

“Design considerations for testing treatment effect heterogeneity in randomized trials with heterogeneous clustering”



Guangyu Tong PhD

Associate Research Scientist
Center for Methods in Implementation and
Prevention Science
Yale School of Public Health

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Via Zoom

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Abstract: An important consideration in the design and analysis of randomized trials in implementation science research is the need to account for correlated outcome observations within groups or clusters. Two typical designs with such consideration are cluster randomized trials and individually randomized group treatment trials. While sample size methods for testing the average treatment effect are available for both designs, methods for detecting treatment effect modification are relatively limited. In this talk, I introduce new sample size methodology for testing treatment effect modification based on either a univariate or multivariate effect modifier while accounting for differences across study arms in the outcome variance, outcome intracluster correlation coefficient (ICC) and cluster size. I will cover cases where the effect modifier is measured at either the individual level or cluster level. With a univariate effect modifier, the closed-form sample size expressions provide insights on optimal allocation of groups or clusters to maximize design efficiency. Overall, the results show that the required sample size for testing treatment effect heterogeneity with an individual-level effect modifier can be affected by unequal ICCs and variances between arms, and accounting for such between-arm heterogeneity leads to more efficient sample size determination. Simulations are carried out to validate the proposed sample size methods, and their applications are illustrated in the context of real trials. At the end of the talk, I will also describe emerging statistical problems arising from my implementation science and other research collaborations.