

BSTA 661: DESIGN OF INTERVENTIONAL STUDIES
INSTRUCTOR: ALISA J. STEPHENS-SHIELDS, PHD, FALL SEMESTER 2019
TUESDAYS AND THURSDAYS, 1:30-2:50 PM, BIOMEDICAL RESEARCH BUILDING II/III 253

SYLLABUS: SEPTEMBER 26, 2019

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

Required Texts: 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 homeworks and one final exam. Grading will be based on the homework (40%), the final exam (40%), and class participation (20%).

Date	Lecture	Topic
October 22	Lecture 1	Introduction to Interventional Studies
October 24	Lecture 2	Phase I Clinical Trials and Pharmacokinetics
October 29	Lecture 3	Phase II Clinical Trials
October 31	Lecture 4	Control Groups and Mechanics of Treatment Assignment
November 5	Lecture 5	Endpoints in Interventional Studies
November 7	Lecture 6	Hypothesis Testing and Effect Estimation
November 12	Lecture 7	Experimental Designs
November 14	Lecture 8	Sample Size and Power for Measured Outcomes
November 19	Lecture 9	Monitoring and Group Sequential Designs
November 21	Lecture 10	Adaptive Designs
November 26	Lecture 11	SMARTs for Adaptive Interventions
December 3	Lecture 12	Statistical Analysis Plans and Reproducible Research
December 5	Lecture 13	Final Exam

Texts Recommended for Further Reading on Clinical Trials

Highly-recommended:

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