BSTA 661: DESIGN OF INTERVENTIONAL STUDIES
INSTRUCTOR: ALISA J. STEPHENS-SHIELDS, PHD, FALL SEMESTER 2021
MONDAYS AND WEDNESDAYS, 1:45-3:15 PM, BLOCKLEY 418

SYLLABUS:

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 (Phase I, II, III), 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 a statistical programming language (e.g. R, SAS). (0.5 course unit, second half of fall semester)

Prerequisites: Permission of instructor

Recommended Text: **Piantadosi S. Clinical Trials: A Methodologic Perspective. Second edition. Wiley, 2005.* Additional readings will be taken from the classic and current literature, including textbooks and journal articles

Supplementary Texts: See next page (optional, students may find helpful for additional reading)

Grading: There will be two homework assignments and a final project. Grading will be based on the homework (60%), final project (20%), and class participation (20%).

Date	Lecture	Topic	Reading	Assignments
September 1	Lecture 1	Introduction to Interventional Studies	6.1-6.3,3.1-3.4	
September 8	Lecture 2	Phase I and Phase II Clinical Trials	10.2,10.3.1-2 10.4.1-5	HW 1 Assigned
September 13	Lecture 3	Control Groups and Mechanics of Treatment Assignment	13	
September 15	Lecture 4	Endpoints in Interventional Studies	8.3-8.7	
September 20	Lecture 5	Hypothesis Testing and Effect Estimation	16.2-16.4	HW 1 Due/HW2 Assigned
September 22	Lecture 6	Estimands in Clinical Trials		
September 27	Lecture 7	Sample Size and Power for Measured Outcomes	11.1-11.5	
September 29	Lecture 8	Experimental Designs	19.1-19.5, 20.1-20.2	HW 2 Due / HW3 Assigned
October 4	Lecture 9	Pragmatic Trials and Other Trial Types		
October 6	Lecture 10	Monitoring and Group Sequential Designs	14.1-14.4	
October 11	Lecture 11	Adaptive Designs		HW 3 Due
October 13	Lecture 12	SMARTs for Adaptive Interventions	Almirall et al. (2011)	
October 18	Lecture 13	Project Presentations		

Texts Recommended for Further Reading on Clinical Trials

Highly-recommended:

- Cook TD, DeMets DL. Introduction to Statistical Methods for Clinical Trials. First edition. Chapman and Hall/CRC, 2007.
- Friedman LM, Furberg CD, DeMets DL. Fundamentals of Clinical Trials, Fourth edition, Springer, 2010.
- Senn S. Statistical Issues in Drug Development. Second edition. Wiley, 2007.

Other Texts on Clinical Trials in General:

- Chow S-C. Controversial Statistical Issues in Clinical Trials. First edition. Chapman and Hall/CRC, 2011.
- Chow S-C, Liu J-P. Design and Analysis of Clinical Trials: Concepts and Methodologies. Second Edition. Wiley, 2004.
- Evans S, Ting N. Fundamental Concepts for Clinical Trial Statisticians. First edition. Chapman and Hall/CRC, 2011.
- Meinert CL. An Insider's Guide to Clinical Trials. First edition. Oxford University Press, 2011.
- Meinert CL. Clinical Trials: Design, Conduct and Analysis. Second edition. Oxford University Press, 2012.
- Peace KE, Chen D-G. Clinical Trial Methodology. First edition. Chapman and Hall/CRC; 2010.
- Pocock SJ. Clinical Trials: A Practical Approach. First edition. Wiley, 1984.
- Redmond CK, Colton T. Biostatistics in Clinical Trials. First edition. Wiley, 2001.
- Spilker WA. Guide to Clinical Trials. First edition. Raven Press, 1991.

Specialized Trial Designs:

- Cayen MN. Early Drug Development: Strategies and Routes to First-in-Human Trials. First edition. Wiley, 2010.
- Crowley J, Green S, Benedetti J, Smith A. Clinical Trials in Oncology. Second Edition. Chapman and Hall/CRC, 2002.
- Hayes RJ, Moulton LH. Cluster Randomised Trials. First edition. Chapman and Hall/CRC, 2009.
- Julious S, Tan S-B, Machin D. An Introduction to Statistics in Early Phase Trials. First edition. Wiley, 2010.
- Rosenberger WF,Lachin JM. Randomization in Clinical Trials: Theory and Practice. First edition. Wiley-Interscience, 2002.
- Senn SS. Cross-over Trials in Clinical Research. Second edition. Wiley, 2002.

Sequential/Adaptive Methods and Monitoring:

- Berry SM, Carlin BP, Lee JJ, Muller P. Bayesian Adaptive Methods for Clinical Trials. First edition. Chapman & Hall/CRC, 2010.
- Ellenberg SS, Fleming TR, DeMets DL. Data Monitoring Committees in Clinical Trials: A Practical Perspective. First edition. Wiley, 2002.
- Herson J. Data and Safety Monitoring Committees in Clinical Trials. First edition. Chapman and Hall/CRC, 2009.
- *Jennison C, Turnbull BW. Group Sequential Tests with Applications to Clinical Trials. First edition. Chapman and Hall/CRC, 2000.*
- Proschan MA, Lan KKG, Wittes JT. Statistical Monitoring of Clinical Trials: A Unified Approach. First edition. Springer, 2010.
- Whitehead J. The Design and Analysis of Sequential Clinical Trials. Second edition. Wiley, 1996.

Related Topics:

- Chen D, Peace KE. Clinical Trial Data Analysis Using R. First edition. Chapman & Hall/CRC, 2010.
- Dmitrienko A,Molenberghs G, Offen W, Chuang-Stein C. Analysis of Clinical Trials Using SAS: A Practical Guide. First edition. SAS Publishing, 2005.
- McFadden E. Management of Data in Clinical Trials. Second edition. Wiley-Interscience, 2007.