THE HONG KONG POLYTECHNIC UNIVERSITY
香港理工大學

DEPARTMENT OF APPLIED MATHEMATICS
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The Hong Kong Polytechnic University
Department of Applied Mathematics

Seminar

An External Control Trial Design and Highly Robust Causal Estimates of Treatment Effect

By

Prof Ming T. TAN
Georgetown University, Washington DC

Abstract

We propose a clinical design and analysis procedure for comparing a new therapy with an external control obtained using real-world data, including disease registry/electronic health records. The method is motivated by a clinical trial comparing the 3-year relapse-free survival (RFS) between locally treated high-risk ocular melanoma patients on adjuvant combination immunotherapy versus a matched contemporaneous control population, where a randomized control trial is not feasible. This talk will present several statistical challenges in the design and analysis of the trial, such as sample size determination for paired survival data and obtaining a robust causal estimate of treatment differences in this nonrandomized trial. Although the doubly robust estimate (DRE) is applicable and provides additional robustness by only requiring either the propensity score or the outcome regression model to be correctly specified, it is known that DRE may give estimates with significant bias and variance, even when the propensity and/or outcome models are mildly misspecified. Therefore, we introduce the highly robust estimate to obtain a causal estimate of the treatment effect, which is shown to have much-enhanced robustness against model specifications, and is generalizable to a variety of estimands of interest. This work is in collaboration with Yuan A, Yin A, Wu T, Rapisuwon S and Aitkins MB.

Biography

Prof Tan is a tenured professor of Biostatistics, Bioinformatics and Biomathematics at Georgetown University School of Medicine. He is the chairperson of the Department of Biostatistics, Bioinformatics and Biomathematics at Georgetown University Medical Center and its Lombardi Comprehensive Cancer Center.

Prof Tan's research covers the design, monitoring, and analysis of clinical trials (in both multi-center and single institutional settings), translational and epidemiological studies. His current research focuses on developing causal inference methods for nonrandomized studies, estimands in the presence of recurrent events, statistical methods for multidrug combinations utilizing experimental data and systems pharmacology, innovative adaptive designs and subgroup analysis in clinical trials, and machine learning predictive modeling, all funded by the NIH R01 and R21.

Prof Tan has served on multiple NIH study sections (such as Clinical Oncology and Biomarker), review and site visit panels (for P30, P50, and P01), Data and Safety Monitoring Boards for government, institution, and pharmaceutical sponsor trials, and has been a member of FDA Advisory Committee (recent term to 2018) and a statistical expert to pharmaceutical and investment companies. He is a Fellow of the American Statistical Association and an elected Member of the International Statistical Institute; and is an Executive Editor of Molecular Carcinogenesis, Associate Editor of Statistics in Medicine, Statistics in Bioscience. He has over 250 peer-reviewed publications split evenly between statistics/biostatistics and biomedical journals.

Date: **20 November 2023 (Monday)**
Time: **02:30-03:30 pm (Hong Kong Standard Time GMT +8)**
Venue: **TU107**
Speaker: **Prof Ming T. TAN, Georgetown University, Washington DC**
Host: **Prof Jian HUANG, The Hong Kong Polytechnic University**

*** * * ALL ARE WELCOME * * ***