

# **PubH 6400: STATISTICS METHODS FOR TRANSLATIONAL & CLINICAL RESEARCH**



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**COURSE INTRODUCTION & SYLLABUS**

# BIOSTATISTICS

BIOSTATISTICS is the Biomedical Version of the TRIAL BY JURY. It can be defined as “the science of dealing with uncertainties using incomplete information.” Obviously, it is an essential component of Biomedical Research; we have to face uncertainties and, most of the times, we have to rely on incomplete information.

**In that analogy, Trial by Jury versus Biostatistics, the “trial phase” corresponds to “data analysis” and “investigation phase corresponds to study design”; and the amount of evidence is the sample size.**

# INFERENCES & VALIDITIES

- Two major levels of inferences are involved in interpreting the results/findings of a study:
  - ❖ The first level concerns Internal validity; the degree to which the investigator draws the correct conclusions about what actually happened in the study.
  - ❖ The second level concerns External Validity (also referred to as generalizability or inference); the degree to which these conclusions could be appropriately applied to people and events outside the study.

# External Validity

# Internal Validity

Truth in  
The Universe

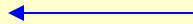
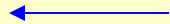
Truth in  
The Study

Findings in  
The Study

Research Question

Study Plan

Study Data



**Validity** is an important concept/component in research. It involves the assessment against accepted absolute standards which are often not available; or in a milder form, to see if the evaluation appears to cover its intended target or targets. **Statistical contributions involve both Internal Validity and External Validity of any research project.**

# STATISTICAL ISSUES

- **Statistics is a way of thinking**, thinking about ways to gather and analyze data.
- The gathering part (i.e. **data collection**) comes before the analyzing part; the first thing a statistician or a learner of statistics does when faced with a biomedical project is data collection (followed by **data management** and **data analysis**).
- Studies may be inconclusive because they were **poorly planned or not enough data** were collected to accomplish the goals and support the hypotheses.

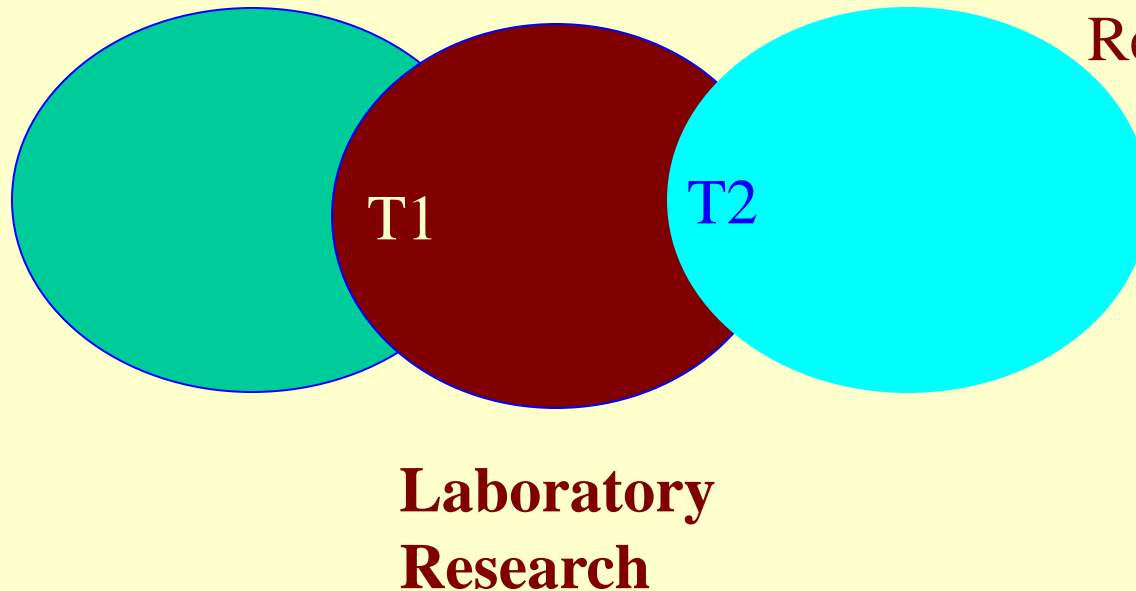
# THE IMPORTANT PHASE

Just as in the case of “Trial by Jury”, the most **important stage** of the “Research Process” is the DESIGN: How & How Much data are collected! Also, It dictates how data should be analyzed. **May be it’s not the question of “how” to collect your data but the decision on “when to do what”!**



Clinical Research

Population  
Research



Studies can be grouped into these areas: Population, Laboratory, and Clinical; plus Translational Research, the component of **basic science** that interacts with clinical (T1) or with population research (T2).

# ABOUT THE COURSE

- **6400: Biostatistics Methods for Translational & Clinical Research**
- **Prerequisites: About a year or two courses of applied statistics**
- **Contents: The course covers some major topics in 4 half-days:**
  - ❖ **Day #1: Diagnostic Medicine,**
  - ❖ **Day #2: Designing Clinical Research,**
  - ❖ **Day #3: Early Phase Clinical Trials, and**
  - ❖ **Day #4: Regression Applications**

# CONTACT INFORMATION

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- **Home Page:  
[www.biostat.umn.edu/~chap](http://www.biostat.umn.edu/~chap)**

# OFFICE HOURS

- **Scheduled office hours: Noon - 1 PM**
- **Some limited other times are available but by appointment.**

# A FEW GOOD BOOKS

There is no textbook; hard to find one that covers all areas. The following are a few good references:

- ❖ “The Statistical Evaluation of Medical Tests for Classification and Prediction” by Margaret Pepe.
- ❖ “Bioassay” by John Hubert, or “Statistical Method in Biological Assay” by David Finney
- ❖ “Clinical Trials in Oncology” by Stephanie Green.

# READING & LEARNING

**There are 9 files are posted on the course's web page; two (2) each for Day #1-3 and three (3) for Day #4. These contain much more materials than the Instructor could cover in class. The first part/files are more basic; the second part/files (Part B) are mostly supplements, read only as much as you can.**

# COURSE EVALUATION

- **Emphasis is on learning, not testing; conducted as one of “seminar courses”; your input are appreciated.**
- **Attendance required – you would fail the course if you miss class more than one day**
- **There will be no class exams. There are 7 “Exercises” (2 in Day1, 2 in Day 2, and 3 in Day 3). These exercises are required for course grading; you have to do at least 6. They are all due on Day (Friday); we’ll go over solution in the very last hour.**

## Problems for **Non-majors**:

- (1) Level of statistics maybe overwhelmed
- (2) Many applications are not in your field or what you think you might need; hard to motivate
- (3) Rather fast pace with not enough details.

Goal: Only to familiarize with the topics; be practical on how much you can learn – and put up with what you feel you might not need



# Problems for **Everybody**:

- (1) No books; reading articles from different sources
- (2) Different learning model; out of “cram & exam taking” mode – and into seminar environment.

Goals: Participation + Discussion + Self learning