

Biost / Stat 578
Special Topics: Statistical Design of Clinical Trials

Syllabus
Spring 2013

Course Overview: This course will address issues related to the statistical design of clinical trials, including such topics as

- selection of statistical estimand;
- randomization strategies, including dynamic balancing and response adaptive randomization;
- group sequential sampling rules, including both frequentist and Bayesian approaches;
- adaptive sampling rules, including response adaptive re-powering of RCT and enrichment; and
- approaches to addressing missing data in RCT.

Instructor : Scott S. Emerson, M.D., Ph.D., Professor of Biostatistics
Office : HSB F673
Phone : 616-6678 (Biostatistics)
Email : semerson@uw.edu
Office hours : (by appointment)

Time and Place : Lectures : MW 8:00a - 9:20a T663

Class Web Pages: <http://www.emersonstatistics.com/b578/>

Prerequisites : Biost 514 / 515, Stat 512 / 513, and Biost 524 (or equivalents)

Computing : Software : R (and RCTdesign package)

Attendance : Required

Assignments : Weekly assignments
One oral report on a special topic

Weekly homeworks (usually assigned on Wednesday and due on Sunday) will involve derivation or simulation of operating characteristics of particular RCT designs. Each student will also be required to present a 20 minute lecture on a special topic.

Grading : CR/NC based on homework's and presentation

Course Objectives

This course provides an overview of the statistical design of RCT. Thus the major objectives of this course are

1. To explore the impact of standard statistical theory on choice of RCT design.
2. To learn and understand the theory behind methodology that is more specific to the RCT setting, including adaptive randomization, sequential sampling, and approaches to handling missing data in RCT.
3. To be able to provide the important elements of the statistical design to grant applications, RCT protocols, statistical analysis plans, interim monitoring plans, clinical trial reports, and scientific publications and presentations.