STAT 854. Biometrics: Methods in Biomedical Studies

X. Joan Hu

Department of Statistics and Actuarial Science
Simon Fraser University

Spring 2023

What to do today (Thursday Jan 5, 2023)?

1. Course Syllabus

2. Introduction

STAT-854. Biometrics: Methods in Biomedical Studies

Instructor: X. Joan Hu (Tel: 778-782-6714)

Lecture: TuTh 16:30-18:20 PT; WMC2268

- ▶ An outline of each lecture will be posted in the course's webpage and canvas-page before the lecture time.

Office Hour: Tue 13:30-14:20 (PT) or by appointment.

Computer Software: *R* and *SAS* are recommended; *R* (URL http://www.r-project.org/) will be used in class.

STAT-854. Biometrics

Textbooks (recommended):

- *Epidemiologic Methods: Studying the Occurrence of Illness*, by TD Koepsell and N.S. Weiss, Oxford University Press, 2003
- *Fundamentals of Clinical Trials*, by LM Friedman, CD Furberg, and DL DeMets, Springer, 2010

Reference Books:

- *Epidemiology* (fourth edition), by L Gordis
- *Clinical Trials: A Methodologic Perspective* (second edition), by S Piantadosi
- More to provide as the course progresses.

STAT-854. Biometrics

COURSE OUTLINE

Part I – Introduction
- ▶ I.1 General Introduction
- ▶ I.2 A Classic Epidemic Example

Part II - Epidemiologic Concepts and Designs
- ▶ II.1 Epidemiologic view of diseases and populations
- ▶ II.2 Measuring disease frequency in population
- ▶ II.3 Design of medical studies: cohort and case-control studies; controlled clinical experiments

STAT-854. Biometrics

Part III - Clinical Trials
- ▶ III.1 Clinical trial design principles: bias control, random error control, randomization, blocking, masking
- ▶ III.2 Types of clinical trials: phases of trial, etc.
- ▶ III.3 Study monitoring: sequential methods.

Part IV - More Advanced Analytic Approaches
- ▶ IV.1 Models and analysis in prospective and retrospective studies
- ▶ IV.2 Important issues: confounding, causal inference, and missing data
- ▶ IV.3 More selected topics: survival analysis, longitudinal analysis, ...

COURSE EVALUATION

Grading Scheme:

- Assignments - 30%; Projects - 70%; Participation - 10%

Elaboration ... ...

- Two homework assignments (15% for each; Weeks 3 and 10)
- Project A (30%; Weeks 5-8)
  - Phase I (10%): to propose for a research project
  - Phase II (20%): to report the project
- Project B (30%; Weeks 12-15)
  - Phase I (10%): to review a research paper
  - Phase II (20%): to present the review (time and place TBA)
- Project C (10%; Week 15)
  - To submit a revision of either the report of Project A or the review for Project B
- Class Participation (bonus upto 10%)

STAT-854. Biometrics

Remarks:

- ▶ Discussions in-class are highly encouraged. The whole class will participate in evaluating the course participation and final presentation.
- ▶ Homework/Project:
    - ▶ No homework/project is accepted after the due time: turn in the available portion, if you cannot complete the whole homework/project in time.
    - ▶ Please turn in your homework/project by uploading it to the relevant canvas page.
    - ▶ Group discussions are encouraged; however, the homework/projects to be evaluated should be independent work.
- ▶ Suggestions for additional (not required) study/practice will be given in class.

Why to study STAT-854. Biometrics?

▶ Many many *biomedical data*: how to analyze them?

▶ How to collect meaningful biomedical data?

$\Longrightarrow$ **STAT-854. Biometrics**

What to study in STAT-854. Biometrics?

(http://www.biometrics.tibs.org/)

The terms "Biometrics" and "Biometry" have been used since early the 20th century to refer to the field of development of statistical and mathematical methods applicable to data analysis problems in the biological sciences.

Examples under the umbrella include designs for/analyses of data from

- ▶ *agricultural field experiments* to compare the yields of different varieties of wheat,
- ▶ *human clinical trials* evaluating the relative effectiveness of competing therapies for disease,
- ▶ *environmental studies* on the effects of air or water pollution on the appearance of human disease in a region or country,
- ▶ and, recently, *identification of individuals* using biological traits, such as those based on retinal or iris scanning, fingerprints, or face recognition.

What to study in STAT-854. Biometrics?

- **Epidemiologic Methods**

- **Clinical Trials**

- **Modern Biostatistical Methods**

Statistical methods for design and analysis in biomedical studies

What is Epidemiology?

- According to Porta (2008), epidemiology is
  "the study of the occurrence and distribution of health related states or events in specified populations, including the study of the determinants influencing such states, and the application of this knowledge to control the health problems."

- Rothman (2012) defines epidemiology as the study of the occurrence of illness.

- Most methods which we will discuss dated from about 1945 - following World War II.

What is A Clinical Trial?

- Friedman et al (2008, Chp1) define a clinical trial as "a prospective study comparing the effect and value of intervention(s) against a control in human beings."

  - experimental vs observational; prospective vs retrospective

  - intervention: a single or combination of diagnositc, preventive, or therapeutic drugs, biologics devices, regimens, or procedures

  - control: the best current standard therapy; if it's not available, no active treatment such as a placebo and no treatment at all.

How to study STAT-854. Biometrics?

- ▶ Master the basic concepts, designs and inference procedures

  - ▶ the basic statistics studied before
  - ▶ the method for the simplest case in a class of problems and then move on

- ▶ Understand the ideas and modelings, know when to use what procedures and how to implement them via R/SAS.

- ▶ Follow the theoretical derivations.

- ▶ If interested, go beyond the required material along its lines.

Don't fall behind.

Part I. Introduction

What is an epidemic?
"the occurrence in a community or region of cases of an illness,
specific health-related behavior, or other health-related events
clearly in excess of normal expectancy" (Porta, 2008)

What to study next?

Part I – Introduction

▶ *I.1 General Introduction*

▶ I.2 An Epidemic Example

Part II - Epidemiologic Concepts and Designs

▶ II.1 Epidemiologic view of diseases and populations

▶ II.2 Measuring disease frequency in population

▶ II.3 Design of medical studies: cohort and case-control studies; controlled clinical experiments