ISBA Biostatistics and Pharmaceutical Section 2023 Paper Competition Award Winners Webinar

Monday, April 29 from 1pm – 3pm CST

Open Access Zoom Link:
https://us02web.zoom.us/j/86144726580?pwd=Wjg2SUpIcXlkb0UyS2hiNFpEMmsydz09

Junior Researcher Award

Bayesian Nonparametric Common Atoms Regression for Generating Synthetic Controls in Clinical Trials

By **Noirrit Chandra** (speaker), email noirrit.chandra@utdallas.edu
(co-authors: A. Sarkar, J. de Groot, Y. Yang & P. Muller)

ABSTRACT: The availability of electronic health records (EHR) has opened opportunities to supplement increasingly expensive and difficult to carry out randomized controlled trials (RCT) with evidence from readily available real world data. In this paper, we use EHR data to construct synthetic control arms for treatment-only single arm trials. We propose a novel nonparametric Bayesian common atoms mixture model that allows us to find equivalent population strata in the EHR and the treatment arm and then resample the EHR data to create equivalent patient populations under both the single arm trial and the resampled EHR. Resampling is implemented via a density-free importance sampling scheme. Using the synthetic control arm, inference for the treatment effect can then be carried out using any method available for RCTs. Alternatively the proposed nonparametric Bayesian model allows straightforward model-based inference. In simulation experiments, the proposed method exhibits higher power than alternative methods in detecting treatment effects, specifically for non-linear response functions. We apply the method to supplement single arm treatment-only glioblastoma studies with a synthetic control arm based on historical trials. https://doi.org/10.1080/01621459.2023.2231581

Best Paper Award

Nonparametric failure time: Time-to-event machine learning with heteroskedastic Bayesian additive regression trees and low information omnibus Dirichlet process mixtures

By **Rodney Sparapani** (speaker), email rsparapa@mcw.edu
(co-authors B. Logan, M. Maiers, P, Laud & E. McCulloch)

ABSTRACT: Many popular survival models rely on restrictive parametric, or semiparametric, assumptions that could provide erroneous predictions when the effects of covariates are complex. Modern advances in computational hardware have led to an increasing interest in flexible Bayesian nonparametric methods for time-to-event data such as Bayesian additive regression trees (BART). We propose a novel approach that we call nonparametric failure time (NFT) BART in order to increase the flexibility beyond accelerated failure time (AFT) and proportional hazard models. NFT BART has three key features: (1) a BART prior for the mean function of the event time logarithm; (2) a heteroskedastic BART prior to deduce a covariate-dependent variance function; and (3) a flexible nonparametric error distribution using Dirichlet process mixtures (DPM). Our proposed approach widens the scope of hazard shapes including nonproportional hazards, can be scaled up to large sample sizes, naturally provides estimates of uncertainty via the posterior and can be seamlessly employed for variable selection. We provide convenient, user-friendly, computer software that is freely available as a reference implementation. Simulations demonstrate that NFT BART maintains excellent performance for survival prediction especially when AFT assumptions are violated by heteroskedasticity. We illustrate the proposed approach on a study examining predictors for mortality risk in patients undergoing hematopoietic stem cell transplant (HSCT) for blood-borne cancer, where heteroskedasticity and nonproportional hazards are likely present. https://doi.org/10.1111/biom.13857