Adaptive Enrichment Designs for Confirmatory Randomized Trials:
Statistical Methods and Software Tools

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1:00pm – 4:00pm

Instructor:
Michael Rosenblum, PhD
Associate Professor of Biostatistics
Johns Hopkins Bloomberg School of Public Health
mrosen@jhu.edu

Background

Most randomized trials are designed to determine average treatment effects for a population. This results in trials that may fail to detect important differences in benefits and harms for subpopulations. For example, standard trial designs are not targeted to determine whether a treatment benefits most patients, benefits only a select few, or benefits some patients and harms others. The impact is that treatment recommendations based on the results of standard trial designs may be suboptimal, leading to poor patient outcomes and wasting healthcare resources. This problem affects virtually all disease areas, since it stems from how randomized trials, the gold standard for evaluating treatments, are currently designed and analyzed.

Randomized trial designs that adaptively change enrollment criteria during a trial, called adaptive enrichment designs, have potential to provide improved information about which subpopulations benefit from new treatments. These trial designs involve multiple populations of interest, e.g., defined in terms of a biomarker or risk score measured at baseline.

Course Description

This course presents an overview of the strengths and limitations of these designs, explains recent advances in statistical methods for these designs, and presents a software tool for optimizing these designs.

Two case studies are presented in the context of treatments for stroke and Alzheimer's disease.

Learning Objectives:

1. Understand benefits and limitations of adaptive enrichment designs
2. Learn about (and see demonstration of) a new user-friendly, free, open-source software tool that automatically searches over certain adaptive and non-adaptive trial designs to find the ones that best address a clinical investigator's scientific goals and resource constraints.

Target Audience

Anyone involved in or interested in the planning and/or analysis of randomized trials. Individuals from industry, government, and academia are all welcome.