BIOS 752 Design and Analysis of Clinical Trials

Instructors:

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Lectures: Tuesday and Thursday, 12:30 PM to 1:45 PM

Help session: Monday 3:00 PM to 4:00 PM

Assessment:
1) MIDTERM EXAM 1 (worth 35% of final grade);
2) FINAL EXAM (worth 35% of final grade);
3) HOMEWORK (worth 30% of final grade). Collaboration by students on homework assignments is allowed. However, copying or simply dividing up assignments among collaborating students is not allowed. Questions concerning these homework assignments can be addressed to the course instructor.

Recommended texts: Matthews, J. N. S. (2006). *Introduction to randomized controlled clinical trials*. Chapman and Hall.
ISBN 978-1-58488-624-2
Proschan, M.M., Lan, G. and Wittes, J.T. (2006) *Statistical monitoring of clinical trials: a unified approa*ch. Springer**.**

Course prerequisite: BIOS 660; BIOS 661 or equivalent.

Lecture 1   Brief History of Clinical Trials. Phases of Clinical Trials.

Lecture 2   Phase I trials in non life-threatening diseases.

Lecture 3   Phase I trials.

Lecture 4   Phase I trials in life-threatening diseases. CRM.

Lecture 5   Phase I trials in life-threatening diseases.

Lecture 6   Phase II A trials.

Lecture 7   Phase II Clinical Trials in Oncology, exact CI.

Lecture 8   Phase II Oncology: Gehan's Two-Stage Design. Simon's Design.

Lecture 9   Phase II Oncology: toxicity monitoring.

              Randomization: Simple, Permuted block.

Lecture 10   Randomization: Practical aspects. Stratification and minimization.

Lecture 11   Randomization: Response adaptive randomization. Design-based inference.

Lecture 12   Multicenter trials. Trial coordination and QA/QC.

Lecture 13   Randomization.

Lecture 14   Sample Size Calculations.

Lecture 15   General issues in Phase III trials. ICH E9.

Lecture 16   Intent-to-treat, LOCF, missing data imputation.

Lecture 17   Intent-to-treat, LOCF, missing data imputation.

Lecture 18   Crossover studies.

Lecture 19   Crossover studies.

Lecture 20   **Midterm**

Lecture 21   Multiplicity (due to multiple endpoints, doses, subgroups).

Lecture 22   Multiplicity.

Lecture 23   Covariance Analysis, parametric and non-parametric methods.

Lecture 24   Early Stopping.

Lecture 25   Early Stopping.

Lecture 26   Early Stopping.

Lecture 27   Equivalency trials, non-inferiority trials.

Lecture 28   Bayesian methods.

Lecture 29   Review.