutical Statistics, now in its 33th year. One of his books: Biopharmaceutical Statistics for Drug Development has been translated into several foreign languages including Japanese, and has been used extensively as 'the bible for drug development' in the Japanese pharmaceutical industry. He has given over 200 invited presentations in the scientific, statistical, medical and pharmaceutical arenas all over the world.



Abstract:

Karl Peace traces his life from its humble beginning in Baker County, located in southwest GA. Born to tenant farmer parents with little education, he presents vivid accounts of sharecroppers' existence in the 1940s and 1950s. He became the first in his family to earn a high school diploma and later a college degree. Upon earning a Ph.D. in Biostatistics, he became the first individual from rural Baker County to claim such a lofty educational status. After a career as a classroom instructor at the college and university level, he accrued a long career in the pharmaceutical industry and made major contributions in the areas of research and clinical development of pharmaceutical compounds. He then founded the CRO:BRCI, and after giving it to his wife, he returned to his undergraduate alma mater to develop a degree program in biostatistics (Master's) and found the Jiann-Ping Hsu College of Public Health – both firsts in the University System of Georgia.

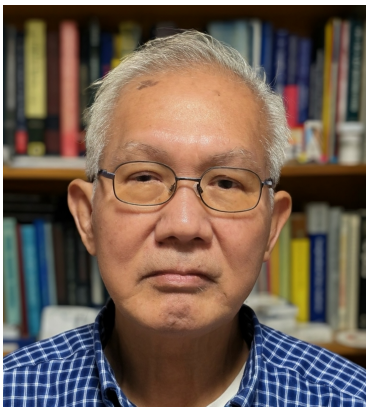
This presentation is an account of a man's life that recognizes achievement under difficult circumstances and the importance of helping others. Notable other events that represent filling needed gaps are highlighted.



10:30 a.m.

Title: Applications of Artificial Intelligence & Machine Learning in Healthcare and Clinical Research

Presenter: Dr. Kao-Tai Tsai



Kao-Tai Tsai obtained his PhD in Mathematical Statistics from University of California, San Diego, and worked at AT&T Bell Laboratories to conduct statistical research, modeling, and exploratory data analysis. He later joined the US FDA and then various pharmaceutical companies, focusing on biostatistics, clinical trial research, and data analysis to address the unmet needs in human health.

He has been active in the statistical profession and has engaged in numerous invited lectures, short courses, presentations, and seminars on practical statistical issues related to clinical trials.

In addition, he was President of the NJ chapter of the ASA, served on the Board of Directors and various committees of ICSA and BASS, and organized symposia for several professional organizations. His recent research focuses on topics in clinical trials, biomarkers, big data analysis, and applications of statistical graphics in data analysis. He is the author of the book Machine Learning for Knowledge Discovery with R, Methodologies for Modeling, Inference and Prediction, published by Chapman and Hall/CRC in 2021.

Abstract:

BASS has been 33 years young and still growing strong after all those years, at the same time, the healthcare and clinical research and other scientific areas also have great advances, respectively. It is critical for us to incorporate the advances of other areas, such as artificial intelligence and machine learning (AI/ML), into healthcare and clinical research to produce greater health benefits for the general public.

In this presentation, the definitions of AI/ML will be described based on the seminal research in computer science, and a general framework of AI/ML life cycle will also be described with the emphasis of interpretability to make it more transparent, to increase the trustworthy, and the confidence of the general users and the regulatory agencies. After this, it will be apparent to users about how the AI/ML framework can be custom-made for various kinds of research. More details will be discussed especially for clinical research to fit the primary theme of the BASS conference. Examples will also be presented for illustrations.

Lunch 12:00 p.m. – 1:00 p.m.

1:00 p.m.

Title: From Statistician to Trusted Drug Development Leader: Statistics, Strategy, and Scientific Judgment

Presenter: Dr. Gary Cline, Cline Consulting



Gary has more than 25 years' experience in the pharmaceutical industry, including roles at ClinTrials, Procter & Gamble, ICON Clinical Research, Celgene and AstraZeneca where he has served on various teams and governance committees. He has had various leadership responsibilities covering data science & AI, strategic research and innovation, biostatistics, decision science, programming and data management across many therapeutic areas. He led Biometrics aspects for discovery and development programs, submissions and commercial activity for many products. He currently leads a consulting business, Cline Consulting, LLC. Recent activity includes serving on the board of the Washington Statistical Society, legal expert and drug development consulting. Gary received his MS in Mathematics at Eastern Kentucky University and PhD in Statistics at the University of Kentucky.

Abstract:

Successful statistical engagement requires more than technical expertise. It depends on methodological breadth, effective communication, sound scientific judgment, and the ability to help multidisciplinary teams make informed decisions under uncertainty. This presentation outlines actions statisticians can take early, middle, and late in their careers to prepare for expert collaboration, using many examples from pharmaceutical development, biotechnology, regulatory strategy, due diligence, and litigation support.

Early-career preparation should emphasize study design, randomization, sample-size determination, programming, data quality, statistical logic and methodology, and reproducible documentation. Mid-career development should expand on honing those skills while broadening and applying insights from early career experiences. Late-career preparation centers on integrating insights across multiple stages of development and therapeutic areas.

2:00 p.m.

Title: Evaluating Machine Learning-Based Causal Inference Methods in Rare-Disease Trials

Presenters: Dr. Ahrim Youn, Sanofi



Ahrim Youn is a Senior Statistical Project Leader at Sanofi, where she provides statistical leadership for the global fitusiran development program. Since joining Sanofi in 2021, she has supported statistical methodology development and novel clinical trial designs, including Phase 2/3 seamless trials, predictive modeling of efficacy and adverse events, dose-response modeling, causal inference applications, and machine learning approaches across multiple therapeutic areas.
Before joining Sanofi, Ahrim held roles at Bristol Myers Squibb/Celgene, The Jackson Laboratory for Genomic Medicine, Columbia University, and the National Cancer Institute.

Her expertise spans oncology and hematology clinical trials, biomarker discovery, statistical genomics, cancer driver gene identification, and integrative pan-cancer analyses. She earned her Ph.D. in Statistics from the University of Washington and has authored numerous publications in clinical trial methodology, causal inference, predictive modeling, statistical genomics, and computational biology.

Abstract:

Background: Rare-disease trials frequently involve small sample sizes, longitudinal outcomes, missing data, and potential reliance on external control arms (ECAs). These features may limit statistical power and challenge the validity of both conventional and machine learning-based causal inference methods. We conducted a simulation study to compare the operating characteristics of causal estimators under conditions motivated by a Phase 3 trial in Gaucher disease type 3.

Methods: Synthetic longitudinal data were generated using distributions informed by trial data, with change in Repeatable Battery for the Assessment of Neuropsychological Status total score as the endpoint. Randomized controlled trial samples of 30 or 60 participants were evaluated under null and alternative treatment effects. Scenarios varied the prognostic strength of baseline covariates, missing-at-random and missing-not-at-random mechanisms, and the presence of an ECA with or without systematic selection bias.

Ten approaches spanning conventional, semiparametric, and machine learning methods were assessed, including mixed models for repeated measures (MMRM), propensity-score matching, inverse probability weighting, g-computation, causal forests, prognostic covariate adjustment (PROCOVA and PROCOVA-MMRM), and point-treatment, longitudinal, and adaptive targeted maximum likelihood estimation (TMLE). Performance was evaluated using bias, variance, Type I error, and power.

Results: In randomized-trial-only analyses, fully covariate-adjusted MMRM provided a favorable balance of stability, Type I error control, and power, particularly when covariates were strongly prognostic. Standard and longitudinal TMLE exhibited Type I error inflation at both sample sizes, associated with anti-conservative variance estimation. G-computation was unstable at N=30 when the number of adjustment covariates was large but performed better at N=60. All methods were biased under missing-not-at-random outcome data, highlighting the limitations of analyses that do not explicitly model nonignorable missingness. Incorporating exchangeable external controls generally improved power. However, under ECA selection bias, simple pooling and several weighting or matching approaches produced substantial bias or Type I error inflation. PROCOVA, g-computation, and, at the larger sample size, adaptive TMLE were comparatively robust to nonexchangeable external populations.

Conclusions: Method complexity did not guarantee superior performance in rare-disease settings. Fully covariate-adjusted MMRM remains a strong choice for small randomized trials, while carefully implemented PROCOVA and g-computation may enable effective external-data borrowing. The validity of ECA analyses depends critically on transportability, positivity, variance estimation, and missing-data assumptions. Simulation-based evaluation tailored to the intendedtrial design should therefore precede the use of machine learning-based causal estimators in confirmatory analyses.

Break - 3:00 p.m.



3:30 p.m.

Title: On Estimands in the Analysis of Safety Data and the Handling of Intercurrent Events

Presenter: Dr. Ed Whalen,Pfizer

Ed Whalen works at Pfizer Inc on clinical studies in the internal medicine group. His statistical work has helped support the development, risk assessment, and clinical trial analyses of several approved drugs including the assessment of pregabalin for presentation at an FDA advisory committee on antiepileptic drugs suicidality risks.

Abstract:

The PHUSE safety estimands working group has worked through the application of the estimand process to safety endpoints in clinical studies. They do so in five main parts: (1) an overall introduction to the estimand process and how it can apply in the safety data setting, (2) specifics on the handling of intercurrent events, (3) moving from estimands to estimators, (4) how to communicate and implement the estimand process, and (5) specific issues such as aggregation across multiple studies. The current presentation will briefly outline the overall estimand process in the safety context then focus primarily on the handling of intercurrent events. Examples, both simple and mildly complex will be presented as well.

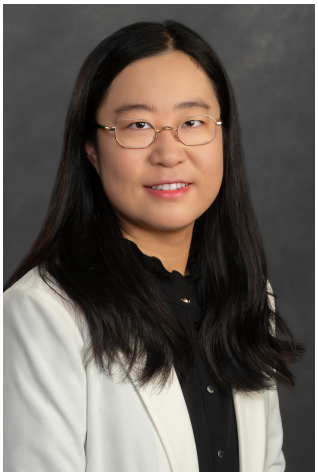
Banquet Dinner - 7:00 PM

Tuesday, November 3

9:00 a.m.

Title: Advancing Precision Medicine: From Targeted Therapy Development to Individualized Treatment Recommendation

Presenter: Dr. Na Bo, Virginia Commonwealth University



Dr. Na Bo is Assistant Professor of Biostatistics at Virginia Commonwealth University. Her research expertise is in causal inference and survival analysis, particularly in estimating heterogeneous treatment effect and subgroup identification. She also works on deep learning methods and applications for survival data.

Abstract:

One crucial aspect of precision medicine is to provide sufficient information for physicians to recommend the most suitable treatment for their patients, which requires understanding heterogeneous treatment effects from a patient-centric perspective.

However, traditional approaches to evaluating treatment effectiveness rely on randomized clinical trials (RCTs) that focus on average treatment effects, often overlooking the treatment heterogeneity driven by unique patient characteristics.

Throughout the presentation, I will focus on a clinical research question: whether the patient could survive longer if he receives a treatment versus an alternative?

With advances in biomarker-guided therapies, modern RCTs focus on developing therapies to target a subgroup of patients. In Part I, we propose a new subgroup identification and inference procedure using a novel efficacy measure, living longer probability (LLP). Using LLP as efficacy measure, we developed a simultaneous inference procedure to identify and infer subgroups with differential treatment effects while appropriately controlling multiplicity. To enhance interpretability, we link LLP to the two-sample survival probability curve, providing an intuitive visualization that maps the control-group survival to the treatment-group survival over time. Extensive simulations were conducted to evaluate Type I error control and power.

We will illustrate the proposed methods using Age-Related Eye Disease Studies (AREDS), RCTs evaluating whether nutritional supplements can delay age-related macular degeneration (AMD)—a progressive, polygenic, and neurodegenerative eye disorder. The proposed approach offers both intuitive visualization and rigorous inference to identify subgroups to be targeted with differential treatment effects.

While subgroup identification provides valuable insights into treatment heterogeneity, it lacks quantifying the individualized treatment effect (ITE) for each patient. In Part II, I will focus on predicting ITE in survival data by proposing an interpretable ITE prediction framework that integrates meta-learners with tree-based methods to estimate the conditional average treatment effect for survival outcomes under causal inference framework and identify predictive subgroups simultaneously. We will evaluate the performance of our method through extensive simulation studies. We will demonstrate its application using AREDS to predict ITE of an antioxidant and mineral supplement on time-to-AMD progression and to identify genetically defined subgroups with enhanced treatment effects. Our method offers a direct interpretation of ITE prediction and provides evidence to support precision healthcare.

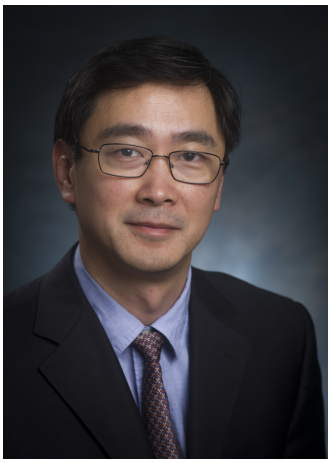
Break 10:00 a.m.



10:30 a.m.

Title: Open Drug Discovery at the Speed of AI

Presenters: Dr. Jake Chen, The University of Alabama at Birmingham



Dr. Jake Y. Chen is the Triton Endowed Professor of Biomedical Informatics and Data Science at the UAB School of Medicine, with joint appointments in Genetics, Computer Science, and Biomedical Engineering, and is the founding director of the Systems Pharmacology AI Research Center (SPARC). Over 25 years, he has advanced AI-driven biomedical informatics, network/systems biology, and computational drug discovery—developing systems pharmacology models, multi-omics AI frameworks, and digital twin simulations that integrate clinical, genomic, and real-world data for precision medicine. He has 200+ peer-reviewed publications and 200+ invited talks worldwide. Dr. Chen also leads and convenes large, multi-institutional collaborations and contributes to national biomedical AI strategy and translational informatics infrastructure.

He serves as the Contact MPI for CONNECT, an NIH U54-funded national AI-infrastructure initiative (2024–2029) focused on building AI-ready biomedical knowledge networks and has advised NIH and the U.S. Congress (via NIDDK panels) while collaborating with FDA efforts related to AI-driven biomedical innovation. A recognized leader in AI-driven systems pharmacology, he is a Fellow of ACMI, AIMBE, and AMIA, an ACM Distinguished Member, and has received honors including the "Top 100 AI Leaders in Drug Discovery and Healthcare" (Deep Knowledge Analytics, 2019) and the CAST-USA Pioneer Award (2023).

Abstract:

Drug discovery remains slow, costly, and failure-prone, even as biomedical data and AI methods rapidly expand. In this talk, I will discuss how "open drug discovery" can be reimagined for the AI era by combining open evidence, AI-ready biomedical data, knowledge graphs, virtual-cell and digital-twin models, and human-supervised AI agents. Rather than presenting AI as a black-box shortcut from prompt to drug, I will focus on a more rigorous open discovery loop: reading the literature, integrating disease mechanisms, prioritizing therapeutic targets, generating candidate molecules or biologics, and identifying the next experiments needed for validation.

I will illustrate this approach with case studies from the Systems Pharmacology AI Research Center, including AI-assisted pancreatic cancer target prioritization and a PHGDH allosteric modulator project for Alzheimer's disease that moved rapidly from a newly published biological insight toward candidate discovery and intellectual-property action. The broader vision is Open Source Drug Discovery 2.0: a model in which open evidence and open tooling accelerate early discovery, while pragmatic IP-gating and translational partnerships help promising candidates move toward real-world therapeutic development.

Lunch 11:30 a.m. - 12:30 p.m.

12:30 p.m.

Title: An Artificial Intelligence (AI) Case Study and Implementation

Presenters: Mr. Zack Wintrob, Emmes



Zack Wintrob is a Biostatistician Manager and PK/PD subject matter expert at Emmes where he brings over a decade of clinical research experience. He has been with Emmes since 2022 where he has focused on infectious disease and antibiotic resistance as well as PK/PD studies. He earned his MA in Biostatistics and MS in Pharmaceutical Science from State University of New York at Buffalo. Prior to joining Emmes, Zack worked at other clinical research organizations and hospitals with a primary focus on emerging modalities and therapy optimization in hematology and oncology.

Abstract:

Artificial intelligence (AI) is being adopted into statistical programming and biostatistics workflows, offering new opportunities to improve efficiency while introducing important governance and quality considerations. This presentation will examine the practical integration of AI within specific statistical workflows, such as analysis dataset specifications and drafting of clinical study report content using table, figure, and listing (TFL)-to-text workflows.

Drawing on real-world implementation experience, the session will discuss where AI can reduce repetitive effort, improve consistency, and accelerate document development, while also highlighting the limitations of current large language models in the context of regulated statistical deliverables. Reinforcing the indispensable role of experienced biostatisticians in not only providing scientific judgment, ensuring statistical validity, and verifying regulatory compliance, but also as essential contributors to the development of AI tools to ensure that AI based technologies enhance workflows rather than disrupt them.

Attendees will gain practical insights into the benefits, risks, and governance considerations associated with deploying AI in clinical development workflows, including traceability, reproducibility, and the necessity for human oversight as well as the advantages of involving biostatisticians in AI development. The presentation will conclude with a framework for responsibly integrating AI into biostatistics workflows that enhances productivity without compromising the scientific rigor and quality expected for regulatory submissions.

1:30 p.m.

Title: A Comparative Evaluation of Bayesian and Frequentist Mediation Analysis in Clinical Trials

Presenter: Dr. Ibrahim Turkoz, Johnson & Johnson



Dr. Turkoz is a Distinguished Scientist in the Statistics and Decision Science group at Johnson & Johnson. Before joining J&J, he worked with several pharmaceutical consulting companies. He has more than 25 years of clinical research experience in the pharmaceutical industry, with involvement across all phases of clinical drug development. His current work focuses on causal inference, comparative effectiveness, and cost-effectiveness research using innovative methods, including pragmatic trials. Dr. Turkoz is a founding member of the International Society for CNS Clinical Trials and Methodology (ISCTM). He has contributed to the development of several rating scales used to measure patient functioning and clinical symptomatology in CNS disorders. In addition to CNS, Dr. Turkoz has supported successful regulatory submissions in pain, immunology, oncology, medical devices, and registries. Dr. Turkoz has authored or coauthored more than 80 articles in peer-reviewed journals.

Abstract:

In clinical trials, mediation analysis elucidates intermediate variables or processes that mediate the relationship between an intervention and health outcomes. This multifaceted approach enhances understanding not only whether an intervention works but how it works, informing researchers, clinicians, and policymakers. Mediation analysis can, for example, examine how changes in biomarker or patient behavior mediate the relationship between drug administration and observed clinical improvements.

When multiple mediators are correlated, standard approaches face challenges: frequentist estimators may underestimate uncertainty, Bayesian estimators can be sensitive to prior specification, and the direct-effect parameter can become weakly identified as mediator correlation increases. Regulators and investigators need guidance on the tradeoffs between bias, variance, and interval calibration when assessing direct and indirect treatment effects in such settings.

We conducted extensive simulations motivated by a depression clinical trial with two mediators representing change in overall depressive symptoms and change in anhedonia, and a functional outcome.

Three estimation strategies were compared: (i) frequentist ordinary least squares (OLS), (ii) Empirical Bayes (EB) with data-derived priors, and (iii) hierarchical shrinkage Bayesian estimation. Simulations varied mediator correlation regimes (rho = 0.3 and rho = 0.7) and used realistic covariate structures and moderate sample sizes. Performance metrics included bias, interval width, interval coverage (nominal 90%), and estimator stability across repeated datasets.

A clear trade-off emerged between bias and uncertainty quantification. OLS estimators exhibited low bias but tended to underestimate variability, especially at higher mediator correlation (rho = 0.7). EB estimators improved coverage but often produced wider intervals and were sensitive to prior centering, sometimes over-regularizing the direct-effect parameter. Hierarchical shrinkage priors provided the most balanced performance: stable estimates with interval coverage closer to nominal levels across correlation regimes. Mediator-related parameters were generally robust across methods, whereas the direct-effect parameter was weakly identified and more sensitive to prior choice as correlation increased.

Presenting both frequentist and Bayesian perspectives offers complementary evidence in regulatory contexts. Hierarchical shrinkage priors are recommended when correlated mediators and moderate sample sizes make interval calibration a priority, while analysts should exercise caution with prior centering in Empirical Bayes approaches.

Break 2:30 p.m. - 3:00 p.m.

Poster Session 3:00 p.m. - 4:00 p.m.

Poster Title: MAPLE: Mapper Based Localized Prediction with Data Driven Cover Selection for High dimensional Data

Authors: Md Moinul Ahsan, Dr. Nitai Mukhopadhyay, Dr. Priyam Das

Virginia Commonwealth University, Department of Biostatistics

Abstract:

High dimensional biomedical data often exhibit nonlinear, heterogeneous, and manifold driven structures that challenge global parametric and tree-based models. We propose MAPLE (mapper-based Adaptive Prediction via Local Estimation), a localized prediction framework grounded in topological data analysis. The method is formulated as a nonparametric estimator of conditional class probabilities that adapts to the intrinsic geometry of the predictor space. Neighborhoods are defined through connectivity in a data-adaptive Mapper graph, enabling localized averaging within graph induced regions that capture complex structures such as branching and multi-scale heterogeneity. We introduce a statistically principled, data driven procedure for cover selection based on a bias–variance trade off, yielding optimal asymptotic scaling for interval widths and overlaps. The framework accommodates binary, nominal, and ordinal outcomes and incorporates a permutation-based variable importance measure to quantify covariate contributions in prediction. We establish theoretical guarantees, including pointwise consistency and Bayes risk consistency under standard regularity conditions. Simulations show that MAPLE consistently outperforms or matches multinomial regression, ordinal regression, and random forest, with the largest gains observed under heterogeneous and high-noise settings. Applications to Parkinson's disease progression (PPMI) and glioma classification (TCGA RNA sequencing) demonstrate strong predictive accuracy and interpretable, topology-aware summaries of underlying data structure.

Poster Title: A Bayesian Likely Responder Approach for the Analysis of Randomized Controlled Trials

Authors: Annan Deng, Dr. Hyung G. Park, Dr. Thaddeus Tarpey, Dr. Carole Siegel

New York University, Department of Population Health and Department of Psychiatry

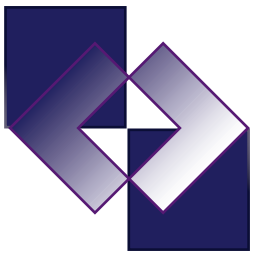
Abstract:

Introduction/Goals: Randomized controlled trials are typically designed and analyzed to estimate an average treatment effect, which can mask clinically important heterogeneity in how individual patients respond to treatment. The Likely Responder (LR) framework addresses this by using baseline covariates to predict a prognostic balancing score and restricting inference on treatment effects to patients predicted to be likely responders. Existing LR and related data-driven subgroup analyses, however, typically treat the first-stage subgroup assignment as fixed when conducting second-stage inference, understating the true uncertainty in the estimated treatment effect. This work develops a Bayesian two-stage approach that integrates subgroup identification with subgroup-specific treatment effect inference while propagating first-stage estimation uncertainty into the second stage.

Methods: We model the prognostic balancing score within a Bayesian regression framework and draw subgroup assignments from their posterior distribution rather than fixing them at a point estimate. Subgroup-specific treatment effects are then estimated conditional on these posterior draws, yielding credible intervals that reflect uncertainty in both subgroup membership and treatment effect. We compare this corrected approach with a naive method that ignores subgroup-estimation uncertainty through simulation studies and an application to the COMPILE trial, a pooled international platform trial of convalescent plasma for hospitalized COVID-19 patients (N = 2,341; N = 2,287 complete cases), stratifying patients into likely, moderate, and unlikely responder subgroups based on estimated prognostic balancing scores.

Results: Simulations show that the naive approach produces confidence intervals that are too narrow, while the proposed Bayesian two-stage method achieves appropriate coverage. In the COMPILE application, for the primary outcome of ventilation or worse by day 14, estimated odds ratios were 0.72 among likely responders, 0.89 among moderate responders, and 1.00 among unlikely responders, indicating a clinically meaningful gradient in treatment benefit across subgroups. Credible intervals from the corrected method were consistently, and appropriately, wider than those from the naive approach.

Conclusions: By explicitly propagating subgroup-estimation uncertainty into treatment effect inference, this framework provides a more honest and better-calibrated account of heterogeneous treatment response than conventional two-stage subgroup analyses. These results are directly relevant to precision-medicine-oriented trial designs and to FDA guidance on enrichment strategies, offering a principled way to characterize which patients are most likely to benefit from a new therapy without overstating the certainty of that classification.



Poster Title: Beyond Gaussian Assumptions: Robust Matrix-variate Regression for Correlated Periodontal Outcomes

Authors: Samuel Soon, Dr. Qingyang Liu, Dr. Victor H. Lachos, and Dr. Dipankar Bandyopadhyay
Virginia Commonwealth University, Department of Biostatistics

Abstract:

Clinical attachment loss (CAL) and probing pocket depth (PPD), the two most important endpoints assessing periodontal disease (PD) status and progression, are collected at multiple tooth sites within subjects. The two measurements are known to be correlated and exhibit non-Gaussian behavior characterized by skewness and heavy tails. Despite this, analysis of PD data is often performed using Gaussian random effects without accounting for response-wise covariance. Although parametric regression models exist to handle the apparent non-Gaussianity of the vectorized multivariate responses, we consider the computationally elegant alternative of matrix-variate non-Gaussian (MVNG) regression, which generalizes a multivariate density to matrix-valued random variables and accommodates skewness through additional parameters. We explore an MVNG linear regression framework for the variance-gamma and normal-inverse Gaussian distributions, which reduces the need for numerical approximations and allows for accurate representations of skewness, kurtosis, and covariance parameters. We circumvent the computational inefficiencies of classical maximum likelihood estimation through an expectation-conditional maximization (ECM) algorithm, and provide model-specific case-deletion influence diagnostics to identify influential subjects and illustrate the robustness of MVNG models. We present simulation studies to study the finite-sample behavior of our model. Furthermore, application to a dataset generated from a clinical PD study reveals the advantages of our proposal, in light of existing alternatives.

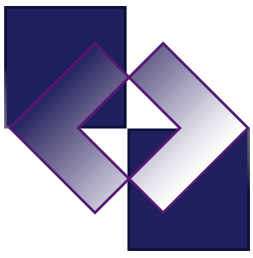


Poster Title: A Balaam-Modified Approach to LARGO-Like Parkinson's Trials: Efficient Estimation of Rasagiline Effects Under Motor Fluctuations

Authors: Purbasha Biswas, Dr. Jing Kersey, Dr. Karl E. Peace
Georgia Southern University, Department of Biostatistics, Epidemiology, and Environmental Health Sciences

Abstract:

The LARGO trial established rasagiline (1 mg/day) as an effective adjunct to levodopa in Parkinson's disease (PD) patients with motor fluctuations, demonstrating clinically meaningful reductions in daily "off-time" (poor or absent motor function) and improvements in Unified Parkinson's Disease Rating Scale (UPDRS)/Clinical Global Improvement (CGI) endpoints compared with placebo. Building on this evidence base, we propose a design strategy that re-uses LARGO-like assumptions while improving statistical efficiency for future PD evaluations of rasagiline versus placebo. Methodologically, we leverage key results emphasizing the Balaam 4-sequence, 2-period schema (AA, AB, BA, BB). Under the standard crossover model with direct and carryover effects, the Balaam direct-effect contrast is constructed so that period effects and carryover effects cancel algebraically, yielding an estimator of the differential direct treatment effect that is not conditional on carryover, unlike the classic Grizzle 2×2 estimator, where the direct effect is biased unless differential carryover is absent. The ASA discussion (Peace) further highlights that incomplete "responders-only crossover" structures (e.g., White-type designs) can reintroduce carryover contamination and reduce efficiency due to response-dependent second-period sample sizes. Using LARGO-motivated parameters for change in daily off-time (rasagiline ≈ −1.2 hours; placebo ≈ −1.0 hours; SD ≈ 2; NI margin example −0.5), we outline a simulation framework comparing a parallel non-inferiority analysis to a Balaam-style crossover to exploit within-subject contrasts while respecting ethical continuity of benefit. These design principles motivate a PD trial blueprint that maintains LARGO's clinical endpoint while reducing sensitivity to carryover and improving inferential efficiency for rasagiline versus placebo. The anticipated contribution is a PD-focused trial strategy that strengthens the interpretability of treatment comparisons by design, separating direct effects from carryover sensitivity while transparently characterizing efficiency trade-offs for realistic PD heterogeneity.



Poster Title: Improving Statistical Inference from Error-Prone Digital Health Data: Applications to Wearable Sensors and Health Outcome Classification

Authors:Andi Mai, Dr. Lan Xue, Dr. Dr. Roger S. Zoh, Dr. Carmen D. Tekwe
Indiana University, Department of Epidemiology and Biostatistics

Abstract:

Introduction/Goals

Wearable sensors and continuous glucose monitoring (CGM) devices are increasingly used in health research, clinical studies, and patient monitoring. However, these data are often subject to measurement error and outcome misclassification, which can lead to biased estimation and unreliable scientific conclusions. The goal of this dissertation is to develop statistical methods that improve inference from error-prone digital health data.

Methods

The first component develops a functional mixed-effects clustering framework that accounts for measurement error in high-frequency wearable sensor data. The proposed method improves recovery of underlying activity patterns and supports more reliable downstream analyses. The second component develops methods for analyzing misclassified health outcomes, motivated by continuous glucose monitoring applications and hypoglycemia classification. The proposed approaches account for imperfect outcome ascertainment and evaluate the impact of misclassification on estimation and prediction.

Results

Simulation studies demonstrate that accounting for measurement error substantially improves clustering performance and downstream inference compared with naïve approaches. In the misclassification setting, the proposed methods reduce bias and improve estimation accuracy under realistic levels of outcome misclassification. Applications to wearable sensor and CGM-related data illustrate the practical utility of the methods.

Conclusions

This dissertation contributes statistical methodology for improving the reliability of digital health data analyses. The proposed methods address challenges that commonly arise in wearable sensors, CGM devices, and other emerging digital measurement technologies.

Poster Title: Evaluating the Benefit of Biomarker Based Treatment With Poisson Outcomes and Hormetic Dose Response

Authors: Seth Rozelle

Augusta University

Abstract:

In clinical settings, patients are sometimes given a particular treatment based on their measurement of a known biomarker. The goal of biomarker guided therapy is to reduce the occurrence of negative outcomes in a population. The process of how to evaluate this decrease, or benefit, has been explored in the context of binary and survival data. In this research, evaluation is extended to Poisson, or count response, outcomes. This is done in both the traditional one crossing point case, and to the unexplored two crossing point case, sometimes called Hormetic Dose Response. A sample size method for these Poisson response cases is developed and subsequently evaluated with Monte Carlo estimates. A sample size calculator has been created in a R Shiny app for the purpose of designing an appropriately sized RCT or simulation to evaluate the benefit of biomarker guided treatment selection for both the one and two crossing point cases.

Poster Title: Association Between FOLR1 Expression and Chemotherapy Response in Patients with Advanced Ovarian Cancer

Authors: Jeremiah Tella, Dr. Michael D. Toboni, Dr. Brett W. Wright, Jaelynn A. Lawrence, Anna Wilbanks, Dr. Emily O'Brien, Dr. Carson Edwards, Nneka R. Ijeli, Purva Pawar, Ananya S. Venkatachalam, Dr. Rebecca C. Arend, Dr. Charity J.Morgan
University of Alabama at Birmingham

Abstract:

Introduction: In the treatment of advanced ovarian cancer, interval debulking surgery (IDS) is commonly performed after neoadjuvant chemotherapy. Chemotherapy Response Score (CRS), considered a standardized grading system, provides a histopathologic measure of treatment response, while CA-125 has been commonly used as the primary ovarian cancer marker. Folate receptor alpha (FOLR1) is expressed in ovarian cancer and is an important therapeutic target. However, data on the relationship between baseline FOLR1 expression and response to neoadjuvant chemotherapy are limited.

Aim: To determine the association between baseline FOLR1 expression, CA-125, and pathologic response to neoadjuvant chemotherapy in patients with advanced ovarian cancer.

Methods: We analyzed patients with advanced ovarian, fallopian tube, or primary peritoneal cancer enrolled in the standard-of-care arm of an ongoing phase II study of carboplatin and mirvetuximab soravtansine in first-line treatment of patients receiving neoadjuvant chemotherapy. These patients were ineligible for the study treatment because they had low FOLR1. The cohort included 29 participants, with clinical, biomarker, and response data. FOLR1 and CA-125 were measured at baseline and at the time of IDS. Pathologic response was assessed using CRS. We also explored whether race or age were significantly associated with biomarker and response outcomes.

Results: Participants had a median age of 67 years (IQR, 55–74); 70% were White and 85% were non-Hispanic. Median baseline FOLR1 was 27% (IQR, 4–45; n=25), and median baseline CA-125 was 501.65 (IQR, 219.10–1,306.20; n=24). Among 14 participants with repeated screening and IDS CA-125 measurements, 13 (93%) had a decrease, with a median reduction of 83.81% (IQR, 13.42–91.12). Among the 15 participants for whom CRS was available, 2 (13%) had CRS1, 7 (47%) had CRS2, and 6 (40%) had CRS3. Baseline FOLR1 was inversely associated with CRS (ρ=-0.698, p=0.0055), with a median FOLR1 of 44.5% and 35% for CRS1 and CRS2, respectively. The median FOLR1 was 1% for CRS3 (exact Kruskal-Wallis p=0.0199). Baseline CA-125 did not differ significantly across CRS groups (p=0.1665), and CA-125 reduction was not associated with CRS (ρ=-0.235, p=0.576).

Conclusion: Lower baseline FOLR1 expression was associated with greater pathologic response to neoadjuvant chemotherapy. Baseline CA-125 and changes in CA-125 were not associated with CRS. These preliminary findings suggest that baseline FOLR1 may be a useful biomarker of pathologic response and warrant confirmation in a larger cohort.

Poster Title: Optimal Local Exchangeability Designs for Bayesian Basket Trials

Authors: Kyle Wu, Dr. Pavlos Msaouel, Dr. Weng Kee Kong
University of California Los Angeles, Department of Biostatistics
The University of Texas, MD Anderson Cancer Center

Abstract:

Purpose: Basket clinical trials are increasingly used in oncology due to their promising potential to accelerate drug development for rare tumor types. Innovative statistical designs can reduce accrual requirements without compromising the ability to detect responsive subgroups or assuming complete exchangeability between tumor types. To promote greater use of such designs in practice, we propose a more flexible statistical framework for basket trials that can accommodate different clinical intuitions.

Methods: The local multisource exchangeability model (LMEM) was proposed by Liu et al.1 Our proposed approach (LMEM2) increases their flexibility by using pairwise similarity metrics to calibrate the degree of information borrowing among baskets. Using simulated basket trials under various scenarios considered in Jing et al.,2 we compare optimal sample size designs generated by our framework, the LMEM framework, the multisource exchangeability model (MEM) framework, the prune-pool framework, and traditional pooled or independent analysis. Operating characteristics are also compared within a two-stage design with pre-specified sample allocations per stage to consider performance when external factors may determine accrual requirements.

Results: In the optimal sample size comparison, LMEM2 reduced Type I error rates compared to LMEM (while maintaining comparable power) and required fewer patients than the prune-pool and MEM designs. Compared to independent Simon's two-stage designs, LMEM2 can reduce the required sample size by more than half. When the sample size in each stage is pre-specified, LMEM2 demonstrated superior power and better Type I error control over the pooled design, or traded power to minimize Type I error rates, comparable to the MEM method.

Conclusion: LMEM2 is a flexible, computationally efficient framework that can simultaneously reduce accrual requirements and detect heterogeneous treatment effects precisely.

2026 BASS Scholars

- Moinul Ahsan - Virginia Commonwealth University
- Purbasha Biswas - Georgia Southern University
- Samuel Soon - Virginia Commonwealth University

BASS Travel Stipend Award Winners:

- Andi Mai - Indiana University
- Annan Deng - New York University
- Seth Rozelle - Augusta University

Wednesday, November 4

8:30 a.m

Title: Missing Data and Sensitivity Analyses

Presenter: Dr. Abel Eshete, FDA

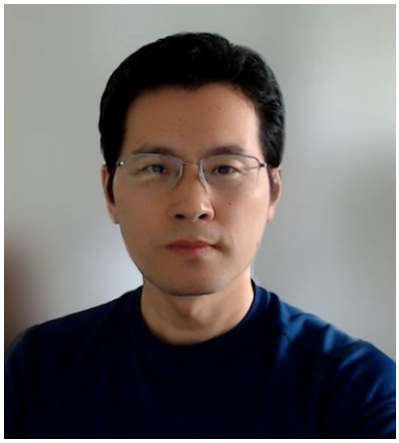


Dr. Abel Eshete is a Lead Mathematical Statistician at the FDA with over two decades of experience in biostatistics and statistical methodology. He earned his Ph.D. in Biostatistics from Hasselt University in Belgium. Prior to joining the FDA, he served as a Research Associate at Harvard University's School of Public Health, where he contributed to the statistical design and analysis for large-scale NIH-funded HIV studies, and as a Researcher at Hasselt University, where he developed statistical methods for biomarker validation and provided consultancy to major pharmaceutical companies. He has also taught as an adjunct professor at George Washington University and Addis Ababa University in Ethiopia. His research interests include surrogate marker validation, longitudinal data analysis, clinical trials, estimand, and missing data.

9:30 a.m.

Title: Statistical Considerations for Surrogate Endpoint Evaluation

Presenter: Dr. Feng Li, FDA



Dr. Feng Li is a Lead Mathematical Statistician in the Division of Biometrics II, Office of Biostatistics, Office of Translational Science, Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). Dr. Li joined FDA in 2009. He is currently the Chair of the Surrogate Endpoints Working Group within the Office of Biostatistics.

Break - 10:30 a.m.

11:00 a.m.

Panel Discussion: *The Impact of Artificial Intelligence in Biopharmaceutical Statistics*

Panelists:

Dr. Robert Perera
Dr. Vipin Arora
Dr. CV Damaraju
Dr. Melvin Munsaka

