Biostatistics in Medical Product Regulation 140.633.81
Final Project

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1 Introduction

1. According to the FDA, guidance documents issued by the FDA "represent FDA's current thinking on a topic." Although these guidance documents "do not create or confer any rights for or on any person and do not operate to bind FDA or the public" and "one can use an alternative approach if the approach satisfies the requirements of the applicable statutes and regulations", they are without question valuable resources for understanding the regulation decision-making process.\(^1\)

2. Four FDA statistics-related guidance documents have been selected for this class to review. All four guidance documents can be found in the online library of this course.

3. As the final course project, students are required to formulate groups, select one of the four guidance documents to review, and make presentation to share their findings with the class.

2 Selected Guidance Documents for Each Group

2.1 Group 1: Adaptive Design Clinical Trials for Drugs and Biologics\(^2\)

1. This is a draft guidance issued by Center for Drug Evaluation and Research (CDER) and Center for Biologics Evaluation and Research (CBER) in 2018.
2. The document is 36 pages long.
3. A group of up to 4 students will review this document.
4. The final presentation should have 25-30 slides and be presented in 20-25 minutes.
5. The group will have 5 minutes for questions from class.

\(^1\)https://www.fda.gov/industry/fda-basics-industry/guidances
\(^2\)https://www.fda.gov/regulatory-information/search-fda-guidance-documents/adaptive-design-clinical-trials-drugs-and-biologics
2.2 Group 2: Non-Inferiority Clinical Trials to Establish Effectiveness Guidance for Industry

1. This is a guidance issued by CDER and CBER in 2016.
2. The document is 56 pages long.
3. A group of up to 6 students will review this document.
4. The final presentation should have 30-35 slides and be presented in 25-30 minutes.
5. The group will have 5 minutes for questions from class.

2.3 Group 3: Reporting Results from Studies Evaluating Diagnostic Tests

1. This is a guidance issued by Center for Devices and Radiological Health (CDRH) in 2007.
2. The document is 39 pages long.
3. A group of up to 4 students will review this document.
4. The final presentation should have 25-30 slides and be presented in 20-25 minutes.
5. The group will have 5 minutes for questions from class.

2.4 Group 4: Multiple Endpoints in Clinical Trials

1. This is a draft guidance issued by CDER and CBER in 2017.
2. The document is 54 pages long.
3. A group of up to 6 students will review this document.
4. The final presentation should have 30-35 slides and be presented in 25-30 minutes.
5. The group will have 5 minutes for questions from class.

3 Milestones

1. Tue, Sep 17, 11:59 PM
   • Deadline for students to formulate groups
   • Discussion forum (Final Project > Group Set Up) recommended for formulating groups
   • Each group should email their list of members to the instructor
   • The instructor may assign a student's group when necessary

2. Mon, Oct 7, 11:59 PM
   • Documents need to be submitted to the instructor to show progress

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4 Review and Presentation Instruction

The goal of the review is to understand the FDA perspectives on specific statistics topics. The presentation should provide both the big picture and sufficient details about the guidance document.

Please consider the following questions in your review and presentation:

• Why did the FDA issue the guidance document?
  – new technology?
  – new statistical methodology?

• Does the guidance introduce specific concepts or terminologies?
  – statistical concepts?
  – clinical trial concepts?
  – specific definitions?

• What statistical concerns does the guidance document try to address?
  – design issues?
  – analysis issues?
  – data issues?

• What examples does the guidance provide to illustrate its major points?

• Discuss points?
5 Final Project Grade

| Points |
|------------------------|-----|
| Group set up in time | 5   |
| Documents showing progress | 10 |
| Final presentation points from instructor | |
| Contents of slides | 20 |
| Presentation | 25 |
| Demonstration of team work | 10 |
| Time management | 5   |
| Answering questions | 5   |
| Final presentation points from class | |
| Group member intra-evaluation | 10 |
| Class evaluation | 10 |
| Total | 100 |

Bonus Point

Inclusion of external relevant information 10

6 Misc

- Topics have been created in the Discussion Forum for each guidance group. Try to use it for exchanging ideas within the group and with the instructor.