Biost 517
Applied Biostatistics I

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The Use of Statistics to Answer Scientific Questions

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Lecture 1:
Course Structure; Overview

September 26, 2007

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Lecture Outline

- Course Structure
- Overview of Setting
  - Scientific method
  - Case study

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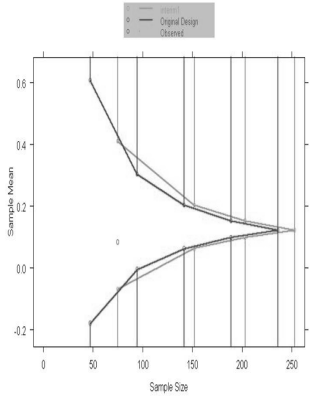
Fair Warning

As You See It



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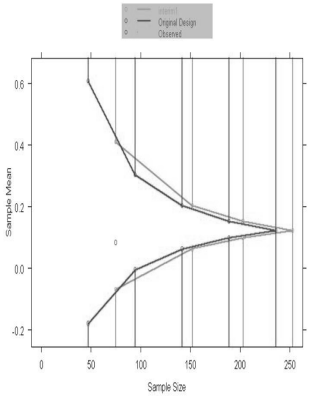
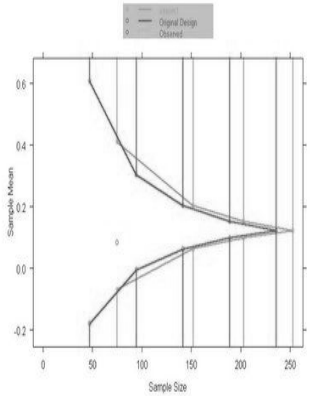
As You See It



As I See It



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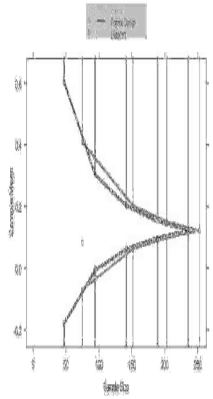
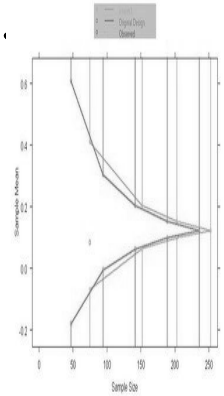
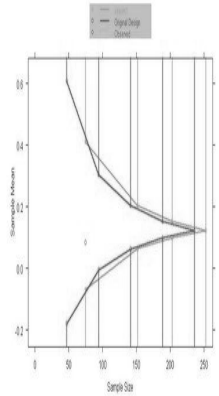
As You See It



Deuteranope



Protanope



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Course Overview

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Course Structure

- Instructor: Scott S. Emerson, M.D., Ph.D.
- TAs: Lin Chen, Daryl Morris
  Rob Wellman
- Time and Place:
  - Lectures: 9:30 - 10:20 am MWF HSB T739
  - Data Analysis:
    - 8:30 - 9:20 am M HSB T530
    - 8:30 - 9:20 am W HSB T473
    - 8:30 - 9:20 am F HSB T531

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Old Dogs, New Tricks

- Recording of Lectures
  - At first: videotape
  - Later: Audio and computer video on web

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Textbook: Rosner

- Fundamentals of Biostatistics (5th ed.)
  - Classical organization
  - Used primarily as a reference
  - (Lectures follow a different organization)

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Computer Software

- Extensively used for data analysis
- Students may use any program that will do what is required, however
  - Stata is used heavily in Biostat 536, 537, 540
  - Help will presume the use of Stata
    - I am conversant in S-Plus (very) and SPSS (enough for this class)
  - Other packages may not compute robust standard errors

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Stata

- Extremely flexible statistical package
  - Interactive
  - Excellent complement of biostatistical methods
- Graphical, report capabilities suboptimal
- Available in microcomputer lab
- Supplementary info on web page
- Syntax introduced in lectures as needed

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Computer Software: Comments

- Designed for people who know statistics, but do not want to write basic functions
  - Tries to be all things to all people
    - Much output that you will not want
    - Much output that I will recommend against

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Guiding Principles

- This is a course in biostatistics, not Stata
  - I will tell you how you can get the statistics I teach you to use
    - There are often multiple ways
  - I will not explain every number that appears on the printout

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Written Homeworks

- Weekly homeworks will involve analysis of real data
  - Questions directed toward specific analyses
    - But questions will still be stated in as scientific terms (as opposed to statistical) terms as possible
  - Work handed in is expected to be organized scientifically
    - I expect nicely formatted tables, figures
    - Unedited Stata output is totally unacceptable

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Errors to Avoid

Unedited Stata output is TOTALLY unacceptable

Errors to Avoid

Any assignments that are handed in should be only your work

Homework Keys

- Keys to the homeworks will be available on the web pages
  - Annotated Stata output will typically be included
  - My answers will typically go beyond what I expected you to do
    - You are responsible for any new information that I provide in the homework keys, even if that information is not otherwise presented in class

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Discussion Section

- Data Analysis Laboratory
  - Data analysis to answer scientific questions
    - You will be given a scientific question and a data set which was collected to try to answer that question
      - Setting is more realistic than that which is given on written homeworks
    - We will discuss the approach to the whole problem
    - Nothing to hand in, but participation in discussion is expected

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Grading

- 25% Homeworks (approx 8)
- 25% One Midterm (in class, closed book)
- 20% Data Analysis and Report
- 30% Final Exam (in class, closed book)

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Course Structure

- Biost 517
  - One response variable; one grouping variable
    - One-, two-, K-sample description and inference
    - Simple regression
  - Stratified description and inference
    - Adjustment for confounding, precision
- Biost 518
  - Multivariable regression

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Biost 517: Topics

- Scientific setting
  - Scientific questions
  - Study structures
  - Statistical role

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Biost 517: Topics

- Descriptive statistics
  - Motivation
  - Types of measurements
  - Univariate summary statistics
  - Univariate depictions of distributions
  - Censored data descriptive statistics
  - Bivariate descriptive statistics

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Biost 517: Topics

- Inferential statistics for two variables
  - Relevant probability
  - Types of statistical inference
    - Bayesian posterior distributions
    - Frequentist sampling distributions
  - Point and interval estimates
  - Hypothesis tests
    - T, chi squared, Fisher's exact, logrank, Wilcoxon
    - Simple regression

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Biost 517: Topics

- Introduction to stratified analyses
  - Confounding, precision, effect modification
  - Descriptive statistics
  - Stratified analyses

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Overview of Setting

Scientific Method

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Purpose of Statistics

- Statistics is about science
  - (Science in the broadest sense of the word)
- Science is about proving things to people
  - (The validity of any proof rests solely on the willingness of the audience to believe it)

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First Stage of Scientific Investigation

- Hypothesis generation
  - Observation
    - Measurement of existing populations
    - Disadvantages:
      - Confounding
      - Limited ability to establish cause and effect

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Further Stages of Scientific Investigation

- Refinement and confirmation of hypotheses
  - Experiment
    - Intervention
    - Elements of experiment
      - Overall goal
      - Specific aims (hypotheses)
      - Materials and methods
      - Collection of data
      - Analysis
      - Interpretation; Refinement of hypotheses

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Do You Need Statistics?

- Two question test (Both must be YES)
  - In a deterministic world, do YOU know how to answer your question?
    - Is the question answerable in the real world?
    - How do you use a number to answer the scientific question?
  - In a world subject to variation, do YOU know how you would answer your question if you had the entire population?

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Ex: Smoking Effect on Lungs

- Association between smoking and lung function in children
  - Long term smoking is associated with lower lung function
  - Are similar effects observed in short term smoking in children?

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Ex: Smoking Effect on FEV

- Scientific question
  - Does smoking lead to lower lung function in kids?
- Study design
  - 654 healthy children
  - Measure smoking by self report
  - Measure lung function by FEV
    - Forced expiratory volume: maximum volume of air that can be exhaled in 1 second

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The Data

SMOKERS

1.953 2.236 3.428 3.208 1.694 3.957 4.789 2.384 3.074 2.387 3.835 2.599 4.756 3.086 4.309 3.413 2.975 3.169 3.343 3.751 2.216 3.078
3.186 3.297 2.304 3.102 2.677 3.297 3.498 2.759 2.953 3.785 2.276 4.637 3.038 3.120 3.339 3.152 3.104 4.045 4.763 3.069 4.506 3.519
3.688 2.679 2.198 3.345 3.082 2.903 3.004 3.406 3.122 3.330 2.608 3.799 4.086 4.070 2.264 4.404 2.278 4.872 4.270 3.727 2.795

NONSMOKERS

1.708 1.724 1.720 1.558 1.895 2.336 1.919 1.415 1.987 1.942 1.602 1.735 2.193 2.118 2.258 1.932 1.472 1.878 2.352 2.604 1.400 1.256
0.839 2.578 2.988 1.404 2.348 1.755 2.980 2.100 1.282 3.000 2.673 2.093 1.612 2.175 2.725 2.071 1.547 2.004 3.135 2.420 1.776 1.931
1.343 2.076 1.624 1.344 1.650 2.732 2.017 2.797 3.556 1.703 1.634 2.570 3.016 2.419 1.569 1.698 2.123 2.481 1.481 1.577 1.940 1.747
2.069 1.631 1.536 2.560 1.962 2.531 2.715 2.457 2.090 1.789 1.858 1.452 3.842 1.719 2.111 1.695 2.211 1.794 1.917 2.144 1.253 2.659
1.580 2.126 3.029 2.964 1.611 2.215 2.388 2.196 1.751 2.165 1.682 1.523 1.292 1.649 2.588 0.796 2.574 1.979 2.354 1.718 1.742 1.603
2.639 1.829 2.084 2.220 1.473 2.341 1.698 1.196 1.872 2.219 2.420 1.827 1.461 1.338 2.090 1.697 1.562 2.040 1.609 2.458 2.650 1.429
1.675 1.947 2.069 1.572 1.348 2.288 1.773 0.791 1.905 2.463 1.431 2.631 3.114 2.135 1.527 2.293 3.042 2.927 2.665 2.301 2.460 2.592
1.750 1.759 1.536 2.259 2.048 2.571 2.046 1.780 1.552 1.953 2.893 1.713 2.851 1.624 2.631 1.819 1.658 2.158 1.789 3.004 2.503 1.933
2.091 2.316 1.704 1.606 1.165 2.102 2.320 2.230 1.716 1.790 1.146 2.187 2.717 1.796 1.335 2.119 1.666 1.826 2.709 2.871 1.092 2.262
2.104 2.166 1.690 2.973 2.145 1.971 2.095 1.697 2.455 1.920 2.164 2.130 2.993 2.529 1.726 2.442 1.102 2.056 1.808 2.305 1.969 1.556
1.072 2.042 1.512 1.423 3.681 1.991 1.897 1.370 1.338 2.016 2.639 1.389 1.612 2.135 2.681 3.223 1.796 2.010 1.523 1.744 2.485 2.335
1.415 2.076 2.435 1.728 2.850 1.844 1.754 1.343 2.303 2.246 2.476 3.239 2.457 2.382 1.640 1.589 2.056 2.226 1.886 2.833 1.715 2.631
2.550 1.912 1.877 1.935 1.539 2.803 2.923 2.358 2.094 1.855 1.535 2.135 1.930 2.182 1.359 2.002 1.699 2.500 2.366 2.069 1.418 2.333
1.514 1.758 2.535 2.564 2.487 1.591 1.624 2.798 1.691 1.999 1.869 1.004 1.427 1.826 2.688 1.657 1.672 2.015 2.371 2.115 2.328 1.495
2.884 2.328 3.381 2.170 3.470 3.058 1.811 2.524 2.642 3.741 4.336 4.842 4.550 2.841 3.166 3.816 2.561 3.654 2.481 2.665 3.203 3.549
3.222 3.111 3.490 3.147 2.520 2.292 2.889 2.246 1.937 2.646 2.957 4.007 2.386 3.251 2.762 3.011 4.305 3.906 3.583 3.236 3.436 3.058
3.007 3.489 2.864 2.819 2.250 4.683 2.352 3.108 3.994 4.393 2.592 3.193 2.346 3.515 2.754 2.720 2.463 2.633 3.048 3.111 3.745 2.094
3.183 3.977 3.354 3.411 3.171 3.887 2.646 2.504 3.587 3.845 2.971 2.891 1.823 2.417 2.175 2.735 4.273 2.976 4.065 2.318 3.596 3.395
2.751 2.673 2.556 2.542 2.608 2.354 1.458 3.795 2.491 3.060 2.545 2.993 3.305 3.774 2.855 2.988 2.498 3.169 2.887 2.704 3.515 3.425
2.287 2.434 2.365 2.696 2.868 2.813 3.255 4.593 4.111 1.916 1.858 3.350 2.901 2.241 4.225 3.223 5.224 4.073 4.080 2.606 4.411 3.791
3.089 2.465 3.200 2.913 4.877 2.358 3.279 2.581 2.347 2.691 2.827 1.873 2.538 2.758 3.050 3.079 2.201 1.858 3.403 3.501 2.578 1.665
2.081 2.974 4.073 4.448 3.984 2.250 2.752 3.680 2.862 3.023 3.681 3.255 3.692 2.356 4.591 3.082 3.258 2.216 3.247 4.324 2.362 2.563
3.206 3.585 4.720 3.331 5.083 2.417 2.364 2.341 3.231 3.078 3.369 3.529 2.866 2.891 3.022 3.127 2.866 2.605 3.056 2.569 2.501 3.320
2.123 3.780 3.847 3.924 2.132 2.752 2.449 3.456 3.073 2.688 3.329 4.271 3.530 2.928 2.689 2.332 2.934 3.110 2.894 2.435 2.838 3.035
4.831 2.812 2.714 3.086 3.519 4.232 2.770 3.341 3.090 2.531 2.822 2.935 2.568 2.387 2.499 4.130 3.001 3.132 3.577 3.222 3.280 2.659
2.822 2.140 4.203 2.997 2.562 3.082 3.806 2.458 2.391 3.141 2.579 2.100 2.785 4.284 2.906 5.102 4.429 4.279 4.500 2.635 3.082 3.387
5.793 3.985 4.220 4.724 3.731 3.500 3.674 5.633 3.645 2.887 3.960 4.299 2.981 4.504 5.638 2.853 3.211