Short Course of the 2025 Workshop on Biostatistics and Bioinformatics

Title: Bayesian Statistics, Designs, Sample Size Estimation, and Decision Making for Clinical Trials

Room 1441, 25 Park Place, Georgia State University, Atlanta, GA 30303

1:30pm-5:30pm, May 9 (Friday)

Yuan Ji, PhD, Professor of Biostatistics, The University of Chicago, 5841 S. Maryland Ave MC2000, Chicago, IL 60637

Abstract:

Bayesian statistics are based on two probabilities, one for data and the other for parameters, to explore their uncertainties. The probability model for data, i.e., the likelihood is common between Bayesian and frequentist, while the probability model for parameter, i.e., the prior is unique to Bayesian. Through Bayes' Theorem, one obtains the posterior probability that quantifies the uncertainty of the parameters given the observed data.

In this short course, I will explore the applications of Bayesian statistics in early- and late-phase clinical trials. For early-phase trials, I will focus on the FDA Project Optimus which has altered the paradigm of oncology dose selection by emphasizing the importance of finding an optimal dose with desirable efficacy and safety. I will provide a comprehensive review and introduction of practical statistical designs for dose optimization, including backfill, randomized comparison, different endpoints for safety and tolerability, and integration of

PK/PD data. For late-phase trials, I will discuss Bayesian models and inference that borrow information across different patient populations (e.g., adults vs pediatrics). More importantly, I will illustrate alternative quantification of statistical errors or substantial evidence, through which regulatory decision making may be founded on different and more practical metrics.

Through the learning provided by this short course, attendees will be exposed to the main ideas and motivations for key innovative statistical designs and strategies. The short course is constructed not long to teach "hows" but "whys", so that attendees will develop ability to assess the pros and cons of different designs for their individual needs in practice. The following topics will be covered with three sessions:

Session 1: Brief review of Bayesian statistics and modeling. This session will expose attendees to some basic concept and techniques of Bayesian statistics as many designs for early-phase oncology trials are Bayesian.

Session 2 Review of dose-optimization designs, including dose finding designs, cohort expansion methods, randomized dose comparison, and integration of PK/PD data.

Session 3: Review of existing and novel Bayesian sample size estimation, decision making, and error quantification in late-phase studies.

Throughout the short course, available software and tools will be illustrated for the course attendees. The short course is expected to run for 4 hours with two breaks.

About the instructor:

Dr. Yuan Ji is Professor of Biostatistics at The University of Chicago. His research focuses on innovative Bayesian statistical methods for translational cancer research. Dr. Ji is author of over 170 publications in peer-reviewed journals including across medical and statistical journals. He is the inventor of many innovative Bayesian adaptive designs such as the mTPI and i3+3 designs, which have been widely applied in dose-finding clinical trials worldwide. His work on cancer genomics has been reported by a large number of media outlets in 2015. He received Mitchell Prize in 2015 by the International Society for Bayesian Analysis. He is an elected fellow of the American Statistical Association, and the Chair of the Biopharm section of the International Socieity of Bayesian Analysis.