

Biost 517
Applied Biostatistics I
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1

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Biost 514
Biostatistics I
.....

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2

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The Use of Statistics to
Answer Scientific Questions
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3

General Philosophy
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“Everything should be as simple as possible,
but no simpler.”

- A. Einstein (paraphrased)

4

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Lecture 1:
Course Structure; Overview

September 30, 2009

5

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

6

Course Overview
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7

Course Structure
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- Instructor: Scott S. Emerson, M.D., Ph.D.
 - » Fair warning
- TAs: Tanya Granston, Fred Boehm
- Time and Place:
 - Lectures: 9:30 - 10:20 am MWF HSB A420
 - Data Analysis:
 - 8:30 - 9:20 am M HSB T530
 - 8:30 - 9:20 am W HSB K069
 - 8:30 - 9:20 am F HSB A420

8

Assumed Prior Knowledge

- Statistical coursework
 - None
 - (If you have had prior courses, unlearn
 - Need for normal data to test means
 - P value as entire summary of analysis
 - Significance testing to detect confounding
 - Overlapping CI signify not statistically significant
 - Wilcoxon as test of median
 - Fisher's exact test preferred in small samples
 - ...)

9

Old Dogs, New Tricks

- Recording of Lectures: Camtasia
 - Audio and computer video on web
 - Posted approximately 24 hours after class
- No guarantees
 - “Mistakes happen”

10

Textbook: Rosner

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

11

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

12

Stata

- Extremely flexible statistical package
 - Interactive
 - Excellent complement of biostatistical methods
- Graphical, report capabilities suboptimal
- Available in microcomputer lab (v 10)
- Supplementary info on web page
- Syntax introduced in lectures as needed

13

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

14

Guiding Principles

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

15

Written Homeworks

- Weekly homeworks: 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
 - Biost 514: supplemental problems

16

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

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- 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

19

Discussion Section

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- 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

20

Grading

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

21

Course Structure

- Biost 517 / 514
 - 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 / 515
 - Multivariable regression

22

Biost 517 / 514: Topics

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

23

Biost 517 / 514: Topics

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

24

Biost 517 / 514: Topics

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- 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

25

Biost 517 / 514: Topics

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

26

Overview of Setting

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Scientific Method

27

Purpose of Statistics

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- 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)

28

First Stage of Scientific Investigation.....

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

29

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

30

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?

31

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?

32

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33

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

34

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 1.802 1.735 2.193 2.118 2.258 1.932 1.472 1.878 2.352 2.604 1.400 1.256 0.639 2.578 2.988 1.404 2.348 1.755 2.980 2.100 1.283 3.002 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 1.126 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 2.318 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 2.168 1.690 2.973 2.145 1.971 2.095 1.697 2.455 1.920 2.164 2.130 2.993 3.529 1.726 2.442 1.102 2.056 1.808 3.305 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 2.865 3.203 3.549 3.222 3.111 3.490 3.147 2.520 2.232 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 3.056 3.007 3.489 3.894 2.919 2.250 4.683 2.352 3.108 3.904 4.393 2.592 3.193 2.346 3.515 2.754 2.720 2.463 2.633 3.046 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 3.365 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 3.690 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 3.083 4.417 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 4.565 3.073 2.688 3.329 2.271 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 2.859 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