University of California, Irvine
Distinguished Speaker
Statistics Seminar

Single Arm Trials with a Synthetic Control
Arm Built from RWD

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Join via Zoom: https://tinyurl.com/4azdp5zh

Randomized clinical trials (RCT) are the gold standard for approvals by regulatory agencies. However, RCT's are increasingly time consuming, expensive, and laborious with a multitude of bottlenecks involving volunteer recruitment, patient truancy, and adverse events. An alternative that fast tracks clinical trials without compromising quality of scientific results is desirable to more rapidly bring therapies to consumers. We propose a model-based approach using nonparametric Bayesian common atoms models for patient baseline covariates. This specific class of models has two critical advantages in this context: First, the models have full prior support, i.e., allow to approximate arbitrary distributions without unreasonable restrictions or shrinkage in specific parametric families; and second, inference naturally facilitates a reweighting scheme to achieve equivalent populations. We prove equivalence of the synthetic and other patient cohorts using an independent separate verification. Failure to classify a merged data set using a flexible statistical learning method such as random forests, support vector machines etc. proves equivalence. We implement the proposed approach in two motivating case studies.