

(Version Friday 19<sup>th</sup> January, 2018; 09:21)

## Biostatistics 619: Clinical Trials, Winter 2018 Fridays, 1pm–4pm; M1152, SPH II

Instructor: Philip S. Boonstra, PhD  
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Time: Fridays, 1pm–4pm  
**No class on Mar 2 (Winter Break)**  
Class Meeting Location: M1152, SPH II  
Office Hours: Mondays 12pm-1pm; Tuesdays 3pm-4pm  
Please contact me if you want to meet at a different time

**Course Description:** This course is designed for individuals with a strong quantitative background who are interested in the statistical design and analysis aspects of clinical trials and the interface between statistics and policy in this area.

**Course Materials:** *None required.* Some material covered in lectures will be based on content from:

Optional {  
· Cook, TD and DeMets, DL (Editors) *Introduction to Statistical Methods for Clinical Trials*, 2nd ed. (2007)  
· Friedman, LM, Furberg, CD, and DeMets, DL. *Fundamentals of Clinical Trials*, 5th ed. (2015) **This is available for free electronically via UM's subscription to Springer Link.**  
· Piantadosi, Steven. *Clinical Trials A Methodologic Perspective*, 3rd ed. (2017)  
· Crowley J and Hoering A (Editors) *Handbook of Statistics in Clinical Oncology*, 3rd ed. (2012)

Throughout the semester, I will also make available relevant papers via [Canvas](#).

### Pre-requisite:

- [Biostat601](#) or equivalent, with a grade of B or higher
- [Biostat651](#) or equivalent, with a grade of B or higher

### Course Goals:

**Profession & Science of Public Health–3** Explain the role of quantitative and qualitative methods and sciences in describing and assessing a population’s health

**Profession & Science of Public Health–6** Explain the critical importance of evidence in advancing public health knowledge

**Competencies:**

**Evidence-based Approaches to Public Health–2** Select quantitative and qualitative data collection methods appropriate for a given public health context

**Evidence-based Approaches to Public Health–3** Analyze quantitative and qualitative data using biostatistics, informatics, computer-based programming and software, as appropriate

**Evidence-based Approaches to Public Health–4** Interpret results of data analysis for public health research, policy or practice

**Topics covered:** Phase I (dose-escalation, including algorithmic and model-based designs), Phase II (early stopping, Bayesian designs, biomarkers), Phase III (randomization, including adaptive randomization, interim analyses, sample size, group-sequential designs, non-inferiority trials). Integrated into lectures and homework will be the use of statistical software to simulate and analyze data from clinical trials.

**Grading:** Approximately nine homework assignments (54%); two quizzes (26%); anonymous feedback provided to instruct (10%); participation in lectures, attendance (10%)

**Anonymous Feedback:** I want to be an effective teacher. Each week I will publish a poll on Canvas asking you to rate the week’s lecture (1–5) and give me anonymous feedback. On each quiz will be a question asking if you’ve taken at least one weekly poll (since the beginning of the semester or since the previous quiz); you will receive the full 10% if you answer ‘yes’ each time.

**Software:** I will teach using (and, as best as I can, offer help with) the R language and environment, which is freely available at <http://cran.us.r-project.org/>.

**Diversity, Equity, and Inclusion:**

SPH is committed to creating classroom environments that are supportive of diversity, equity and inclusion

**Academic Conduct:**

The faculty and staff of the School of Public Health believe that the conduct of a student registered or taking courses in the School should be consistent with that of a professional person. Courtesy, honesty, and respect should be shown by students toward faculty members, guest lecturers, administrative support staff, community partners, and fellow students. Similarly, students should expect faculty to treat them fairly, showing respect for their ideas and opinions and striving to help them achieve maximum benefits from their experience in the School.

Student academic misconduct refers to behavior that may include plagiarism, cheating, fabrication, falsification of records or official documents, intentional misuse of equipment or materials (including library materials), and aiding and abetting the perpetration of such acts. Please visit <http://www.rackham.umich.edu/current-students/policies/academic-policies> for the full SPH Code of Academic Integrity and further definition of these terms.

**Student Well-being:**

SPH faculty and staff believe it is important to support the physical and emotional well-being of our students. If you have a physical or mental health issue that is affecting your performance or participation in any course, and/or if you need help connecting with University services, please contact the instructor or the Office of Academic Affairs. Please visit <http://www.sph.umich.edu/students/current/#wellness> for more information.

**Student Accommodations:**

Students should speak with their instructors before or during the first week of classes regarding any special needs. Students can also visit the Office of Academic Affairs for assistance in coordinating communications around accommodations. Students seeking academic accommodations should register with Services for Students with Disabilities (SSD). SSD arranges reasonable and appropriate academic accommodations for students with disabilities. Please visit <https://ssd.umich.edu/topic/our-services> for more information on student accommodations.

Students who expect to miss classes, examinations, or other assignments as a consequence of their religious observance shall be provided with a reasonable alternative opportunity to complete such academic responsibilities. It is the obligation of students to provide faculty with reasonable notice of the dates of religious holidays on which they will be absent. Please visit <http://www.provost.umich.edu/calendar/> for the complete University policy.

**Course Calendar:**

- Overview
- Phase 1: Algorithmic Dose-escalation
- Phase 1: Model-based Dose-escalation
- Phase 1: Advanced Topics
- Phase 2: Gehan, Simon Two-Stage Designs
- Phase 2: Bayesian Designs; Randomization
- Phase 3: Sample Size
- Phase 3: Surrogate Outcomes
- Phase 3: Randomization (Fixed, Adaptive)
- Phase 3: Covariate Adjustment
- Phase 3: Designs (Group Sequential, Factorial, Crossover, Non-inferiority)