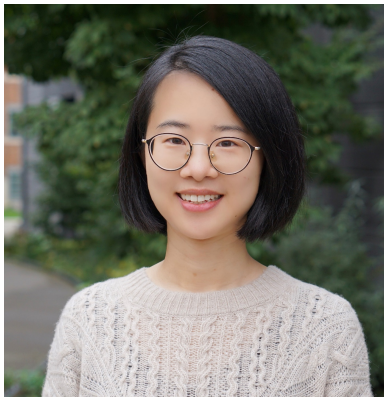
Department of Biostatistics and Medical Informatics Seminar



Ting Ye, PhD

Assistant Professor
Department of Biostatistics
University of Washington

Friday, October 25, 2024
12:00-1:00 pm
Biotechnology Center Auditorium
or via Zoom
https://uwmadison.zoom.us/j/97615509019

From Estimands to Robust Inference of Treatment Effect in Platform Trials

Abstract: A platform trial is an innovative clinical trial design that uses a master protocol (i.e., one overarching protocol) to evaluate multiple treatments in an ongoing manner and can accelerate the evaluation of new treatments. However, the flexibility that marks the potential of platform trials also creates inferential challenges. Two fundamental challenges are the precise definition of treatment effects and the robust and efficient inference on these effects. In this work, we make a key contribution by, for the first time, clearly stating how to construct a clinically meaningful estimand. This estimand characterizes the treatment effect as a contrast of the expected outcomes between two treatments in a population of concurrently eligible participants—the largest population that preserves the integrity of randomization. Then, we develop weighting and post-stratification methods for estimation of treatment effects with minimal assumptions. To fully leverage the efficiency potential of data from concurrently eligible participants, we also consider a model-assisted approach for baseline covariate adjustment to gain efficiency while maintaining robustness against model misspecification. We derive and compare asymptotic distributions of proposed estimators in theory and propose robust variance estimators. The proposed estimators are empirically evaluated in a simulation study and illustrated using the SIMPLIFY trial.

Bio: Dr. Ting Ye is an Assistant Professor in the Department of Biostatistics at the University of Washington. She completed her PhD in Statistics at University of Wisconsin-Madison in 2019 and was a postdoctoral researcher at the Department of Statistics, University of Pennsylvania. Her current research focuses on the design and analysis of complex innovative clinical trials and causal inference in biomedical big data.

School of Medicine and Public Health
UNIVERSITY OF WISCONSIN–MADISON