

From Joint Models to Making go/no-go decisions: a framework for inference and decision making in early clinical trials

Sunday, October 20, 2019

(1 PM to 5 PM)

In this workshop, Eric Novik and Stan (mc-stan.org) developers Daniel Lee and Krzysztof Sakrejda, will cover the modern analysis workflow including generative models, parameter inferences, prior and posterior predictive distributions, and utility functions in the context of joint time to event and biomarker models. We will cover the following topics.

- **Overview of the Bayesian workflow:** We will cover the Bayesian workflow starting with data simulations and ending with making decisions.
- **Discussion of joint models:** as implemented in the R package `rstanarm` starting from an introduction to the event and biomarker submodels and the rationale for their inclusion.
- **Intro to Data and EDA:** We will discuss the simulated data sets available for model exploration and share them with participants and introduce approaches to EDA particularly relevant to joint models
- **Model fitting and diagnostics:** Guided models fitting using the `stan_jm` function in the `rstanarm` package. We will focus on model diagnostics and goodness of fit/calibration in the joint model context.
- **Prediction and visualization:** Participants will be guided in creating predictions based on the joint model in different use cases and visualizing them for model exploration and communication.
- **Decision problem and framework:** Discussion of the decision theory framework as it applied to the pharmaceutical context and frame some standard questions asked of medical trials in terms of decision theory.
- **Decision calculations and summary:** Participants will be guided in constructing a utility function for the decision problem and completing the calculations required to arrive at a quantitative summary of the problem that summarizes their stated cost-benefit trade-offs.

Registration Fee: Industry \$400, Academia \$200, Trainee \$100