



# The Hong Kong Polytechnic University Department of Applied Mathematics

## **Statistics and Data Science Online Colloquium Series**

Principal Stratification as a Tool for Estimating the Overall Average Treatment Effect in Randomized Clinical Trials with All-or-None Compliance

#### By

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### Abstract

Noncompliance is a common problem in randomized clinical trials that threatens to undermine the benefit of randomization. A popular approach to the noncompliance problem is principal stratification, which provides a simple method to estimate the complier-average treatment effect (CATE) under appropriate conditions. However, the CATE may be difficult to interpret in clinical practice. Here we propose to use principal stratification as a tool to estimate the overall average treatment effect (ATE) in clinical trials with all-or-none compliance. The use of principal stratification makes it unnecessary to assume that all confounders are measured, a key assumption for most existing methods that estimate the ATE through confounding adjustment. The proposed approach requires the availability of baseline covariates that are sufficient to explain any difference between the CATE and the ATE, so that the covariate-specific CATE and ATE may be assumed equal. Under this assumption, the overall ATE can be estimated by averaging an estimate of the covariate-specific CATE over observed covariate values. Estimation of the covariate-specific CATE can be carried out using several different methods based on different modeling assumptions. The different methods are compared in a simulation study and applied to the Multiple Risk Factor Intervention Trial.

## **Bibliography**

Dr. Zhiwei Zhang is Mathematical Statistician in the Biometric Research Program of the U.S. National Cancer Institute. He received his PhD in Biostatistics in 2003 from the University of Pittsburgh. His research interests include causal inference, precision medicine, and clinical trial design and analysis. Dr. Zhang has published over 100 peer-reviewed articles, mostly in statistical journals. He is an elected fellow of the American Statistical Association.

Date : 21 May 2021 (Friday) Time : 09:30-10:30 (Hong Kong Standard Time GMT +8) Venue : Online Talk via Zoom (Meeting ID: 977 3116 4557, Passcode: 0521) Speaker : Dr. Zhiwei Zhang, National Cancer Institute Host : Dr. Catherine Liu, The Hong Kong Polytechnic University Click to join : https://polyu.zoom.us/j/97731164557?pwd=WTB6NXR6SnpORVk0cllIMVR0Ui9HZz09

