Syllabus for BIST 0660 Spring 2013
‘Introduction to Clinical Trials: Design and Analysis of Medical Experiments’ (Concepts and Methods)

Instructors:
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Office Hours - by Appointment

Prerequisites:
PHCO 0504 (Introduction to Biostatistics), BIST 0654 (Biostatistical Computing I). BIST 0661 (Regression Analysis). Knowledge in Regression methods very essential.

■ A self-study review homework will be assigned at the first class (today)

Overview: This is an introductory level course on clinical trials. The focus is on the concepts, processes, and certain statistical methods in the design, conduct, analyses, and reporting of clinical trials. Some statistical methods and medical principles will be given. Students are expected to participate in group discussions during the class.

After completing this course, the students should be able to
- participate in a clinical trial team in either an academic or an industrial setting,
- function as a team member and contribute in the understanding and development of a trial protocol,
- follow up the design and conduct, as well as assist in basic statistical analyses of efficacy and safety data,
- apply the knowledge learned in this course to read published clinical trial papers critically.

TEXTBOOKS
Required:
- Friedman, Furberg and DeMets: Fundamentals of Clinical Trials
  Springer-Verlag, New York, NY. (ISBN#0-8151-3356-1)

(Optional References):
- Cook and DeMets: Introduction to Statistical Methods for Clinical Trials
- C. L. Meinert: Clinical Trials: Design, Conduct and Analysis
- “Practical Statistics For Medical Research” By D. G. Altman, Chapman & Hall/CRC
  1997 (ISBN # 0-412-27630-5)
- “The Design and Analysis of Clinical Experiments” By J. L. Fleiss, Wiley & Sons
  1986 (ISBN # 0-471-82047-4)
- “Clinical Trials: A Methodologic Perspective” By Steven Piantadosi, Wiley & Sons
  1997 (ISBN # 0-471-16393-7)

In addition, complementary materials will be handed out in the class.

Journals: Controlled Clinical Trials; Statistics in Medicine; BMJ; Biometrics; Statistical Methods in Medical Research; DIA Journal

Useful Web Sites:

http://www.umdnj.edu/riteweb/
http://www.fda.gov/cder/
http://www.fda.gov/cder/about/history/

Grading:

Attendance and participation: 15%
Home work assignment: 20%
Quiz: 15%
Mid-term: 20%
Final: 30%
Possible Topics: (But only selected few can be covered in this course, not necessarily follow closely the following sequence either – for some good reason)

1. Overview:
   • Rationale and justification of clinical trials
   • Types of clinical trials: by phase, by design, by disease, by organization.
   • Several key issues in clinical trials: ethics, informed consent,
   • Key concepts: Validity, bias, and generalization of results.

2. Planning clinical trials:
   • Protocol – structure, writing and review board.
   • Sample size, endpoint and designs.
   • Recruitment of investigators, trial center organization, role of statisticians.
   • Enrollment of patients.
   • Randomization and blinding.
   • Case Report Form design.

3. Conducting and monitoring clinical trials.
   • Data collection, quality assurance, auditing.
   • Patient’s compliance of protocol issues
   • Reasons for monitoring; danger of over-monitoring
   • Interim analyses – type/purpose of interim analyses, various sequential methods, issues and DSMB, Adaptive designs.

4. Statistical Methods
   • For diagnosis
   • For evaluating reliability of instruments
   • Baseline data
   • Covariates
   • Subgroups
   • Continuous and categorical
   • Use of cut-points
   • Multiplicity
   • Repeated measures
   • Survival data
   • Meta-Analysis

5. Report writing, graphics, publications – review/evaluation/comparison of literature