SYLLABUS

Course	**Biostatistics 238, Spring 2016**
Meeting Days/Location	Lectures: Tuesday, Thursday 9-10.50am, CHS 41-268 Discussion: Thursday 5-5.50pm, CHS A1-241
Instructor Information	Weng Kee Wong, PhD Department of Biostatistics School of Public Health CHS 51-239B University of California, Los Angeles Phone: (310) 206-9622 Email: wkwong@ucla.edu Office Hours: Mondays 10-11 am or by appointment
Course Description	This class covers introductory material for design and analysis of clinical trials, including adaptive methods for studying early and late randomized trials. This course is designed primarily for students pursuing graduate degrees in biostatistics; it includes statistical theory and uses linear algebra.
Prerequisites	Preferred preparation is Biostatistics 200A, 200B and 200C. Previous coursework in linear algebra is also required.
References	1 Clinical Trials: A Practical Approach by J. S. Pocock. John Wiley, 1983. 2 Fundamentals of Clinical Trials (3rd edition) by L. M. Friedman, C. D. Furberg and D. L. DeMets. Springer 1998. 3 Clinical Trials: A Methodologic Perspective by S. Piantadosi. John Wiley, 2005. 4 Adaptive Design Methods in Clinical Trials by S. C. Chow and M. Chang. Chapman and Hall/CRC, 2007. 5 Radmonization in Clinical Trials: Theory and Practice by W. F. Rosenberger and J. M. Lachin. Wiley. 2002 6 Randomized Controlled Clinical Trials by Matthews, J. N. S. Chapman and Hall.2008 7 Data Management Using Stata: A Practical Handbook by Michael Mitchell. Stata Press, 2010.
Homework Assignments	There will be regular homework assignments that will include a mix of problems, some to be solved by hand and some involving computing and writing computer codes.
Grading Policy	Grades for the course will be determined based on the following weighting:

Homework	25%
Attendance and Discussion	5%
Oral Presentation	30%
Written Project Report	40%

Topics (subject to change)

Topics	**Supplementary material and journal articles (vary)**
Week 1: Tuesday Lecture 1: Describe class structure: organization, expectation and coursework. Overview of clinical trials via a video and some useful websites. (ICH and ongoing trials in the world) Thursday Lecture 2: Scope and objectives of clinical trials, brief intro to epidemiology and various outcome measures with some mathematical details.	
Week 2: Tuesday Lecture 3: Phases of clinical trials - statistical issues and methods for Phase I and Phase 2 trials. Thursday Lecture 4: Design Protocol for a real trial.	

Week 3: Tuesday Lecture 5: Phase II clinical trials: Gehan's two-stage Design, Simon's Two Stage Design. Thursday Lecture 6: Phase III clinical trials: why are clinical trials needed; issues to consider before designing a clinical trial; ethical issues, randomized clinical trials (RCT) and the ITT (intent-to-treat) principle.	
Week 4: Tuesday Lecture 7: Randomization for design-based inference; fixed allocation randomization: simple, permuted block and stratified randomization. Blinding: principles and general considerations. Thursday Lecture 8: Adaptive randomization procedures: Efron biased coin design, urn model, minimization method of Pocock and Simon.	
Week 5: Tuesday Lecture 9: An exemplary analysis of a longitudinal data set using GEE. Thursday Lecture 10: Response adaptive randomization, covariate-adaptive randomization and other mechanics of randomization.	
Week 6: Tuesday Lecture 11: Optimal design theory for randomization purposes. Thursday Lecture 12: Optimal design theory for randomization purposes.	
Week 7: Tuesday Lecture 11: Sample size calculation for various types of clinical trials and outcomes. Thursday Lecture 12: Sample size calculation for various types of clinical trials and outcomes.	
Week 8: Tuesday Lecture 15: Multiplicity issues in clinical trials. Thursday Lecture 16: Multiplicity issues in clinical trials.	
Week 9: Tuesday Lecture 15: Presentations from 2 students. Thursday Lecture 16: Presentations from 2 students.	
Week 10: Tuesday Lecture 15: Presentations from 2 students. Thursday Lecture 16: Presentations from 2 students.	

Learning objectives and competencies

Learning objectives	MS competencies	DrPH competencies	PhD competencies
1. Able to identify different types of discrete data that arise naturally in the health sciences and appreciate their unique features and use in public health research.	A2 Formulate a public health question in statistical terms	B2 Formulate a public health question in statistical terms.	A2 Formulate a public health or scientific question in statistical terms
Able to use the statistical software package STATA to efficiently manage, summarize and analyze data, including all gate-	A3 Identify the strengths and weaknesses of different study designs to address public health and scientific questions; communicate	B1 Collaborate with researchers to formulate the aims of a public health research project. B2 Formulate a public health question in statistical terms.	A1 Collaborate with researchers to formulate the aims of a public health research project. A3 Identify the strengths and weaknesses of different study

keeping activities in an on-going study.	these issues to public health researchers A6 Conduct appropriate statistical analyses of study data and interpret the results. A8 Use statistical software to answer research questions and communicate the results to other research professionals. C4 Develop ability to use new and evolving computational and digital technologies into biostatistical work.	B3 Identify the strengths and weaknesses of different study designs to address public health and scientific questions; communicate these issues to public health researchers. B6 Conduct appropriate statistical analyses of study data and interpret the results. E5 Develop ability to evaluate and incorporate new and evolving computational and digital technologies into biostatistical work.	designs to address public health and scientific questions; communicate these issues to public health researchers. A5 Conduct appropriate statistical analyses of study data and interpret the results. A8 Research biostatistical methods and computational resources for collaborative research. B5 Develop ability to evaluate and incorporate new and evolving computational and digital technologies into biostatistical work.
Able to apply different statistical techniques for analyzing various types of data problem.	A7 Effectively communicate the assumptions and results of analyses through oral and written communications to the collaborative team. B1 Gauge the statistical skill set of an audience to appropriately customize the level of biostatistical presentations. B2 Effectively communicate statistical concepts and reasoning to public health collaborators. B3 Learn to write and disseminate substantive field publications and communicate the statistical portion of the methodology to a substantive field audience. B4 Learn digital tools useful for communication.	B7 Effectively communicate the assumptions and results of analyses through oral and written communications to the collaborative team. C4 Effectively communicate statistical concepts and reasoning to public health collaborators. D2 Write and present effective and clear reports or publications about the application of statistical methods to health problems. D5 Understand and be able to effectively communicate the public health significance of the problems being addressed.	A6 Effectively communicate the assumptions and results of analyses through oral and written communications to the collaborative team. C1 Gauge the statistical skill set of an audience to appropriately customize the level of biostatistical presentations. C2 Effectively communicate statistical concepts and reasoning to public health collaborators. C3 Learn to write and disseminate substantive field publications and communicate the statistical portion of the methodology to a substantive field audience. C4 Learn digital tools useful for communication. D4 Effectively communicate statistical concepts and reasoning to public health collaborators. D6 Learn to write and publish substantive field publications and communicate the statistical portion of the methodology to a substantive field audience. D7 Learn current and future digital tools useful for communication.