

M21- 617: Study Design and Clinical Trials (Spring, 2020)

Course master/Instructor: Amber Salter, PhD & Chuck Goss, PhD
Office Hours: By appointment
Email: amber@wustl.edu and cwgoss@wustl.edu

Semester & time(s): Second half of spring semester;
two 3-hour lectures per week (03/17/20 - 05/05/20)

Schedule: Tuesdays & Thursdays 1:30-4:30 pm

Place: Room 502, Becker Library

Credits: 3

Objective: The course will focus on statistical and epidemiological concepts of study design and clinical trials. Topics include: different phases of clinical trials, various types of medical studies (observational studies, retrospective studies, cross-over design, factorial design, and group sequential design and power analysis, along with statistical methods for the various types of studies. Study management, randomization method and survey data analysis are also addressed. Students will be expected to write up a proposed design for a study of their choice, and to practice power analysis/sample size estimation during lab sessions. Permission of the Course Master required.

Prerequisites: M21-560 Biostatistics I and M21-570 Biostatistics II or the equivalent as determined by the course masters.

Format: Lecture and computer lab

Grade Criteria:	Attendance	(15%)
	Assignments/Labs	(25%)
	Quizzes	(20%)
	Project	(40%)
	Report (5 pages)	(30%)
	Presentation	(10%)

Grading scale: A: 90-100, B: 80-89, C: 70-79; D: 60-69; F: 59 and below

Textbook:

Required: Cook, Thomas D. DeMets, David L. (eds) Introduction to Statistical Methods for Clinical Trials. Chapman & Hall, 2008 (ISBN 13: 978-1-58488-027-1)

Recommended:

Piantadosi, Steven. Clinical Trials: A Methodologic Perspective, 3rd Edition. Wiley, 2017 (ISBN-13: 978-0-471-72781-1)

Tentative Lecture Schedule: SPR 2020:
Tues/Thurs 1:30pm – 4:30pm

Date	Lecture	Topic	Readings	
3/17	1	Introduction; Defining the question (Part I);	Cook: 1.1-1.3	Salter
3/19	2	Defining the question (Part II); Epidemiology concepts	Cook: Ch 2	Salter
3/24	3	Bias, Reliability, Randomization (lecture) + lab	Cook: Ch 5	Salter
3/26	4	Clinical Trials: Phase I-II	Cook: 1.4-1.9, 3.1	Salter
3/31	5	Clinical Trials: Phase III-IV	Cook: 3.2,	Salter
4/2	6	Crossover design; Factorial design, Noninferiority design	Cook: Ch 6	Goss
4/7	7	1:30 – 3:00 pm: Statistical Concern; Surrogate endpoints; biomarkers; Reporting clinical trials: Part I	Cook: 4.3, 11.2	Goss
		3:00 – 4:30 pm Project discussion		Salter& Miller
4/9	8	1:30 – 3:00 pm: Statistical Concern; Surrogate endpoints; biomarkers; Reporting clinical trials: Part II		Goss
		3:00 – 4:30 pm: Project discussion		Salter& Miller
4/14	9	Data Collection (lecture) & REDCap demonstration + lab	Cook: 3.3	Steger-May
4/16	10	Power: introduction, one-sample and two-sample for continuous outcome, frequency data, correlation (lecture) + lab	Cook: 4.1, 4.2	Chen
4/21	11	Power: equivalence trials, regression, survival, and repeated measures (lecture) + lab	Cook: 4.1, 4.6-4.8	Chen
4/23	12	1:30 – 3:00 pm: Missing Data and Reporting	Cook: 11.3-11.6, Ch 12	Goss
		3:00 – 4:30 pm: Project discussion		Salter& Miller
4/28	13	Survey Data + lab		Lu
4/30	14	Final project Presentations		Salter& Goss
5/5	15	Final project Presentations		Salter& Goss

Assignments

Assignments will be due at the beginning of class on the specified due date.

Project Requirement

1. Groups: 1-2 persons
2. Write an essay/report (Statistical Analysis Plan): 5-page limit
3. Project discussions: Participation in project discussions & brainstorming is required
4. Presentations (team, or individual)

Competencies: Students who complete this course will learn concepts and critical study design issues in medical studies and clinical trials, optimal study designs, power analysis, study management issues, and randomization methods. They will gain skills in study design by writing a study design proposal and increase their knowledge of power analysis via practice sessions in the lab and final project.

1. To teach concepts and critical study design issues in medical studies and clinical trials;
2. To encourage students to read the recent literatures;
3. To introduce study management issues and randomization method;
4. To apply skills by writing a study design proposal;
5. To increase the knowledge of power analysis via practice sessions in the lab and final project;

Class Participation

Readings assigned for each class should be read ahead of the class and students should be prepared to discuss the material from readings. All interactions in class will be civil, respectful, and supportive of an inclusive learning environment for all students. Please speak to Dr. Salter, Dr. Rao, or Ms. Freels about any concerns you may have about classroom participation and classroom dynamics.

Course Policies and Information for Students

INCLUSIVE LEARNING ENVIRONMENT STATEMENT

The best learning environment—whether in the classroom, studio, laboratory, or fieldwork site—is one in which all members feel respected while being productively challenged. At Washington University in St. Louis, we are dedicated to fostering an inclusive atmosphere, in which all participants can contribute, explore, and challenge their own ideas as well as those of others. Every participant has an active responsibility to foster a climate of intellectual stimulation, openness, and respect for diverse perspectives, questions, personal backgrounds, abilities, and experiences, although instructors bear primary responsibility for its maintenance.

A range of resources is available to those who perceive a learning environment as lacking inclusivity, as defined in the preceding paragraph. If possible, we encourage students to speak directly with their instructor about any suggestions or concerns they have regarding a particular instructional space or situation. Alternatively, students may bring concerns to another trusted advisor or administrator (such as an academic advisor, mentor, department chair, or dean). All classroom participants—including faculty, staff, and students—who observe a bias incident affecting a student may also file a report (whether personally or anonymously) utilizing the online Bias Report and Support System.

1. ATTENDANCE POLICY

Class attendance is required. As a courtesy to other students, please arrive on time. More than two unexcused absences from class may result in a lower grade in attendance.

2. PENALTIES FOR LATE WORK and REQUESTS FOR EXTENSIONS

Late assignments may result in a deduction of points for each day late (including weekends) unless prior approval is obtained from the instructor or a compelling situation prevents prior approval (i.e. documented health issues or family emergencies).

3. ETHICS/VIOLATIONS OF ACADEMIC INTEGRITY: Ethical behavior is an essential component of learning and scholarship. Students are expected to understand, and adhere to, the University's academic integrity policy: wustl.edu/policies/undergraduate-academic-integrity.html. Students who violate this policy will be referred to the Academic Integrity Policy Committee. Penalties for violating the policy will be determined by the Academic Integrity Policy committee, and can include failure of the assignment, failure of the course, suspension or expulsion from the University. If you have any doubts about what constitutes a violation of the Academic Integrity policy, or any other issue related to academic integrity, please ask your instructor.

- Always cite your sources when you present ideas and/or language that you have not developed yourself, including material from class lectures and discussions.
- Violation of this policy includes collaborating on assignments where collaboration is not allowed and/or utilizing notes, texts, etc. on any assignment where use of such materials is not allowed.

Resources for Students

1. **DISABILITY RESOURCES:** If you have a disability that requires an accommodation, please speak with instructor and consult the **Disability Resource Center** at Cornerstone (cornerstone.wustl.edu/). Cornerstone staff will determine appropriate accommodations and will work with your instructor to make sure these are available to you.
2. **WRITING ASSISTANCE:** For additional help on your writing, consult the expert staff of **The Writing Center** (writingcenter.wustl.edu) in Olin Library (first floor). It can be enormously helpful to ask someone outside a course to read your essays and to provide feedback on strength of argument, clarity, organization, etc.
3. **THE UNIVERSITY'S PREFERRED NAME POLICY FOR STUDENTS**, with additional resources and information, may be found here: registrar.wustl.edu/student-records/ssn-name-changes/preferred-name-policy/preferred-name-policy-student/.
4. **ACCOMMODATIONS BASED UPON SEXUAL ASSAULT:** The University is committed to offering reasonable academic accommodations to students who are victims of sexual assault. Students are eligible for accommodation regardless of whether they seek criminal or disciplinary action. Depending on the specific nature of the allegation, such measures may include but are not limited to: implementation of a no-contact order, course/classroom assignment changes, and other

academic support services and accommodations. If you need to request such accommodations, please direct your request to Kim Webb (kim_webb@wustl.edu), Director of the Relationship and Sexual Violence Prevention Center. Ms. Webb is a confidential resource; however, requests for accommodations will be shared with the appropriate University administration and faculty. The University will maintain as confidential any accommodations or protective measures provided to an individual student so long as it does not impair the ability to provide such measures.

If a student comes to me to discuss or disclose an instance of sexual assault, sex discrimination, sexual harassment, dating violence, domestic violence or stalking, or if I otherwise observe or become aware of such an allegation, I will keep the information as private as I can, but as a faculty member of Washington University, I am required to immediately report it to my Department Chair or Dean or directly to Ms. Jessica Kennedy, the University's Title IX Coordinator. If you would like to speak with the Title IX Coordinator directly, Ms. Kennedy can be reached at [\(314\) 935-3118](tel:3149353118), jwkennedy@wustl.edu, or by visiting her office in the Women's Building. Additionally, you can report incidents or complaints to Tamara King, Associate Dean for Students and Director of Student Conduct, or by contacting WUPD at [\(314\) 935-5555](tel:3149355555) or your local law enforcement agency.

You can also speak confidentially and learn more about available resources at the Relationship and Sexual Violence Prevention Center by calling [\(314\) 935-8761](tel:3149358761) or visiting the 4th floor of Seigle Hall.

5. **BIAS REPORTING:** The University has a process through which students, faculty, staff and community members who have experienced or witnessed incidents of bias, prejudice or discrimination against a student can report their experiences to the University's Bias Report and Support System (BRSS) team. See: brss.wustl.edu
6. **MENTAL HEALTH:** Mental Health Services' professional staff members work with students to resolve personal and interpersonal difficulties, many of which can affect the academic experience. These include conflicts with or worry about friends or family, concerns about eating or drinking patterns, and feelings of anxiety and depression. See: shs.wustl.edu/MentalHealth

Disclaimer

The instructor reserves the right to make modifications to this information throughout the semester.