BSTA 661 Design of Interventional Studies (Fall 2023)

Description: This course is designed for graduate students in statistics or biostatistics interested in the issues underlying the design of interventional studies. General topics include designs for various types of clinical trials, endpoints and control groups, statistical inference in interventional studies, sample size determination, and design considerations for adaptive designs and interventions. Regulatory and ethical issues will also be covered. Students should have a working knowledge of basic biostatistical principles and familiarity with the R statistical programming language. (0.5 course unit, fall semester)

Prerequisites: Permission of instructor

Recommended Texts: Both are available as e-books through the Penn library

1. **FFD**: Friedman LM, Furberg CD, DeMets DL, et al. *Fundamentals of Clinical Trials 5th edition*. Cham: Springer International Publishing. 2015. DOI: 10.1007/978-3-319-18539-2.

2. **STCT** Proschan MA. *Statistical Thinking in Clinical Trials*. Chapman and Hall/CRC, 2021.

Also recommended: **Piantadosi S. Clinical Trials: A Methodologic Perspective. Second edition. Wiley, 2005*

Not all references to readings are complete. More detail will be provided during the semester.

Goals:

1. Provide introductory information about issues involved in designing and analyzing a interventional study, particularly in human subjects.
2. Introduce quantitative problems in clinical trials with the goal of having students 'think statistically' about these problems.
3. Have students work effectively in small groups. Clinical trials are highly collaborative; a good trialist is adept at communicating with colleagues and developing solutions to problems.

Assessment:

In-class Group Assignments (45% final grade): Each week will include some time in class to work with other students on problems posed in class. These 'in-class' assignments will be posted prior to each lecture. In-class assignments will require a write-up of your work including any work not completed in class. Most weeks will have two assignments corresponding to the two class meetings. Each group of students will submit their two in-class assignments by noon on Monday of the following week. The in-class assignments will be graded for participation and completion (thought given to responding carefully to each problem) rather than 'correctness' of content. Please do these assignments carefully as they are key to your understanding of the course content.

Please ask your instructor for help with understanding problems.

While you may use your laptop or phone to get background information about a question, please don't use ChatGPT or other AI to get answers to questions, at least initially in class. Getting a Chat GPT

answer will interfere with development of your own critical thinking skills. In addition, CANVAS will use TurnItIn to check for signs that your work is not original.

Attendance: Please try to attend all in-class meetings; if you miss a class, please contact your instructor to determine how to make up the work.

Individual Assignments (20% final grade): On two occasions you will submit individual, rather than grouped responses. Individual assignments will replace one of the group assignments that week. This will allow you to write up your own work. Dates to be announced.

Late Policy: Assignments will be docked 25% for each day late.

Final Exam (35% final grade): The final exam is open-book, closed-electronics and in-class. Questions will be modelled after the assignments.

Meetings: M/W 1:45-3:15 418 Blockley (EXCEPT Monday 11 September 2:00-3:30)

Office Hours: Dr. Putt after class Wednesday (621 Blockley) or Thursdays at 4 PM (Zoom). Please don't hesitate to provide feedback or ask for help!

Teaching Assistant: DH Lee. Office hours to be announced.

Lecture/Meeting	Date	Suggested Readings
1 (Online) Syllabus Topic Review	30 Aug	
2 Ethical Questions in Clinical Trials	6 Sept	FFD Ch. 2
3 Populations, Questions, Endpoints	11 Sept 2:3:30	FFD Ch. 3
4 Study Design	13 Sept	FFD Ch. 5
5 Randomization and Blinding	18 Sept	FFD Ch. 6 STCT Ch. 5
6 Statistical Inference: Randomization Tests	20 Sept	STCT Ch.6
7 Statistical Inference: Precision	25 Sept	STCT Ch. 6
8 Sample Size & Power	27 Sept	STCT Ch.8
9 Multiplicity & Subgroup Analysis	2 Oct	
10 Interim Monitoring	5 Oct	STCT Ch. 10
11 Dr. Devan Mehrotra (Merck & Company) Clinical Trials in Drug Development	9 Oct	
12 Early Phase Studies	11 Oct	
13 Questions, Review	16 Oct	
14 Final Exam (In-class)	18 Oct	