UA College of Medicine-Phoenix

Biostatistics and Study Design Service

Introduction

Basics

- Statistical support is available to UA COM-PHX faculty (any category) for their research projects, contingent upon approval by COM-PHX Research Administration
- Statistical support is available to UA COM-PHX students for their scholarly projects
- There is no charge for unfunded research and grant applications
- Our website

Contact Information

- An intake form is required for each project:
  - Request for Statistical Assistance for faculty members
  - Request for Statistical Assistance for medical students (related to the Scholarly Project)
- E-mail address for the biostatistical support team: COMPHX-Biostat@email.arizona.edu

Team Members

- Janet Foote, PhD, Assistant Professor, Public Health, UA Mel and Enid Zuckerman College of Public Health (MEZCOPH)
- Chengcheng Hu, PhD, Associate Professor, Public Health and Director, Biostatistics, Phoenix Campus, MEZCOPH
- Paul Kang, MPH, Biostatistician, MEZCOPH
- David Margraf, Graduate Research Assistant, MEZCOPH
- Other faculty members from MEZCOPH

Scope

We provide statistical support at all stages of a research project

- Study design
- Study conduct
- After Completion of Study Follow-up/data Collection

Study Design

- Review the research protocol (feasible aims/hypotheses and appropriate outcome measures)
- Identification of a proper experimental design
- Sample size calculation and power analysis
- Data monitoring plan (if needed)
- Statistical analysis plan

Study Design (cont.)

- Randomization procedure (if needed)
- Advice on database design
- Review the data collection forms
- For grant proposals, IRB submissions or Scholarly Project prospectuses, draft the section on statistical considerations outlined above.

Experimental Design

- Prospective study
- Retrospective study
- Cohort study
- Case-control study
- Cross-sectional study/survey
- etc., etc.

Sample Size Determination

- If the primary objective is to test a hypothesis like whether a treatment has a beneficial effect compared to placebo, the sample size calculation is based on power, the chance to identify a treatment effect of a certain level
- If the primary objective is to estimate a parameter, the sample size calculation is based on precision of the estimation

Steps of Hypothesis Testing

1) State the research/alternative hypothesis (e.g. drug has an effect compared to placebo)
2) State the null hypothesis (e.g. no drug effect)
3) Identify a test statistic based on data
4) Calculate p-value: if the null hypothesis is true, the probability of observing a value of the test statistic as extreme as or more extreme than the actually observed value
5) If the p-value is smaller than the pre-determined alpha level or significance level (usually 0.05), reject the null hypothesis and conclude that the alternative hypothesis is true

Power of a Statistical Test

- Power is the probability that an effect of a certain size can be detected in the hypothesis testing procedure
- Larger effect size generally leads to higher power and hence requires smaller sample size
- Power also depends on the variability of the outcome measure for subjects in the target population
- For the same effect size, smaller variability in the outcome measure leads to higher power and hence requires smaller sample size

Effect Size and Variability

- Knowledge about effect size and variability in the target population is needed to determine the sample size
- Such information may be obtained through literature search
- If no prior information is available a pilot study is recommended to acquire such knowledge
- A rough “guesstimate” or a range for the parameters is often sufficient

Effect Size and Variability (cont.)

- The effect size used in power analysis needs to be realistic for the population under study
- The effect size also needs to be “clinically significant”, i.e., a change of that size makes a meaningful difference in the health status of a study participant
- For example, we might have enough power to detect a 3 mmHg difference in SBP under different conditions but that change is well within the range of biological variation

Power Analysis

- Sample size is the minimum number of study participants required to achieve a certain power (at least 80%, preferably 90%) for assumed effect size and level of variability
- After the sample size is determined, if there is uncertainty in the assumed effect size and/or variability, the power needs to be calculated for a number of possible scenarios with each parameter taking different values in a reasonable range

Sample Size for Estimation Problem

- Estimator of any parameter of interest is accompanied by a precision measure of the estimator, like a 95% confidence interval (CI)
- Increasing the sample size will shrink the CI
- When the primary objective is to estimate a parameter, sample size is based on a certain level of precision to be achieved (measured, for example, by width of CI)
- Expected range of the parameter and variability in the outcome measure need to be obtained from literature review or pilot data

Statistical Analysis Plan

- Descriptive analysis: summary statistics, graphical presentation of data
- Comparison of certain parameter across different groups
- Testing and estimation of association between different variables
- Prediction models to make valid projections of an outcome for a particular individual based on a set of variables

Database Management Issues Before Study Starts

- Identify variables to address the aims
- Development of data collection forms
- Design and construction of databases
- Editing, error correction and data quality control procedures
- Procedures for routine operational reporting
- Procedures for producing analytic datasets
- Data security

Statistical/Data Management Issues During Study Conduct

- Routine monitoring
  - Accrual, randomization
  - Data completeness
  - Toxicity monitoring
- Interim analyses
  - Safety
  - Efficacy
  - Futility

Statistical/Data Management Issues After Data Collection is Complete

- Data cleaning, queries
- Data analysis
- Manuscript/report preparation: statistical method section, results section (tables and figures)
- Work with investigators on interpretation of the results and discussion

When to Contact Biostat Support?

- You are encouraged to contact us as early as possible, preferably at the beginning of the design phase
- Prior to submitting your proposal to the funding agency and/or IRB
- Before you pilot your survey / start data collection
- As you are monitoring your study
- As you are preparing to analyze data

Information You Need to Have

- Overall research question
- Target population
- Specific aims
- Outcome measures and other relevant variables
- Approach
- Effect size and variability from the literature/pilot data
- Your timeline

What We Expect from You

- Co-authorship is expected on papers and abstracts for any meaningful intellectual contribution from biostatistician(s)
- Grant proposal: effort of biostatistician(s) to be included if statistical expertise is needed in the grant application and proposed research
- End of project: please notify us of any presentations, posters, papers, reports, or grant submissions resulting from our work.