PROGRAMME OF THE EIGHTIETH ANNUAL DEMING CONFERENCE ON APPLIED STATISTICS

Sponsored by
Deming Conference Organization
AMERICAN STATISTICAL ASSOCIATION: Biopharmaceutical Section

December 8 – December 12, 2025: Three-Day Conference plus Two-Day Short Course
Tropicana Casino and Resort, Havana Tower, Atlantic City, NJ

Three Keynotes on December 8-10, 2025

1: **Shaping the Future: Advancing the Value and Protecting the Integrity of Clinical Trials, Prof. Scott Evans**
2: **Tufts CSDD – DIA Survey Results on AI Adoption in Drug Development, Prof. Mary Jo Lamberti**
3: **Optimising Group Sequential and Adaptive Designs: Where Frequentist meets Bayes, Prof. Christopher Jennison and Bruce Turnbull**

Twelve Sessions of Tutorials on December 8-10, 2025

Two Short Courses on December 11-12, 2025

1. **Classification and Regression Trees by Example by Prof. Wei-Yin Loh, University of Wisconsin, Madison**
2. **Group Sequential and Adaptive Methods for Clinical Trials by Christopher Jennison, University of Bath and Bruce Turnbull, Cornell University**

A $5,000 college scholarship will be awarded to an undergraduate spouse, child, stepchild, or grandchild of a registrant

ONSITE REGISTRATION WILL BE ON THE FOURTH FLOOR OF THE HAVANA TOWER. It will start at 6:00 pm on Sunday December 7th and will be followed by a one-hour reception with cold drinks and snacks. It will continue at 7:00 AM Monday December 8th through Thursday December 11th. THREE-DAY REGISTRANTS WILL RECEIVE A DIGITAL COPY OF THE HANDOUTS FOR ALL SESSIONS.

RECEIPTS and a CERTIFICATE OF ATTENDANCE will be distributed at the conference. Register and pay for both the conference and the hotel online as early as possible at www.demingconference.org. This gives you an instant receipt. Only if absolutely necessary, mail a check with your completed registration form on the last page in this program. If checks aren't postmarked on or before the early discounted registration date, you will be charged the next higher amount. E-Mail Cancellations sent to registrar@demingconference.org will be accepted until November 16th for a separate $50 fee for both the conference and courses. Afterwards, there will be no refunds but substitution of another registrant is permissible. Book orders can't be cancelled. If a registrant cancels, his or her ordered books can be picked up at the site or would be mailed.

We are soliciting abstract proposals for posters for the Deming Conference 2025. The Poster Presentation forum allows participants to submit their research concepts and issues of relevance for peer review in biostatistics. Poster sessions, which will be held on all 3 days of the conference, allow attendees to discuss the specifics of an abstract with the author in a small group setting. There will be five poster awards. Students pursuing a doctorate degree in Biostatistics/Statistics may apply to receive a Deming Student Scholar Award and present a poster on their doctoral thesis at the conference. This award will be offered to up to three students from US Universities which will include travel, accommodation and registration fee to the 3-day conference (Dec 8 – 10, 2025). Once an award is granted, the students must present a poster on their doctoral thesis at the conference. Submissions will be accepted through Saturday, October 4, 2025. Poster abstracts can be emailed to sofia.paul@cellcentric.com and/or yuanyuan.guo1@lilly.com or submitted online for consideration.

Meeting facilities are in the Tropicana's Havana Tower state-of-the-art complex with 502 nonsmoking rooms where attendees stay in soundproof climate-controlled rooms with direct-dial phones, cable color TV, coffee makers, hairdryers, refrigerators, safe, iron and board, complimentary wireless internet and gorgeous views of the Atlantic City skyline.

- **There is a guest check in desk on the 3rd floor of the Havana Tower and meeting facilities are on the 4th floor.**
- **It's one of the largest NJ hotels, with 2,079 rooms, elegant public areas, exclusive retail shops and fine dining.**
- **It's on the beach with a complimentary heated indoor pool on the sixth floor of the South Tower.**
- **It has free Wi-Fi in the rooms and conference floor. The casino is in a separate building connected by a bridge.**

Eightieth (80th) Annual Deming Conference on Applied Statistics
Tropicana Casino and Resort, Havana Tower, Atlantic City, NJ
Sponsored by the Deming Conference Organization and AbbVie Inc.

Sunday December 7, 2025, Registration and Reception: 6:00 ⇒ 7:30 PM

Monday December 8, 2025, Registration: 7:00 ⇒ 8:00 AM and Hot Breakfast 7:00 ⇒ 7:50 AM

December 8, 8:00 ⇒ 9:00 AM Keynote: Shaping the Future: Advancing the Value and Protecting the Integrity of Clinical Trials, Prof. Scott Evans
Moderator: Alfred H. Balch

Session A (9am-12pm)	**Session B (9am-12pm)**
Selected Topics of Statistics in Clinical Drug Development: A Statistical and Regulatory Perspective Naitee Ting, Veramed Moderator: Din Chen	The DOOR is Open: Patient-centric, Pragmatic Benefit-Risk Evaluation in Clinical Trials Scott Evans and Toshimitsu Hamasaki, George Washington *University* Moderator: Alfred H Balch
Lunch (On Your Own) 12:00 ⇒ 2:00 PM	
Session C (2pm-5pm) ♣	**Session D (2pm-5pm) ♣**
Analysis of Recurrent Events Data in Medicine, Engineering, Marketing, and Other Fields Wayne B. Nelson, Wayne Nelson Statistical Consulting and Training Moderator: Din Chen	Introduction to Quantitative Decision Making in Drug Development Jerry Weaver, Teva and Joe Ibrahim, UNC Moderator: Naitee Ting

Monday December 8, 7:00 PM Speaker's and Awards Dinner (Optional Added Fee Event)

Tuesday December 9. 2025, Registration: 7:00 ⇒ 8:00 AM and Hot Breakfast 7:00 ⇒ 7:50 AM

December 9, 8:00 ⇒ 9:00 AM Keynote: Tufts CSDD – DIA Survey Results on AI Adoption in Drug Development, Prof. Mary Jo Lamberti
Moderator: Jingjing Ye

Session E (9am-12pm) ♣	**Session F (9am-12pm) ♣**
Advances in Interpretation of Patient-Reported Outcomes Joseph C. Cappelleri, Pfizer Moderator: Wenjin Wang	Targeted machine learning for reliable evidence generation from clinical studies Mark van der Laan, UC Berkeley and Susan Gruber, TL Revolution Moderator: Weili He
Lunch (On Your Own) 12:00 ⇒ 2:00 PM	
Session G (2pm-5pm) ♣	**Session H (2pm-5pm) ♣**
Unleashing the Power of Machine Learning and Deep Learning to Accelerate Clinical Development Li Wang, Yunzhao Xing, and Sheng Zhong, AbbVie Moderator: Jingjing Ye	Recent Developments and Innovations in Health Technology Assessment Weili He, AbbVIe and Xiang Zhang, CSL Behring Moderator: Bill Wang

Wednesday December 10, 2025, Registration: 7:00 ⇒ 8:00 AM and Hot Breakfast 7:00 ⇒ 7:50 AM

December 10, 8:00 ⇒ 9:00 AM: Keynote: Optimising Group Sequential and Adaptive Designs: Where Frequentist meets Bayes, Prof. Christopher Jennison and Bruce Turnbull
Moderator: Ivan F Chan

Session I (9am-12pm) ♣	**Session J (9am-12pm) ♣**
ICH E20 Adaptive designs for clinical trials - Scientific guideline Christopher Jennison, University of Bath, Vladimir Dragalin, Johnson & Johnson, and Bruce Turnbull, Cornell University Moderator: Bill Wang	Application of Data Borrowing in Clinical Trials and Regulatory Submissions Jerry Li, Inna Perevozskaya and Ivan F Chan, BMS Moderator: Alfred H Balch
Lunch (On Your Own) 12:00 ⇒ 2:00 PM	
Session K (2pm-5pm)	**Session L (2pm-5pm)**
Statistical Designs and Considerations for Dose Optimization Ying Yuan, MD Anderson and Philip He, Daiichi Sankyo Moderator: Kaylan Ghosh	Critical Thinking and Creative Analogies in Statistics, Science, and Technology: Essential Skills for the AI Era Mark Chang, Boston University Moderator: Ivan F. Chan

Thursday December 11, 2025, Registration: 7:00 ⇒ 8:00 AM and Hot Breakfast: 7:00 ⇒ 7:50 AM

Short Course 8:00⇒9:30 Lecture / **9:30⇒9:50** *Break* / **9:50⇒11:20 Lecture** / **11:20⇒12:40** *Lunch on Your Own* / **12:40⇒2:10 Lecture** / **2:10⇒2:30** *Break* / **2:30⇒4:00 Lecture** / **4:00⇒4:20** *Break* / **4:20⇒5:00 Lecture**

Classification and Regression Trees by Example Wei-Yin Loh, University of Wisconsin, Madison Moderator: Din Chen	Group Sequential and Adaptive Methods for Clinical Trials 🕮 Christopher Jennison, University of Bath and Bruce Turnbull, Cornell University Moderator: Bill Wang

Friday December 12, 2025, Hot Breakfast: 7:00 ⇒ 7:50 AM

Short Course (Continued) 8:00 - 9:30 Lecture / **9:30 - 9:50** Break / **9:50 - 11:20 Lecture** / **11:20 - 11:40** Break / **11:40 - 1:10 Lecture**

All tutorial and short course titles, presenters and moderators from 1970 onwards are on www.demingconference.org

🕮 Session is based on a recently published text that is available either for a discounted price or is included in the price of the short course registration

♣ Sessions will have their breaks extended by 15 minutes for Poster Presentations

TRAVEL TO THE DEMING CONFERENCE

Air Travel

When searching for flights, compare Atlantic City International Airport (ACY) and Philadelphia International Airport (PHL) to find the best fares and connections.

- ACY is currently served by Spirit Airlines, with Allegiant Air beginning service in late 2025. From ACY, frequent jitneys run into Atlantic City for about $3, and taxis cost roughly $10–12 to the Tropicana.
- PHL offers far more flight options. For ground transportation, the most budget-friendly choice is SEPTA to 30th Street Station in Philadelphia, connecting to NJ Transit's Atlantic City Rail Line. The trip takes over two hours but is economical. For faster door-to-door service, private shuttle companies operate from PHL to Atlantic City (reservations required; check pricing and numbers directly as rates vary). Car rentals are available at both airports, though a car is not strictly necessary once you are in Atlantic City.

Newark Airport is generally not recommended unless it offers significant savings on international flights. Travel from Newark to Atlantic City by public transit takes about three hours, usually by NJ Transit Bus #67 (via Toms River) or train connections.

Rail

NJ Transit's Atlantic City Rail Line offers 14 daily trips between Atlantic City and Philadelphia, with connections to Amtrak, NJ Transit's Northeast Corridor to New York City, SEPTA at 30th Street Station, and PATCO at Lindenwold. Free shuttle buses meet all trains and provide direct service to the Tropicana. Schedules are available at njtransit.com.

Direct weekend service from New York City was formerly offered by ACES Train, but this service is no longer operating. Travelers from NYC should instead connect via NJ Transit to Philadelphia and transfer.

Bus

The Tropicana Casino Transportation Department (888-275-1212, option 1) can confirm if casino bus service is available from your area—some routes run up to 200 miles. Many casino line-run buses provide rebates or bonuses to offset the cost. Greyhound also operates service to Atlantic City with promotions such as open return or slot-play credits (luckystreakbus.com).

Once in town, the Atlantic City Jitney runs along Pacific Avenue for about $3, making it easy to connect between casinos and hotels.

Driving

From the Garden State Parkway, New Jersey Turnpike, or Philadelphia, take the Atlantic City Expressway to Exit 2 (Black Horse Pike, Route 40/322) into Atlantic City. Turn left on Arctic Avenue at the first light over the bridge, follow Arctic Avenue to Brighton Avenue, then turn right. After crossing Atlantic Avenue, the entrance to "The Quarter" Garage (Havana Tower) will be on your left.

Self-parking or valet for hotel guests is typically $25-40 per day with unlimited in-and-out privileges. Check Caesars.com/parking for the rate.

Meals & Conference Hospitality

For details about restaurants and facilities, see tropicana.net. The Tropicana features 20+ dining options and multiple attractions.

Committee Members

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Conference Speakers Biography

Ivan S.F. Chan has extensive experience in the pharmaceutical industry. He is VP and Head of Oncology Biostatistics, Global Biometrics & Data Sciences at Bristol Myers Squibb, leading the global statistical support for oncology development. Prior to joining BMS, Ivan was VP and Head of Statistical Sciences at AbbVie, and he previously worked at Merck Research Laboratories where he led the global statistical support for vaccines. Ivan received his Ph.D. in Biostatistics from the University of Minnesota. He is an elected Fellow of the American Statistical Association (ASA) and an elected Fellow of the Society for Clinical Trials (SCT). He currently serves as Co-Chair of Deming Conference on Applied Statistics and Executive Director of the International Society for Biopharmaceutical Statistics. Ivan has previously served as the President of the International Chinese Statistical Association and the Program Chair of the ASA Biopharmaceutical Section. He has 90+ publications in statistical and clinical journals.

Mark Chang, PhD, is the founder of AGInception, an organization dedicated to artificial intelligence research. He is a Fellow of the American Statistical Association with over 25 years of experience as a statistician in both the biopharmaceutical industry and academia, having held positions ranging from Scientific Fellow to Senior Vice President. As an adjunct professor at Boston University, Dr. Chang has supervised PhD students on research topics such as adaptive clinical trial design and artificial intelligence. His broad research interests span adaptive clinical trials, AI, the principles of scientific methods, paradoxes, and various issues in modern biostatistics and software development. Dr. Chang has published 15 books, including most recently Artificial Intelligence for Drug Development, Precision Medicine, and Healthcare (2020); Foundation, Architecture, and Prototyping of Humanized AI (2023); and Critical Thinking and Creative Analogies in Statistics, Science, and Technology—Essential Skills for the AI Era (2025). He served on editorial boards of several statistical journals and is the Series Editor of the Chapman & Hall/CRC Biostatistics Book Series. He is also the co-founder of the International Society for Biopharmaceutical Statistics and has served as co-chair of the BIO Adaptive Clinical Trial Design Working Group.

Vladimir Dragalin is a Vice President and Scientific Fellow at Janssen, Pharmaceutical Companies of Johnson and Johnson. He is an adaptive designs expert with 25 years' experience in developing the statistical methodology of adaptive designs, with over 12 years' experience in pharmaceutical industry including positions at GlaxoSmithKline, Wyeth, Pfizer and at leading CROs Quintiles and Aptiv Solutions. Dr. Dragalin is a Fellow of the American Statistical Association and Member of the Drug Information Association, the Society for Clinical Trials, the International Society for CNS Clinical Trials and Methodology, and an Associate Editor of Journal of Biopharmaceutical Statistics. He is actively involved in the PhRMA Working Group on Adaptive Designs and the PhRMA Working Group on Adaptive Dose Ranging Studies (currently under DIA) and an elected member of the PhRMA Biostatistics and Data Management Technical Group.

Scott Evans is a Professor and Founding Chair of the Department of Biostatistics and Bioinformatics and the Director of the Biostatistics Center at Milken Institute School of Public Health of the George Washington University. He is the: Director of the Statistical and Data Management Center for the Antibacterial Resistance Leadership Group (ARLG) funded by NIAID/NIH; the PI of the Coordinating Center for the Exercise and Nutrition Interventions to Improve Cancer Treatment-Related Outcomes (ENICTO) in Cancer Survivors Consortium funded by the NCI/NIH, and the co-PI of the Data Coordinating Center of the Clamp OR Delay among neonates with Congenital Heart Disease (CORD-CHD) clinical trial funded by the NHLBI/NIH. He is the author of more than 250 publications and four books on clinical trials. He served as: the Guest Editor of a mini-Series on DSMBs for the *NEJM Evidence*; a member of an FDA Advisory Committee; and the past-President of the Society for Clinical Trials (SCT). He is a recipient of the Mosteller Statistician Award, the Zackin Distinguished Collaborative Statistician Award, the Founders Award from the American Statistical Association (ASA), an elected member of the International Statistical Institute (ISI), and is a Fellow of the ASA, SCT, and the Infectious Disease Society of America (IDSA).

Susan Gruber, PhD, MPH, MS, is the founder of Putnam Data Sciences, a statistical consulting and data analytics consulting firm. Her work focuses on the development and application of data-adaptive methodologies for improving the quality of evidence generated by observational and randomized health care studies. Prior to forming Putnam Data Sciences, Dr. Gruber was the Director of the Biostatistics Center in the Department of Population Medicine at Harvard Pilgrim Health Care and Harvard Medical School, and former Senior Director of the IMEDS Methods program at the Reagan Udall Foundation for the FDA.

Toshimitsu Hamasaki is a Professor of Biostatistics at the Biostatistics Center and the Department of Biostatistics and Bioinformatics at the Milken Institute School of Public Health, George Washington University. His research focuses on the design, monitoring, analysis, and reporting of clinical trials. He has authored more than 200 peer-reviewed publications and four textbooks on statistical methods in clinical trials. Dr. Hamasaki served as Editor-in-Chief of Statistics in Biopharmaceutical Research, an Official Publication of the American Statistical Association (ASA), from 2000 to 2025. He was also a member of the Steering Committee for the Adaptive designs CONSORT Extension Project, an initiative to extend the CONSORT Statement for adaptive clinical trials, and participated in the ICH-E5 Guideline Implementation Working Group as a representative of the Japan Pharmaceutical Manufacturers Association. Dr. Hamasaki is an elected member of the International Statistical Institute and a Fellow of the ASA and the Society for Clinical Trials.

Philip He, PhD, is a statistician at Daiichi Sankyo, Inc., with nearly two decades of experience in oncology drug development, spanning early-phase trials through regulatory approvals. He has led statistical teams in the successful development of multiple cancer therapies, including chemotherapy, tyrosine kinase inhibitors, monoclonal antibodies, and immunotherapies. Passionate about advancing oncology clinical development, Dr. He actively contributes to the scientific community through volunteer service in professional organizations, editorial and peer-review work, and conference organization. He has published extensively on topics such as adaptive designs, estimands, dose optimization, and Bayesian statistics. Dr. He currently serves as Head of Early Phase Statistics at Daiichi Sankyo and co-chairs the DahShu Innovative Design and Scientific Working Group (IDSWG) Oncology Team (https://oncologytrialdesign.org/), where he collaborates with industry peers to advance innovative trial designs. In recent work, the IDSWG Oncology Team contributed a book chapter on dose optimization in oncology.

Weili He, PhD, has over 25 years of experience working in the biopharmaceutical industry. She is currently a Distinguished Research Fellow and head of Medical Affairs and Health Technology Assessment (HTA) statistics at AbbVie. She has a PhD in Biostatistics. Weili's areas of expertise span across clinical trials, real-world studies and evidence generations, statistical methodologies in clinical trials, observational research, innovative adaptive designs, and benefit-risk assessment. She is the lead or co-author of more than 60 peer-reviewed publications in statistics or medical journals and lead editor of three books on adaptive design, benefit-risk assessment, and RWE, respectively. She is the co-founder and co-chair of the American Statistical Association (ASA) Biopharmaceutical Section (BIOP) Real-world Evidence Scientific Working Group (SWG) from 2018 to 2022. She is also the founder and co-chair of a newly formed ASA BIOP HTA SWG. Weili is the BIOP Chair-Elect, Chair, and Past Chair from 2020-2022. She is also an Associate Editor of Statistics in Biopharmaceutical Research since 2014, and an elected Fellow of ASA since 2018.

Joseph G. Ibrahim is an Alumni Distinguished Professor of Biostatistics at the University of North Carolina. Dr. Ibrahim's areas of research focus are Bayesian inference, missing data, meta-analysis, network meta-analysis, Cancer research, and clinical trials. With over 37 years of experience working in Bayesian methods, Dr. Ibrahim directs the UNC Laboratory for Innovative Clinical Trials. He is also the Director of Graduate Studies in UNC's Department of Biostatistics. He is an Elected Fellow of ASA, IMS, ISBA, ISI, and RSS. He has published over 390 research papers, mostly in the top statistical journals. Dr. Ibrahim was awarded the 2024 Samuel S. Wilks Award. He has co-authored two advanced graduate-level books on Bayesian survival analysis and Monte Carlo methods in Bayesian computation and he is the PI of several grants from the NIH for his research on Bayesian methods, missing data, and cancer research. Joseph C. Cappelleri earned his MS in statistics from the City

University of New York (Baruch College), PhD in psychometrics from Cornell University, and MPH in epidemiology from Harvard University. Dr. Cappelleri is an executive director of biostatistics and the head of Health Economics & Outcomes Research Statistics in the Statistical Research and Data Science Center at Pfizer Inc, where he is the recipient of the Craig A. Saxton Clinical Development Excellence Award (Pfizer's highest accolade). As an adjunct professor, he has served on the faculties at Brown University (biostatistics), Tufts Medical Center (medicine), and the University of Connecticut (statistics). Among the most published authors and most prolific medical researchers in the history of Pfizer and the pharmaceutical industry, Dr. Cappelleri has co-authored hundreds and hundreds of publications (and external presentations) on clinical and methodological topics, including regression-discontinuity designs, meta-analysis, and health measurement scales. He is the lead author of the book "Patient-Reported Outcomes: Measurement, Implementation and Interpretation" and has co-authored or co-edited five other books. Dr. Cappelleri is an elected Fellow of the American Statistical Association (ASA), elected recipient of the Long-Term Excellence Award from the Health Policy Statistics Section of the ASA, and elected recipient of the Avedis Donabedian Outcomes Research Lifetime Achievement Award from ISPOR -- The Professional Society for Health Economics and Outcomes Research.

Christopher Jennison is Professor of Statistics at the University of Bath, UK. He was awarded his PhD from Cornell University for research into the sequential analysis of clinical trials and has continued to work in this area for the past 25 years. He has published extensively on group sequential methods and adaptive designs. His book with Professor Bruce Turnbull, "Group Sequential Methods with Applications to Clinical Trials", is a standard text on this topic and is widely used by practising statisticians. Professor Jennison's research is informed by experience of clinical trial analysis at the Dana Farber Cancer Institute, Boston and a broad range of consultancy with Medical Research institutes and pharmaceutical companies in Europe, America and Asia. He has made numerous presentations at international conferences, in which he sets out to describe novel statistical methodology and its application to the design and analysis of clinical trials.

Mary Jo Lamberti, PhD is a Research Associate Professor at Tufts University School of Medicine and the Director of Sponsored Research at Tufts CSDD. She has extensive experience benchmarking drug development and operating practices. Her research focuses on a variety of areas including patient recruitment and retention in clinical trials, clinical research workforce issues, outsourcing and partnerships, and investigative site initiation and management. In addition, she has examined the impact of technologies and digital solutions in clinical trials. Dr. Lamberti has been a frequent speaker at global industry conferences and has published extensively. She holds a B.A. from Wellesley College and a Ph.D. from Boston University.

Jerry Li, PhD, is a Director and TA Lead in Oncological Biostatistics at Global Biostatistics and Data Sciences (GBDS), BMS. Jerry leads statistical support for the clinical development of multiple assets including BMS/BioNTech strategically partnered anti-PD1 and VEGF program for clinical trial designs including phase2/3 seamless design, interactions with worldwide health authorities, and life cycle management of the assets. Jerry established and co-led the Dynamic Borrowing Working Group at BMS and co-authored the BMS internal guidance on this topic. Jerry also co-founded and co-chaired the ASA BIOP Dynamic Borrowing Scientific Working Group consisting of over two dozen members from health authority, academia and biopharmaceutical industry. Prior to BMS, Jerry was at Merck and Daiichi Sankyo following working at the FDA. He has held positions with increasing responsibilities in multiple therapeutic areas including oncology, neurosciences, immunology, and infectious disease and demonstrated a track record of successful regulatory approvals. In addition to dynamic borrowing, Jerry is also interested in dose optimization, phase 2/3 seamless design, statistical modeling of disease-modifying treatment effect, and properties of log-rank test following covariate-adaptive randomization in oncology trials. Jerry received his Ph.D. in statistics from the University of Maryland, College Park, and also holds an advanced degree in biomedical sciences.

Wayne Nelson is a leading expert on analysis of reliability and accelerated test data. He consults on applications, gives training courses, and works as an expert witness. For 24 years he consulted across the General Electric Co. and received the Dushman Award of GE Corp. R&D for developments and applications of product reliability data analysis. He was elected a Fellow of the Amer. Statistical Assoc. (1973), the Amer. Soc. for Quality (1983), and the Inst. of Electrical and Electronics Engineers (1988) for his innovative statistical developments for reliability data modeling and analysis. He was awarded the 2005 Lifetime Achievement Award of the IEEE Reliability Society and the 2003 Shewhart and 2010 Shainin Medals of ASQ for his developments of reliability methodology and contributions to reliability education, and the 2018 Hahn Award of ASA for outstanding service to clients. He has received nine Outstanding Presentation Awards from ASA. He authored three highly regarded books *Applied Life Data Analysis* (Wiley 2004), *Accelerated Testing* (Wiley 2004), *Recurrent Events Data Analysis* (SIAM 2003), two ASQ booklets, and 140 literature publications.

Inna Perevozskaya, Ph.D. is currently Head of Statistical Methdology and Innovation at BMS. The mission of the group is to provide strategic and methodological support to cross-functional trial design teams across the portfolio of all BMS medicines. Inna has been a core member of Adaptive Design WG since its inception in 2006. for the latter, she co-led a sub-team dedicated to simulation best practices across industry, which won Statistics in Biopharmaceutical Research best paper award. She is also an ASA Fellow, Associate Editor for Statistics in Biopharmaceutical Research, and has been elected Program Chair for the Executive Committee of the ASA Biopharmaceutical Section. Prior to joining BMS, Inna held positions of increasing responsibility and leadership within Merck, Wyeth, Pfizer and GSK. She holds an MS in Mathematics degree from Moscow State University and a PhD in Statistics from the University of Maryland, where she specialized in novel dose-escalation designs for oncology. Her research/consulting experience has resulted in 30+ publications in peer reviewed journals and several awards.

Naitee Ting is a Fellow of American Statistical Association (ASA). He is currently Vice President of Veramed. Naitee is also an Adjunct Professor of Department of Statistics at University of Connecticut, Adjunct Professor of Department of Biostatistics at Columbia University. He joined Veramed in October, 2024. Before Veramed, Naitee has been with Boehringer Ingelheim Pharmaceuticals, Inc. (BI) for 15 years, and he was working at Pfizer Inc. for 22 years (1987-2009). Naitee received his Ph.D. in 1987 from Colorado State University (major in Statistics). He has an M.S. degree from Mississippi State University (1979, Statistics) and a B.S. degree from College of Chinese Culture (1976, Forestry) at Taipei, Taiwan. Naitee published articles in Technometrics, Statistics in Medicine, Drug Information Journal, Journal of Statistical Planning and Inference, Journal of Biopharmaceutical Statistics, Biometrical Journal, Statistics and Probability Letters, and Journal of Statistical Computation and Simulation. His book "Dose Finding in Drug Development" was published in 2006 by Springer, and is considered as the leading reference in the field of dose response clinical trials. The book "Fundamental Concepts for New Clinical Trialists", co-authored with Scott Evans, was published by CRC in 2015. Another book "Phase II Clinical Development of New Drugs", co-authored with Chen, Ho, and Cappelleri was published in 2017 (Springer). Naitee is an adjunct professor of Columbia University, University of Connecticut, and Colorado State University. Naitee has been an active member of both the ASA and the International Chinese Statistical Association (ICSA).

Bruce Turnbull received the B.S. from Cambridge University in 1967 and the Ph.D. from Cornell University in 1971. After serving on the faculty at Stanford University and at the University of Oxford, he joined Cornell University in 1976, where he is currently Professor in the School of Operations Research and Information Engineering where he previously served as Acting Director. From 2000--2002, he was founding Chairman of the newly formed Department of Statistical Science and currently also holds a professorial appointment there. In 1979 he was awarded the Snedecor Memorial Award by the American Statistical Association in recognition of his research. He has authored over 130 publications and is the co-author of a book on statistical procedures for monitoring clinical trials. He has been a consultant to many organizations, including the Oak Ridge National Laboratory, Institute for Energy Analysis; and various pharmaceutical companies. Turnbull has served on the Board of Directors of the National Institute of Statistical Sciences, and on the Expert Review Panel for the National Toxicology Program Board of Scientific Counselors. He is on the Data and Safety Monitoring Committees for several major national and international clinical trials in the areas of cancer, heart disease, pulmonary disease and AIDS sponsored by the National Institutes of Health and by the Veterans Administration. He has served on the editorial board of a number of statistical journals and is currently editor of the Chapman and Hall book series on biostatistics. He is a Fellow of the American Statistical Association and of the Royal Statistical Society. Professor Turnbull also has emeritus status in the School of Operations Research and Information Engineering in Cornell's College of Engineering.

Mark van der Laan is the Jian-Ping Hsu/Karl E. Peace Professor of Biostatistics and Statistics at the University of California, Berkeley. He has made contributions to survival analysis, semiparametric statistics, multiple testing, and causal inference. He also developed the targeted maximum likelihood methodology and general theory for super-learning. He is a founding editor of the Journal of Causal Inference and International Journal of Biostatistics. He has authored 4 books on targeted learning, censored data and multiple testing, authored over 300 publications, and graduated 45 Ph.D. students. He received his Ph.D. from Utrecht University in 1993 with a dissertation titled "Efficient and Inefficient Estimation in Semiparametric Models". He received the COPSS Presidents' Award in 2005, the Mortimer Spiegelman Award in 2004, and the van Dantzig Award in 2005.

Li Wang, PhD, is currently Senior Director and Head of Statistical Innovation group in AbbVie. Li is leading Design Advisory which provides strategic and quantitative consulting as requested to all Development teams in all Therapeutic Areas to facilitate innovative thinking and complex innovative design evaluation. Li also leads Clinical Trial Innovation capability in AbbVie to drive Machine Learning and Advanced Analytics research and application in Development. Prior to this senior leadership role, he led Immunology and Solid Tumor statistical design and strategy discussions and multiple ML, RWE and Bayesian innovation projects from 2017 to 2019. From 2006 to 2017, he contributed to and subsequently led several NDAs and SNDAs including blockbusters Eliquis, Onglyza and Rinvoq. He is enthusiastic in teaching statistical courses to non-statisticians and investigating/promoting novel statistical and machine learning methodologies.

Jerry Weaver is currently the Global Statistics Therapeutic Head of Immunology and Head of Non-clinical Statistics at Teva. He has over 30 years of industry experience and has worked at well-established pharmaceutical companies that include Bristol-Myers Squibb, Celgene, Novartis, and Pfizer. His leadership and experience in clinical development spans the therapeutic areas of immunology, fibrosis, oncology, neuroscience, cardiovascular, and anti-infectives; with medical affairs/ market access support in hematology/ oncology and immunology. As a leader in clinical statistics, he has provided statistical strategies on clinical development programs, helped design a multitude of phase 1-4 clinical trials, interacted with global regulatory agencies, participated in advisory committee meetings, supported labeling negotiations, and partnered with key opinion leaders in statistics. Jerry has a track record of six successful global submissions, three of which were under his oversight as a therapeutic area head at Celgene and Bristol-Myers Squibb. Jerry received his graduate degree in statistics from the University of Iowa in which he studied under his advisor Robert V. Hogg. His interests include design of experiments, dose response modeling, quantitative go/ no go decision making, and Bayesian methods. Ying Yuan is the Bettyann Asche Murray Distinguished Professor and Chair of the Department of Biostatistics at the University of Texas MD Anderson Cancer Center. Dr. Yuan is internationally renowned for his pioneering research in innovative Bayesian adaptive designs, including early-phase trials, seamless trials, biomarker-guided trials, and basket and platform trials. The designs and software developed by Dr. Yuan's lab (www.trialdesign.org) have been widely adopted by medical research institutes and pharmaceutical companies. Among these, the BOIN design, developed by Dr. Yuan's team, is a groundbreaking oncology dose-finding method recognized by the FDA as a fit-for-purpose drug development tool. The BOP2 design is also widely used for Phase II trials and toxicity monitoring. Dr. Yuan is a member of the FDA Advisory Committee for Genetic Metabolic Diseases, an elected Fellow of the American Statistical Association, and the lead author of two books: *Bayesian Designs for Phase I-II Clinical Trials* and *Model-Assisted Bayesian Designs for Dose Finding and Optimization*, both published by Chapman & Hall/CRC.

Yunzhao Xing, PhD, is the Associate Director of Statistical Innovation at AbbVie, holding a PhD in Material Science from the University of North Carolina at Chapel Hill and a background in Physics. Prior to AbbVie, he served as a senior scientist at Halliburton, focusing on sensor modeling and simulation. Since joining AbbVie in 2018, Yunzhao has led numerous successful projects in machine learning, deep learning, and image processing. His skill set encompasses web scraping, simulation modeling, and interactive web application development, making him a pivotal contributor to AbbVie's Statistical Innovation Group. Yunzhao is recognized for his commitment to pushing the boundaries of statistical innovation.

Xiang Zhang, PhD, is the Head of Medical Affairs and HTA Statistics at CSL Behring, where he leads a team of statisticians focused on generating evidence to support product launches and commercialization efforts, including HTA submissions and non-interventional studies. He also co-leads the Forum for Observational Research Excellence at CSL Behring, which offers expertise to internal stakeholders on leveraging real-world data (RWD) and real-world evidence (RWE) for clinical development, regulatory submissions, and product commercialization. Dr. Zhang's research interests center on the development and application of methodologies for real-world data analysis. He has authored or co-authored over 40 peer-reviewed publications and a book titled "Real World Health Care Data Analysis: Causal Methods and Implementation Using SAS." He holds a Ph.D. in Statistics from the University of Kentucky.

Sheng Zhong, PhD, is the Director of Statistics at AbbVie Inc. He received his Ph.D. in Statistics from the University of Chicago. At AbbVie, he led multiple innovative predictive modeling projects across different fields such as clinical trial enrollment duration forecasting, virtual controls based on targeted learning in single-arm trials, and predictive clinical safety monitoring based on structured and text data. His recent works have led to multiple publications and manuscripts under review. Before joining AbbVie in 2016, Dr. Zhong worked at a big data analytics start-up for heavy machine equipment maintenance, where his work led to 3 US patents.

Monday December 8, 2025, 9:00 – 12:00 PM

Section A

Selected Topics of Statistics in Clinical Drug Development: A Statistical and Regulatory Perspective

Naitee Ting , Veramed

Moderator: Din Chen

Abstract

Clinical drug development is a complex process that involves years of investment and high failure rate. There are many areas that statistics can contribute to improve the efficiency of drug development, ranging from early phase dose finding to confirmatory trial designs. Novel statistical approaches have been implemented in recent years to improve this process. There have been notable shifts in the regulatory landscape supporting these approaches, such as the FDA complex innovative design (CID), model informed drug development (MIDD) programs. In this short course, we will provide a broad perspective of how such statistical innovations contribute to clinical drug development. We will discuss relevant regulatory guidance. Topics to be covered include early phase dose finding, dose ranging, confirmatory adaptive designs, multiplicity, covariate adjustment, Go/NoGo decision making, Bayesian borrowing, estimand and missing data. We will provide examples from recent trials and drug reviews/approvals.

Session B

The DOOR is Open: Patient-centric, Pragmatic Benefit-Risk Evaluation in Clinical Trials

Scott Evans and Toshimitsu Hamasaki, George Washington *University*

Moderator: Alfred H Balch

Abstract:

Randomized clinical trials (RCTs) are the gold standard for evaluating the benefits and harms of interventions. However commonly used design and analysis approaches to RCTs often are not suited to answering the most important research questions to inform medical decision-making. Typically, efficacy and safety are evaluated in silos, one outcome at a time. However this approach: fails to incorporate associations between or the cumulative nature of multiple outcomes in individual patients, suffers from competing risk complexities during interpretation of individual outcomes, fails to recognize important gradations of patient responses, suboptimal evaluates treatment effect heterogeneity based on a single endpoint rather than benefit: risk considerations, and since efficacy and safety analyses are often conducted on different populations, generalizability is unclear. The Council for International Organizations of Medical Sciences (CIOMS) released "Benefit-risk balance for medicinal products" in 2025. Included within the report are two new points of emphasis: (1) transitioning benefit-risk evaluation as a post-hoc exercise to incorporating benefit-risk considerations into clinical trial design, and (2) a pragmatic patient-centric approach to benefit-risk assessment reflecting how benefits and harms are experienced by patients to better align with the goals of informing clinical practice. The desirability of outcome ranking (DOOR) is a paradigm for the design, analysis, and interpretation of clinical trials and other research studies based on patient-centric benefit-risk evaluation, developed to address these issues and advance clinical trial science. In this paradigm outcomes are used to analyze patients rather than patients being used to analyze outcomes. The experiences of trial participants in different treatment arms are compared by the desirability of the overall patient outcome, increasing pragmatism and addressing the most important "real world" question to aid clinical decision-making: how do resulting patient experiences, when comprehensively considering benefits and harms, compare between therapeutic alternatives? We describe the DOOR paradigm; define guiding principles to maximize replicability, robustness, objectivity, transparency, and pragmatism; outline a recommended statistical analysis plan; illustrate application with examples; and demo a freely available online application for implementing the recommended DOOR analyses and the design of studies implementing DOOR.

Monday Lunch (On Your Own) 12:00 PM - 2:00 PM

2:00 - 5:00 PM

Session C ♣

Analysis of Recurrent Events Data in Medicine, Engineering, Marketing, and Other Fields

Dr. Wayne B. Nelson, Wayne Nelson Statistical Consulting and Training

Moderator: Din Chen

Abstract

Recurrent events data observed over time on sample units include the number and costs of recurrent disease episodes in patients, repairs of products, sales to customers on Amazon.com, and births of babies to statisticians. Analysis of such data requires special statistical models and methods not covered in basic courses. This tutorial presents a general simple and informative nonparametric model and plot for analyzing such recurrence data on numbers or costs of recurrences. The plots and analyses are applied to data on numbers and costs of bladder tumor and herpes recurrences, car transmission repairs, childbirths to statisticians, sales resulting from promotions on amazon.com, and other applications. Computer programs that calculate and make the plots with confidence limits are surveyed.

The plots provide:

1. An estimate of the average number or cost of recurrences per sample unit during a period of interest. Examples include the number and treatment cost of disease recurrences, product repairs during warranty or design life, and profit on Amazon.com sales promotions.
2. The behavior of the population recurrence rate – does it increase or decrease with population age? This information is useful for decisions on product burn-in, overhaul, and retirement and on planning patient treatments.
3. Predictions of the future number or cost of recurrences for a unit or the population. This is useful for predicting warranty costs of products and the demand for their replacement parts and for predicting patient treatment costs for recurrent diseases.
4. A comparison of data sets from different populations; this is used to decide which disease treatments and product designs, materials, treatments, environments, etc., produce lower recurrence rates, and which amazon.com promotions yield more sales.
5. Unsought, useful information.

Session D ♣

Introduction to Quantitative Decision Making in Drug Development

Jerry Weaver, Teva and Joe Ibrahim, UNC

Moderator: Naitee Ting

Ab**stract**

Making decisions within drug development programs requires careful consideration of emerging data as well as leveraging established scientific beliefs such that the chances of success can be optimized while ensuring responsible stewardship of the research and development organization (i.e., how to best allocate limited capital). This gives rise to an area of statistical sciences which we refer to as Quantitative Decision Making (QDM). In essence, it can be conducted at various stages of drug development which impact study level decisions (such as interim analyses for stopping the trial for futility or making mid-stage design adaptations), project level decisions (such as interim analyses at the study level that results in launching another study thus accelerating the development program), or even portfolio level decisions (such as choosing or prioritizing a pipeline strategy with respect to optimizing net present value) by providing probabilities of success to help guide the decision making process. One critical aspect of implementing effective QDM is establishing clear decision criteria, aligned with the development strategy, prior to the incorporation of emerging data. Another aspect includes understanding and applying appropriate statistical methods which typically have a Bayesian element.

In this session the following topics will be presented:

- Target product profile and the QDM decision criteria framework
- Communication and presentation of go/ no go operating characteristics in QDM
- Conditional and unconditional probabilities as illustrated with power versus expected power (i.e., assurance)
- Bayesian philosophy and principles
- Constructing prior distributions either from study data or through the elicitation of expert opinion
- Implementation of Bayesian models
- Examples on various endpoint types (binary, continuous, counts, time-to-event)
- Monitoring a trial at multiple interims for futility
- Mid-trial design adaptations given interim results
- Development program acceleration given interim results
- Probability of pharmacological success at the end of phase 1 for dose selection in phase 2 and 3
- Probability of success in phase 3 for an in-licensing opportunity

Speaker's Dinner (Optional Added Fee Event) Monday 7PM

Tuesday December 3, 2025, 9:00 – 12:00 PM

Session E ♣
Advances in Interpretation of Patient-Reported Outcomes

Joseph C. Cappelleri, Pfizer

Moderator: Wenjin Wang

Abstract
This presentation provides a review mainly on two broad approaches – anchor-based and distributed-based – aimed at enriching the understanding and meaning of patient-reported outcome scores. Anchor-based approaches use a measure (external to the targeted patient-reported outcome of interest) that is well interpretable and correlated with the targeted patient-reported outcome. Examples include percentages based on thresholds, cutoff scores based on severity, criterion-group interpretation, statistical significance and clinical significance, content-based interpretation, and clinically meaningful change and difference. Distributed-based approaches rely strictly on the distribution of data. Examples include effect size, probability of relative benefit, and cumulative distribution functions. A third approach on interpretation – mediation analysis – is also highlighted and illustrated to assess the effect of treatment on an outcome variable (which may or may not be a patient-reported outcome) indirectly through the effect of a mediator variable (which may or may not be a patient-reported outcome) and otherwise directly through other effects. Applications are based on real-life and simulated examples.

Session F ♣
Targeted machine learning for reliable evidence generation from clinical studies

Mark van der Laan, UC Berkeley and Susan Gruber, TL Revolution

Moderator: Weili He

Abstract
Targeted Learning (TL) unifies causal inference, machine learning (ML) and statistical theory to
provide a framework for evaluating causal effects from clinical studies. Randomized controlled trials (RCTs) and real-world data (RWD) studies require adequate power, unbiased estimation, adequate control of the Type-I error rate, and good confidence interval coverage. TL provides a causal estimation roadmap that guides the design, analysis, and interpretation of clinical studies to address these challenges. Advances in ML play a crucial role in developing actionable evidence to support decision making.

The first half of the tutorial will use case studies to familiarize attendees with the TL causal estimation roadmap and practical application of targeted maximum likelihood estimation (TMLE) and super learning (SL), a general, flexible approach to ML.

The second half of the course covers three recent developments in targeted machine learning. The highly adaptive lasso (HAL) is the first general nonparametric MLE, offering superior rates of convergence and efficiency without traditional constraints of parametric modeling. The Adaptive (A)-TMLE is an estimator that can optimally combine RCT and external data. Unlike other data fusion methods, A-TMLE is guaranteed to be as efficient as an efficient estimator of the RCT data alone. The final innovation concerns deep learning for solving complex estimation problems concerning the effects of static and dynamic treatment strategies over time, adjusting for time dependent confounders.

Tuesday Lunch (On Your Own) 12:00 AM - 2:00 PM

2:00 - 5:00 PM

Session G ♣
Unleashing the Power of Machine Learning and Deep Learning to Accelerate Clinical Development

Li Wang, Yunzhao Xing, and Sheng Zhong, AbbVie
Moderator: Jingjing Ye

Abstract
With the rapid advancement of machine learning (ML) and deep learning (DL) methodology in the last decade, the performances of prediction tasks in many computer science fields (e.g., imaging processing, natural language processing) have been greatly improved especially thanks to generative AI and transformers. There are many applications of ML and DL in drug discovery side. However, the impact of ML/DL in the field of clinical development has been relatively limited. Hence, we would like to propose a short course to educate, motivate and encourage the use of ML/DL especially in clinical development. Since we are statisticians by training, we would like to provide statistical perspectives on ML/DL as well.

The course starts with an overview of ML/DL methodology evolution over time and the related key concepts (e.g., back-propagation, hyperparameter tuning, train-test split, key performance metrics including AUC, precision, recall, F1 etc.). Then we will talk about the close relationship between ML and statistics. We will then introduce the latest developments in image processing and natural language processing, together with their novel applications in clinical development from our recent real projects and submitted papers to illustrate the vast potential of ML/DL in clinical development.

In terms of the course outline, the materials of the course are divided into three sections:

1. General ML/DL methodology and relationship between ML and statistics
2. Image processing and applications: deep convolutional neural networks (DCNN), object detection and segmentation, Region-based CNN (R-CNN), YOLO, and applications (e.g. psoriasis area and severity prediction)
3. Natural language processing and applications: word embeddings (word2vec), recurrent neural networks and language models, self-attention and transformers, pre-train and fine-tune paradigm and applications (e.g., adverse drug event prediction)

Session H ♣
Recent Developments and Innovations in Health Technology Assessment

Weili He, AbbVie and Xiang Zhang, CSL Behring
Moderator: Bill Wang

Abstract
Health Technology Assessment (HTA) is crucial for evaluating clinical and economic (and sometimes humanistic) values of new healthcare technologies to the related healthcare system, including medications, devices, and prevention strategies. It provides national health authorities with evidence-based information for informed decisions on reimbursement and pricing relative to other marketed therapies. Despite varying country requirements, HTA's core principles consistently highlight the importance of statisticians' involvement. To address this need, the ASA Biopharmaceutical Section's HTA Scientific Working Group (SWG) conducted a global assessment of the HTA landscape. In this session, we present insights from Phase I of the HTA SWG's work. We will outline the HTA framework, explore its history, examine requirements of different HTA bodies, discuss consolidation of PICOs (Population, Intervention, Comparator, Outcome) under new EU HTA regulations, and highlight challenges and opportunities in HTA submissions, focusing on statisticians' leadership roles in addressing these challenges and utilizing those opportunities. The tutorial's second part will discuss early integration of HTA/payer needs in trial design to enhance the ability to generate fit-for-purpose evidence by identifying and addressing evidence gaps during study design, along with HTA methodologies for evaluating clinical effectiveness, cost-effectiveness, and quality of life of new health technologies.

Wednesday December 4, 2025, 9:00 – 12:00 PM

Session I ♣
ICH E20 Adaptive designs for clinical trials - Scientific guideline

Christopher Jennison, University of Bath, Vladimir Dragalin, Johnson & Johnson, and Bruce Turnbull, Cornell University

Moderator: Bill Wang

Abstract
The Draft ICH E20 Guideline lays down general principles for the conduct of adaptive designs. The scope of this Guideline is extensive, covering group sequential tests, adaptation using combination tests or the conditional error principle, trials that test multiple hypotheses, and adaptive designs that combine all these features.

In this tutorial, we shall start by reviewing the methods used to construct adaptive designs and giving examples of their application. We shall explain the five principles put forward in the Guideline and discuss their implications. We shall present topics that emerge from this discussion: the logistical challenges in implementing an adaptive design; the requirement for a trial to contribute to the wider drug development program, going beyond testing the null hypothesis of no treatment effect; implications of the Guidelines for the use of Bayesian methods; methods for producing unbiased estimates and computing confidence intervals on termination of an adaptive trial.

The session will conclude with a question-and-answer session.

Session J ♣
Application of Data Borrowing in Clinical Trials and Regulatory Submissions

Jerry Li, Inna Perevozskaya and Ivan F Chan, BMS

Moderator: Alfred H Balch

Abstract
Data borrowing can bring significant benefits to expedite drug development bringing life-saving drugs to patients. Specifically, data borrowing can overcome challenges when patients are difficult to enroll, reduce the size/duration/risk of a new trial ensuring adequate power, have great operational and cost saving benefits, and boost the power and improve the efficiency of analysis for a trial with a limited sample size.
This tutorial will introduce the approaches of both frequentist and Bayesian borrowing. Frequent methods including propensity-score matching or weighting will be covered. Various Bayesian methods, including the power prior, commensurate prior, meta-analytic predictive (MAP) prior, robust MAP prior (RMAP) and self-adaptive mixture (SAM) prior, will be elaborated. Case studies and the regulatory landscape will also be introduced.

Wednesday Lunch (On Your Own) 12:00 AM – 2:00 PM

2:00 - 5:00 PM

Session K
Statistical Designs and Considerations for Dose Optimization

Ying Yuan, MD Anderson and Philip He, Daiichi Sankyo
Moderator: Kaylan Ghosh

Abstract
The U.S. FDA launched Project Optimus to reform dose-finding practices by shifting the focus from identifying the maximum tolerated dose (MTD) to determining the optimal biological dose (OBD) that offers the best risk-benefit profile. In this talk, I will provide an overview of strategies for dose optimization, including efficacy-integrated and two-stage approaches, illustrated with real-world examples. I will also discuss recent advances in the design of randomized dose optimization trials and highlight the role of backfilling as a flexible and efficient tool in this process. Additionally, I will address the complexities of dose optimization in the context of multiple indications, multiple agents, and combination therapies. Finally, I will introduce software tools that support the practical implementation of these methodologies.

Session L
Critical Thinking and Creative Analogies in Statistics, Science, and Technology: Essential Skills for the AI Era

Mark Chang, Boston University
Moderator: Ivan F. Chan

Abstract
In the AI era, questions often matter more than answers, adaptability trumps rigidity, and interconnections outweigh siloed expertise.

This book tutorial presents a stimulating blend of critical thinking and creative analogy to reframe complex scientific, technological, and statistical concepts. Merging established viewpoints with bold, unconventional insights, it offers participants a deeper and more intuitive understanding of modern challenges. Drawing from fields such as biostatistics, medicine, socioeconomics, and education, the tutorial demonstrates how abstract reasoning can illuminate real-world decisions.

Rather than focusing directly on AI tools, this course equips participants to thrive in an AI-driven world by cultivating the human strengths of reasoning and analogy. Through paradoxes and surprising case studies, we explore how deep thinking can generate promising research ideas and reveal hidden insights in decision-making, healthcare, and scientific discovery.

This tutorial is intended for strategic thinkers as well as practical statisticians and scientists who value critical thinking and analogical insight.

Topics to be Covered

- Digital Twins in Clinical Trials and Healthcare: Opportunities and Challenges
- Drug Repositioning: Revelation from the "Fair Game"
- Insights from Game Theory and Stochastic Decision Processes
- Identifying Bias through Critical Thinking
- Recursive Functions as Self-Referencing Analogies
- What Constitutes Scientific Evidence? – The Similarity Principle
- Analogy-Driven Humanized AI and Future Outlook

TWO SIMULTANEOUS SHORT COURSES THURSDAY AND FRIDAY, DECEMBER 11-12, 2025

Short Course Registration includes (1) a hot breakfast and two refreshment breaks each day; (2) handouts and (3) the textbook. No registrations will be accepted without payment in full. We will refund full tuition if courses are canceled due to insufficient registration.

Thursday Schedule 8:00⇒9:30 Lecture / **9:30⇒9:50** *Break* / **9:50⇒11:20 Lecture** / **11:20⇒12:40** *Lunch on Your Own* / **12:40⇒2:10 Lecture** / **2:10⇒2:30** *Break* / **2:30⇒4:00 Lecture** / **4:00⇒4:20** *Break* / **4:20⇒5:00 Lecture**

Friday Schedule 8:00 - 9:30 Lecture / **9:30 - 9:50** Break / **9:50 - 11:20 Lecture** / **11:20 - 11:40** *Break* / **11:40 - 1:10 Lecture**

Classification and Regression Trees by Example

Wei-Yin Loh, University of Wisconsin, Madison

Moderator: Din Chen

Abstract

Since the first regression tree algorithm was published in JASA more than 60 years ago (Morgan & Songuist, 1963), many newer algorithms have appeared, with significant improvements in capability, power, and speed. Theoretical foundations, such as asymptotic consistency, have also been established. As a result, modern tree algorithms are often the preferred "low-level learners" in ensemble methods such as forests and gradient boosting machines.

This course is a broad overview of the current state of the art for both newcomers and experienced users. No prior knowledge of the subject is required. Real datasets are used to motivate, illustrate, and compare algorithms (including CART, RPART, CTREE, randomForest, Ranger, Cforest, and GUIDE) on their strengths (prediction accuracy, variable selection, and importance ranking), weaknesses (over and underfitting, selection biases), and features (applicability to missing data, longitudinal, multivariate, and censored responses, subgroup identification, propensity score estimation, circular or periodic predictor variables, such as angles and time of day, and regression trees with linear splits and linear fits, and applications to explainable AI). There will be demos of free software, if time permits.

Wei-Yin Loh is Professor of Statistics at the University of Wisconsin, Madison. His research interests are in bootstrap theory and methodology and algorithms for classification and regression trees. Loh is a fellow of the American Statistical Association and the Institute of Mathematical Statistics, and a consultant to government and industry. He is a recipient of the Reynolds Award for teaching, the U.S. Army Wilks Award for statistics research and application, an Outstanding Science Alumni Award from the National University of Singapore, and visiting fellowships from AbbVie, IBM and the Bureau of Labor Statistics.

Group Sequential and Adaptive Methods for Clinical Trials 🕮

Christopher Jennison, University of Bath and Bruce Turnbull, Cornell University

Moderator: Bill Wang

Abstract

In the first part of this course we shall review group sequential designs, in particular, error spending tests that can handle unpredictable levels of information at each analysis. We shall discuss more recent developments in: accommodating delayed observations ("pipeline" data) at interim analyses; trials with multiple arms or multiple endpoints; controlling bias when reporting results of a trial with early stopping. Statistical software will be used to illustrate the methods and examples.

The course will then move on to adaptive designs based on combination tests or the conditional error principle and combining these methods with closed testing procedures to create adaptive designs that test multiple hypotheses while controlling the family-wise error rate. We shall discuss applications including sample size re-estimation, Phase II/III seamless designs, multi-arm multi-stage designs, and enrichment designs. Adaptations may follow rigid rules that are pre-specified in the protocol, or a more flexible approach may be followed with the possibility of unplanned changes at interim analyses. The complexity and flexibility of an adaptive design has implications for the inferences that can be draw on its conclusion: we shall explain what is achievable using current methods and note areas where further work is needed.

Participants will be invited to discuss their experiences in implementing adaptive designs, interacting with regulators, or serving on Data and Safety Monitoring Boards.

Christopher Jennison is Professor of Statistics at the University of Bath, UK. He was awarded his PhD from Cornell University for research into the sequential analysis of clinical trials and has continued to work in this area for the past 25 years. He has published extensively on group sequential methods and adaptive designs. His book with Professor Bruce Turnbull, "Group Sequential Methods with Applications to Clinical Trials", is a standard text on this topic and is widely used by practicing statisticians. Professor Jennison's research is informed by experience of clinical trial analysis at the Dana Farber Cancer Institute, Boston and a broad range of consultancy with Medical Research institutes and pharmaceutical companies in Europe, America and Asia. He has made numerous presentations at international conferences, in which he sets out to describe novel statistical methodology and its application to the design and analysis of clinical trials.

Bruce Turnbull received the B.S. from Cambridge University in 1967 and the Ph.D. from Cornell University in 1971. After serving on the faculty at Stanford University and at the University of Oxford, he joined Cornell University in 1976, where he is currently Professor in the School of Operations Research and Information Engineering where he previously served as Acting Director. From 2000--2002, he was founding Chairman of the newly formed Department of Statistical Science and currently also holds a professorial appointment there. In 1979 he was awarded the Snedecor Memorial Award by the American Statistical Association in recognition of his research. He has authored over 130 publications and is the co-author of a book on statistical procedures for monitoring clinical trials. He has been a consultant to many organizations, including the Oak Ridge National Laboratory, Institute for Energy Analysis; and various pharmaceutical companies. Turnbull has served on the Board of Directors of the National

		Institute of Statistical Sciences, and on the Expert Review Panel for the National Toxicology Program Board of Scientific Counselors. He is on the Data and Safety Monitoring Committees for several major national and international clinical trials in the areas of cancer, heart disease, pulmonary disease and of AIDS sponsored by the National Institutes of Health and by the Veterans Administration. He has served on the editorial board of a number of statistical journals and is currently editor of the Chapman and Hall book series on biostatistics. He is a Fellow of the American Statistical Association and of the Royal Statistical Society. Professor Turnbull also has emeritus status in the School of Operations Research and Information Engineering in Cornell's College of Engineering.

THREE KEYNOTES

(Monday, Tuesday, and Wednesday Morning, December 8-10, 2025)

Keynote 1:	*Keynote 2:*	*Keynote 3:*
Shaping the Future: Advancing the Value and Protecting the Integrity of Clinical Trials **Prof. Scott Evans** Moderator: Alfred H. Balch	**Tufts CSDD – DIA Survey Results on AI Adoption in Drug Development** **Prof. Mary Jo Lamberti** Moderator: Jingjing Ye	**Optimising Group Sequential and Adaptive Designs: Where Frequentist meets Bayes** **Prof. Christopher Jennison and Bruce Turnbull** Moderator: Ivan F Chan
Abstract Clinical trials are our most reliable tool for understanding the benefits and harms of interventions on patients, and provide the most dependable evidence to inform clinical practice. It is a time of great concern for losing the benefits that distinguish clinical trials as the pinnacle of evidence, yet also a time of great opportunity to improve their value. Let us harmonize the unwavering commitment for the foundational scientific principles and objectivity necessary for robust apex evidence, and adopt the spirit of thoughtful carpe diem. Ideas for advancing this vision are discussed. These include placing increased interest on research questions of a pragmatic origin to match their clinical importance, and adjusting our approaches to address these questions.	**Abstract** The Tufts Center for the Study of Drug Development (CSDD) and the Drug Information Association (DIA) collaborated on a research study with a working group of 16 biopharmaceutical and contract research organizations examining the current use of artificial intelligence and machine learning to support the continuum of drug development with a focus on clinical operations and development. The global survey findings will be discussed in detail in this presentation and participants will gain insight into the level of adoption of artificial intelligence across biopharmaceutical companies and CROs and learn about the top activities where AI/ML is being implemented and utilized. Participants will also learn about the effectiveness of the impact of AI/ ML including time savings, cost, and deployment of organizational resources. Last, the challenges to the implementation of AI/ML will be discussed as well as the opportunities for the pharmaceutical industry.	**Abstract** In designing a group sequential clinical trial to test the treatment effect on a primary endpoint, one may search for an optimal design subject to type I error and power requirements. We shall illustrate how the solution to this frequentist problem involves solving a Bayes sequential decision problem. Conversely, if one starts with a Bayesian formulation but calibrates the design to guarantee a type I error condition, the result should match an optimized frequentist procedure. Underlying this equivalence are theorems which prove that the class of admissible frequentist designs is the same as the class of Bayes optimal designs. From a practical perspective, this relationship is helpful in computing optimal designs. At a philosophical level, we see that, when implemented well, the two approaches to inference should produce the same results. A more complex trial can have multiple treatments, multiple endpoints, or subgroups of patients, so multiple hypotheses may be tested. Examples include seamless Phase 2-3 trials with treatment selection and adaptive enrichment trials. In such cases, control of the family-wise error rate is usually required. Although the relation between optimal frequentist and Bayesian designs is not so straightforward here, there are design strategies that combine the two approaches. One may start with a Bayesian design and calibrate features of this to achieve a frequentist error rate. Alternatively, one can define a class of frequentist designs that control the family-wise error rate and use Bayesian methods to optimize within this class.