Math 654: Design and Analysis of Clinical Trials

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General Information

- Introduction
- Go through the syllabus and other important information about the course
- Big picture of the course
- Office: 210B Cullimore Hall
- Contact information: most convenient way is email
- Office hours: Th 1:30 pm-3:30 pm, or by appointment

Textbook

- **Textbook:** Alex Dmitrienko, Ajit C. Tamhane, and Frank Bretz (2010). *Multiple Testing Problems in Pharmaceutical Statistics,* Chapman & Hall.
- **Other references**
  - ICH Guidances E4, E8, E9, and E10.
  - Thomas Cook and David DeMets (2008). Introduction to Statistical Methods for Clinical Trials, Chapman & Hall/CRC.
  - Lawrence Friedman, Curt Furberg, and David DeMets (1999). *Fundamentals of Clinical Trials,* Springer.
  - Stefan Wellek (2010). *Testing Statistical Hypotheses of Equivalence and Noninferiority,* CRC Press.
  - Michael Proschan, Gordan Lan, and Janet Wittes (2010). *Statistical Monitoring of Clinical trials,* Springer.
  - Frank Bretz, Torsten Hotheorn, and Peter Westfall (2010). *Multiple Cpomparisons using R,* CRC Press.

Email List

- Email list for the class, to deliver syllabus, lecture slides/notes, homework/solutions, exam/solutions and some important notifications
- Computing: We will freely use **SAS** and **R**.
- Will talk more about computing when we need it

Big Picture of the Course

- Part I: Introduction to Clinical Trials
- Part II:
  - Phase I – III Clinical Trials
  - Randomization
  - Sample Size Calculation
  - Early Stoping of Clinical Trials
- Part III:
  - General Multiple Comparisons Methodology
  - Analysis of Multiple Endpoints
  - Gatekeeping Strategy in Clinical Trials
- Midterm (Parts I – II)
- Paper Presentation
- Research Project (Parts I – III)

Course Evaluation

- Letter grade will be given based on Homework(20%)+ Paper Presentation(15%) + Midterm(30%)+ Project(35%)
- Homework: bi-weekly. 5 homeworks in total.
- Midterm: two hours, in-class, open book/notes. Tentatively scheduled at 3/11
- Paper presentation: Tentatively scheduled in the last half hour of each class
- Project presentation: Tentatively scheduled at 5/6
- No Final Exam.

Paper Presentation

- Form a group of two members
- For each group, I will send you one paper.
- Read the paper carefully and present it in front of the class
- For other groups, also read the paper carefully and prepare one or two questions for the presenters.

Project

- Find a research question relevant to clinical trials yourself
- Interesting and complicated to some extent
- Use the statistical methods you have learnt in this class to analyze it
- Demonstrate the performance of your proposed method through real data analysis
- Write a report, 8-10 pages
- Give a presentation in front of the class

Any questions/comments?