BBSW2022 PROGRAM BOOK

Nov 3-4, Crowne Plaza Hotel, Foster City, CA 94404

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Agenda

Day 1: November 3, 2022 (Thursday)

Time	Speaker	Topic
7:30-8:30 AM	**Registration, Breakfast and Networking**	
8:30-9:00 AM	**Welcome and Opening Remarks**	
9:00-10:00 AM	**Keynote Speech: Lost in Translation and How to Fix it** L.J. Wei (Professor, Harvard University)	
10:00-10:30 AM	**Break**	
10:30 AM-12:00 PM	**Technical Session #1: Biomarker and Imaging Analyses** Chair: Tuan Nguyen (Alector)	
10:30-11:00 AM	Shivaani Kummar (OHSU)	Biomarker Driven Drug Development – Opportunities and Challenges
11:00-11:30 AM	Xiao Li (Genentech)	When Spatial Statistics Meets Digital Pathology – Integrating Spatial Statistics Frameworks in Histopathology Image Analysis
11:30 AM-12:00 PM	Meijuan Li (Eisai)	Driver Mutation Variant Allele Frequency in Circulating Tumor DNA and Association with Clinical Outcome in NSCLC Patients with EGFR or KRAS Mutated Tumors
12:00-1:00 PM	**Lunch and Networking**	
1:00-2:30 PM	**Career Session: Effectively Managing Your Career** Moderator: Tara Maddala (Pandora Bio)	
	Fan Zhang (AstraZeneca)	Change is Constant: Seeking it, Embracing it, Navigating it
	Ning Leng (Genentech)	Finding Your Career Lattice
2:30-3:30 PM	**Break and Poster**	
3:30-5:30 PM	**Technical Session #2: Real World Evidence** Chair: Haijun Ma (Exelixis)	
3:30-4:00 PM	Mark van der Laan (UC Berkeley)	Targeted Learning and Causal Inference for Integrating Real World Evidence into the Drug Approval Process and Safety Analysis
4:00-4:30 PM	Dave Miller (Unlearn)	Prognostic Covariate Adjustment: A Novel Method to Reduce Trial Sample Sizes While Controlling Type I Error
4:30-5:00 PM	Hui Wang (VA Palo Alto)	Pragmatic Use of the Electronic Medical Records in Clinical Research from the Veterans Affairs Healthcare – A Closed Healthcare System
5:00-5:30 PM	Brian Hobbs (UT Austin)	Does Real-World Evidence Have a Role in Precision Oncology?
5:30-6:30 PM	**Raffle, Mixer, and Live Performance by "Average Lovers"**	
6:30-8:30 PM	**Dinner Panel – Leadership with the C-Suite** Moderator: Tara Maddala (CEO, Pandora Bio)	

	Dave Miller (Chief Scientific Officer, Unlearn), Stacey Lindborg (Chief Development Officer, Brainstorm Cell Therapeutics), Yali Li (Chief Data Officer, Function Oncology)

Day 2: November 4, 2022 (Friday)

Time	Speaker	Topic
8:00-8:45 AM	**Registration, Breakfast and Networking**	
8:45-9:00 AM	**Day 2 Opening Remarks**	
9:00-10:00 AM	**Keynote Speech: Imagine Where Your Unique Potential and the Discipline of Analytics Could Take You: Ready to Catch Your Wave?** Stacy Lindborg (EVP, Brainstorm Cell Therapeutics)	
10:00-10:30 AM	**Break**	
10:30 AM-12:00 PM	**Technical Session #3: Innovative Trial Design and Methodology** Chair: Godwin Yung (Genentech)	
10:30-11:00 AM	Cong Chen (Merck)	Adaptive 2-in-1 Phase 2/3 Designs – Time to Hedge and Have Fun
11:00-11:30 AM	Heng Xu (Nektar)	Adaptive Endpoints Selection with Application in Rare Disease
11:30 AM-12:00 PM	Lei Nie (FDA)	Bayesian Dynamic Borrowing Methods in Clinical Trials
12:00-1:30 PM	**Lunch, Networking, and Poster**	
1:30-1:45 PM	**Poster Awards**	
1:45-3:45 PM	**Technical Session #4: Artificial Intelligence and Machine Learning** Chair: Rita Lopatin (Grail)	
1:45-2:15 PM	Earl Hubbell (Grail)	Learning to Detect Cancer in Epigenetic Space
2:15-2:45 PM	Michel Friesenhahn (Genentech)	The Unreasonable Effectiveness of Additive Models for Tabular Data
2:45-3:15 PM	Mark Chang (AGInception)	Humanized and Medical AI
3:15-3:45 PM	Haoda Fu (Eli Lilly)	25 Misuse Data Examples in the Era of AI and Machine Learning
3:45-4:00 PM	**Closing Remarks**	
4:00-5:30 PM	**Raffle and Happy Hour**	

Abbreviation: OHSU = Oregon Health & Science University. UC = University of California. VA = Veterans Affairs. UT = University of Texas. FDA = U.S. Food and Drug Administration.

Vendor Exhibits all day for both days.

Keynote Talks

L.J. Wei (Professor, Harvard University)

Topic: Lost in Translation and How to Fix it

Abstract: One of the main goals of conducting a clinical, comparative study is to obtain robust, clinically interpretable treatment effect estimates with respect to harm-benefit perspectives at the patient's level via efficient and reliable quantitative procedures. Like translational medicine, to accomplish this goal, it is important to know how to effectively translate new developments in basic data science research into clinical practice. Unfortunately, some commonly used statistical procedures are not translational. That is, results of the analysis may be misinterpreted or difficult to comprehend. A notorious example is use of the p-value for clinical decision making, which is not an appropriate quantifier for assessing the clinical utility of a new therapy or strategy. In this talk, we will discuss several translational problems and present possible remedies.

Bio: Professor L.J. Wei' is professor at the Harvard University School of Public Health. He received his PhD degree from the University of Wisconsin Madison in 1975. His research is in the area of developing statistical methods for the design and analysis of clinical trials. In 1977-78, he first introduced the "urn design" for two-arm sequential clinical studies. In 1979, he proposed a response adaptive design, a randomized version of Marvin Zelen's play the winner rule. In 1982, Wei and his colleagues presented a rather flexible monitoring scheme, which has become a classical reference for the literature in interim analysis for clinical trials.

Dr. Wei has developed numerous methods for analyzing data with multiple outcomes or repeated measurements obtained from study subjects. In particular, his "multivariate Cox procedures" to handle multiple event times have become very popular. He and his colleagues are also responsible for developing alternative models to the Cox proportional hazards model for analyzing survival observations. One example is the analysis methods based on restricted mean survival time.

More recently, Dr. Wei has devoted his effort to developing translational statistics, linking the statistical analysis with clinical interpretability together. Professor Wei has also won numerous awards including Spiegelman Award for Outstanding Statistical Research in Public Health, Mosterller Statistician of the Year, Wilks Memorial Award and more. Dr. Wei is elected fellow of ASA and IMS.

Stacy Lindborg (EVP, Brainstorm Cell Therapeutics)

Topic: Imagine Where Your Unique Potential and the Discipline of Analytics could Take You: Ready to Catch Your Wave?

Abstract: Healthcare jobs topped the list of the highest-paying occupations, and the sector's future is very bright. According to the U.S. Bureau of Labor Statistics, employment in healthcare occupations is projected to grow 16% from 2020 to 2030—adding about 2.6 million new jobs. According to multiple surveys, the loosely defined role of quantitative analyst comes in around 15 out of the top 20 jobs in health care and around 16 of the top 100 jobs spanning all job sectors, just below CEO (10) and dentists (14, 15). It's safe to say jobs that leverage sophisticated analytics offer great earning potential and the platform to contribute in powerful ways to society. How does your career thus far reflect these numbers? Are you already soaring or might you be seeking to catch your wave?

In our time together we'll reflect on qualities that position you well for advancement across your career, including to C-Suite roles. We'll also consider qualities that have the potential to bias your candidacy for broader leadership roles before turning to some concepts that have provided a firm foundation across my career as a leader.

Bio: Stacy Lindborg, PhD, is a globally recognized leader skilled at leading teams and creating strategic alliances with cross-functional and external partners to drive innovation within drug development. Currently Executive Vice President & Chief Development Officer at Brainstorm Cell Therapeutics. She is a member of Brainstorm's Executive Leadership Team and is accountable for driving the creation of clinical development strategies from first-in-human (FIH) through registration – including framing innovative development paths and identifying the best opportunities, ensuring evidence is generated as required by key constituents (scientific community, regulatory, commercial/payer) and resourcing them adequately so they can be realized.

Most recently, she was Vice President & Global Analytics and Data Sciences Head at Biogen – responsible for evidence generation as well as statistical and epidemiological support for RD and marketed products. Serving on all R&D governance bodies, she was active in guiding the firm's long-term vision for growth and stimulating innovative platforms increasing Biogen's productivity. Previous positions include: Head of R&D strategy at Lilly, responsible for characterizing the productivity of the portfolio, driving key R&D strategy projects including the R&D component of annual corporate Long-Range Plan; and General Product Management Leadership (responsible for R&D, Commercial and Manufacturing plans). Stacy is passionate about analytics and loves to operate at the intersection of innovative Statistics and R&D strategy. She has been a student of leadership styles across her career.

Dr. Lindborg earned a Ph.D. in Statistics from Baylor University and has been elected as Fellow of the American Statistical Association. She is currently serving on the Scientific Advisory Board for Cytel, and as an independent board member for MIT Catalyst. She is on the Board of Directors for Celsion Corporation and the Massachusetts Down Syndrome Congress.

BBSW2022 President-Elect

Whedy Wang, PhD (Alector)

Bio: Whedy has over 25 years of experience in the biopharmaceutical industry, including directing biometrics efforts in more than ten NDA and sNDA submissions, three advisory committee meetings and multiple EX-US submissions, all leading to successful approvals. Whedy is currently VP of Biometrics & Digital Science at Alector, a biotech company that is on a mission to slow the progression of neurodegenerative diseases and to one day prevent their occurrence. Prior to joining Alector, she held several senior positions including Vice President of Biometrics and Data Science at Theravance Biopharma, VP of Biometrics at Gilead Sciences Palo Alto (formerly CV Therapeutics), Senior Vice President of Bioinformatics at Orexigen, and Executive Director of Biometrics at Affymax.

At her current as well as previous job postings, Whedy provided strategic input and biometrics oversight to U.S. and EU development and commercial efforts. As a member of the executive team at CV Therapeutics and Orexigen, Whedy contributed to the development of corporate strategy and led life cycle management planning. Additionally, Whedy was the global project leader for Lexiscan® and Ranexa® where her contributions included presenting development rationale and product life cycle management plan to the Board of Directors, and potential EU and Asia partners. Whedy also played a key role in business development discussions that led to successful collaborations such as royalty financing of $185 million for Lexiscan® Injection with TPG-Axon Capital, and license agreement for Ranexa® in 68 countries including EU with the Menarini Group. Whedy holds a M.P.H. in Epidemiology, and a Ph.D. in Biostatistics, both from the University of Michigan, Ann Arbor.

Whedy thrives to be a leader who inspires and empowers others to be their best!! She lives in Palo Alto with her husband and 3 toddler girls. Her favorite daily routine includes reading bedtime stories and playing Animal Crossing with her daughters.

Invited Talks

Mark Chang, PhD (AGInception)

Humanized and Medical AI

Abstract: Humanized or Human-level AI thinks and behaves like humans, while Medical AI involves the applications of AI technology in drug development and healthcare to help improve health outcomes and patient experiences.

Discuss how to deal with the fundamental issues such as self-awareness, consciousness, emotion, creativity, and language-understanding, the principles that serve as the backbone of AI methods, and main approaches to humanized and medical AI.

Discuss why we need the small-data instead of big-data AI in precision medicine and how to use the similarity principle to develop machine-learning and apply it to clinical trials for precision medicine with an example.

Bio: Dr. Mark Chang is the founder of AGInception for Artificial General Intelligence Research. Previously, Dr. Chang served as Sr. Vice President, Strategic Statistical Consulting at Veristat, Vice President of Biometrics at AMAG Pharmaceuticals and director and scientific fellow at Millennium/Takeda Pharmaceuticals. Dr. Chang has over 25 years of experience as a statistician and extensive experience in NDA and working regulatory agencies. Dr. Chang is a fellow of the American Statistical Association and an adjunct professor of Biostatistics at Boston University. He is a co-founder of the International Society for Biopharmaceutical Statistics. He served as co-chair of the Biotechnology Industry Organization (BIO) Adaptive Design Working Group, and on ASA Biopharmaceutical Section, Multiregional Clinical Trial (MRCT) Expert Group, PhRMA Adaptive Design and Biomarker Working Groups. Dr. Chang served on the editorial boards for Statistical Journals. He has published 11 books, including Artificial Intelligence for Drug Development, Precision Medicine, and Healthcare, Innovative Strategies, Statistical Solutions and Simulations for Modern Clinical Trials, Monte Carlo Simulation for the Pharmaceutical Industry, Adaptive Design Theory and Implementation Using SAS and R, Adaptive Design Method for Clinical Trials, Modern Issues and Methods in Biostatistics, Paradoxes in Scientific Inference, and Principles of Scientific Methods.

Cong Chen, PhD (Merck)

Adaptive 2-in-1 Phase 2/3 Designs – Time to Hedge and Have Fun

Abstract: A common practice in contemporary oncology drug development is to detect efficacy signal in a multi-cohort single-arm Phase 1 trial after dose-finding, and follow-up those cohorts deemed promising directly with a randomized-controlled Phase 3 trial. Single-arm Phase 1 data is fraught with caveats. Despite its critical role in conventional drug development, randomized-controlled Phase 2 trials have been routinely skipped, missing out an opportunity to better characterize the safety profile, establish a solid clinical proof-of-concept and optimize Phase 3 trial design. A mid-trial futility analysis is routinely implemented in a Phase 3 trial to mitigate the risk, but in fear of terminating it wrongly it is often conducted too late (or futility bar too low). This aggressive approach of a straight Phase 3 design is not sustainable and has already led to multiple

high-profile setbacks in the immune-oncology field (e.g., epacadostat in melanoma, tiragolumab in small-cell lung cancer) with many more not making it to headline news.

Unless Phase 1 data is exceptional, a more prudent approach is to conduct an adaptive Phase 2/3 trial. By mimicking the sequential Phase 2/3 design, the decision to Phase 3 is based on smaller sample size than in a mid-trial Phase 3 futility analysis, making it possible to pause or stop the development program earlier to reduce unnecessary patient exposure in case of underwhelming treatment effect. While adaptive Phase 2/3 design intends to reduce the risk of a false Go-decision of a straight Phase 3 design, it inevitably increases the risk of a false No-Go decision. A false No-Go decision may be made when the Go-No Go bar is set too high and continued follow-up shows greater treatment effect. Unbeknown to biostatisticians until more recently, without inflating the overall Type I error, Phase 2 data can be not only included in Phase 3 analysis but also legitimately declared positive after a false No-Go decision to Phase 3 (aka 2-in-1 design). Unlike futility stopping in a Phase 3 trial, a No-Go decision to Phase 3 in an adaptive Phase 2/3 design does not automatically mean a failed study. With the statistical rigor preserved, a positive outcome at end of the Phase 2 component of an adaptive Phase 2/3 trial has the same merit as a standalone Phase 2 trial. In case of a false No-Go decision, a positive outcome at end of the Phase 2 component, when adequately powered, may be used to justify for accelerated approval (AA), consistent with FDA's growing interest in relying more on randomized-controlled trials than single-arm trials for AA (e.g., upcoming Project FrontRunner).

In this talk, we will propose a prototype 2-in-1 design built upon a recent study and discuss the application of similar adaptive Phase 2/3 designs.

Bio: Dr. Cong Chen is Executive Director of Early Oncology Development Statistics at Merck & Co., Inc. He joined Merck in 1999 after graduating from Iowa State University with a Ph.D. in Statistics. He also holds a MS degree in Mathematics from Indiana University at Bloomington and a BS degree in Probability and Statistics from Peking University, PR China.

As head of the group, he oversees the statistical support of oncology early clinical development and translational biomarker research at Merck. Prior to taking the role in March 2016, he led the statistical support for the development of pembrolizumab (KEYTRUDA), a paradigm changing anti-PD-1 immunotherapy, and played a pivotal role in accelerating its regulatory approvals.

Dr. Chen is a Fellow of American Statistical Association, an Associate Editor of Statistics in Biopharmaceutical Research, a member of Cancer Clinical Research Editorial Board and a co-leader of the DIA Small Population Work Stream. He has published over 100 papers and 10 book chapters on design and analysis of clinical trials, has given multiple short courses on design and analyses of clinical trials and was twice invited to give an oral presentation at the AACR Annual Meeting in recent years on design strategies for oncology drug development

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Michel Friesenhahn, PhD (Genentech)

The Unreasonable Effectiveness of Additive Models for Tabular Data

Abstract: Predictive machine learning has become widespread in medical research. One important consideration is to achieve good performance on relevant metrics. Since nature is complex, it's reasonable to expect that to get good performance the model must also be complex. However, it is also important in many cases to explain how the models work for insight and trust. Therefore, the goals of modeling have expanded from being primarily performance-based to looking for machine learning approaches that deliver both performance and model explanations.

To achieve these goals, we will start with challenging the following commonly held beliefs:

1. Trees have outstanding intrinsic interpretability
2. Blackbox models are inherently complex and poorly interpretable
3. Blackbox models often or usually outperform additive models
4. The SHapley Additive eXplanation (SHAP) method solves the model explanation problem

We will discuss our perspective on how to think about model explainability and why we think each of these beliefs is incorrect. As an alternative to providing post hoc explanations, we propose a new machine learning pipeline that takes as an input a blackbox model and extracts an intrinsically interpretable model that does not lose performance.

Bio: Dr. Michel Friesenhahn is a statistician who has over 20 years of industry experience, including the last 16 years at Roche. Prior to Roche he worked at Chiron and Bayer developing molecular diagnostics. At Roche he has broad experience ranging from non-clinical to clinical development. There he has supported programs in ophthalmology, Alzheimer's Disease, asthma, and pain. After a decade working directly in drug development, Michel moved to methodology development.

His current work focuses on exploratory and predictive modeling methods, performance metrics, and how to use predictive models to improve clinical development, particularly for ophthalmology and Alzheimer's Disease. Michel completed both his undergrad and graduate education at UC Berkeley.

Haoda Fu, PhD (Eli Lilly)

25 Misuse Data Examples in the Era of AI and Machine Learning

Abstract: Scientific progress depends on good research, and good research needs good statistical analytics. However statistical analysis is tricky to get right, even for the best and brightest of us. You would be surprised how many scientists are doing it wrong. We picked top mistakes from both internal and external examples. Those mistakes have caused significant waste and wrong decisions.

Bio: Dr. Haoda Fu is an Associate Vice President and an Enterprise Lead for Machine Learning, Artificial Intelligence, and Digital Connected Care at Eli Lilly and Company. Dr. Haoda Fu is a Fellow of ASA (American Statistical Association), and an IMS Fellow (Institute of Mathematical Statistics). He is also an adjunct professor of biostatistics department, University of North Carolina Chapel Hill and Indiana University school of medicine.

Dr. Fu received his Ph.D. in statistics from University of Wisconsin - Madison in 2007 and joined Lilly after that. Since he joined Lilly, he is very active in statistics methodology research. He has more than 90 publications in the areas, such as Bayesian adaptive design, survival analysis, recurrent event modeling, personalized medicine, indirect and mixed treatment comparison, joint modeling, Bayesian decision making, and rare events analysis. In recent years, his research area focuses on machine learning and artificial intelligence. His research has been published in various top journals including JASA, JRSS, Biometrika, Biometrics, ACM, IEEE, JAMA, Annals of Internal Medicine etc. He has been teaching topics of machine learning and AI in large industry conferences including teaching this topic in FDA workshop. He was board of directors for statistics organizations and program chairs, committee chairs such as ICSA, ENAR, and ASA Biopharm section.

Brian Hobbs, PhD (UT Austin)

Does Real-World Evidence Have a Role in Precision Oncology?

Abstract: The FDA instituted a program for Accelerated Approval in 1992, which allowed for approvals on the basis of surrogate endpoints for drugs treating serious conditions that filled an unmet medical need. The Food and Drug Administration Safety Innovations Act, passed in 2012, allows accelerated approvals for appropriate drugs and indications by evaluating the effects of drugs on surrogate markers. More recently, the FDA has established three additional pathways to speed the review process for emerging therapies. These changes prompted innovations in trial design with master protocol and seamless designs. Immune checkpoint inhibitors (ICIs) have yielded promising therapies for patients experiencing refractory cancers. Trials evaluating ICIs made extensive use of phase Ib, enrolling hundreds and even more than one thousand patients into dose expansion cohorts following dose-escalation spanning multiple tumor types. This represents a departure from conventional drug development strategies, for which dose expansion cohorts were used in roughly 25% of phase trials. Moreover, in 2021 two drugs, Atezolizumab and Durvalumab, were voluntarily withdrawn from accelerated approvals for PD-L1 inhibition in advanced or metastatic bladder cancer. This presentation considers the statistical implications of expansive, uncontrolled early phase trials and discusses the potential role for real-world evidence in this setting.

Bio: Dr. Brian Hobbs completed a doctoral degree in biostatistics at the University of Minnesota and then joined The University of Texas MD Anderson as an Assistant Professor of biostatistics. He was promoted to Associate in 2017, and then recruited to Cleveland Clinic to found a Section of Cancer Biostatistics. He joined The University of Texas Dell Medical School in August 2020 as a tenured Associate Professor. The Eastern North American Region of International Biometric Society selected his thesis paper for the John Van Ryzin Award in 2010. In 2016, Dr. Hobbs was selected by The University of Minnesota for the Emerging Leader Award, an honor bestowed on alumni on the basis of impactful contributions within 10 years of graduating from one of The School of Public Health's 20 programs. Recognized as an expert in clinical oncology research methodology, in 2017 Dr. Hobbs was invited to lead the publication of National Cancer Institute's Clinical Trials Design Task Force with the goal of providing national, consensus recommendations for first-in-human cancer drug trials that use seamless designs. In 2019, Dr. Hobbs was invited to describe recent advances and current issues with master protocol designs in the Journal of Clinical Oncology Precision Oncology. In 2020, he was invited to contribute to an article for Nature Reviews Clinical Oncology describing the current state of tumor agnostic trials. In 2021, Dr. Hobbs was invited to review the landscape of basket trials in the Journal of Clinical Oncology.

Earl Hubbell, PhD (Grail)

Learning to Detect Cancer in Epigenetic Space

Abstract: Blood-based multi-cancer early detection (MCED) tests that use sequencing coupled with machine-learning classifiers to detect and localize cancer signals in cell-free DNA (cfDNA) are now a reality. Signals seen in such tests may be limited to a single cancer or shared among broad groups of cancers. Leveraging a shared signal could allow detection of cancers that may not have been available for classifier training, which is ideal for population use. Using a targeted methylation-based assay and machine-learning classifier, we evaluated the generalizability of MCED test detection across cancers.

The MCED test assesses cfDNA methylation patterns to predict the presence of cancer and a cancer signal origin (CSO). We performed two types of computational experiments: the first retrained the classifier excluding all cancers of a given type from the training dataset; the second retrained the classifier only on cancers from one anatomic region (ie, lung or colorectal, separately). Because fewer samples were available for training in exclusion experiments, a comparable classifier using all cancers was created by downsampling to match learning curves for training. Performance was evaluated on samples from excluded cancers and compared to that when those cancers were included.

Despite exclusion from training, performance in solid cancers was preserved (median of 95% of the previous detection rate). Without a trained CSO category, detected cancers were classified into surrogate CSOs with similar biological characteristics. Detection rate in hematologic cancers dropped noticeably (by 38% for lymphoid neoplasm and 48% for plasma cell neoplasm) when excluded from training, suggesting differences in signal between hematologic cancers and others. Finally, training only on a single cancer resulted in a median of 94% of the previous detection rate in non-lung samples (lung training) or 86% in non-colorectal samples (colorectal training), indicating strong generalizability of cancer detection even when trained only on cancers from a single organ.

Epigenetic changes like DNA methylation have been called a “hallmark of cancer.” This research suggests that some epigenetic patterns found in methylation represent a shared cancer signal allowing detection of various solid cancers by MCED tests, even those not included in training. Consequently, MCED tests based on this shared signal may be able to detect multiple cancers, including very rare cancers, in a screening population.

Bio: Dr. Earl Hubbell is currently Distinguished Scientist, Bioinformatics (and Data Science) at GRAIL, LLC, a subsidiary of Illumina. GRAIL is a healthcare company dedicated to detecting more cancers early when they are more treatable and potentially curable. He has previously worked in sequencing development at Ion Torrent, and microarrays at Affymetrix. He received his BS in Mathematics from Caltech and a PhD in Applied Mathematics from USC.

Shivaani Kummar, PhD (OHSU)

Biomarker Driven Drug Development – Opportunities and Challenges

Abstract: Increasing understanding of cancer biology and development of novel therapeutic strategies has led to unprecedented increase in drug approvals and push to personalize treatment based on presence of biomarkers. Assessment of pharmacokinetics and pharmacodynamics, development and incorporation of biomarkers, and patient enrichment strategies have necessitated a change both in the preclinical package needed to support bringing an agent forward for clinical development and in the clinical trial design. I will discuss the opportunities and challenges in pursuing a biomarker driven approach to drug development.

Bio: Dr. Shivaani Kummar is DeArmond Endowed Chair of Cancer Research, Division Chief of Hematology and Medical Oncology, co-Director of the Center of Experimental Therapeutics, and Associate Director for Clinical and Translational Research, Knight Cancer Institute, Oregon Health & Science University. In her prior positions she served as head of Early Clinical Trials Development in the Office of the Director, Division of Cancer Treatment and Diagnosis, National Cancer Institute, and Professor and Director of the Phase I Clinical Research and Translational Oncology Programs, Stanford University. She specializes in conducting pharmacokinetic and pharmacodynamic driven first-in-human trials tailored to make early, informed decisions regarding the suitability of novel molecular agents for further clinical investigation. She is a member of scientific planning committees of national and international professional organizations and has authored over 150 peer reviewed publications, 9 book chapters, and co-edited a book on 'Novel Designs of Early Phase Trials for Cancer Therapies'. She is currently a member of the American Association for Cancer Research Exploratory IND/Phase 0 Clinical Trials Task Force, Scientific Program Annual Meeting Committee 2023 and Cancer Progress Report 2022 Steering Committee.

Meijuan Li, PhD (Eisai)

Driver Mutation Variant Allele Frequency in Circulating Tumor DNA and Association with Clinical Outcome in NSCLC Patients with EGFR or KRAS Mutated Tumors

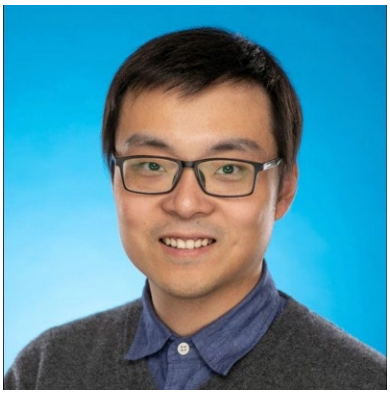
Abstract: ctDNA has much potential utility in drug development including predicting patient survival or cancer recurrence, predicting treat response, monitoring patient disease status or treatment response, and detecting early stage cancer. Actionability on the continuous output of variant allele frequency (VAF) of ctDNA has not been well characterized in the clinical setting, limiting its use. In this talk, the association of allele frequency of clinically relevant short variants in circulating free DNA (cfDNA) with overall survival (OS) and real-world progress free survival (rwPFS) for patients using real-world data was also assessed. The results of the association analysis indicated that VAF of the predictive biomarker mutation was negatively correlated with OS of NSCLC patients who were under different treatment options, and the association between mutation VAF and rwPFS was not significant. The results from this study may support ctDNA as a potential surrogate endpoint of the clinical outcome.

Bio: Dr. Meijuan Li has extensive and diverse experience in statistics, molecular biology, cancer biomarker development, and FDA regulation of precision medicine products, diagnostic devices, and radiology therapeutic devices etc. She currently is VP, Head of Biostatistics, Eisai Oncology Business Group. She assumes strategic leadership of the OBG Biostatistics line function. In this capacity, she is accountable for all developmental programs, clinical studies, regulatory submissions, and business development programs etc.

Before joining Eisai, Dr. Li spent about two and half years at Foundation Medicine (FMI) where she served as VP, Head of Biomarkers and Biometrics and led development in the following areas: Statistics, Data Management, and Biomarker development and analysis. She played a critical role in the first FMI regulatory filing and approval for blood based NGS assay as well as in the expansion of FMI's liquid and solid companion diagnostic portfolio. In addition, she was instrumental in developing several important bioinformatics algorithms including reversion mutation caller algorithm and FDA approved CDx, MSI detection algorithm.

Prior to joining industry, Dr. Li worked at the FDA for approximately 11 years holding various leadership positions of increasing responsibility. She oversaw all biostatistical aspects of pre- and post-approval studies pertaining to the regulation of biomarkers for oncology products, neurology products, radiology devices (diagnostic and therapeutic), and combination medical products etc. She worked on numerous projects such as artificial pancreas and glucose monitoring devices, digital pathology whole slide imaging system, digital mammography imager, hematology, and microbiology diagnostic devices.

Dr. Li has co-authored numerous publications and book chapters on various technical topics including Bayesian statistics, survival analysis, missing data, biomarker and CDx, ctDNA, real world data, and personalized medicine. She is the leading statistician in the field of companion diagnostic devices in precision medicine and was named as "the Statisticians of the Month, 2020" by Medical Devices and Diagnostics Section, American Statistical Association due to her significant contributions to statistics of medical devices and diagnostics. In addition, she is the holder of several granted or submitted patents (joint) on novel biomarker discovery or bioinformatics algorithms.



Xiao Li, PhD (Genentech)

When Spatial Statistics Meets Digital Pathology – Integrating Spatial Statistics

Abstract: With the advance of imaging technology, digital pathology imaging of tumor tissue slides is becoming a routine clinical procedure for cancer management. This process produces massive information that captures histological details in high resolution, including spatial structure of tissue components and their microenvironment interactions. Several reports have demonstrated the prognostic and predictive value of the spatial characterization of the TME, and the spatial arrangement and architecture of different cells across different types of cancers. However, these reports either focused on simple density or distance measures, implying a lack of analytic framework that comprehensively characterizes spatial TME heterogeneity. In this talk, I will present a spatial statistics empowered analytical pipeline that generates system-level features of cell-cell interaction by modeling histopathology images as spatial data. Our data application further showed that the outcome-agnostic pipeline that embeds spatial information shows better prediction performance when compared to conventional approaches in several clinical utilities.

Bio: Dr. Xiao Li obtained his PhD in Biostatistics and Data Science from The University of Texas Health Science Center at Houston, where he focused on methodology development of Bayesian Non-parametric and Spatial modeling on Cancer Radiomics.

Dr. Li started his career as a senior biostatistician (biomarker focused) at Gilead Sciences. Later he joined Genentech as a Data Scientist. He is now a Principal Data Scientist at Data, Analytics and Imaging group at Personalized Healthcare, Genentech/Roche. At work, He mainly focuses on utilizing AI & statistical learning to develop clinical support products for drug discoveries. Specifically, this includes developing software/tools to enable patient prognosis risk stratification; collaborating cross-functionally to generate translational insights, i.e., biomarker discoveries. He has several publications and related patents under review in these areas. He is also interested in leveraging Real-world data for clinical trial designs. Dr. Li is the co-PI of an FDA funded project that applies novel statistical approaches to develop a decision framework for hybrid randomized controlled trial designs.

Dave Miller (Unlearn)

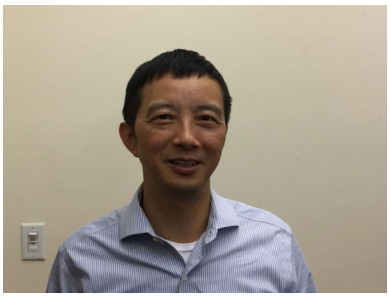
Prognostic Covariate Adjustment: A Novel Method to Reduce Trial Sample Sizes While Controlling Type I Error

Abstract: Randomized controlled trials often require large sample sizes to obtain statistical power. Faster clinical trials can be achieved by augmenting a small study cohort with external datasets of untreated patients. Unfortunately, statistical methods that merge subjects into the trial placebo arm directly tend to inflate the Type I error rate in the presence of unmeasured confounders.

Prognostic Covariate Adjustment (PROCOVA), a procedure that EMA has recently qualified for use in pivotal studies, incorporates learning from historical data while maintaining Type I error. For each trial subject, the model generates a Prognostic Digital Twin: a clinical prediction of the subject's outcome if they were randomized to the control arm. The Prognostic Digital Twin is a multivariate distribution of the trial's outcomes of interest. The expected value for a single outcome at a single timepoint is a prognostic score that can be used as a powerful covariate to estimate treatment effects more precisely.

PROCOVA decreases the variance of the treatment effect as a function of r, where r is the Pearson correlation between prognostic score and observed trial outcomes in each study arm. Rather than reducing the variance with the same sample size, one may choose to reduce the sample size while maintaining the same variance as a larger study. We present a reanalysis of a Phase II Alzheimer's Disease trial, where we achieve a similar standard error to the original study with a 23% sample size reduction for the placebo arm.

Bio: Dave Miller is the Chief Science Officer at Unlearn, overseeing the Clinical, Regulatory, and Biostatistics function. He is a biostatistician with expertise in prognostic models, clinical trials, observational studies, and diagnostic devices. Prior to joining Unlearn, he was Head of Biostatistics & Epidemiology for Verily Life Sciences. Other past roles include Lead Program Biostatistician at Genomic Health and Sr Dir of Medical Affairs Statistical Analysis at ICON Clinical Research. Dave received his bachelors and masters in Statistics from Carnegie Mellon. He has co-authored over 100 peer-reviewed papers in medical journals.



Lei Nie, PhD (FDA)

Challenges and Opportunities in Rare Genetic Disease Drug Development

Abstract: Small patient populations, slow disease progression, heterogenous or even variable disease presentation are among the chief obstacle to successfully developing therapies for patients with rare genetic diseases. These obstacles lead to major challenges in establishing efficacy endpoints, selecting endpoints, determining the magnitude and types of error rate controls for multiple endpoints. Through elaborating these challenges, we also identify potential solutions and opportunities.

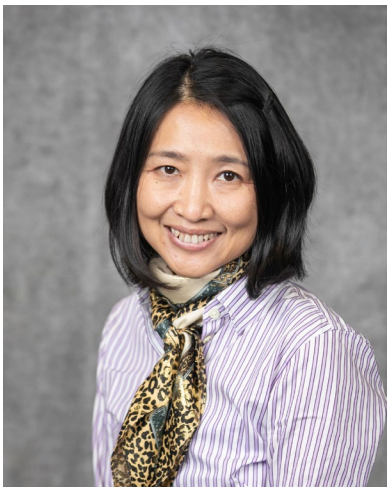
Bio: Dr. Lei Nie is the Director of Division of Biometrics IV in the Office of Biostatistics, OTS/CDER/FDA. He is interested in learning, developing and promoting innovative statistical methods in drug development. He is an elected fellow of the American Statistical Association and authored/co-authored more than 100 peer reviewed journal publications.

Mark van der Laan, PhD (UC Berkeley)

Targeted Learning and Causal Inference for Integrating Real World Evidence into the Drug Approval Process and Safety Analysis

Abstract: Targeted Learning represents a general roadmap for accurately translating the real world into a formal statistical estimation problem, and a corresponding template for construction of optimal machine learning based estimators of any desired target causal estimand combined with formal statistical inference. It is flexible by being able to incorporate high dimensional and diverse data sources. To optimize finite sample performance, it can be tailored towards the precise experiment and statistical estimation problem in question, while being theoretically grounded, optimal, and benchmarked. We provide a motivation, explanation, and overview of targeted learning; the key role of super-learning; and discuss SAP construction based on targeted learning. We also discuss a Sentinel and FDA RWE demonstration project of targeted learning.

Bio: Dr. Mark van der Laan is the Jiann-Ping Hsu/Karl E. Peace Professor in Biostatistics and Statistics at the University of California, Berkeley. His research interests include censored data, causal inference, genomics, observational studies and adaptive designs. He has led the development of two general statistical approaches: Super Learning and Targeted Learning. Targeted Learning improves on typical current statistical practice by avoiding reliance on wrong model assumptions, and its capability to target any question of interest. Targeted Learning improves on machine learning or other data adaptive methods by 1) targeting arbitrary questions of interest, 2) being more precise and unbiased and 3) providing formal statistical inference in terms of confidence intervals. In 2005 he was awarded the Committee of Presidents of Statistical Societies (COPSS) Presidential Award in recognition of outstanding contributions to the statistics profession. He also received the 2004 Spiegelman Award and 2005 van Dantzig Award. He is co-founder of the international Journal of Biostatistics and Journal of Causal Inference. He has authored various books on Targeted Learning, Censored Data and Multiple Testing, published over 400 publications, and mentored 60 Ph.D. students and 30 postdoctoral fellows.



Hui Wang, PhD (VA Palo Alto)

Pragmatic Use of the Electronic Medical Records in Clinical Research from the Veterans Affairs Healthcare – A Closed Healthcare System

Abstract: This talk will share the experiences on the use of the VA EHR data in clinical research studies – its advantages, limitations, and challenges – through real-world examples. As an early adopter of the EHR system in its care system, VA has one of the best EHR data in the nation. The VA EHR data was collected within a closed healthcare system with uniform standard that made it well-suited for research use. We will discuss types of studies suitable to use EHR data, the issues and challenges we encountered when using EHR data such as data standardization, quality assessment and bias control, integration of EHR data with other data sources such as clinical trial data, and the differences between the VA EHR data and the commercially available databases on market.

Bio: Dr. Hui Wang has worked in the field of clinical research as a biostatistician for the past 15 years. She serves as a mathematical statistician in the Veterans Affairs Cooperative Studies Program, where she works with the VA's EHR data to conduct clinical research studies with VA investigators. Before that, she was a faculty biostatistician at the Department of Pediatrics, Stanford Medical School. Dr. Wang received her Ph.D. degree in Statistics from UCLA and her postdoctoral training from UC Berkeley. She recently founded a company that explores the application of real-world data in clinical development for pharmaceutical companies.

Heng Xu, PhD (Nektar)

Adaptive Endpoints Selection with Application in Rare Disease

Abstract: In rare diseases, there are many unanswered questions that are critical to clinical development. Among them, one important question is how to choose primary endpoints that translate into meaningful improvement of health outcomes for patients while maximizing trial probability of success at the same time. A natural history study is often recommended by regulatory agencies, following this, traditional approach has dampened enthusiasm for many drug developers because it entails much higher cost and longer timeline. We propose to use an innovative design that allows adaptation on primary endpoint(s) so that the learning stage of the disease can be done within the pivotal trial itself through a subset of patients (i.e., informational cohort). The overall family wise error rate will be controlled through the use of combination test following partition test principle. A case example in patients with Pompe disease is used to show that the proposed innovative design maintains robust power across treatment effect scenarios while traditional fixed design bears the high risk of failure due to incorrect endpoint selection. Even if multiple endpoints can be included as primary, the proposed innovative design can still improve power over traditional designs by optimizing alpha allocations in cases with differential treatment effects.

Bio: Dr. Heng Xu is a biostatistician at Nektar Therapeutics in San Francisco. He obtained his PhD in Mathematical Statistics at Purdue University, and his research interests focus on adaptive designs and event projections. He will present his collaborative work on adaptive endpoint selection with application in rare diseases.

Career Panel Session

Ning Leng, PhD (Genentech)

Bio: Ning Leng is a People and Product Lead in Product Development Data Sciences in Roche-Genentech. Ning joined Roche-Genentech in 2016 as a statistician in the oncology early development and personalized healthcare group. Ning has worked on both early and late phase oncology development, with a special interest in utilizing diverse data sources and advanced methodologies to generate insights for personalized healthcare. Ning is an advocate of automation, open sourcing and open collaboration in pharma. She is also passionate in connecting people and helping people find new interests and opportunities. She serves as Roche representative on the R consortium board, co-leads the R consortium R submission working group, and co-leads the Meetup series of the Bay-area Biotech-pharma Statistics Workshop (BBSW). Ning holds a B.S. in Information and Computing Science from Beijing Institute of Technology and a Ph.D. in Statistics from University of Wisconsin-Madison.

Abstract: Today's fast changing environment brings ambiguity and uncertainty to how we work. At the same time, these challenges provide an unprecedented amount of opportunities and flexibility that enable us to define our personalized career path. In this talk, I will share my journey of exploring my 'career lattice', by navigating through computational genomics, clinical statistics and data science software development.

Ruixiao Lu, PhD (Alumis)

Bio: Ruixiao Lu, Ph.D. is currently VP, Head of Biostatistics, at Alumis Inc. a precision medicine company with the mission to transform the lives of patients with autoimmune diseases, overseeing the Biostatistics and Statistical Programming function. Prior to Alumis, she was VP, Head of Statistics, Data Science and Clinical Data Management at Quantum Leap Healthcare Collaborative (QLHC), the sponsor for the large-scale clinical trials using master protocols such as I-SPY for Breast Cancer and I-SPY COVID. Before she transitioned to pharma industry, she held increasing responsibilities over the years at Genomic Health, Inc (GHI) which was subsequently acquired by Exact Sciences, an industry leader in personalized cancer diagnostics, leading product development and medical affairs programs and in the end as the Director and functional head for the statistics and programming. She also contributed to business development evaluation and provided strategic input to product lifecycle management throughout her career.

Ruixiao has been dedicated to promoting statistics and data sciences through many of her leadership positions in various non-profit organizations including BBSW. She is currently the Treasurer and Board Member, and the Vice Chair of District 6, for the American Statistical Association (ASA). She had also held key officer positions in the San Francisco Bay Area Chapter of ASA, including President, and received ASA Outstanding Chapter Service Award for her years of service. Ruixiao is a co-founder and Board of Director for DahShu, a 501(c)(3) non-profit organization whose mission is to promote the research and education for data science.

Ruixiao received her B.S. in Statistics from Peking University, and her Ph.D. in Statistics, with Emphasis in Biostatistics, from University of California, Davis.

Fan Zhang, PhD (AstraZeneca)

Bio: Fan Zhang is the Head of Late Hematology Statistics, Oncology Biometrics, at AstraZeneca following the integration of Acerta Pharma, where she was the Vice President of Biometrics at Acerta Pharma, a Member of AstraZeneca Group, and led Biostatistics, Clinical Data Management and Statistical Programming functions and successfully achieved Calquence approvals in CLL in multiple countries simultaneously under FDA RTOR and Project Orbis pilots.

Fan has over two decades of biopharma experience, including 18 years in oncology drug development for solid tumors and hematologic malignancies. Prior to joining Acerta, she grew and led the Data & Statistical Sciences group at AbbVie's Redwood City and South San Francisco sites to support the oncology early development programs.

Before AbbVie, Fan spent over 10 years at Genentech with increasing responsibilities leading biostatistics in late stage oncology development and global regulatory submissions that resulted in multiple successful product launches. Fan spent her formative years in the biopharma industry at Eli Lilly where she was the lead statistician for pivotal phase III clinical trials in neuroscience indications.

Fan received her M.S. and Ph.D. in Biostatistics with a minor in HIV/AIDS Research from UCLA School of Public Health.

Abstract: Change is constant: Seeking it, Embracing it, Navigating it.

We likely all have experienced changes throughout our professional journey at different stages of our career. The causes of these changes may vary: sometimes we proactively seek changes; oftentimes we get involved in changes; and more commonly, changes have a way to find us whether we like them or not. In this talk, I share a few inflection points on my own journey, the decisions I made and actions I took, as well as my reflection on the lessons learned that I have found useful to help guide me on dealing with future changes.

C-Suite Panel Session

Yali Li, PhD (Chief Data Officer, Function Oncology)

Bio: Yali is a statistics and data science leader dedicated to transforming data into innovation, vision, decisions, and products, with a focus on integrating data insights into diagnostics development. She is currently the Chief Data Officer of Function Oncology, driving data strategies for

developing the next generation precision medicine. Prior to Function Oncology, she has been building and leading teams of data science talent for over 12 years in translational research, precision medicine, and cancer diagnostics at various pharmaceutical/biotech companies, including Foundation Medicine and Thrive. Yali's experience spans Biostatistics and Data Science, Biomarker and Clinical Development, and Molecular Diagnostics. She received her Ph.D. in Biostatistics and M.S. in Molecular Biology and Microbiology from Case Western Reserve University.

Stacy Lindborg, PhD (Chief Development Officer, Brainstorm Cell Therapeutics)

Bio: Stacy Lindborg, PhD, is a globally recognized leader skilled at leading teams and creating strategic alliances with cross-functional and external partners to drive innovation within drug development. Currently Executive Vice President & Chief Development Officer at Brainstorm Cell Therapeutics. She is a member of Brainstorm's Executive Leadership Team and is accountable for driving the creation of clinical development strategies from first-in-human (FIH) through registration – including framing innovative development paths and identifying the best opportunities, ensuring evidence is generated as required by key constituents (scientific community, regulatory, commercial/payer) and resourcing them adequately so they can be realized.

Most recently, she was Vice President & Global Analytics and Data Sciences Head at Biogen – responsible for evidence generation as well as statistical and epidemiological support for R&D and marketed products. Serving on all R&D governance bodies, she was active in guiding the firm's long-term vision for growth and stimulating innovative platforms increasing Biogen's productivity. Previous positions include: Head of R&D strategy at Lilly, responsible for characterizing the productivity of the portfolio, driving key R&D strategy projects including the R&D component of annual corporate Long-Range Plan; and General Product Management Leadership (responsible for R&D, Commercial and Manufacturing plans).

Stacy is passionate about analytics and loves to operate at the intersection of innovative Statistics and R&D strategy. She has been a student of leadership styles across her career.

Dr. Lindborg earned a Ph.D. in Statistics from Baylor University and has been elected as Fellow of the American Statistical Association. She is currently serving on the Scientific Advisory Board for Cytel, and as an independent board member for MIT Catalyst. She is on the Board of Directors for Celsion Corporation and the Massachusetts Down Syndrome Congress.

Tara Maddala, PhD (CEO, Pandora Bio)

Bio: Tara is CEO of Pandora Bio, a mental and behavioral health startup utilizing biometric data for early detection of mental illness. Tara is also an expert consultant and principal of TMBiostats LLC, providing clinical strategy and statistical advice and serving on scientific advisory boards. Previously, Tara served as Vice President of Clinical Development at Delfi Diagnostics and was responsible for clinical trials serving the mission of developing a new class of high-performance, affordable liquid biopsy tests for early cancer detection. Before Delfi, Tara was the Vice President of Biometrics at GRAIL, leading a team of statisticians/data scientists, data managers, and biosample managers. Tara has also led the Biostatistics team at Genomic Health (GHI) and Clinimetrics, a CRO. She is co-inventor on several machine learning cancer genomic patents and has co-authored approximately 20 peer-reviewed publications and over 40 congress presentations. In addition to BBSW, Tara volunteers with Young Women in Bio, regularly lectures for the UCSF-Berkeley Translational Medicine program, and is an advisor to the UCSF Center for Translational and Policy Research on Personalized Medicine. She holds a PhD in Biostatistics from The University of Texas and Engineering BS and MS degrees from The University of Florida and Georgia Tech.

Dave Miller (Chief Science Officer, Unlearn)

Bio: Dave Miller is the Chief Science Officer at Unlearn, overseeing the Clinical, Regulatory, and Biostatistics function. He is a biostatistician with expertise in prognostic models, clinical trials, observational studies, and diagnostic devices. Prior to joining Unlearn, he was Head of Biostatistics & Epidemiology for Verily Life Sciences. Other past roles include Lead Program Biostatistician at Genomic Health and Sr Dir of Medical Affairs Statistical Analysis at ICON Clinical Research. Dave received his bachelors and masters in Statistics from Carnegie Mellon. He has co-authored over 100 peer-reviewed papers in medical journals.

Session Chairs and Organizers

Rita Lopatin (Grail)

Bio: Rita has 25 years of industry experience focusing on cancer screening, genomic test development in oncology and medical devices. She has led statistical activities on multiple phase 2, 3 and 4 studies, and provided strategic and operational input on the development of long range plans, product offerings and regulatory submissions.

Currently Rita is a Senior Director of Biostatistics at GRAIL, LLC, which is an industry leader in multi-cancer early detection. Rita leads statistical activities across several studies and supports regulatory, marketing and MSL interactions. She also leads a cross-functional team coordinating activities and resources across different programs and provides strategic input to lifecycle planning. Prior to Grail, Rita was a Lead Program Biostatistician (Director level) at Genomic Health, where she was a lead biostatistician on liquid biopsy program and led cross-functional teams for development of novel molecular tests in bladder, colon and renal cancer programs.

Rita received her BS in economic cybernetics from Novosibirsk State University (Russia) and MS in Statistics, minor in Biostatistics, from University of Minnesota. Outside of work, she enjoys hiking, kayaking and traveling around the world.

Haijun Ma, PhD (Exelixis)

Bio: Dr. Haijun Ma is the Head of Biostatistics at Exelixis Inc. Before joining Exelixis, she worked at Amgen Inc. and Nektar Therapeutics, where she had roles of increasing responsibilities. She worked in different therapeutic areas and drug development stages ranging from Phase I to post-marketing. She has experience with clinical trials and observational studies using real world data. She is active in promoting good statistical practices and developing statistical methods to tackle practical problems. Her current research interests are in study designs, statistical methods for oncology trials, Bayesian statistics and RWD/RWE. She has published peer reviewed manuscripts and book chapters. She earned her Ph.D. in biostatistics at the University of Minnesota – Twin Cities.

Tara Maddala, PhD (Pandora Bio)

Bio: Tara is CEO of Pandora Bio, a mental and behavioral health startup utilizing biometric data for early detection of mental illness. Tara is also an expert consultant and principal of TMBiostats LLC, providing clinical strategy and statistical advice and serving on scientific advisory boards. Previously, Tara served as Vice President of Clinical Development at Delfi Diagnostics and was responsible for clinical trials serving the mission of developing a new class of high-performance, affordable liquid biopsy tests for early cancer detection. Before Delfi, Tara was the Vice President of Biometrics at GRAIL, leading a team of statisticians/data scientists, data managers, and biosample managers. Tara has also led the Biostatistics team at Genomic Health (GHI) and Clinimetrics, a CRO. She is co-inventor on several machine learning cancer genomic patents and has co-authored approximately 20 peer-reviewed publications and over 40 congress presentations. In addition to BBSW, Tara volunteers with Young Women in Bio, regularly lectures for the UCSF-Berkeley Translational Medicine program, and is an advisor to the UCSF Center for Translational and Policy Research on Personalized Medicine. She holds a PhD in Biostatistics from The University of Texas and Engineering BS and MS degrees from The University of Florida and Georgia Tech.

Tuan Nguyen, PhD (Alector)

Bio: Tuan Nguyen is currently a Director of Biostatistics at Alector. He has 12+ years of pharmaceutical industry experience in statistical methodologies and modeling in biomarker analyses supporting phase 1-3 clinical trials in Oncology, Liver, Respiratory and Neurodegenerative therapeutic areas. Before joining Alector, he had worked at Eli Lilly, Gilead, Nektar and Theravance, with special interest in data science/machine learning methods in the context of multivariate prediction and integrative analyses/data mining/subgroup identification. Tuan holds a Ph.D. in Statistics from Rice University.

Godwin Yung, PhD (Genentech)

Bio: Godwin Yung is a Principal Statistical Methodologist in the statistical methods, collaboration, and outreach (MCO) group at Genentech/Roche. As such, he conducts methods research (e.g., survival), enables the use of the most appropriate statistical methodology in pharmaceutical development at his company (e.g., adaptive designs, borrowing external information, surrogate endpoints), and collaborates with external stakeholders to advance the field of statistics (e.g., Oncology Estimand Working Group, BBSW, Statistics in Pharmaceuticals). Godwin received his B.A. in Mathematics from Reed College and Ph.D. in Biostatistics from Harvard University.

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company is committed to advancing innovative medicines to prevent and treat life-threatening diseases, including HIV, viral hepatitis and cancer. Gilead operates in more than 35 countries worldwide, with headquarters in Foster City, California. To learn more about our current career openings, visit https://www.gilead.com/careers/careers-at-gilead

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Cytel enables decision-makers in the life sciences to unlock the full potential of their products. From navigating uncertainty to proving value, Cytel's 30 years of global expertise in consulting, data-driven analytics, and industry-leading software helps biotechs and pharmaceuticals transform intelligence into confident decisions. We have an uncompromising commitment to scientific rigor and high standards of operational excellence, which are channeled through our locations in North America, Europe, the United Kingdom, and Asia. Together, we enable our clients to deliver the therapies that propel us forward.

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Loxo@Lilly aims to create medicines that make life better for all those affected by cancer around the world. Bringing together the focus and spirit of a biotech with the scale, resources, and heritage of Lilly, our team is focused on rapidly delivering impactful new medicines for people with cancer. Our approach centers on creating oncology medicines that unequivocally show early signs of clinical activity and will matter to patients.

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We are headquartered in Vancouver, BC with offices in Seattle, Toronto, Calgary, India, and Ireland. We work with clients predominantly in the Life Sciences sector including Nanostring, Roche, Genentech, Kite Pharma, CRISPR Therapeutics, Seagen and Kaiser Permanente. We are partnered with AWS, Snowflake and are one of the few RStudio Full-service partners in the world.

We have experience working on molecular, cellular, and animal studies across a multitude of disease areas such as oncology, immunology, virology, and ophthalmology that have led to publications in leading peer-reviewed scientific journals. This, coupled with our understanding of mature and emerging next-generation sequencing methodologies and biocomputing technologies, affords us the ability to develop sophisticated analysis pipelines to interpret complex pre-clinical and clinical data.

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visualize single-cell spatial transcriptomics; and developing next-generation computational models for multimodal genomic assays, including CRISPR screens and phased variants in ctDNA in cancer patients.

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- Engineering

RESOLUTIX

Resolutix supports the pharmaceutical industry with cost-effective, innovative solutions and talented individuals who can be trusted to process the clinical trial data to generate fast, accurate results. With Sponsor-managed strategic sourcing, we provide reliable, efficient consultants to work on sponsor studies remotely. Our team specializes in data analysis and standardization of clinical trial data in support of FDA, EMA, and PMDA submissions and more. Our resource pool includes specialists with over 150 years of combined experience in CDISC implementation. Our team consists of hard-working and passionate individuals who believe in achieving faster and accurate results through innovative solutions. We've consistently been recognized for our exceptional outcomes and service, like fast turn-around of CDISC packages for Phase 1 studies and SEND implementation for pre-clinical trials. Our consultants bring in a wide range of experience with multiple software packages, including but not limited to, SAS, R, MatLab, Python, Tableau, and Winnolin.

SimulStat provides statistical programming, biostatistics, and real-world data services to the pharmaceutical and biotechnology industry. Whether you need a consultant SAS programmer/biostatistician/epidemiologist or an entire FSP team, we can provide the resources you need. We also provide off-site biostatistics and statistical programming outsourcing solutions for projects your infrastructure may not be able to support.

Co-founded by two seasoned statisticians with >20 years of experiences in pharmaceutical companies, Stat4ward (www.stat4ward.com) provides consulting services in the following areas

- Companion diagnostics (CDx) development and submission. Stat4ward has been involved in >10 PMA submissions. Supports include
    - Analytical validation studies. Study design and analysis according to CLSI guidelines
    - Clinical bridging SAP
    - Submission documents
    - Interactions with FDA
- LDT/IVD. Stat4ward has supported multiple 510k and De Novo submissions, and also supported many CLIA submissions. Supports include
    - Analytical validation studies
    - Algorithm development
    - Clinical validation studies
    - Publications
    - Interaction with FDA/CLIA/Medicare
- Biomarkers. Stat4ward has deep expertise in supporting exploratory biomarker studies. We have deep understanding of different assay technologies, e.g.
    - Omics (e.g. NGS, proteomics, Nanostring)
    - Flow Cytometry
    - Multiplex ELISA (e.g. Luminex, MSD)
    - High content imaging
- CMC
    - Analytical method development, validation, maintenance
    - Stability
    - Lot release
    - Spec limits
- Preclinical
    - In vitro studies using different assay technologies
    - In vivo studies using different animal models (e.g. mouse clinical trial, xenograft)
- Others
    - Immunogenicity cutpoint

- Adaptive design
- MCP-MOD
- Clinical trials (all phases)

Silver Sponsors

The 2022 BBSW workshop is sponsored by the American Statistical Association's Biopharmaceutical Section.

Jazz Pharmaceuticals' purpose is to innovate to transform the lives of patients and their families.

We are focused on developing life-changing medicines for people with serious diseases — often with limited or no options — so they can live their lives more fully. By transforming biopharmaceutical discoveries into novel medicines, we are working to give people around the world the opportunity to redefine what's possible – to make the "small wins" big again.

We are a fully integrated, global biopharmaceutical company with a diverse portfolio of marketed medicines and novel product candidates for adults and children, and expertise in two key therapeutic areas: neuroscience and oncology. We are an industry leader in treating sleep disorders and epilepsy, and in oncology we are investigating and delivering medicines for hard-to-treat hematologic malignancies and solid tumors. We use our deep knowledge of and commitment to these fields to innovate and identify new solutions where none previously existed.

Seagen is a global, multi-product biotechnology company dedicated to developing and commercializing transformative cancer medicines. As the industry leader in antibody-drug conjugate (ADC) technology, we pioneered a new generation in the science of harnessing antibodies to deliver cell-killing agents directly to cancer cells. Seagen's dedication to improving the lives of cancer patients goes beyond science, and we believe that every employee has a role in contributing to this mission. By working together with a shared dedication and diverse perspectives, we are able to reach our full potential and make a real difference in the world. Seagen is a fast-growing company and has an abundance of opportunities available for you to grow your career. Put your passion to work at Seagen and join us!

WU CONSULTING, INC.

Wu Consulting, Inc. (WCI) was founded in 1996, based in King of Prussia, PA and San Francisco, CA. It specializes in providing statistical support to its pharmaceutical clients in its expertise's area of Biostatistics, SAS Programming, and Data Management. Its FSP team has been recognized as top-notched stat and SAS experts in the pharmaceutical industry.

If you are looking for a highly qualified and reliable functional service provider for your organization to provide skilled and highly experienced biostatistician and programming consultants, or if you are a consultant and would like to join WCI's team to work for its FSP clients, please contact:

Wei Zhang
Wu Consulting, Inc.
(610) 761-1740, Email:
wei@wuconsulting.com

For more information about Wu Consulting, Inc. please go to its website: www.wuconsulting.com

UNLEARN

Good for patients. Good for sponsors.

A SCIENCE FIRST COMPANY

Do Good Work

Founded in 2017, Unlearn brings together a world-class team of experts across pharma, medtech, physics, and business who share a vision of using machine learning and regulatory acceptable biostatistics methods to improve clinical trials for the benefit of patients and sponsors.

SMALLER, POWERFUL TRIALS WITHOUT BIAS

TwinRCT™

A TwinRCT™ is a randomized trial that uses machine learning to achieve a higher probability of success with a smaller number of patients. As in a traditional RCT, patients are randomized to a treatment group and a control group, but, unlike a traditional trial, a digital twin is computed for every patient. The treatment effects for the primary and secondary outcomes can all be estimated with greater precision after correcting for a prognostic score derived for each patient from the digital twins.

GET IN TOUCH

Reach out to us to learn more about leveraging TwinRCTs™ to enable smaller, more efficient trials.

TwinRCT™s accelerate clinical trials across all disease areas including:

CNS

- Alzheimer's Disease
- Multiple Sclerosis
- Amyotrophic Lateral Sclerosis
- Huntington Disease
- Parkinson's Disease

I&I

- Systemic Lupis Erythematosus
- Rheumatoid Arthritis
- Psoriasis
- Crohn's Disease
- Ulcerative Colitis

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TRUSTED STATISTICAL METHODOLOGY

PROCOVA™

In March 2022, the European Medicines Agency (EMA) published a draft qualification opinion on our 3-step PROCOVA™ procedure. The draft opinion provides the regulatory framework for planning and conducting a Phase 2 or Phase 3 TwinRCT™.

"CHMP qualifies PROCOVA™ as prognostic score adjustment and the proposed procedures as described in a handbook for trial statisticians could enable increases in power or precision of treatment effect estimates in phase 2 and 3 clinical trials with continuous outcomes."

– EMA March 2022 Draft Qualification Opinion for PROCOVA™

SCAN THIS CODE TO ACCESS A SUMMARY OF THE DRAFT QUALIFICATION AND A HANDBOOK ON HOW TO APPLY OUR PROCOVA PROCEDURE

AN INNOVATIVE MULTI-YEAR PARTNERSHIP

Merck KGaA

Darmstadt, Germany

In February 2022, Unlearn and Merck KGaA signed a multi-year partnership to accelerate late-stage immunology clinical trials leveraging TwinRCTs™ to facilitate smaller control arms and generate appropriate evidence to support regulatory decisions.

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